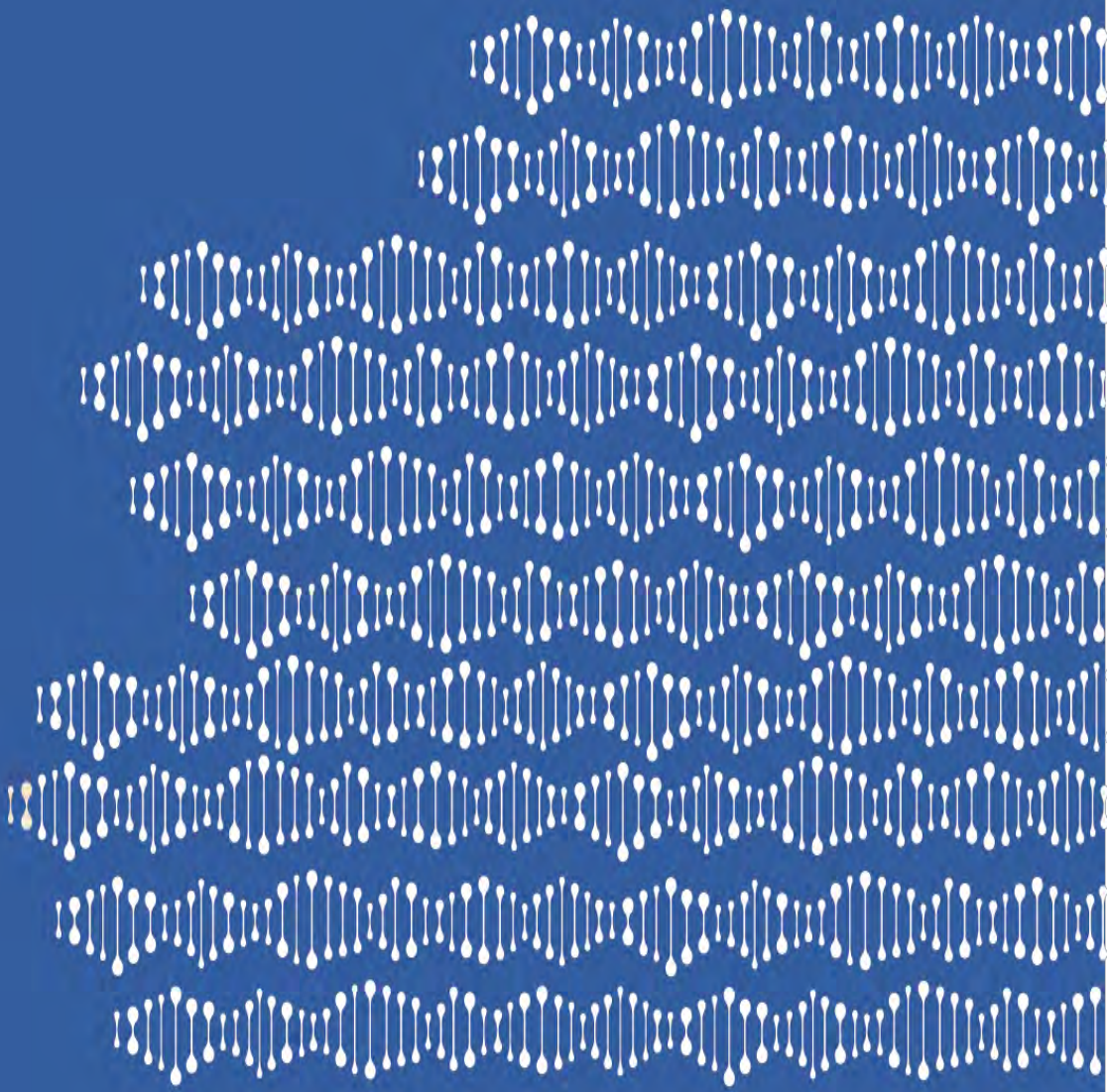
CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

Oversight Committee Meeting

February 15, 2017



CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

Summary Overview of the February 15, 2017, Oversight Committee Meeting

This summary provides an overview of major agenda items and background on key issues for Committee consideration at the February 15, 2017, Oversight Committee meeting.

CEO Report
Wayne Roberts will present the CEO's report and address issues including a personnel update, including a discussion of Governor Abbott's freeze on state agency hiring, action items from the November 16th Oversight Committee meeting, the FY 2016 annual report, a legislative update, and report on FY 2017 grant award funds available. Mr. Roberts will also present his annual report required by Tex. Health & Safety Code § 102.260(c).

Chief Compliance Officer Report
Vince Burgess will report on the status of required grantee reports, financial status report reviews, annual grantee certifications, desk reviews and site visits as well as grantee training and technical assistance.

Chief Scientific Officer Report and Grant Award Recommendations
Dr. James Willson will provide an update on the Academic Research Program and present the Program Integration Committee's five award recommendations for Recruitment of Established Investigators and First-Time, Tenure-Track Faculty recruitment grants totaling $22 million. Dr. Willson will also present the PIC's recommendation increasing the grant award amount previously approved by the Oversight Committee for CPRIT grant RP170259.

CPRIT will not publicly disclose information related to the Academic Research grant applications recommended for funding until the Oversight Committee meeting. The information is available to board members through a secure electronic portal.

Chief Prevention and Communications Officer and Grant Award Recommendations
Dr. Becky Garcia will update the Oversight Committee on the Prevention Program activities as well as an update on the agency's communications activities. She will present the Program Integration Committee's nine award recommendations totaling $12,024,696.

CPRIT will not publicly disclose information related to the Prevention Program grant applications recommended for funding until the Oversight Committee meeting. The information is available to board members through a secure electronic portal.

Chief Product Development Officer Report
Mr. Mike Lang will provide an update on the Product Development Program. He will also present proposed policy changes to the standard revenue sharing terms and matching fund requirements for grantees receiving a second award. Information regarding proposed contract

amendments have been provided under separate cover and will be discussed in closed session with CPRIT's General Counsel.

Appointments - Scientific Research and Prevention Programs Committee and Advisory Committee on Childhood Cancers
The Chief Executive Officer has provisionally appointed two new members to CPRIT's Scientific Research and Prevention Programs Committees. CPRIT's statute requires the Oversight Committee to approve the CEO's recommendations before the appointment is final. Biographical sketches for the appointees are included in the board packet. In addition, Mr. Roberts has provisionally appointed 12 new members of the Advisory Committee on Childhood Cancer.

Internal Auditor Report
Weaver and Tidwell, CPRIT's internal auditor, will provide an internal audit update.

Advisory Committee Reports
Both the University Advisory Committee and Advisory Committee on Childhood Cancers will present their annual reports to the Oversight Committee. The committees' charters require each to provide updates to the Oversight Committee annually.

Amendments to 25 TAC Chapters 701 - 703
Ms. Doyle will present the final order approving two amendments to Chapter 703 that the Oversight Committee provisionally approved at the November meeting. If approved, the amendments will become effective in March.

Ms. Doyle will also present six proposed changes to the agency's administrative rules. Texas Health and Safety Code § 102.108 authorizes the Oversight Committee to implement rules to administer CPRIT's statute. Legal staff will bring back these rule changes to the Oversight Committee for final approval in May after the public has an opportunity to comment on the proposed rule changes.

Chief Operating Officer Report
Heidi McConnell will discuss the operating budget, performance measures, and debt issuance history for the first quarter of FY 2017.

Subcommittee Business
Presiding Officer Geren will present his recommendation for new members of the Prevention Program Subcommittee and for new chairs of the Audit Subcommittee Chair and Prevention Program Subcommittee.

CANCER PREVENTION & RESEARCH INSTITUTE OF TEXAS

Oversight Committee Meeting Agenda

Texas Higher Education Coordinating Board
1200 E. Anderson Lane, Austin, TX 78752
Board Room 1.170

February 15, 2017
11:00 a.m.

The Oversight Committee may discuss or take action regarding any item on this agenda, and as authorized by the Texas Open Meetings Act, Texas Government Code Section 551.001 et seq., may meet in closed session concerning any purpose permitted by the Act. Anyone wishing to offer public comments must notify the Chief Executive Officer in writing prior to the start of the meeting. The Committee may limit the time a member of the public may speak.

1. Call to Order
2. Roll Call/Excused Absences
3. Adoption of Minutes from the November 17, 2016 meeting **TAB 1**
4. Public Comment
5. Grantee Presentations **TAB 2**
6. Chief Executive Officer Report **TAB 3**
   - CEO Report Pursuant to Health & Safety Code § 102.260(c)
7. Chief Compliance Officer Report **TAB 4**
8. Chief Scientific Officer Report and Grant Award Recommendations **TAB 5**
   - RP170259 Contract Amendment
9. Chief Prevention and Communications Officer Report and Grant Award Recommendations **TAB 6**
10. Chief Product Development Officer Report **TAB 7**
    - Standard Revenue Sharing Terms
    - Matching Requirements for Second Awards
    - RP110508/DP160057 Contract Amendment
    - CP120038 Contract Amendment
    - RP101219/DP140067 Contract Amendment
11. Scientific Research and Prevention Program Committee Appointments **TAB 8**
12. Advisory Committee on Childhood Cancers Appointments **TAB 9**
13. Internal Auditor Report **TAB 10**
14. Advisory Committee on Childhood Cancers – Annual Report **TAB 11**
15. University Advisory Committee – Annual Report **TAB 12**
16. Amendments to 25 T.A.C. Chapters 701 and 703 **TAB 13**
    - Final Order Approving Amendments to Chapter 703
    - Proposed Amendments to Chapters 701 and 703 and Authorization to Publish in *Texas Register*
17. Chief Operating Officer Report **TAB 14**
18. Subcommittee Business
19. Compliance Investigation Pursuant to Health & Safety Code § 102.2631
20. Consultation with General Counsel
21. Future Meeting Dates and Agenda Items **TAB 15**
22. Adjourn

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

Oversight Committee Meeting
November 16, 2016

1. Call to Order

A quorum being present, Presiding Officer Geren called the Oversight Committee to order at 10:02 a.m.

2. Roll Call/Excused Absences

Committee Members Present:
Angelos Angelou
Pete Geren
Donald (Dee) Margo
Amy Mitchell
Bill Rice, M.D.
Craig Rosenfeld, M.D.
Ned Holmes
Cynthia Mulrow, M.D.

Committee Members Absent:
Will Montgomery

MOTION:
On a motion made by Mr. Holmes and seconded by Ms. Mitchell, the Oversight Committee unanimously voted to excuse the absence of Mr. Montgomery.

3. Adoption of Minutes from August 17, 2016 and September 14, 2016 meetings

MOTION:
On a motion made by Dr. Mulrow and seconded by Mr. Margo, the Oversight Committee unanimously voted to approve the minutes of the Oversight Committee meetings held on August 17, 2016, and September 14, 2016.

4. Public Comment

Presiding Officer Geren noted there are no requests for public comment.

5. Grantee Presentation

Presiding Officer Geren noted that grantee presentations will become a regular part of CPRIT Oversight Committee meetings. He called on Dr. James Willson, Chief Scientific Officer, to introduce Dr. Thomas Yankeelov.

Dr. Willson introduced CPRIT Grantee Dr. Thomas Yankeelov, Professor at The University of Texas at Austin, Biomedical Engineering Department. Dr. Yankeelov is a CPRIT grantee recruited to The University of Texas as an established investigator in December 2015. He is leader of the Strong Institute's Cancer Research Program and also Director of the Computational Oncology Program in the Texas Institute of Computational Engineering and Science. His research focus is on using computational biology, advanced in vivo imaging, and mathematical modeling. His clinical research has an overall goal of improving patient care by employing advanced imaging methods for the early identification, assessment, and prediction of tumor response to therapy. He develops tumor forecasting methods by integrating advanced imaging technologies with patient-specific data and builds predictive, multi-scale biophysical models of tumor growth with the purpose of optimizing therapies for the individual cancer patient.

Dr. Yankeelov summarized his research to develop tumor forecasting methods by integrating advanced imaging technologies with other patient-specific data to build predictive, multi-scale biophysical models of tumor growth to optimize therapy on a patient basis, and to provide leadership in these areas to the community.

The first clinical trial is a community partnership model and includes The University of Texas at Austin, Seton Hospital, Austin Neurological Associates, and Texas Oncology.

Dr. Yankeelov confirmed that this clinical trial includes all breast cancers. He is planning on conducting a clinical trial on metastatic brain cancer in January 2017. Other cancers will be included as his research develops.

When asked if there were plans to include more data than just that collected by an MRI, Dr. Yankeelov explained that when a MRI PET machine is available they will be able to collect more data without subjecting patients to multiple scans.

6. Chief Executive Officer Report

Wayne Roberts, Chief Executive Officer, introduced Chris Cutrone, CPRIT's new Senior Communications Specialist. Mr. Cutrone's responsibilities will include press releases, editing of documents, and relationships with media. Mr. Cutrone most recently worked at the Texas Health and Human Services Commission, Office of the Inspector General.

Mr. Roberts explained that action items from the August 17 and September 14 meetings and the resulting staff responses were included in the meeting materials. There were no questions.

Mr. Roberts confirmed there will be sufficient FY 2017 funds available for all awards being presented today.

Mr. Roberts reported on bills that have been pre-filed on behalf of CPRIT. Senator Nelson pre-filed SB 81 to address operational issues. Senator Watson filed SB 224 to extend CPRIT's sunset date by two years. Representative Sarah Davis is addressing both operational and sunset issues in HB 63. In addition, she filed HB 84, a companion to sunset bill SB 224. The operational bills address:

- clarification of the requirement that Oversight Committee members file personal financial statements with the Ethics Commission;
- authorization of the Oversight Committee to meet in closed session to discuss management, acquisition or sale of equity, or other revenue sharing discussions and to allow a staff member or a contracted representative to serve on grantee boards when appropriate;
- clarification of the language in the statute requiring CPRIT to allocate 10% of funding to the Prevention Program;
- authorization of the Oversight Committee to transfer management and disposition of CPRIT's royalty obligations and equity ownership to the Texas Treasury Safekeeping Trust Company; and
- elimination of the requirement that the Oversight Committee publicly report political donations.

There were no questions for Mr. Roberts.

7. Chief Scientific Officer Report and Grant Award Recommendations

Dr. James Willson, Chief Scientific Officer, reported that the Academic Research Program funded grants totaling $206 million in FY 2016. Of the total funding, Dr. Willson stated approximately $70 million was spent on the recruitment of outstanding cancer researchers to Texas. Of the 111 Research awards in FY 2016, 85 awards and 88% of the funds awarded addressed Oversight Committee targeted priorities.

Academic Research - Proposed Awards

Dr. Willson reported that 170 applications were received for the most recent Academic Research grant review cycle. Of those 170 applications, the Scientific Review Council (SRC) recommended 57 projects totaling $71,256,343. The Program Integration Committee (PIC) voted to defer 11 of the projects recommended by the SRC to a future FY 2017 meeting, pending sufficient funding. Dr. Willson presented 46 projects totaling $61,223,240 in 8 slates corresponding to the grant mechanisms.

The recommendations presented for the Oversight Committee's consideration were:

- 4 Early Translational Research Awards totaling $3,974,486
- 20 Individual Investigator Research Awards (IIRA) totaling $17,892,210

- 7 IIRA Childhood and Adolescent Cancers totaling $8,035,738
- 3 IIRA Computational Biology totaling $2,634,668
- 5 IIRA Prevention and Early Detection totaling $5,819,500
- 5 Research Training Awards totaling $14,866,638
- 1 Recruitment of Established Investigators totaling $6,000,000
- 1 Recruitment of First-Time, Tenure Track Faculty Members totaling $2,000,000

Dr. Willson also reported on the program priorities addressed by the grant recommendations:

- 35 address a Broad Range Of Innovative, Investigator-Initiated Research Projects
- 7 address Enhance Texas' Research Capacity and Life Sciences Infrastructure
- 10 address Childhood Cancers
- 9 address Prevention and Early Detection
- 5 address Computational Biology and Analytic Methods
- 4 address Rare Or Intractable Cancers
- 8 address Cancers of Importance in Texas (Liver)
- 6 address Disparities

In response to a question about the success rate of Computational Biology applications, Dr. Willson said that applying mathematics, statistics, and computer science to the biological sciences requires interactions between different sciences that have not traditionally collaborated. However, this environment is changing with recruitment of CPRIT Scholars, like Dr. Yankeelov, whose research involves applying computational science to cancer research. CPRIT-sponsored training initiatives are also catalyzing these interactions in Dallas and the Texas Medical Center. Oversight Committee policies have been implemented that may improve success rates in the future.

Compliance Certification

Mr. Vince Burgess, Chief Compliance Officer, presented his certification of the review process for the proposed grant awards recommended to the Oversight Committee at this meeting. He stated he had reviewed the compliance pedigrees for the grant applications submitted to CPRIT for the following mechanisms:

- Recruitment of Established Investigators
- Recruitment of First-Time, Tenure-Track Faculty Members
- Early Translational Research Awards
- Individual Investigator Research Awards
- Individual Investigator Research Awards for Cancer in Children and Adolescents
- Individual Investigator Research Awards for Computational Biology
- Individual Investigator Research Awards for Prevention and Early Detection
- Research Training Awards
- Texas Company Product Development Awards

Mr. Burgess stated that he had conferred with staff at CPRIT and SRA, International (SRA), CPRIT's contracted third-party grants administrator, regarding academic research and product development research awards and studied the supporting grant review documentation, including third-party observer reports for the peer review meetings.

Mr. Burgess noted that Wayne Roberts, the Chief Executive Officer, granted Mr. Michael Lang, the Chief Product Development Officer, a waiver from the general prohibition against communicating with applicants. The Oversight Committee was notified of the waiver on October 28, 2016. The waiver pertains to the two companies recommended for Product Development Research awards and allowed Mr. Lang to discuss with each applicant the possibility of reducing their budgets. Neither applicant was given unfair advantage.

Mr. Burgess reported that he was satisfied that the application review process that resulted in the above mechanisms recommended by the Program Integration Committee (PIC) followed applicable laws and agency administrative rules. He certified the academic research and product development research award recommendations for the Oversight Committee's consideration.

Conflict of Interest Notification

Presiding Officer Geren noted for the record that Mr. Angelou reported a conflict of interest with two applications submitted by The University of Texas at Austin. The applications identified by Mr. Angelou were an Early Translation Research Award and an Individual Investigator – Prevention and Early Detection Award. The Oversight Committee agreed to take up the award recommendations together in one vote, with the exception of the award recommendations for The University of Texas at Austin, so that Mr. Angelou could vote on the recommendations without conflicts.

Academic Research Grant Award Recommendations

App ID	Award Mech.	Application Title	PI	PI Organization	Budget
RP170067	RTA	The Future of Cancer Research: Training Program for Basic and Translational Scientists	Keyomarsi, Khandan	The University of Texas M. D. Anderson Cancer Center	$4,000,000
RP170427	ETRA	Ambient Mass Spectrometry for Preoperative Molecular Diagnosis of Thyroid Fine Needle Aspirate Biopsies	Schiavinato Eberlin, Livia	The University of Texas at Austin	$983,586

App ID	Award Mech.	Application Title	PI	PI Organization	Budget
RP170466	IIRA	Targeting the Inflammatory Cancer Stem Cell Microenvironment of Triple Negative Breast Cancer with Leukocyte-mimetic Nanovesicles	Tasciotti, Ennio	The Methodist Hospital Research Institute	$896,951
RP170233	IIRA	K-ras Spatiotemporal Dynamics: Novel Therapeutic Targets	Hancock, John	The University of Texas Health Science Center at Houston	$900,000
RP170496	IIRA	Targeting a Growth and Survival Pathway in Bone Tumor Cells.	Gregory, Carl	Texas A&M University System Health Science Center	$864,971
RP170314	IIRA	Biodegradable nanoclusters for molecular cancer imaging	Sokolov, Konstantin	The University of Texas M. D. Anderson Cancer Center	899,553
RP170593	RTA	Computational Cancer Biology Training Program	Pettitt, B. Montgomery	The University of Texas Medical Branch at Galveston	$3,999,285
RP170074	IIRACCA	Molecular Epidemiology and Somatic Alterations Driving Acute Lymphoblastic Leukemia in Down Syndrome	Rabin, Karen	Baylor College of Medicine	$1,200,000
RP170401	IIRA	Targeting The Glycolysis Pathway To Overcome Resistance To Cancer Immunotherapy	Hwu, Patrick	The University of Texas M. D. Anderson Cancer Center	$900,000
RP170207	IIRACCA	BBB-penetrating redox-responsive smart drugs and exploiting the MGMT-driven S-phase checkpoint for chemotherapy of childhood brain cancers	Srivenugopal, Kalkunte	Texas Tech University Health Sciences Center	$1,173,149
RP170231	IIRA	Identifying vulnerabilities in mutant p53 driven tumorigenesis	Lozano, Guillermina	The University of Texas M. D. Anderson Cancer Center	$869,197

App ID	Award Mech.	Application Title	PI	PI Organization	Budget
RP170399	IIRA	Elimination of hypoxia sensitizes resistant solid tumors to immunotherapy	Curran, Michael	The University of Texas M. D. Anderson Cancer Center	$899,993
RP170040	IIRA	Exploiting DNA repair defects using intensity modulated proton therapy	Sawakuchi, Gabriel	The University of Texas M. D. Anderson Cancer Center	$899,889
RP170295	IIRAP	Developing Effective Epigenetic Biomarkers to Identify Individuals with High Risk of Cancer	Waterland, Robert	Baylor College of Medicine	$1,052,089
RP170095	IIRAP	Exercise as an aid to smoking cessation in anxiety vulnerable adults	Smits, Jasper	The University of Texas at Austin	$891,623
RP170470	IIRACCA	OCT4/c-MYC axis as a mechanism of resistance to 13-cis retinoic acid in neuroblastoma	Kang, Min	Texas Tech University Health Sciences Center	$1,125,638
RP170146	IIRA	B cell receptor signaling intersects with angiogenesis in diffuse large B cell lymphoma	Aguiar, Ricardo	The University of Texas Health Science Center at San Antonio	$900,000
RP170493	IIRAP	For Our Children: A tailored multi-level intervention for parents and healthcare providers to increase HPV vaccination rates	Fernandez, Maria	The University of Texas Health Science Center at Houston	$1,487,683
RP170245	ETRA	Discovery of antibody-drug conjugates targeting a receptor broadly expressed in solid tumors	Liu, Qingyun	The University of Texas Health Science Center at Houston	$1,000,000
RP170330	IIRA	A novel GRK3-EZH2 regulatory pathway in prostate cancer progression	Li, Wenliang	The University of Texas Health Science Center at Houston	$900,000
RP170250	IIRA	Regulation of 53BP1 by novel 53BP1-binding proteins in DNA repair	Chen, Junjie	The University of Texas M. D. Anderson Cancer Center	$900,000

App ID	Award Mech.	Application Title	PI	PI Organization	Budget
RP170126	IIRA	A Novel Pathway to Reduce BRCA1-Associated Breast Cancer Risk	Hu, Yanfen	The University of Texas Health Science Center at San Antonio	$900,000
RP170114	IIRA	Mechanisms of melanoma metastasis	Morrison, Sean	The University of Texas Southwestern Medical Center	$892,521
RP170537	ETRA	Identification of novel immune targets and neoantigens for development of immunotherapy for breast cancer	Wang, Rongfu	The Methodist Hospital Research Institute	$999,995
RP170508	IIRAP	Structural modeling of peptide-HLA complexes presenting a melanoma-associated antigen for cross-reactivity assessment	Kavraki, Lydia	Rice University	$900,000
RP170510	IIRACCA	Telomere Maintenance Mechanisms in Neuroblastoma	Reynolds, Charles	Texas Tech University Health Sciences Center	$1,058,246
RP170336	IIRA	Preclinical Analyses of NAD Kinase as a Redox Vulnerability for the Treatment of Pancreatic Cancer.	Scott, Kenneth	Baylor College of Medicine	$875,757
RP170066	ETRA	Oncolytic Immunotherapy for Gliomas and Cancer Metastases in the Era of Checkpoint Regulation	Fueyo, Juan	The University of Texas M. D. Anderson Cancer Center	$990,905
RP170382	IIRA	Primary Cilia in Cell Cycle Control and Tumorigenesis	Zhong, Qing	The University of Texas Southwestern Medical Center	$900,000
RP170259	RTA	CPRIT Cancer Prevention Research Training Program	Chang, Shine	The University of Texas M. D. Anderson Cancer Center	$2,071,403
RP170564	IIRA	Super-resolution imaging of tumor angiogenesis in deep tissue with high specificity and sensitivity	Yuan, Baohong	The University of Texas at Arlington	$900,000

App ID	Award Mech.	Application Title	PI	PI Organization	Budget
RP170079	IIRA	Palbociclib synergizes with autophagy inhibitors to induce senescence in breast cancer	Keyomarsi, Khandan	The University of Texas M. D. Anderson Cancer Center	$900,000
RP170301	RTA	Osteopathic Scholars in Cancer Research (OSCR)	Vishwanatha, Jamboor	University of North Texas Health Science Center at Fort Worth	$799,055
RP170071	IIRAP	Genetic Epidemiology and Molecular Basis of Cancer Predisposition in Pediatric Rhabdomyosarcoma	Lupo, Philip	Baylor College of Medicine	$1,488,105
RP170366	IIRA	Optimizing Chemoradiation Strategies by Tumor Metabolism Interrogation	Lai, Stephen	The University of Texas M. D. Anderson Cancer Center	$899,996
RP170317	IIRA	Developing Effective Immunotherapeutic Strategies for Advanced Uveal Melanoma	Woodman, Scott	The University of Texas M. D. Anderson Cancer Center	$899,507
RP170307	IIRA	Biomarker-Based Treatment Of Poor Prognostic Mesenchymal Subtype in Gastric Cancer	Lee, Ju-Seog	The University of Texas M. D. Anderson Cancer Center	$893,875
RP170152	IIRACCA	Targeting the HNF4A and WNT/Beta-catenin pathways in childhood malignant yolk sac tumors.	Amatruda, James	The University of Texas Southwestern Medical Center	$1,169,499
RP170144	IIRACB	Effective Exploitation Of Structural Data For Oncology	Ioerger, Thomas	Texas A&M Engineering Experiment Station	$900,000
RP170169	IIRACCA	High throughput combinatory drug screening for pediatric medulloblastomas with a dysregulated EZH2 pathway	Li, Xiao-Nan	Baylor College of Medicine	$1,198,726
RP170488	IIRACCA	Mechanisms of Notch Dysregulation in Pediatric Osteosarcoma	Lee, Brendan	Baylor College of Medicine	$1,110,480

App ID	Award Mech.	Application Title	PI	PI Organization	Budget
RP170387	IIRACB	Development and Validation of a Network-guided, Multi-objective Optimization Model for Cancer Data Analysis.	Liu, Zhandong	Baylor College of Medicine	$889,679
RP170170	IIRACB	Prediction of nuclear export signals in proteins	Grishin, Nick	The University of Texas Southwestern Medical Center	$844,989
RP170345	RTA	UTHSCSA Cancer Research Training Program	Oyajobi, Babatunde	The University of Texas Health Science Center at San Antonio	$3,996,895

ETRA: Early Translational Research Awards
IIRA: Individual Investigated Research Awards
IIRACCA: Individual Investigated Research Awards for Childhood and Adolescent Cancers
IIRACB: Individual Investigated Research Awards for Computational Biology
IIRAP: Individual Investigated Research Awards for Prevention and Early Detection
RTA: Research Training Awards

*RP170259 – Research Training Award: SRC recommended the following budget reductions: Reduce number of trainees from 9 to 6 Post-Doctoral trainees per year; reduce funding for training program manager to 0.5 FTE (from proposed 1.0 FTE). The award amount in this table reflects these changes.

Academic Research Recruitment Grant Award Recommendations

App ID	Candidate	Mechanism	Organization	Budget
RR170005	Maura Gillison	REI	The University of Texas M.D. Anderson Cancer Center	$6,000,000
RR170003	Srinivas, Malladi	RFTFM	The University of Texas Southwestern Medical Center	$2,000,000

REI: Recruitment of Established Investigators
RFTFM: Recruitment of First-Time Tenure Track Faculty Members

MOTION:
On a motion made by Dr. Mulrow and seconded by Mr. Margo, the Oversight Committee unanimously voted to approve the Program Integration Committee's recommendations for an Early Translational Research Award, and an Individual Investigator – Prevention and Early Detection Award to The University of Texas at Austin (application numbers RP170427 and RP170095).

Presiding Officer Geren noted for the record that Mr. Angelou did not vote on these recommendations.

MOTION:
On a motion made by Mr. Holmes and seconded by Dr. Mulrow, the Oversight Committee unanimously voted to approve the Program Integration Committee's remaining 44 recommendations for the eight slates of academic research awards.

MOTION:
On a motion made by Dr. Rosenfeld and seconded by Dr. Mulrow, the Oversight Committee unanimously voted to approve the delegation of contract negotiation authority to the Chief Executive Officer and CPRIT staff, and authorized the Chief Executive Officer to sign the contracts on behalf of CPRIT.

8. Chief Product Development Officer Report and Grant Award Recommendations

Mr. Michael Lang, Chief Product Development Officer, updated the Oversight Committee on the status of Product Development Review Cycles 16.2 and 17.1. He also reported on the meeting of the Product Development Advisory Committee (PDAC). The PDAC discussed several issues, including the Product Development Research/Scientific Research funding ratio, royalty rates for therapeutic companies versus drug and device companies, maintaining the current $20 million award cap and eligibility of previous CPRIT Product Development Research grantees for future awards.

Mr. Lang outlined a proposal for CPRIT to modify the royalty structure for devices, diagnostics, and services to 2.5% with 2.5X cap. This change should increase CPRIT attractiveness to lower margin industry sectors, such as devices, diagnostics, and services, increasing their application rate. Mr. Lang explained that the Product Development Research Subcommittee will discuss the issue and may bring a recommendation to a future meeting.

In response to a question, Mr. Lang stated that while it cannot be determined how many companies from other health sectors did not submit applications due to the royalty rate structure, 75% of CPRIT awardees are in the therapeutics sector.

In response to a question about the funding split between product development and academic research, Presiding Officer Geren explained that some members have not had an opportunity to consider the issue. He suggested deferring Oversight Committee discussion until the February meeting to allow the Research Subcommittee and Product Development Subcommittee time to consider the issue and prepare a combined recommendation. He asked that the Product Development Advisory Committee prepare a recommendation also. Mr. Geren said the subcommittees should also discuss the issue of the award caps.

An Oversight Committee member asked CPRIT's exposure to liability if someone sues a CPRIT company over its product. Ms. Kristen Doyle, Deputy Executive Officer and General Counsel, responded that CPRIT as a state entity has sovereign immunity and qualified immunity protect the state and its officers against liability. CPRIT's contract also makes it clear that CPRIT is not responsible for actions taken by grantees in any of its programs.

Grant Award Recommendations

Mr. Lang presented the Program Integration Committee's recommendations for two Texas Company Product Development Research Awards totaling $32,146,716 to Bellicum Pharmaceuticals ($16,946,716), and Molecular Templates ($15,200,000). Both companies revised their budget requests from the original applications after the Program Integration Committee had made its recommendations. Because the companies made progress on the projects since the submission of the applications, their budget requirements decreased through the budget review process. Mr. Lang noted that the CEO granted him a waiver pursuant to Texas Administrative Code § 702.19(c), allowing him to communicate with the companies after the PIC recommendations to negotiate budget reductions.

Mr. Lang explained that Bellicum Pharmaceuticals ("Bellicum") is developing a cancer treatment based on a lead compound BPX-501. This company is studying the use of the compound in combination with αβ T-cell depleted grafts for the treatment of acute myeloid leukemia. The BPX-105 therapy provides an alternative for patients when a HLA-matched donor is not available. The scientific rationale underlying the project is highly rated by the review panel, receiving an overall score of 3.1. Bellicum's headquarters are in Houston, Texas. The company previously received a CPRIT grant in 2011.

Mr. Lang reported that the scientific rationale underlying the Molecular Templates product development research project is also highly rated by the review panel, receiving an overall score of 1.7. Molecular Templates is developing a lead compound, MT-4019ND, a modified fragment of an antibody that inactivates ribosome activity. The company is developing MT-4019ND to treat multiple myeloma. Molecular Templates is located in Georgetown, Texas. The company previously received a CPRIT grant in 2012.

Program Priorities Addressed by Grant Recommendations

Mr. Lang reported the program priorities addressed by the grant recommendations, explaining that one project may address more than one program priority. These priorities include:

- Funding projects at Texas companies and relocating companies that are most likely to bring important products to the market
- Providing funding that promotes the translation of research at Texas institutions into new companies able to compete in the marketplace
- Identifying and funding projects to develop tools and technologies of special relevance to cancer research, treatment, and prevention
- Early translational research (priority across programs)
- Enhance Texas' research capacity and life science infrastructure (priority across programs)
- Rare and intractable cancers, including childhood cancers (Academic Research priority)

There were no questions for Mr. Lang.

Presiding Officer Geren noted that Oversight Committee members did not report any conflicts of interest with these two awards. He noted that Mr. Burgess had certified previously the Product Development Research Awards along with the Academic Research Awards.

Product Development Research
Grant Award Recommendations

Application ID	Company Name	Project	Maximum Budget
DP160057	Bellicum Pharmaceuticals, Inc.	Clinical Evaluation of a Novel T Cell Therapy (BPX-501) for the Treatment of Children and Adults with AML	$16,946,716
DP16071	Molecular Templates, Inc.	A Novel Compound Targeting CD38 for Treatment of Multiple Myeloma	$15,200,000

MOTION:
On a motion made by Dr. Rice and seconded by Dr. Rosenfeld, the Oversight Committee unanimously voted to approve the Program Integration Committee's recommendations for Bellicum Pharmaceutical and Molecular Templates with revised budget amounts as recommended by the Chief Product Development Officer. The revised budget amount for Bellicum Pharmaceutical is $16,946,716. The revised budget amount for Molecular Templates is $15,200,000.

MOTION:
On a motion made by Mr. Angelou and seconded by Ms. Mitchell, the Oversight Committee unanimously voted to approve the delegation of contract negotiation authority to the Chief Executive Officer and CPRIT staff, and authorized the Chief Executive Officer to sign the contracts on behalf of CPRIT.

Presiding Officer Geren reported that Mr. Roberts notified the Oversight Committee by letter on November 9, 2016, that he would seek authority to disburse grant funds in advance to the companies approved in the Oversight Committee's previous motion. There were no questions for Mr. Roberts.

MOTION:
On a motion made by Dr. Rosenfeld and seconded by Dr. Mulrow, pursuant to the General Appropriations Act, Article IX, Section 4.03(a), the Oversight Committee unanimously voted to authorize CPRIT to disburse grant funds via advance payments to Bellicum Pharmaceuticals and Molecular Templates upon execution of the award contracts and the successful completion of tranches.

9. Chief Prevention and Communications Officer Report

Prevention Report

Dr. Rebecca Garcia informed the Oversight Committee that CPRIT awarded a total of $26,938,196 for 26 Prevention grants in FY 2016. She updated the status of FY 2017 Prevention Program review cycles 1 and 2. Dr. Garcia highlighted a new RFA that CPRIT plans to release November 17, 2016 for Tobacco Control and Lung Cancer Screening.

Presiding Officer Geren asked Dr. Garcia to comment on smokeless tobacco use, which appears to be a bigger problem in rural, underserved areas. Dr. Garcia explained that the data suggests that smokeless tobacco, primarily chewed tobacco, is used more in rural and farm settings. The new RFA, on Tobacco Control and Lung Cancer Screening, will accept applications for youth tobacco prevention and adult tobacco cessation programs as well as lung cancer screening for high risk smokers. Programs for youth can be different from adult smoking cessation programs. Another area of interest included in the RFA is the use of electronic nicotine delivery devices, or e-cigs. According to the latest survey data, there has been a significant increase in the use of e-cigs by youth who have never smoked tobacco.

Communications Report

Dr. Garcia reported on the Communications activities, including earned media, publicizing grant awards announcements, media coverage of CPRIT, ongoing projects, and events/meetings attended.

10. Agenda Item 12 – Internal Auditor Report

Ms. Alyssa Martin, Internal Auditor, presented the required FY 2016 Internal Audit Annual Report. She reported all state agencies are required to submit an Internal Audit Annual Report. CPRIT will submit the report to state oversight agencies following Oversight Committee approval.

Ms. Martin said the CPRIT Audit Subcommittee has reviewed the report and it contains all the required elements. Included in the annual report are the four internal audit reports the Oversight Committee approved in Fiscal Year 2016 and two audits that followed-up on prior findings. Also included are other audits not performed by the Internal Auditor. These audits include the grant monitoring audits performed by CohnReznick for the Compliance Program, and the independent third party observer reports performed by Grant Thornton at the peer review panels to ensure conflicts of interest and staff participation meet requirements.

Another element of the annual report is the Internal Audit Plan for 2017. The Oversight Committee reviewed and approved the plan at the September 2016 meeting. The updated risk assessment was reported for areas considered high risk, even if not on the 2017 audit plan.

Presiding Officer Geren reported that on October 7, 2016, the Audit Subcommittee met and considered these recommendations. Mr. Margo, Chair of the Audit Subcommittee, explained that the subcommittee discussed the report thoroughly and unanimously recommends Oversight Committee approval.

MOTION:
On a motion made by Mr. Margo and seconded by Dr. Rosenfeld, the Oversight Committee unanimously voted to approve the FY 2016 Internal Audit Annual Report.

Presiding Officer Geren announced a brief recess for lunch.

The Oversight Committee recessed at 12:11 p.m.

The Oversight Committee reconvened at 12:42 p.m.

11. Agenda Item 10 – Scientific Research and Prevention Program Committee Appointments

Mr. Roberts presented his three appointments to the Scientific Research and Prevention Programs Committee, which included three recommendations for the Prevention Program peer review panels:

- Kevin T. Brady, M.P.H.
- Gregory Connolly, D.M.D., M.P.H.
- Michael P. Eriksen, Sc.M., Sc.D.

Mr. Roberts reported that the Product Development Review Council (PDRC) added a new member, Colin Turnbull, Ph.D., to the PDRC. Mr. Roberts explained that no action is needed for this PDRC appointment because Dr. Turnbull is already an appointed member of the Scientific Research and Prevention Programs Committee.

MOTION:
On a motion made by Dr. Rosenfeld and seconded by Mr. Holmes, the Oversight Committee unanimously voted to approve the Scientific Research and Product Development Program Panel appointments.

12. Agenda Item 11 – FY 2017 Program Priorities

Presiding Officer Geren referred the Oversight Committee members to the handout, *2017 Program Priorities*, which outlines the process used to develop the priorities, the scope of the project, and CPRIT's long-term vision. He called upon the chairs for each program subcommittee to present the recommendations.

Academic Research
Dr. Rice, Chair of the Academic Research Subcommittee, reported that the subcommittee reviewed the Academic Research program priorities and made minor adjustments to the

current priorities. He asked Dr. Willson to discuss the subcommittee deliberations. Dr. Willson explained that in addition to current priorities, the subcommittee discussed how, within a limited budget, to communicate CPRIT's highest priorities to the research community, Oversight Committee members, and staff. In that effort, the subcommittee recommended moving the recruitment of outstanding cancer investigators to Texas into the top position on the priority list and core facilities into the second position. The changes reflect that these areas of investment will have a long-lasting legacy. The 2017 priorities are:

- Recruitment of outstanding cancer researchers to Texas
- Investment in core facilities
- A broad range of innovative, investigator-initiated research projects
- Prevention and early detection
- Computational biology and analytic methods
- Childhood cancers
- Population disparities and cancers of importance in Texas

Prevention

Dr. Mulrow, Chair of the Prevention Subcommittee, explained that the subcommittee had three program priority areas that are similar to current priorities. The subcommittee noted that identifying priorities based on areas where significant cancer incidence and mortality disparities exist focuses the program further on areas of greatest need and greatest potential for impact. Data on cancer incidence, mortality and disparities (geographic, ethnic, etc.) are reviewed annually to identify priorities and identify areas of emphasis. This information informs the development of RFAs and informs programmatic decisions during the Peer Review Council level of review. The 2017 priorities are:

- Populations disproportionately affected by cancer incidence, mortality or cancer risk prevalence
- Geographic areas of the state disproportionately affected by cancer incidence, mortality or cancer risk prevalence
- Underserved populations

Product Development Research

Dr. Rosenfeld, Chair of the Product Development Research Subcommittee, said the subcommittee reviewed the program priorities. The priorities are similar to previous years and the subcommittee recommended approval by the full Oversight Committee. The 2017 priorities are:

- Funding novel projects that offer therapeutic or diagnostic benefits not currently available; i.e., disruptive technologies
- Funding projects addressing large or challenging unmet medical needs
- Investing in early stage projects when private capital is least available
- Stimulating commercialization of technologies developed at Texas institutions
- Supporting new company formation in Texas or attracting promising companies to Texas that will recruit staff with life science expertise, especially experienced C-level staff to develop seed clusters of life science expertise at various Texas locations

- Providing appropriate return on Texas taxpayer investment

There were no questions for Dr. Rice, Dr. Mulrow or Dr. Rosenfeld.

MOTION:
On a motion made by Dr. Rice and seconded by Ms. Mitchell, the Oversight Committee unanimously voted to approve the FY 2017 Program Priorities.

13. Amendments to 25 T.A.C. Chapters 701-703

Kristen Doyle, Deputy Executive Officer and General Counsel, presented the proposed rule changes for final approval, followed by new proposed rule changes for publication in the *Texas Register*.

Ms. Doyle explained that at its August meeting the Oversight Committee preliminarily approved the rule changes proposed for final adoption today. She reported that Texas Tech University System submitted a comment regarding a reference to a type of audit that has been superseded. Ms. Doyle recommended against making a change to the final rule at this time. She explained that CPRIT will address Texas Tech's proposed change as a new proposed amendment.

MOTION:
On a motion made by Dr. Rice and seconded by Mr. Margo, the Oversight Committee unanimously voted to approve the final orders adopting rule changes to Texas Administrative Code Chapters 701-703.

Ms. Doyle presented two new proposed amendments to Texas Administrative Code Chapter 703 for the Oversight Committee's preliminary approval. She explained that the proposed rules will be published in the *Texas Register* and on the CPRIT website for public comments. She will present any comments received at the February 2017 Oversight Committee meeting.

MOTION:
On a motion made by Dr. Rosenfeld and seconded by Mr. Holmes, the Oversight Committee unanimously voted to approve the proposed changes to Texas Administrative Code Chapters 701-703 for publication in the *Texas Register*.

14. Plan for Management of Royalty/Equity Portfolio

Ms. Doyle reported that much of what CPRIT now funds will not be commercialized and sold on the market for many years. CPRIT's statute requires CPRIT to include revenue sharing terms in every grant contract. When CPRIT funds early translational research or basic science projects, the universities or companies may not receive any revenue from the project for 10-15 years. CPRIT needs a system to track all grants and data regarding new products being commercialized, patent applications filed, etc., with a goal of knowing when the grantees will owe revenue sharing payments to CPRIT. Ms. Doyle explained that CPRIT currently relies on self-reporting by the academic institutions and companies.

Ms. Doyle reported that CPRIT and the Texas Treasury Safekeeping Company (the Trust Company), a part of the Comptroller's Office, have met and discussed management of assets. The Trust Company has relevant expertise. When the Emerging Technology Fund (ETF) program ended, the ETF funded companies and their ongoing obligations transferred to the Trust Company. The Trust Company increased staff to manage those assets.

The legislative change proposed in SB 81 and HB 63 transfers the final disposition of equity generated by CPRIT grants and royalty streams to the Trust Company. If approved, final decision-making authority regarding disposition of assets resulting from CPRIT grants would transfer to the Trust Company.

In the event that legislation does not pass, CPRIT will contract with a third party to provide that expertise and final decisions would remain with the Oversight Committee. CPRIT is also seeking legislation to allow the Oversight Committee to go into closed session to consider and discuss equity disposition.

In response to a question about whether the researcher or the institution self-reports revenue, Ms. Doyle explained that the institution as the grantee contacts CPRIT when payments are due. To date, CPRIT has collected approximately $3.1 million from 8-10 grantees.

There were no further questions for Ms. Doyle.

15. Contract Approvals

Ms. Heidi McConnell, Chief Operations Officer, presented the CPRIT staff recommendation to approve an amendment to the contract with SRA International, Inc., a CSRA Company (CSRA), in the amount of $1,226,787. The FY 2017 CSRA contract is currently $7,038,659. This amendment would increase the contract by 18% and allow the agency to increase the contract up to 25% if necessary. This contract amendment would require approval by the LBB.

The amendment is for modified services including:

1) A Service Organization Control (SOC) 2, type 2 report on the grants management platform to assess the system and the suitability of the design of controls;
2) Enhancements to the grants management platform to address functionality such as updating reporting capabilities, adding a workflow reset, adding grantee out of office delegation assignment, and status changes for grants that decline an award or are terminated early;
3) The addition of consensus paragraphs to peer review summary statements for academic and product development research applications;
4) Review and objective assessment of patient advocate peer reviewer applications submitted to CPRIT; and
5) The additional administration of grant application supporting documentation in the grants management system, currently maintained outside the system, in order to produce an automated grant pedigree.

These additional services are necessary to improve efficiency and effectiveness of the grant management processes and address an internal audit finding.

In response to a question, Ms. McConnell reported that CPRIT does not own the software, but does own the data. CPRIT receives a nightly download of all from the software applications so, should it be necessary to change vendors, CPRIT could move the data into a new system.

Ms. Doyle presented the CPRIT recommendation that the Oversight Committee approve outside counsel contracts for FY 2017 for Vinson & Elkins, LLP ($125,000) and Baker Botts, LLP ($125,000). These firms will join Yudell Isidore, PLLC, in providing legal advice and evaluation services regarding intellectual property and revenue sharing agreements associated with CPRIT grants during FY 2017. The Office of the Attorney General must approve all outside counsel agreements prior to contract execution.

Although CPRIT seeks contract approval for FY 2017, the request for proposals includes an option to renew the three outside counsel contracts for up to four additional one-year periods. The renewal periods, if exercised, will extend the outside counsel contract through August 31, 2020. If CPRIT decides to exercise its option to extend one or more of the contracts, CPRIT staff will seek approval from the Oversight Committee and the Office of the Attorney General at the appropriate time.

Having multiple outside counsel contracts is necessary to ensure CPRIT has counsel available in the event of conflicts of interest.

There were no questions for Ms. McConnell or Ms. Doyle.

MOTION:
On a motion made by Mr. Angelou and seconded by Mr. Holmes, the Oversight Committee unanimously voted to approve the contract amendment to the FY 2017 grants management contract with SRA International, Inc.

MOTION:
On a motion made by Mr. Angelou and seconded by Ms. Mitchell, the Oversight Committee unanimously voted to approve outside counsel contracts with Vinson & Elkins and Baker Botts.

16. Chief Operating Officer Report

Ms. McConnell presented the Chief Operating Office Report on CPRIT's Financial Overview for FY 2016, Quarter 4, including:

CPRIT Financial Overview for FY 2016, Quarter 4

FY 2016, Quarter 4 Operating Budget
For the fourth quarter of FY 2016, CPRIT expended or encumbered approximately $16.3 million, or 93%, of the agency's $17.7 million administrative budget between the Indirect Administration and Grant Review and Award Operations strategies. During the quarter, CPRIT received $870,368 in revenue sharing payments which were deposited into the General Revenue Fund (0001), bringing the total revenue sharing collected for FY 2016 to $921,686. Total revenue sharing payments received since CPRIT's inception through the end of August 2016 is approximately $3.1 million.

FY 2016, Quarter 4 Performance Measures
In October 2016, CPRIT reported performance to the LBB on all five required measures. CPRIT met or exceeded the targeted performance in four of the five performance measures. CPRIT did not achieve the target for the Number of People Served by Institute Funded Prevention and Control Activities because the grant activities have not changed from year to year and the agency did not expect to meet the targeted number that had been doubled from the prior year.

Debt Issuance History
The Texas Public Finance Authority (TPFA) issued $60 million in commercial paper notes on CPRIT's behalf in August 2016 bringing the total commercial paper notes issued for FY 2016 to $147.5 million. In FY 2017, TPFA also issued $58 million in commercial paper notes in October on CPRIT's behalf.

FY 2016 Annual Financial Report
CPRIT completed the FY 2016 Annual Financial Report (AFR) on November 4, 2016, well ahead of the November 20th deadline and submitted the report to the Office of the Comptroller of Public Accounts, Governor's Office, Legislative Budget Board, and State Auditor's Office. The McConnell & Jones LLP audit team, the independent audit firm engaged to perform CPRIT's annual financial audit, reviewed the documentation related to the FY 2016 AFR. The results of that audit were expected in early December to be reported to the Audit Subcommittee at a special meeting on December 13.

There were no questions for Ms. McConnell.

17. Chief Compliance Officer Report

Mr. Burgess presented the Chief Compliance Officer report that provides an overview of compliance activities over the last quarter as well as some highlights from FY 2016.

Implementation of the FY 2017 Grantee Risk Assessment has begun. The Grantee Risk Assessment, which assesses financial exposure, entity maturity and prior experience, is used to determine whether a grantee will receive a desk review, on-site review, or other type of compliance monitoring.

Mr. Burgess highlighted the following activities:

Training & Support
CPRIT staff conducted a new grantee training for Texas State University in San Marcos on October 6, 2016. Additionally, CPRIT staff conducted a grantee training webinar on October 12, 2016, with approximately 130 grantee staff in attendance. This webinar was the third conducted for grantees this calendar year to meet new annual compliance training requirements. As of this most recent training webinar, all active grantees had met the training requirement for this year.

FY16 Compliance Program Activities
Mr. Burgess reported that during FY2016, the Compliance Program strengthened existing compliance functions and identified additional compliance activities to support the integrity and transparency of CPRIT's agency processes.

- Grant Recipient Report Monitoring – The number of delinquent reports decreased from 52 in September 2015 to 7 in August 2016, an 85% decline.
- Compliance Monitoring Reviews (Desk and On-site) – The Compliance team performed over 330 compliance reviews during FY 2016, which is an increase of over 500% from the 63 reviews completed in FY 2015.
- Training and Education – CPRIT staff conducted two training webinars, three new grantee trainings, participated in UT Southwestern Medical Center's Research Demonstration Training Series, and the National Council of University Research Administrator's (NCURA) Region V Meeting. These efforts led to over 500 grantee staff being trained.

There were no questions for Mr. Burgess.

18. Subcommittee Business
19. Compliance Investigation Pursuant to Health & Safety Code § 102.2631
20. Consultation with General Counsel

Presiding Officer Geren stated there was no business to discuss for standing items 17, 18, and 19.

21. Future Meeting Dates and Agenda Items

Presiding Officer Geren announced the next regular Oversight Committee meeting is scheduled for February 15, 2017, at 10:00 a.m.

Presiding Officer Geren announced that this was Dr. Mulrow's last meeting with the CPRIT Oversight Committee. Mr. Geren read a resolution on her behalf. Mr. Roberts presented her with a Silver Star with the Texas Seal inscribed with her service years.

MOTION:
On a motion made by Dr. Rice and seconded by Mr. Margo, the Oversight Committee unanimously voted to approve the resolution for Dr. Mulrow.

22. Adjourn

MOTION:
There being no further business, the Oversight Committee unanimously approved a motion to adjourn made by Presiding Officer Geren and seconded by Dr. Rosenfeld.

Meeting adjourned at 1:30 p.m.

_____ _____

Signature Date

Keith Argenbright, MD is Professor and Chief of Community Health Sciences at University of Texas Southwestern Medical Center.

Dr. Argenbright also serves as Director at Moncrief Cancer Institute, an affiliate of the NCI-designated UT Southwestern Harold C. Simmons Comprehensive Cancer Center. Moncrief Cancer Institute is a non-profit, community-based cancer prevention and support center, providing services spanning the cancer continuum of care, including public education and outreach, cancer prevention and early detection, behavioral and nutritional counseling, genetic testing and counseling, financial advocacy, survivorship services and population research. Since the inception of its initial prevention program in 2010, Moncrief Cancer Institute has been the recipient of $49 million in local, state and federal awards.

Dr. Argenbright is a graduate of the University of Oklahoma and Tulane University School of Medicine. He completed a family practice residency at John Peter Smith Hospital and a Master of Medical Management at Carnegie Mellon University.

In 2014, he earned the UT Regents' Outstanding Teaching Award. Considered the top teaching prize in the UT system, it is one of the largest teaching award programs in the country.

Eric Poma, Ph.D., has been with Molecular Templates as its Chief Executive Officer and Chief Scientific Officer since its inception. He was most recently Vice President, Business Development at Innovive Pharmaceuticals, a clinical stage oncology company that was acquired in 2008. Prior to Innovive,

Dr. Poma was Assistant Vice President, Business Development at ImClone Systems (acquired by Lilly). Dr. Poma holds a PHD in Microbiology and Immunology from the University of North Carolina and an MBA in Finance from the Stern School of Business.

CANCER PREVENTION & RESEARCH INSTITUTE OF TEXAS

MEMORANDUM

TO:	OVERSIGHT COMMITTEE MEMBERS
FROM:	WAYNE R. ROBERTS, CHIEF EXECUTIVE OFFICER
SUBJECT:	AGENDA ITEM 6, CHIEF EXECUTIVE OFFICER REPORT
DATE:	FEBRUARY 7, 2017

As of this writing the Chief Executive Officer Report for the February 15, 2017, Oversight Committee (OC) meeting will consist of the following items:

- Personnel update, including discussion of Governor Abbott's freeze on state agency hiring
- Action Items from the November 16, 2016, OC meeting (Attachment 1)
- 2016 Annual Report (distributed to you at the meeting)
- CEO Report Pursuant to Health & Safety Code § 102.260(c)
- Legislative update
- Report on "FY 2017 Grant Award Funds Available" (see following attachment)
- Other topics may be added as warranted

In addition, for your reference, copies of the CPRIT Activities Update for December and January provided to you previously are included at the end of this tab. These reports are provided to you in months in which the OC does not meet.

*****

CPRIT has awarded **1,114** grants totaling **$1.761 billion**

- 172 prevention awards totaling $169.1 million
- 911 academic research and product development research awards totaling $1.592 billion

Of the $1.592 billion in academic research and product development awards,

- 30.0% of the funding ($476.7 million) supports clinical research projects
- 27.1% of the funding ($431.9 million) supports translational research projects
- 24.2% of funding ($385.0 million) supports recruitment awards
- 15.0% of the funding ($239.1 million) supports discovery stage research projects
- 3.7% of funding ($59.3 million) supports training programs.

CPRIT has 15 open Requests for Applications (RFAs)

- 3 Research Recruitment
- 5 Academic Research
- 2 Product Development Research
- 5 Prevention

Attachment 1

Action Items from November 16, 2016, Oversight Committee Meeting

Discuss funding ratio between Academic Research and Product Development Research at respective Oversight Committee Subcommittees. Convene a teleconference or other meetings with the two subcommittee chairs as to a proposed funding split for full Oversight Committee discussion at the February 15, 2017, meeting.

Both the Academic Research and Product Development Research OC Subcommittees discussed the historical funding ratio and possible changes. Both recommended postponing further discussion until the May 17, 2017, one year after the current ratio was discussed.

Discuss additional revenue sharing proposals with the Oversight Committee Product Development Subcommittee and bring recommendations, if any, to the February 15, 2017, meeting.

Modifying the current revenue sharing proposals for diagnostic and device companies was discussed with the Product Development Research Subcommittee and recommendations are being presented at the February 15, 2017, OC meeting.

In addition, limiting additional awards to prior product development research awardees was discussed. A recommendation to increase the required match for companies with second awards is being presented at the February 15, 2017, OC meeting.

Discuss product development research award funding caps with the Oversight Committee Product Development Subcommittee and bring recommendations, if any, to the February 15, 2017, meeting.

Product development research award caps were discussed with the Product Development Research Subcommittee and no action was taken. Current policy which remains in effect is that award funding caps are addressed through product development research Requests for Applications.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

To:	**OVERSIGHT COMMITTEE MEMBERS**
From:	**WAYNE ROBERTS, CHIEF EXECUTIVE OFFICER**
Subject:	**FY 2016 REPORT ON MERIT AND PROGRESS OF PROGRAMS PURSUANT TO TEXAS HEALTH & SAFETY CODE § 102.260(C)**
Date:	**FEBRUARY 8, 2017**

Summary

Fiscal year 2016 marked the halfway point in CPRIT's constitutional funding authorization. Texas' decision in 2007 decision to provide $3 billion for cancer research and prevention remains the only such commitment in the United States. Key metrics indicate that CPRIT is affecting Texas' national standing in both cancer research and the biomedical industry. In FY 2016 (FY 2016), the Oversight Committee approved 143 grants totaling $274,260,908.[1] Texas Health and Safety Code § 102.260(c) requires the Chief Executive Officer to report at least annually to the Oversight Committee on the progress and continued merit of each research program. CPRIT's Academic Research Program, Prevention Program and Product Development Research Program showed progress and merit in FY 2016.

This report provides an overview illustrating the progress made in advancing CPRIT's mission to create and expedite innovation in cancer research and cancer prevention. Aligning program activities with the program priorities adopted by the Oversight Committee is a good gauge of progress and merit. This report highlights each program's implementation of the FY 2016 program priorities. CPRIT's FY 2016 *Annual Report* and quarterly *Achievements Report* provide more information on CPRIT awards.

With regard to progress made by individual grant projects within each of CPRIT's three programs, Texas Administrative Code § 703.21 requires all CPRIT grantees to submit progress reports at least annually. Outside experts evaluate these progress reports to ensure that the grantee has made sufficient progress and should continue work under the grant. To the extent that an expert reviewer determines that a grant project is not making sufficient progress, CPRIT may take a number of actions, including contract termination. CPRIT did not terminate any award during FY2016 for lack of sufficient progress.

[1] Unless specifically noted, all grant awards and amounts discussed in this report reflect the awards approved by the Oversight Committee in FY 2016 that are either under contract with a grantee or the grantee has not declined the award as of the date of this memo.

Academic Research Program

CPRIT's Academic Research Program supports innovative and meritorious projects that are discovering new information about cancer that can lead to prevention, early detection, and cures; translating new and existing discoveries into practical advances in cancer diagnosis and treatment; and increasing the prominence and stature of Texas in the fight against cancer.

In FY 2016, CPRIT's Academic Research Program awarded 51 Individual Investigator Research Awards, 21 High Impact-High Risk research grants, 6 Core Facilities Support Awards, 7 Multi-Investigator Research Awards, 4 Research Training Awards, and 25[2] Recruitment Awards to Texas institutions. The total amount of Academic Research awards approved by the Oversight Committee in FY 2016 and under contract was $211,602,887.

Academic Research Program Priorities

The Oversight Committee adopted the following FY 2016 program priorities for the Academic Research Program:

- A broad range of innovative, investigator-initiated academic research projects;
- Prevention and early detection;
- Computational biology and analytic methods;
- Rare and intractable cancers, including childhood cancers;
- Population disparities and cancers of importance in Texas (Lung, Cervix, Liver); and
- Recruit outstanding cancer researchers to Texas.

This was the second full year that the Oversight Committee's program priorities have been in place. These priorities have influenced Requests for Applications (RFAs) and funding decisions. For example, childhood and adolescent cancers research comprises 10% of CPRIT's research portfolio grants, which is more than twice the national rate. This is one result of the Oversight Committee's decision to prioritize rare and intractable cancers, including childhood cancers. Similarly, the number of Academic Research grants awarded for research to improve prevention and early detection of cancer in Texas has increased to $23 million.

The table below shows the program priorities met by the Academic Research Program grants.

[2] As of August 31, 2016, 17 recruitment contracts were executed and eight contracts were pending acceptance.

Academic Research Program Priorities	# Grants*	Award Totals
A broad range of innovative, investigator- initiated academic research projects	84	$91,000,814
Recruit outstanding cancer researchers to Texas	25	$76,520,000
Childhood cancers	20	$26,984,209
Prevention and early detection	17	$23,272,828
Computational biology and analytic methods	9	$24,600,567
Rare and intractable cancers	31	$42,255,865
Population disparities and cancers of importance in Texas (Lung, Cervix, Liver)	38	$32,982,826

* Some grants address more than one priority.

Seventeen recruits accepted positions at Texas institutions, for a total of $49 million in recruitment grant awards. CPRIT is building a critical mass of cancer researchers in Texas by supporting recruitment of cancer scientists and clinicians as cancer research scholars to academic institutions in Texas. Since its inception through August 31, 2016, CPRIT has supported the recruitment of 113 outstanding cancer researchers to 17 academic institutions throughout Texas. This program has been highly successful in enhancing Texas' cancer research efforts and increasing the external visibility of the state in this field, which ultimately benefits the life sciences infrastructure in Texas.

Prevention Program

Experts report that we are able to prevent nearly half of all cancers. However, the ability to reduce cancer rates depends, in part, on more broadly applying evidence-based prevention strategies currently available. CPRIT's Prevention Program supports effective, evidence-based prevention programs to underserved populations in the state. Prevention Program grants help Texans reduce the risk of getting cancer, identify cancers earlier, and assist people in finding cancer treatment. These efforts ease the burden of cancer in Texas. Comparing 2008 data to the latest information available in 2013, Texas has seen a drop in cancer death rates by 13%, which translates to more than 3,300 deaths prevented.

There were 79 Prevention Program projects active throughout Texas in FY 2016. The Oversight Committee approved 26 new grants during the fiscal year totaling $26,938,196. By the end of FY 2016, CPRIT has supported $169.1 million in Prevention Program grants to provide more than 3.1 million education and clinical prevention services in all 254 counties since 2010. These services include tobacco cessation, genetic testing and counseling, vaccinations and survivor care services. Cumulatively, through FY 2016, Texans have received more than 1.74 million clinical prevention services. Screenings and diagnostics for breast, cervical, colorectal, and liver cancer

account for more than 726,264 of the clinical services. Also, the CPRIT-funded screenings detected 4,833 cancer precursors and found 2,280 cancers. More than 288,403 recipients have received their first cancer screenings from CPRIT projects. These numbers highlight the impact CPRIT has in Texas communities.

In addition to the impact on the health of people in Texas, the Prevention Program grants also improve the healthcare system and foster greater collaborations. Health system improvements include reducing wait times for diagnostic testing, reducing the number of people lost to follow-up, implementing patient reminder systems, enhancing electronic medical records, and training a cadre of community health care workers to help educate and navigate people through the system. These grants stimulate greater collaboration among academic institutions, community organizations, and clinics.

Prevention Program Priorities

The Oversight Committee adopted the following FY 2016 program priorities for the Prevention Program:

- Prioritize populations and geographic areas of greatest need, greatest potential for impact;
- Focus on underserved populations; and
- Increase targeting of preventive efforts to areas where significant disparities in cancer incidence or mortality in the state exist.

CPRIT released six Prevention Program RFAs in FY 2016 including one on Colorectal Cancer Prevention Coalitions and another on Cancer Prevention and Navigation to Clinical Services. The table below reflects how active projects in FY 2016 address Prevention Program priorities.

Prevention Program Priorities	**# Grants***	**Total Amount**
Prioritize populations and geographic area of greatest need, greatest potential for impact	60	$91,000,814
Focus on underserved populations	76	$101,305,994
Increase targeting of preventive efforts to areas where significant disparities in cancer incidence or mortality in the state exist	41	$53,027,117

* Some grants address more than one priority.

Product Development Research Program

CPRIT's Product Development Research Program funds innovative and scientifically meritorious product development projects with the potential of translating research discoveries into commercial products that can benefit cancer patients. During FY 2016, the Oversight Committee approved three Product Development Research awards totaling $53,933,366.

CPRIT has 20 company grants active in FY 2016. Twelve CPRIT-funded company projects conducted clinical trials in FY 2016, reaching cancer patients in Texas with innovative, early stage treatments. The Product Development Research program benefits not only cancer patients, but like CPRIT's recruitment grants, the Product Development Research awards are an important component in building the life sciences infrastructure and community in Texas.

Since 2010 through August 31, 2016, CPRIT companies raised $1.28 billion in follow-on funding from other investors, indicating private sector confidence in the high quality, merit-based peer review and due diligence review process. During FY 2016, two CPRIT-funded companies received FDA Orphan Drug Designation status and a third gained FDA fast track designation for its lead candidate. Orphan drug status means that the drug has a longer period of government exclusivity, therefore attracting more investment. One product development grantee was added to the NASDAQ Biotech Index and another raised $43.7 million with its initial public offering in April. These follow-on investments and activities testify to the quality of the CPRIT-funded projects and CPRIT's review process.

Product Development Research Program Priorities

The Oversight Committee adopted the following FY 2016 priorities for the Product Development Research Program:

- Funding projects at Texas companies and relocating companies that are most likely to bring important products to the market;
- Providing funding that promotes the translation of research at Texas institutions into new companies able to compete in the marketplace; and
- Identifying and funding projects to develop tools and technologies of special relevance to cancer research, treatment, and prevention.

The table below depicts the program priorities fulfilled by the three Product Development Research grants awarded in FY 2016.

Product Development Program Priorities	# Grants	Award Totals
Funding projects at Texas companies and relocating companies that are most likely to bring important products to the market	3	$53,933,966
Providing funding that promotes the translation of research at Texas institutions into new companies able to compete in the marketplace	1	$18,668,717
Identifying and funding projects to develop tools and technologies of special relevance to cancer research, treatment, and prevention	N/A	N/A

The three companies funded in FY 2016 work in Texas to bring important products to market. Two companies awarded grants in FY 2016 relocated to Texas: Aravive Biologics (formerly Ruga Corporation, from San Francisco to Houston) and Salarius Pharmaceuticals (from Connecticut to Houston). The third company, Pelican Therapeutics will transition from a virtual company to a bricks and mortar company in Texas. One of the company projects approved in FY 2016 plans a collaboration with academic institutions in Texas, promoting the translation of research into Texas-based companies. The company specializes in developing novel drugs for rare pediatric cancers, including Ewing's Sarcoma.

Conclusion

CPRIT's three programs show merit and progress and should continue operations. The work conducted under the purview of CPRIT's programs is part of an iterative cycle with observations emerging from the laboratory making their way to the public and back again to the laboratory. Essential players in this cycle are basic scientists, physician scientists, clinical researchers, product development entrepreneurs, public health professionals, health care providers, patients, community organizations, early stage companies, and research institutions across Texas.

FY 2017 GRANT AWARD FUNDS AVAILABLE

General Obligation Bond Proceeds

	Prevention	Academic / Product Development Research	Prevention Percentage Based on Available Award Appropriations	Operating Budget	Total Appropriations
Available Appropriated Funds	$ 28,319,312	$ 254,879,810		$ 16,800,878	$ 300,000,000
Unexpended Bond Proceeds Carry Forward		$ -			$ -
Unexpended Balance Carry Forward		$ -			
Approved Adjustment to Operating Costs		$ -		$ -	
Appropriations Transfer to DSHS		$ (2,969,554)		$ 2,969,554	
Adjusted Appropriations	$ 28,319,312	$ 251,910,256		$ 19,770,432	$ 300,000,000
Total Available for All Grants			$ 280,229,568		
Calculated 10% for Prevention Grants of Total Available Grant Funding			$ 28,022,957		
Adjustment for 10% Prevention Grants Limit	(296,355)	$ 296,355			
Adjustment to Address Avg Prevention Historical Limit	(1,851,835)	$ 1,851,835			
Revised Adjusted Appropriations	26,171,122	$ 254,058,446		$ 19,770,432	$ 300,000,000

	Prevention Grants	Academic Research Grants	PD Research Grants			
Total Available for Grant Awards (Total GO Bond Proceeds Less Operating Budget)	$ 26,171,122	$ 190,543,834	$ 63,514,612			$ 280,229,568
Announced Grant Awards						
9/14/16 AR Core Facilities Awards		$ 16,062,539	$ -			
9/14/16 AR Recruitment Awards		$ 34,000,000	$ -			
11/16/16 PDR Awards-2 companies			$ 32,146,716			
11/16/16 AR Awards-Translational Research		$ 3,974,486				
11/16/16 AR Awards-IIRA		$ 17,892,210				
11/16/16 AR Awards-Childhood/Adolescent Cancers		$ 8,035,738				
11/16/16 AR Awards-Computational Biology		$ 2,634,668				
11/16/16 AR Awards-Prevention and Early Detection		$ 5,819,500				
11/16/16 AR Awards-Research Training		$ 14,866,638				
11/16/16 AR Recruitment Awards		$ 8,000,000				
Announced Grant Award Subtotal	$ -	$ 111,285,779	$ 32,146,716	$ -		$ 111,285,779
Grant Award Adjustments						
Declined Recruit Award (MDACC-Ye) 9/2016 Slate		$ (2,000,000)				$ (2,000,000)
Declined IIRA (BCM-Scott) 11/2016 Slate		$ (875,757)				$ (875,757)
Declined Recruit Award (MDACC-Clarke) 9/2016 Slate		$ (6,000,000)				$ (6,000,000)
Revised Grant Award Subtotal	$ -	$ 102,410,022	$ 32,146,716			$ 102,410,022
Available Funds January 2017	$ 26,171,122	$ 88,133,812	$ 31,367,896			$ 114,304,934
Pending Grants-PIC Recommendations						
2/15/17 AR Recruitment Awards		$ 22,000,000	$ -			
2/15/17 Increase to AR Award RP170259		$ 576,748				
2/15/17 Prevention Awards	$ 12,024,696	$ -				
Pending Award Subtotal	$ 12,024,696	$ 22,576,748	$ -			$ 34,601,444
Total Potential Grant Funding Committed	$ 12,024,696	$ 124,986,770	$ 32,146,716			$ 137,011,466
Available Funds as of Feb. 16, 2016	$ 14,146,426	$ 65,557,064	$ 31,367,896			$ 143,218,102
11/2016 PIC Deferred AR Grant Applications		$ 10,033,103				
2/2017 PIC Deferred PRV Grant Applications	$ 1,500,000					

Operating Budget Detail	
Indirect Administration	$ 3,030,652
Grant Review & Award Operations	$ 13,770,226
Subtotal, CPRIT Operating Costs	$ 16,800,878
Cancer Registry Operating Cost Transfer	$ 2,969,554
Total, Operating Costs	19,770,432

CPRIT MANAGEMENT DASHBOARD
FISCAL YEAR 2017

	SEPT	OCT	NOV	DEC	JAN	FEB	MAR	APR	MAY	JUN	JUL	AUG	CUMULATIVE (ANNUAL)	CUMULATIVE (TO DATE)
ACCOUNTABILITY														
Announced Grant Awards			48										48	
New Grant Contracts Signed	9	15	6	4	21								55	
New Grant Contracts In Negotiation			41										41	
Grant Reimbursements Processed (#)	147	182	186	289	216								1,020	
Grant Reimbursements Processed ($)	$ 16,840,484	$ 13,844,271	$ 15,610,663	$ 25,547,229	$ 24,395,194								$ 96,237,841	
Revenue Sharing Payments Received	$ 4,000	$ -	$ 11,862	$ -	$ -								$ 15,862	$ 3,151,065
Total Value of Grants Contracted ($)	$ 30,061,230	$ 29,635,362	$18,107,181	$ 2,866,290	$ 35,989,029								$ 116,659,092	
Grants Awarded (#)/ Applications Rec'd (#)	12%	12%	13%	13%	13%									
Debt Issued ($)/Funding Awarded ($)	64%	67%	64%	64%	67%									
Grantee Compliance Trainings/Monitoring Visits	0	4	3	0	0								7	
Awards with Delinquent Reimbursement Submission (FSR)			1											63
Awards with Delinquent Matching Funds Verification			0											22
Awards with Delinquent Progress Report Submission			2											31
IA Agency Operational Recommendations Implemented	19	19	19	19	19									
IA Agency Operational Recommendations In Progress	19	19	19	19	19									
Open RFAs	11	3	5	10	10									
Prevention Applications Received	36	0	0	0	0								36	676
Product Development Applications Received	19	0	0	0	0								19	363
Research Applications Received	2	2	3	3	169								179	5,447
Help Desk Calls/Emails	230	247	167	110	254								1,008	
MISSION														
ACADEMIC RESEARCH PROGRAM														
Number of Research Grants Awarded (Annual)			46										46	
Recruited Scientists Announced														167
Recruited Scientists Accepted														123
Recruited Scientists Contracted														111
Published Articles on CPRIT-Funded Projects (#)														
Jobs Created & Maintained (#)														
Trainees in CPRIT-Funded Training Programs (#)														
Open Clinical Trials (#)														
Number of Patents Resulting from Research														
Number of Patent Applications														

CPRIT MANAGEMENT DASHBOARD
FISCAL YEAR 2017

	SEPT	OCT	NOV	DEC	JAN	FEB	MAR	APR	MAY	JUN	JUL	AUG	CUMULATIVE (ANNUAL)	CUMULATIVE (TO DATE)
Number of Investigational New Drugs														
PRODUCT DEVELOPMENT RESEARCH PROGRAM														
Number of Product Development Grant Awarded (Annual)			2										2	
Life Science Companies Recruited (in TX)														9
Published Articles on CPRIT-Funded Projects														
Number of Jobs Created & Maintained														
Open Clinical Trials (#)														7
Number of Patents Resulting from Research														
Number of Patent Applications														
Number of Investigational New Drugs														
PREVENTION PROGRAM														
Number of Prevention Grant Awarded (Annual)			0										0	
People Served by CPRIT-Funded Prevention and Control Activities			181,686										181,686	
People Served through CPRIT-Funded Education and Training			89,885										89,885	
People Served through CPRIT-Funded Clinical Services			91,801										91,801	
TRANSPARENCY														
Total Website Hits (Sessions)	5,975	5,618	7,019	5,137	8,089								31,838	
Total Unique Visitors to Website (Users)	4,485	4,009	4,768	3,608	5,563								22,433	

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO:	OVERSIGHT COMMITTEE MEMBERS
FROM:	WAYNE R. ROBERTS, CHIEF EXECUTIVE OFFICER
SUBJECT:	CPRIT ACTIVITIES UPDATE – JANUARY 2017
DATE:	FEBRUARY 2, 2017

Topics in the memo include preparation for the February Oversight Committee meeting, recent milestones in our fight against cancer, CPRIT staffing, legislative and related briefings, Compliance, Program, and Operations updates.

Preparation for the February Oversight Committee Meeting

The Oversight Committee will meet February 15 at 10:00 a.m. in the Board Room 1.170 of the Texas Higher Education Coordinating Board, 1200 E. Anderson Lane, Austin, Texas 78752. We have used this room previously when space at the Capitol was unavailable. Please allow yourself extra time in order to park. The final agenda for the Oversight Committee meeting will be posted by February 7, 2017; a tentative agenda is attached.

You should have received an email from CPRIT on February 1 with a link and password to access the Program Integration Committee's recommendations via the grant award portal. The portal has supporting documentation regarding each project proposed for an award, including the application, CEO affidavit, summary statement, and grant pedigree. A summary of the award slate will also be available through the portal. Please allow time to complete the individual conflict of interest checks and review the supporting material.

Oversight Committee members will receive an electronic copy of the agenda packet by February 8. Hard copies of the agenda packet will be available at the meeting.

Pursuant to instruction from the November 16, 2016, Oversight Committee meeting, the Academic Research and Product Development Research subcommittees met to discuss the funding ratio between the two programs. Both subcommittees unanimously recommended to postpone this discussion until the May 17, 2017, OC meeting. Additional discussion will occur in subcommittees prior to the May meeting.

Recent Milestones in the Fight Against Cancer

CPRIT Grantees in the News

- Martin Matzuk, PhD, director of the CPRIT funded Center for Drug Discovery at Baylor College of Medicine, was named a Fellow in the National Academy of Inventors (NAI). NAI

Fellows are academic leaders who demonstrate a prolific spirit of innovation in creating or facilitating outstanding inventions and innovations that make a tangible impact on the quality of life, economic development, and welfare of society. Dr. Matzuk holds 13 U.S. patents and has published over 325 papers. His individual research has set the groundwork for creating drugs for treatment of ovarian cancer. Dr. Matzuk is a member of the Dan L. Duncan Comprehensive Cancer Center at Baylor, and a member of the National Academy of Sciences.

- Andres Nevarez, a bioinformatics graduate student at UT Southwestern is one of 34 graduate students selected by the Howard Hughes Medical Institute for a 2016 Gilliam Fellowship in Advanced Study of Bioinformatics. Mr. Nevarez is pursuing his training in the bioinformatics program developed at UT Southwestern by CPRIT Scholar Gaudenz Danuser, PhD. Mr. Nevarez's project uses advanced computer vision to detect the spread of skin cancer.

- Scott Kopetz, MD, PhD, CPRIT grantee and associate professor at MD Anderson reported the results of a clinical trial that shows significantly better outcomes for patients with a treatment-resistant form of metastatic colorectal cancer. The results, reported on at the 2017 American Society of Clinical Oncology Gastrointestinal Cancers Symposium, showed that 67 percent of patients with a colon cancer with a BRAF mutation who were treated with the drug vemurafenib responded. Only 22 percent of patients who received standard treatment had this response. About 60,000 people per year are diagnosed in the U.S. with metastatic colorectal cancer and about 7 percent have the BRAF mutation. This research could help thousands of people who previously had no effective course of treatment. While this trial was not directly supported by CPRIT funds, Dr. Kopetz has been funded by CPRIT to study the molecular mutations like BRAF in over 750 metastatic colon cancers.

- Maria Jibaja-Weiss, Matt Anderson and Jane Montealegre of Baylor College of Medicine were Honorable Mention for the Hearst Health Prize for Excellence in Population Health. The application was based on their collaborative CPRIT-funded projects in the Harris Health System in Houston. Achievements resulting from their Cervical Cancer Screening Program for High Risk Uninsured and Underinsured Women in Harris County include increasing the proportion of Harris Health patients (162,443/year) compliant with cervical cancer screening from 53 to 83 percent, better than the national average, and increasing the proportion of cervical cancers diagnosed in its earliest stage from 63 to 73 percent, saving Harris County an estimated $2.3 million annually.

Notable CPRIT Supported Research and Prevention Accomplishments

- Immatics US Inc. has signed a research collaboration and exclusive license agreement with Amgen to develop next-generation, T-cell engaging bio-specific immunotherapies targeting multiple cancers. Amgen is one of the world's leading independent biotechnology companies. The collaboration will combine Immatics' target discovery and T-cell receptor capabilities with Amgen's clinical development, manufacturing and commercial expertise to develop and commercialize novel oncology drugs. Immatics will receive an upfront fee of $30 million plus development, regulatory and commercial milestone payments for each program and tiered royalties payments. A substantial research collaboration such as this is one of the most important milestones for any biotech company because it shows the new technology is valued by experts. It also provides development capital and a distribution channel. Immatics received a Product Development Research grant in 2015.

- ESSA Pharma Inc. announced continued progress on their Phase 1 / 2 clinical study for prostate cancer. ESSA is developing EPI-506 for the treatment of castration resistant prostate cancer ("CRPC") in patients whose disease progresses despite treatment with current therapies. EPI-506 disrupts the signaling pathway that drives prostate cancer growth. The company announced the ongoing Phase 1 / 2 study is now in the 5th cohort and to date treatment has been well-tolerated. Phase 2 study approval has been received pending final Phase 1 data review. ESSA received a Product Development Research grant in 2014.

- Aravive Biologics (formerly Ruga Corporation) announced that newly published research indicated that Aravive's engineered decoy receptor may block Zika virus infection. The novel compound in development by Aravive for cancer treatment binds to a cell receptor that scientists believe may be a key "survival switch" that allows tumors to grow and metastasize. By binding to the tumor survival switch, the Aravive compounds acts as a decoy, slowing the tumor growth and making it more vulnerable to radiation and other therapies. The company reports that in addition to its cancer applications, the new research suggests that Aravive's decoy receptor may also be able to block infection by Zika and related viruses, such as the dengue virus. Aravive received a Product Development Research grant in 2015. The company relocated from San Francisco to Houston as a result of the CPRIT award.

- Dr. Joshua Mendell, CPRIT Scholar and Professor of Molecular Biology at UT Southwestern has discovered an important mechanism for how proteins are regulated with implications for how cancer develops. The work, published in the journal *Nature*, reveals how tiny microRNA molecules find their partner messenger RNAs on a crowded cellular dancefloor to regulate the production of proteins. This discovery is important because the microRNA pathway is critically important to health and disease, serving as a kind of volume control for genes by dialing down the expression of specific proteins. For example, Dr. Mendell previously found that defects in the microRNA pathway contribute to certain childhood cancers and specific microRNAs can accelerate or inhibit cancer by regulating tumor suppressor or tumor promoting genes.

- Dr. Ron DePinho, CPRIT grantee and President of MD Anderson, reported in the journal *Nature* on a gene target to help treat pancreatic, colon and stomach cancers. Like urban

traffic patterns that offer multiple routes to a destination, cells have redundant pathways for critical cell survival processes. DePinho's team found that loss of one of these survival pathways - the metabolic gene malic enzyme 2 (ME2) - leaves a pancreatic cancer cell dependent on an alternative pathway, ME3. The loss of ME2 sets up a cancer-specific vulnerability in the pancreatic cancer cell that can be exploited by developing a drug that selectively targets the partner pathway – ME3 in this example. Because normal cells retain the ME2 gene, they are not vulnerable to targeting ME3, while the cancer cell is. What makes this report important is that a large number of pancreatic, colon and stomach cancers have lost the ME2 gene and might be vulnerable to targeting ME3. The MD Anderson team is now pursuing development of a drug to target ME3.

- Dr. Kenneth Westover, CPRIT Scholar and Assistant Professor of Biochemistry and Radiation Oncology at UT Southwestern Medical Center has uncovered the chemical process behind the anti-cancer properties of a spicy Indian pepper plant called the long pepper, whose suspected medicinal properties date back thousands of years. The secret lies in a chemical called Piperlongumine (PL), which has shown activity against many cancers including prostate, breast, lung, colon, lymphoma, leukemia, primary brain tumors, and gastric cancer. Using x-ray crystallography, Dr. Westover showed how PL is transformed after being ingested to an active drug that silences a gene called GSTP1. The GSTP1 gene produces a detoxification enzyme that is often overly abundant in tumors and serves to protect the cancer cell. The study is published in the *Journal of Biological Chemistry*.

- UT Southwestern Simmons Cancer Center researchers, Drs. Robert Bachoo, Ralf Kittler, Bruce Mickey, and Elizabeth Maher, have found a way to inhibit the growth of glioblastoma, a type of brain cancer with low survival rates. Their CPRIT and NIH supported research found that gene mutations that the pharmaceutical industry and oncologists have focused on to treat glioblastoma are essential only for starting tumor growth. Once the tumor has advanced to the stage where patients seek treatment, they found that these mutations are no longer required for continued tumor growth and as a consequence are not effective targets for treatment. Instead, they found that the same proteins that regulate normal brain development are responsible for sustaining the growth of glioblastoma. Most exciting is their finding that when these proteins are inhibited glioblastomas stop growing. This discovery is important because it focuses researchers on a new target for the treatment of glioblastoma. For example, they found that mithramycin, an FDA approved drug for controlling serum calcium but not previously evaluated in brain cancers, inhibits these targets and stops tumor growth in mice, barring human glioblastomas. The discovery reported in January's *Cell Reports* could lead to development of a new therapy that will increase survival time for glioblastoma patients.

- Dr. Navkiran Shokar at Texas Tech University Health Sciences Center at El Paso, leads a comprehensive community-based program providing education, outreach, no-cost screening and diagnostic tests, and navigation services to address obstacles to colorectal cancer screening. Through this program almost 9,000 people were screened for colorectal cancer: 363 had diagnostic colonoscopies and 219 of these had pre-cancerous polyps removed during the procedure. Due to its initial success, the project has now expanded to 19 additional West Texas counties

Personnel and Immediate Hiring Freeze as of January 31, 2017

Governor Abbott imposed on January 31 an immediate hiring freeze at all state agencies and institutions of higher education to last through the end of the 2017 fiscal year. As of January 31, CPRIT has 32 authorized full-time equivalent (FTE) positions, of which 29 are filled. CPRIT was screening and interviewing candidates to fill the two open Grant Accountant positions and one open Grant Specialist position when the hiring freeze was announced. We have stopped those efforts while we seek clarification from the Governor's Office regarding the application of the hiring freeze to CPRIT and, if so, whether a waiver from the freeze is appropriate because the State Auditor specifically identified these positions as necessary in his 2013 CPRIT audit.

The hiring freeze may impact CPRIT grantees at state institutions of higher education, including CPRIT Scholars applicants that are in the midst of the recruiting process.

On January 4, 2017, I completed the third of three executive management training sessions with the Assistant Dean for Professional Development of the LBJ School of Public Affairs.

Legislative Briefings and CPRIT Outreach

- Kristen Doyle, Heidi McConnell and I discussed proposed CPRIT legislation with Representative Sarah Davis' staff on January 4.

- Ms. Doyle, Ms. McConnell and I discussed proposed CPRIT legislation with Senator Jane Nelson's staff on January 5.

- The 85th Texas Legislature convened at noon on January 10. The 140 day regular session will end at 11:59 p.m. on May 29.

- Ms. McConnell, Ms. Doyle and I briefed Representative Giovanni Capriglione on CPRIT's activities and legislative issues on January 11.

- Dr. Rebecca Garcia and Ramona Magid conducted a webinar for the American Cancer Society (ACS) Texas Health Systems Department on January 11. ACS had requested this webinar for their staff who work with CPRIT grantees and were interested in learning more about CPRIT.

- Dr. Garcia and Ms. Magid met with new staff of the Texas Comprehensive Cancer Control Program (TCCCP) on January 12. This program receives funding from the Centers for Disease Control and Prevention to implement comprehensive cancer control in Texas by increasing the coordination, integration and implementation of cancer activities through a network of cancer stakeholders at the community level.

- On January 13 Ms. Doyle and I briefed staff of Senator Van Taylor on CPRIT's activities and legislative issues.

- Senator Jane Nelson filed Senate Bill 1 and the Speaker's Office released the House draft General Appropriations Act on January 17. These will be the two budget bills for the session. Briefly, CPRIT is recommended at the requested level with authorization for three additional compliance program grant specialists.

- Dr. Garcia presented an update on CPRIT activities at the MD Anderson Executive Advisory Panel Meeting in Houston on January 19.

- Ms. Magid, Senior Program Manager for Prevention, and I discussed cancer and prevention funding concerns in the South Texas State Planning Region with representatives of the City of Laredo on January 23.

- Ms. Doyle, Ms. McConnell and I briefed staff of Senator Sylvia Garcia on CPRIT's activities and legislative issues on January 26.

- Ms. Doyle and I briefed Representative Cindy Burkett on CPRIT's activities and legislative issues on January 31.

- Ms. Doyle and I briefed staff of Senator Kelly Hancock on CPRIT's activities and legislative issues on January 31

- CPRIT testified on Senate Bill 1 (General Appropriations Act) at the Senate Finance Committee on February 1. Ms. McConnell and I made the prepared presentation. Senator Uresti had one question concerning prevention awards for smoking cessation and we will respond in writing to the committee. Cam Scott of the American Cancer Society Cancer Action Network provided oral testimony and reported on a poll his organization had conducted that indicates strong public support for CPRIT and cancer research in Texas. Written testimony was provided but we do not have copies of the material at this time.

- Additional individual legislator meetings are being scheduled.

FY 2016 Annual Report

The *CPRIT 2016 Annual Report* was delivered to the Governor and all members of the Legislature on January 27. Hard copies of this statutorily required report will be provided to you at the February 15 Oversight Committee meeting.

Compliance Program Update

Submission Status of Required Grant Recipient Reports

A delinquent report is produced by CPRIT's grant management system (CGMS) each week; this is the primary source used by CPRIT's compliance staff to follow up with grantees. CPRIT typically has 550+ grants that are either active or wrapping up grant activities and receives an average of 570 grantee reports each month.

As of the most recent CGMS report (January 23, 2017), five required grantee reports from four entities have not been filed in the system by the set due date. Of the five delinquent reports, three (60%) are Academic Research grants, one (20%) is a Prevention grant, and one (20%) is a Product Development grant. In most cases, CPRIT does not disburse grant funds until the required reports are filed. In some instances, grantee institutions may be ineligible to receive a future award if required reports are not submitted. CPRIT's grant compliance specialists and grant accountants continue to review and process incoming reports and reach out to grantees to promptly resolve filing issues.

FSR Reviews

CPRIT's Grant Compliance Specialists performed 87 second-level reviews of grantee Financial Status Reports (FSRs) during the month of January. Eleven (13%) FSRs required resubmission due to insufficient or inaccurate documentation submitted by the grantee. CPRIT's grant accounting staff completes the initial review of the FSRs and supporting documentation before routing them to the compliance specialists for final review and disposition.

Desk Reviews

Eleven desk reviews have been performed during the month of January. Desk-based financial monitoring/reviews are conducted during the course of grant awards to verify that grantees expend funds in compliance with specific grant requirements and guidelines. Desk reviews may target an organization's internal controls, procurement and contracting procedures and practices, current and past fiscal audits, subcontracting monitoring, and timeliness of required grantee report submission. Grant Compliance Specialists are working with 11 grantees to remediate desk review findings.

On-site Reviews

Grant compliance staff performed two on-site reviews during the month of January covering Product Development, Research, and Prevention grants. On-site reviews typically include an examination of the grantee's financial and administrative operations, procurement and inventory procedures, personnel policies and procedures, payroll and timesheet policies, travel policies and records, and single audit compliance. Grant Compliance Specialists are working with three grantees to remediate on-site review findings.

Annual Compliance Attestation (Self-Certification)

Grantees are required to submit an annual self-certification demonstrating compliance with statutory and administrative grant requirements, CPRIT's policies and procedures, the grant contract, and the Uniform Grant Management Standards (UGMS). This opportunity to self-report, in the form of a checklist, provides a baseline of grantee compliance and allows Grant Compliance Specialists to work proactively with grantees towards full compliance prior to a desk review or on-site review. Compliance staff is working with six grantees to submit the required attestation.

Training & Support

An Oversight Committee Ethics and Compliance training module was developed and delivered via an online portal in December 2016. The training covered the Code of Conduct and Ethics (general provisions and gifts), Conflict of Interest Policy, Non-Disclosure Agreement, and other required statements and certifications. Each section, except the overview section, contained links to substantive material and checkpoint questions. Respondents were required to answer correctly questions in each section in order to pass the training. All Oversight Committee members completed the required training prior to the due date.

A grantee training webinar has been scheduled for March 9, 2017. The webinar will focus on administrative rules changes, grantee reporting requirements, compliance program activities, and the grant closeout process. This webinar is in support of the annual compliance training requirement that the Authorized Signing Official (ASO) and at least one other employee from each grantee organization must attend an annual compliance training by November 1 of each year.

Academic Research Program Update

FY2018 Cycle 1 Request for Academic Research Applications
CPRIT announced five Requests for Applications (RFAs) on January 5, 2017. Applications may be submitted March 15, 2017 through June 8, 2017. Grant recommendations for FY 2018 Cycle 1 awards are expected to be presented to the Oversight Committee in February 2018. The five RFAs are as follows:

- **Individual Investigator Research Awards (IIRA) (RFA R-18.1 IIRA)**
  Supports applications for innovative research projects addressing critically important questions that will significantly advance knowledge of the causes, prevention, and/or treatment of cancer. Areas of interest include laboratory research, translational studies, and/or clinical investigations. Competitive renewal applications accepted.
  Award: Up to $300,000 per year.
  Duration: Maximum 3 years.

- **IIRA Childhood and Adolescent Cancers (RFA R-18.1-IIRACCA)**
  Supports applications for innovative research projects addressing questions that will advance knowledge of the causes, prevention, progression, detection, or treatment of cancer in children and adolescents. Laboratory, clinical, or population-based studies are all acceptable. CPRIT expects the outcome of the research to reduce the incidence, morbidity, or mortality from cancer in children and/or adolescents in the near or long term. Competitive renewal applications accepted.
  Award: Up to $300,000 per year. Applicants that plan on conducting a clinical trial as part of the project may request up to $500,000 in total costs.
  Duration: Maximum 4 years.

- **IIRA Computational Biology (RFA R-18.1-IIRACB)**
  Supports applications for innovative mathematical or computational research projects addressing questions that will advance our knowledge in any aspect of cancer. Areas of interest include data analysis of cellular pathways, microarrays, cellular imaging, cancer imaging or genomic, proteomic, and metabolomics databases; descriptive mathematical models of cancer, as well as mechanistic models of cellular processes and interactions and use of artificial intelligence approaches to build new tools for mining cancer research and treatment databases.
  Award: Up to $300,000 per year.
  Duration: Maximum 3 years.

- **IIRA Prevention and Early Detection (RFA R-18.1-IIRAP)**
  Supports applications for innovative research projects addressing questions that will advance knowledge of the causes, prevention, early-stage progression, and/or early detection of cancer. Research may be laboratory, clinical, or population-based, and may include behavioral/intervention, dissemination or health services/outcomes research to reduce cancer incidence or promote early detection. Competitive renewal applications accepted.
  Award: Up to $300,000 per year for laboratory and clinical research; Up to $500,000 per year for population-based research.
  Duration: Maximum 3 years.

- **IIRA Clinical Translation (RFA R-18.1 – IIRACT)**
  Supports applications for innovative clinical research that will lead to a better understanding of the clinical efficacy of a cancer therapy or diagnostic device. Applications submitted under this mechanism should propose innovative clinical studies that are hypothesis-driven and involve patients enrolled prospectively on a clinical trial or involve analyses of biospecimens from patients enrolled on a completed trial for which the outcomes are known.
  Award: Up to $400,000 per year for a maximum of 3 years for laboratory and clinical research; Up to $600,000 per year for up to 4 years if research includes the conduct of clinical trials.
  Duration: Maximum 4 years.

Educational Webinar Planned for February 16

The Academic Research Program will host a question and answer webinar on Individual Investigator Research Awards for Clinical Translation on February 16, 2017. This forum will provide an opportunity for potential applicants to ask questions regarding the award mechanism. The webinar will be recorded, archived and available for view on the CPRIT webpage following the event.

Clinical Trials Data Repository

The Academic Research Program in collaboration with the Product Development Research Program has worked with CPRITs third party IT vendor to develop a clinical trials data module for CPRIT awardees conducting clinical trials. The data module, scheduled to launch in early February 2017, will provide important data on clinical trials outcomes, including the aggregate number of patients enrolled in clinical trials in Texas because of CPRIT funding. Other data

collected include type of research, primary purpose, primary anatomic site, phase of trial, and the focus of the trials. The data module will also collect demographic data including race and ethnicity.

Advisory Committee Meetings
The University Advisory Committee and the Advisory Committee for Childhood Cancers met January 17 and January 23, respectively. The agenda for both meetings included developing content for required annual reports and presentations to the Oversight Committee at its February meeting. Dr. Rice attended and actively participated in both meetings.

Product Development Research Program Update

FY 2017 Cycle 1 Product Development Research Applications
Twenty-five Product Development Research applications were submitted for the first cycle of FY 2017. Peer reviewers at the screening teleconference selected eight of the 25 companies to present at the in-person peer review meeting. Following the in-person presentations, three companies were selected for due diligence. The PDRC met to review the diligence reports for the three companies. The PDRC did not recommend any companies for Oversight Committee consideration.

FY 2017 Cycle 2 Product Development Research Applications
RFAs for FY 2017 Cycle 2 Product Development Research awards will be accepted through February 9.

Program Policies
The Product Development Subcommittee met January 16 to discuss Product Development Research program policies. The Subcommittee will recommend the Oversight Committee approve a revision to CPRIT's standard revenue sharing terms and a change to matching fund requirements for grantees receiving their second Product Development Research grant.

Company Contract Status
Mike Lang expects to recommend the Oversight Committee approve contract terms and contract amendments for two companies, Fujifilm Diosynth Biotechnologies (formerly Kalon Biotherapeutics) and Bellicum Pharmaceuticals, at the February 15 Oversight Committee meeting.

Prevention Program Update

FY 2017 Cycle 1 Prevention Applications
Five RFAs for Cycle 17.1 were released in May 2016. CPRIT received 36 applications by the August 30 deadline. After administrative review, 5 were withdrawn and 31 applications requesting $36,684,532 were assigned to the review panels. Two peer review panels met December 5 - 8 in Dallas.

The Prevention Review Council (PRC) conducted a programmatic review January 20 and forwarded their recommendations to the Program Integration Council (PIC). The PRC considers

geographic distribution, cancer type, type of project and potential for impact in addition to scores assigned by the peer review panels. The PIC met January 31 forwarded their recommendations to the Oversight Committee for consideration at the February 15 Oversight Committee meeting.

FY 2017 Cycle 2 Prevention Applications

The following RFAs were released on November 17:

- Evidence-Based Cancer Prevention Services
- Dissemination of CPRIT-Funded Cancer Control Interventions
- Cancer Prevention Promotion and Navigation to Clinical Services
- Colorectal Cancer Coalition
- Tobacco Control and Lung Cancer Screening

Submissions are due March 2 with peer review meetings taking place in June. Recommendations will be presented to the Oversight Committee in August 2017.

Dr. Garcia and Ramona Magid held a webinar January 11 to present the FY 2017 Cycle 2 funding opportunities and answer questions; 120 people participated. The webinar was recorded, archived and is available on the CPRIT website.

Other activities

The prevention program performance measures report for the Legislative Budget Board was submitted January 9.

Senate Bill 200, Section 2.32, 84th Legislature in 2015 directed the Health & Human Services Commission to collaborate with the Department of State Health Services and CPRIT to develop a strategic plan to reduce the morbidity and mortality from HPV associated cancers. The final report has been submitted to the Legislature and is available on the HHSC website at https://hhs.texas.gov/sites/hhs/files//Human-Papillomavirus-Strategic-Plan_1.pdf.

As a member of the advisory board, Dr. Garcia participated in the January 9 Texas Health Improvement Network Advisory Meeting in Austin. The network was established to address the urgent health care challenges and improve the health care system in Texas.

Communications

Projects and Events

- Press Briefings: CPRIT program officers briefed reporters from *Chemical and Engineering News*, the *Austin American Statesman* and the *Dallas Morning News* on CPRIT activities and its programs. A briefing is being rescheduled with the *San Antonio Express News/Houston Chronicle*. *Chemical and Engineering News* ran a story on CPRIT I their January 30, 2017 issue.
- Communications plans for 2017 include working with CPRIT grantees to promote their work in conjunction with cancer awareness months. January is cervical cancer awareness month, and activities included pitching stories about CPRIT funded cervical cancer screening and HPV vaccination/education programs in local media markets. Results to date include television coverage in El Paso with all major networks, and one network in the Rio Grande

Valley. Similar efforts will be made in February (National Cancer Prevention Month) and March (Colorectal Cancer Awareness Month).

- Staff edited the *CPRIT 2016 Annual Report* and distributed it to the Governor and legislators January 27.
- The *Achievements Report* is being redesigned for 2017, and the new format will be released in February after the Oversight Committee meeting.
- A new one page report entitled, *Texans Conquer Cancer*, was created as a companion piece to the *Achievements Report* for use with various audiences and is available for download on the CPRIT website.
- Content for the new CPRIT website continues to be migrated from the current to the new website, which is scheduled for a February launch.
- Staff continues to respond to requests for information and prepare legislative briefing materials.

Operations and Finance Update

The Bond Review Board (BRB) considered a long-term debt funding request from the Texas Public Finance Authority (TPFA) for $375 million in CPRIT general obligation bond proceeds at a planning meeting on January 9. The funding request includes refunding as long-term debt of $269 million in outstanding commercial paper issued on CPRIT's behalf since April 2016 and $106 million of new money for anticipated CPRIT issuances through the remainder of FY 2017. TPFA determined that it was in the best interest of the state to not only complete refunding of CPRIT's outstanding commercial paper but also issue the remaining amount needed for agency expenses during the year because the 20-year fixed bond rate is moving up and is now approximately 3.5%. The BRB approved on a 2-1 vote (Comptroller Hegar) the funding at its board meeting on January 19, 2017. Heidi McConnell and I attended both BRB meetings.

Heidi McConnell, Kristen Doyle, and I also participated in a due diligence meeting held by the legal counsel for the underwriters of the $375 million general obligation and refunding bonds on January 17. Ms. Doyle and Ms. McConnell responded to questions about the agency while the TPFA Executive Director Lee Deviney addressed questions about the bonds and other administrative related to state conditions.

Beginning January 9, the Weaver audit team was at CPRIT's office performing field work for the internal audit over training programs.

Upcoming Subcommittee Meetings

The dates and times for the upcoming February Oversight Committee subcommittee meetings are listed below.

Subcommittee	Date & Time
Board Governance	February 2 at 10:00 a.m.
Audit	February 6 at 10:00 a.m.
Prevention	February 7 at 10:00 a.m.
Scientific Research	February 8 at 10:00 a.m.
Product Development	February 9 at 10:00 a.m.
Nominations	February 10 at 10:30 a.m.

An agenda, call-in information and supporting material will be sent to the subcommittees one week prior to the meeting date.

*****

CPRIT has awarded **1,114** grants totaling **$1.761 billion**

- 172 prevention awards totaling $169.1 million
- 911 academic research and product development research awards totaling $1.592 billion

Of the $1.592 billion in academic research and product development awards,

- 30.0% of the funding ($476.7 million) supports clinical research projects
- 27.1% of the funding ($431.9 million) supports translational research projects
- 24.2% of funding ($385.0 million) supports recruitment awards
- 15.0% of the funding ($239.1 million) supports discovery stage research projects
- 3.7% of funding ($59.3 million) supports training programs.

CPRIT has 15 open Requests for Applications (RFAs)

- 3 Research Recruitment
- 5 Academic Research
- 2 Product Development Research
- 5 Prevention

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO:	OVERSIGHT COMMITTEE MEMBERS
FROM:	WAYNE R. ROBERTS, CHIEF EXECUTIVE OFFICER
SUBJECT:	CPRIT ACTIVITIES UPDATE – DECEMBER 2016
DATE:	JANUARY 5, 2017

Topics in the memo include preparation for the February Oversight Committee meeting, recent milestones in our fight against cancer, CPRIT staffing, legislative and related briefings, Compliance, Program, and Operations updates.

Preparation for the February Oversight Committee Meeting

Pursuant to instruction from the November 16, 2016, Oversight Committee meeting, staff will arrange meetings in January and February for the OC Subcommittees on Academic Research and Product Development Research to discuss the funding ratio between the two programs. The two subcommittee chairs will also meet to discuss any proposed changes to the funding split. We anticipate full Oversight Committee review and possible action at the February 15, 2017, meeting.

Recent Milestones in the Fight Against Cancer

CPRIT Grantees in the News

- Dr. Hashem El-Serag was named the Margaret M. and Albert B. Alkek Chair of the Department of Medicine at Baylor College of Medicine. Dr. El-Serag is the principal investigator of the CPRIT Texas Hepatocellular Carcinoma Consortium, a multi-investigator research award to address the high morbidity and mortality of liver cancer in Texas.

- Ken Westover, M.D., Ph.D., received the V Scholar Plus award to continue developing a drug to inhibit KRAS mutations. More than 30 percent of all human cancers – including 95 percent of pancreatic cancers and 45 percent of colorectal cancers – are driven by mutations of the RAS family of genes. This award, which extends grant funding for exceptional V Scholars, recognizes Dr. Westover's progress in developing a novel treatment against cancers with KRAS mutations. Dr. Westover is an Assistant Professor at UT Southwestern Medical Center and recipient of a CPRIT First Time Tenure Track Faculty award.

- UT Health Science Center at San Antonio (Dr. Barbara Turner – PP150079), Dr. David Lakey of the UT System and the Department of State Health Services are bringing together hepatocellular carcinoma (HCC) and Hepatitis C (HCV) thought leaders across Texas to

form a collaborative to align efforts across the state and to develop messages to share with policy makers.

- Dr. Subhasis Misra (PP150031) of Texas Tech University Health Sciences Center hosted a media event in Amarillo in support of the "80% by 2018" colorectal cancer screening initiative. The event was covered by all local stations and featured a survivor who was screened through the CPRIT program. This initiative has provided a foundation for others to promote colon cancer screening. Representative Four Price, Amarillo Mayor Paul Harpole, and staff from Senator Seliger's and Representative Ken King's offices attended this event.

Notable CPRIT Supported Research and Prevention Accomplishments

- Dr. Chengcheng "Alec" Zhang and his collaborators at the UT Southwestern Medical Center reported in the journal *Nature Medicine* that intermittent fasting inhibits the development and progression of acute lymphoblastic leukemia (ALL) in a mouse model. The mechanism responsible for this finding is related to leptin, the hormone made by fat cells that regulates hunger and energy levels. Based on their findings they plan to move quickly to human clinical trials in patients with ALL.

- Over 7,500 attendees from 90 countries attended the San Antonio Breast Cancer Symposium in early December. The University of Texas Health Science Center at San Antonio's Cancer Therapy & Research annual symposium is arguably the year's most influential breast cancer meeting. Gail Tomlinson, M.D., Ph.D., reported on the impact of the CPRIT prevention grant project, "*GRACIAS Texas: Genetic Risk Assessment for Cancer in All South Texas,"* which identifies individuals in South Texas and the Lower Rio Grande Valley at very high risk of developing breast cancer and gives them the opportunity to help prevent developing cancers.

- CPRIT Established Investigator, Thomas Yankeelov Ph.D., embarked on a two-year clinical study funded by CPRIT and the National Cancer Institute to use magnetic resonance imaging for early predictions on how an individual patient's breast cancer may respond to therapy. The study will enroll 100 women with newly diagnosed breast cancers and is conducted in partnership with community based oncologists and radiologists at Seton Healthcare, Texas Oncology, and Austin Radiological Association.

- Dr. Jinming Gao, Professor of Oncology, Pharmacology and Otolaryngology with the Harold C. Simmons Comprehensive Cancer Center and Dr. Baran Sumer, Associate Professor of Otolaryngology, have invented a transistor-like threshold sensor that can illuminate cancer tissue, helping surgeons more accurately distinguish cancerous from normal tissue. In the latest study published in *Nature Biomedical Engineering*, researchers were able to demonstrate the ability of the nanosensor to illuminate tumor tissue in multiple mouse models. The new technology may be able to help radiologists reduce false rates in imaging, and assist cancer researchers with non-invasive monitoring of drug responses. CPRIT academic research grants support Dr. Gao and Dr. Sumer's work at UT Southwestern Medical Center. In addition, Dr. Gao and Dr. Sumer are scientific co-founders of OncoNano

Medicine, Inc. OncoNano Medicine received a Product Development grant in 2014 to help fund development of the sensor.

- Aravive Biologics, formerly Ruga Corporation, announced that new preclinical data reported in the *Journal of Cancer Investigation* shows Aravive's novel compound increases tumor sensitivity to radiation therapy and check-point immuno-oncology agents. The preclinical research indicates that novel compound in development by Aravive binds to a cell receptor that scientists believe may be a key "survival switch" that allows tumors to grow and metastasize. By binding to the tumor survival switch, the Aravive compounds acts as a decoy, slowing the tumor growth and making it more vulnerable to radiation and other therapies. Strong anti-cancer activity has been shown against acute myeloid leukemia, and ovarian, pancreatic and breast tumors. Aravive received a Product Development grant awarded in late 2015 to fund preclinical research and clinical trials. The company relocated from San Francisco to Houston as a result of the CPRIT award.

- Asuragen, a CPRIT Product Development grant recipient in 2012, launched a new lung cancer diagnostic test kit that can detect both common and rare versions of the disease. The next generation sequencing panel provides the most comprehensive and sensitive research tool for examining more than 100 clinically relevant gene arrangements for non-small cell lung cancer. The company credits support from CPRIT for development of the new diagnostic kit. The molecular diagnostics company also presented extensive new scientific and clinical studies at the Association for Molecular Pathology annual meeting. The presentations by Asuragen scientists and collaborators covered a broad range of topics, from their latest technology discoveries to advances in genetic disease testing, oncology disease monitoring, and next generation sequencing technologies for profiling solid and liquid biopsies.

- DNAtrix Therapeutics reports the first patients have been treated in a multicenter Phase 2 trial investigating its novel cancer therapy in patients with recurrent glioblastoma. Currently, there is no cure or adequate treatment for recurrent glioblastoma. Scientists have modified the common cold virus, called adenovirus, so that it can recognize and kill cancer cells without harming normal brain tissue. Multiple clinical studies have shown the company's lead oncolytic adenovirus has a favorable safety profile, strong tumor-killing potential and can trigger a patient's antitumor immune response. DNAtrix also recently presented new clinical data at the annual meeting of the Society for Neuro-Oncology. DNAtrix received a Product Development grant in 2014 to fund this work.

- Formation Biologics (formerly Armada Pharmaceuticals) will begin enrolling patients in a Phase I/II study at the START Center for Cancer Care in San Antonio, the company's initial clinical site. The Phase I/II clinical trial will study the safety and efficacy of the company's lead compound, an antibody-drug conjugate, against solid tumors including breast cancer, triple-negative breast cancer, ovarian cancer, and other cancers of epithelial origin. Antibody-drug conjugates pair an antibody designed to target a particular tumor marker with a cancer drug. Once the antibody finds the tumor marker, the cancer cell absorbs the antibody with the cytotoxin that kills the cancer cell. By specifically targeting cancer cells, antibody-drug

conjugates have fewer side effects and may require fewer drug doses than traditional chemotherapy. Formation Biologics received a Product Development grant in 2015 to support this research.

- Medicenna Therapeutics will start a Phase 2 clinical trial for the treatment of recurrent glioblastoma, the most common and uniformly fatal form of brain cancer. Medicenna's immunotherapy treatment uses a two-pronged "molecular Trojan Horse" approach to target and eliminate tumor cells while boosting a therapeutic immune response in patients. The novel fusion protein has received Fast Track Designation from the FDA and Orphan Drug Status from both the FDA and European Medicines Agency. Earlier results from three Phase 1 and 2a clinical trials in 66 patients with glioblastoma showed potent anti-tumor effects without drug-related systemic toxicity in the majority of patients. Medicenna received a Product Development grant in 2015 to support the early clinical trials.

Personnel Changes and Job Openings

As of December 31, 2016, CPRIT has 32 authorized full-time equivalent (FTE) positions, of which 31 are filled.

- Freddy Ruiz, Grant Accountant, moved to the vacant Grant Compliance Specialist position.

- Gerald Green, Grant Accountant, resigned effective January 3. Both Grant Accountant positions are posted through January 6, 2017. In the interim, we will use temporary staff to fill these vacancies until permanent staff are hired.

- Oralia Huggins, Grant Compliance Specialist, resigned effective January 3. The position is posted with a closing date of January 17, 2017.

On November 29, I participated in the second of three executive management training sessions with the Assistant Dean for Professional Development of the LBJ School of Public Affairs. The third and final session is scheduled for January 4.

Legislative Briefings and CPRIT Outreach

- Kristen Doyle and I briefed Senator Donna Campbell on CPRIT's activities and legislative issues on November 17.

- Dr. Jim Willson, Michael Lang and Dr. Patty Moore visited the University of Houston on November 18 to meet with campus departmental leadership and cancer investigators to preview upcoming CPRIT initiatives and to discuss opportunities for awards. The day included a highly interactive 90 minute town hall meeting with UH campus cancer investigators and faculty from two predominantly minority universities – UH-Downtown and Prairie View A&M University.

- Dr. Willson, Dr. Moore, Ms. Doyle and I discussed CPRIT and cancer-related issues with representatives of the Texas Biomedical Research Institute (Texas Biomed) at our office on November 21

- On November 30 Heidi McConnell and I briefed the Lieutenant Governor's staff on CPRIT's schedule of debt payments on outstanding and expected general obligation bonds.

- Dr. Becky Garcia met with grantees and fellows at the University of Houston on December 1 to discuss opportunities for CPRIT cancer prevention funding.

- Dr. Garcia attended a seminar on December 2 at Rice University regarding HPV-Related Cancers: Opportunities for Cancer Prevention.

- Oversight Committee member Dr. Bill Rice, Ms. Doyle and I met with representatives of the Livestrong Foundation on December 5 to discuss CPRIT activities and cancer-related issues.

- At the request of the Governor's Office, I participated in a reception held in Austin on December 5 for the Belgian trade delegation, which included Her Royal Highness Princess Astrid.

- At the request of State Representative Sarah Davis, Ms. Doyle and I discussed CPRIT Product Development Research Grant processes with a potential California applicant on December 8.

- On December 8 Ms. Doyle, Ms. McConnell and I briefed Senator Kel Seliger on CPRIT's activities and legislative issues.

- Mr. Lang presented an overview of CPRIT's Product Development Research program at the BionorthTX Breakfast Forum held in Fort Worth on December 8. BionorthTX is an industry association providing shared services to biotech companies in the DFW area. More than 60 people, mostly prospective CPRIT grant applicants, attended Mr. Lang's presentation.

- On December 12 Ms. Doyle, Ms. McConnell and I briefed Representative Larry Gonzales on CPRIT's activities and legislative issues.

- Dr. Garcia, Ms. Doyle, Chris Cutrone and I briefed representatives of the Texas Cancer Partnership on December 14 regarding CPRIT's legislative requests.

Compliance Program Update

Submission Status of Required Grant Recipient Reports

A delinquent report is produced by CPRIT's grant management system (CGMS) each week; this is the primary source used by CPRIT's compliance staff to follow up with grantees. CPRIT typically has 550+ grants that are either active or wrapping up grant activities and receives approximately 570 grantee reports each month.

As of the most recent CGMS report (December 20, 2016), 22 required grantee reports from eight entities have not been filed in the system by the set due date. Nineteen of the 22 delinquent reports are Financial Status Reports that were not filed by the due date, but are in the 30 day grace period. Of the 22 delinquent reports, 16 (73%) are Academic Research grants, five (23%) are Prevention grants, and one (5%) is a Product Development Research grant. In most cases, CPRIT does not disburse grant funds until the required reports are filed. In some instances, grantee institutions may be ineligible to receive a future award if required reports are not submitted. CPRIT's grant compliance specialists and grant accountants continue to review and process incoming reports and reach out to grantees to resolve promptly filing issues.

Financial Status Report Reviews

CPRIT's Grant Compliance Specialists performed 330 second level reviews of grantee Financial Status Reports (FSRs) during the months of November and December. Eighteen FSRs required resubmission due to insufficient or inaccurate documentation submitted by the grantee. CPRIT's grant accounting staff completes the initial review of the FSRs and supporting documentation before routing them to the compliance specialists for final review and disposition.

Desk Reviews

Thirty-five desk reviews were performed during the months of November and December. Desk-based financial monitoring/reviews are conducted during the course of grant awards to verify that grantees expend funds in compliance with specific grant requirements and guidelines. Desk reviews may target an organization's internal controls, procurement and contracting procedures and practices, current and past fiscal audits, subcontracting monitoring, and timeliness of required grantee report submission. Grant Compliance Specialists are working with twenty-two grantees to remediate desk review findings.

On-site Reviews

Grant compliance staff performed four on-site reviews during the first quarter of FY 2017 covering Product Development Research and Prevention grants. On-site reviews typically include an examination of the grantee's financial and administrative operations, procurement and inventory procedures, personnel policies and procedures, payroll and timesheet policies, travel policies and records, and single audit compliance. Grant Compliance Specialists are working with three grantees to remediate on-site review findings.

Single Audit Tracking

As part of ongoing monitoring efforts, grant compliance specialists track the submission of grantees' independent audit reports and the resolution of issues identified in these reports. Grantees who expend $750,000 or more in state awards in the grantee's fiscal year must submit a single independent audit, a program specific audit, or an agreed upon procedures engagement. The grantee must submit the independent audit report and any findings to CPRIT within 30 days of receipt, but no later than 270 days after the grantee's fiscal year.

There are currently five grantees with outstanding audit findings. Grantees have 30 days from the receipt of the audit to submit supporting documentation to demonstrate remediation efforts. Grant Compliance Specialists worked with two grantees to remediate fully audit report findings.

There are currently no grantees with a delinquent audit report or a delinquent Corrective Action Plan (CAP). Grantees are unable to receive reimbursements or advances if they are delinquent in filing the required audit and corrective action plan, unless the grantee's request for additional time, submitted on or before the due date of the audit, was approved by CPRIT's CEO.

Training & Support

CPRIT staff conducted new grantee training for Lone Star Community Health on November 30, 2016. In addition to a brief overview of CPRIT's history and mission, the training covered grantee reporting requirements, an overview of the compliance program, and a hands-on navigation of CPRIT's online grants management system.

Academic Research Program Update

FY 2017 Academic Recruitment Cycles 17.3 - 17.6

The Scientific Review Council (SRC) reviews recruitment applications monthly. Recruitment award recommendations for applications reviewed by the SRC in November – January (Review Cycles 17.3, 17.4, 17.5 and 17.6) will be presented at the February 2017 Oversight Committee Meeting.

FY 2017 Cycle 2 Request for Academic Research Applications

CPRIT began accepting applications October 17, 2016, for Core Facility and High Impact/High Risk Research awards (see descriptions of the awards below). The application portal will close January 16. Grant recommendations for FY 2017 Cycle 2 awards are expected to be presented to the Oversight Committee in August, 2017.

- Core Facilities Support Awards (RFA R-17.2- CFSA) establish or enhance core facilities (laboratory, clinical, population-based, or computer-based) that directly support cancer research programs to advance knowledge of the causes, prevention, and/or treatment of cancer or improve quality of life for patients with and survivors of cancer. Award: Up to $3 million (total costs) for the first 2 years and up to $1 million (total costs) for each subsequent year. Maximum duration: 5 years.

- High Impact/High-Risk Research Awards (RFA R-17.2-HIHR) provide short-term funding to explore the feasibility of high-risk projects that, if successful, contribute major new insights into the etiology, diagnosis, treatment, or prevention of cancers. Award: Up to $200,000 (total costs). Maximum duration: 2 years.

Product Development Research Program Update

FY 2017 Cycle 1 Product Development Research Applications

Three companies are currently undergoing due diligence, the last step in the Product Development review process before the Product Development Review Council (PDRC) creates a list of recommended awards. The PDRC's recommendations will be presented to the Program Integration Committee and the Oversight Committee in February.

FY 2017 Cycle 2 Product Development Research Applications

On December 22, 2016, CPRIT released two request for applications for FY 2017 Cycle 2 Product Development Research awards. CPRIT will accept applications through its electronic portal January 5 – February 9. Applicants may request up to $20 million in CPRIT funding, but the RFA admonishes applicants that, "while all requests for funding must be well justified, a funding request at or near the maximum amount will be heavily scrutinized. Such a request must be exceptionally well justified to warrant dedicating a large percentage of CPRIT's product development research budget to the applicant's project." Companies that have previously received a CPRIT award are eligible to apply in this cycle; however, the RFA notes that CPRIT reserves the right to seek a higher matching funds contribution (*i.e,* CPRIT will contribute $1.00 in award funds for every $1.00 contributed by the company) from companies that receive a second award.

Other Activities

Following up on the preliminary discussion at the November Oversight Committee meeting, Mr. Lang is developing a proposed recommendation regarding an addition to CPRIT's standard revenue sharing terms to better accommodate device and diagnostic companies. In addition, Mr. Lang and Ms. Doyle have been negotiating appropriate revenue sharing terms with Fujifilm Diosynth Biotechnologies (formerly Kalon Biotherapeutics) and Bellicum Pharmaceuticals. These items, along with pros and cons of award funding caps, will be presented to the Product Development Research subcommittee for consideration and recommendation. Action on these items, if any, will be on the agenda for the February 15, 2017, Oversight Committee meeting.

Prevention Program Update

FY 2017 Cycle 1 Prevention Awards

Five RFAs for FY 2017 Cycle 1 were released in May 2016. Thirty-six applications were submitted and after administrative review, five were withdrawn from consideration. The remaining 31 applications, requesting $36.7 million, were assigned to two review panels that met in Dallas December 5–8, 2016.

Mechanism	# of Applications	Requested Funding
Cancer Prevention Promotion and Navigation to Clinical Services	4	**$** 1,588,990
Competitive Continuation/Expansion	10	$ 13,780,345
Dissemination of CPRIT-Funded Cancer Control Interventions	1	$ 300,000
Evidence-Based Cancer Prevention Services	16	$ 1,015,197
Total	**31**	**$ 36,684,532**

The Prevention Review Council (PRC) meets January 20, 2017, for programmatic review and to develop recommendations for the Program Integration Council (PIC). The PRC considers geographic distribution, cancer type, type of project and potential for impact in addition to scores forwarded by the review panels. Dr. Garcia and Ramona Magid are preparing background information on the active portfolio needed for their deliberations. The PIC recommendations will go to the Oversight Committee in February 2017.

FY 2017 Cycle 2 Prevention Awards

The following RFAs were release on November 17, 2016:

- Evidence-Based Cancer Prevention Services
- Dissemination of CPRIT-Funded Cancer Control Interventions
- Cancer Prevention Promotion and Navigation to Clinical Services
- Colorectal Cancer Coalition
- Tobacco Control and Lung Cancer Screening

The Tobacco Control and Lung Cancer Screening RFA is new this cycle and seeks proposals for programs on tobacco prevention and cessation, as well as screening for early detection of lung cancer. Through release of this RFA, we hope to stimulate more programs across Texas increasing access for underserved populations and reducing the incidence and mortality rates of tobacco-related cancers.

Submissions are due March 2, 2017, with peer review in June. Recommendations will be presented to the Oversight Committee in August 2017.

Other Activities

Quarterly progress reports were submitted by December 15 and are being reviewed; the performance measures report for the Legislative Budget Board is due January 9, 2017.

Senate Bill 200 of the 84th Legislature directed the Health & Human Services Commission to collaborate with the Department of State Health Services and CPRIT on a strategic plan to reduce the morbidity and mortality from HPV associated cancers. Dr. Garcia and Ms. Magid attended meetings, helped draft, reviewed and submitted comments to the final draft of the HPV Strategic Plan. The report was due to the Legislature on December 31, 2016.

Advisory Committee Meetings

- The Oversight Committee approved expanding the membership of the Advisory Committee for Childhood Cancer (ACCC) in late 2015. ACCC chair Dr. Susan Blaney has submitted nominations to CPRIT for Oversight Committee consideration and approval at the upcoming Oversight Committee meeting in February. The ACCC, joined by the provisional members, met telephonically on October 26, 2016. The 12 new members are:
  - Meaghan Granger, MD, Cook Children's
  - Cindy Schwartz, MD Anderson Cancer Center
  - Mohamad Al-Rahawan, MD, MPH, Texas Tech Health Sciences Center
  - James Amatruda, MD, PhD, UT Southwestern

- Greg Aune, MD, MD Anderson -2nd Member
- Sheila Thampi, MD, Children's Hospital of San Antonio
- Juan Carlos Bernini, MD, Vannie Cook Clinic, McAllen
- Julie Luke, CPNP, Methodist Children's Hospital of South Texas
- Lisa Hartman, MD, El Paso Children's Hospital
- Virginia Harod, MD, Dell Children's Hospital
- Stan Goldman, MD, Medical City Dallas Hospital
- Barkat Hooda, MD, UTMB.

- The University Advisory Committee (UAC) unanimously approved Dr. Michelle Barton as Vice Chair. The UAC will meet January 17, 2017, to develop content for its required annual report and presentation. Texas A&M University System Chancellor John Sharp appointed Dr. Carrie Byington to serve as a member of the UAC. She assumes the position of Vice Chancellor for Health Services, Dean of the College of Medicine and Senior Vice President for the TAMU Health Science Center in January 2017. Regretfully, Dr. Michael Conn of the Texas Tech University Health Sciences Center died on November 26, 2016. His replacement appointment is pending.

Communications

Projects and Events

- *Website Redesign:* The original aggressive timeline has slipped due to competing office priorities and is now targeted for completion in February.

- *2017 Biennial Conference*: The conference will occur November 13-14, 2017, at the Austin Renaissance hotel. We are drafting requests for proposals for a registration and abstract management system. Staff has also begun considering potential speakers.

- *2016 Annual Report*: Work continues on this required report due to the Legislature by January 31, 2017.

- *Achievements Report*: A November Achievements report was issued following the Oversight Committee meeting. The February 2017 edition will be streamlined and redesigned.

- A new CPRIT Fact Sheet was created as a companion to the Achievements report for use with various audiences.

- The Communication team met December 16 to develop plans for communications activities during the first half of 2017.

- Staff continues to respond to requests for information and prepare legislative briefing materials.

- The innovative work in nanotechnology by CPRIT grantees Dr. Jinming Gao and Dr. Baran Sumer of UT Southwestern Medical Center was spotlighted in the *Dallas Morning News* on December 20, 2016. They are recipients of two Academic Research grants and a Product Development Research grant, which were mentioned. Chris Cutrone, CPRIT's Senior Communications Specialist, worked with UT Southwestern communications staff to develop the story and press release. Dr. Willson was quoted in the article.

Operations and Finance Update

The 2016 financial audit performed by McConnell & Jones, LLP was completed on December 5, 2016, with no audit findings. The audit was reviewed with the Audit Subcommittee at a specially called meeting on December 13, 2016. Pursuant to the discussion at the November Oversight Committee meeting, the Audit Subcommittee accepted the report on behalf of the Oversight Committee and it has been submitted to the Comptroller of Public Accounts and the State Auditor's Office. Copies of this audit along with the 2016 internal audits and the 2016 Annual Financial Report were mailed to Oversight Committee members for your records. The audits are also available on CPRIT's website.

Upcoming Subcommittee Meetings

The dates and times for the upcoming February Oversight Committee subcommittee meetings are listed below.

Subcommittee	Date & Time
Board Governance	February 2 at 10:00 a.m.
Audit	February 6 at 10:00 a.m.
Prevention	February 7 at 10:00 a.m.
Scientific Research	February 8 at 10:00 a.m.
Product Development	February 9 at 10:00 a.m.
Nominations	February 10 at 10:30 a.m.

An agenda, call-in information and supporting material will be sent to the subcommittees one week prior to the meeting date.

*****

CPRIT has awarded **1,114** grants totaling **$1.761 billion**

- 172 prevention awards totaling $169.1 million
- 911 academic research and product development research awards totaling $1.592 billion

Of the $1.592 billion in academic research and product development awards,

- 30.0% of the funding ($476.7 million) supports clinical research projects
- 27.1% of the funding ($431.9 million) supports translational research projects
- 24.2% of funding ($385.0 million) supports recruitment awards
- 15.0% of the funding ($239.1 million) supports discovery stage research projects
- 3.7% of funding ($59.3 million) supports training programs.

CPRIT has 10 open Requests for Applications (RFAs)

- 3 Research Recruitment
- 2 Academic Research
- 5 Prevention
- 2 Product Development Research

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO:	OVERSIGHT COMMITTEE MEMBERS
FROM:	VINCE BURGESS, CHIEF COMPLIANCE OFFICER
SUBJECT:	CHIEF COMPLIANCE OFFICER REPORT
DATE:	FEBRUARY 6, 2017

Submission Status of Required Grant Recipient Reports

A delinquent report is produced from CPRIT's grant management system (CGMS) each week; this is the primary source used by CPRIT's compliance staff to follow up with grantees. CPRIT typically has 550+ grants that are either active or wrapping up grant activities and receives an average of 570 grantee reports each month.

As of the most recent CGMS report (January 31, 2017), 18 required grantee reports from eight entities have not been filed in the system by the set due date. Of the 18 delinquent reports, six (33%) are Academic Research grants, five (28%) are Prevention grants, and seven (39%) are Product Development grants. In most cases, CPRIT does not disburse grant funds until the required reports are filed. In some instances, grantee institutions may be ineligible to receive a future award if required reports are not submitted. CPRIT's grant compliance specialists and grant accountants continue to review and process incoming reports and reach out to grantees to promptly resolve filing issues.

FSR Reviews

CPRIT's Grant Compliance Specialists performed 132 second-level reviews of grantee Financial Status Reports (FSRs) during the month of January. Thirteen (10%) FSRs required resubmission due to insufficient or inaccurate documentation submitted by the grantee. CPRIT's grant accounting staff completes the initial review of the FSRs and supporting documentation before routing them to the compliance specialists for final review and disposition.

Desk Reviews

Eleven desk reviews have been performed during the month of January. Desk-based financial monitoring/reviews are conducted during the course of grant awards to verify that grantees

expend funds in compliance with specific grant requirements and guidelines. Desk reviews may target an organization's internal controls, procurement and contracting procedures and practices, current and past fiscal audits, subcontracting monitoring, and timeliness of required grantee report submission. Grant Compliance Specialists are working with 11 grantees to remediate desk review findings.

On-site Reviews

Grant compliance staff performed two on-site reviews during the month of January covering Product Development, Research, and Prevention grants. On-site reviews typically include an examination of the grantee's financial and administrative operations, procurement and inventory procedures, personnel policies and procedures, payroll and timesheet policies, travel policies and records, and single audit compliance. Grant Compliance Specialists are working with three grantees to remediate on-site review findings.

Annual Compliance Attestation (Self-Certification)

Grantees are required to submit an annual self-certification demonstrating compliance with statutory and administrative grant requirements, CPRIT's policies and procedures, the grant contract, and the Uniform Grant Management Standards (UGMS). This opportunity to self-report, in the form of a checklist, provides a baseline of grantee compliance and allows Grant Compliance Specialists to proactively work with grantees towards full compliance prior to a desk review or on-site review. Compliance staff is currently working with three grantees to remediate attestation findings.

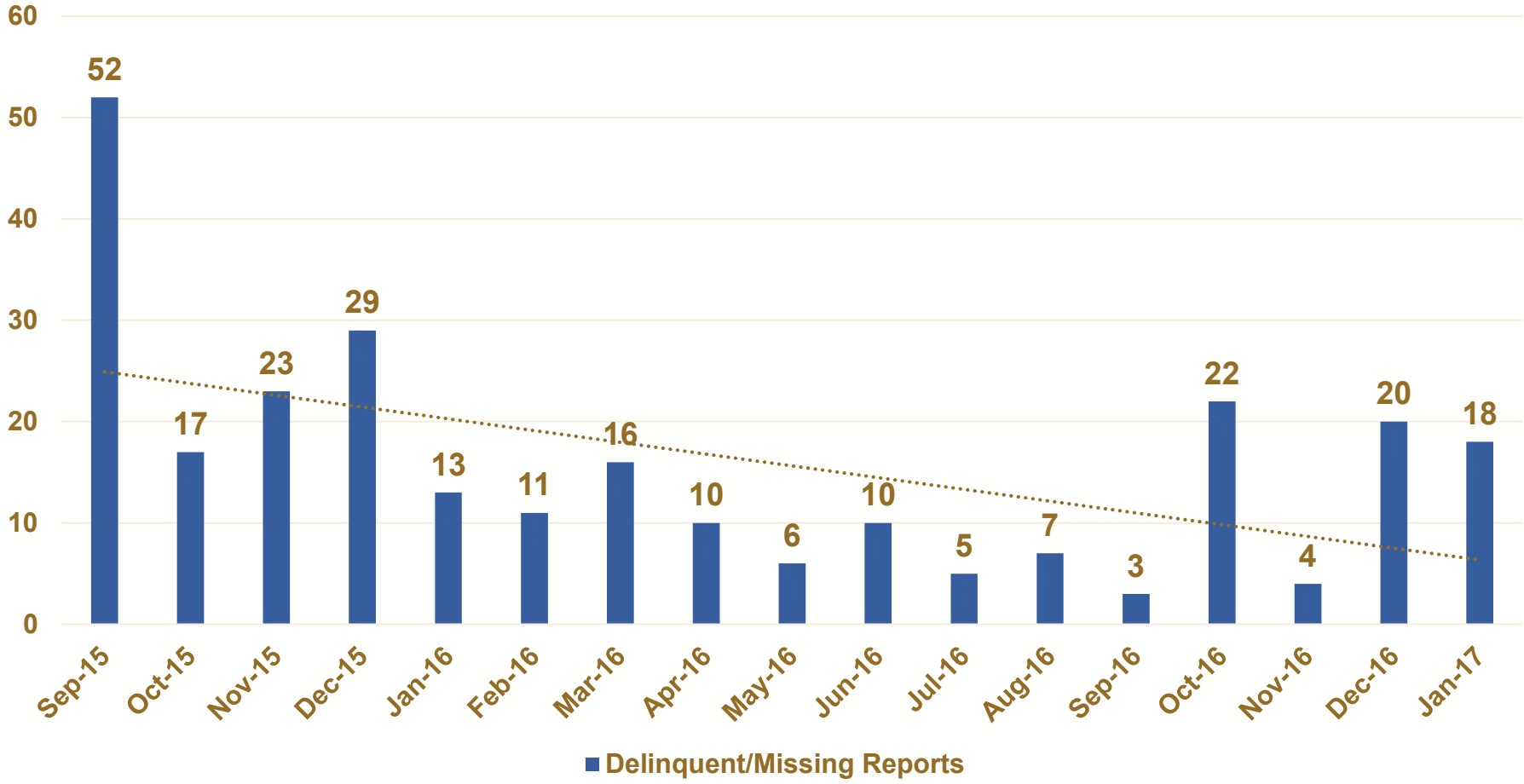
Training & Support

An Oversight Committee Ethics and Compliance training module was developed and delivered via an online portal in December 2016. The training covered the Code of Conduct and Ethics (general provisions and gifts), Conflict of Interest Policy, Non-Disclosure Agreement, and other required statements and certifications. Each section, except the overview section, contained links to substantive material and checkpoint questions. Respondents were required to successfully answer questions in each section in order to pass the training. All Oversight Committee members completed the required training prior the due date.

A grantee training webinar has been scheduled for March 9, 2017. The webinar will focus on administrative rules changes, grantee reporting requirements, compliance program activities, and the grant closeout process. This webinar is in support of the annual compliance training requirement which states that the Authorized Signing Official (ASO) and at least one other

employee from each grantee organization must attend an annual compliance training by November 1 of each year.

Grant Recipient Report Monitoring – 9-15 thru 1-17 Delinquent/Missing Reports



Reports Submitted: Approximately 6,800/Annually, Average 570/Monthly

CANCER PREVENTION & RESEARCH INSTITUTE OF TEXAS

MEMORANDUM

TO: OVERSIGHT COMMITTEE MEMBERS

FROM: JAMES WILLSON, M.D., CHIEF SCIENTIFIC OFFICER

SUBJECT: ACADEMIC RESEARCH PROGRAM UPDATE

DATE: JANUARY 25, 2017

Recruitment Impact

As displayed in table 1, to date CPRIT has recruited 113 outstanding cancer researchers to Texas who collectively enhance Texas' cancer research capacity and life science infrastructure. The table also presents the peer-reviewed funding awarded to CPRIT Scholars from Federal and other national funding agencies.

Table 1

	CPRIT Scholars Recruited to Texas		Extramural Funding to Date
First Time Tenure Track	71		$73,000,000
Rising Stars	15		$36,000,000
Established Investigators	27		$73,000,000
Total	**113**		**$182,000,000**

Accolades for CPRIT Scholars:

- ✓ 8 members National Academies of Sciences; Medicine; Engineering
- ✓ 2 Members Howard Hughes Medical Institute
- ✓ 2 National Cancer Institute Outstanding Investigators
- ✓ 2015 Lasker DeBakey Clinical Medical Research Award
- ✓ 2015 American Society of Clinical Oncology's Gianni Bonadonna Breast Cancer Award
- ✓ 2016 American Society of Clinical Oncology Distinguished Achievement Award
- ✓ 2016 O'Donnell Award in Medicine, Academy of Medicine, Engineering and Science of Texas

FY17 Academic Research Grant and Recruitment Applications Under Review

FY17 Academic Research Cycle 2 and Recruitment Cycles 17.5, 17.6, 17.6 and 17.8 are currently under review. *Table 2* displays data by applications received for two Requests for Applications (RFAs) which closed for application receipt on January 17, 2017. The Chief Scientific Officer is currently assigning applications to appropriate peer review panels. Full scientific reviews will be conducted April 19-26 in Dallas. The Scientific Review Council and Program Integration Committee recommendations will be presented at the August 16, 2017 Oversight Committee meeting.

Table 2: 17.2 Academic Research RFA Submission Data

Funding Mechanism	Applications Started	Applications Submitted
Core Facilities Support Awards	25	24
High-Impact /High Risk Awards	159	143
Total	184	167

FY 2018 Cycle 1 Request for Academic Research Applications

CPRIT announced and posted five Requests for Applications (RFAs) on January 5, 2017. Application receipt opening date is March 15, 2017 with a closing date of June 8, 2017. Grant recommendations for FY2018 Cycle 1 awards are expected to be presented to the Oversight Committee in February 2018. The five RFAs are as follows:

- **Individual Investigator Research Awards (IIRA) (RFA R-18.1 IIRA)**
  Supports applications for innovative research projects addressing critically important questions that will significantly advance knowledge of the causes, prevention, and/or treatment of cancer. Areas of interest include laboratory research, translational studies, and/or clinical investigations. Competitive renewal applications accepted.
  Award: Up to $300,000 per year.
  Duration: Maximum 3 years.

- **IIRA Childhood and Adolescent Cancers (RFA R-18.1-IIRACCA)**
  Supports applications for innovative research projects addressing questions that will advance knowledge of the causes, prevention, progression, detection, or treatment of cancer in children and adolescents. Laboratory, clinical, or population-based studies are all acceptable. CPRIT expects the outcome of the research to reduce the incidence, morbidity, or mortality from cancer in children and/or adolescents in the near or long term. Competitive renewal applications accepted.
  Award: Up to $300,000 per year. Applicants that plan on conducting a clinical trial as part of the project may request up to $500,000 in total costs.
  Duration: Maximum 4 years.

- **IIRA Computational Biology (RFA R-18.1-IIRACB)**
  Supports applications for innovative mathematical or computational research projects addressing questions that will advance our knowledge in any aspect of cancer. Areas of interest include data analysis of cellular pathways, microarrays, cellular imaging, cancer imaging or genomic, proteomic, and metabolomics databases; descriptive mathematical models of cancer, as well as mechanistic models of cellular processes and interactions and use of artificial intelligence approaches to build new tools for mining cancer research and treatment databases.
  Award: Up to $300,000 per year.
  Duration: Maximum 3 years.

- **IIRA Prevention and Early Detection (RFA R-18.1-IIRAP)**
  Supports applications for innovative research projects addressing questions that will advance knowledge of the causes, prevention, early-stage progression, and/or early detection of cancer. Research may be laboratory, clinical, or population-based, and may include behavioral/intervention, dissemination or health services/outcomes research to reduce cancer incidence or promote early detection. Competitive renewal applications accepted.
  Award: Up to $300,000 per year for laboratory and clinical research; Up to $500,000 per year for population-based research.
  Duration: Maximum 3 years.

- **IIRA Clinical Translation (RFA R-18.1 – IIRACT)**
  Supports applications for innovative clinical research that will lead to a better understanding of the clinical efficacy of a cancer therapy or diagnostic device. Applications submitted under this mechanism should propose innovative clinical studies that are hypothesis-driven and involve patients enrolled prospectively on a clinical trial or involve analyses of biospecimens from patients enrolled on a completed trial for which the outcomes are known.
  Award: Up to $400,000 per year for a maximum of 3 years for laboratory and clinical research; Up to $600,000 per year for up to 4 years if research includes the conduct of clinical trials.
  Duration: Maximum 4 years.

Advisory Committee Meetings

Both the University Advisory Committee (UAC) and Advisory Committee for Childhood Cancers met in January (January 17, 2017 and January 23, 2017 respectively). The agenda for both meetings included developing content for required annual reports and presentations to the Oversight Committee. Dr. Rice attended and actively participated in both meetings.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO: OVERSIGHT COMMITTEE MEMBERS

FROM: JAMES WILLSON, MD, CHIEF SCIENTIFIC OFFICER

SUBJECT: ACADEMIC RESEARCH AWARD FUNDING MODIFICATION RECOMMENDATION

DATE: FEBRUARY 1, 2017

Summary and Recommendation

The Academic Research Program and the Program Integration Committee requests funding for Award RP170259 be increased to $2,648,151 to accurately reflect the reduction in budget that was recommended by the Scientific Review Council on October 13, 2016. RP170259 was approved by the Oversight Committee on November 16, 2016.

Discussion

The Scientific Review Council (SRC) and Program Integration Committee recommended five Research Training Awards totaling $14,866,638 with subsequent approval by the Oversight Committee on November 16, 2016. For one of these awards (RP170259) the SRC recommended the following budget reductions: Reduce number of trainees from 9 to 6 Post-Doctoral trainees per year, reduce funding for training program manager to 50% (from proposed 100% FTE) and reduce budget to reflect a reduction of 3 trainees/year . The award amount approved by the Oversight Committee on November 16, 2016 ($2,071,403) reflected the budget reduction.

When calculating the revised award amount, CPRIT did not include fringe benefit costs for 6 Post-Doctoral trainees. The Academic Research Program is requesting the funding for Award RP170259 be increased by $576,748 to $2,648,151 to correct this omission. The revised amount incorporates the SRC recommendations and is less than the budget originally requested in the grant application.

Research Training Award Recommendation

ID	Score	Title	PI	Organization	Current Award	Recommended Award	Priorities
RP170259	2.6	CPRIT Cancer Prevention Research Training Program	Chang, Shine	The University of Texas M. D. Anderson Cancer Center	**$2,071,403**	**$2,648,151**	Prevention

PO Box 12097 | Austin, Texas 78711 | PH: 512-463-3190 | F: 512-475-2563 | cprit.texas.gov

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO:	OVERSIGHT COMMITTEE MEMBERS
FROM:	REBECCA GARCIA, PHD, CHIEF PREVENTION AND COMMUNICATIONS OFFICER
SUBJECT:	PREVENTION PROGRAM UPDATE
DATE:	FEBRUARY 6, 2017

FY 2017 Cycle 1

Five RFAs for Cycle 17.1 were released in May 2016. Applications were due August 30. We received 36 applications. After administrative review, 5 were withdrawn and 31 applications requesting $36,684,532 were assigned to the review panels. Two peer review panels met December 5 - 8 in Dallas.

The Prevention Review Council (PRC) conducted a programmatic review January 20 and forwarded their recommendations to the Program Integration Council (PIC). The PRC considers geographic distribution, cancer type, type of project and potential for impact in addition to scores assigned by the peer review panels. The PIC met January 31 and will forward their recommendations to the Oversight Committee for consideration on February 15, 2017.

FY 2017 Cycle 2

The following RFAs were released on November 17:

- Evidence-Based Cancer Prevention Services
- Dissemination of CPRIT-Funded Cancer Control Interventions
- Cancer Prevention Promotion and Navigation to Clinical Services
- Colorectal Cancer Coalition
- Tobacco Control and Lung Cancer Screening

Submissions are due March 2 with peer review meetings taking place in June. Recommendations will be presented to the Oversight Committee in August 2017.

Dr. Garcia and Ms. Magid held a webinar Jan 11 to present the FY2017 cycle 2 funding opportunities and answer questions. 120 people participated in the webinar.

Other activities

The prevention program performance measures report for the Legislative Budget Board was submitted January 9.

Senate Bill 200, Section 2.32, 84th Legislature, 2015 directed HHSC to collaborate with DSHS and CPRIT to develop a strategic plan to reduce the morbidity and mortality from HPV associated cancers. The final report has been submitted to the Legislature and is available on the HHSC website at https://hhs.texas.gov/sites/hhs/files//Human-Papillomavirus-Strategic-Plan_1.pdf

Dr. Garcia and Ms. Magid conducted a webinar for the American Cancer Society (ACS) Texas Health Systems Department on Jan. 11. ACS had requested this webinar for their staff who work with CPRIT grantees and were interested in learning more about CPRIT.

Meetings

- As a member of the advisory board, Dr. Garcia participated in the Jan 9 Texas Health Improvement Network Advisory Meeting in Austin. The network was established to address the urgent health care challenges and improve the health care system in the state.
- Dr. Garcia and Ms. Magid met with new staff of the Texas Comprehensive Cancer Control Program (TCCCP) on Jan 12. This program receives funding from the Centers for Disease Control and Prevention (CDC) to implement comprehensive cancer control in Texas by increasing the coordination, integration and implementation of cancer activities in the state through a network of cancer stakeholders at the community level.
- Dr. Garcia presented an update on CPRIT activities at the MD Anderson Executive Advisory Panel Meeting in Houston on Jan 19.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO: OVERSIGHT COMMITTEE MEMBERS

FROM: REBECCA GARCIA, PH.D. CHIEF PREVENTION AND COMMUNICATIONS OFFICER

SUBJECT: COMMUNICATIONS UPDATE

DATE: FEBRUARY 15, 2017

The following report provides an overview of the agency's communications activities from Nov. 17, 2016 through Feb. 15, 2017.

Earned Media

The communication team conducted individual media outreach to secure positive coverage for CPRIT, including a feature in the Houston Business Journal on Dec. 9. The Houston Chronicle also featured CPRIT in an article on Feb. 3 about a poll commissioned by the American Cancer Society regarding the importance of CPRIT in Texas.

A 2017 media pitch plan was developed to highlight CPRIT's impact correlating with each cancer awareness month. For Cervical Cancer Awareness Month in January, the team secured seven broadcast and two online stories featuring Dr. Rebecca Garcia in the Rio Grande Valley as well as CPRIT-funded programs through Texas Tech Health Sciences Center in the El Paso market.

Grant Awards Announcement: Following the Oversight Committee's approval of grant awards at its November meeting, CPRIT distributed a press release on Nov. 16 to local, regional and national outlets announcing 46 academic research grants and two product development research grants.

Coverage: (Nov. 5, 2016 – Feb. 6, 2017)

- 25 articles featured CPRIT
- 80 additional articles mentioned CPRIT (stories primarily focused on work of grantees)

Coverage Highlights: (see clipped articles following report)

- November 17, 2016, *Xconomy*, Bellicum, Molecular Templates Granted $32M for Immuno-Oncology Work
- November 21, 2016, *Houston Business Journal,* San Francisco Biotech Company

Confirms Relocation to Houston Following CPRIT Grant

- December 9, 2016, *Houston Business Journal,* Texas Innovations in Health Care: Houston Institutions Lead the Way in CPRIT Funding
- December 19, 2016, *Dallas Morning News,* UT Southwestern Researchers Seek to Light up Cancer Cells so Surgeons can Better see Where to Cut
- January 17, 2017, *KVEO-TV NBC McAllen,* Cervical Cancer Mortality Rates High Along Texas-Mexico Border
- January 24, 2017, *KDBC-TV CBS El Paso,* Borderland Women 30% More Likely to Die of Cervical Cancer
- February 3, 2017, *Houston Chronicle,* Poll: Texans Favor Extension of Cancer-Fighting Agency

Projects and Events

- Press Briefings: CPRIT program officers briefed reporters from Chemical and Engineering News, the Austin American Statesman and the Dallas Morning News on CPRIT activities and its programs. A briefing is being rescheduled with the San Antonio Express News/Houston Chronicle. Chemical and Engineering News ran a story on CPRIT for their February edition.
- Dr. Garcia and Chris Cutrone traveled to El Paso to coordinate media interviews at Texas Tech Health Science Center El Paso highlighting the cervical cancer prevention work of CPRIT grantee, Dr. Shokar. Oversight Committee member Dee Margo was also present.
- Dr. Garcia and Dr. Argenbright from UT Southwestern Medical Center will be interviewed in February for National Cancer Prevention month.
- The 2016 Annual Report has been completed and was delivered to the Legislature on January 27, 2017.
- The Achievements Report is being redesigned for 2017, and a new version of it will be released in February.
- A new one page report entitled, *Texans Conquer Cancer*, was created as a companion piece to the Achievements report for use with various audiences, and is available for download on the CPRIT website.
- Staff continues to respond to requests for information and prepare legislative briefing materials.

Xconomy

Bellicum, Molecular Templates Granted $32M for Immuno-Oncology Work

David Holley
November 17th, 2016

Xconomy Texas — *Austin* — The Cancer Prevention and Research Institute of Texas has awarded about $32 million in grants to two Texas immuno-oncology drug developers working on blood cancer treatments.

Bellicum Pharmaceuticals (NASDAQ: **BLCM**) has been awarded $16.9 million to work on developing its T-cell **therapy** called BPX-501 for acute myeloid leukemia, says CPRIT, the cancer research institute. **Houston-based Bellicum went public in 2014**, raising almost $140 million in an IPO that it used for new hires, its facilities, and clinical trials. It has four ongoing clinical trials for BXP-501.

As Xconomy has previously reported, **Bellicum's BPX-501 can be used** in stem cell **transplants**, and is designed to make other potentially risky cancer treatments safer for patients by preventing graft-versus-host disease—when the body thinks a transplanted cell is foreign.

Meanwhile, Molecular Templates, which is based just north of Austin in Georgetown, TX, is receiving $15.2 million for its treatment for multiple myeloma. The private company is developing **antibodies** that use the immune system to find and kill cancer cells that express a glycoprotein, CD38. The **treatment** is listed as preclinical on Molecular Templates' Website.

Former employees of ImClone Systems now lead Molecular Templates, **including** its president and chief financial officer, Jason Kim, and its CEO and chief scientific officer, Eric Poma. The company **raised** a $12 million Series C funding round in 2014 from AJU IB Investment, Excel Ventures, and Santé Ventures.

The new grants were funded through CPRIT's product development research program. CPRIT also gave out $53 million in other early stage research grants to various Texas academic institutions.

Those grants include studies looking at areas of cancer such as large B-cell lymphoma and genetic mutations in breast cancer at the University of Texas Health Science Center at San Antonio, as well as cancer imaging, DNA repair, and advanced uveal melanoma (among many others) at the M.D. Anderson Cancer Center in Houston. (You can read about all the grants **here**.)

http://www.xconomy.com/texas/2016/11/17/bellicum-molecular-templates-granted-32m-for-immuno-oncology-work/

HOUSTON BUSINESS JOURNAL

San Francisco biotech company confirms relocation to Houston following CPRIT grant

Nov 21, 2016, 8:09am CST

Joe Martin
Reporter
Houston Business Journal

San Francisco-based Ruga Corp. has confirmed its planned relocation to Houston and expects to hire following the acceptance of a $20 million grant from the Cancer Prevention and Research Institute of Texas.

Ruga, which has rebranded to Aravive Biologics Inc., was awarded the grant in November 2015 to further the development of its myeloid leukemia treatment. The company has been in transition over the past couple months and plans to continue to move some of its staff over in the coming months, said Aravive CEO Ray Tabibiazar.

"We have had large support from the local biotech organizations like BioHouston," Tabibiazar said. "It's a small biotech community, so everyone knows each other, and everyone's trying to help out. It's been good."

For now, Aravive is housed at 2 Houston Center, but the company expects to relocate in January. Tabibiazar declined to disclose where Aravive will settle, as the company is finalizing an agreement for new space. Aravive expects to have 18 people split between Houston and San Francisco, he added.

Aravive is a preclinical company focusing on acute myeloid leukemia. It expects to submit an investigational new drug application with the Food and Drug Administration by the end of 2017.

Aravive's drug development status is one of the primary reasons the company decided to accept the grant and relocate, Tabibiazar said.

"(Houston) has a very vibrant cancer research environment," Tabibiazar said. "Houston is probably one of the top, if not the top, place to do drug development. It will be fun to have these interactions."

http://www.bizjournals.com/houston/news/2016/11/21/san-francisco-biotech-company-confirms-relocation.html

HOUSTON BUSINESS JOURNAL

Texas Innovations in Health Care: Houston institutions lead the way in CPRIT funding

Dec 9, 2016, 12:20pm CST

Joe Martin

Reporter
Houston Business Journal

Houston companies, research institutions and hospitals have received more than $900 million from the Cancer Prevention and Research Institute of Texas since 2009, helping elevate Houston as a leading research hub in the fight to eliminate cancer.

CPRIT began under former Gov. Rick Perry as a public funding source aimed at making Texas the leading state for cancer innovation and research. So far, the program has awarded $1.68 billion over 1,070 grants to 98 institutions, organizations and companies, and will provide $3 billion in funding before it winds down, according to CPRIT.

Houston, with its massive conglomeration of health care institutions, naturally became a leader in the program, bringing in more CPRIT dollars than any other hub in Texas. Two of Houston's top cancer research centers, including the University of Texas M.D. Anderson Cancer Center and the Baylor College of Medicine, which is not affiliated with Baylor University or Baylor Scott & White in Dallas, have raised a combined $529.38 million since 2009, according to Houston Business Journal research.

The grants stretch across a broad array of uses. Rice University, for example, has received more than $40 million in CPRIT funding. While that is much smaller in comparison to M.D. Anderson, it's been instrumental in bolstering Rice's faculty. In May 2015, for example, Rice was able to recruit Natasha Kirienko, a Harvard Medical School and Massachusetts General Hospital cancer researcher, to Houston through a $2 million CPRIT grant. There are dozens of other examples of CPRIT grants being used to attract researchers to Houston and elsewhere in the state.

For the bigger players, CPRIT is used as a significant means for funding research. M.D. Anderson, which has been rated as the No. 1 cancer hospital in the country, has used its $317 million in grants to recruit researchers from all over the country to Houston, and moved forward numerous forms of research in breast, lung, prostate and other cancers.

Perhaps one of the more promising aspects for CPRIT is its ability to attract and retain biotechnology companies in the Lone Star State. In Houston alone, CPRIT has helped draw at least 14 companies since its formation, one of which – Bellicum Pharmaceuticals (Nasdaq: BLCM) – has gone public.

"If you're in the oncology space, CPRIT has got to be No. 1 on your list of organizations to talk to," said David Arthur, CEO of Houston-based Salarius Pharmaceuticals, which moved here after a CPRIT grant.

How they do it

Houston's ability to receive and handle nearly half of all CPRIT dollars is due in large part to its long history of being the home to clinical health care research. Institutions like M.D. Anderson, the Houston Methodist Research Institute, Baylor College of Medicine, the University of Texas Medical Branch in Galveston and others have cultivated reputations as some of the better research institutions around the country – cancer and otherwise. That has created a strong ecosystem built around clinical research that includes thousands of researchers, doctors and lab techs clamoring for cash to fund their work.

"You have a situation where M.D. Anderson in Houston and (University of Texas Southwestern Medical Center in Dallas) have massive research infrastructure. They will submit, in any round of funding, requests (for) 40 applications. It's a numbers game," CPRIT CEO Wayne Roberts said.

Granted

The Houston metro area is by far the leader in the state with respect to the number and amount of grants awarded by the Cancer Prevention & Research Institute of Texas. Researchers – including companies and academic institutions – in the Houston area have received more than 500 CPRIT grants totaling a little more than $900 million, according to data from CPRIT.

Below are the top 10 grant recipients from the Houston area.

Joe Martin covers technology, money and law for the Houston Business Journal. Follow him on Twitter for more.

Organization	No. of awards	Total amount
University of Texas M.D. Anderson Cancer Center	218	$316.9 million
Baylor College of Medicine	154	$213.1 million
University of Texas Health Science Center at Houston	43	$57.2 million
Rice University	21	$43.1 million
Methodist Hospital Research Institute	16	$34.1 million
Statewide Clinical Trials Network of Texas	1	$25.2 million
University of Texas Medical Branch at Galveston	25	$23.4 million
University of Houston	16	$21.9 million
Ruga Corp.	1	$20 million

Source: Cancer Prevention & Research Institute of Texas. Current as of October 2016

http://www.bizjournals.com/houston/news/2016/12/09/texas-innovations-in-health-care-houston.html

DALLASNEWS
Powered by The Dallas Morning News

UT Southwestern researchers seek to light up cancer cells so surgeons can better see where to cut

Sabriya Rice, Business of Healthcare Reporter

HEALTH CARE DEC 19

Researchers in Dallas are developing a new technology that they hope will literally shed light on a problem that has frustrated cancer surgeons worldwide.

The removal of cancerous tumors through surgery is an often life-saving treatment option--- but it's not an exact science.

"It's always a balance between taking out too much or too little tissue," explained Dr. Baran Sumer, a surgeon focused on cancers of the head and neck at the University of Texas Southwestern Medical Center.

Oncologists want to remove as much cancerous tissue from the body as possible so the disease does not continue to grow or spread. But removing too much can result in deformations and other complications for patients.

Sumer and a team of researchers from the Simmons Comprehensive Cancer Center developed a technology called a pH Nanosensor, an injection that seeks out cancerous areas in the body and causes them to light up.

The goal is to help surgeons more precisely determine how much to cut and possibly reduce the need for extra procedures.

In findings published Monday in Nature Biomedical Engineering, the injection had positive results in a study of mice with tumors of the head, neck and breast.

While promising, it's important to note that the research is very preliminary. It's too early to tell whether it will have the same effect in humans.

"That's going to be the big question mark," said Dr. Otis Brawley, chief medical officer for the American Cancer Society. "There are many reasons on a immunologic and cellular level why it might work in a mouse, but not in human."

Still, local researchers may be on the forefront understanding the technology's potential.

In 2015, they were awarded a grant from the National Cancer Institute totalling more than $1.8 million for a five-year period.

UTSW has also received two research grants from the Cancer Prevention and Research Institute of Texas. Dallas-based OncoNano Medicine, a UTSW spinoff company that wants to commercialize the technology, has received $6 million in CPRIT funding.

"They have a pathway toward realizing the potential," said CPRIT's chief scientific officer, Dr. James Willson. The researchers have focused on head-and-neck and breast cancers, but the problem of precision is faced by surgeons for many different types of cancers, he said.

Here's how it works. Cancer tumors have a pH that is slightly more acidic than normal tissue. "It's one of the few distinguishing characteristics that cuts across all cancer types," explained Jinming Gao, PhD, a bioengineer working on the the design aspect of the probe.

The fluorescent sensor targets the different pH signals, thus making them glow so they are easier to distinguish from normal tissue.

The recent study found that surgeons were able to excise more of the cancerous tissue in most of the 18 mice after they had been illuminated. The long-term survival of the mice was also improved.

While many questions remain, UT Southwestern is among a handful of entities turning to technologies that can light up cancer cells.

For example, Seattle Children's Hospital is researching a "molecular flashlight" for brain cells. Researchers from North Carolina and Massachusetts developed a probe called LUM015 that causes concentrations of cancerous tissues to become fluorescent.

The Dallas researchers are seeking approval from the Food and Drug Administration to begin early-stage clinical trials to test their injection in humans in 2017 and identify potential side effects.

Future studies will then need to evaluate whether attempts to spot and remove all areas of disease is effective with metastasized cancers, added Brawley. "Surgical cherry-picking," he says, has not helped patients in the past, though he encourages additional research.

Finding cancers cells that have metastasized and spread throughout the body has been one of the biggest challenges for cancer detection and diagnosis. "Drugs frequently don't go to every place the tumor exists. It hits about 95 percent…and that's not enough," Brawley said.

http://www.dallasnews.com/business/health-care/2016/12/19/ut-southwestern-researchers-seek-light-cancer-cells-surgeons-can-better-see-cut

Cervical Cancer Mortality Rates High Along Texas-Mexico Border

By: Amy Martinez |
Posted: Jan 17, 2017 04:08 PM CST
Updated: Jan 17, 2017 07:02 PM CST

RIO GRANDE VALLEY, Texas - Women living along the Texas-Mexico border have a 30% higher Cervical Cancer mortality rate. That is compared to non-border regions. One of the biggest culprits is a lack of education on the topic.

We spoke to doctors who say they're working on new ways to reduce the risk of Cervical Cancer.

Dr. Rebecca Garcia, Cancer Prevention and Research Institute of Texas said, "We're funding a lot of programs across the state that will give women who don't have other resources, access to Cervical Cancer screening."

Regular Cervical Cancer screenings are one of the easiest ways to detect and prevent cancer in women.

But with so many living without health insurance in the valley, doctors are taking matters into their own hands.

Dr. Carlos Herrera, Gynecologic Oncology said, "With the new DNA technology, we can detect the virus at very small quantities. And hopefully one day, we will have kits just like pregnancy tests that will detect women who have this virus."

Dr. Herrera says our close proximity to Mexico is another reason why we're seeing a growing number of women diagnosed with the disease.

"We also mimic what's going on over there. That means, not a lot of screening, not a lot of education on the matter, and what happens is the rate of cervical cancer is exactly like it's a third world country."

The Cancer Prevention and Research Institute of Texas is currently funding programs for the Human Papillomavirus vaccine because HPV is one of the main causes of Cervical Cancer.

Dr. Rebecca Garcia, Cancer Prevention and Research Institute of Texas, "It's recommended children ages 11-12 get vaccinated against the HPV virus. And this will really protect your children from getting cancer in the future."

Currently doctors say booster shots to protect against HPV and Cervical Cancer are not necessary.

Smoking and having multiple sexual partners significantly increase the chance of contracting HPV and Cervical Cancer.

http://www.rgvproud.com/news/local-news/cervical-cancer-mortality-rates-high-along-texas-mexico-border/641579770

Borderland women 30% more likely to die of cervical cancer

by Ashley Claster | Tuesday, January 24th 2017

EL PASO, Texas (CBS4/KFOX14) -- — Women living in the Borderland are significantly more at risk to die of cervical cancer, particularly Hispanics.

El Pasoan Candra Viezcas, who has been through cervical cancer before, hopes other women get screened. Cervical cancer is preventable through vaccines and regular Pap smears.

"I encourage them not to wait any longer to save their lives at no cost," Viezcas said.

The Texas Tech Health Science Center of El Paso said women living along the border between Texas and Mexico have a 30 percent higher cervical cancer mortality rate compared with non-border counties.

"About 40 percent of women in our area don't have access to health insurance," said Dr. Navkiran Shokar, from TTUHSC.

Shokar saidx there are about 50 cases of cervical cancer in El Paso County every year, and about 15 of those women do not survive.

"The main barrier to women in this community to getting tested is access to services because lack of insurance and lack of programs. So this program really addresses that major barrier," Shokar said.

TTUHSC has two programs funded by the Cancer Prevention and Research Institute of Texas. Through those programs, the center has been able to provide 1,700 Pap smears to women in El Paso and Hudspeth County, and 1,400 vaccinations for HPV, which causes cervical cancer.

Viezcas said she was glad to have that program around.

"Here they do the exams for free and they are very friendly. They make you feel welcome," Viezcas said.

The center helps women before, during and after a diagnosis. It even provides transportation for women who are far away from medical services. The center provides free Pap smears, follow-up testing and diagnostic testing, as well as free HPV vaccines. Vaccines are recommended for girls and boys between the ages of 9 and 26.

http://cbs4local.com/news/local/borderland-women-30-more-likely-to-die-of-cervical-cancer

HOUSTON CHRONICLE

Poll: Texans favor extension of cancer-fighting agency

By Todd Ackerman, Houston Chronicle | February 3, 2017

Four years after scandal almost caused it to be dismantled and four years before it's scheduled to close down, a strong majority of Texans favor the continuation of the state cancer-fighting agency, according to a new poll.

The poll, commissioned by the American Cancer Society, found nearly three-fourths of respondents said it is more important to renew the taxpayer-funded, $3 billion program than to save tax dollars. Seventeen percent favored saving taxpayer dollars and 9 percent either said they didn't know or declined to answer.

"There are few issues that unite voter opinion in Texas like the desire for our state to remain at the forefront of the fight against cancer and to maintain the work of the Cancer Prevention and Research Institute of Texas," said Cam Scott, government relations director of the Texas chapter of ACS' Cancer Action Network. "With 116,200 Texans estimated to receive a cancer diagnosis this year, it is critical the state maintains this momentum to end cancer. It is an all-too common enemy that necessitates a sustained response from our elected leaders."

The support for CPRIT's extension was up slightly from the last time the poll was taken. In December 2014, 70 percent preferred the program's continuation; in the new poll, 74 percent did.

Under current statute, CPRIT is scheduled to award its last grants by Aug. 31, 2020, and shut down in 2021. Currently, it still has $1.33 billion left to award.

The Legislature will consider conflicting bills about CPRIT's future this session. Rep. Sarah Davis, R-Houston, has sponsored a bill to extend the agency's lifespan by two years and Sen. Charles Schwertner, R-Georgetown, is again expected to file one calling for CPRIT to develop a plan for "self-sufficiency" so voters will not be asked to pony up another $3 billion when it ends.

CPRIT was launched in 2009 after voters approved a bond issue in 2007 to fund the program. The legislation calls for the agency to allocate $300 million a year for 10 years for cancer prevention and efforts to develop cures. (Because of the yearly ceiling, CPRIT would leave $150 million on the table if it is not extended.)

The program ran smoothly until 2012 to early 2013, when mismanagement and improprieties were discovered in three grants totaling $56 million. Top elected officials subsequently shut the program down for 10 months and pulled its funding from the state budget before restoring it at the 11th hour. A reform bill passed at the same time removed its governing board and installed more stringent safeguards. There have been no problems since then.

Go here to view more details on the poll, including numbers that show it enjoys strong support from men, women, young, old and "even Tea Party supporters."

http://www.houstonchronicle.com/local/prognosis/article/Three-fourths-of-Texans-favor-extension-of-CPRIT-10906809.php?cmpid=gsa-chron-result

RESEARCH FUNDING

A vote against cancer

Texas institute set up by referendum is halfway through its cancer R&D funding mission

ANN M. THAYER, C&EN HOUSTON

In 2007, 61% of Texas voters approved Proposition 15, thereby authorizing the state government to issue up to $3 billion in bonds over 10 years to fund cancer prevention, research, and product commercialization efforts. Their votes also created the Cancer Prevention & Research Institute of Texas (CPRIT, pronounced see-prit) to coordinate this initiative.

CPRIT at a glance

- **Year founded:** 2007
- **Headquarters:** Austin, Texas
- **Number of grants awarded:** 1,114
- **Value of grants awarded:** $1.76 billion
- **Grants by type: 82%** research, **15%** prevention, **3%** product development
- **Researchers recruited:** 127

Note: Figures reported as of Jan. 17, 2017.

The first call for applications went out in 2009, and CPRIT began awarding funds in 2010. But within two years, the institute was engulfed in a controversy over its peer review process. The storm involved scientific, commercial, and political interests and caused a halt in funding for about a year. Ultimately, legislative reforms that addressed the grant and oversight processes got CPRIT running again in 2013.

Despite the setback, CPRIT's mission has remained expediting "discoveries and innovations across Texas to reduce the burdens of cancer." So far, third-party contractors report that the institute's investment in research, screening, and related activities has been generating substantial economic impacts. Ten years after the vote, CPRIT says it is helping position Texas as a national leader in cancer research.

Texas is not the first state where voters have chosen to fund scientific R&D. In 2004, 59% of voters passed the California Stem Cell Research & Cures Initiative. The act authorized $3 billion in bonds and created the California Institute for Regenerative Medicine, which has disbursed about $1.4 billion toward research and facilities.

What makes CPRIT different is its disease focus. "We strive every year to demonstrate that we are creating a cancer research ecosystem in which the research is leading to meaningful impacts and translation into practice," Chief Scientific Officer James K. V. Willson says. This ecosystem includes research programs, prevention services, clinical studies, companies, education, and jobs.

Recipients so far include 98 academic institutions, nonprofit organizations, and private companies, all located in Texas. CPRIT can distribute up to $300 million in grants per year for a total of $3 billion by 2021. The institute reached the $1.5 billion halfway point in 2016. Bills are in the Texas legislature to push the sunset date from 2021 out to 2023, which would give CPRIT time to disburse the full amount.

About 10% of CPRIT's annual merit-based grant funding goes toward prevention activities, and about 18% targets product development. The remaining 72% is put into academic research. It's an amount roughly equal to what is invested in Texas by the National Cancer Institute (NCI) annually.

About two-thirds of CPRIT's academic research awards are to individual investigators, and the rest are to institutions for recruiting scientists. The success rate is about 10% for individuals and closer to 30% for facility and faculty recruitment grants.

"CPRIT funds have catalyzed something quite extraordinary that happened in Texas in the past five years," Willson says. The state's major academic institutions, including several University of Texas sites, have dramatically boosted their cancer-related research expenditures. These gains have come not only through CPRIT but also increasingly from sources outside Texas.

As a former cancer center director at the UT Southwestern Medical Center, Willson witnessed firsthand a transformation he says was enabled by CPRIT seed funding. Since CPRIT's founding, centers at UT Southwestern and Baylor College of Medicine have been recognized as comprehensive NCI centers. The UT MD Anderson Cancer Center was the only Texas institution to already have the designation. Along with the UT Health Sci-

Recruited

CPRIT grants have helped attract these leading scientists to Texas institutions.

James P. Allison, UT MD Anderson Cancer Center

Gang Bao, Rice University

Daniel J. Leahy, UT Austin

K.C. Nicolaou, Rice University

John L. Wood, Baylor University

CREDIT: COURTESY OF UT AUSTIN (ALLISON & LEAHY); JEFF FITLOW/RICE UNIVERSITY (BAO); RICE UNIVERSITY (NICOLAOU); COURTESY OF JOHN WOOD

ence Center at San Antonio, Texas is home to four of 69 NCI centers and ranks fourth after California (10), New York (seven), and Pennsylvania (five).

CPRIT has also raised Texas's profile by providing $385 million to help state institutions recruit 127 cancer researchers, including seven National Academies members, Willson points out. Through its Scholars program, single awards of up to $6 million—and sometimes a bit more—target the move of prominent established investigators, rising stars, and first-time, tenure-track faculty and their labs to Texas.

This funding helps add to research infrastructure and the ability to train new scientists. UT Austin, for example, is building a cryo-electron microscope facility after CPRIT grants brought in Daniel J. Leahy, a prominent structural biologist previously at the Johns Hopkins University School of Medicine, and David Taylor, who was a postdoc at the University of California, Berkeley, working on structural studies of CRISPR gene-editing systems. The institute has also supported 30 multiuser facilities.

Meanwhile, CPRIT's product development program has invested $329 million through 31 grants, mostly to small companies. About 10% of applicants are selected for awards. As part of the final contracts, grantee matching funds have added another $165 million, and the follow-on funding that these companies have raised totals more than $1.3 billion.

Most of CPRIT's commercial investments support new drugs, along with some diagnostics and medical devices. "Research provides the scientific underpinnings upon which technologies are developed, but until those technologies are developed, we don't advance cancer care," says Mike Lang, CPRIT's chief product development officer.

Houston-based Bellicum Pharmaceuticals, which has licenses to technology from Baylor College of Medicine, has received two grants from CPRIT. A $16.9 million grant awarded in November will support Phase II clinical studies of its lead T-cell therapy, BPX-501. A three-year, $5.6 million grant in 2011 supported preclinical development.

The first award helped catalyze Bellicum's Series B financing in 2014 and the expansion of the company, says Chief Executive Officer Thomas J. Farrell. "The level of diligence that CPRIT had done provided some reassurance to the private investors we were working with at the time," he recalls. By the end of 2014, the company went public. CPRIT support "was directly and objectively instrumental in getting to where we are today," he says.

Aravive Biologics is literally where it is because of CPRIT. In accepting a $20 million grant last year, the company, formerly known as Ruga Corp., agreed to relocate to Houston. The money will help move its high-affinity, soluble Fc-fusion protein, Aravive-S6, to the clinic as a possible treatment for acute myelogenous leukemia and solid tumors.

With technology originating from Stanford University, Aravive is looking forward to partnering with academic centers in Texas to conduct clinical trials, CEO Ray Tabibiazar says. "Texas has a very good track record of being able to recruit patients and do the clinical research required for successful drug development."

"There is an amazing amount of knowledge and expertise in cancer and outside of cancer," along with capital to support the science, says Tabibiazar, who has a medical and venture investing background. But unlike established bioscience hubs, Texas currently lacks a management base with a track record of creating and building multiple companies, he says. That expertise may develop given time.

Beyond its primary mission, Lang says, "CPRIT also has the opportunity to be an economic stimulus to the state, and we endeavor to do that." In fact, CPRIT has relocated 10 companies to Texas and says it has helped create nearly 400 jobs while maintaining more than 4,000. In 2015, CPRIT's activities generated about $378 million in combined state and local tax receipts, according to an outside analysis.

Returns to the state are baked in, participants say, because CPRIT's grants come with strings attached. As with many academic licenses, access to intellectual property for research purposes may be stipulated. And for companies and institutions, milestones are set along with return obligations, often in the form of royalties on sales of any eventual products aided by CPRIT funding.

Companies describe a multistep application process that is extensive, tightly managed, and up to a year long. It involves a review of the company's science, intellectual property, business, and development plans, as well as any impacts on Texas. To minimize conflicts of interest, multiple panels of out-of-state experts review each aspect.

The whole submission and review process is probably more extensive than those required for a lot of venture investments, Tabibiazar says. "But it is understandable because it is taxpayer money and, at the end of the day, you are accountable to the people of Texas." ■

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO:	OVERSIGHT COMMITTEE MEMBERS
FROM:	MICHAEL LANG, CHIEF PRODUCT DEVELOPMENT OFFICER
SUBJECT:	PRODUCT DEVELOPMENT PROGRAM REPORT
DATE:	FEBRUARY 8, 2017

Summary

The Product Development Research Program's first review cycle of FY 2017 concluded without any award recommendations from the Product Development Review Council (PDRC). The second cycle of FY 2017 is accepting applications, with recommendations expected for the August Oversight Committee. Memos reflecting the program policies changes approved by the Product Development Subcommittee at the January 16th meeting are attached for your review. Awards contracts for the most recent awards are awaiting execution. Proposed changes to two contracts will be discussed at the February 13 subcommittee meeting.

Review Cycle Status

FY 2017 Cycle 1 Product Development Research Applications

Applicants submitted 25 proposals by the August deadline. After review and discussion at the screening teleconference, the panel members selected eight of the 25 companies to present at the in person peer review meeting. Three companies were selected for due diligence after the in-person presentations. The PDRC reviewed the diligence reports and did not recommend any of the three for funding.

FY 2017 Cycle 2 Product Development Research Applications

Applicants may submit proposals for FY 2017 Cycle 2 Product Development Research awards through February 9, 2017. Peer reviewers will evaluate the applications between March and July 2017. Grant award recommendations will be presented for Oversight Committee consideration at the August 2017 meeting.

Program Policies

At its meeting on January 16, 2017, the Product Development Subcommittee approved program policy changes for Oversight Committee consideration. I have attached memos reflecting the recommended changes to revise the standard revenue sharing terms and to increase the matching requirements for second awards. I have provided these memos to the Oversight Committee members in the meeting packet.

Company Contract Negotiations

Award contracts for the two companies approved at the November meeting and one contract approved at the May meeting are in negotiation.

- Molecular Templates - We expect imminent execution of the contract with the company, which will include the standard revenue sharing terms.

- Pelican Therapeutics – The Company informed us that they are in negotiations with a significant potential investor. The investment is at least partially contingent upon the CPRIT award. CPRIT has met with Pelican and the investor to discuss the contract terms. The investor is securing internal approvals. Our assessment is that Pelican will execute the award contract shortly.

- Bellicum Pharmaceuticals - The award approved for the company in November will continue the work on the same compound that was the subject of Bellicum's first award. CPRIT based the first award on substantially different revenue sharing terms than the standard revenue sharing terms currently in place. Multiple royalty obligations based on different revenue sharing terms could be problematic and confusing. Kristen Doyle and I met with Bellicum representatives to address this issue. I have attached our proposal for revenue sharing terms to address both awards. We recommend that the Oversight Committee approve these changes at the February meeting. Once approved, Bellicum will execute the second contract and we will amend the first contract.

In addition to the three awards awaiting contract execution, Kristen and I have been working with Fujifilm Diosynth Biotechnologies (formerly Kalon Biotherapeutics) to revise the current revenue sharing terms. I have attached our proposal, which is consistent with the original intent of the grant award – to support services for Texas' growing life sciences industry – while also ensuring that CPRIT participates in a share of the revenue generated. We will present a contract modification that includes a revenue sharing agreement to the Oversight Committee for review and approval.

Proposed Rule Change to the Product Development Review Process

I have attached a proposed change to CPRIT's administrative rule § 703.6(e)(4)(B). The change reflects that the Product Development Review Council (PDRC) will decide the applications moving forward to due diligence. The PDRC's decision is based on the applications recommended for due diligence by the peer review panel(s). The amendment revises the current process where the review panel recommends the applications for due diligence. The PDRC requests the change to enhance the review process.

Appointment of Dr. Leila Alland to the Scientific Research and Prevention Programs Panel

Wayne Roberts has provisionally appointed Dr. Leila Alland as a member of the Scientific Research and Prevention Programs panel. She will serve as an expert peer reviewer for the Product Development Research Program. Her biosketch is attached. The Nominations Subcommittee will consider Dr. Alland's appointment and recommend approval to the Oversight Committee.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO:	OVERSIGHT COMMITTEE MEMBERS
FROM:	MICHAEL LANG, CHIEF PRODUCT DEVELOPMENT OFFICER
SUBJECT:	REVISION TO STANDARD REVENUE SHARING TERMS
DATE:	JANUARY 31, 2017

Summary and Recommendation

The Product Development Subcommittee recommends that the Oversight Committee approve a change to the standard revenue sharing terms for Product Development grants made to companies that are developing services, diagnostics, or devices. The change modifies the standard revenue sharing royalty rate to 2.5%. The revenue sharing obligation decreases to 0.5% once the grantee has made revenue sharing payments totaling 2.5X the grant amount. Royalty obligation ceases when any governmental grant of exclusivity (*e.g.* patents) expires.

The changed terms recognize the smaller profit margins for non-therapeutic companies. Revising the standard revenue sharing terms for services, diagnostics, and device firms may incentivize more companies to apply to CPRIT and diversify the Product Development portfolio. The standard revenue sharing terms for therapeutic companies receiving CPRIT grants remain the same as originally adopted in January 2015. I have attached the revised standard agreement recommended for approval to this memo.

Background – CPRIT's Standard Revenue Sharing Terms

CPRIT's statute requires the Oversight Committee to establish standards requiring all grant awards to be subject to an agreement that allows the state to share in the proceeds realized from projects undertaken with grant funds. The standards should balance the state's opportunity to benefit through revenue sharing with the need to ensure that medical research is not unreasonably hindered and should not remove the incentive for further development.

The Oversight Committee approved standard revenue sharing terms in January 2015 requiring grantees to pay a 3% – 5% royalty rate until 4X the amount of the grant award is paid to Texas. Once the 4X cap is reached, the grantee must pay a continuing royalty of 0.5% until any governmental grant of exclusivity (*e.g.* patents) expires. Early stage investment in biotech is often based on a royalty return. Successful projects pay a portion of revenue to the investor or licensor. Unsuccessful programs provide no return. This minimizes the burden on pre-revenue companies while providing substantial upside return from successful programs. Most licenses

and philanthropic support are based on royalty returns while most private investments are based on equity ownership.

The standard revenue sharing terms for Product Development grants meet the Oversight Committee's established criteria:

- Simple and understandable
- Provide the State of Texas with a fair and reasonable rate of return on its investment in keeping with:
    - Its role as a provider of financing only (vs. other assistance provided by VCs, including access to network leads for additional capital, business and technical guidance, board members, etc.)
    - Its goals of stimulating company formation and job growth in Texas, and
    - Its lower cost of capital in comparison with other investors
- Facilitate state participation in any blockbuster returns on a product developed with CPRIT funds
- Fall within a range of venture capital and non-profit industry standards, and
- Do not deter follow-on investment in the company by corporate or venture capital investors

The terms are designed so that CPRIT receives greater return on companies with greater sales. Revenue sharing amounts increase in steps 3% to 5% based upon the amount of cumulative revenue. By incorporating steps into the revenue sharing provisions, CPRIT intends to provide for flexibility for all companies.

Discussion – Standard Revenue Sharing Terms Have a Disparate Impact

CPRIT's standard revenue sharing royalty terms provide consistency and transparency. However, CPRIT applies these standard terms across different industry sectors with diverse attributes, resulting in disparate impacts. For example, profit margins vary widely across industry sectors, as reflected on the table below.

Health Care Industry Sector	Net Profit Margin
Drug Manufacturers - Major	21.4
Biotechnology	19.7

Drug Manufacturers - Other	18.5
Medical Instruments and Supplies	11.7
Healthcare Information Services	8.9
Drug Delivery	8.3
Diagnostic Laboratories	6.7

Source: Yahoo Finance

A 4% royalty rate consumes 20% of the profits from a biotech company. The same 4% royalty consumes 60% of the profits of a diagnostic lab. Besides profit margins, the therapeutics industry generally has higher development costs, longer development cycles, and increased development risk.

Because of the different impacts, CPRIT's standard revenue sharing terms are more attractive to some sectors. The terms will be most attractive to high risk, high profits industry sectors such as drugs and other therapeutics. These same terms may create a disincentive for industry sectors with smaller profit margins. To help demonstrate the differences, CPRIT has financially modeled typical therapeutic vs. diagnostic investments based on our standard royalty terms, as reflected in the table below.

Financial Model of Typical Therapeutic vs. Diagnostic Award

Model Inputs	**Therapeutic**	**Diagnostics**	**Notes**
CPRIT Investment $M	15	5	Therapeutics awards are typically larger
Est. Annual Revenue $M	500	100	Therapeutics revenue is typically higher
Cumulative CPRIT Return $M	75	24	4X cap is effected earlier for therapeutics reducing cumulative royalty return
Model Outputs			
NPV of Attrition Adjusted. Royalty -$M	4.9	5.3	Therapeutics have higher attrition rates which reduces net royalty
Portion of Total Revenue Paid as Royalty	1.67%	2.4%	Therapeutics have higher revenue but pay lower royalty due to cap
Industry Profit Margin	19.7%	6.7%	Therapeutics profit margins

			are 3x higher than Diagnostics
Portion of Cumulative Profits Paid as Royalty	9%	36%	Therapeutics pay larger portion of profits as royalty than Diagnostics

The financial model shows that the 4X cap in CPRIT's standard revenue sharing terms substantially reduces the royalty paid by therapeutics companies while minimally impacting other sectors. The model estimates the revenue shared paid to CPRIT by a therapeutics company with a $15 million CPRIT grant at 9% of profits, while other sectors will pay 36% of profits for $5 million investment.

The disparate impact may ultimately affect the type of companies that devote the effort to apply for a CPRIT award. CPRIT's Product Development portfolio invests 90% of its grant award funds in therapeutics. There is a revolution underway in cancer therapeutics. New therapeutics are dramatically improving survival in certain cancers. Investing in these companies is consistent with CPRIT's mission.

However, a similar innovation revolution is underway in cancer diagnostics. Molecular diagnostic techniques are providing a deeper understand of disease mechanisms. This allows for earlier and more effective interventions. Prophylactic and earlier interventions are typically more cost-effective, have higher efficacy and fewer side effects than later stage therapies. Devices and diagnostics also have much shorter development times. Growing the number of funded companies in these sectors may help CPRIT show more outcomes that are successful earlier than therapeutic sector investments.

When the Oversight Committee adopted the standard revenue sharing terms in 2015, the step-up in required revenue sharing from 3% to 5% based upon the company's cumulative revenues was intended to accommodate the different impacts between industry sectors. In practice, CPRIT's share of its portfolio invested in services, diagnostics, and devices remains much smaller than the share of therapeutic development projects.

Recommendation – Adopt Industry-Specific Revenue Sharing Terms

The Product Development Subcommittee recommends setting the revenue sharing percentage for services, diagnostic, and device companies at 2.5%, with a cap on the total amount of revenue shared with the state at 2.5X the grant amount, subject to 0.5% royalty tail. Increasing the number of CPRIT-funded services, diagnostic, and device firms is consistent with CPRIT's mission. Revising the revenue sharing terms to better accommodate the disparate impact may incentivize more applications and ultimately increase the number of funded grants in these sectors.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

CPRIT Product Development General Contract Term Sheet
(Proposed changes are in red)

Revenue Sharing for Therapeutics Programs: Until 4X the amount of the grant monies distributed to the grantee is paid to Texas, the revenue sharing percentage for all products and services subject to revenue sharing shall be:

- 3% of Revenue for Cumulative Revenues greater than $5 million and less than $500 million,
- 4% of Revenue for Cumulative Revenues of $500 million or more but less than $1 billion, and
- 5% of Revenue for Cumulative Revenue of $1 billion or more.

"Cumulative Revenue" is the sum of all Revenue in all years and quarters up to the quarter in which the revenue sharing is being paid. The definition of "Revenue" is given below.

Continuing Royalty for Therapeutics Programs: After 4X the amount of the grant monies distributed to the grantee is paid to Texas, the revenue sharing percentage for all products and services subject to revenue sharing shall be reduced to 0.5%, but cannot be reduced further by any provision for stacking or adjustment.

Revenue Sharing for Devices, Diagnostics, Services, and Other Programs: Until 2.5X the amount of the grant monies distributed to the grantee is paid to Texas, the revenue sharing percentage for all products and services subject to revenue sharing shall be 2.5% of Cumulative Revenue.

Continuing Royalty for Devices, Diagnostics, Services, and Other Programs: After 2.5X the amount of the grant monies distributed to the grantee is paid to Texas, the revenue sharing percentage for all products and services subject to revenue sharing shall be reduced to 0.5%, but cannot be reduced further by any provision for stacking or adjustment.

Stacking Provision: The above revenue sharing percentages may be diminished by 0.5% for every one percent of royalty necessary to be paid to a third party to sell a product or service, but in no case shall be reduced to less than one-half of what would otherwise be due.

Equity: Nothing herein prohibits CPRIT from negotiating an equity share in addition to or in lieu of revenue sharing or continuing royalty terms when deemed appropriate by the Oversight Committee and a company.

Termination of Revenue Sharing: All revenue sharing obligations under the contract for any particular Commercial Product or Commercial Service in a given venue shall terminate for that Commercial Product or Commercial Service in that venue when there is not, or there no longer exists, any governmental grant of exclusivity for the Commercial Product or Commercial Service in that venue. In the event that the Commercial Product or Commercial Service will not have a government grant of exclusivity, CPRIT may negotiate appropriate revenue sharing terms and termination, if any, of revenue sharing payments for Oversight Committee approval.

Definition of Revenue: "Revenue means the gross consideration, whether cash or non-cash (for example, but not by way of limitation, securities, direct equity interest, indirect equity interest, trade or barter considerations, and the like), received from Sales to a Third Party by RECIPIENT or its licensees (including without limitation, any milestone fees, license fees, sublicense fees, or assignment fees), net of: (a) trade or quantity discounts or rebates, credits, allowances or refunds given for rejected or returned Commercial Products or Commercial Services, (b) any sales, value-added or other tax or governmental charge levied on the sale, transportation or delivery of a Commercial Product or Commercial Service (but excluding any income tax owed by the RECIPIENT or its licensees), and (c) any separately stated charges for freight, postage, shipping, and insurance. The foregoing notwithstanding, any consideration: (i) received and used by RECIPIENT or its licensees for the purposes of research or development, or (ii) received from Sales made solely in the performance of clinical trials designed to obtain regulatory approval for a Commercial Product or Commercial Service, or (iii) received by RECIPIENT or its licensees from Sales made for compassionate use where no profit was obtained by RECIPIENT or its licensees shall not be included in this term."

CPRIT will make it clear in the final contract document that there will be no revenue sharing of milestones or other monies prior to the approval of a Product.

*****

These are standard terms that will be applicable to most Product Development grants. However, special circumstances, at CPRIT's determination, may justify individually negotiating one or more terms with the grantee at the time of or following execution of the award contract. In the event that CPRIT staff determines that the project may be reasonably characterized as a combination project (*i.e.* a combination of a therapeutic with a device, diagnostic, service or other program), CPRIT's Oversight Committee will make the final determination about the appropriate revenue sharing terms.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

TO:	OVERSIGHT COMMITTEE MEMBERS
FROM:	MICHAEL LANG, CHIEF PRODUCT DEVELOPMENT OFFICER
SUBJECT:	MATCHING FUND REQUIREMENTS FOR REPEAT GRANTEES
DATE:	JANUARY 31, 2017

Summary and Recommendation

The Product Development Subcommittee recommends that the Oversight Committee approve a change to the required amount of matching funds for Product Development grantees who are receiving their second CPRIT award. Grantees are currently required to dedicate to the grant project $1 of their own funds for every $2 of CPRIT grant award funds. The recommended proposal increases the grantee's matching fund obligation to $1 for every $1 contributed by CPRIT.

Background

The contract for every CPRIT research grant award includes a statutorily required matching funds provision. The grantee must dedicate at least $1 of their own funds toward the grant project for every $2 contributed by CPRIT. Although Academic Research grantees may fulfill the matching requirement via federal indirect cost credits, Product Development grantees must demonstrate and verify specific capital outlays to meet the matching obligation.

CPRIT does not restrict previous grantees from applying for and receiving a second grant award from CPRIT. To date, three CPRIT Product Development grantees have received a second CPRIT award. For these three companies, the first awards funded preclinical and early Phase I work. The second awards are funding Phase I and II clinical trials.

Discussion

Although the Product Development program reviews far more worthy grant applications than it can fund, CPRIT staff and the Product Development Subcommittee do not recommend restricting grantees with scientifically meritorious projects from submitting a second grant application. These grantees are typically conducting Phase I and II clinical trials, which are expensive, but move the potential therapy further along the research and development continuum. Grantees receiving their second awards have ongoing operations and stronger track records, both of which contribute toward the grantee's ability to raise additional funds. Staff and

the Product Development Subcommittee recommend increasing the matching fund requirement from a 1:2 to a 1:1 ratio for second grantees. Because the grantee's proportionate share of project costs is increasing, more CPRIT funds are available for other earlier stage Product Development grants.

Implementation

The Board Governance Subcommittee recommends a rule change if the Oversight Committee agrees with the recommendation. The rule change will be effective for the projects recommended for funding at the August 2017 meeting of the Oversight Committee.

Recommendations for Product Development Research Peer Review Panels

- Leila Alland, M.D.

Recommendations for Academic Research Peer Review Panels

- Jean-Perre Issa, M.D.

PO Box 12097 | Austin, Texas 78711 | PH: 512-463-3190 | F: 512-475-2563 | cprit.texas.gov

Leila Alland, M.D.
Chief Medical Officer, Tarveda Therapeutics

Leila Alland, M.D. brings more than 15 years of pharmaceutical drug development experience with a focus on oncology. Dr. Alland has broad experience as a clinical leader developing both small and large molecule oncology products. Dr. Alland is currently serving as Chief Medical Officer of Tarveda Therapeutics, Inc., (Watertown, MA), with a focus on developing miniaturized drug conjugates for the treatment of patients with solid tumor malignancies. Prior to joining Tarveda, Dr. Alland served as Vice President and Head of Oncology Early Clinical Development at AstraZeneca where she led numerous clinical stage programs through Phase 1/2 development, including six first-in-human programs and a Phase 2 registration study supporting the approval of Tagrisso. At Bristol-Myers Squibb, Dr. Alland served as clinical head for multiple early phase oncology programs spanning tumor targeted and immuno-oncology therapies. Dr. Alland conducted her pediatric hematology and oncology training at Memorial Sloan Kettering Cancer Center and then joined the faculty of the Albert Einstein College of Medicine as Assistant Professor where her cancer biology research resulted in publications in *Nature*, *Nature Genetics* and *Cell*. She received her medical degree at New York University School of Medicine and her B.A. in Biology at the University of Pennsylvania.

BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors.
Follow this format for each person. **DO NOT EXCEED FIVE PAGES.**

NAME: Jean-Pierre J. Issa, M.D.

eRA COMMONS USER NAME (credential, e.g., agency login): JPISSA

POSITION TITLE: Professor of Medicine

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable. Add/delete rows as necessary.)*

INSTITUTION AND LOCATION	DEGREE *(if applicable)*	Completion Date MM/YYYY	FIELD OF STUDY
The American University of Beirut, Beirut,	B.S.	1980-83	Biology
The American University of Beirut, Beirut,	M.D.	1983-87	Medicine

A. Personal Statement

I trained in medical oncology at Johns Hopkins, where I started my research career in the field of epigenetics and cancer. I am currently Professor at Temple University and Director of the Fels Institute for Cancer Research and Molecular Biology. I am also co-leader of the Cancer Epigenetics Program at the Fox Chase Cancer Center (part of Temple Health). My laboratory and clinical/translational interests are in the area of epigenetics, with particular emphasis on the role of DNA methylation and histone modifications in aging, cancer development and as a target for prevention and therapy for cancer. Some of the findings from my laboratory include the first description of age-related promoter hypermethylation (1994), the discovery of the CpG Island Methylator Phenotype (1999), bench to bedside clinical trials of the hypomethylating drug decitabine (2004) which contributed to its eventual FDA approval (2006), the discovery of methylation-independent gene silencing in cancer by Polycomb group proteins (2008) and the discovery of microbiome-DNA methylation interactions (2014). I have also been involved in studies of environmental and lifestyle effects on DNA methylation, the identification of epigenetic field defects as early markers of neoplasia risk, whole epigenome studies with discovery of markers for screening and prognosis, and screening for epigenetic acting drugs to treat patients with malignancies. Google Scholar calculates an H-index of 110 and >46,000 citations for my publications (https://scholar.google.com/citations?user=ujjcPF0AAAAJ&hl=en).

Examples of relevant publications that include research in breast cancer:

1. Raynal NJ, (et al.), Issa JP. Targeting Calcium Signaling Induces Epigenetic Reactivation of Tumor Suppressor Genes in Cancer. Cancer Res. 2016 Mar 15;76(6):1494-505.
2. Pathiraja TN, Nayak SR, Xi Y, Jiang S, Garee JP, Edwards DP, Lee AV, Chen J, Shea MJ, Santen RJ, Gannon F, Kangaspeska S, Jelinek J, Issa JP, Richer JK, Elias A, McIlroy M, Young LS, Davidson NE, Schiff R, Li W, Oesterreich S. Epigenetic reprogramming of HOXC10 in endocrine-resistant breast cancer. Sci Transl Med. 2014 Mar 26;6(229):229ra41.

3. Issa JP. Aging and epigenetic drift: a vicious cycle. J Clin Invest. 2014 Jan;124(1):24-9.
4. Malouf GG, (et al), Issa JP. Architecture of epigenetic reprogramming following Twist1-mediated epithelial-mesenchymal transition. Genome Biol. 2013 Dec 24;14(12):R144.
5. Kondo Y, (et al), Issa JP. Gene silencing in cancer by histone H3 lysine 27 trimethylation independent of promoter DNA methylation. Nat Genet. 2008 Jun;40(6):741-50.

B. Positions and Honors

Positions and Employment

1995-1999	Assistant Professor, Johns Hopkins Oncology Center, Baltimore, Maryland
1999-9/2003	Associate Professor, M.D. Anderson Cancer Center, Houston, Texas
9/2003-8/2011	Professor, M.D. Anderson Cancer Center, Houston, Texas
9/2011-Present	Professor and Director, Fels Institute for Cancer Research, Temple University
9/2012-Present	Co-Leader, Cancer Epigenetics Program, Fox Chase Cancer Center, Temple Health

Honors (selected)

Scholar, Sidney Kimmel Foundation for Cancer Research, 1997
American Society for Clinical Investigation, 2003
Bessie McGoldrick Professorship in Clinical Cancer Care, 2005
Frei Award for Translational Research, MD Anderson (MDACC), 2007
American Cancer Society Clinical Research Professor 2007-2017
The Dallas Fort Worth Living Legend Faculty Achievement Award in Basic Science Research, MDACC, 2007
Richard and Hinda Rosenthal Memorial Award, American Association for Cancer Research, 2011
American Association of Physicians, 2015

C. Contributions to Science

1. **Discovery of age-related DNA methylation drift.** DNA methylation is a mediator of epigenetic cellular inheritance and is involved in multiple physiologic conditions (development, differentiation, stemness etc.) as well as pathologic conditions (cancer, developmental diseases etc.). Biochemical measurements of total DNA methylation (e.g. 5-methylcytosine content) had suggested alterations in the process in cancer and aging but the significance of these findings were obscure until technology was developed to study specific genes. In the 1980's and 90's, it became clear that DNA methylation of many genes was altered in cancer and a hypothesis was advanced that promoter DNA methylation could serve as a mechanism to stably silence tumor-suppressor genes in cancer. In 1994, I described for the first time that human aging was associated with gains in promoter DNA methylation of a gene (*ERα*) in normal appearing colonic tissues and proposed that age-related methylation disturbances predispose to diseases such as cancer (Issa *et al*, 1994) (>1,000 citations). My lab later showed that age-related DNA methylation changes affected multiple genes and were tissue specific (Ahuja *et al*, 1998) (>500 citations), that both hypermethylation and hypomethylation could be observed (hence the term drift) and that the process was conserved from mice to humans (Maegawa *et al*, 2010) and finally that age-related methylation was the clearest explanation for methylation disturbances in cancer (Maegawa *et al*, 2014). The study of age-related methylation drift exploded in the past few years with hundreds of publications confirming and extending my lab's original findings, and it is currently one of the hottest topics in aging research. A Pubmed search for Aging DNA methylation reveals over 1200 papers.
   - Ahuja N, Li Q, Mohan AL, Baylin SB, **Issa JP** (1998) Aging and DNA methylation in colorectal mucosa and cancer. Cancer Res 58(23): 5489-94
   - **Issa JP**, Ottaviano YL, Celano P, Hamilton SR, Davidson NE, Baylin SB (1994) Methylation of the oestrogen receptor CpG island links ageing and neoplasia in human colon. Nat Genet 7(4): 536-40
   - Maegawa S, Hinkal G, Kim HS, Shen L, Zhang L, Zhang J, Zhang N, Liang S, Donehower LA, **Issa JP** (2010) Widespread and tissue specific age-related DNA methylation changes in mice. Genome Res 20(3): 332-40
   - Maegawa S, Gough SM, Watanabe-Okochi N, Lu Y, Zhang N, Castoro RJ, Estecio MR, Jelinek J, Liang S, Kitamura T, Aplan PD, **Issa JP** (2014) Age-related epigenetic drift in the pathogenesis of MDS and AML. Genome Res 24(4): 580-91

2. **Discovery of the CpG Island Methylator Phenotype (CIMP).** When it was proposed by the labs of Dr. Stephen Baylin (my mentor) and Dr. Peter Jones that DNA methylation could be exploited by cancer cells to silence tumor-suppressor genes, many scientists were skeptical. It was argued that DNA methylation changes were an artifact of transformation and cell culture and could not provide the same selective advantage as mutations in causing cancer. In 1998, I discovered that a subset of colon cancers was characterized by intense aberrant DNA methylation of multiple genes, a phenomenon which I named CIMP (Toyota *et al*, 1999) (>1800 citations). My lab proposed that this non-Gaussian distribution of aberrant DNA methylation proves that it is selected for and not an artifact of transformation (Issa, 2004) (>800 citations). Moreover, I compared CIMP to genetic instability phenotypes, and I proposed the hypothesis that CIMP (and by extension aberrant DNA methylation) could have a traceable cause such as a mutation. My lab subsequently showed for the first time that CIMP was associated with distinct genetic changes (Toyota *et al*, 2000), that it was present in many other cancers (e.g. gastric cancer) and that a combined genetic/epigenetic classifier divided colon cancer into very distinct and clinically relevant subsets (Shen *et al*, 2007). While CIMP was initially controversial, the concept was validated in colon cancer and extended by TCGA studies to multiple other malignancies where it was shown to be highly prognostic, potentially caused by Krebs cycle alterations in some cases, while a primary molecular abnormality in other cases. A Pubmed search for CIMP/CpG Island Methylator Phenotype reveals over 750 papers.
   - **Issa JP** (2004) CpG island methylator phenotype in cancer. Nat Rev Cancer 4(12): 988-93
   - Shen L, Toyota M, Kondo Y, Lin E, Zhang L, Guo Y, Hernandez NS, Chen X, Ahmed S, Konishi K, Hamilton SR, **Issa JP** (2007) Integrated genetic and epigenetic analysis identifies three different subclasses of colon cancer. Proc Natl Acad Sci U S A 104(47): 18654-9
   - Toyota M, Ahuja N, Ohe-Toyota M, Herman JG, Baylin SB, **Issa JP** (1999) CpG island methylator phenotype in colorectal cancer. Proc Natl Acad Sci U S A 96(15): 8681-6
   - Toyota M, Ohe-Toyota M, Ahuja N, **Issa JP** (2000) Distinct genetic profiles in colorectal tumors with or without the CpG island methylator phenotype. Proc Natl Acad Sci U S A 97(2): 710-5

3. **Clinical/translational studies of epigenetic therapy.** A paradox in the early days of trying to introduce epigenetic acting drugs in the clinic was the perceived lack of efficacy of DNA methylation inhibitors that were synthesized in the 1960's and tested clinically in the 1970's and 1980's. This was a consistent criticism of the field, along with concerns about non-specificity of the approach and potential mutagenesis of the drugs (and of hypomethylation induction). However, pre-clinical studies showed that DNA methylation inhibitors had biphasic effects – demethylation at low doses and DNA damage/cytotoxicity at high doses and I reasoned that this may have confounded some of the early clinical trials which were based on determining maximally tolerated doses. Focusing on the DNA methylation inhibitor decitabine, I led a clinical/translational group that designed a clinical trial where we showed that low doses of decitabine were more active clinically than the MTD (Issa *et al*, 2004) (>600 citations). This was followed by the pivotal study that led to decitabine's FDA approval in the USA (Kantarjian *et al*, 2006) (>900 citations) and a dose-optimization study that led to the currently used dose schedule of the drug (Kantarjian *et al*, 2007) (>600 citations). In that same trial, we also showed that sustained demethylation of the P15 tumor suppressor was associated with response (same reference). More recently, I led a collaboration between our "epigenetics" Stand Up to Cancer/AACR team and the Astex company to design and complete a molecularly guided phase I study of the new hypomethylation drug SGI110 (Issa *et al*, 2015). The field of epigenetic therapy of cancer is now highly active with dozens of drugs in clinical trials.
   - **Issa JP**, Garcia-Manero G, Giles FJ, Mannari R, Thomas D, Faderl S, Bayar E, Lyons J, Rosenfeld CS, Cortes J, Kantarjian HM (2004) Phase 1 study of low-dose prolonged exposure schedules of the hypomethylating agent 5-aza-2'-deoxycytidine (decitabine) in hematopoietic malignancies. Blood 103(5): 1635-40
   - Kantarjian H, **Issa JP**, Rosenfeld CS, Bennett JM, Albitar M, DiPersio J, Klimek V, Slack J, de Castro C, Ravandi F, Helmer R, Shen L, Nimer SD, Leavitt R, Raza A, Saba H (2006) Decitabine improves patient outcomes in myelodysplastic syndromes: results of a phase III randomized study. Cancer 106(8): 1794-803
   - Kantarjian H, Oki Y, Garcia-Manero G, Huang X, O'Brien S, Cortes J, Faderl S, Bueso-Ramos C, Ravandi F, Estrov Z, Ferrajoli A, Wierda W, Shan J, Davis J, Giles F, Saba HI, **Issa JP** (2007) Results of a randomized study of 3 schedules of low-dose decitabine in higher-risk myelodysplastic syndrome and chronic myelomonocytic leukemia. Blood 109(1): 52-7

- Issa JP, Roboz G, Rizzieri D, Jabbour E, Stock W, O'Connell C, Yee K, Tibes R, Griffiths EA, Walsh K, Daver N, Chung W, Naim S, Taverna P, Oganesian A, Hao Y, Lowder JN, Azab M, Kantarjian H. Safety and tolerability of guadecitabine (SGI-110) in patients with myelodysplastic syndrome and acute myeloid leukaemia: a multicentre, randomised, dose-escalation phase 1 study. Lancet Oncol. 2015 Sep;16(9):1099-110.

4. **DNA methylation and histone code interactions.** Both DNA methylation and silencing histone marks (H3K9me2, H3K27me3) are epigenetic processes but their interactions remain a matter of debate. An association between gene marking by polycomb group specific proteins and susceptibility to DNA hypermethylation in cancer (as well as other studies in model organisms) led many to believe that DNA methylation is simply a consequence of chromatin remodeling. Studying DNA methylation - chromatin interactions at cancer-specific loci, my lab showed (for the first time for cancer silencing) that DNA methylation was closely linked to H3K9me2 (Kondo *et al*, 2003) but inversely correlated with H3K27me3 (Kondo *et al*, 2008) (>440 citations), which contradicted earlier thinking about polycomb triggering DNA methylation. These data were confirmed by many labs. Furthermore, we showed that DNA demethylation alone is not sufficient for gene expression (chromatin remodeling is required) and, in turn, chromatin remodeling alone can lead to high level gene expression despite the persistence of DNA methylation (Raynal *et al*, 2012; Si *et al*, 2010). Altogether, our data strongly suggest that DNA methylation and histone code changes are independent processes and confirmed early hypotheses that suggested that the main role of DNA methylation is to provide a memory of the gene silencing state.
   - Kondo Y, Shen L, Cheng AS, Ahmed S, Boumber Y, Charo C, Yamochi T, Urano T, Furukawa K, Kwabi-Addo B, Gold DL, Sekido Y, Huang TH, **Issa JP** (2008) Gene silencing in cancer by histone H3 lysine 27 trimethylation independent of promoter DNA methylation. Nat Genet 40(6): 741-50
   - Kondo Y, Shen L, **Issa JP** (2003) Critical role of histone methylation in tumor suppressor gene silencing in colorectal cancer. Mol Cell Biol 23(1): 206-15
   - Raynal NJ, Si J, Taby RF, Gharibyan V, Ahmed S, Jelinek J, Estécio MR, **Issa JP** (2012) DNA methylation does not stably lock gene expression but instead serves as a molecular mark for gene silencing memory. Cancer Res 72(5): 1170-81
   - Si J, Boumber YA, Shu J, Qin T, Ahmed S, He R, Jelinek J, **Issa JP** (2010) Chromatin remodeling is required for gene reactivation after decitabine-mediated DNA hypomethylation. Cancer Res 70(17): 6968-77

5. **Epigenomics.** My laboratory has been involved in DNA methylation profiling studies for 20 years, including method development and studies of methylation/environment interactions as well as methylation/outcomes correlations. For example, my laboratory developed the LINE1 methylation assay as a surrogate for global methylation levels (Yang *et al*, 2004) (>570 citations). My group was among the first to show a link between chronic inflammation and aberrant DNA methylation (Issa *et al*, 2001) (>500 citations), to suggest that aberrant DNA methylation is present in “fields” of cancer predisposition (Shen *et al*, 2005) (>300 citations) and to demonstrate geographic variation in aberrant DNA methylation, suggesting an environmental effect (Shen *et al*, 2002) (>200 citations).
   - **Issa JP**, Ahuja N, Toyota M, Bronner MP, Brentnall TA (2001) Accelerated age-related CpG island methylation in ulcerative colitis. Cancer Res 61(9): 3573-7
   - Shen L, Ahuja N, Shen Y, Habib NA, Toyota M, Rashid A, **Issa JP** (2002) DNA methylation and environmental exposures in human hepatocellular carcinoma. J Natl Cancer Inst 94(10): 755-61
   - Shen L, Kondo Y, Rosner GL, Xiao L, Hernandez NS, Vilaythong J, Houlihan PS, Krouse RS, Prasad AR, Einspahr JG, Buckmeier J, Alberts DS, Hamilton SR, **Issa JP** (2005) MGMT promoter methylation and field defect in sporadic colorectal cancer. J Natl Cancer Inst 97(18): 1330-8
   - Yang AS, Estécio MR, Doshi K, Kondo Y, Tajara EH, **Issa JP** (2004) A simple method for estimating global DNA methylation using bisulfite PCR of repetitive DNA elements. Nucleic Acids Res 32(3): e38

A full bibliography can be found at: http://www.ncbi.nlm.nih.gov/sites/myncbi/jean-pierre.issa.1/bibliography/40796930/public/?sort=date&direction=ascending

D. Research Support

ACTIVE

P50 (Kantarjian/Issa) 08/01/2013-07/31/2018
NIH/NCI
Specialized Program of Research Excellence (SPORE) in Leukemia
The major goal of project-1 is to identify drugs that reverse epigenetic silencing and conduct clinical trials of these drugs/combinations in AML and MDS.
Role: Overall Co-PI of the SPORE and PI, project 1
No Overlap

CRP-13-307-06(Issa) 07/01/2012-6/30/2018
ACS
Clinical Research Professorship "Epigenetic Therapy"
The goal of this career development-type award is to study (1) the efficacy of combined epigenetic therapy (DNA methylation inhibitors + histone deacetylase inhibitors) in solid tumors and their potentiation potential on the activity of other drugs; (2) DNA methylation changes in cancers and possible prediction of response to therapy; and (3) to discover specific drugs that potentiate the activity of epigenetic-acting drugs.
Role: PI
No Overlap

1R01CA165052-01(Issa) 09/30/2012- 07/31/2017
NIH/NCI
Regulator of Cancer-Specific DNA Hypermethylation
The goal of this grant is to understand the mechanisms controlling aberrant CpG island methylation in cancer.
Role: PI
No Overlap

Ellison Medical Foundation (Issa) 11/14/2012 – 11/13/2016
Epigenetic Drift as a Modifier of Lifespan
The goal of this grant is to propose the hypothesis that epigenetic drift modifies lifespan by reducing stem cell fitness and plasticity.
Role: PI; No Overlap

Astex (Issa) 11/1/2014 – 10/31/2016
Genomics and epigenomics of response to SGI-110 in AML and MDS
The goal of this grant is to identify genetic/epigenetic markers that could be used to stratify AML/MDS patients and correlate the DNA methylation genetic mutation profiles with response and survival using SGI-110.
Role: PI; No Overlap

AACR/Stand Up to Cancer (Issa) 10/1/2014 – 09/30/2017
Van Andel Stand up to Cancer DREAM Team in Epigenetic Therapy
The goal of this grant is to develop an infrastructure to conduct clinical trials involving epigenetic therapy drugs, including clinical and translational research support.
Role: PI in the team led by Drs. Steve Baylin and Peter Jones and site PI
No Overlap

FCCC/NIH P30 (Issa) 08/12/2016 – 07/31/2017
Comprehensive Cancer Center Program at Fox Chase
The goal of this grant is provides salary support only for Dr. Issa to co-lead the Cancer Epigenetics Program in the Fox Chase Cancer Center Support Grant.

No Overlap

Completed in the Past 3 Years

01 (Johns Hopkins lead institution; Issa project leader MDACC) 12/1/2009 – 11/30/2013
AACR Stand Up to Cancer Dream Team: "Bringing epigenetic therapy to the forefront of cancer management"
The central focus of this multi-institutional team effort is to bring the promise of epigenetic therapy, targeting reversal of abnormal gene silencing, to full clinical practice for major types of human cancer.

1R01DE022015-01 (MPI, Issa and Pan) 08/01/2011-07/31/2016
NIH
Epigenetic Mechanisms of Neuropathic Pain
The goal of this grant is to understand the role of epigenetic changes in the pathogenesis of neuropathic pain and to test epigenetic therapy as pain treatment.
Role: PI (MPI grant)
No Overlap

CURRICULUM VITAE
Jean-Pierre Issa, M.D.

Updated Dec 2016

PERSONAL

HOME ADDRESS:
Philadelphia, PA,

BUSINESS ADDRESS: Fels Institute for Cancer Research, Temple University
3307 N Broad St
Philadelphia, PA 19140
215-707-4307

BIRTHPLACE: Zahle, Lebanon

CITIZENSHIP STATUS: United States

EDUCATION AND TRAINING

UNDERGRADUATE SCHOOL

The American University of Beirut Beirut, Lebanon	7/1980 to 6/1983	BS, 1983, Biology

GRADUATE/PROFESSIONAL SCHOOL

The American University of Beirut, Beirut, Lebanon	7/1983 to 6/1987	MD, 1987, Medicine

GRADUATE MEDICAL EDUCATION

Residency in Internal Medicine	7/1987–6/1990 Good Samaritan Hospital, Baltimore, MD
Fellowship in Medical Oncology	7/1990–6/1994 Johns Hopkins University, Baltimore, MD

LICENSURE

Active

Pennsylvania	MD444160	09/15/2011-present

Inactive

Texas	L8381	11/2007–11/2011
Maryland	D39335	1990-1999

BOARD CERTIFICATION

American Board of Internal Medicine	1990
Medical Oncology	1993

ACADEMIC HONORS AND AWARD

Dean's Honor List,	1980–1987
B.S. with Distinction,	1983
Outstanding Resident Award,	1990
Merit Award, American Society of Clinical Oncology Meeting,	1994
Young Investigator Award, American Society of Clinical Oncology,	1995
Scholar, Sidney Kimmel Foundation for Cancer Research,	1997–1999
Scholar, George and Barbara Bush Foundation,	2000–2003
Teacher of the Year, Division of Cancer Medicine, University of Texas M.D. Anderson Cancer Center	2001
Scholar, Sidney Kimmel Foundation Symposium for Cancer Research	2002
Elected to membership, The American Society for Clinical Investigation	2003
Bessie McGoldrick Professorship in Clinical Cancer Care University of Texas M.D. Anderson Cancer Center	2005-2011
Emil Frei III Award for Excellence in Translational Research, Division of Medicine, University of Texas M.D. Anderson Cancer Center	2006
American Cancer Society Clinical Research Professorship	2007-2017
MD Anderson Trust Fund, University of Texas M.D. Anderson Cancer Center	2007–2012
The Dallas Fort Worth Living Legend Faculty Achievement Award in Basic Science Research, University of Texas M.D. Anderson Cancer Center	2007
Nagy Sahyoun Prize, American University of Beirut	2009
ALMA Community Enrichment Award, American Lebanese Medical Association	2010
35th Annual Richard and Hinda Rosenthal Memorial Award, American Association for Cancer Research (AACR)	2011
Scientific Research Award, American Cancer Society Greater Philadelphia	2014
Elected to membership, The American Association of Physicians	2015

ACADEMIC APPOINTMENTS

Instructor, Tumor Biology Division, The Johns Hopkins University School of Medicine, Baltimore, MD	1/1994–1/1995
Assistant Professor, Tumor Biology Division, The Johns Hopkins University School of Medicine, Baltimore, MD	1/1995–1/1999
Associate Professor, Leukemia Department, Division of Cancer Medicine, The University of Texas M.D. Anderson Cancer Center, Houston, TX	1/1999–1/2003
Professor, Department of Leukemia, The University of Texas M. D. Anderson Cancer Center, Houston, TX	1/2003–8/2011
Professor, Department of Medicine Temple University School of Medicine Philadelphia, PA	9/2011-present

Professor, Cancer Epigenetics Program — 1/2013-present
Fox Chase Cancer Center
Temple Health, Philadelphia, PA

Administrative Appointments/Responsibilities

Chief, Section of Translational Research, Department of Leukemia, — 8/1999 – 8/2011
The University of Texas M. D. Anderson Cancer Center,
Houston, TX

Co-Director, Center for Cancer Epigenetics — 2007–8/2011
Institute of Basic Science Research
The University of Texas M. D. Anderson Cancer Center
Houston, TX

Program Leader, Hematologic Malignancies Program, — 7/1/2007 – 8/2010
The University of Texas M. D. Anderson Cancer Center,
Houston, TX

Director, Fels Institute for Cancer Research and Molecular Biology — 9/1/2011-present
Temple University
Philadelphia, PA

Program Co-Leader, Cancer Epigenetics Program, — 9/2012-present
Fox Chase Comprehensive Cancer Center
Temple Health
Philadelphia, PA

PRESENT TITLE AND AFFILIATION

Primary Appointment
American Cancer Society Clinical Research Professor
Professor of Medicine
Director, Fels Institute for Cancer Research and Molecular Biology
Temple University School of Medicine
Philadelphia, PA

Dual/Joint/Adjunct Appointment
Professor, Fox Chase Comprehensive Cancer Center
Temple Health
Philadelphia, PA

Consultantships and External Advisory Boards
EPA Expert Panel on Arsenic Carcinogenicity, Washington, DC, Member, 1/1997–12/1997
Breast Cancer SPORE, Johns Hopkins, External advisory board, 2005-2010
Leukemia SPORE, Ohio State, External advisory board, 2008-2013
National Cancer Institute – Leukemia Steering Committee, Consultant, 2009-2014
CPRIT Lonestar P01, External advisory board, 2011-present
National Scientific Advisory Council, American Federation for Aging Research, 2014-present

Kidney Cancer SPORE, MD Anderson, External advisory board, 2013
Karmanos Cancer Institute, External advisory board, 2016-present
Ovarian cancer P01, Wistar Institute, External advisory board, 2016-present

Institutional Committee Activities (MD Anderson Cancer Center)
Division of Medicine Subcommittee on Basic Research, Member, 1/2000–1/2003
Institutional Biosafety committee, Member, 1/2000–1/2003
IRG Committee, Member, 1/2001–1/2004
Clinical Research Committee II, Member, 1/2002–1/2003
Recruitment for Lymphoma Chair, Committee 2003
Promotion and Tenure Committee, Member, 1/2003–1/2006
Promotion and Tenure Committee, Vice - chair, 1/2004–1/2005
Post-doctoral Advisory Committee, Member, 1/2005–12/2005
Recruitment of the Provost, EVP and CAO, Advisory Committee, 1/2006–12/2006
Translational Research Subcommittee, Division of Cancer Medicine, Chair, 2007
Recruitment for Epidemiology Chair, Committee, 2009
Internal Advisory Board, UT MDACC Multiple Myeloma SPORE, 9/2009 – 2011
Endowed Positions Committee, 2009 – 2011

Institutional Committee Activities (Temple/Fox Chase)
Recruitment for Department of Medicine Chair, Committee 2012
Fox Chase/Temple Integration committee, 2013
Vice-Provost for Research Advisory Committee, Temple University, 2013-present
Dean's Research Advisory Committee, Temple University SOM, 2014-present
Fox Chase Cancer Center, Internal Advisory Board, 2015-present

RESEARCH

Grants and Contracts

Funded
1R01CA165052-01 (Issa) 09/30/2012-09/29/2017; 1.2 calendar
NIH/NCI
Regulator of Cancer-Specific DNA Hypermethylation
The goal of this grant is to understand the genetic/sequence and epigenetic/chromatin determinants of cancer specific DNA hypermethylation.

1R01DE022015-01 (MPI, Issa and Pan) 08/01/2011-07/31/2016; 1.2 calendar
NIH
Epigenetic Mechanisms of Neuropathic Pain
The goal of this grant is to understand the role of epigenetic changes in the pathogenesis of neuropathic pain and to test epigenetic therapy as pain treatment.

3P50 CA100632-06 (Kantarjian/Issa) 8/5/2003-4/30/2018; 2.64 calendar
NIH/NCI
Specialized Program of Research Excellence (SPORE) in Leukemia
Overall co-PI, Leader of Project 1, Co-Leader of Core A (admin), Co-PI of Core B (lab), and co-Director of Career Development and Research Development

The major goal of project-1 (Issa) is to clone genes hypermethylated in drug resistant AML and conduct a clinical trial of reversing drug resistance using decitabine followed by chemotherapy.

ACS (Issa) 7/1/2007-6/30/2018; 1.2 calendar
American Cancer Society
Clinical Research Professorship "Epigenetic Therapy"
The goal of this career development-type award is to study (1) the efficacy of combined epigenetic therapy (DNA methylation inhibitors + histone deacetylase inhibitors) in solid tumors and their potentiation potential on the activity of other drugs; (2) DNA methylation changes in cancers and possible prediction of response to therapy; and (3) to discover specific drugs that potentiate the activity of epigenetic-acting drugs.

Ellison Medical Foundation 10/01/2012-09/30/2016; .96 calendar
Epigenetic Drift as a Modifier of Lifespan
The goal of this grant is to propose the hypothesis that epigenetic drift modifies lifespan by reducing stem cell fitness and plasticity.

Stand Up to Cancer Grant 2014-2017 1.2 calendar
AACR/Van Andel Stand Up to Cancer Dream Team: "Epigenetics"
The central focus of this multi-institutional team effort is to bring the promise of epigenetic therapy, targeting reversal of abnormal gene silencing, to full clinical practice for major types of human cancer. Dr. Issa is a Principal in this team.

Completed (past 3 years)

2P01 CA046939-20A2 (Champlin PI; Issa Proj 6 PI) 12/1/2009–11/30/2015; 1.8 calendar
NIH/NCI
The Therapy of CML, Project 6 "The CML Epigenome - Implications for Therapy"
The goal of the program project is to study the CML epigenome, and use the results to identify agents that act through epigenetic parameters as well as use epigenetic markers to predict treatment outcome.

Stand Up to Cancer Grant (Johns Hopkins lead institution; Issa project leader Temple) 12/1/2009 – 6/30/2013 1.2 calendar
AACR/Stand Up to Cancer Dream Team: "Bringing epigenetic therapy to the forefront of cancer management"
The central focus of this multi-institutional team effort is to bring the promise of epigenetic therapy, targeting reversal of abnormal gene silencing, to full clinical practice for major types of human cancer.

5R01 CA121104-03 (Issa) 9/1/2007 - 7/31/2012 1.2 calendar
NIH/NCI
Randomized study of combined epigenetic therapy
The goal is to test, in a randomized setting, combined epigenetic therapy (decitabine +/- valproic acid), with both molecular and clinical endpoints.

5P01 CA108631-04 (Kantarjian - PI) (Issa Leader Project 3) 6/21/2005 – 5/31/2011 .12 calendar
NIH/NCI

New Approaches to the Biology and Treatment of MDS
The overall goal of this project is to conduct translational research in the biologic, genetic and clinical aspects of MDS to improve understanding, therapy, and prognosis.

1 U24 CA143883-01 (Weinstein; Issa Collaborator) 09/01/2009-08/31/2011 .6 calendar
NIH/NCI
Integrative Pipeline for Analysis & Translational Application of TCGA Data (GDAC)
The overarching goals are to develop information resources for basic understanding of cancer biology and to aid in the identification of new biomarkers and biosignatures for 'personalization' of cancer management. The technological challenges are great, but the bioinformatic challenge of converting profile data into knowledge that improves patient outcomes is even greater.

Astex 2013-2015
Epigenetic studies in patients treated with SGI-110
This contract covers correlative studies in MDS/AML patients treated with SGI-110.

CS2007-19540 (Issa) 2/7/2007 – 2/28/2011 0.12 calendar
Eisai
2006-0686 "Phase II Randomized Study of Low-Dose Decitabine (5-AZA-2'-Deoxycytidine) with or without Valproic Acid in Myelodysplastic Syndrome (MDS) and Acute Myelogenous Leukemia"
The goal is to study the safety and efficacy of low-dose decitabine alone and in combination with valproic acid in MDS and AML.

CCTS (Hwu) 3/6/2008 - 3/5/2011 0.12 calendar
MDACC
The Biologic Effects of Decitabine and Pegylated Interferon in Patients with Advanced Melanoma
The goal is to ascertain if the combination of decitabine and interferon will increase expression of tumor antigens in melanoma tissue by reversal of methylation induced gene silencing, and if increased expression will lead to upregulated T-cell anti-tumor responses and consequent clinical benefit for melanoma patients.

RP100233 (Myers PI, Issa Project Leader) 8/1/2010-8/31/2011 .6 calendar
CPRIT Individual Investigator Award
Comprehensive Analysis of Genetic and Epigenetic Changes in Oral Cancer
Principal Investigator, 25%, SPORE in Leukemia, 5P50 CA 100632, NIH/NCI, 9/1/2008–4/30/2013, $9,200,000 ($1,889,715 to Issa Lab)

Completed

Principal Investigator, Kimmel Scholar Award, 1997–1999, $200,000

Principal Investigator, Clinical Implications of DNA methylation changes in leukemia, Leukemia and Lymphoma Society, 12/1/1997–11/30/2000, $200,000

Principal Investigator, Causes of a Global Hypermethylator Phenotype in Colorectal Cancer, RPG-99-098-01-MGO , American Cancer Society (ACS Research Grant), 1/1/1999–12/31/2001, $300,000

Co-Investigator, Folate Supplementation and DNA Methylation in the Colon, R01 CA59005, NIH/NCI, 8/1/1999–7/31/2003, $129,972

Co-Investigator, 1%, Colon Cancer Prevention Program Project, 2 PO2 CA41108, NIH/NCI, PI - Dr. Issa is Co-PI of project 2, 9/14/2000–7/31/2010, $17,463,410 ($70,364 for Issa lab)

Principal Investigator, Prognostic Significance of Methylation Profiling in Colorectal Cancer, R01 CA89245-01, NIH/NCI, 1/1/2001–2/28/2004, $166,000/year

Principal Investigator, 10%, Methylated CpG Island Amplification for Methylation Profiling of Neoplasms, PAR 99 100, NIH/NCI, 1/1/2001–3/31/2005, $559,142

Co-Investigator, Phase I Studies of Targeted Anti-Cancer Therapies, 3 U01 CA062461 10 S1, NIH/NCI, 3/27/2003–1/31/2008, $666,422

Principal Investigator, 10%, Epigenetic silencing and resistance to Imatinib mesylate in CML, CM020027, DOD, 7/1/2003–7/31/2006, $496,689 ($165,563/year)

Principal Investigator, 25%, Specialized Programs of Research Excellence, 1 P50 CA100632-01, NIH/NCI, 8/5/2003–10/30/2008, $9,331,895 ($1,866,379/year)
Co-Investigator, 1%, Phase I/II Study of 5-Aza-2'-Deoxycytidine and Valproic Acid in Patients with Relapsed/Refractory Leukemias or Myelodysplastic Syndromes, 1R21CA105771-01A, NIH/NCI, PI - Garcia-Manero, 9/1/2003–8/31/2007 ($250,000/year)
Co-Investigator, 1%, Prognostic Value of Methylation of Cell Cycle Controlling Genes in Adult Acute Lymphocytic Leukemia, 2 R21 CA100067, NIH/NCI, PI - Garcia-Manero, 9/1/2003–8/31/2007 ($100,000/year)

Principal Investigator, 15%, Diet and DNA Methylation in Colon Mucosa and Adenomas, CA105346-01, NIH/NCI, 7/13/2004–6/30/2008, $1,000,000 ($250,000/year)

Principal Investigator, 5%, Changes in Tumor DNA Methylation with Decitabine, R21 CA112895, NIH/NIC, 6/1/2005–5/31/2007, $500,000 ($60,468/year)
Principal Investigator, 5%, DNA Methylation as an Epigenetic Factor in the Development and Progression of Polycythemia Vera, MP043015, DOD, 9/15/2005–8/14/2009, ($225,032)
Co-Investigator, Phase I Studies of Targeted Anti-Cancer Therapy; 2005-0723 " A Phase 1 Trial of SAHA (NSC 701852) and Decitabine (IND 50733, NSC 127716) In Patients with Relapsed, Refractory or Poor Prognosis Leukemia," 2U01CA062461-15, NIH/NCI, PI - Kurzrock, 4/16/2008–10/23/2009

Clinical Protocols

Ongoing

Co-PI, Phase I/II studies of SGI-110 in patients with MDS and AML, Astex, 2012-present

Funded/Completed

Principal Investigator, Phase I Biologic Study of Decitabine (5-Aza-2-Deoxycitidine, DAC) In Hematopoietic Malignancies, ID00-045, 2000–2001

Principal Investigator, Phase II Study of Imatinib Mesylate (Gleevec, STI571) (NSC#716051) and Decitabine (5-AZA-2'-Deoxycitidine) (NSC#127716), An NCI-Supplied Agent, in Chronic Myelogenous Leukemia in Accelerated and Blastic Phases, Protocol#5737, ID02-205, 2000–2003

Principal Investigator, A Randomized, Open-Label, Phase III Trial of Decitabine (5-Aza-2' -Deoxycytidine) versus Supportive Care in Adults with Advanced-Stage Myelodysplastic Syndrome, D-0007, 2002

Principal Investigator, A Phase II Multicenter Study Of Decitabine (5-AZA-2'-Deoxycytidine) In Chronic Myelogenous Leukemia Accelerated, Blast and Chronic Phase Refractory to Imatinib Mesylate (STI 571), (Three Protocols), DM02-133, DM02-134 & DM02-135, 2002–2003

Principal Investigator, Phase I Trial of SAHA (NSC 701852) and Decitabine (IND 50733, NSC 127716) in Patients with Relapsed, Refractory or Poor Prognosis Leukemia, 2005-0723, 2005

Principal Investigator, Phase I Study Of 5-AZA-2'-DEOXYCYTIDINE and DEPSIPEPTIDE in Patients with Relapsed/Refractory Leukemia, Myelodysplastic Syndromes, Or Myeloproliferative Disease, NCI Protocol #5563, 2005
Principal Investigator, Randomized phase I/II Study of 5-Azacytidine in Combination with Cytosine Arabinoside in Patients with Relapsed/Refractory Acute Myelogenous Leukemia or High Risk Myelodysplastic Syndrome, 2005-0291, 2005

Principal Investigator, Phase II Randomized Study of Low-Dose Decitabine (5-AZA-2'-Deoxycytidine) with or without Valproic Acid in Myelodysplastic Syndrome (MDS) and Acute Myelogenous Leukemia, 2006-0686, 2006

Principal Investigator, A Phase I Clinical Trial of Vorinostat in Combination with Decitabine in Patients with Acute Myelogenous Leukemia or Myelodysplastic Syndrome., 2006-1096, 2007, $89,062, Merck

Patents and Technology Licenses

Patents: Issued

United States Patent 6,783,933
Issa August 31, 2004
CACNA1G polynucleotide, polypeptide and methods of use therefor
Inventors: Issa; Jean-Pierre (Houston, TX)
Assignee: The Johns Hopkins University School of Medicine (Baltimore, MD)
Family ID: 23575700
Appl. No.: 09/398,522
Filed: September 15, 1999

United States Patent 7,427,476
Issa September 23, 2008
PITX2 polynucleotide, polypeptide and methods of use therefor
Inventors: Issa; Jean-Pierre (Houston, TX)
Assignee: The Johns Hopkins University School of Medicine (Baltimore, MD)
Family ID: 23575700
Appl. No.: 10/930,301
Filed: August 30, 2004

United States Patent 7,700,324

Issa , et al. April 20, 2010
Methylated CpG island amplification (MCA)
Inventors: Issa; Jean-Pierre (Timonium, MD), Baylin; Stephen (Baltimore, MD), Toyota; Minoru (Baltimore, MD)
Assignee: The Johns Hopkins University School of Medicine (Baltimore, MD)
Family ID: 26804186
Appl. No.: 09/309,175
Filed: May 10, 1999

United States Patent 8,133,986
Issa , et al. March 13, 2012
Methylated CpG island amplification (MCA)
Inventors: Issa; Jean-Pierre (Timonium, MD), Baylin; Stephen (Baltimore, MD), Toyota; Minoru (Baltimore, MD)
Assignee: The Johns Hopkins University School of Medicine (Baltimore, MD)
Family ID: 26804186
Appl. No.: 12/763,917
Filed: April 20, 2010

United States Patent 8,609,343
Chung; Woonbok ; et al. December 17, 2013
DETECTION OF BLADDER CANCER
Inventors: Chung; Woonbok; (Haverford, PA) ; Czerniak; Bogdan A.; (Houston, TX) ; Issa; Jean-Pierre; (Philadelphia, PA)
Family ID: 46795770
Filed: March 12, 2012

United States Patent 8,642,271
Markowitz; Sanford D. ; et al. February 4, 2014
Aberrant Methylation of C60RF150 DNA Sequences In Human Colorectal CancerInventors:Markowitz; Sanford D.; (Pepper Pike, OH) ; Moinova; Helen; (Beachwood, OH) ; Myeroff; Lois; (Chardon, OH) ; Issa; Jean-Pierre; (Bellaire, TX) ; Maeda; Osamu; (Nagoya, JP)
Family ID: 44224918
Filed: August 27, 2010

Patents: Filed and/or pending

United States Patent Application 20060019270
Yang; Allen S. ; et al. January 26, 2006
Global DNA methylation assessment using bisulfite PCR
Inventors: Yang; Allen S.; (Valenica, CA) ; Issa; Jean-Pierre; (Bellaire, TX) ; Estecio; Marcos; (Houston, TX)
Assignee: Board of Regents The University of Texas System
Family ID: 35657651
Serial No.: 096453
Series Code: 11
Filed: April 1, 2005

United States Patent Application 20080234223
Kind Code A1

Yang; Allen S. ; et al. September 25, 2008
N4 MODIFICATIONS OF PYRIMIDINE ANALOGS AND USES THEREOF
Inventors: Yang; Allen S.; (Valencia, CA) ; Marquez; Victor; (Los Angeles, CA) ; Byun; Hyang-Min; (Glendale, CA) ; Issa; Jean-Pierre; (Bellaire, TX)
Assignee: UNIVERSITY OF SOUTHERN CALIFORNIA
Los Angeles
CA
Family ID: 39344894
Serial No.: 929556
Series Code: 11
Filed: October 30, 2007

Patents: Invention disclosures

MDA06-121, A series of cell lines that can be used to screen for factors (drugs, molecules, natural substances) that reverse gene silencing by DNA methylation and histone deacetylation, United States, MDA06-121, 1/12/2007, Filed
MD Anderson, Digital Restriction Enzyme Analysis of Methylation (DREAM), United States, MDA09-032, Filed

Technology Licenses
United States Patent 6,783,933 CACNA1G polynucleotide, polypeptide and methods of use therefor (Licensed to Epigenomics)
United States Patent 7,427,476 PITX2 polynucleotide, polypeptide and methods of use therefor (Licensed to Epigenomics)
United States Patent 7,700,324 and 8,133,986 Methylated CpG island amplification (MCA) (Licensed to Epigenomics)

Grant Reviewer/Service on Study Sections

- PAR061 study section, Reviewer, 2002
- PO1 review study section, Reviewer, 2002
- NCI Cancer Prognosis & Prediction study section, Reviewer, October 23-24, 2003
- Site Visit, NCI Intramural program, Laboratory of Immunobiology, Member, October 1-3, 2003
- NCI Innovative Molecular Analysis Technologies Study Section, Member, July 27-28, 2004
- NCI Metabolism section, Reviewer 2004
- Site Visit, NCI Intramural program, Surgery Branch, Member, September 8-10, 2004
- Site Visit, NCI Laboratory of Biosystems and Cancer, Member September 19-21, 2004
- VA merit grants, Hong Kong development grants, MRC program reviews, Ad Hoc Reviewer, 2004
- Ad Hoc Reviewer Cancer Research UK grants, Reviewer, 2005
- Epigenetics of Neurobiology and Addiction, Reviewer, May 15-16, 2006
- Innovative Technologies for the Molecular Analysis of Cancer Review Committee, Reviewer, March 22-23, 2006
- Technology Development in Epigenetics, NIH Center for Scientific Review Special Emphasis Panel, ZRG1 CB-B (50), NIH, Reviewer, July 17, 2008

- Cancer Genetics Study Section, NIH Center for Scientific Review, ad-hoc Reviewer, February 9-10, 2009, ad-hoc reviewer, 2011
- MCH Study Section, NIH Center for Scientific Review, Reviewer, ad-hoc 2012, permanent member 2013-2019
- Special Emphasis Panel II Study Section, NIH Center for Scientific Review, adhoc reviewer 2012, 2013, 2014
- ACSO, Young Investigator Awards grant reviewer, 2013-2014

EDITORIAL AND REVIEW ACTIVITIES

Editor/Service on Editorial Board(s)

Editorial Board, Cancer Biology & Therapy, 2001–
Editorial Board, Clinical Cancer Research, 2002–2012
Editorial Board, Cancer Research, 2003–2012
Editorial Board, Molecular Cancer Research, 2003–2009
Editorial Board, Current Cancer Therapy Reviews, 2004–
Editorial Board, Clinical Leukemia, 2006
Editorial Board, JNCI, 2010-present
Editorial Board, Journal of Clinical Oncology, 2009–2015
Senior Editor, Cancer Prevention Research, 2008–present
Senior Editor, Molecular Cancer Research, 2009–2013
Associate Editor, Clinical Epigenetics, 2010-2014
Editorial Board, Cancer Research, 2015–

Journal Reviewer

Reviewer for, Science, Nature, Nature Genetics, Nature Medicine, NEJM, Leukemia, Cancer Research, Blood, Carcinogenesis, Clinical Cancer Research, Gut, Gastroenterology, American Journal of Pathology, JNCI, Cancer, MCB, PNAS, JBC, JCI, Leukemia Research, Human Genetics, Oncogene, Laboratory Investigation, American Journal of Pharmacogenomics, CEBP, CaPR, MCR etc.

PROFESSIONAL MEMBERSHIPS/ACTIVITIES

American Association for Cancer Research (1992-Present)
American Society of Clinical Oncology (1992-Present)
American Society of Hematology (2002-Present)
American Society for Clinical Investigation (2003-Present)
American Association of Physicians (2015-present)

MENTORSHIP

Since 2004, I have mentored over 100 students, post-doctoral fellows, junior faculty members and visiting scientists. Six students received a PhD while working in my lab at MD Anderson and 5 students are currently working towards a PhD in my lab at Temple University. Some of the individuals who trained in my laboratory include:

- Dr. Minoru Toyota (post-doctoral fellow 1995-1999), who became chair of Biochemistry at Sapporo Medical University in Japan [Dr. Toyota passed away in 2011]
- Dr. Nita Ahuja (post-doctoral fellow 1996-1999), who is currently Associate Professor and Deputy Chair, Department of Surgery, Johns Hopkins Hospital

- Dr. Lanlan Shen (post-doctoral fellow 1999-2005), currently Associate Professor, Baylor School of Medicine
- Dr. Guillermo Garcia Manero (mentored junior faculty 2001-2003), currently Professor and Chief MDS section, leukemia Department, MD Anderson
- Dr. Yutaka Kondo (post-doctoral fellow 2002-2007), currently Professor and Dept Chair, Nagoya City University
- Dr. Betsy Plimack (Masters in clinical research, 2007-2009), currently Associate Professor, Fox Chase Cancer Center
- Dr. Yanis Boumber (PhD student, 2000-2004), currently Assistant Professor, Fox Chase Cancer Center
- Dr. Noel Raynal (post-doctoral fellow 2009-2013), currently Assistant Professor, Hopital Ste Justine, Montreal.

RESEARCH SUMMARY

Personal Statement: I trained in medical oncology at Johns Hopkins, where I started my research career in the field of epigenetics and cancer. I am currently Professor at Temple University and Director of the Fels Institute for Cancer Research. I am also co-leader of a program in Cancer Epigenetics at the Fox Chase Cancer Center (now part of Temple Health). My laboratory and clinical/translational interests are in the area of epigenetics, with particular emphasis on the role of DNA methylation and histone modifications in aging, cancer development and as a target for prevention and therapy for cancer. Some of the findings from my laboratory include the first description of age-related promoter hypermethylation (1994), the discovery of the CpG Island Methylator Phenotype (1999), bench to bedside clinical trials of the hypomethylating drug decitabine (2004) which contributed to its eventual FDA approval (2006), and the discovery of methylation-independent gene silencing in cancer by Polycomb group proteins (2008). I have also been involved in studies of environmental and lifestyle effects on DNA methylation, the identification of epigenetic field defects as early markers of neoplasia risk, whole epigenome studies with discovery of markers for screening and prognosis, and screening for epigenetic acting drugs to treat patients with malignancies. Google Scholar calculates an H-index of 110 and >46,000 citations for my publications (https://scholar.google.com/citations?user=ujjcPF0AAAAJ&hl=en)

CONTRIBUTIONS TO SCIENCE

1. **Discovery of age-related DNA methylation drift.** DNA methylation is a mediator of epigenetic cellular inheritance and is involved in multiple physiologic conditions (development, differentiation, stemness etc.) as well as pathologic conditions (cancer, developmental diseases etc.). Biochemical measurements of total DNA methylation (e.g. 5-methylcytosine content) had suggested alterations in the process in cancer and aging but the significance of these findings were obscure until technology was developed to study specific genes. In the 1980's and 90's, it became clear that DNA methylation of many genes was altered in cancer and a hypothesis was advanced that promoter DNA methylation could serve as a mechanism to stably silence tumor-suppressor genes in cancer. In 1994, I described for the first time that human aging was associated with gains in promoter DNA methylation of a gene (*ERα*) in normal appearing colonic tissues and proposed that age-related methylation disturbances predispose to diseases such as cancer (Issa *et al*, 1994) (>1,000 citations). My lab later showed that age-related DNA methylation changes affected multiple genes and were tissue specific (Ahuja *et al*, 1998) (>500 citations), that both hypermethylation and hypomethylation could be observed (hence the term drift) and that the process was conserved from mice to humans (Maegawa *et al*, 2010) and

finally that age-related methylation was the clearest explanation for methylation disturbances in cancer (Maegawa *et al*, 2014). The study of age-related methylation drift exploded in the past few years with hundreds of publications confirming and extending my lab's original findings, and it is currently one of the hottest topics in aging research. A Pubmed search for Aging DNA methylation reveals over 1200 papers.

- Ahuja N, Li Q, Mohan AL, Baylin SB, **Issa JP** (1998) Aging and DNA methylation in colorectal mucosa and cancer. Cancer Res 58(23): 5489-94
- **Issa JP**, Ottaviano YL, Celano P, Hamilton SR, Davidson NE, Baylin SB (1994) Methylation of the oestrogen receptor CpG island links ageing and neoplasia in human colon. Nat Genet 7(4): 536-40
- Maegawa S, Hinkal G, Kim HS, Shen L, Zhang L, Zhang J, Zhang N, Liang S, Donehower LA, **Issa JP** (2010) Widespread and tissue specific age-related DNA methylation changes in mice. Genome Res 20(3): 332-40
- Maegawa S, Gough SM, Watanabe-Okochi N, Lu Y, Zhang N, Castoro RJ, Estecio MR, Jelinek J, Liang S, Kitamura T, Aplan PD, **Issa JP** (2014) Age-related epigenetic drift in the pathogenesis of MDS and AML. Genome Res 24(4): 580-91

2. **Discovery of the CpG Island Methylator Phenotype (CIMP).** When it was proposed by the labs of Dr. Stephen Baylin (my mentor) and Dr. Peter Jones that DNA methylation could be exploited by cancer cells to silence tumor-suppressor genes, many scientists were skeptical. It was argued that DNA methylation changes were an artifact of transformation and cell culture and could not provide the same selective advantage as mutations in causing cancer. In 1998, I discovered that a subset of colon cancers was characterized by intense aberrant DNA methylation of multiple genes, a phenomenon which I named CIMP (Toyota *et al*, 1999) (>1800 citations). My lab proposed that this non-Gaussian distribution of aberrant DNA methylation proves that it is selected for and not an artifact of transformation (Issa, 2004) (>800 citations). Moreover, I compared CIMP to genetic instability phenotypes, and I proposed the hypothesis that CIMP (and by extension aberrant DNA methylation) could have a traceable cause such as a mutation. My lab subsequently showed for the first time that CIMP was associated with distinct genetic changes (Toyota *et al*, 2000), that it was present in many other cancers (e.g. gastric cancer) and that a combined genetic/epigenetic classifier divided colon cancer into very distinct and clinically relevant subsets (Shen *et al*, 2007). While CIMP was initially controversial, the concept was validated in colon cancer and extended by TCGA studies to multiple other malignancies where it was shown to be highly prognostic, potentially caused by Krebs cycle alterations in some cases, while a primary molecular abnormality in other cases. A Pubmed search for CIMP/CpG Island Methylator Phenotype reveals over 750 papers.

- **Issa JP** (2004) CpG island methylator phenotype in cancer. Nat Rev Cancer 4(12): 988-93
- Shen L, Toyota M, Kondo Y, Lin E, Zhang L, Guo Y, Hernandez NS, Chen X, Ahmed S, Konishi K, Hamilton SR, **Issa JP** (2007) Integrated genetic and epigenetic analysis identifies three different subclasses of colon cancer. Proc Natl Acad Sci U S A 104(47): 18654-9
- Toyota M, Ahuja N, Ohe-Toyota M, Herman JG, Baylin SB, **Issa JP** (1999) CpG island methylator phenotype in colorectal cancer. Proc Natl Acad Sci U S A 96(15): 8681-6
- Toyota M, Ohe-Toyota M, Ahuja N, **Issa JP** (2000) Distinct genetic profiles in colorectal tumors with or without the CpG island methylator phenotype. Proc Natl Acad Sci U S A 97(2): 710-5

3. **Clinical/translational studies of epigenetic therapy.** A paradox in the early days of trying to introduce epigenetic acting drugs in the clinic was the perceived lack of efficacy of DNA methylation inhibitors that were synthesized in the 1960's and tested clinically in the 1970's and 1980's. This was a consistent criticism of the field, along with concerns about non-specificity of the approach and potential mutagenesis of the drugs (and of hypomethylation induction). However, pre-clinical studies showed that DNA methylation inhibitors had biphasic effects – demethylation at low doses and DNA damage/cytotoxicity at high doses and I reasoned that this may have confounded some of the early clinical trials which were based on determining maximally tolerated doses. Focusing on the DNA methylation inhibitor decitabine, I led a clinical/translational group that designed a clinical trial where we showed that low doses of decitabine were more active clinically than the MTD (Issa *et al*, 2004) (>600 citations). This was followed by the pivotal study that led to decitabine's FDA approval in the USA (Kantarjian *et al*, 2006) (>900 citations) and a dose-optimization study that led to the currently used dose schedule of the drug (Kantarjian *et al*, 2007) (>600 citations). In that same trial, we also showed that sustained demethylation of the P15 tumor suppressor was associated with response (same reference). More recently, I led a collaboration between our "epigenetics" Stand Up to Cancer/AACR team and the Astex company to design and complete a molecularly guided phase I study of the new hypomethylation drug SGI110 (Issa, Lancet Oncology 2015, accepted for publication). The field of epigenetic therapy of cancer is now highly active with dozens of drugs in clinical trials.

   - **Issa JP**, Garcia-Manero G, Giles FJ, Mannari R, Thomas D, Faderl S, Bayar E, Lyons J, Rosenfeld CS, Cortes J, Kantarjian HM (2004) Phase 1 study of low-dose prolonged exposure schedules of the hypomethylating agent 5-aza-2'-deoxycytidine (decitabine) in hematopoietic malignancies. Blood 103(5): 1635-40
   - Kantarjian H, **Issa JP**, Rosenfeld CS, Bennett JM, Albitar M, DiPersio J, Klimek V, Slack J, de Castro C, Ravandi F, Helmer R, Shen L, Nimer SD, Leavitt R, Raza A, Saba H (2006) Decitabine improves patient outcomes in myelodysplastic syndromes: results of a phase III randomized study. Cancer 106(8): 1794-803
   - Kantarjian H, Oki Y, Garcia-Manero G, Huang X, O'Brien S, Cortes J, Faderl S, Bueso-Ramos C, Ravandi F, Estrov Z, Ferrajoli A, Wierda W, Shan J, Davis J, Giles F, Saba HI, **Issa JP** (2007) Results of a randomized study of 3 schedules of low-dose decitabine in higher-risk myelodysplastic syndrome and chronic myelomonocytic leukemia. Blood 109(1): 52-7

4. **DNA methylation and histone code interactions.** Both DNA methylation and silencing histone marks (H3K9me2, H3K27me3) are epigenetic processes but their interactions remain a matter of debate. An association between gene marking by polycomb group specific proteins and susceptibility to DNA hypermethylation in cancer (as well as other studies in model organisms) led many to believe that DNA methylation is simply a consequence of chromatin remodeling. Studying DNA methylation - chromatin interactions at cancer-specific loci, my lab showed (for the first time for cancer silencing) that DNA methylation was closely linked to H3K9me2 (Kondo *et al*, 2003) but inversely correlated with H3K27me3 (Kondo *et al*, 2008) (>440 citations), which contradicted earlier thinking about polycomb triggering DNA methylation. These data were confirmed by many labs. Furthermore, we showed that DNA demethylation alone is not sufficient for gene expression (chromatin remodeling is required) and, in turn, chromatin remodeling alone can lead to high level gene expression despite the persistence of DNA methylation (Raynal *et al*, 2012; Si *et al*, 2010). Altogether, our data strongly suggest that DNA methylation and histone code changes are independent processes and confirmed early hypotheses that suggested that the main role of DNA methylation is to provide a memory of the gene silencing state.

- Kondo Y, Shen L, Cheng AS, Ahmed S, Boumber Y, Charo C, Yamochi T, Urano T, Furukawa K, Kwabi-Addo B, Gold DL, Sekido Y, Huang TH, **Issa JP** (2008) Gene silencing in cancer by histone H3 lysine 27 trimethylation independent of promoter DNA methylation. Nat Genet 40(6): 741-50
- Kondo Y, Shen L, **Issa JP** (2003) Critical role of histone methylation in tumor suppressor gene silencing in colorectal cancer. Mol Cell Biol 23(1): 206-15
- Raynal NJ, Si J, Taby RF, Gharibyan V, Ahmed S, Jelinek J, Estécio MR, **Issa JP** (2012) DNA methylation does not stably lock gene expression but instead serves as a molecular mark for gene silencing memory. Cancer Res 72(5): 1170-81
- Si J, Boumber YA, Shu J, Qin T, Ahmed S, He R, Jelinek J, **Issa JP** (2010) Chromatin remodeling is required for gene reactivation after decitabine-mediated DNA hypomethylation. Cancer Res 70(17): 6968-77

5. **Epigenomics.** My laboratory has been involved in DNA methylation profiling studies for 20 years, including method development and studies of methylation/environment interactions as well as methylation/outcomes correlations. For example, my laboratory developed the LINE1 methylation assay as a surrogate for global methylation levels (Yang *et al*, 2004) (>570 citations). My group was among the first to show a link between chronic inflammation and aberrant DNA methylation (Issa *et al*, 2001) (>500 citations), to suggest that aberrant DNA methylation is present in "fields" of cancer predisposition (Shen *et al*, 2005) (>300 citations) and to demonstrate geographic variation in aberrant DNA methylation, suggesting an environmental effect (Shen *et al*, 2002) (>200 citations).

   - Issa JP, Ahuja N, Toyota M, Bronner MP, Brentnall TA (2001) Accelerated age-related CpG island methylation in ulcerative colitis. Cancer Res 61(9): 3573-7
   - Shen L, Ahuja N, Shen Y, Habib NA, Toyota M, Rashid A, Issa JP (2002) DNA methylation and environmental exposures in human hepatocellular carcinoma. J Natl Cancer Inst 94(10): 755-61
   - Shen L, Kondo Y, Rosner GL, Xiao L, Hernandez NS, Vilaythong J, Houlihan PS, Krouse RS, Prasad AR, Einspahr JG, Buckmeier J, Alberts DS, Hamilton SR, Issa JP (2005) MGMT promoter methylation and field defect in sporadic colorectal cancer. J Natl Cancer Inst 97(18): 1330-8
   - Yang AS, Estécio MR, Doshi K, Kondo Y, Tajara EH, Issa JP (2004) A simple method for estimating global DNA methylation using bisulfite PCR of repetitive DNA elements. Nucleic Acids Res 32(3): e38

A full bibliography can be found at: http://www.ncbi.nlm.nih.gov/sites/myncbi/jean-pierre.issa.1/bibliography/40796930/public/?sort=date&direction=ascending

PUBLICATIONS

Research articles

1. **Issa JP**, Vertino PM, Wu J, Sazawal S, Celano P, Nelkin BD, Hamilton SR, Baylin SB. Increased cytosine DNA-methyltransferase activity during colon cancer progression. J Natl Cancer Inst 85(15):1235-40, 8/1993. PMID:8331684

2. Wu J, **Issa JP**, Herman J, Bassett DE, Jr, Nelkin BD, Baylin SB. Expression of an exogenous eukaryotic DNA methyltransferase gene induces transformation of NIH 3T3 cells. Proc Natl Acad Sci U S A 90(19):8891-5, 10/1993. PMID:8415627
3. Ottaviano YL, **Issa JP**, Parl FF, Smith HS, Baylin SB, Davidson NE. Methylation of the estrogen receptor gene CpG island marks loss of estrogen receptor expression in human breast cancer cells. Cancer Res 54(10):2552-5, 5/1994. PMID:8168078
4. **Issa JP**, Ottaviano YL, Celano P, Hamilton SR, Davidson NE, Baylin SB. Methylation of the Estrogen Receptor CpG island links ageing and neoplasia in human colon. Nat Genet 7(4):536-40, 8/1994. PMID:7951326
5. Vertino PM, **Issa JP**, Pereira-Smith OM, Baylin SB. Stabilization of DNA methyltransferase levels and CpG island hypermethylation precede SV40-induced immortalization of human fibroblasts. Cell Growth Differ 5(12):1395-402, 12/1994. PMID:7696189
6. Wales MM, Biel MA, el Deiry W, Nelkin BD, **Issa JP**, Cavenee WK, Kuerbitz SJ, Baylin SB. p53 activates expression of HIC-1, a new candidate tumour suppressor gene on 17p13.3. Nat Med 1(6):570-7, 6/1995. PMID:7585125
7. Herman JG, Merlo A, Mao L, Lapidus RG, **Issa JP**, Davidson NE, Sidransky D, Baylin SB. Inactivation of the CDKN2/p16/MTS1 gene is frequently associated with aberrant DNA methylation in all common human cancers. Cancer Res 55(20):4525-30, 10/1995. PMID:7553621
8. **Issa JP**, Zehnbauer BA, Civin CI, Collector MI, Sharkis SJ, Davidson NE, Kaufmann SH, Baylin SB. The estrogen receptor CpG island is methylated in most hematopoietic neoplasms. Cancer Res 56(5):973-77, 3/1996. PMID:8640788
9. Belinsky SA, Nikula KJ, Baylin SB, **Issa JP**. Increased cytosine DNA-methyltransferase activity is target-cell-specific and an early event in lung cancer. Proc Natl Acad Sci U S A 93(9):4045-50, 4/1996. PMID:8633014
10. Lapidus RG, Ferguson AT, Ottaviano YL, Parl FF, Smith HS, Weitzman SA, Baylin SB, **Issa JP**, Davidson NE. Methylation of estrogen and progesterone receptor gene 5' CpG islands correlates with lack of estrogen and progesterone receptor gene expression in breast tumors. Clin Cancer Res 2(5):805-10, 5/1996. PMID:9816234
11. **Issa JP**, Baylin SB, Belinsky SA. Methylation of the estrogen receptor CpG island in lung tumors is related to the specific type of carcinogen exposure. Cancer Res 56(16):3655-8, 8/1996. PMID:8706002
12. **Issa JP**, Vertino PM, Boehm CD, Newsham IF, Baylin SB. Switch from monoallelic to biallelic human IGF2 promoter methylation during aging and carcinogenesis. Proc Natl Acad Sci U S A 93(21):11757-62, 10/1996. PMID:8876210
13. Herman JG, Civin CI, **Issa JP**, Collector MI, Sharkis SJ, Baylin SB. Distinct patterns of inactivation of p15INK4B and p16INK4A characterize the major types of hematological malignancies. Cancer Res 57(5):837-41, 3/1997. PMID:9041182
14. **Issa JP**, Zehnbauer BA, Kaufmann SH, Biel MA, Baylin SB. HIC1 hypermethylation is a late event in hematopoietic neoplasms. Cancer Res 57(9):1678-81, 5/1997. PMID:9135007
15. Ahuja N, Mohan AL, Li Q, Stolker JM, Herman JG, Hamilton SR, Baylin SB, **Issa JP**. Association between CpG island methylation and microsatellite instability in colorectal cancer. Cancer Res 57(16):3370-4, 8/1997. PMID:9269998
16. Li Q, Jedlicka A, Ahuja N, Gibbons MC, Baylin SB, Burger PC, **Issa JP**. Concordant methylation of the ER and N33 genes in glioblastoma multiforme. Oncogene 16(24):3197-202, 6/1998. PMID:9671399
17. Herman JG, Umar KiA, Polyak K, Graff JR, Ahuja N, **Issa JP**, Markowitz S, Willson JK, Hamilton SR, Kinzler KW, Kane MF, Kolodner RD, Vogelstein B, Kunkel TA, Baylin SB. Incidence and functional consequences of hMLH1 promoter hypermethylation in colorectal carcinoma. Proc Natl Acad Sci U S A 95(12):6870-5, 6/1998. PMID:9618505

18. Mikovits JA, Young HA, Vertino P, **Issa JP**, Pitha PM, Turcoski-Corrales S, Taub DD, Petrow CL, Baylin SB, Ruscetti FW. Infection with human immunodeficiency virus type 1 upregulates DNA methyltransferase, resulting in de novo methylation of the gamma interferon (IFN-gamma) promoter and subsequent downregulation of IFN-gamma production. Mol Cell Biol 18(9):5166-77, 9/1998. PMID:9710601
19. Ahuja N, Li Q, Mohan AL, Baylin SB, **Issa JP**. Aging and DNA methylation in colorectal mucosa and cancer. Cancer Res 58(23):5489-94, 12/1998. PMID:9850084
20. **Issa JP**, Kantarjian H, Mohan A, O'Brien S, Cortes J, Pierce S, Talpaz M. Methylation of the ABL1 promoter in chronic myelogenous leukemia: lack of prognostic significance. Blood 93(6):2075-80, 3/1999. PMID:10068681
21. Li Q, Kopecky KJ, Mohan A, Willman CL, Appelbaum FR, Weick JK, **Issa JP**. Estrogen receptor methylation is associated with improved survival in adult acute myeloid leukemia. Clin Cancer Res 5(5):1077-84, 5/1999. PMID:10353741
22. Toyota M, Ho C, Ahuja N, Jair KW, Li Q, Ohe-Toyota M, Baylin SB, **Issa JP**. Identification of differentially methylated sequences in colorectal cancer by methylated CpG island amplification. Cancer Res 59(10):2307-12, 5/1999. PMID:10344734
23. Li Q, Ahuja N, Burger PC, **Issa JP**. Methylation and silencing of the Thrombospondin-1 promoter in human cancer. Oncogene 18(21):3284-9, 5/1999. PMID:10359534
24. Toyota M, Ahuja N, Ohe-Toyota M, Herman JG, Baylin SB, **Issa JP**. CpG island methylator phenotype in colorectal cancer. Proc Natl Acad Sci U S A 96(15):8681-6, 7/1999. PMID:10411935
25. Toyota M, Ho C, Ohe-Toyota M, Baylin SB, **Issa JP**. Inactivation of CACNA1G, a T-type calcium channel gene, by aberrant methylation of its 5' CpG island in human tumors. Cancer Res 59(18):4535-41, 9/1999. PMID:10493502
26. Post WS, Goldschmidt-Clermont PJ, Wilhide CC, Heldman AW, Sussman MS, Ouyang P, Milliken EE, **Issa JP**. Methylation of the estrogen receptor gene is associated with aging and atherosclerosis in the cardiovascular system. Cardiovasc Res 43(4):985-91, 9/1999. PMID:10615426
27. Toyota M, Ahuja N, Suzuki H, Itoh F, Ohe-Toyota M, Imai K, Baylin SB, **Issa JP**. Aberrant methylation in gastric cancer associated with the CpG island methylator phenotype. Cancer Res 59(21):5438-42, 11/1999. PMID :10554013
28. Toyota M, Ohe-Toyota M, Ahuja N, **Issa JP**. Distinct genetic profiles in colorectal tumors with or without the CpG island methylator phenotype. Proc Natl Acad Sci U S A 97(2):710-5, 1/2000. PMID:10639144
29. Ueki T, Toyota M, Sohn T, Yeo CJ, **Issa JP**, Hruban RH, Goggins M. Hypermethylation of multiple genes in pancreatic adenocarcinoma. Cancer Res 60(7):1835-9, 4/2000. PMID:10766168
30. Ying AK, Hassanain HH, Roos CM, Smiraglia DJ, **Issa JP**, Michler RE, Caligiuri M, Plass C, Goldschmidt-Clermont PJ. Methylation of the estrogen receptor-alpha gene promoter is selectively increased in proliferating human aortic smooth muscle cells. Cardiovasc Res 46(1):172-9, 4/2000. PMID:10727665
31. Esteller M, Toyota M, Sanchez-Cespedes M, Capella G, Peinado MA, Watkins DN, **Issa JP**, Sidransky D, Baylin SB, Herman JG. Inactivation of the DNA repair gene O6-methylguanine-DNA methyltransferase by promoter hypermethylation is associated with G to A mutations in K-ras in colorectal tumorigenesis. Cancer Res 60(9):2368-71, 5/2000. PMID:10811111
32. Toyota M, Shen L, Ohe-Toyota M, Hamilton SR, Sinicrope FA, **Issa JP**. Aberrant methylation of the Cyclooxygenase 2 CpG island in colorectal tumors. Cancer Res 60(15):4044-8, 8/2000. PMID:10945606
33. **Issa JP**, Ahuja N, Toyota M, Bronner MP, Brentnall TA. Accelerated age-related CpG island methylation in ulcerative colitis. Cancer Res 61(9):3573-7, 5/2001. PMID:11325821

34. Toyota M, Kopecky KJ, Toyota MO, Jair KW, Willman CL, **Issa JP**. Methylation profiling in acute myeloid leukemia. Blood 97(9):2823-9, 5/2001. PMID:11313277
35. Luo RZ, Peng H, Xu F, Bao J, Pang Y, Pershad R, **Issa JP**, Liao WS, Bast RC, Jr, Yu Y. Genomic structure and promoter characterization of an imprinted tumor suppressor gene ARHI. Biochim Biophys Acta 1519(3):216-22, 6/2001. PMID:11418188
36. Rashid A, Shen L, Morris JS, **Issa JP**, Hamilton SR. CpG island methylation in colorectal adenomas. Am J Pathol 159(3):1129-35, 9/2001. PMID:11549606
37. Peng H, Shen N, Qian L, Sun XL, Koduru P, Goodwin LO, **Issa JP**, Broome JD. Hypermethylation of CpG islands in the mouse asparagine synthetase gene: relationship to asparaginase sensitivity in lymphoma cells. Partial methylation in normal cells. Br J Cancer 85(6):930-5, 9/2001. PMID:11556848
38. Anderson GR, Brenner BM, Swede H, Chen N, Henry WM, Conroy JM, Karpenko MJ, **Issa JP**, Bartos JD, Brunelle JK, Jahreis GP, Kahlenberg MS, Basik M, Sait S, Rodriguez-Bigas MA, Nowak NJ, Petrelli NJ, Shows TB, Stoler DL. Intrachromosomal genomic instability in human sporadic colorectal cancer measured by genome-wide allelotyping and inter-(simple sequence repeat) PCR. Cancer Res 61(22):8274-83, 11/2001. PMID:11719460
39. Ueki T, Toyota M, Skinner H, Walter KM, Yeo CJ, **Issa JP**, Hruban RH, Goggins M. Identification and characterization of differentially methylated CpG islands in pancreatic carcinoma. Cancer Res 61(23):8540-6, 12/2001. PMID:11731440
40. Moinova HR, Chen WD, Shen L, Smiraglia D, Olechnowicz J, Ravi L, Kasturi L, Myeroff L, Plass C, Parsons R, Minna J, Willson JK, Green SB, **Issa JP**, Markowitz SD. HLTF gene silencing in human colon cancer. Proc Natl Acad Sci U S A 99(7):4562-7, 4/2002. PMID:11904375
41. Kikuchi T, Toyota M, Itoh F, Suzuki H, Obata T, Yamamoto H, Kakiuchi H, Kusano M, **Issa JP**, Tokino T, Imai K. Inactivation of p57KIP2 by regional promoter hypermethylation and histone deacetylation in human tumors. Oncogene 21(17):2741-9, 4/2002. PMID:11965547
42. On-On Chan A, Broaddus RR, Houlihan PS, **Issa JP**, Hamilton SR, Rashid A. CpG island methylation in aberrant crypt foci of the colorectum. Am J Pathol 160:1823-30, 5/2002. PMID:12000733
43. Shen L, Ahuja N, Shen Y, Habib NA, Toyota M, Rashid A, **Issa JP**. DNA methylation and environmental exposures in human hepatocellular carcinoma. J Natl Cancer Inst 94(10):755-61, 5/2002. PMID:12011226
44. Soria JC, Lee HY, Lee JI, Wang L, **Issa JP**, Kemp BL, Liu DD, Kurie JM, Mao L, Khuri FR. Lack of PTEN expression in non-small cell lung cancer could be related to promoter methylation. Clin Cancer Res 8(5):1178-84, 5/2002. PMID:12006535
45. Garcia-Manero G, Bueso-Ramos C, Daniel J, Williamson J, Kantarjian HM, **Issa JP**. DNA methylation patterns at relapse in adult acute lymphocytic leukemia. Clin Cancer Res 8(6):1897-903, 6/2002. PMID:12060634
46. Garcia-Manero G, Daniel J, Smith TL, Kornblau SM, Lee MS, Kantarjian HM, **Issa JP**. DNA methylation of multiple promoter-associated CpG islands in adult acute lymphocytic leukemia. Clin Cancer Res 8(7):2217-24, 7/2002. PMID:12114423
47. Kantarjian HM, Talpaz M, O'Brien S, Smith TL, Giles FJ, Faderl S, Thomas DA, Garcia-Manero G, **Issa JP**, Andreeff M, Kornblau SM, Koller C, Beran M, Keating M, Rios MB, Shan J, Resta D, Capdeville R, Hayes K, Albitar M, Freireich EJ, Cortes JE. Imatinib mesylate for Philadelphia chromosome-positive, chronic-phase myeloid leukemia after failure of interferon-alpha: follow-up results. Clin Cancer Res 8(7):2177-87, 7/2002. PMID:12114418
48. Kantarjian HM, O'Brien S, Cortes JE, Smith TL, Rios MB, Shan J, Yang Y, Giles FJ, Thomas DA, Faderl S, Garcia-Manero G, Jeha S, Wierda W, **Issa JP**, Kornblau SM, Keating M, Resta D, Capdeville R, Talpaz M. Treatment of philadelphia chromosome-positive, accelerated-phase chronic myelogenous leukemia with imatinib mesylate. Clin Cancer Res 8(7):2167-76, 7/2002. PMID:12114417

49. Shen L, Kondo Y, **Issa JP**, Garcia-Manero G. Lack of p21(CIP1) DNA methylation in acute lymphocytic leukemia. Blood 100(9):3432-3; author reply 3433-4, 11/2002. PMID:12412576
50. On-On Chan A, **Issa JP**, Morris JS, Hamilton SR, Rashid A. Concordant CpG Island Methylation in Hyperplastic Polyposis. American Journal of Pathology 160:529-536, 2002. PMID:11839573
51. Garcia-Manero G, Jeha S, Daniel J, Williamson J, Albitar M, Kantarjian HM, **Issa JP**. Aberrant DNA methylation in pediatric patients with acute lymphocytic leukemia. Cancer 97(3):695-702, 2/2003. PMID:12548613
52. On-On Chan A, Kim SG, Bedeir A, **Issa JP**, Hamilton SR, Rashid A. CpG island methylation in carcinoid and pancreatic endocrine tumors. Oncogene 22:924-34, 2/2003. PMID:12584572
53. Shen L, Kondo Y, Hamilton SR, Rashid A, **Issa JP**. P14 methylation in human colon cancer is associated with microsatellite instability and wild-type p53. Gastroenterology 124(3):626-33, 3/2003. PMID:12612901
54. Shen L, Toyota M, Kondo Y, Obata T, Daniel S, Pierce S, Imai K, Kantarjian HM, **Issa JP**, Garcia-Manero G. Aberrant DNA methylation of p57KIP2 identifies a cell-cycle regulatory pathway with prognostic impact in adult acute lymphocytic leukemia. Blood 101(10):4131-6, 5/2003. PMID:12586619
55. Kim SG, Chan AO, Wu TT, **Issa JP**, Hamilton SR, Rashid A. Epigenetic and genetic alterations in duodenal carcinomas are distinct from biliary and ampullary carcinomas. Gastroenterology 124(5):1300-10, 5/2003. PMID:12730870
56. Toyota M, Sasaki Y, Satoh A, Ogi K, Kikuchi T, Suzuki H, Mita H, Tanaka N, Itoh F, **Issa JP**, Jair KW, Schuebel KE, Imai K, Tokino T. Epigenetic inactivation of CHFR in human tumors. Proc Natl Acad Sci U S A 100(13):7818-23, 6/2003. PMID:12810945
57. Yuan J, Luo RZ, Fujii S, Wang L, Hu W, Andreeff M, Pan Y, Kadota M, Oshimura M, Sahin AA, **Issa JP**, Bast RC, Jr, Yu Y. Aberrant methylation and silencing of ARHI, an imprinted tumor suppressor gene in which the function is lost in breast cancers. Cancer Res 63(14):4174-80, 7/2003. PMID:12874023
58. Kondo Y, **Issa JP**. Enrichment for histone H3 lysine 9 methylation at Alu repeats in human cells. J Biol Chem 278(30):27658-62, 7/2003. PMID:12724318
59. Colella S, Shen L, Baggerly KA, **Issa JP**, Krahe R. Sensitive and quantitative universal Pyrosequencing methylation analysis of CpG sites. Biotechniques 35(1):146-50, 7/2003. PMID:12866414
60. Li H, Myeroff L, Smiraglia D, Romero MF, Pretlow TP, Kasturi L, Lutterbaugh J, Rerko RM, Casey G, **Issa JP**, Willis J, Willson JK, Plass C, Markowitz SD. SLC5A8, a sodium transporter, is a tumor suppressor gene silenced by methylation in human colon aberrant crypt foci and cancers. Proc Natl Acad Sci U S A 100(14):8412-7, 7/2003. PMID:12829793
61. Frazier ML, Xi L, Zong J, Viscofsky N, Rashid A, Wu EF, Lynch PM, Amos CI, **Issa JP**. Association of the CpG island methylator phenotype with family history of cancer in patients with colorectal cancer. Cancer Res 63(16):4805-8, 8/2003. PMID:12941799
62. Fujii S, Luo RZ, Yuan J, Kadota M, Oshimura M, Dent SR, Kondo Y, **Issa JP**, Bast RC, Jr, Yu Y. Reactivation of the silenced and imprinted alleles of ARHI is associated with increased histone H3 acetylation and decreased histone H3 lysine 9 methylation. Hum Mol Genet 12(15):1791-800, 8/2003. PMID:12874100
63. Kantarjian HM, O'Brien S, Cortes J, Giles FJ, Faderl S, **Issa JP**, Garcia-Manero G, Rios MB, Shan J, Andreeff M, Keating M, Talpaz M. Results of decitabine (5-aza-2'deoxycytidine) therapy in 130 patients with chronic myelogenous leukemia. Cancer 98(3):522-8, 8/2003. PMID:12879469
64. Cavalli LR, Urban CA, Dai D, de Assis S, Tavares DC, Rone JD, Bleggi-Torres LF, Lima RS, Cavalli IJ, **Issa JP**, Haddad BR. Genetic and epigenetic alterations in sentinel lymph nodes metastatic

lesions compared to their corresponding primary breast tumors. Cancer Genet Cytogenet 146(1):33-40, 10/2003. PMID:14499694
65. Yang AS, Estecio MR, Garcia-Manero G, Kantarjian HM, **Issa JP**. Comment on "Chromosomal instability and tumors promoted by DNA hypomethylation" and "Induction of tumors in nice by genomic hypomethylation." Science 302(5648):1153; author reply 1153, 11/2003. PMID:14615517
66. Kim SG, Wu TT, Lee JH, Yun YK, **Issa JP**, Hamilton SR, Rashid A. Comparison of epigenetic and genetic alterations in mucinous cystic neoplasm and serous microcystic adenoma of pancreas. Mod Pathol 16(11):1086-94, 11/2003. PMID:14614047
67. Belinsky SA, Klinge DM, Stidley CA, **Issa JP**, Herman JG, March TH, Baylin SB. Inhibition of DNA methylation and histone deacetylation prevents murine lung cancer. Cancer Res 63(21):7089-93, 11/2003. PMID:14612500
68. Obata T, Toyota M, Satoh A, Sasaki Y, Ogi K, Akino K, Suzuki H, Murai M, Kikuchi T, Mita H, Itoh F, **Issa JP**, Tokino T, Imai K. Identification of HRK as a target of epigenetic inactivation in colorectal and gastric cancer. Clin Cancer Res 9(17):6410-8, 12/2003. PMID:14695142
69. Kondo Y, Shen L, **Issa JP**. Critical role of histone methylation in tumor suppressor gene silencing in colorectal cancer. Mol Cell Biol 23(1):206-15, 2003. PMID:12482974
70. Youssef EM, Lotan D, **Issa JP**, Wakasa K, Fan YH, Mao L, Hassan K, Feng L, Lee JJ, Lippman SM, Hong WK, Lotan R. Hypermethylation of the retinoic acid receptor-beta(2) gene in head and neck carcinogenesis. Clin Cancer Res 10(5):1733-42, 3/2004. PMID:15014026
71. **Issa JP**, Garcia-Manero G, Giles FJ, Mannari R, Thomas D, Faderl S, Bayar E, Lyons J, Rosenfeld CS, Cortes J, Kantarjian HM. Phase 1 study of low-dose prolonged exposure schedules of the hypomethylating agent 5-aza-2'-deoxycytidine (decitabine) in hematopoietic malignancies. Blood 103(5):1635-40, 3/2004. PMID:14604977
72. Youssef EM, Chen XQ, Higuchi E, Kondo Y, Garcia-Manero G, Lotan R, **Issa JP**. Hypermethylation and silencing of the putative tumor suppressor Tazarotene-induced gene 1 in human cancers. Cancer Res 64(7):2411-7, 4/2004. PMID:15059893
73. Kondo Y, Shen L, Yan PS, Huang TH, **Issa JP**. Chromatin immunoprecipitation microarrays for identification of genes silenced by histone H3 lysine 9 methylation. Proc Natl Acad Sci USA 101(19):7398-403, 5/2004. PMID:15123805
74. Maruya S, **Issa JP**, Weber RS, Rosenthal DI, Haviland JC, Lotan R, El-Naggar AK. Differential methylation status of tumor-associated genes in head and neck squamous carcinoma: incidence and potential implications. Clin Cancer Res 10(11):3825-30, 6/2004. PMID:15173091
75. Youssef EM, **Issa JP**, Lotan R. Regulation of RARbeta1 expression in head and neck cancer cells by cell density-dependent chromatin remodeling. Cancer Biol Ther 3(10):1002-6, 10/2004. PMID:15467435
76. Yang AS, Estecio MR, Doshi K, Kondo Y, Tajara EH, **Issa JP**. A simple method for assessing global DNA methylation using bisulfite PCR of repetitive DNA elements. Nucleic Acids Res 32:e38, 2004. PMID:14973332
77. Youssef EM, Estecio MR, **Issa JP**. Methylation and regulation of expression of different retinoic acid receptor beta isoforms in human colon cancer. Cancer Biol Ther 3(1):82-6, 2004. PMID:14726683
78. Murai M, Toyota M, Satoh A, Suzuki H, Akino K, Mita H, Sasaki Y, Ishida T, Shen L, Garcia-Manero G, **Issa JP**, Hinoda Y, Tokino T, Imai K. Aberrant DNA methylation associated with silencing BNIP3 gene expression in haematopoietic tumours. Br J Cancer 92(6):1165-72, 3/2005. PMID:15756280
79. **Issa JP**, Gharibyan V, Cortes J, Jelinek J, Morris G, Verstovsek S, Talpaz M, Garcia-Manero G, Kantarjian HM. Phase II study of low-dose decitabine in patients with chronic myelogenous leukemia resistant to imatinib mesylate. J Clin Oncol 10:3948-56, 6/2005. PMID:15883410

80. Canalli AA, Yang H, Jeha S, Hoshino K, Sanchez-Gonzalez B, Brandt M, Pierce S, Kantarjian H,**Issa JP**, Garcia-Manero G. Aberrant DNA methylation of a cell cycle regulatory pathway composed of P73, P15 and P57KIP2 is a rare event in children with acute lymphocytic leukemia. Leuk Res 29(8):881-5, 8/2005. PMID:15978938
81. Jelinek J, Oki Y, Gharibyan V, Bueso-Ramos C, Prchal JT, Verstovsek S, Beran M, Estey E, Kantarjian HM, **Issa JP**. JAK2 mutation 1849G>T is rare in acute leukemias but can be found in CMML, Philadelphia chromosome-negative CML, and megakaryocytic leukemia. Blood 106(10):3370-3, 11/2005. PMID:16037387
82. Beach,R., Chan,A.O., Wu,T.T., White,J.A., Morris,J.S., Lunagomez,S., Broaddus,R.R., **Issa JP**, Hamilton,S.R., and Rashid,A. BRAF mutations in aberrant crypt foci and hyperplastic polyposis. Am.J.Pathol 166:1069-1075, 2005. PMID:15793287
83. Chan AO, Soliman AS, Zhang Q, Rashid A, Bedier A, Houlihan PS, Mokhtar N, Al-Masri N, Ozbeck U, Yaghan R, Kandilci A, Omar S, Kapran Y, Dizdaroglu F, Bondy ML, Amos CI, **Issa JP**, Levin B, Hamilton SR. Differing DNA methylation patterns and gene mutation frequencies in colorectal carcinomas from Middle Eastern countries. Clin Cancer Research 11(23):8281-7, 2005. PMID:16322286
84. Shen L, Kondo Y, Rosner GL, Xiao L, Hernandez NS, Vilaythong J, Houlihan PS, Krouse PS, Prasad AR, Einsphar JG, Buckmeier J, Alberts DS, Hamilton SR, **Issa JP**. MGMT promoter methylation and field defect in sporadic colorectal cancer. J Natl Cancer Inst 97(18):1330-8, 2005. PMID:16174854
85. Chirieac,L.R., Shen,L., Catalano,P.J., **Issa JP**, and Hamilton,S.R. Phenotype of microsatellite-stable colorectal carcinomas with CpG island methylation. Am.J.Surg.Pathol 29:429-436, 2005. PMID:15767794
86. Akino,K., Toyota,M., Suzuki,H., Mita,H., Sasaki,Y., Ohe-Toyota,M., **Issa JP**, Hinoda,Y., Imai,K., and Tokino,T. The Ras effector RASSF2 is a novel tumor-suppressor gene in human colorectal cancer. Gastroenterology 129:156-169, 2005. PMID:16012945
87. Yang AS, Doshi KD, Choi SW, Mason JB, Mannari RK, Gharybian V, Luna R, Rashid A, Shen L, Estecio MR, Kantarjian HM, Garcia-Manero G, **Issa JP**. DNA methylation changes after 5-aza-2'-deoxycytidine therapy in patients with leukemia. Cancer Res 66:5495-503, 5/2006. PMID:16707479
88. Estecio MR, Youssef EM, Rahal P, Fukuyama EE, Gois-Filho JF, Maniglia JV, Goloni-Bertollo EM, **Issa JP**, Tajara EH. LHX6 is a sensitive methylation marker in head and neck carcinomas. Oncogene 25:5018-26, 8/2006. PMID:16899610
89. Gollob JA, Sciambi CJ, Peterson BL, Richmond T, Thoreson M, Moran K, Dressman HK, Jelinek J, **Issa JP**. Phase I trial of sequential low-dose 5-aza-2'-deoxycytidine plus high-dose intravenous bolus interleukin-2 in patients with melanoma or renal cell carcinoma. Clin Cancer Res 12(15):4619-27, 8/2006. PMID:16899610
90. Tabe Y, Konopleva M, Kondo Y, Contractor R, Jin L, Ruvolo V, Tsutsumi-Ishii Y, Miyake K, Miyake N, Ohsaka A, Nagaoka I, **Issa JP**, Andreeff M. PML-RARalpha and AML1-ETO translocations are rarely associated with methylation of the RARbeta2 promoter. Ann Hematol 85:689-704, 10/2006. PMID:16832676
91. Garcia-Manero G, Kantarjian HM, Sanchez-Gonzalez B, Yang H, Rosner G, Verstovsek S, Rytting M, Wierda WG, Ravandi F, Koller C, Xiao L, Faderl S, Estrov Z, Cortes J, O'Brien S, Estey E, Bueso-Ramos C, Fiorentino J, Jabbour E, **Issa JP**. Phase 1/2 study of the combination of 5-aza-2'-deoxycytidine with valproic acid in patients with leukemia. Blood 108:3271-9, 11/2006. PMID:16882711
92. Yu,Y., Luo,R., Lu,Z., Wei,F.W., Badgwell,D., **Issa JP**, Rosen,D.G., Liu,J., and Bast,R.C., Jr. Biochemistry and biology of ARHI (DIRAS3), an imprinted tumor suppressor gene whose expression is lost in ovarian and breast cancers. 407:455-468, 2006. PMID:16757345

93. Kantarjian H, **Issa JP**, Rosenfeld CS, Bennett JM, Albitar M, Dipersio J, Klimek V, Slack J, de Castro C, Ravandi F, Helmer R, Shen L, Nimer SD, Leavitt R, Raza A, Saba H. Decitabine improves patient outcomes in myelodysplastic syndromes: results of a phase III randomized study. Cancer 106(8):1794-803, 2006. PMID:16532500
94. Montero AJ, Diaz-Montero CM, Mao L, Youssef EM, Estecio M, Shen L, **Issa JP**. Epigenetic Inactivation of EGFR by CpG Island Hypermethylation in Cancer. Cancer Biol Ther 5(11):1494-1501, 2006. PMID:17369752
95. Shu J, Jelinek J, Chang H, Shen L, Qin T, Chung W, Oki Y, **Issa JP**. Silencing of bi-directional promoters by DNA methylation in tumorigenesis. Cancer Research 66(10):5077-84, 2006. PMID:16707430
96. Liu TX, Becker MW, Jelinek J, Wu WS, Deng M, Mikhalkevich N, Hsu K, Bloomfield CD, Stone RM, DeAngelo DJ, Galinsky IA, **Issa JP**, Clarke MF, Look AT. Chromosome 5q deletion and epigenetic suppression of the gene encoding alpha-catenin (CTNNA1) in myeloid cell transformation. Nat Med 13(1):78-83, 1/2007. PMID:17159988
97. Kim J, Kim JY, Song KS, Lee YH, Seo JS, Jelinek J, Goldschmidt-Clermont PJ, **Issa JP**. Epigenetic changes in estrogen receptor beta gene in atherosclerotic cardiovascular tissues and in-vitro vascular senescence. Biochim Biophys Acta 1772:72-80, 1/2007. PMID:17110088
98. Shen L, Guo Y, Chen X, Ahmed S, **Issa JP**. Optimizing annealing temperature overcomes bias in bisulfite PCR methylation analysis. Biotechniques 2007 Jan;42(1):48, 50, 52
99. Kantarjian HM, O'Brien S, Shan J, Aribi A, Garcia-Manero G, Jabbour E, Ravandi F, Cortes J, Davisson J, **Issa JP**. Update of the decitabine experience in higher risk myelodysplastic syndrome and analysis of prognostic factors associated with outcome. Cancer 109(2):265-73, 1/2007. PMID:17133405
100. Kantarjian HM, O'Brien S, Huang X, Garcia-Manero G, Ravandi F, Cortes J, Shan J, Davisson J, Bueso-Ramos CE, **Issa JP**. Survival advantage with decitabine versus intensive chemotherapy in patients with higher risk myelodysplastic syndrome: comparison with historical experience. Cancer 109(6):1133-7, 2/2007. PMID:17315156
101. Oki Y, Kantarjian HM, Gharibyan V, Jones D, O'Brien S, Verstovsek S, Cortes J, Morris GM, Garcia-Manero G, **Issa JP**. Phase II study of low-dose decitabine in combination with imatinib mesylate in patients with accelerated or myeloid blastic phase of chronic myelogenous leukemia. Cancer 109:899-906, 3/2007. PMID17236224
102. Boumber YA, Kondo Y, Chen X, Shen L, Gharibyan V, Konishi K, Estey E, Kantarjian H, Garcia-Manero G, **Issa JP**. RIL, a LIM Gene on 5q31, Is Silenced by Methylation in Cancer and Sensitizes Cancer Cells to Apoptosis. Cancer Res 67(5):1997-2005, 3/2007. PMID:17332327
103. Huang,X., Biswas,S., Oki,Y., **Issa JP**., and Berry,D.A. A parallel phase I/II clinical trial design for combination therapies. Biometrics 63:429-436, 2007. PMID:17688495
104. Kwabi-Addo,B., Chung,W., Shen,L., Ittmann,M., Wheeler,T., Jelinek,J., and **Issa JP**. Age-related DNA methylation changes in normal human prostate tissues. Clin.Cancer Res 13:3796-3802, 2007. PMID:17606710
105. Kondo,Y., Shen,L., Suzuki,S., Kurokawa,T., Masuko,K., Tanaka,Y., Kato,H., Mizuno,Y., Yokoe,M., Sugauchi,F., Hirashima,N., Orito,E., Osada,H., Ueda,R., Guo,Y., Chen,X., **Issa JP**, and Sekido,Y. Alterations of DNA methylation and histone modifications contribute to gene silencing in hepatocellular carcinomas. Hepatol.Res 37:974-983, 2007. PMID:17538160
106. Shen L, Catalano PJ, Benson AB 3rd, O'Dwyer P, Hamilton SR, **Issa JP**. Association between DNA Methylation and Shortened Survival in Patients with Advanced Colorectal Cancer Treated with 5-Fluorouracil Based Chemotherapy. Clin Cancer Res 13(20):6093-8, 2007. PMID:17947473
107. Feng W, Shen L, Wen S, Rosen DG, Jelinek J, Hu X, Huang S, Huang M, Liu J, Sahin AA, Hunt KK, Bast RC Jr, Shen Y, **Issa JP**, Yu Y. Correlation between CpG methylation profiles and

hormone receptor status in breast cancers. Breast Cancer Res 9(4):R57, 2007. PMID:17764565
108. Wang J, Bhutani M, Pathak AK, Lang W, Ren H, Jelinek J, He R, Shen L, **Issa JP**, Mao L. Delta DNMT3B variants regulate DNA methylation in a promoter-specific manner. Cancer Res 67(22):10647-52, 2007. PMID:18006804
109. Shen L, Kondo Y, Ahmed S, Boumber Y, Konishi K, Guo Y, Chen X, Vilaythong JN, **Issa JP**. Drug sensitivity prediction by CpG island methylation profile in the NCI-60 cancer cell line panel. Cancer Res 67(23):11335-43, 2007. PMID:18056460
110. Qin T, Youssef EM, Jelinek J, Chen R, Yang AS, Garcia-Manero G, **Issa JP**. Effect of cytarabine and decitabine in combination in human leukemic cell lines. Clin Cancer Res 13(14):4225-32, 2007. PMID:17634552
111. Yanada M, Huang X, Garcia-Manero G, O'brien S, Ravandi F, Borthakur G, Faderl S, **Issa JP**, Kantarjian H, Estey E. Effect of haematological improvement on survival in patients given targeted therapy as initial treatment of acute myeloid leukaemia or high-risk myelodysplastic syndrome. Br J Haematol 138(4):555-7, 2007. PMID:17593249
112. Temam,S., Kawaguchi,H., El Naggar,A.K., Jelinek,J., Tang,H., Liu,D.D., Lang,W., **Issa JP**, Lee,J.J., and Mao,L. Epidermal growth factor receptor copy number alterations correlate with poor clinical outcome in patients with head and neck squamous cancer. J.Clin.Oncol 25:2164-2170, 2007. PMID:17538160
113. Lee S, Jeong J, Majewski T, Scherer SE, Kim MS, Tuziak T, Tang KS, Baggerly K, Grossman HB, Zhou JH, Shen L, Bondaruk J, Ahmed SS, Samanta S, Spiess P, Wu X, Filipek S, McConkey D, Bar-Eli M, **Issa JP**, Benedict WF, Czerniak B. Forerunner genes contiguous to RB1 contribute to the development of in situ neoplasia. Proc Natl Acad Sci U S104(34):13732-7, 2007. PMID:17702869
114. Shen L, Kondo Y, Guo Y, Zhang J, Zhang L, Ahmed S, Shu J, Chen X, Waterland RA, **Issa JP**. Genome-wide profiling of DNA methylation reveals a class of normally methylated CpG islands promoters. PLoS Genet 3(10):e181, 2007. PMID:17967063
115. Estecio MR, Yan PS, Ibrahim AE, Tellez CS, Shen L, Huang TH, **Issa JP**. High-throughput methylation profiling by MCA coupled to CpG island microarray. Genome Res 2007 Oct;17(10):1529-36. Epub 2007 Sep 4. PMID:17785535
116. Choi SH, Byun HM, Kwan JM, **Issa JP**, Yang AS. Hydroxycarbamide in combination with azacitidine or decitabine is antagonistic on DNA methylation inhibition. Br J Haematol 138(5):616-23, 2007. PMID:17686055
117. Choi IS, Estecio MR, Nagano Y, Kim DH, White JA, Yao JC, **Issa JP**, Rashid A. Hypomethylation of LINE-1 and Alu in well-differentiated neuroendocrine tumors (pancreatic endocrine tumors and carcinoid tumors). Mod Pathol 20(7):802-10, 2007. PMID:17483816
118. Oki Y, Jelinek J, Shen L, Kantarjian HM, **Issa JP**. Induction of hypomethylation and molecular response after decitabine therapy in patients with chronic myelomonocytic leukemia. Blood 2008 Feb 15;111(4):2382-4. Epub 2007 Nov 30. PMID:18055864
119. Shen L, Toyota M, Kondo Y, Lin E, Zhang L, Guo Y, Hernandez NS, Chen X, Ahmed S, Konishi K, Hamilton SR, **Issa JP**. Integrated genetic and epigenetic analysis identifies three different subclasses of colon cancer. Proc Natl Acad Sci U S A 104(47):18654-9, 2007. PMID:18003927
120. Estecio MR, Gharibyan V, Shen L, Ibrahim AE, Doshi K, He R, Jelinek J, Yang AS, Yan PS, Huang TH, Tajara EH, **Issa JP**. LINE-1 Hypomethylation in Cancer Is Highly Variable and Inversely Correlated with Microsatellite Instability. PLoS ONE 2:e399, 2007. PMID:17476321
121. Oki,Y., Jelinek,J., Beran,M., Verstovsek,S., Kantarjian,H.M., **Issa JP**. Mutations and promoter methylation status of NPM1 in myeloproliferative disorders. Haematologica 91:1147-1148, 2007. PMID:16870548

122. Tabe Y, Konopleva M, Kondo Y, Contractor R, Tsao T, Konoplev S, Shi Y, Ling X, Watt JC, Tsutsumi-Ishii Y, Ohsaka A, Nagaoka I, **Issa JP**, Kogan SC, Andreeff M. PPARgamma-Active Triterpenoid CDDO Enhances ATRA-Induced Differentiation in APL. Cancer Biol Ther 6(12):Sept 4 [epub ahead of print], 2007. PMID:18075297
123. Konishi K, Shen L, Wang S, Meltzer SJ, Harpaz N, **Issa JP**. Rare CpG Island Methylator Phenotype in Ulcerative Colitis-Associated Neoplasias. Gastroenterology 132(4):1254-60, 2007. PMID:17408633
124. Kantarjian H, Oki Y, Garcia-Manero G, Huang X, O'Brien S, Cortes J, Faderl S, Bueso-Ramos C, Ravandi F, Estrov Z, Ferrajoli A, Wierda W, Shan J, Davi J, Giles F, Saba H, **Issa JP**. Results of a Randomized Study of Three Schedules of Low-Dose Decitabine in Higher Risk Myelodysplastic Syndrome and Chronic Myelomonocytic Leukemia. Blood 109:52-57, 2007. PMID:16882708
125. Jelinek,J., **Issa JP**, He,R., Cmejla,R., Cmejlova,J., and Pospisilova,D. RPS19 and JAK2 genes are not silenced by DNA methylation in diamond blackfan anemia. J.Pediatr.Hematol.Oncol 29:207-208, 2007. PMID:17356406
126. Soriano AO, Yang H, Faderl S, Estrov Z, Giles F, Ravandi F, Cortes J, Wierda WG, Ouzounian S, Quezada A, Pierce S, Estey EH, **Issa JP**, Kantarjian HM, Garcia-Manero G. Safety and clinical activity of the combination of 5-azacytidine, valproic acid and all-trans retinoic acid in acute myeloid leukemia and myelodysplastic syndrome. Blood 110:2302-2308, 2007. PMID:17596541
127. Gao W, Kondo Y, Shen L, Shimizu Y, Sano T, Yamao K, Natsume A, Goto Y, Ito M, Murakami H, Osada H, Zhang J, **Issa JP**, Sekido Y. Variable DNA methylation patterns associated with progression of disease in hepatocellular carcinomas. Carcinogenesis 29(10). e-Pub 7/16/2008. PMID:18632756
128. Qin T, Jelinek J, Si J, Shu J, **Issa JP**. Mechanisms of resistance to 5-aza-2'-deoxycytidine in human cancer cell lines. Blood. 2009 Jan 15;113(3):659-67. Epub 2008 Oct 17. PMID: 18931345 PMID:18931345
129. Kroeger H, Jelinek J, Estecio MR, He R, Kondo K, Chung W, Zhang L, Shen L, Kantarjian HM, Bueso-Ramos CE, **Issa JP**. Aberrant CpG island methylation in acute myeloid leukemia is accentuated at relapse. Blood, 2008 Aug 15;112(4):1366-73. Epub 2008 Jun 3. PMID::18523155
130. Kantarjian H, O'Brien S, Ravandi F, Cortes J, Shan J, Bennett JM, List A, Fenaux P, Sanz G, **Issa JP**, Freireich EJ, Garcia-Manero GJ. Proposal for a new risk model in myelodysplastic syndrome that accounts for events not considered in the original international prognostic scoring system. Cancer, 2008 Sep 15; 113 (6): 1351-61. PMID:18618511
131. Kuang SQ, Tong WG, Yang H, Lin W, Lee MK, Fang ZH, Wei Y, Jelinek J, **Issa JP**, Garcia-Manero G. Genome-wide identification of aberrantly methylated promoter associated CpG islands in acute lymphocytic leukemia. Leukemia 8:1529-38, 8/2008. e-Pub 6/2008. PMID:18528427
132. Zuo X, Shen L,**Issa JP**, Moy O, Morris JS, Lippman SM, Shureiqi I. 15-Lipoxygenase-1 transcriptional silencing by DNA methyltransferase-1 independently of DNA methylation. FASEB J 2008 Jun;22(6) :1981-92. Epub 2008 Jan 15. PMID:18198215
133. Borthakur,G., Ahdab,S.E., Ravandi,F., Faderl,S., Ferrajoli,A., Newman,B., **Issa JP** and Kantarjian,H. Activity of decitabine in patients with myelodysplastic syndrome previously treated with azacitidine. Leuk.Lymphoma 49:690-695, 2008. PMID:18398735
134. Boumber YA, Kondo Y, Chen X, Shen L, Guo Y, Tellez C, Estécio MR, Ahmed S, **Issa JP**. An Sp1/Sp3 binding polymorphism confers methylation protection. PLoS Genet 4(8):e1000162, 2008. PMID:18725933

135. Kondo Y, Shen L, Ahmed S, Boumber Y, Sekido Y, Haddad BR, **Issa JP**. Downregulation of histone H3 lysine 9 methyltransferase G9a induces centrosome disruption and chromosome instability in cancer cells. PLoS ONE 3(4):e2037, 2008. PMID:18446223
136. Jelinek, J., Li,J., Mnjoyan, Z., **Issa JP**, Prchal, J.T., and Afshar-Kharghan, V. Epigenetic control of PRV-1 expression on neutrophils. Exp.Hematol 35:1677-1683, 2008. PMID:17976520
137. Suehiro,Y., Wong,C.W., Chirieac,L.R., Kondo,Y., Shen,L., Webb,C.R., Chan,Y.W., Chan,A.S., Chan,T.L., Wu,T.T., Rashid,A., Hamanaka,Y., Hinoda,Y., Shannon,R.L., Wang,X., Morris,J., **Issa JP**, Yuen,S.T., Leung,S.Y., and Hamilton,S.R. Epigenetic-Genetic Interactions in the APC/WNT, RAS/RAF, and P53 Pathways in Colorectal Carcinoma. Clin.Cancer Res. 14:2560-2569, 2008. PMID:18451217
138. Kondo Y, Shen L, Cheng A, Ahmed S, Boumber Y, Charo C, Yamochi T, Urano T, Furukawa K, Kwabi-Addo B, Gold D, Sekido Y, Huang T, **Issa JP**. Gene silencing in cancer by histone H3 lysine 27 tri-methylation independent of promoter DNA methylation. Nature Genetics 40(6):741-50, 2008. PMID:18488029
139. Chung,W., Kwabi-Addo,B., Ittmann,M., Jelinek,J., Shen,L., Yu,Y., **Issa JP**. Identification of novel tumor markers in prostate, colon and breast cancer by unbiased methylation profiling. PLoS.ONE 3:e2079, 2008. PMID:18446232
140. Feng,W., Marquez,R.T., Lu,Z., Liu,J., Lu,K.H., **Issa JP**, Fishman,D.M., Yu,Y., and Bast,R.C., Jr. Imprinted tumor suppressor genes ARHI and PEG3 are the most frequently down-regulated in human ovarian cancers by loss of heterozygosity and promoter methylation. Cancer 112:1489-1502, 2008. PMID:18286529
141. Lee,E.S., **Issa JP**, Roberts,D.B., Williams,M.D., Weber,R.S., Kies,M.S., and El Naggar,A.K. Quantitative Promoter Hypermethylation Analysis of Cancer-Related Genes in Salivary Gland Carcinomas: Comparison with Methylation-Specific PCR Technique and Clinical Significance. Clin.Cancer Res., 14:2664-2672, 2008. PMID:18451230
142. Majewski,T., Lee,S., Jeong,J., Yoon,D.S., Kram,A., Kim,M.S., Tuziak,T., Bondaruk,J., Lee,S., Park,W.S., Tang,K.S., Chung,W., Shen,L., Ahmed,S.S., Johnston,D.A., Grossman,H.B., Dinney,C.P., Zhou,J.H., Harris,R.A., Snyder,C., Filipek,S., Narod,S.A., Watson,P., Lynch,H.T., Gazdar,A., Bar-Eli,M., Wu,X.F., McConkey,D.J., Baggerly,K., **Issa JP**, Benedict,W.F., Scherer,S.E., and Czerniak,B. Understanding the development of human bladder cancer by using a whole-organ genomic mapping strategy. Lab Invest, 2008. Jul;88(7):694-721. Epub 2008 May 5. PMID:18458673
143. De Padua Silva L, de Lima M, Kantarjian H, Faderl S, Kebriaei P, Giralt S, Davisson J, Garcia-Manero G, Champlin R, **Issa JP**, Ravandi F. Feasibility of allo-SCT after hypomethylating therapy with decitabine for myelodysplastic syndrome. Bone Marrow Transplant. e-Pub 1/2009. PMID:19151791
144. Kim MJ, White-Cross JA, Shen L, **Issa JP**, Rashid A. Hypomethylation of long interspersed nuclear element-1 in hepatocellular carcinomas. Mod Pathol 22(3). e-Pub 1/2009. PMID:19136926
145. Huang X, Ning J, Li Y, Estey E, **Issa JP**, Berry DA. Using short-term response information to facilitate adaptive randomization for survival clinical trials. Stat Med, 2009 May 30;28(12):1680-9. PMID:19326367
146. Heinrichs S, Kulkarni RV, Bueso-Ramos CE, Levine RL, Loh ML, Li C, Neuberg D, Kornblau SM,**Issa JP**, Gilliland DG, Garcia-Manero G, Kantarjian HM, Estey EH, Look AT. Accurate detection of uniparental disomy and microdeletions by SNP array analysis in myelodysplastic syndromes with normal cytogenetics. Leukemia. e-Pub 4/2009. PMID:19387468
147. Figueiredo JC, Grau MV, Wallace K, Levine AJ, Shen L, Hamdan R, Chen X, Bresalier RS, McKeown-Eyssen G, Haile RW, Baron JA,**Issa JP**. Global DNA Hypomethylation (LINE-1) in

the Normal Colon and Lifestyle Characteristics and Dietary and Genetic Factors. Cancer Epidemiol Biomarkers Prev 18(4):1041-9, 4/2009. e-Pub 3/2009. PMID:19336559

148. Ware CB, Wang L, Mecham BH, Shen L, Nelson AM, Bar M, Lamba DA, Dauphin DS, Buckingham B, Askari B, Lim R, Tewari M, Gartler SM, **Issa JP**, Pavlidis P, Duan Z, Blau CA. Histone deacetylase inhibition elicits an evolutionarily conserved self-renewal program in embryonic stem cells. Cell Stem Cell 4(4):359-69, 4/2009. PMID:19341625
149. Aparicio A, North B, Barske L, Wang X, Bollati V, Weisenberger D, Yoo C, Tannir N, Horne E, Groshen S, Jones P, Yang A,**Issa JP**. LINE-1 methylation in plasma DNA as a biomarker of activity of DNA methylation inhibitors in patients with solid tumors. Epigenetics 4(3). e-Pub 4/2009. PMID:19421002
150. Watanabe Y, Kim HS, Castoro RJ, Chung W, Estecio MR, Kondo K, Guo Y, Ahmed SS, Toyota M, Itoh F, Suk KT, Cho MY, Shen L, Jelinek J,**Issa JP**. Sensitive and Specific Detection of Early Gastric Cancer with DNA Methylation Analysis of Gastric Washes. Gastroenterology. 2009 Jun;136(7):2149-58. Epub 2009 Apr 16. PMID:19375421
151. Tellez CS, Shen L, Estécio MR, Jelinek J, Gershenwald JE, **Issa JP**. CpG island methylation profiling in human melanoma cell lines. Melanoma Res 19(3):146-55, 6/2009. PMID:19441164
152. Stewart DJ, **Issa JP**, Kurzrock R, Nunez MI, Jelinek J, Hong D, Oki Y, Guo Z, Gupta S, Wistuba II. Decitabine effect on tumor global DNA methylation and other parameters in a phase I trial in refractory solid tumors and lymphomas. Clin Cancer Res 15(11):3881-8, 6/2009. e-Pub 5/2009. PMID:19407036
153. Konishi K, Shen L, Jelinek J, Watanabe Y, Ahmed S, kaneko K, Kogo M, Takano T, Imawari M, Hamilton SR, **Issa JP**, Concordant DNA methylation in synchronous colorectal carcinomas. Cancer Prev Res (Phila Pa) 2 (9):814-22, 9/2009, e-Pub 9/8/2009. PMID:19737982
154. Goto Y, Shinjo K, Kondo Y, Shen L, Toyota M, Suzuki H, Gao W, An B, Fujii M, Murakami H, Osada H, Taniguchi T, Usami N, Kondo M, Hasegawa Y, Shimokata K, Matsuo K, Hida T, Fujimoto N, Kishimoto T, **Issa JP**, Sekido Y. Epigenetic profiles distinguish malignant pleural mesothelioma from lung adenocarcinoma. Cancer Res. 2009 Dec 1;69 (23):9073-82. Epub 2009 Nov 3. PMID:19887624
155. Kantarjian H, O'Brien S, Ravandi F, Borthakur G, Faderl S, Bueso-Ramos C, Abruzzo L,Pierce S, Shan J, **Issa JP**, Garcia-Manero G. The heterogeneous prognosis of patients with myelodysplastic syndrome and chromosome 5 abnormalities: how does it relate to the original lenalidomide experience in MDS? Cancer 2009 Nov 15;115(22):5202-9. PMID: 19691096
156. Shen L, Kantarjian H, Guo Y, Lin E, Shan J, Huang X, Berry D, Ahmed S, Zhu W, Pierce S, Kondo Y, Oki Y, Jelinek J, Saba H, Estey E, **Issa JP**. DNA Methylation Predicts Survival and Response to Therapy in Patients With Myelodysplastic Syndromes. J Clin Oncol. 2010 Feb 1;28(4):605-13. Epub 2009 Dec 28.
157. Ravandi F, **Issa JP**, Garcia-Manero G, O'Brien S, Pierce S, Shan J, Borthakur G, Verstovsek S, Faderl S, Cortes J, Kantarjian H. Superior outcome with hypomethylating therapy in patients with acute myeloid leukemia and high-risk myelodysplastic syndrome and chromosome 5 and 7 abnormalities. Cancer. 2009 Dec 15;115(24):5746-51, PMID: 19795507 [PubMed - in process]
158. Borthakur G, Huang X, Kantarjian H, Faderl S, Ravandi F, Ferrajoli A, Torma R, Morris G, Berry D, **Issa JP**. Report of a phase 1/2 study of a combination of azacitidine and cytarabine in acute myelogenous leukemia and high-risk myelodysplastic syndromes. Leuk Lymphoma. 2010 Jan;51(1):73-8 PMID: 20017599 [PubMed - in process]
159. Cleyrat C, Jelinek J, Girodon F, Boissinot M, Ponge T, Harousseau JL, **Issa JP**, Hermouet S. JAK2 mutation and disease phenotype: a double L611V/V617F in cis mutation of JAK2 is

associated with isolated erythrocytosis and increased activation of AKT and ERK1/2 rather than STAT5. Leukemia. 2010 Feb 25. [Epub ahead of print] PubMed PMID: 20182460.

160. An B, Kondo Y, Okamoto Y, Shinjo K, Kanemitsu Y, Komori K, Hirai T, Sawaki A, Tajika M, Nakamura T, Yamao K, Yatabe Y, Fujii M, Murakami H, Osada H, Tani T,Matsuo K, Shen L, **Issa JP**, Sekido Y. A characteristic methylation profile in CpG island methylator phenotype-negative distal colorectal cancers. Int J Cancer.2010 Feb 3. [Epub ahead of print] PubMed PMID: 20131317.
161. Maegawa S, Hinkal G, Kim HS, Shen L, Zhang L, Zhang J, Zhang N, Liang S, Donehower LA, **Issa JP**. Widespread and tissue specific age-related DNA methylation changes in mice. Genome Res. 2010 Mar;20(3):332-40. Epub 2010 Jan 27. PubMed PMID: 20107151.
162. Suzuki H, Igarashi S, Nojima M, Maruyama R, Yamamoto E, Kai M, Akashi H, Watanabe Y, Yamamoto H, Sasaki Y, Itoh F, Imai K, Sugai T, Shen L, **Issa JP**, Shinomura Y, Tokino T, Toyota M. IGFBP7 is a p53-responsive gene specifically silenced in colorectal cancer with CpG island methylator phenotype. Carcinogenesis. 2010 Mar;31(3):342-9. Epub 2009 Jul 28. PubMed PMID: 19638426.
163. Batty GN, Kantarjian H, **Issa JP**, Jabbour E, Santos FP, McCue D, Garcia-Manero G, Pierce S, O'Brien S, Cortés JE, Ravandi F. Feasibility of therapy with hypomethylating agents in patients with renal insufficiency. Clin Lymphoma Myeloma Leuk. 2010 Jun;10(3):205-10. PubMed PMID: 20511166.
164. Jabbour E, Garcia-Manero G, Batty N, Shan J, O'Brien S, Cortes J, Ravandi F, **Issa JP**, Kantarjian H. Outcome of patients with myelodysplastic syndrome after failure of decitabine therapy. Cancer. 2010 Aug 15;116(16):3830-4. PubMed PMID: 20564137.
165. Kwabi-Addo B, Wang S, Chung W, Jelinek J, Patierno SR, Wang BD, Andrawis R, Lee NH, Apprey V, **Issa JP**, Ittmann M. Identification of differentially methylated genes in normal prostate tissues from African American and Caucasian men. Clin Cancer Res. 2010 Jul 15;16(14):3539-47. Epub 2010 Jul 6. PubMed PMID: 20606036.
166. Si J, Boumber YA, Shu J, Qin T, Ahmed S, He R, Jelinek J, **Issa JP**. Chromatin remodeling is required for gene reactivation after decitabine-mediated DNA hypomethylation. Cancer Res. 2010 Sep 1;70(17):6968-77. Epub 2010 Aug 16. PubMed PMID: 20713525; PubMed Central PMCID: PMC2932851.
167. Estécio MR, Gallegos J, Vallot C, Castoro RJ, Chung W, Maegawa S, Oki Y, Kondo Y, Jelinek J, Shen L, Hartung H, Aplan PD, Czerniak BA, Liang S, **Issa JP**. Genome architecture marked by retrotransposons modulates predisposition to DNA methylation in cancer. Genome Res. 2010 Oct;20(10):1369-82. Epub 2010 Aug 17. PubMed PMID: 20716667; PubMed Central PMCID: PMC2945186.
168. Wallace K, Grau MV, Levine AJ, Shen L, Hamdan R, Chen X, Gui J, Haile RW, Barry EL, Ahnen D, McKeown-Eyssen G, Baron JA, **Issa JP**. Association between folate levels and CpG Island hypermethylation in normal colorectal mucosa. Cancer Prev Res (Phila). 2010 Dec;3(12):1552-64. PubMed PMID: 21149331; PubMed Central PMCID: PMC3058541.
169. Spannhoff A, Kim YK, Raynal NJ, Gharibyan V, Su MB, Zhou YY, Li J, Castellano S, Sbardella G, **Issa JP**, Bedford MT. Histone deacetylase inhibitor activity in royal jelly might facilitate caste switching in bees. EMBO Rep. 2011 Mar;12(3):238-43. Epub 2011 Feb 18. PubMed PMID: 21331099; PubMed Central PMCID: PMC3059907.
170. Fini L, Piazzi G, Daoud Y, Selgrad M, Maegawa S, Garcia M, Fogliano V, Romano M, Graziani G, Vitaglione P, Carmack SW, Gasbarrini A, Genta RM, **Issa JP**, Boland CR, Ricciardiello L. Chemoprevention of intestinal polyps in ApcMin/+ mice fed with western or balanced diets by drinking annurca apple polyphenol extract. Cancer Prev Res (Phila). 2011 Jun;4(6):907-15. Epub 2011 Mar 7. PubMed PMID: 21383028.
171. Cogdell D, Chung W, Liu Y, McDonald JM, Aldape K, **Issa JP**, Fuller GN, Zhang W. Tumor-associated methylation of the putative tumor suppressor AJAP1 gene and association

between decreased AJAP1 expression and shorter survival in patients with glioma. Chin J Cancer. 2011 Apr;30(4):247-53. PubMed PMID: 21439246.
172. Pathiraja TN, Shetty PB, Jelinek J, He R, Hartmaier R, Margossian AL, Hilsenbeck SG, **Issa JP**, Oesterreich S. Progesterone receptor isoform-specific promoter methylation: association of PRA promoter methylation with worse outcome in breast cancer patients. Clin Cancer Res. 2011 Jun 15;17(12):4177-86. Epub 2011 Apr 1. PubMed PMID: 21459801.
173. Ahn JB, Chung WB, Maeda O, Shin SJ, Kim HS, Chung HC, Kim NK, **Issa JP**. DNA methylation predicts recurrence from resected stage III proximal colon cancer. Cancer. 2011 May 1;117(9):1847-54. doi: 10.1002/cncr.25737. Epub 2010 Nov 18. PubMed PMID: 21509761.
174. Chung W, Bondaruk J, Jelinek J, Lotan Y, Liang S, Czerniak B, **Issa JP**. Detection of bladder cancer using novel DNA methylation biomarkers in urine sediments. Cancer Epidemiol Biomarkers Prev. 2011 Jul;20(7):1483-91. Epub 2011 May 17. PubMed PMID: 21586619; PubMed Central PMCID: PMC3132294.
175. Jelinek J, Gharibyan V, Estecio MR, Kondo K, He R, Chung W, Lu Y, Zhang N, Liang S, Kantarjian HM, Cortes JE, **Issa JP**. Aberrant DNA methylation is associated with disease progression, resistance to imatinib and shortened survival in chronic myelogenous leukemia. PLoS One. 2011;6(7):e22110. Epub 2011 Jul 8. PubMed PMID: 21760961; PubMed Central PMCID: PMC3132778.
176. Watanabe Y, Castoro RJ, Kim HS, North B, Oikawa R, Hiraishi T, Ahmed SS, Chung W, Cho MY, Toyota M, Itoh F, Estecio MR, Shen L, Jelinek J, **Issa JP**. Frequent Alteration of MLL3 Frameshift Mutations in Microsatellite Deficient Colorectal Cancer. PLoS One. 2011;6(8):e23320. Epub 2011 Aug 11. PubMed PMID: 21853109; PubMed Central PMCID: PMC3154922.
177. Qin T, Castoro R, El Ahdab S, Jelinek J, Wang X, Si J, Shu J, He R, Zhang N, Chung W, Kantarjian HM, **Issa JP**. Mechanisms of resistance to decitabine in the myelodysplastic syndrome. PLoS One. 2011;6(8):e23372. Epub 2011 Aug 17. PubMed PMID: 21858090; PubMed Central PMCID: PMC3157379.
178. Konishi K, Watanabe Y, Shen L, Guo Y, Castoro RJ, Kondo K, Chung W, Ahmed S, Jelinek J, Boumber YA, Estecio MR, Maegawa S, Kondo Y, Itoh F, Imawari M, Hamilton SR, **Issa JP**. DNA methylation profiles of primary colorectal carcinoma and matched liver metastasis. PLoS One. 2011;6(11):e27889. Epub 2011 Nov 21. PubMed PMID: 22132162; PubMed Central PMCID: PMC3221680.
179. Challen GA, Sun D, Jeong M, Luo M, Jelinek J, Berg JS, Bock C, Vasanthakumar A, Gu H, Xi Y, Liang S, Lu Y, Darlington GJ, Meissner A, **Issa JP**, Godley LA, Li W, Goodell MA. Dnmt3a is essential for hematopoietic stem cell differentiation. Nat Genet. 2011 Dec 4;44(1):23-31. doi: 10.1038/ng.1009. PubMed PMID: 22138693.
180. Raynal NJ, Si J, Taby RF, Gharibyan V, Ahmed S, Jelinek J, Estécio MR, **Issa JP**. DNA methylation does not stably lock gene expression but instead serves as a molecular mark for gene silencing memory. Cancer Res. 2012 Mar 1;72(5):1170-81. Epub 2012 Jan 4. PubMed PMID: 22219169; PubMed Central PMCID: PMC3294136.
181. Bhardwaj A, Song HW, Beildeck M, Kerkhofs S, Castoro R, Shanker S, De Gendt K, Suzuki K, Claessens F, **Issa JP**, Orgebin-Crist MC, Wilkinson MF. DNA demethylation-dependent AR recruitment and GATA factors drive Rhox5 homeobox gene transcription in the epididymis. Mol Endocrinol. 2012 Apr;26(4):538-49. Epub 2012 Feb 9. PubMed PMID: 22322598; PubMed Central PMCID: PMC3327359.
182. Yamazaki J, Taby R, Vasanthakumar A, Macrae T, Ostler KR, Shen L, Kantarjian HM, Estecio MR, Jelinek J, Godley LA, **Issa JP**. Effects of TET2 mutations on DNA methylation in chronic myelomonocytic leukemia. Epigenetics. 2012 Feb;7(2):201-7. PubMed PMID: 22395470; PubMed Central PMCID: PMC3335912.

183. Tsai HC, Li H, Van Neste L, Cai Y, Robert C, Rassool FV, Shin JJ, Harbom KM, Beaty R, Pappou E, Harris J, Yen RW, Ahuja N, Brock MV, Stearns V, Feller-Kopman D, Yarmus LB, Lin YC, Welm AL, **Issa JP**, Minn I, Matsui W, Jang YY, Sharkis SJ, Baylin SB, Zahnow CA. Transient low doses of DNA-demethylating agents exert durable antitumor effects on hematological and epithelial tumor cells. Cancer Cell. 2012 Mar 20;21(3):430-46. PubMed PMID: 22439938; PubMed Central PMCID: PMC3312044.
184. Zhang Y, Shu J, Si J, Shen L, Estecio MR, **Issa JP**. Repetitive elements and enforced transcriptional repression co-operate to enhance DNA methylation spreading into a promoter CpG-island. Nucleic Acids Res. 2012 May 17. [Epub ahead of print] PubMed PMID: 22600741.
185. Boumber Y, Kantarjian H, Jorgensen J, Wen S, Faderl S, Castoro R, Autry J, Garcia-Manero G, Borthakur G, Jabbour E, Estrov Z, Cortes J, **Issa JP**, Ravandi F. A randomized study of decitabine versus conventional care for maintenance therapy in patients with acute myeloid leukemia in complete remission. Leukemia. 2012 Jun 5. doi: 10.1038/leu.2012.153. [Epub ahead of print] PubMed PMID: 22665218.
186. Xu J, Shetty PB, Feng W, Chenault C, Bast RC Jr, **Issa JP**, Hilsenbeck SG, Yu Y. Methylation of HIN-1, RASSF1A, RIL and CDH13 in breast cancer is associated with clinical characteristics, but only RASSF1A methylation is associated with outcome. BMC Cancer. 2012 Jun 13;12(1):243. [Epub ahead of print] PubMed PMID: 22695491.
187. Ulrich CM, Toriola AT, Koepl LM, Sandifer T, Poole EM, Duggan C, McTiernan A, **Issa JP**. Metabolic, hormonal and immunological associations with global DNA methylation among postmenopausal women. Epigenetics. 2012 Sep 1;7(9). [Epub ahead of print] PubMed PMID: 22869041.
188. Estecio MR, Gallegos J, Dekmezian M, Lu Y, Liang S, **Issa JP**J. SINE Retrotransposons Cause Epigenetic Reprogramming of Adjacent Gene Promoters. Molecular Cancer Research, 2012 Sep 20. [Epub ahead of print] PubMed PMID: 22952045.
189. Jelinek J, Liang S, Lu Y, He R, Ramagli LS, Shpall EJ, Estecio MR, **Issa JP**. Conserved DNA methylation patterns in healthy blood cells and extensive changes in leukemia measured by a new quantitative technique. Epigenetics. 2012 Oct 17;7(12). [Epub ahead of print] PubMed PMID: 23075513.
190. Yamazaki J, Estecio MR, Lu Y, Long H, Malouf GG, Graber D, Huo Y, Ramagli L, Liang S, Kornblau SM, Jelinek J, **Issa JP**. The epigenome of AML stem and progenitor cells. Epigenetics. 2012 Dec 18;8(1). [Epub ahead of print] PubMed PMID: 23249680.
191. Pickering CR, Zhang J, Yoo SY, Bengtsson L, Moorthy S, Neskey DM, Zhao M, Ortega Alves MV, Chang K, Drummond J, Cortez E, Xie TX, Zhang D, Chung W, **Issa JP**, Zweidler-McKay PA, Wu X, El-Naggar AK, Weinstein JN, Wang J, Muzny DM, Gibbs RA, Wheeler DA, Myers JN, Frederick MJ. Integrative genomic characterization of oral squamous cell carcinoma identifies frequent somatic drivers. Cancer Discov. 2013 Apr 25. [Epub ahead of print] PubMed PMID: 23619168.
192. Cancer Genome Atlas Research Network. Genomic and epigenomic landscapes of adult de novo acute myeloid leukemia. N Engl J Med. 2013 May 30;368(22):2059-74. doi: 10.1056/NEJMoa1301689. Epub 2013 May 1. Erratum in: N Engl J Med. 2013 Jul 4;369(1):98. PubMed PMID: 23634996.
193. Natsume A, Ito M, Katsushima K, Ohka F, Hatanaka A, Shinjo K, Sato S, Takahashi S, Ishikawa Y, Takeuchi I, Shimogawa H, Uesugi M, Okano H, Kim SU, Wakabayashi T, **Issa JP**, Sekido Y, Kondo Y. Chromatin Regulator PRC2 Is a Key Regulator of Epigenetic Plasticity in Glioblastoma. Cancer Res. 2013 Jul 15;73(14):4559-4570. Epub 2013 May 29. PubMed PMID: 23720055.
194. Lanikova L, Kucerova J, Indrak K, Divoka M, **Issa JP**, Papayannopoulou T, Prchal JT, Divoky V. β-Thalassemia Due to Intronic LINE-1 Insertion in the β-Globin Gene (HBB): Molecular

Mechanisms Underlying Reduced Transcript Levels of the β-Globin(L1) Allele. Hum Mutat. 2013 Jul 22. doi: 10.1002/humu.22383. [Epub ahead of print] PubMed PMID: 23878091.
195. Tahara T, Maegawa S, Chung W, Garriga J, Jelinek J, Estécio MR, Shibata T, Hirata I, Arisawa T, **Issa JP**. Examination of whole blood DNA methylation as a potential risk marker for gastric cancer. Cancer Prev Res (Phila). 2013 Aug 13. [Epub ahead of print] PubMed PMID: 23943784.
196. Taube JH, Malouf GG, Lu E, Sphyris N, Vijay V, Ramachandran PP, Ueno KR, Gaur S, Nicoloso MS, Rossi S, Herschkowitz JI, Rosen JM, **Issa JP**, Calin GA, Chang JT, Mani SA. Epigenetic silencing of microRNA-203 is required for EMT and cancer stem cell properties. Sci Rep. 2013;3:2687. doi: 10.1038/srep02687. PubMed PMID:24045437; PubMed Central PMCID: PMC3776231.
197. Okamoto Y, Shinjo K, Shimizu Y, Sano T, Yamao K, Gao W, Fujii M, Osada H, Sekido Y, Murakami S, Tanaka Y, Joh T, Sato S, Takahashi S, Wakita T, Zhu J, **Issa JP**, Kondo Y. Hepatitis Virus Infection Affects DNA Methylation in Mice with Humanized Livers. Gastroenterology. 2013 Oct 30. doi:pii: S0016-5085(13)01563-1. 10.1053/j.gastro.2013.10.056. [Epub ahead of print] PubMed PMID: 24184133.
198. Tahara T, Yamamoto E, Madireddi P, Suzuki H, Maruyama R, Chung W, Garriga J, Jelinek J, Yamano HO, Sugai T, Kondo Y, Toyota M, **Issa JP**, Estécio MR. Colorectal Carcinomas with CpG Island Methylator Phenotype 1 Frequently Contain Mutations in Chromatin Regulators. Gastroenterology. 2013 Nov 6. doi:pii:S0016-5085(13)01578-3. 10.1053/j.gastro.2013.10.060. [Epub ahead of print] PubMed PMID: 24211491.
199. Malouf GG, Taube JH, Lu Y, Roysarkar T, Panjarian S, Estecio MR, Jelinek J, Yamazaki J, Raynal NJ, Long H, Tahara T, Tinnirello A, Ramachandran P, Zhang XY, Liang S, Mani SA, **Issa JP**. Architecture of epigenetic reprogramming following Twist1-mediated epithelial-mesenchymal transition. Genome Biol. 2013 Dec 24;14(12):R144. [Epub ahead of print] PubMed PMID: 24367927.
200. Tahara T, Yamamoto E, Suzuki H, Maruyama R, Chung W, Garriga J, Jelinek J, Yamano HO, Sugai T, An B, Shureiqi I, Toyota M, Kondo Y, Estécio MR, **Issa JP**. Fusobacterium in colonic flora and molecular features of colorectal carcinoma. Cancer Res. 2014 Mar 1;74(5):1311-8. doi: 10.1158/0008-5472.CAN-13-1865. Epub 2014 Jan 2. PubMed PMID: 24385213.
201. Stewart DJ, Nunez MI, Jelinek J, Hong D, Gupta S, **Issa JP**, Wistuba II, Kurzrock R. Decitabine impact on the endocytosis regulator RhoA, the folate carriers RFC1 and FOLR1, and the glucose transporter GLUT4 in human tumors. Clin Epigenetics. 2014 Jan 9;6(1):2. doi: 10.1186/1868-7083-6-2. PubMed PMID: 24401732; PubMed Central PMCID: PMC3895853.
202. Maegawa S, Gough SM, Watanabe-Okochi N, Lu Y, Zhang N, Castoro RJ, Estecio MR, Jelinek J, Liang S, Kitamura T, Aplan PD, **Issa JP**. Age-related epigenetic drift in the pathogenesis of MDS and AML. Genome Res. 2014 Apr;24(4):580-91. doi: 10.1101/gr.157529.113. Epub 2014 Jan 10. PubMed PMID: 24414704; PubMed Central PMCID: PMC3975058.
203. Pathiraja TN, Nayak SR, Xi Y, Jiang S, Garee JP, Edwards DP, Lee AV, Chen J, Shea MJ, Santen RJ, Gannon F, Kangaspeska S, Jelinek J, **Issa JP**, Richer JK, Elias A, McIlroy M, Young LS, Davidson NE, Schiff R, Li W, Oesterreich S. Epigenetic reprogramming of HOXC10 in endocrine-resistant breast cancer. Sci Transl Med. 2014 Mar 26;6(229):229ra41. doi: 10.1126/scitranslmed.3008326. PubMed PMID: 24670685.
204. Plimack ER, Desai JR, **Issa JP**, Jelinek J, Sharma P, Vence LM, Bassett RL, Ilagan JL, Papadopoulos NE, Hwu WJ. A phase I study of decitabine with pegylated interferon α-2b in advanced melanoma: impact on DNA methylation and lymphocyte populations. Invest New Drugs. 2014 May 31. [Epub ahead of print] PubMed PMID:24875133.
205. Jin C, Lu Y, Jelinek J, Liang S, Estecio MR, Barton MC, **Issa JP**. TET1 is a maintenance DNA demethylase that prevents methylation spreading in differentiated cells. Nucleic Acids Res. 2014 May 29. pii: gku372. [Epub ahead of print] PubMed PMID: 24875481.

206. Cui Y, Hausheer F, Beaty R, Zahnow C, **Issa JP**, Bunz F, Baylin SB. A Recombinant Reporter System for Monitoring Reactivation of an Endogenously DNA Hypermethylated Gene. Cancer Res. 2014 May 29. pii: canres.2287.2013. [Epub ahead of print] PubMed PMID: 24876104.
207. Kirschbaum M, Gojo I, Goldberg SL, Bredeson C, Kujawski LA, Yang A, Marks P, Frankel P, Sun X, Tosolini A, Eid JE, Lubiniecki GM, **Issa JP**. A phase 1 clinical trial of vorinostat in combination with decitabine in patients with acute myeloid leukaemia or myelodysplastic syndrome. Br J Haematol. 2014 Jul 8. doi:10.1111/bjh.13016. [Epub ahead of print] PubMed PMID: 25040094.
208. Stewart DJ, Nunez MI, Jelinek J, Hong D, Gupta S, Aldaz M, **Issa JP**, Kurzrock R, Wistuba II. Impact of decitabine on immunohistochemistry expression of the putative tumor suppressor genes FHIT, WWOX, FUS1 and PTEN in clinical tumor samples. Clin Epigenetics. 2014 Jul 3;6(1):13. doi: 10.1186/1868-7083-6-13. eCollection 2014. PubMed PMID: 25024751; PubMed Central PMCID: PMC4094901.
209. Tsimberidou AM, Said R, Culotta K, Wistuba I, Jelinek J, Fu S, Falchook G,Naing A, Piha-Paul S, Zinner R, Siddik ZH, He G, Hess K, Stewart DJ, Kurzrock R, **Issa JP**. Phase I study of azacitidine and oxaliplatin in patients with advanced cancers that have relapsed or are refractory to any platinum therapy. Clin Epigenetics. 2015 Mar 17;7(1):29. doi: 10.1186/s13148-015-0065-5. eCollection 2015. PubMed PMID: 25806091; PubMed Central PMCID: PMC4371799.
210. van Esterik JC, Vitins AP, Hodemaekers HM, Kamstra JH, Legler J, Pennings JL, Steegenga WT, Lute C, Jelinek J, **Issa JP**, Dollé ME, van der Ven LT. Liver DNA methylation analysis in adult female C57BL/6JxFVB mice following perinatal exposure to bisphenol A. Toxicol Lett. 2014 Oct 17;232(1):293-300. doi: 10.1016/j.toxlet.2014.10.021. [Epub ahead of print] PubMed PMID: 25455458.
211. **Issa JP**, Garcia-Manero G, Huang X, Cortes J, Ravandi F, Jabbour E, Borthakur G, Brandt M, Pierce S, Kantarjian HM. Results of phase 2 randomized study of low-dose decitabine with or without valproic acid in patients with myelodysplastic syndrome and acute myelogenous leukemia. Cancer. 2015 Feb 15;121(4):556-61. doi: 10.1002/cncr.29085. Epub 2014 Oct 21. PubMed PMID: 25336333; PubMed Central PMCID: PMC4320000.
212. Yamazaki J, Jelinek J, Lu Y, Cesaroni M, Madzo J, Neumann F, He R, Taby R, Vasanthakumar A, Macrae T, Ostler KR, Kantarjian HM, Liang S, Estecio MR, Godley LA, **Issa JP**. TET2 mutations affect non-CpG island DNA methylation at enhancers and transcription factor binding sites in chronic myelomonocytic leukemia. Cancer Res. 2015 May 13. pii: canres.0739.2014. [Epub ahead of print] PubMed PMID:25972343.
213. Fujii S, Srivastava V, Hegde A, Kondo Y, Shen L, Hoshino K, Gonzalez Y, Wang J, Sasai K, Ma X, Katayama H, Estecio MR, Hamilton SR, Wistuba I, **Issa JJ**, Sen S. Regulation of AURKC expression by CpG island methylation in human cancer cells. Tumour Biol. 2015 May 20. [Epub ahead of print] PubMed PMID: 25990457.
214. Tefferi A, Kantarjian H, Rajkumar SV, Baker LH, Abkowitz JL, Adamson JW, Advani RH, Allison J, Antman KH, Bast RC Jr, Bennett JM, Benz EJ Jr, Berliner N, Bertino J, Bhatia R, Bhatia S, Bhojwani D, Blanke CD, Bloomfield CD, Bosserman L, Broxmeyer HE, Byrd JC, Cabanillas F, Canellos GP, Chabner BA, Chanan-Khan A, Cheson B, Clarkson B, Cohn SL, Colon-Otero G, Cortes J, Coutre S, Cristofanilli M, Curran WJ Jr, Daley GQ, DeAngelo DJ, Deeg HJ, Einhorn LH, Erba HP, Esteva FJ, Estey E, Fidler IJ, Foran J, Forman S, Freireich E, Fuchs C, George JN, Gertz MA, Giralt S, Golomb H, Greenberg P, Gutterman J, Handin RI, Hellman S, Hoff PM, Hoffman R, Hong WK, Horowitz M, Hortobagyi GN, Hudis C, **Issa JP**, Johnson BE, Kantoff PW, Kaushansky K, Khayat D, Khuri FR, Kipps TJ, Kripke M, Kyle RA, Larson RA, Lawrence TS, Levine R, Link MP, Lippman SM, Lonial S, Lyman GH, Markman M, Mendelsohn J, Meropol NJ, Messinger Y, Mulvey TM, O'Brien S, Perez-Soler R, Pollock R, Prchal J, Press O,

Radich J, Rai K, Rosenberg SA, Rowe JM, Rugo H, Runowicz CD, Sandmaier BM, Saven A, Schafer AI, Schiffer C, Sekeres MA, Silver RT, Siu LL, Steensma DP, Stewart FM, Stock W, Stone R, Storb R, Strong LC, Tallman MS, Thompson M, Ueno NT, Van Etten RA, Vose JM, Wiernik PH, Winer EP, Younes A, Zelenetz AD, LeMaistre CA. In Support of a Patient-Driven Initiative and Petition to Lower the High Price of Cancer Drugs. Mayo Clin Proc. 2015 Aug;90(8):996-1000. doi: 10.1016/j.mayocp.2015.06.001. Epub 2015 Jul 23. PubMed PMID: 26211600.

215. Malouf GG, Tahara T, Paradis V, Fabre M, Guettier C, Yamazaki J, Long H, Lu Y, Raynal NJ, Jelinek J, Mouawad R, Khayat D, Brugières L, Raymond E, **Issa JP**. Methylome sequencing for fibrolamellar hepatocellular carcinoma depicts distinctive features. Epigenetics. 2015 Sep 2;10(9):872-81. doi: 10.1080/15592294.2015.1076955. PubMed PMID: 26224146.
216. **Issa JJ**, Roboz G, Rizzieri D, Jabbour E, Stock W, O'Connell C, Yee K, Tibes R, Griffiths EA, Walsh K, Daver N, Chung W, Naim S, Taverna P, Oganesian A, Hao Y, Lowder JN, Azab M, Kantarjian H. Safety and tolerability of guadecitabine (SGI-110) in patients with myelodysplastic syndrome and acute myeloid leukaemia: a multicentre, randomised, dose-escalation phase 1 study. Lancet Oncol. 2015 Aug 18. pii: S1470-2045(15)00038-8. doi: 10.1016/S1470-2045(15)00038-8. [Epub ahead of print] PubMed PMID: 26296954.
217. Foy JP, Pickering CR, Papadimitrakopoulou VA, Jelinek J, Lin SH, William WN, Jr, Mitchell FT, Wang J, Lang W, Feng L, Zhang L, Kim ES, Fan YH, Hong WK, El-Naggar A, Lee JJ, Myers JN, **Issa JJ**, Lippman SM, Mao L, Saintigny P. New DNA methylation markers and global DNA hypomethylation are associated with oral cancer development. Cancer Prev Res (Phila). 2015 Sep 4. pii: canprevres.0179.2014. [Epub ahead of print] PubMed PMID: 26342026.
218. Yanxun Xu, Xiaofeng Zheng, Yuan Yuan, Estecio MR, **Issa JP**, Peng Qiu, Yuan Ji, Shoudan Liang. BM-SNP: A Bayesian Model for SNP Calling Using High Throughput Sequencing Data. IEEE/ACM Trans Comput Biol Bioinform. 2014 Nov-Dec;11(6):1038-44. doi: 10.1109/TCBB.2014.2321407. PubMed PMID: 26357041.
219. Qin T, Si J, Raynal NJ, Wang X, Gharibyan V, Ahmed S, Hu X, Jin C, Lu Y, Shu J, Estecio MR, Jelinek J, **Issa JP**. Epigenetic synergy between decitabine and platinum derivatives. Clin Epigenetics. 2015 Sep 11;7(1):97. doi:10.1186/s13148-015-0131-z. eCollection 2015. PubMed PMID: 26366234; PubMed Central PMCID: PMC4567801.
220. Jin C, Qin T, Barton MC, Jelinek J, **Issa JJ**. Minimal Role of Base Excision Repair in TET-Induced Global DNA Demethylation in HEK293T Cells. Epigenetics. 2015 Oct 6:0. [Epub ahead of print] PubMed PMID: 26440216.
221. Kopetz S, Desai J, Chan E, Hecht JR, O'Dwyer PJ, Maru D, Morris V, Janku F, Dasari A, Chung W, **Issa JJ**, Gibbs P, James B, Powis G, Nolop KB, Bhattacharya S, Saltz L. Phase II Pilot Study of Vemurafenib in Patients With Metastatic BRAF-Mutated Colorectal Cancer. J Clin Oncol. 2015 Oct 12. pii: JCO.2015.63.2497. [Epub ahead of print] PubMed PMID: 26460303.
222. Kropf P, Barnes G, Tang B, Pathak A, **Issa JJ**. Healthcare utilization and costs associated with tyrosine kinase inhibitor switching in patients with chronic myeloid leukemia. Leuk Lymphoma. 2015 Oct 20:1-21. [Epub ahead of print] PubMed PMID: 26480922.
223. Lowder JN, Taverna P, **Issa JJ**. Will next-generation agents deliver on the promise of epigenetic hypomethylation therapy? Epigenomics. 2015 Nov 6. [Epub ahead of print] PubMed PMID: 26541345.
224. Laumet G, Garriga J, Chen SR, Zhang Y, Li DP, Smith TM, Dong Y, Jelinek J, Cesaroni M, **Issa JP**, Pan HL. G9a is essential for epigenetic silencing of K(+) channel genes in acute-to-chronic pain transition. Nat Neurosci. 2015 Dec;18(12):1746-55. doi: 10.1038/nn.4165. Epub 2015 Nov 9. PubMed PMID: 26551542; PubMed Central PMCID: PMC4661086.
225. Yamazaki J, Taby R, Jelinek J, Raynal NJ, Cesaroni M, Pierce SA, Kornblau SM, Bueso-Ramos CE, Ravandi F, Kantarjian HM, **Issa JP**. Hypomethylation of TET2 Target Genes Identifies a

Curable Subset of Acute Myeloid Leukemia. J Natl Cancer Inst. 2015 Nov 13;108(2). pii: djv323. doi: 10.1093/jnci/djv323. PubMed PMID: 26568194.
226. Bouwmeester MC, Ruiter S, Lommelaars T, Sippel J, Hodemaekers HM, van den Brandhof EJ, Pennings JL, Kamstra JH, Jelinek J, **Issa JJ**, Legler J, van der Ven LT. Zebrafish embryos as a screen for DNA methylation modifications after compound exposure. Toxicol Appl Pharmacol. 2015 Dec 19;291:84-96. doi:10.1016/j.taap.2015.12.012. [Epub ahead of print] PubMed PMID: 26712470.
227. Raynal NJ, Lee JT, Wang Y, Beaudry A, Madireddi P, Garriga J, Malouf GG, Dumont S, Dettman EJ, Gharibyan V, Ahmed S, Chung W, Childers WE, Abou-Gharbia M, Henry RA, Andrews AJ, Jelinek J, Cui Y, Baylin SB, Gill D, **Issa JJ**. Targeting calcium signaling induces epigenetic reactivation of tumor suppressor genes in cancer. Cancer Res. 2015 Dec 30. pii: canres.2391.2014. [Epub ahead of print] PubMed PMID: 26719529.
228. Kleb B, Estécio MR, Zhang J, Tzelepi V, Chung W, Jelinek J, Navone NM, Tahir S, Marquez VE, **Issa JP**, Maity S, Aparicio A. Differentially methylated genes and androgen receptor re-expression in small cell prostate carcinomas. Epigenetics. 2016 Feb 18:1-10. [Epub ahead of print] PubMed PMID: 26890396.
229. Overman MJ, Morris V, Moinova H, Manyam G, Ensor J, Lee MS, Eng C, Kee B, Fogelman D, Shroff RT, LaFramboise T, Mazard T, Feng T, Hamilton S, Broom B, Lutterbaugh J, **Issa JP**, Markowitz SD, Kopetz S. Phase I/II study of azacitidine and capecitabine/oxaliplatin (CAPOX) in refractory CIMP-high metastatic colorectal cancer: evaluation of circulating methylated vimentin. Oncotarget. 2016 Aug 16. doi: 10.18632/oncotarget.11317. [Epub ahead of print] PubMed PMID: 27542211.
230. Sato T, Cesaroni M, Chung W, Panjarian S, Tran A, Madzo J, Okamoto Y, Zhang H, Chen X, Jelinek J, **Issa JJ**. Transcriptional Selectivity of Epigenetic Therapy in Cancer. Cancer Res. 2016 Nov 22. pii: canres.0834.2016. [Epub ahead of print] PubMed PMID: 27879268.

Invited Articles

1. Belinsky SA, Nikula KJ, Baylin SB, **Issa JP**. A microassay for measuring cytosine DNA methyltransferase activity during tumor progression. Toxicol Lett 82-83:335-40, 12/1995. PMID:8597074
2. **Issa JP**, Baylin SB, Herman JG. DNA methylation changes in hematologic malignancies: biologic and clinical implications. Leukemia 11 Suppl 1:S7-11, 3/1997. PMID:9130685
3. Lengauer C, **Issa JP**. The role of epigenetics in cancer. DNA Methylation, Imprinting and the Epigenetics of Cancer--an American Association for Cancer Research Special Conference. Las Croabas, Puerto Rico, 12-16 1997 December. Mol Med Today 4(3):102-3, 3/1998. PMID:9575489
4. Baylin SB, Herman JG, Graff JR, Vertino PM, **Issa JP**. Alterations in DNA methylation: a fundamental aspect of neoplasia. Adv Cancer Res 72:141-96, 1998. PMID:9338076
5. **Issa JP**. Aging, DNA methylation and cancer. Crit Rev Oncol Hematol 32(1):31-43, 9/1999. PMID:10586353
6. Toyota M, **Issa JP**. CpG island methylator phenotypes in aging and cancer. Semin Cancer Biol 9(5):349-57, 10/1999. PMID:10547343
7. Ahuja N, **Issa JP**. Aging, methylation and cancer. Histol Histopathol 15(3):835-42, 7/2000. PMID:10963127
8. Garcia-Manero G and **Issa JP**J. CpG island methylation in cancer. Cancer Research Alert 1(12):133-136, 2000.

9. Toyota M, **Issa JP**. The role of DNA hypermethylation in human neoplasia. Electrophoresis 21(2):329-33, 2000. PMID:10675010
10. Santini V, Kantarjian H , **Issa JP**J. DNA methylation in hematologic malignancies-pathophysiology and therapeutic implications. Annals of Int. Med 134:573-586, 2001. PMID:11281740
11. **Issa JP**. Epigenetic variation and human disease. J Nutr 132(8 Suppl):2388S-2392S, 8/2002. PMID:12163698
12. Shen L, **Issa JP**J. Epigenetics in Colorectal Cancer. Current Opinion in Gastroenterology 18:68-73, 2002. PMID:17031233
13. **Issa JP**. Age-related epigenetic changes and the immune system. Clin Immunol 109(1):103-8, 10/2003. PMID:14585281
14. **Issa JP**. Decitabine. Curr Opin Oncol 15(6):446-51, 11/2003. PMID:14624227
15. Rashid A, **Issa JP**. CpG island methylation in gastroenterologic neoplasia: a maturing field. Gastroenterology 127(5):1578-88, 11/2004. PMID:15521024
16. **Issa JP**. CpG island methylator phenotype in cancer. Nat Rev Cancer 4(12):988-93, 12/2004. PMID: 15573120
17. Kondo Y, **Issa JP**. Epigenetic changes in colorectal cancer. Cancer Metastasis Rev 23(1-2):29-39, 2004. PMID:15000147
18. List AF, Vardiman J, **Issa JP**, DeWitte TM. Myelodysplastic syndromes. Hematology Am Soc Hematol Educ Program:297-317, 2004. PMID:15561689
19. Kantarjian HM, **Issa JP**. Decitabine dosing schedules. Semin Hematol 42(3 Suppl 2):S17-22, 7/2005. PMID:16015500
20. **Issa JP**, Byrd JC. Decitabine in chronic leukemias. Semin Hematol 42(3 Suppl 2):S43-9, 7/2005. PMID:16015505
21. Toyota M, **Issa JP**. Epigenetic changes in solid and hematopoietic tumors. Semin Oncol 32(5):521-30, 10/2005. PMID:16210093
22. **Issa JP**, Kantarjian HM, Kirkpatrick P. Azacitidine. Nat Rev Drug Discov 4:275-6, 2005. PMID:15861567
23. **Issa JP**. DNA methylation in the treatment of hematologic malignancies. Clin Adv Hematol Oncol. 3(9):684-6, 2005. PMID:16224441
24. **Issa JP**, Kantarjian HM. Emerging role of epigenetic therapy: focus on decitabine. Semin Hematol Jul;42(2 Suppl 2)(S1-2), 2005. PMID:16015499
25. Garcia-Manero G, **Issa JP**. Histone deacetylase inhibitors: a review of their clinical status as antineoplastic agents. Cancer Invest 23(7):635-42, 2005. PMID:16305991
26. **Issa JP**. Optimizing therapy with methylation inhibitors in myelodysplastic syndromes: dose, duration, and patient selection. Nat.Clin.Pract.Oncol 2 Suppl 1:S24-S29, 2005. PMID:16341237
27. Oki Y, **Issa JP**. Review: recent clinical trials in epigenetic therapy. Rev Recent Clin Trials 1(2):169-82, 5/2006. PMID:18473969
28. Oki Y, Aoki E, **Issa JP**. Decitabine--bedside to bench. Crit Rev Oncol Hematol 61:140-52, 2/2007. PMID:17023173
29. Plimack ER, Kantarjian HM, **Issa JP**. Decitabine and its role in the treatment of hematopoietic malignancies. Leuk Lymphoma 48(8):1472-81, 2007. PMID:17701577
30. **Issa JP**. DNA methylation as a therapeutic target in cancer. Clin Cancer Res 13(6):1634-7, 2007. PMID:17363514
31. Konishi K, **Issa JP**. Targeting aberrant chromatin structure in colorectal carcinomas. Cancer J 13(1):49-55, 2007. PMID:17464246
32. Oki Y, **Issa JP**. Treatment options in advanced myelodysplastic syndrome, with emphasis on epigenetic therapy. Int J Hematol 86(4):306-14, 2007. PMID:18055336

33. Jabbour E, **Issa JP**, Garcia-Manero G, Kantarjian H. Evolution of decitabine development: accomplishments, ongoing investigations, and future strategies. Cancer 112(11):2341-51, 6/2008. PMID:18398832
34. Kantarjian H, O'Brien S, Cortes J, Wierda W, Faderl S, Garcia-Manero G, **Issa JP**, Estey E, Keating M, Freireich EJ. Therapeutic advances in leukemia and myelodysplastic syndrome over the past 40 years. Cancer 113(7 Suppl):1933-52, 10/1/2008. PMID:18798533
35. Batty N, Malouf GG, **Issa JP**. Histone deacetylase inhibitors as anti-neoplastic agents. Cancer Lett. e-Pub 4/2009. PMID:19345475
36. Estecio MR, **Issa JP**. Tackling the methylome: recent methodological advances in genome-wide methylation profiling. Genome Med. 2009 Nov 16;1(11):106 PMID:19930617
37. Santos FP, Kantarjian H, Garcia-Manero G, **Issa JP**, Ravandi F., Decitabine in the treatment of myelodysplastic syndromes. Expert Rev Anticancer Ther. 2010 Jan;10(1):9-22 PMID: 20014881
38. **Issa JP**, Kantarjian HM. Targeting DNA methylation. Clin Cancer Res. 2009 Jun 15;15(12):3938-46. Epub 2009 Jun 9. PubMed PMID: 19509174; PubMed Central PMCID: PMC2732562.
39. **Issa JP**. Epigenetic changes in the myelodysplastic syndrome. Hematol Oncol Clin North Am. 2010 Apr;24(2):317-30. Review. PubMed PMID: 20359628; PubMed Central PMCID: PMC2848959.
40. Bryan J, Jabbour E, Prescott H, Garcia-Manero G, **Issa JP**, Kantarjian H. Current and future management options for myelodysplastic syndromes. Drugs. 2010 Jul 30;70(11):1381-94. doi: 10.2165/11537920-000000000-00000. Review. PubMed PMID: 20614946.
41. Kondo Y, **Issa JP**. DNA methylation profiling in cancer. Expert Rev Mol Med. 2010 Jul 28;1e23. Review. PubMed PMID: 20663272.
42. Taby R, **Issa JP**. Cancer epigenetics. CA Cancer J Clin. 2010 Nov-Dec;60(6):376-92. Epub 2010 Oct 19. Review. PubMed PMID: 20959400.
43. Estécio MR, **Issa JP**. Dissecting DNA hypermethylation in cancer. FEBS Lett. 2011 Jul 7;585(13):2078-86. Epub 2010 Dec 10. Review. PubMed PMID: 21146531.
44. Boumber Y, **Issa JP**. Epigenetics in cancer: what's the future? Oncology (Williston Park). 2011 Mar;25(3):220-6, 228. Review. PubMed PMID: 21548464.
45. Beck S, Bernstein BE, Campbell RM, Costello JF, Dhanak D, Ecker JR, Greally JM, **Issa JP**, Laird PW, Polyak K, Tycko B, Jones PA; for the AACR Cancer Epigenome Task Force. A Blueprint for an International Cancer Epigenome Consortium. A Report from the AACR Cancer Epigenome Task Force. Cancer Res. 2012 Dec 15;72(24):6319-6324. Epub 2012 Nov 27. PubMed PMID: 23188507.
46. Yamazaki J, **Issa JP**. Epigenetic aspects of MDS and its molecular targeted therapy. Int J Hematol. 2012 Oct 10. [Epub ahead of print] PubMed PMID: 23054654.
47. **Issa JP.** The myelodysplastic syndrome as a prototypical epigenetic disease. Blood. 2013 May 9;121(19):3811-7. doi: 10.1182/blood-2013-02-451757. Review. PubMed PMID: 23660859; PubMed Central PMCID: PMC3650703.
48. **Issa JP**. Aging and epigenetic drift: a vicious cycle. J Clin Invest. 2014 Jan 2;124(1):24-9. doi: 10.1172/JCI69735. Epub 2014 Jan 2. Review. PubMed PMID: 24382386; PubMed Central PMCID: PMC3871228.
49. Sapienza C, **Issa JP**. Diet, Nutrition, and Cancer Epigenetics. Annu Rev Nutr. 2016 Jul 17;36:665-81. doi: 10.1146/annurev-nutr-121415-112634. PubMed PMID: 27022771.
50. Jones PA, **Issa JP**, Baylin SB. Targeting the cancer epigenome for therapy. Accepted for publication, Nat Rev Genet. 2016 Sep 15;17(10):630-41. doi: 10.1038/nrg.2016.93. PubMed PMID:27629931.

Editorials

1. **Issa JP**, Baylin SB. Epigenetics and Human Disease. (News and Views). Nature Medicine 2:281-282, 1996.
2. **Issa JP**. Methylation and Prognosis-of Molecular Clocks and Hypermethylator Phenotypes. Clin. Cancer Res 9:2879-81, 2003.
3. Yang AS, **Issa JP**. Mitoxantrone: a hypomethylating agent? Cancer Biol Ther 2(3):264-5, May-Jun, 5/2003.
4. **Issa JP**, Shen L, Toyota M. CIMP, at last. Gastroenterology 129(3):1121-4, 2005.
5. Plimack ER, Stewark DJ,**Issa JP**. Combining epigenetic and cytotoxic therapy in the treatment of solid tumors. J Clin Oncol 25(29):4519-21, 2007.
6. **Issa JP**. Colon cancer: it's CIN or CIMP. Clin Cancer Res 14(19):5939-40, 10/1/2008.
7. **Issa JP**. Cancer prevention: epigenetics steps up to the plate. Cancer Prev Res (Phila PA), 2008 Sep;1(4):219-22. Epub 2008 Mar 19.
8. **Issa JP**, Garber JE. Time to think outside the (genetic) box. Cancer Prev Res (Phila). 2011 Jan;4(1):6-8. PubMed PMID: 21205738.
9. **Issa JP**, Just W. Epigenetics. FEBS Lett. 2011 Jul 7;585(13):1993. Epub 2011 Jun 17. PubMed PMID: 21693122.
10. **Issa JP**. Epigenetic variation and cellular Darwinism. Nat Genet. 2011 Jul 27;43(8):724-6. doi: 10.1038/ng.897. PubMed PMID: 21792236.
11. **Issa JP**. DNA methylation as a clinical marker in oncology. J Clin Oncol. 2012 Jul 10;30(20):2566-8. Epub 2012 May 7. PubMed PMID: 22564986.

Book Chapters

1. **Issa JP**J. Hypermethylator phenotypes in aging and cancer. In: DNA Alterations in Cancer: Genetic and Epigenetic Changes. Ed(s) M. Ehrlich. 1999: BioTechniques Books, Eaton Publishing, 311-322, 1999.
2. Issa J-PJ. CpG Island methylation in aging and cancer. In: Curr Top Microbiol Immunol. 249, 101-18, 2000.
3. Issa J-PJ. The epigenetics of colorectal cancer. In: Proceedings of the N. Y. Academy of Sciences. 910, 140-53, 2000.
4. Toyota M , Issa J-PJ. Methylated CpG Island Amplification for Methylation Analysis and Cloning Differentially Methylated Sequences. In: In Methods in Molecular Biology: DNA Methylation Protocols. 200, 101-110, 2002.
5. Issa J-PJ. The Aging Epigenome. The Epigenome: Molecular Hide and Seek. In: Prolonging Life. Wiley Publishing, 2002.
6. Yu Y, Fuji S, Yuan J, Luo RZ, Wang L, Bao J, Kadota M, Oshimura M, Dent SR, Issa J-PJ, Bast RC. Epigenetic regulation of ARHI in Breast and Ovarian Cancer Cells. In: Proceedings of the NY Academy of Sciences. 983, 268-277, 2003.
7. Garcia-Manero G, **Issa JP**J. The role of DNA hypomethylating agents in cancer treatment. In: Progress In Oncology 2004. 6, 131-158, 2004.
8. Yu Y, Luo R, Lu Z, Feng W, Badgwell D, **Issa JP**J, Rosen D, Liu J, Bast R. Biochemistry and Biology of ARHI (DIRAS3), and Imprinted Tumor Suppressor Gene Whose Expression is Lost in Ovarian and Breast Cancers. In: Methods In Enzymology. 407, 455-468, 2006.
9. Oki Y and **Issa JP**. Epigenetic mechanisms in AML - a target for therapy. Cancer Treat Res. 2010;145:19-40.
10. **Issa JP.** Age-related variation in DNA methylation. In: Epigenetic Epidemiology. Ed. Karin Michels, 2012, Springer.

11. **Issa JP.** DNA methylation inhibitors. In: Molecular Oncology. Ed. Edward Gelmann, Charles Sawyers and Frank Rauscher III. 2014, Cambridge University Press.
12. Raynal NJM and **Issa JPJ.** DNA Methyltransferase Inhibitors. In: Drug Discovery in Cancer Epigenetics. Ed. Gerda Egger and Paola Arimondo. 2016, Academic Press, Elsevier.
13. Fasan O, Boland P, Kropf P and **Issa JPJ**. Epigenetics and Epigenetic Therapy of Cancer. In: Targeted Therapy in Translational Cancer Research. Ed. Tsimberidou AM, Kurzrock R and Anderson KC, 2016, Wiley Blackwell.
14. Kelly A and **Issa JPJ**. Epigenetics and Cancer. In: Epigenetics, Energy Balance, and Cancer Ed. Berger N, 2016, Springer.
15. Sato T, **Issa JPJ** and Kropf P. DNA Hypomethylating Drugs in Cancer Therapy. In: Perspectives on Chromatin Deregulation in Cancer. Ed. Scott A. Armstrong, Steven Henikoff, and Christopher R. Vakoc, 2016, Cold Spring Harbor Laboratory Press.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

Recommendations for Advisory Committee on Childhood Cancers (ACCC)

- Mohamad Al-Rahawan, M.D., MPH
- James Amatruda, M.D., Ph.D.
- Greg Aune, M.D.
- Juan Carlos Bernini, M.D.
- Stan Goldman, M.D.
- Meaghan Granger, M.D.
- Virginia Harod, M.D.
- Lisa Hartman, M.D.
- Barkat Hooda, M.D.
- Julie Luke, CPNP
- Cindy Schwartz, M.D., MPH
- Sheila Thampi, M.D

PO Box 12097 | Austin, Texas 78711 | PH: 512-463-3190 | F: 512-475-2563 | cprit.texas.gov

BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors.
Follow this format for each person. **DO NOT EXCEED FIVE PAGES.**

NAME: Mohamad Al-Rahawan

eRA COMMONS USER NAME (credential, e.g., agency login):

POSITION TITLE: Division Chief of Pediatric Hematology Oncology

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable. Add/delete rows as necessary.)*

INSTITUTION AND LOCATION	DEGREE *(if applicable)*	Completion Date MM/YYYY	FIELD OF STUDY
Damascus University Medical School, Damascus, Syria	MD	12/1998	Medicine
St John Hospital and Medical Center, Detroit, MI	Residency	07/2003	Pediatric
University of Virgina Health System, Charlottesville, VA	Fellowship	07/2004	Pediatric Hem/Onc
Children's National Medical Center, Washington, DC and NCI, NIH, Rockville, MD	Fellowship	07/2007	Pediatric Hem/Onc
George Washington University, School of Public Health and Health Sciences, Washington, DC	MPH	05/2007	Public Health

A. Personal Statement

I am a Board Certified Pediatric Hematologist Oncologist with clinical expertise in treating childhood cancer and blood disorders. In the 7 years preceding my arrival to Texas Tech University Health Sciences Center, I served as Attending Pediatric Hematologist/Oncologist, Division Head, and Associate Professor of Clinical Pediatrics at the University of Illinois College of Medicine in Peoria, IL. I also served as adjunct faculty at St. Jude Children's in Memphis, TN. My research experience includes expression of cell cycle markers in inherited marrow failures, the impact of anemia on the quality of life in children receiving cancer treatment, hematological anomalies in diabetic children, and safe implementation of electronic medical records in pediatric oncology practice. Also during my time in Peoria, I functioned as a clinical pediatric hematologist oncologist member of the Children's Oncology Group. I am board-certified in Pediatric Hematology Oncology. I consider myself a general pediatric hematologist oncologist with advanced skills in solid tumors and bone marrow failure. I enjoy working with patients and their families and I have been commended by my team for my interpersonal skills.

B. Positions and Honors

Positions:

8/2007 – 9/2014 Staff Physician, St Jude Midwest Affiliate, Peoria, IL

8/2007 – 9/2014 Attending Pediatric Hematologist/Oncologist, Children's Hospital of Illinois, OSF-St. Francis Medical Center, Peoria, IL
8/2007 – 9/2014 Adjunct Faculty, St Jude's Children's Research Hospital, Memphis, TN
8/2007 – 3/2014 Assistant Professor of Clinical Pediatrics, University of Illinois College of Medicine at Peoria, Peoria, IL
7/2009 – 12/2013 Student, Resident and Visiting Fellow Rotation Director, Pediatric Hematology/Oncology, University of Illinois College of Medicine at Peoria, Peoria, IL
7/2011 – 6/2012 Assistant Division Head, Division of Pediatric Hematology Oncology, University of Illinois College of Medicine at Peoria, Peoria, IL
7/2012 – 9/2014 Medical Director of the St. Jude Midwest Affiliate Clinic, St Jude Midwest Affiliate, Peoria, IL
7/2012 – 9/2014 Division Head, Division of Pediatric Hematology Oncology, University of Illinois College of Medicine at Peoria, Peoria, IL
4/2014 – 9/2014 Associate Professor of Clinical Pediatrics, University of Illinois College of Medicine at Peoria, Peoria, IL
9/2014 – Present Attending Pediatric Hematologist/Oncologist, Covenant Women's and Children's Hospital, Lubbock, TX
9/2014 – Present Attending Pediatric Hematologist/Oncologist, University Medical Center, Lubbock, TX
9/2014 – Present Associate Professor of Clinical Pediatrics, Department of Pediatrics, Texas Tech Health Sciences Center, Lubbock, TX
9/2014 – Present Division Chief of Pediatric Hematology Oncology, Department of Pediatrics, Texas Tech Health Sciences Center, Lubbock, TX

Honors:

6/1999 Higher Education Scholar of the Year, Ministry of Education, Damascus, Syria

3/2003 Best Research Project in Pediatrics (Short Course Prednisone vs. IV IgG in ITP), St. John Hospital and Medical Center, Detroit, MI

6/2009 Annual Best Crowd Draw for Grand Rounds, University of Illinois College of Medicine at Peoria, Peoria, IL

6/2010 Annual Pediatric Residency Program Subspecialist Teaching Award, University of Illinois College of Medicine at Peoria, Peoria, IL

6/2011 Annual Pediatric Residency Program Overall Teaching Award, University of Illinois College of Medicine at Peoria, Peoria, IL

6/2012 Annual Pediatric Residency Program Subspecialist Teaching Award, University of Illinois College of Medicine at Peoria, Peoria, IL

11/2012 Annual Faculty Evaluation fourth Highest Rank in the Pediatric Department, Pediatric Faculty Performance Review Committee, University of Illinois College of Medicine at Peoria, Peoria, IL

11/2014 Sixth Annual Celebration of Excellence Recognition, University of Illinois College of Medicine at Peoria, Peoria, IL

6/2015 Annual Pediatric Residency Program, Subspecialist of the Year Award, Texas Tech University Health Sciences Center, Lubbock, TX

C. Contribution to Science

Descriptive and predictive variables in children with bone marrow failure:
During my years of fellowship in pediatric hematology oncology, I worked with Dr. Blanch Alter at the National Institutes of Health (NIH). My main focus was on cell cycle markers in the marrow of these

patients and their predictive value in diagnosis, response to therapy and outcome afterwards. This contribution resulted in the following publications:

Al-Rahawan MM, Giri N, Alter BP. Intensive Immunosuppression Therapy for Aplastic Anemia Associated with Dyskeratosis Congenita. *Int J Hematol.* **2006** Apr;83(3):275-276

Al-Rahawan MM, Alter BP, Bryant BJ, Elghetany MT. Bone Marrow Cell Cycle Markers in Inherited Bone Marrow Failure Syndromes. *Leukemia Research.* **2008** dec;32(12): 1793-9

Hutson, SP, Han, PK, Hamilton, JG, Rife, SC, **Al-Rahawan MM,** Moser RP, Duty SP, Anand S, Alter BP, The Use of haematopoietic stem cell transplantation in Fanconi Anaemia Patients: A survey of decision making among families in the US and Canada. *Health Expect.* **2013**, Apr 29

Hodgkin disease in pediatrics:
As a junior faculty, I worked with one of my mentors, Dr. Pedro de Alarcon, on a few projects in the study of Hodgkin disease. We reviewed the progress in this field and collaborated with others to publish in peer reviewed journals and online sources. This contribution resulted in the following publications:

Al-Rahawan MM, de Alarcon PA. Gemcitabine and Vinorelbine Therapy for Patients with Hodgkin Lymphoma. *Pediatric Health.* **2009** Dece;3(6): 525-532.

de Alarcon PA, Fernandez KS, **Al-Rahawan MM**, Metzger M. Pediatric Hodgkin Lymphoma. Medscape Reference. Updated April 30, **2015**. Available at: http://emedicine.medscape.com/article/987101-overview.

Genotype-phenotype relations in rare syndromes:
As a clinician, I encountered rare manifestations of rare syndromes. Occasionally, the manifestations were never described before. I collaborated with experts in the field to describe the genotype of the phenotype I encountered. We have successfully published some of our findings in case report formats. This contribution resulted in the following publications:

Al-Rahawan MM, Chute DJ, So-Church K, Gripp KW, Stabley DL, McDaniel NL, Wilson WG, Waldron PE. Hepatoblastoma and Heart Transplantation in a Patient with Cardio-Facio-Cutaneous Syndrome. *Am J Med Genet A.* **2007** Jul 1; 143(13):1481-8

Gripp KW, Lin AE, Nicholson L, Allen W, Cramer A, Jones KL, Kutz W, Peck D, Rebolledo MA, Wheeler PG, Wilson W, **Al-Rahawan MM,** Stabley DL, Sol-Church K. Further delineation of the phenotype resulting from BRAF or MEK1 germline mutations helps differentiate cardio-facio-cutaneous syndrome from Costello syndrome. Am J Med Genet A. **2007** Jul 1;143(13):1472-80.

Shah KM, Pratt EI, **Al-Rahawan MM,** Abraham RS. Novel Combination of ITGB2 Mutations Causing Leukocyte Adhesion Deficiency Type 1 (LAD-1). *J Pediatric Infectious Diseases.* **2011,** Aug 2(6): 141-148

Howard K, Hall CP, **Al-Rahawan MM.** Wiskott-Aldrich Syndrome: Description of a New Gene Mutation Without Immunodeficiency. *J Pediatr Hematol Oncol. **2016** Mar;38(2):163.*

Supervised medical student, resident trainee and junior faculty project publications:
As a medical educator, I contributed to the professional development of multiple learners and collegues. Many of the projects that resulted from this role were published in poster or manuscript forms. Examples of this are below:

Al-Rahawan MM, Gray BM, Mitchell CS, Smith SD. Thoracic vertebral osteomyelitis with paraspinous mass and intraspinal extension: an atypical presentation of cat scratch disease. *Pediatr Radiol.* **2012** Jan;42(1): 116-9.

Al-Rahawan MM, Siebert JD, Mitchell CS, Smith SD. Durable Complete Response to Chemotherapy in an Infant with a Clivus Chordoma. *Pediatr Blood Cancer.* **2012** Aug;59(2): 323-5 doi: 10.1002/pbc.23297

Prus KM**, Al-Rahawan MM**, Recovery of Native Erythropoietin Production in a Patient with Erythropoietin-Associated Pure Red Cell Aplasia, *Pediatr Nephrol.* **2014,** Jan:29(1): 161-2

Howard K, Averitt G, **Al-Rahawan MM**, Levent F. Neonatal Disseminated HSV-2 Triggering Hemophagocytic Lymphohistiocytosis: A Case Report. *J Pediatric Infect Dis Soc.* **2015.** In Review

Complete list of publications:
https://www.ncbi.nlm.nih.gov/myncbi/browse/collection/50833023/?sort=date&direction=ascending.

D. Research Support

05/2013-09/2014 Bar EK, Kyle Mays, Julie Kasap, **Al-Rahawan MM.** Evaluating the Impact of EMR Implementation and Pharmacy Support on the Pediatric Hematology-Oncology Physician Ordering of Chemotherapy, University of Illinois College of Medicine at Peoria, Peoria, IL. Co-PI**.** In process

07/2012-Present Anderson S, Baumann H, Drawbridge N, Barr EK, Aguilar A, **Al-Rahawan MM.** Exploring the Effect of Hemoglobin, HbF, Reticulocyte Count and EPO Levels on HbA1c in Type I and Type II Diabetic Children, Texas Tech University Health Sciences Center; Lubbock, TX. PI**.** Pending Funding

10/2015-Present Principal investigator, Passport for Care CPRIT Grant, Texas Tech University Health Sciences Center; Lubbock, TX

11/2015-Present Coinvestigator, Banking of Tissue and Establishing Continuous Cell Lines and Xenografts from Neoplasia" (SPOC Tissue Bank), Texas Tech University Health Sciences Center; Lubbock, TX

BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors.
Follow this format for each person. **DO NOT EXCEED FIVE PAGES.**

NAME: James Francis Amatruda, MD, PhD

eRA COMMONS USER NAME (credential, e.g., agency login): JAMATR

POSITION TITLE: Associate Professor of Pediatrics, Molecular Biology and Internal Medicine; Nearburg Family Professor of Pediatric Oncology Research

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable. Add/delete rows as necessary.)*

INSTITUTION AND LOCATION	DEGREE *(if applicable)*	YEAR(s)	FIELD OF STUDY
Harvard University	A.B.	June, 1986	Biochemistry
Washington University	M.D.	May, 1993	Medicine
Washington University	Ph.D.	May, 1993	Cell Biology
Brigham and Women's Hospital, Boston, MA	Residency	1993-1996	Internal Medicine
Dana-Farber/Partners Cancer Care	Fellowship	1997-1999	Medical Oncology
Boston Children's/Harvard Medical School	Post-doc	1999-2004	Cancer biology/genetics

A. Personal Statement

My background, research interests and leadership experience make me ideally suited to serve as co-Principal Investigator leading the Center for Pediatric Cancer Systems Biology.

Research Interest: As a physician-scientist, I see firsthand the devastating effects of pediatric cancers and have focused my research on understanding the molecular origins of childhood cancer with a view toward developing better treatments. As a postdoctoral fellow, I pioneered the use of zebrafish as a model of human cancer. In my own laboratory, I have continued to identify and develop new models of cancer in the zebrafish, and have translated these results into better understanding of the biology of human cancers. My group discovered novel BMP receptor mutations as a cause of germ cell tumors in zebrafish, and went on to investigate the role of BMP signaling in human germ cell tumors. We made the first zebrafish model of Ewing sarcoma by transgenically expressing the human Ewing sarcoma oncoprotein, EWS-FLI1, in zebrafish, and demonstrated that the fish model recapitulates key features of the human disease. As a complement to studies developing zebrafish genetic models, we have also carried out genomic characterization of childhood cancers, including germ cell tumor and Wilms tumor of the kidney. To facilitate the investigation of the biology of pediatric cancers, I have established close collaboration with pathologists, and serve on the Advisory Committee for the Pediatric Specimen Biorepository at UT Southwestern. A specific, long-term goal of my work is to develop model systems that better recapitulate the range of cancer cell phenotypes I encounter in my patients, especially those the drive adverse clinical outcomes.

Leadership: At UT Southwestern, I organize and lead a working group of 5 tenured or tenure-track faculty at UT Southwestern who are investigating different aspects of Ewing sarcoma using highly complementary approaches. I also serve as Associate Division Director for Research in the Division of Pediatric Hematology-Oncology, charged with helping to bridge the gap between the lab and the clinic, specifically by fostering collaborations between clinicians and basic scientists. As part of my commitment to this goal, two years ago I became the Assistant Director of the UT Southwestern Medical Scientist Training Program, which has been continuously funded by NIH for the past 34 years and which has a long track record of successfully producing career physician-scientists. I am a member of the Ewing Sarcoma Biology Committee in the Children's Oncology Group (COG), the national cooperative group that oversees all U.S. clinical trials in pediatric oncology. I also chair the COG Rare Tumors Biology committee and am a member of the steering committee for the COG's Solid Malignancies Integrated Translational Science Center. I directly participate in the design and institution of clinical trials for childhood germ cell tumors and other rare tumors. For these reasons, I am

ideally suited not only to help guide the U54 Center, but also to ensure that the results are incorporated into novel clinical trials going forward, thus helping to directly translate the results into improved patient care.

B. Positions and Honors

Positions and Employment

1986-1993	Medical Scientist Training Program, Washington University, St. Louis, MO.
1993-1994	Intern, Department of Internal Medicine, Brigham and Women's Hospital, Boston, MA
1994-1996	Resident, Department of Internal Medicine, Brigham and Women's Hospital, Boston, MA
1996-1997	Visiting Scientist, lab of Dr. Piero Benedetti, Istituto di Biologia Cellulare Consiglio Nazionale delle Ricerche, Rome, Italy
1997-1999	Clinical Fellow in Medicine/Oncology, Dana-Farber/Partners Cancer Care
1999-2004	Instructor in Medicine and Pediatrics, Harvard Medical School
2005-2012	Assistant Professor of Pediatrics, Molecular Biology and Internal Medicine University of Texas Southwestern Medical Center, Dallas, TX
2005-	Attending Physician, Hematology-Oncology, Children's Medical Center, Dallas, TX
2009-	Chair, Germ Cell Tumor Biology Sub-committee, Children's Oncology Group
2010-	Chair, Rare Tumors Biology Sub-committee, Children's Oncology Group.
2013-	Associate Professor of Pediatrics, Molecular Biology and Internal Medicine with tenure University of Texas Southwestern Medical Center
2014-	Assistant Director, Medical Scientist Training Program
2014-	Associate Division Director for Research, Division of Pediatric Hematology-Oncology

Other Experience and Professional Memberships

2005-	Ad hoc reviewer, *Development, Developmental Biology, PNAS, Disease Models and Mechanisms, Developmental Dynamics, PLOS Genetics*, *FASEB Journal, Journal of Pathology* and *Nature Genetics*
2011	NIH Peer Review committee, ZRG1 CB-Z (56) *ad hoc* member
2012-	Member, American Association for Cancer Research
2013	NIH Peer Review committee, Provocative Questions R21
2014-	Development, Differentiation and Cancer Study Section, American Cancer Society
2014-	Scientific Advisory Board, Pablove Foundation
2015-	President, Zebrafish Disease Models Society
2015	NIH Peer Review committee, ZCA1 RPRB-M (O2)
2015-	Scientific Advisory Board, William G. Forbeck Foundation and Curing Kids Cancer
2015-	Coursemaster, "Macromolecules" section, 1st-year Medical School Curriculum, UTSW

Honors and Awards

1991	Spencer T. and Ann W. Olin Medical Scientist Fellowship
1992	AFCR Medical Student Award for Excellence, Washington University
1993	*Alpha Omega Alpha*, Washington University
1994	Arnold Dunne Award for Outstanding Intern, Brigham and Women's Hospital
1996	Yamagiwa-Yoshida Postdoctoral Fellowship, International Union Against Cancer
1997	Adriano Buzzati-Traverso Foundation Prize, Rome, Italy
2003	William Guy Forbeck Foundation Scholar Award
2007-	Outstanding Lecturer. Awarded by the UT Southwestern 1st-year Med School Class (2007, 2009, 2010, 2011, 2012, 2013, 2014, 2015)
2013	Regent's Outstanding Teaching Award, University of Texas System

C. Contributions to Science

1. **Zebrafish as a cancer model**. As a post-doctoral fellow in Leonard Zon's laboratory at Children's Hospital Boston, I was among the first investigators to adopt the zebrafish system as a genetic model for cancer gene discovery. Zebrafish are ideally suited for this purpose, since the fish are susceptible to cancer and other human diseases, and are amenable to large-scale screens and forward-genetic approaches. Working with a graduate student colleague, Jen Shepard, I mutagenized zebrafish strains and carried out a forward-genetic screen to identify genes regulating embryonic cell proliferation. Through the screen, we identified two key cell-cycle regulators (*mybl2* and *espl2),* essential for embryonic development, both of which proved to be novel, haploinsufficient tumor suppressors (Shepard *et al.*, 2007; Shepard *et al.*, 2005). Moving to my independent faculty position at UT Southwestern, I have continued to use the fish system to

dissect cancer pathways. We described the identification of a zebrafish Cdc25a mutant, which gave novel insight into the role of the ATM-DNA Damage pathway during normal embryonic development (Verduzco *et al.*, 2012). More recently, we used transgenic approaches to make the first zebrafish model of Ewing's Sarcoma, a lethal bone tumor, identifying key oncogenic mediators via a cross-species comparative approach. Finally, in collaboration with my colleague John Abrams, I used zebrafish to demonstrate a completely novel function of p53 in suppression of germline retroelement transposition (Wylie *et al.*, 2015) These studies established the zebrafish as a powerful system for cancer biology studies.

a. Shepard JL*, **Amatruda JF***, Stern HM, Subramanian A, Finkelstein D, Ziai J, Finley KR, Pfaff KL, Hersey C, Zhou Y, Barut B, Freedman M, Lee C, Spitsbergen J, Neuberg D, Weber G, Golub TR, Glickman JN, Kutok JL, Aster JC, Zon LI. A zebrafish bmyb mutation causes genome instability and increased cancer susceptibility. Proc Natl Acad Sci U S A. 2005;102(37):13194-9. PMCID: 1198999 (*equal contribution).
b. Leacock SW, Basse AN, Chandler GL, Kirk AM, Rakheja D, **Amatruda JF**. A zebrafish transgenic model of Ewing's sarcoma reveals conserved mediators of EWS-FLI1 tumorigenesis. Dis Mod Mech. 2012;5(1):95-106. PMCID: 3255547.
c. Verduzco D, Dovey JS, Shukla AA, Kodym E, Skaug BA, **Amatruda JF**. Multiple isoforms of CDC25 oppose ATM activity to maintain cell proliferation during vertebrate development. Mol Can Res. 2012;10(11):1451-61. PMCID: 3511848.
d. Wylie A, Jones AE, D'Brot A, Lu W-J, Kurtz Pm, Moran JV, Rakheja D, Chen KS, **Amatruda JF,** Abrams JM. p53 genes act to restrain mobile elements. Genes Dev. 2016;30(1):64-77. PMID: 26701264.

2. **Molecular mechanisms of germ cell tumor.** We used forward-genetic screening to identify zebrafish that developed germ cell tumors (GCTs) with high penetrance; the first animal model of this disease (Neumann *et al.*, 2009). GCTs occur in infants, children and young adults, and testicular GCT is the most common cancer of young men, but the molecular pathogenesis of these tumors is unknown. We identified a mutation in the Type IB BMP receptor, *bmpr1bb*, as the cause of the zebrafish GCTs (Neumann *et al.*, 2011). BMPs (Bone Morphogenetic Proteins), members of the TGF-beta superfamily, play important roles in development and differentiation, but have not been linked to GCTs. Extending our work from the fish model to human tumors, we showed that the BMP pathway is misregulated in human GCTs as well (Fustino *et al.*, 2011). These studies identified BMP signaling as a key node in GCT differentiation and a promising target for novel therapies. In further studies of human GCTs, we performed genome-wide methylation analysis and identified mechanisms driving Wnt/beta-catenin signaling in the tumors (Amatruda *et al.*, 2013).
   a. Neumann JC, Dovey JS, Chandler GL, Carbajal L, **Amatruda JF**. Identification of a heritable model of testicular germ cell tumor in the zebrafish. Zebrafish. 2009;6(4):319-27. PMCID: 2811880.
   b. Neumann JC, Chandler GL, Damoulis VA, Fustino NJ, Lillard K, Looijenga L, Margraf L, Rakheja D, **Amatruda JF**. Mutation in the type IB bone morphogenetic protein receptor Alk6b impairs germ-cell differentiation and causes germ-cell tumors in zebrafish. Proc Natl Acad Sci U S A. 2011;108(32):13153-8. PMCID: 3156187.
   c. Fustino N, Rakheja D, Ateek CS, Neumann JC, **Amatruda JF**. Bone morphogenetic protein signalling activity distinguishes histological subsets of paediatric germ cell tumours. Int J Androl. 2011;34(4 Pt 2):e218-33.
   d. **Amatruda JF**, Ross JA, Christensen B, Fustino NJ, Chen KS, Hooten AJ, Nelson H, Kuriger JK, Rakheja D, Frazier AL, Poynter JN. DNA methylation analysis reveals distinct methylation signatures in pediatric germ cell tumors. BMC Cancer. 2013;13:313. PMCID: 3701494.

3. **Cancer genomics.** As an important complement to zebrafish genetics and clinical GCT studies, we have performed genomic characterization of childhood cancers. Through genome-wide RNA interference, we identified the Stk11/Lkb1 kinase as a novel regulator of both Wnt and Hedgehog signaling (Jacob *et al.*, 2011). We used genomic copy number analysis to identify mechanisms driving rhabdomyosarcoma, a muscle cancer (Paulson *et al.*, 2011). Recently, we used whole-exome sequencing to identify novel mutations in microRNA-processing genes in Wilms tumor, the most common childhood cancer (Rakheja *et al.*, 2014). We demonstrated the mechanism by which the mutations promote tumor formation. Finally, in a collaborative project focused on sarcoma, we participated in the identification of novel internal tandem duplications of BCOR in clear cell sarcoma of the kidney (Roy *et al.*, 2015). These studies directly identified

new candidates for targeted therapy of human cancer, and were the basis for our successful participation in the UTSW Kidney Cancer SPORE application.

a. Jacob LS, Wu X, Dodge ME, Fan CW, Kulak O, Chen B, Tang W, Wang B, **Amatruda JF**, Lum L. Genome-wide RNAi screen reveals disease-associated genes that are common to Hedgehog and Wnt signaling. Sci Signal 2011;4(157):ra4. PMCID: 3790583.
b. Paulson V, Chandler G, Rakheja D, Galindo RL, Wilson K, **Amatruda JF**, Cameron S. High-resolution array CGH identifies common mechanisms that drive embryonal rhabdomyosarcoma pathogenesis. Genes, Chromosomes Cancer. 2011;50(6):397-408.
c. Rakheja D, Chen KS, Liu Y, Shukla AA, Schmid V, Chang TC, Khokhar S, Wickiser JE, Karandikar NJ, Malter JS, Mendell JT, **Amatruda JF**. Somatic mutations in DROSHA and DICER1 impair microRNA biogenesis through distinct mechanisms in Wilms tumours. Nature Commun 2014;2:4802.
d. Roy A, Kumar V, Zorman B, Fang E, Haines KM, Doddapaneni H, Hampton OA, White S, Bavle AA, Patel NR, Eldin KW, John Hicks M, Rakheja D, Leavey PJ, Skapek SX, **Amatruda JF**, Nuchtern JG, Chintagumpala MM, Wheeler DA, Plon SE, Sumazin P, Parsons DW. Recurrent internal tandem duplications of BCOR in clear cell sarcoma of the kidney. Nat Commun. 2015 Nov 17;6:8891. doi: 10.1038/ncomms9891. PMCID: PMC4660214.

4. **Collaborative studies in germ cell tumor**. To increase the possibilities of translating basic science insights into new treatment options for germ cell tumors, I have worked with the Children's Oncology Group and national and international collaborators in a series of comprehensive clinical studies to identify factors contributing to germ cell tumor clinical outcome and patient survival. These studies set the stage for an improved combined clinical-molecular risk stratification of GCTs, now in development.
   a. Poynter JN, **Amatruda JF**, Ross JA. Trends in incidence and survival of pediatric and adolescent patients with germ cell tumors in the United States, 1975 to 2006. Cancer. 2010;116(20):4882-91.
   b. Stoneham SJ, Hale JP, Rodriguez-Galindo C, Dang H, Olson T, Murray M, **Amatruda JF**, Thornton C, Arul GS, Billmire D, Krailo M, Stark D, Covens A, Hurteau J, Stenning S, Nicholson JC, Gershenson D, Frazier AL. Adolescents and young adults with a "rare" cancer: getting past semantics to optimal care for patients with germ cell tumors. Oncologist. 2014;19(7):689-92. PMCID: 4077446.
   c. Frazier AL, Hale JP, Rodriguez-Galindo C, Dang H, Olson T, Murray MJ, **Amatruda JF**, Thornton C, Arul GS, Billmire D, Shaikh F, Pashankar F, Stoneham S, Krailo M, Nicholson JC. Revised risk classification for pediatric extracranial germ cell tumors based on 25 years of clinical trial data from the United kingdom and United States. J Clin Oncol. 2015;33(2):195-201. PMCID: 4279239.
   d. Rescorla FJ, Ross JH, Billmire DF, Dicken BJ, Villaluna D, Davis MM, Krailo M, Cullen JW, Olson TA, Egler RA, **Amatruda JF**, Rodrigues-Galindo C, Frazier AL. Surveillance after initial surgery for Stage I pediatric and adolescent boys with malignant testicular germ cell tumors: Report from the Children's Oncology Group. J Pediatr Surg. 2015 Jun;50(6):1000-3. PubMed PMID: 25812445.

Complete List of Published Work in MyBibliography:
http://www.ncbi.nlm.nih.gov/sites/myncbi/james.amatruda.1/bibliography/40062395/public/?sort=date&direction=descending

OTHER SUPPORT

1P50CA196516-01-A1 (Brugarolas) 08/01/16 - 7/31/21 0.3 calendar
NIH/NCI $1,337,017
The University of Texas Southwestern Medical Center SPORE in Kidney Cancer
Career Enhancement Program (Amatruda, PI)
This award supports the Career Development Program associated with the Kidney Cancer SPORE.
The major goal of this project is to be provided by Dr. Amatruda
Role: Program Director

1 P50 CA196516-01-A1 (Project 4) 08/01/16 – 7/31/21 1.6 calendar
NIH $1,337,017
UTSW SPORE in Kidney Cancer
Prognostic Significance and Therapeutic Potential of DROSHA Mutations in Wilms Tumor

The goal of this award is to study mechanisms of tumorigenesis by mutation of DROSHA in the developing kidney.
Role: Leader

UTHSC SA/CPRIT Subct; RFA R-16-CFSA-2 (Skapek) 06/01/16 – 05/31/21 1.2 calendar
UTHSC, San Antonio/CPRIT $450,621
Texas Pediatric Patient Derived Xenograft Facility
The major goals of this project is to oversee the collection and shipment of viable tumor biopsy and leukemia specimens for the development of patient derived xenograft models of childhood cancer and oversee the molecular characterization of those models developed through this core.
Role: Collaborator

Dana-Farber/St. Baldrick's Subcontract (Amatruda) 07/01/15 – 06/30/20 1.0 calendar
Dana-Farber Cancer Institute $176,839
Malignant Germ Cell Tumors International Consortium
The goal of this project is to bring together clinical and molecular data on malignant germ cell tumors to improve risk stratification and to identify drivers of disease progression.

RP160249 (Mendell, PI) 03/01/16 – 02/29/20 0.6 calendar
CPRIT $285,000
DIS3L2 in Childhood Wilms Tumor: Mechanism to Medicines
This grant tests the role of the DIS3L2 nuclease in Perlman Syndrome and childhood Wilms tumor, with the goal of developing new therapeutic approaches.
Role: Collaborator

Alex's Lemonade Stand Young Investigator (Chen, Fellow) 07/01/16 – 06/30/19 0 calendar
Alex's Lemonade Stand Foundation $50,000
The role of miRNA impairment in Wilms tumor formation
The goal is to define how mutations in microRNA processing genes drive Wilms tumor formation.
Role: Mentor

Alex's Lemonade Stand Innovation (Amatruda) 09/01/16 – 08/31/18 0.6 calendar
Alex's Lemonade Stand Foundation $117,224
DICER1-driven Cancers: Models, Mechanisms and Therapies
The goals are to create physiologic models of DICER1-mutant tumors in the zebrafish and to screen a 250,000-compound library to identify small molecules capable of restoring the activity of mutant DICER1.

RP120685-C1 – Core 1 (Amatruda) 08/31/12 – 08/31/18 1.2 calendar
CPRIT $158,148
C1: Central Sarcoma Processing Core
The goal of this award is to facilitate real-time identification of actionable mutations in sarcomas.
Role: Co-PI

RP120685-P3 – Project 3 (Amatruda) 08/31/12 – 08/31/18 1.2 calendar
CPRIT $337,712
P3: Functional Validation of Actionable Mutations in Sarcoma Genetic Model Systems
The goal of this grant is to use Drosophila and Zebrafish genetic models to identify and validate mutations that cooperate with oncogenic fusion proteins in the pathogenesis of sarcomas.
Role: Co-PI

ALSF Young Investigator Award (Kendall, Fellow) 07/01/15 – 06/30/17 0 calendar
Alex's Lemonade Stand Foundation $50,000
Zebrafish Modeling of PAX3-FOXO1 Driven Rhabdomyosarcoma
This is a post-doctoral fellowship for Dr. Genevieve Kendall, who will make zebrafish models of rhabdomyosarcoma.

Note: Fellow's research project is funded by both the American Association for Cancer Research and Alex's Lemonade Stand Foundation.
Role: Mentor

5 U10 CA 180884-03 (Adamson) 03/01/16 – 02/28/17 0.6 calendar
Children's Hospital of Philadelphia $10,665
COG Solid Malignancy Integrated Translational Science Center Grant
This award supports Dr. Amatruda's work on the Children's Oncology Group SM-ITSC Committee.
Role: Co-Investigator

5 U10 CA 180886-03 (Adamson) 03/01/16 – 02/28/17 0.2 calendar
Children's Hospital of Philadelphia $5,377
NIH National Clinical Trials Network (NCTN) Grant (U10CA180886)
This award supports Dr. Amatruda's work on the Children's Oncology Group Rare Tumors Committee.
Role: Subcommittee Vice-Chair

5 R21 CA187516-02 (Amatruda) 07/01/14 – 12/31/16 0.6 calendar
NIH/NCI $131,278
A novel functional genomic pipeline for target identification in sarcoma
The aims of this grant are to use a zebrafish model of Ewing's Sarcoma to functionally characterize candidate cooperating oncogenes derived from analysis of human tumor genomic analysis. A second aim is to conduct small-molecule screens to identify inhibitors of the EWS-FLI1 oncoprotein.
Role: PI

REACH Award (Amatruda) 12/31/13 – 12/30/16 0.84 calendar
Alex's Lemonade Stand Foundation $55,973 **(NCE)**
B-lapachone as a novel targeted therapy for ATRT and other pediatric cancers
The goal of this grant is to evaluate B-lapachone as a treatment for solid tumors of children
Role: PI

Ewing Sarcoma Research Program (Amatruda) 12/01/15 – 11/30/17 0.4 calendar
1 Million for Anna Foundation $125,000
Determining the cell of origin of Ewing Sarcoma in a zebrafish genetic model
The goals of this project are to discover, validate and institute new treatments for Ewing Sarcoma that are more effective and less toxic than current therapies. Dr. Amatruda will use zebrafish genetic models to identify the cell of origin of Ewing sarcoma, a key step to better understanding and treatment of the disease.

Gregory J. Aune, MD, PhD, FAAP
Stephanie Edlund Distinguished Professor in Pediatric Cancer Research

Greehey Children's Cancer Research Institute
University of Texas Health Science Center San Antonio
Office Phone: 210-562-9154
Office Fax: 210-562-9135
aune@uthscsa.edu

Employment Status:
November 2010- present

- Assistant Professor, Tenure-track, Department of Pediatrics, Division of Hematology-Oncology. University of Texas Health Science Center San Antonio. Greehey Children's Cancer Research Institute

Education:

- Pediatrics Hematology/Oncology Fellowship (2008-2010) University of Texas Health Science Center San Antonio, Christus Santa Rosa Children's Hospital, Greehey Children's Cancer Research Institute
- Pediatrics Residency (2006-08) Johns Hopkins Hospital
- Pediatrics Internship (2005-06) Johns Hopkins Hospital
- MD (2005) University of Texas-Houston Medical School
- PhD (2005) University of Texas-Houston Graduate School of Biomedical Sciences
    - PhD dissertation research conducted at the National Cancer Institute, Bethesda, MD
    - Immediate Advisor: Yves Pommier, MD, PhD, Chief, Laboratory of Molecular Pharmacology
    - PhD Dissertation: *RNA Polymerase II Large Subunit Degradation Induced by Ecteinascidin 743: Molecular Characterization and Subsequent Rational Investigation of Antitumor Mechanisms in Cancers with Clinical Response*
- Pacific Lutheran University (PLU), Tacoma, WA (1993-1997), Summa Cum Laude
    - B.S. Chemistry (Biochemistry emphasis)
    - B.A. Biology
- Colfax High School, Colfax, WA (1988-1993) Valedictorian

Academic Distinctions:

Faculty:

- Stephanie Edlund Distinguished Professor in Pediatric Cancer Research (2015)
- St. Baldrick's Scholar. St. Baldrick's Foundation (2014)
- Hyundai Hope Scholar. Hyundai Hope on Wheels Foundation (2014)
- KL2 Scholar. Institute for Integration of Medicine and Science. Clinical and Translational Science Award. University of Texas Health Science Center at San Antonio (2012-2014)
- Winner. Cancer Therapy and Research Center. Images in Cancer Research Competition (2012)

Residency:

- Morbidity and Mortality Committee. Johns Hopkins Hospital Department of Pediatrics 2007-2008

Graduate:

- American Legion Auxiliary Fellowship in Cancer Research (2004)
- University of Texas-Houston Graduate School of Biomedical Sciences Travel Award (2004)
- Sowell-Huggins Endowed Scholar in Cancer Research (2004)
- RW Butcher Achievement Award (2004)
- American Legion Auxiliary Fellowship in Cancer Research (2003)
- Cancer Research Training Award (CRTA) Fellowship. National Cancer Institute, Center for Cancer Research, Laboratory of Molecular Pharmacology (2001-2005)
- Torrison Scholarship -- awarded to four first year medical students in the U.S. with aspirations to enter biomedical research (1997)

Undergraduate:

- Finalist University of Texas Southwestern Medical Scientist Training Program (1997)
- Judge Bertil E. Johnson Pre-medicine Scholarship -- awarded to most outstanding junior pre-medicine major at PLU (1996)
- Barry M. Goldwater Scholarship- PLU nominee, National competition (1995 and 1996)
- PLU Presidential Scholar (1993-97)
- PLU Alumni Merit Scholar (1993-97)
- National Science Scholar Semifinalist (1993)

Secondary:

- Colfax High School Valedictorian (1993)
- Washington Scholar - recipient of full 4-year tuition to Public University of choice or matching amount to Private University -- Statewide competition (1993)

Science Related Employment History:

- University of Texas Health Science Center San Antonio, Greehey Children's Cancer Research Institute. Principal Investigator (2010-present)
- University of Texas Health Science Center San Antonio, Greehey Children's Cancer Research Institute. Mentor: Gail E. Tomlinson, MD, PhD (2008-2010)
- Cancer Research Training Award (CRTA) Fellow- National Cancer Institute, Center for Cancer Research, Laboratory of Molecular Pharmacology. Advisor: Yves Pommier, MD, PhD (2001-2005)
- Summer Research Fellowship Program- National Cancer Institute, Yves Pommier, MD, PhD, Chief, NCI Laboratory of Molecular Pharmacology (Summer 2000)
- Graduate Student Lab Rotation – UT MD Anderson Cancer Center, David MacConkey, Ph.D. (Summer 1998)
- Graduate Student Lab Rotation – UT MD Anderson Cancer Center, William Plunkett, Ph.D. (Summer 1997)
- PLU- Organic Chemistry teaching assistant (1995-97), General Biology teaching assistant (1994), Microbiology and immunology research lab assistant (1994-97)

Primary Areas of Expertise

Pre-clinical Models of Late Health Effects in Childhood Cancer Survivors

There is growing evidence in the medical literature that clearly quantifies the frequency, scope, and severity of late health effects in long-term survivors of childhood cancer. The vast majority of these findings have been obtained from well-designed retrospective epidemiologic studies in large cohorts of childhood cancer survivors. While these studies have clearly established links between treatment exposures and the development of severe disease decades later, little is known about the underlying molecular and cellular mechanisms that drive the pathologic progression of organ dysfunction following early-life chemotherapy and radiation exposure. With this is mind, my laboratory has focused on developing relevant pre-clinical models that recapitulate these clinical scenarios and facilitate the scientific investigation of cellular and molecular mechanisms in the shortened lifespan of mice. We have successfully established a pediatric mouse model of anthracycline-induced cardiac toxicity that can be used to address relevant clinical questions and obtained competitive peer-reviewed funding to carry out these studies.

Analysis of Cardiac Function in Mice by Echocardiography

I am the director of the Greehey Children's Cancer Research Institute Shared Resource for Cardiac Function Analysis. To date, we have completed over 3,000 echocardiographic assessments of mice ranging in age from two weeks to adult. We are generating data for a variety of projects and scientists through numerous collaborative projects. These include evaluating diabetic cardiomyopathy, doxorubicin-induced cardiotoxicity in NELF-deficient mice, a mouse model of Kawasaki's syndrome, a mouse model of bronchopulmonary dysplasia, and a mouse model of streptococcal pneumonia-induced cardiac injury.

Clinical Care of Long-term Survivors of Childhood Cancer

For the past six years, I have provided comprehensive clinical care for long-term survivors of childhood cancer in the South Texas Cancer Survivorship Program at University Hospital in San Antonio, TX. During this time, I have worked to expand our local cohort to approximately 350 long-term survivors of pediatric cancer. In addition, I led successful completion of a pilot study evaluating the sensitivity of cardiac MRI as a tool to detect subclinical heart damage in anthracycline-exposed patients (funded by a $50,000 CTSA pilot grant). On a national level, I am intimately involved in collaborative efforts to establish research networks to reduce cardiac health morbidities in survivors. These include the COG Cardiometabolic Task Force and the NCI Community Oncology Cardiotoxicity Task Force. Finally, I have participated in updating the COG Late Effects evidence-based follow-up guidelines for cardiac and pulmonary organ systems.

Pediatric Cancer Advocacy and Oncology Science Policy

I am a national leader in childhood cancer advocacy efforts. In San Antonio, I am a leader in local fundraising and awareness efforts. Since 2010, I have spearheaded efforts by the St. Baldrick's Foundation and For the Kids Dance Marathon at the University of Texas San Antonio that have raised over $830,000 for childhood cancer patients and research efforts. Most recently, my appointment to the National Cancer Institute Council of Research Advocates (NCRA) was announced by NCI Director Dr. Harold Varmus at a White House briefing on childhood cancer (weblink). In May 2015, I was invited to speak at the 68th World Health Assembly in Geneva, Switzerland and urge the World Health Organization to declare childhood cancer a worldwide problem (weblink). I am a science policy advisor for the National Coalition of Cancer Survivorship, the COG representative for the Alliance for Childhood Cancer, and a member of the Scientific Advisory Board for the Canines-N-Kids Foundation. Finally, I am a standing member of the St. Baldrick's Foundation national advocacy committee and serve on the Board of Directors for the American Childhood Cancer Organization.

Publications:

1. **Aune GJ**, Furuta T, and Pommier Y. Ecteinascidin 743: A novel anticancer drug with a unique mechanism of action. *Anti-Cancer Drugs* 2002; 13: 1-12.
2. Furuta T, Ueda T, **Aune GJ**, Sarasin A, Kraemer KH, and Pommier Y. Transcription Coupled-Nucleotide Excision Repair as a Determinant of Cisplatin Sensitivity of Human Cells. Cancer Res 2002 Sep 1;62(17):4899-90.
3. Furuta T, Takemura H, Liao Z, **Aune GJ**, Redon C, Sedelnikova O, Pilch D, Rogaku E, Celeste A, Tang Chen H, Nussenzweig A, Aladjem M, Bonner W, and Pommier Y. Phosphorylation of Histone H2AX and activation of Mre11, Rad50, and Nbs1 in Response to Replication-Dependent DNA-Double-strand Breaks Induced by Mammalian DNA Topoisomerase I cleavage complexes. J Biol Chem. 2003 May 30;278(22):20303-12.
4. Furuta T, Hayward RL, Meng LH, Takemura H, **Aune GJ**, Bommer WM, Aladjem, Kohn KW, and Pommier Y. p21CKDN1A allows the repair of replication-mediated DNA double-strand breaks induced by topoisomerase I and inactivated by the checkpoint kinase inhibitor 7-hydroxystaurosporine. Oncogene. 2006 May 11; 25 (20). 2839-49.
5. **Aune, GJ**. Wilms Tumor. Pediatrics in Review. 2008 April. 29(4):142-3.
6. **Aune, GJ**. Fluids and Electrolytes. Harriet Lane Handbook. pp. 301-326.
7. **Aune GJ,** Kazutaka T, Sordet O, Guirouilh-Barbat J, Antony S, Bohr W, and Pommier Y. Von Hippel-Lindau and Transcription-coupled Nucleotide Excision Repair Dependent Degradation of RNA Polymerase II in Response to Trabectedin. *Clinical Cancer Research*. 2008 Oct 15;14(20):6449-55.
8. Policarpio-Nicolas ML, Valente PT, **Aune GJ**, Higgins RA. Isolated vaginal myeloid sarcoma in a 16-year-old girl. *Ann Diagn Pathol*. 2011 Jun 7.
9. Patterson NL, Rugmani PI, Li Y, Andrews TG, **Aune GJ**, Lange RA, and Lindsey ML. Using Proteomics to Uncover Matrix Metalloproteinase Roles in Extracellular Matrix Remodeling. Proteomics Clinical Applications. 2013.
10. Andrews, TG, Lindsey, ML, Lange, RA, and **Aune, GJ.** Cardiac Assessment In Pediatric Mice: Strain Analysis as a Diagnostic Measurement. Echocardiography. September 2013.
11. Pan, H, Qin, K, Guo, Z, Ma, Y, April C, Gao, X, Andrews, TG, Bokov, A, Zhang, J, Chen, Y, Weintraub, S, Fan, J, Wang, D, Hu, Y, **Aune, GJ,** Lindsey, ML, and Li R. RNA Polymerase II Pausing Factor NELF Controls Energy Homeostasis in Cardiomyocytes. Cell Reports. April 10, 2014.
12. Lindsey, ML, Lange, RA, Parsons H, Andrews, TG, and **Aune GJ**. The Tell-Tale Heart: Molecular and Cellular Responses to Childhood Anthracycline Exposure. American Journal of Physiology: Heart and Circulatory Physiology. September 2014.

Published Abstracts:

1. Furuta F, Ueda T, **Aune G**, Kohlhagen G, Kraemer K, and Pommier Y. Transcription-coupled nucleotide excision repair as a determinant of cisplatin activity. *Proceedings of the American Association for Cancer Research.* 43: 2002.
2. Furuta F, Takemura H, Hayward L, **Aune G,** Liao Z, Aladjem M, Bonner W, and Pommier Y. The cell cycle checkpoint abrogator 7-hydroxystaurosporin (UCN-01), enhances phosphorylation of histone H2AX and abrogates p21 induction in response to camptothecin. *Proceedings of the American Association for Cancer Research.* 44: 2003.
3. Sordet O, **Aune G**, and Pommier Y. The topoisomerase I inhibitor camptothecin induces the phosphorylation of RNA polymerase II subunit. *Proceedings of the American Association for Cancer Research.* 44: 2003.
4. **Aune G,** Sordet O, Furuta T, Antony S, Bohr V and Pommier Y. Modifications in RNA Polymerase II induced by ecteinasidin 743 are associated with the poisoning of transcription-coupled nucleotide excision repair. *Proceedings of the American Association for Cancer Research.* 44: 2003.

5. **Aune G,** Vasselli J, Linehan W, and Pommier Y. Sensitivity to the Novel Anticancer Agent Ecteinascidin 743 Requires VHL-dependent Degradation of RNA Polymerase II Large Subunit. *Proceedings of the American Association for Cancer Research.* 45: 2004.
6. **Aune G,** Sordet O, Bohr V, and Pommier Y. RNA Polymerase II Degradation Induced by Ecteinascidin 743 is Dependent on Transcription-coupled Nucleotide Excision Repair and Predicts Drug Sensitivity. *Proceedings of the American Association for Cancer Research*. 45: 2004
7. **Aune GJ**, Rao VA, AchiriMofor ND, Merchant MD, Merino ME, Mackall CL, and Pommier YG. EWS-Fli1 Fusion Protein Expression in Ewing's Sarcoma Tumors is Inhibited by Ecteinascidin 743. *Pediatric Academic Societies' Annual Meeting.* 2005.
8. **Aune GJ,** Dickson, F, and Tomlinson, GE. Retrospective Review of Cardiovascular Health in Pediatric Cancer Survivors in South Texas. *Cancer Prevention Research Institute of Texas Annual Meeting. 2011.*
9. **Aune GJ,** Dickson, F, and Tomlinson, GE. Retrospective Review of Cardiovascular Health in Pediatric Cancer Survivors in South Texas. *12th International Conference on Long-Term Complications of Treatment of Children and Adolescents for Cancer*. 2012
10. **Aune GJ,** Andrews T, and Lindsey ML. Analysis of Cardiac Function in a Pediatric Mouse Model of Doxorubicin-Induced Cardiotoxicity Using Echocardiographic Strain Imaging. *Cancer and the Heart.* 2012.
11. Andrews T, Lindsey ML, and **Aune GJ** Analysis of Cardiac Function in a Pediatric Mouse Model of Doxorubicin-Induced Cardiotoxicity Using Echocardiographic Strain Imaging. *National Translational Sciences Meeting.* 2013.
12. Andrews TG, Lindsey ML, Lange RA, and **Aune GJ**. Pediatric Mouse Model of Anthracycline-Induced Cardiotoxicity. *13th International Conference on Long-Term Complications of Treatment of Children and Adolescents for Cancer*. 2013
13. Lindsey, ML, Lange, RA, Andrews, TG, and **Aune GJ.** Pediatric Mouse Model of Anthracycline-induced Cardiac Toxicity. *Keystone Symposia- Fibrosis: From Bench to Bedside*. 2014.
14. Andrews TG, Lindsey ML, Lange RA, and **Aune GJ**. Pediatric Mouse Model of Anthracycline-Induced Cardiotoxicity. *National Translational Sciences Meeting.* 2014.

Invited Scientific Presentations:

1. **Aune G,** Vasselli J, Linehan W, and Pommier Y. Sensitivity to the Novel Anticancer Agent Ecteinascidin 743 Requires VHL-dependent Degradation of RNA Polymerase II Large Subunit. *2004 American Association for Cancer Research National Meeting.* Experimental and Molecular Therapeutics Minisymposium 39.
2. **Aune G,** Sordet O, Bohr V, and Pommier Y. RNA Polymerase II Degradation Induced by Ecteinascidin 743 is Dependent on Transcription-coupled Nucleotide Excision Repair and Predicts Drug Sensitivity. *2004 American Association for Cancer Research National Meeting*. Experimental and Molecular Therapeutics Minisymposium 13.
3. **Aune GJ,** Dickson, F, and Tomlinson, GE. Retrospective Review of Cardiovascular Health in Pediatric Cancer Survivors in South Texas. Cancer Prevention Research Institute of Texas Annual Meeting. (2011)
4. San Antonio Cardiovascular Proteomics Center. Invited Speaker. *Analysis of Cardiac Function in a Pediatric Mouse Model of Doxorubicin-Induced Cardiotoxicity Using Echocardiographic Strain Imaging*. (August 2012)
5. Department of Pediatrics Grand Rounds. University of Texas Health Science Center at San Antonio. Invited Speaker. *The Tell-Tale Heart: Late Effects in Pediatric Cancer Survivors*. (September 2012)
6. Cancer Prevention and Population Sciences Program. Cancer Therapy and Research Center. University of Texas Health Science Center at San Antonio. Invited Speaker. *Cancer Therapy in the 21st Century: The Tell-Tale Heart.* (November 2012)

7. Greehey Children's Cancer Research Institute Annual Retreat. University of Texas Health Science Center at San Antonio. Invited Speaker. *Pediatric Mouse Model of Anthracycline Cardiac Toxicity.* (February 2013)
8. Trinity University Department of Biology Seminar. Series. Invited Speaker. *Pediatric Mouse Model of Anthracycline Cardiac Toxicity.* (April 2013)
9. Cancer Therapy and Research Center Public Forum. University of Texas Health Science Center at San Antonio. Invited Speaker. *Pediatric Cancer Survivorship in the 21st Century.* (May 2013)
10. University of Texas Southwestern Medical School. Division of Cardiology Seminar Series. Invited Speaker. *Pediatric Mouse Model of Anthracycline-Induced Cardiac Toxicity.* (July 2013)
11. Voelcker Scholars Summer Program Seminar Series. Invited Speaker. *Pediatric Mouse Model of Anthracycline Cardiac Toxicity.* (July 2013)
12. Children's Oncology Group Cardiometabolic Task Force. Invited Speaker. *Preclinical Models to Study Anthracycline-Induced Cardiotoxicity.* (January 2014)
13. Center for Pregnancy and Newborn Research Seminar Series. University of Texas Health Science Center at San Antonio. Invited Speaker. *Modeling Anthracycline-induced Cardiac Injury in Young Mice.* (April 2014)
14. Department of Pediatrics Grand Rounds. University of Texas Health Science Center at San Antonio. Invited Speaker. *Deviant Chromosome Engineering by miRNAs.* (July 2014)
15. Coalition Against Childhood Cancer Webinar. *Incorporating Genomics into Pediatric Cancer Trials: A Summary of the NCI Workshop on Pediatric Cancer Genomics for Advocates*. (March 2015)
16. CPRIT's Panel Discussion on "Cancer: The Emperor of All Maladies" CPRIT. Austin, TX (March 2015)
17. 68th World Health Assembly, Non-communicable diseases. *Putting the Fight against Childhood Cancer on the Global Health Agenda*. World Health Organization, Geneva, Switzerland (Invited Speaker, May 2015)
18. Childhood Cancer Summit, United States Congressional Childhood Cancer Caucus, Alliance for Childhood Cancer, Washington, DC (Invited Speaker, June 2015)
19. Pediatric Cancer Action Days, Alliance for Childhood Cancer, Washington, DC (Keynote Speaker, June 2015)
20. Coalition Against Childhood Cancer Annual Meeting. *The Lifelong Burden of Successful Childhood Cancer Therapy*. Washington, DC (Invited Speaker, June 2015)
21. San Antonio Military Medical Center Pediatric Grand Rounds. *The Lifelong Burden of Successful Childhood Cancer Therapy*. San Antonio, TX (Invited Speaker, October 2015)
22. Gerontological Society of America Annual Meeting. *The Lifelong Burden of Successful Childhood Cancer Therapy*. Orlando, FL (Invited Speaker, November 2015)
23. NCCS Cancer Policy Roundtable. *The Empowered Patient: How Do Patients Define and Receive the Care They Value*. Washington, DC (April 2016)
24. 7th Annual Congressional Childhood Cancer Caucus Summit. Invited Survivor Panelist. September 2016.
25. Chasing Cancer. Washington Post Live Forum. Invited Panelist. December 2016.

Invited Speeches:

1. Thomas Jefferson High School for Science and Technology Health Sciences Club: Guest Lecturer: *Careers in Biomedical Sciences.* (2002)
2. American Legion Auxiliary Fellowship in Cancer Research: Awards Luncheon (2003)
3. Sowell-Huggins Scholarship in Cancer Research: Awards Luncheon (2004)
4. American Legion Auxiliary Fellowship in Cancer Research: Awards Luncheon (2004)
5. Johns Hopkins Hospital Department of Pediatrics Grand Rounds Warm-up. *Pediatric Cancer Therapy: The Gift that Keeps on Giving.* (2006)
6. Invited Commencement Speaker- University Texas San Antonio Center for Professional Excellence (2010)

7. University of Texas San Antonio Health Science Center Department of Pediatrics Grand Rounds. *Post Transplant Lymphoproliferative Disorder*. (2010)
8. South Texas Cancer Survivorship Program Outreach Day at San Antonio Seaworld. Invited Speaker. *Pediatric Cancer Therapy: The Gift that Keeps on Giving* (2010)
9. Greehey Children's Cancer Research Institute -- AFLAC-Macy's Holiday Fund Raising Check Presentation. Invited Speaker. (2010)
10. For the Kids Dance Marathon at University of Texas San Antonio -- Student Leader Kickoff. Invited Speaker and Organizer of Motivational Program. (2010)
11. Greehey Children's Cancer Research Institute – Hyundai Hope on Wheels Check Presentation. Recipient and Invited Speaker. (2010)
12. For the Kids Dance Marathon at University of Texas San Antonio – Dance Marathon Keynote Speaker for FTK Family Hour. (2010)
13. For the Kids Dance Marathon at University of Texas San Antonio -- Student Leader Kickoff. Invited Speaker. (2011)
14. San Antonio Cardiovascular Proteomics Center. Invited Speaker. *Creating and Implementing a Junior Faculty Career Plan.* (March 2012)
15. San Antonio Youth Leadership Program. Invited Speaker and Lab Tour Guide. *Careers in Biomedical Science*. (June 2014)
16. Pediatric Translational Working Group. Institute for Drug Development. University of Texas Health Science Center at San Antonio. *Preclinical Evaluation of Chemotherapy-induced Cardiotoxicity.* (August 2014)
17. University Hospital. Greehey Children's Cancer Research Institute – Hyundai Hope on Wheels Check Presentation. Recipient and Invited Speaker. (September 2014)

Scientific Meetings and Poster Presentations:

Faculty:

1. Children's Oncology Group Fall Meeting. (September 2011). **Aune GJ,** Dickson, F, and Tomlinson, GE. Retrospective Review of Cardiovascular Health in Pediatric Cancer Survivors in South Texas.
2. Medical Student Research Day. (October 2011). **Aune GJ,** Dickson, F, and Tomlinson, GE. Retrospective Review of Cardiovascular Health in Pediatric Cancer Survivors in South Texas.
3. Cancer Prevention Research Institute of Texas Annual Meeting. (November 2011). **Aune GJ,** Dickson, F, and Tomlinson, GE. Retrospective Review of Cardiovascular Health in Pediatric Cancer Survivors in South Texas.
4. Frontiers in Translational Science. (February 2012). **Aune GJ,** Dickson, F, and Tomlinson, GE. Retrospective Review of Cardiovascular Health in Pediatric Cancer Survivors in South Texas.
5. 12th International Conference on Long-Term Complications of Treatment of Children and Adolescents for Cancer. (June 2012) **Aune GJ,** Dickson, F, and Tomlinson, GE. Retrospective Review of Cardiovascular Health in Pediatric Cancer Survivors in South Texas.
6. Cancer Prevention Research Institute of Texas Annual Meeting. (October 2012). **Aune GJ,** Andrews T, and Lindsey ML. Analysis of Cardiac Function in a Pediatric Mouse Model of Doxorubicin-Induced Cardiotoxicity Using Echocardiographic Strain Imaging.
7. Cancer and the Heart. (November 2012). **Aune GJ,** Andrews T, and Lindsey ML. Analysis of Cardiac Function in a Pediatric Mouse Model of Doxorubicin-Induced Cardiotoxicity Using Echocardiographic Strain Imaging.
8. Medical Student Research Day. (November 2012). Lam JP, Frausto-Garcia E, Assanasen C, and **Aune GJ.** Retrospective Chart Review of Antimetabolite Toxicities in Pediatric Cancer Patients. **Lam- 2nd Place in Poster Competition**
9. Frontiers in Translational Science. (February 2013). Andrews T, Lindsey ML, and **Aune GJ.** Pediatric Mouse Model of Anthracycline-induced Cardiac Toxicity

10. Frontiers in Translational Science. (February 2013). Andrews T, Lindsey ML, and **Aune GJ.** Cardiac Assessment in Pediatric Mice by Strain Analysis.
11. National Translational Sciences Meeting. (April 2013). Analysis of Cardiac Function in a Pediatric Mouse Model of Doxorubicin-Induced Cardiotoxicity Using Echocardiographic Strain Imaging.
12. Department of Pediatrics Research Day. (May 2013). **Aune GJ,** Andrews T, and Lindsey ML. Analysis of Cardiac Function in a Pediatric Mouse Model of Doxorubicin-Induced Cardiotoxicity Using Echocardiographic Strain Imaging.
13. Department of Pediatrics Research Day. (May 2013). Andrews T, Lindsey ML, and **Aune GJ.** Cardiac Assessment in Pediatric Mice by Strain Analysis.
14. Department of Pediatrics Research Day. (May 2013). Lam JP, Frausto-Garcia E, Assanasen C, and **Aune GJ.** Retrospective Chart Review of Antimetabolite Toxicities in Pediatric Cancer Patients.
15. 13th International Conference on Long-Term Complications of Treatment of Children and Adolescents for Cancer. (June 2013) Andrews TG, Lindsey ML, Lange RA, and **Aune GJ**. Pediatric Mouse Model of Anthracycline-Induced Cardiotoxicity.
16. Texas Regional CTSA Consortium Annual Meeting. (November 2013) Andrews TG, Lindsey ML, Lange RA, and **Aune GJ**. Pediatric Mouse Model of Anthracycline-Induced Cardiotoxicity. **Aune-2nd Place in Poster Competition**
17. Keystone Symposia- Fibrosis: From Bench to Bedside. (March 2014) Lindsey, ML, Lange, RA, Andrews, TG, and **Aune GJ. A** Pediatric Mouse Model of Anthracycline-induced Cardiac Toxicity.
18. National Translational Sciences Meeting. (April 2014). Andrews TG, Lindsey ML, Lange RA, and **Aune GJ**. Pediatric Mouse Model of Anthracycline-Induced Cardiotoxicity.
19. Frontiers in Translational Science. (April 2014). Lindsey, ML, Lange, RA, Andrews, TG, and **Aune GJ.** A Pediatric Mouse Model of Anthracycline-induced Cardiac Toxicity.
20. Frontiers in Translational Science. (April 2014). Andrews TG and **Aune GJ.** Greehey Shared Resource for Cardiac Function Assessment.
21. Department of Pediatrics Research Day. (May 2014). Lindsey, ML, Lange, RA, Andrews, TG, and **Aune GJ.** A Pediatric Mouse Model of Anthracycline-induced Cardiac Toxicity.
22. Department of Pediatrics Research Day. (May 2014). Greehey Shared Resource for Cardiac Function Assessment.

Graduate:

- Pediatric Academic Societies' Annual Meeting (May 2005). **Aune GJ**, Rao VA, AchiriMofor ND, Merchant MD, Merino ME, Mackall CL, Pommier YG. EWS-Fli1 Fusion Protein Expression in Ewing's Sarcoma Tumors is Inhibited by Ecteinascidin 743.
- NIH Young Investigators Retreat (March 2004). **Aune GJ**, Vasselli JR, Linehan WM, Pommier YG. VHL-dependent Degradation of RNA Polymerase II Large Subunit Enhances Sensitivity to the Novel Anticancer Agent Ecteinascidin 743.
- NIH Research Festival (October 2003): **Aune G**, Sordet O, Antony S, Bohr V, Pommier Y. Exposure to the Novel Anticancer Agent Ecteinascidin 743 Causes TC-NER Dependent Degradation of RNA Polymerase II.
- NIH Young Investigators Retreat (February 2003) **Aune GJ,** Sordet O, Furuta T, Antony S, Bohr VA, Pommier Y. Modifications in RNA Polymerase II Induced by Ecteinascidin-743 are Associated With the Poisoning of Transcription-coupled Nucleotide Excision Repair.
- NIH Research Festival (October 2002): A**une G**, Sordet O, Pommier Y. Exposure to the Novel Anticancer Agent Ecteinascidin 743 Causes Degradation of RNA Polymerase II.
- National MD, PhD Student Conference (July 2002): **Aune G**, Pommier Y, Exposure to the Novel Anticancer Agent Ecteinascidin 743 Causes Degradation of RNA Polymerase II.

Undergraduate:

- PLU Chemistry Department Senior Seminar (March 1997)

"Further Characterization and Purification of Mycoplasma protein p95-105"
- Murdock Foundation Research Symposium (November 1996- poster presentation)
  "Further Characterization of Mycoplasma protein p95-105"
- Murdock Scholars Summer Research Meetings (August 1996)
  "Further Characterization of Mycoplasma protein p95-105
- PLU Natural Sciences Festival (Presenter -- 1995)
  Biology: "Tuberculosis and the Luciferase Reporter System"
  Chemistry: "Spectral Analysis of Chemical and Enzymatic Reduction of Vanillin

Mentorship, Teaching, and Educational Activities:
- Mentor. The University of Texas Health Science Center at San Antonio MD/PhD Program Mentee: Brian Iskra. (UTHSCSA Medical School Class of 2021)
- Mentor. The University of Texas Health Science Center at San Antonio MD/PhD Program Mentee: Trevi Mancilla. (UTHSCSA Medical School Class of 2020)
- For the Kids Dance Marathon at University of Texas San Antonio. Faculty Advisor 2010-present.
- For the Kids Dance Marathon at UTSA and Fourth Circle Fund. Oncology Coordinator. (2010-present)
- Faculty Leader. The University of Texas Health Science Center at San Antonio MD/PhD Program Veritas Orange Mentorship Group. 2012 to present.
- Member. The University of Texas Health Science Center at San Antonio MD/PhD Program Steering Committee. 2013-present
- Member. The University of Texas Health Science Center at San Antonio MD/PhD Program Admissions Committee. 2013-present
- Director. Department of Pediatrics, Division of Hematology-Oncology Journal Club. 2011-16.
- Member. Department of Pediatrics, Division of Hematology-Oncology Fellowship Admissions Committee. 2011-present.
- Member. Greehey Children's Cancer Research Institute Equipment Committee. 2013-present.
- Mentor. The University of Texas Health Science Center at San Antonio Medical Student Research Program. Mentee: Dia Hazra (UTHSCSA Medical School Class of 2017)
- Mentor. The University of Texas Health Science Center at San Antonio Voelcker Summer Scholars Program. Mentee: Bryan Martinez (Thomas Edison High School Class of 2017)
- Mentor. The University of Texas Health Science Center at San Antonio Medical School Longitudinal Preceptorship Program. Mentee: John Demis (UTHSCSA Medical School Class of 2017)
- Mentor. The University of Texas Health Science Center at San Antonio Medical School Longitudinal Preceptorship Program. Mentee: Erin Foster (UTHSCSA Medical School Class of 2016)
- Mentor. The University of Texas Health Science Center at San Antonio Medical Student Summer Research Program. Mentee: Phillip Lam (UTHSCSA Medical School Class of 2015)
- Mentor. Greehey Children's Cancer Research Institute Undergraduate Summer Research Program. Mentee: Macartney Welborn. (Southern Methodist University Class of 2015)
- Mentor. The University of Texas Health Science Center at San Antonio Medical Student Summer Research Program. Mentee: Forrestine Dickson (UTHSCSA Medical School Class of 2014)
- Co-chair. St. Baldrick's San Antonio Committee. 2012-present.
- Faculty presenter and lab tour guide for the University of Texas Health Science Center at San Antonio Development Office Estate Planners Forum. 2012.
- Grant Seekers 2.0. Participant. 2013-present
- Grant Writing with New Investigators. Participant and peer reviewer. 2011-present
- University of Texas Health Science Center San Antonio Medical Student Research Day. Poster Session Judge. 2011

- Faculty Recruitment Interviewer: Kyle Orwig, PhD. Candidate for UTHSCA Stem Cell Endowed Chair. 2011.
- Seattle Children's Hospital: Pediatric Cancer Male Infertility Project. *Patient Educational Video Production*. Keynote Interviewee and Participant. 2011. http://www.youtube.com/watch?v=WDw_EdENA2Q
- University of Texas Health Science Center San Antonio, Department of Pediatric Hematology/Oncology. Fellows Lecture Series. *Acute Myelogenous Leukemia.* (2011-present)
- University of Texas Health Science Center San Antonio, Department of Pediatric Hematology/Oncology. Fellows Lecture Series. *Bone Marrow Failure Syndromes.* (2011-present)
- University of Texas Health Science Center San Antonio, Department of Pediatric Hematology/Oncology. Fellows Lecture Series. *Biostatistics.* (2011-present)
- University of Texas Health Science Center San Antonio, Department of Pediatric Hematology/Oncology. Resident Lecture Series. *Abdominal Tumors.* (2009-2011)
- University of Texas Health Science Center San Antonio, Department of Pediatric Hematology/Oncology. Resident Lecture Series. *Late Effects of Pediatric Cancer Therapy* (2009-present)
- Invited Reviewer *Molecular Cancer Therapeutics* (2005-2008)
- Invited Reviewer, *Cancer Research* (2005-2008)
- Invited Reviewer *Clinical Cancer Research* (2005-2008)
- Invited Reviewer (supervised by PhD adviser), *Clinical Cancer Research* (2004)
- Invited Reviewer (supervised by PhD adviser), *Oncology Research Anti-cancer Drug Design* (2004)
- Howard Hughes Medical Institute-NIH Montgomery County Internship Program. Mentor: 2003-04. Student, Nerg AchiriMofor

Professional Service:

2011-	Ad Hoc Reviewer: Circulation, Physiologic Genomics, JOVE, American Journal of Physiology, Comprehensive Physiology, Journal of Molecular and Cellular Cardiology, Journal of the American Heart Association, Journal of Adolescent and Young Adult Oncology
2012-	American Heart Association Basic Cardiovascular Sciences Study Section
2012	Judge. Department of Medicine Research Day
2013-	Children's Oncology Group Cardiometabolic Task Force: Founding member
2013-	NCI Community Cardiotoxicity Task Force: Founding member
2013-	Director, Greehey Shared Resource for Cardiac Function Assessment (Core mouse echocardiography facility for the GCCRI and main medical school campus)
2013-14	Judge. Department of Pediatrics Research Day
2014-	UTHSCSA MD/PhD Program Admissions Committee
2014-	UTHSCSA MD/PhD Program Steering Committee
2014-	Department of Pediatrics Strategic Planning Committee. Member. Research and Compensation Subcommittees
2014-	Reviewer, UTHSCA/CTSA/IIMS KL2 Scholar Review Panel
2014-	Member, NCI Council of Research Advocates. Appointed by NCI Director, Dr. Harold Varmus
2015-	Cancer Policy Advisor. National Coalition for Cancer Survivorship.
2015-	Member, American Childhood Cancer Organization Board of Directors
2015-	Member, St. Baldrick's Foundation National Advocacy Committee
2016-	Director, UTHSCSA MD/PhD Program Bench-to-Bedside Seminar Series
2016-	Member, Canines-N-Kids Scientific Advisory Board
2016-	COG Representative, Alliance for Childhood Cancer

2016- Member, Cancer Prevention and Research Institute of Texas Advisory Committee on Childhood Cancer

Media Exposure:

- News Brief. **Health Science Center News**. *HSC Receives $50,000 from St. Baldrick's Foundation.* January 2012.
- Blog Post. **The FTK Blog.** *THON Sets the Tone for 2012 FTK Dance Marathon at UTSA.* February 29, 2012. http://ftkdmatutsa.wordpress.com/tag/greg-aune/
- Interview. **Texas Public Radio**. For the Report. "*Adult Stem Cell Regulations Approved by the Texas Medical Board.*" (April 2012)
- Panel Participant. **KABB Fox 29 San Antonio**. *Focus on South Texas: Cancer Survivorship*. June 2012
- Feature Article. **Pacific Lutheran University Scene Magazine**. *Late-night Lesson Leads to Career Studying Chemotherapy*. Spring 2012. http://issuu.com/plu-archives/docs/2011-2012_v.42_no.1-3/122
- Feature Article. **Mission Magazine**. University of Texas Health Science Center at San Antonio. *Taking it to heart: Researcher felt cancer firsthand.* Fall 2013. http://uthscsa.edu/mission/article.asp?id=874
- Feature Story. **St. Baldrick's Foundation Blog**. *Working with Heart: St. Baldrick's Researcher Survived Childhood Cancer as a Teen.* January 28, 2014. http://www.stbaldricks.org/blog/post/working-with-heart-st-baldricks-researcher-survived-childhood-cancer-as-a-teen/
- Feature Story. **St. Baldrick's Foundation Blog**. *7 Steps to Building a St. Baldrick's Event from a Veteran VEO.* February 24, 2014. http://www.stbaldricks.org/blog/post/7-steps-to-building-a-st-baldricks-event-from-a-veteran-veo/
- Featured Investigator. **St. Baldrick's 2014 Summer Grants Announcement Video**. *These Pediatric Oncologists Weren't Expecting This Great News.* July 16, 2014. http://www.stbaldricks.org/blog/post/these-pediatric-oncologists-werent-expecting-this-great-news-video
- Feature Story: **University of Texas Health Science Center at San Antonio**, "Our Stories". *Saving Pediatric Cancer Survivors.* July 2012. http://www.uthscsa.edu/stories/cardiac-research-pediatric
- Feature Story. **BioNews Texas** *UTHSC Researchers Study Pediatric Cancer Treatment Consequences in Adult Survivors*. July 21, 2014. *http://bionews-tx.com/news/2014/07/21/uthsc-researchers-study-pediatric-cancer-treatment-consequences-in-adult-survivors/*
- Feature Story. **BioNews Texas** *UT Health Science Center Researcher Receives St. Baldrick's Foundation Grant to Study Long-Term Effects of Chemotherapy on Children.* August 13, 2014. http://bionews-tx.com/news/2014/08/13/ut-health-science-center-researcher-receives-st-baldricks-foundation-grant-to-study-long-term-effects-of-chemotherapy-on-children/
- Feature Story. **San Antonio Business Journal**. *UT Health Science Center to Benefit from Cancer Research Funding.* September 5, 2014. http://www.bizjournals.com/sanantonio/news/2014/09/05/ut-health-science-center-to-benefit-from-cancer.html
- Feature Story. **BioNews Texas** *Hyundai Gives $250,000 to Cardiac Disease Study in Childhood Cancer Survivors. September 10, 2014.* http://bionews-tx.com/news/2014/09/10/hyundai-gives-250000-to-cardiac-disease-study-in-childhood-cancer-survivors/
- Feature Story. **BioNews Texas** *National Cancer Institute Names UTHSCSA Pediatric Hematologist-Oncologist to Council of Research Advocates. September 23, 2014.* https://bionews-tx.com/news/2014/09/23/national-cancer-institute-names-uthscsa-pediatric-hematologist-oncologist-to-council-of-research-advocates/

- Congressional Childhood Cancer Caucus Website. *Caucus Applauds NCI for Naming Pediatric Oncologist to Council of Research Advocates*. September 30, 2014. https://childhoodcancer-mccaul.house.gov/press-release/caucus-applauds-nci-naming-pediatric-oncologist-its-council-research-advocates
- The Source. *President's Precision Medicine Initiative has San Antonio Connection.* February 11, 2015. http://tpr.org/post/source-presidents-precision-medicine-initiative-has-san-antonio-connection#stream/0
- San Antonio Express News. *Childhood cancer survivor active in Obama medical initiative.* February 9, 2015. http://www.expressnews.com/business/health-care/article/Childhood-cancer-survivor-active-in-Obama-s-6072101.php
- St. Baldrick's Foundation Blog. *Reality Check: We are Nowhere Close to Solving the Problem of Childhood Cancer.* July 6, 2016. *https://medium.com/@StBaldricksFoundation/reality-check-we-are-nowhere-close-to-solving-the-problem-of-childhood-cancer-bf0c1a08e403#.86jkqzdhd*
- Invited Panelist Washington Post Live Forum. "Chasing Cancer." December 6, 2016.

Research Support:

Completed:

- Principal Investigator: NIH Pediatric Loan Repayment. *DNA repair gene polymorphisms and cancer predisposition.* $70,000. 2009-2011
- Principal Investigator: Hyundai Hope on Wheels. Recipient with Gail E. Tomlinson, MD, PhD and Joke Beuten, PhD. $100,000. 2010
- Principal Investigator: NIH Pediatric Loan Repayment. *Genetic Susceptibility to Late Effects of Cancer Therapy in Pediatric Cancer Survivors.* $70,000. 2011-2013
- Principal Investigator: St. Baldrick's Foundation Infrastructure Grant. *Increasing Pediatric Oncology Clinical Trial Enrollment in South Texas.* Principal Investigator: $50,000. 2012-2014
- Principal Investigator: CTSA/IIMS KL2 Scholar. *Identification of Candidate Regulatory miRNAs in Cardiac Fibroblasts Exposed to Doxorubicin.* $250,000. 2012-2014
- Co-Principal Investigator (Lead PI: Helen Parsons): CTSA/IIMS Pilot Grant. *Epidemiology of MRI-confirmed Cardiac Late Effects in Anthracycline Exposed Survivors of Childhood Cancer*. $50,000. 2014.
- Co-Principal Investigator (Lead PI: Rong LI): CTSA/IIMS Pilot Grant. *BRCA1 and its Cofactor in Anthracycline Cardiotoxicity Among Pediatric Cancer Survivors*. $50,000. 2014.

Active:

- Principal Investigator: Hyundai Hope Scholar Grant. *Preclinical Efficacy of Long-term Cardiac Protection by Dexrazoxane*. $250,000. 2014-2016.
- Principal Investigator: St. Baldrick's Foundation Scholar Grant. *Evaluation of the Long-term Cardiac Toxicity of Liposomal Doxorubicin.* $330,000. 2014-2017.
- Principal Investigator (MPI: Rong Li- contact PI): National Cancer Institute R21. *BRCA1 and its cofactor in chemotherapy-associated cardiotoxicity.* $406,671
- Principal Investigator: Pablove Foundation Childhood Cancer Research Seed Grant. *Preclinical Evaluation of Anthracycline Equivalency.* $50,000.

Professional Courses:

- The Jackson Lab. Bar Harbor, Maine. *In vivo* Cardiovascular Assessment in the Mouse. Completed in May 2013.

Medical Licensing and Certification:

- American Board of Pediatrics Subspecialty Board- Hematology/Oncology -- 2011
- American Board of Pediatrics -- General Pediatrics -- 2009

- Medical License: State of Texas: M9820
- Pediatric Advanced Life Support
- USMLE Step 3: Pass, November 2006
- USMLE Step 2 CS: Pass, April 2005
- USMLE Step 2 CK: Pass, June 2000
- USMLE Step 1: Pass, June 1999

Organizations:

- American Association for Cancer Research- Associate Member- 2002-present
- American Society of Clinical Oncology- 2010-present
- American Society of Pediatric Hematology Oncology- Member- 2009- present
- Children's Oncology Group- Member- 2009- present
- American Academy of Pediatrics- Associate Member- 2002- present
- American Medical Association: Member- 2000-2005
- American Medical Student Association: Member- 1997-2005
- Texas Medical Association: Member- 1997-2001

Volunteer Service:

- For the Kids Dance Marathon- Annual Participant 2010-present
- St. Baldrick's San Antonio- Annual Head Shavee and Fundraiser. 2012-present. (Personally raised $30,085 for Childhood Cancer Research)
- Kick-it for CureSearch. Participant 2012.
- Leukemia Lymphoma Society Light the Night Walk. Participant 2011
- Little Heroes Prom. Participant. 2010-2011
- Habitat for Humanity (April 1998)
- Camp Goodtimes (For children afflicted with cancer: 1995)
- Whitman Convalescent Center (Volunteer entertainment: 1992-1993)
- American Cancer Society Cancer Drive (1991-1993)

CURRICULUM VITAE

JUAN CARLOS BERNINI, M.D.

Vannie E.Cook Jr. Cancer Hematology Oncology Clinic
McAllen, Texas 78503
Telephone: 956/661-9840
Facsimile: 956/661-9841
E-mail: jcbernin@txch.org

CURRENT POSITION:

May 2012 – Present:	Medical Director, Vannie E. Cook Jr. Children's Cancer and Hematology Oncology Clinic, McAllen, Texas **Associate** Professor Baylor College of Medicine, Houston, Texas
March 2001 – April 2012:	Medical Director, Vannie E. Cook Jr. Children's Cancer and Hematology Oncology Clinic, McAllen, Texas **Assistant** Professor Baylor College of Medicine, Houston, Texas
September 2007 – Present:	Clinical Assistant Professor, Pediatrics, Hematology-Oncology, UT Health Science Center San Antonio

PREVIOUS AFFILIATIONS:

Jun 1997 – March 2001:	Pediatric Hematology/Oncology; Associate Director, Cook Children's Specialty Clinic, Midland, Texas

EDUCATION:

Senior Pediatric Resident

Oct 1995 – Jan 1997	Senior Pediatric Resident at Texas A&M College of Medicine and Scott & White Memorial Hospital

Fellowship

Nov 1990 – Sept 1995	Pediatric Hematology/Oncology, University of Texas Southwestern Medical School and Children's Medical Center at Dallas, Texas

Pediatric Residency

Jan 1989 – Nov 1990	Attending physician at National Children's Hospital of Costa Rica

Feb 1986 – Jan 1989	University of Costa Rica, Children's National Hospital San Jose, Costa Rica

Rotating Internship

Jan – Mar 1984:	Obstetrics and Gynecology, San Vicente de Paul Hospital, Heredia, Costa Rica
Apr – Jun 1984:	Pediatrics, Children's National Hospital of Costa Rica, San Jose, Costa Rica
July – Sept 1984	Surgery, Tony Facio Hospital, Limon, Costa Rica
Oct – Dec 1984	Internal Medicine, San Rafael Hospital, Alajuela, Costa Rica

Medical School

Jan 1980 – Dec 1984:	"Escuela Autonoma de Ciencias Medicas de Centro America", Affiliated to the "Universidad Autonoma de Centro America", San Jose, Costa Rica

SPECIALTY CERTIFICATION:

1998	American Board of Pediatrics, Sub board, Pediatric Hematology/Oncology Pediatric Hematology/Oncology (No. 001364)
1997	American Board of Pediatrics (No. 061014)

CURRENT MEDICAL LICENSURE:

State of Texas – No. K0262

MEDICAL CERTIFICATIONS:

USMLE 3 (May 1996)
USMLE 2 (Oct 1995)
USMLE 1 (Aug 1995)
ECFMG (July 1990)

MEMBERSHIP IN PROFESSIONAL SOCIETIES:

American Academy of Pediatrics, Fellow Member

Children's Oncology Group (COG)
American Society of Hematology (ASH)
American Society of Pediatric Hematology (ASPHO)

COMMITTEES:

2017-Present	Nominee, Cancer Prevention and Research Institute of Texas Advisory Committee on Childhood Cancers
2017-Present	Member, Doctors Hospital at Renaissance Board of Governors
2017-Present	Member, Doctors Hospital at Renaissance Institutional Review Board Committee
2017-Present	Vice-Chair, Doctors Hospital at Renaissance Oncology Therapeutic Oversight Committee
2017-Present	Alternate Member, Doctors Hospital at Renaissance Emergency Medicine Services Committee
2015-Present	Member, Doctors Hospital at Renaissance Cancer Committee
2015-Present	Member, Doctors Hospital at Renaissance Continuing Medical Education Committee
2015-Present	Member, Doctors Hospital at Renaissance Graduate Medical Education Committee
2015-Present	Member, Doctors Hospital at Renaissance Joint Conference Ad Hoc Committee
2015-Present	Member, Doctors Hospital at Renaissance Pediatric GME Subcommittee
2015-Present	Member, Doctors Hospital at Renaissance Pediatric Specialty Committee
2010-Present	Member, Doctors Hospital at Renaissance Utilization Review Committee
2009-Present	Member, Doctors Hospital at Renaissance Pharmacy and Therapeutics Committee

2009-Present	Member, Cancer Prevention and Research Institute of Texas (CPRIT) Scientific and Prevention Advisory Council
2004-Present	Member, Vannie Cook Foundation Board
2004-Present	Member, Doctors Hospital at Renaissance Pediatric Services Committee
2015-2016	Member, Doctors Hospital at Renaissance Clinical Informatics Committee
2015-2016	Member, Doctors Hospital at Renaissance Clinical Research and IRB Development Committee
2015-2016	Member, Doctors Hospital at Renaissance GME Committee for Residency Development Subcommittee
2015-2016	Chair, Doctors Hospital at Renaissance Oncology Therapeutic Oversight Committee
2015-2016	Member, Doctors Hospital at Renaissance Quality Improvement Ad Hoc Committee
2015-2016	Member, Doctors Hospital at Renaissance Radiation Oncology Subcommittee
2010-2016	Member, Doctors Hospital at Renaissance Pathology Lab Users Committee
2010-2014	Member, Doctors Hospital at Renaissance Ethics Committee
2009-2014	Member, Doctors Hospital at Renaissance Tumor Board Committee
2004-2008	Member, Doctors Hospital at Renaissance Radiation Oncology Committee
2007-2014	Member, Doctors Hospital at Renaissance Chemotherapy Safety Committee
2008-2009	Member, Doctors Hospital at Renaissance Governing Board of Pediatrics
2005-2006	Chairman, Doctors Hospital at Renaissance Pediatric

	Committee
2005-2006	Member, Doctors Hospital at Renaissance Medical Executive Committee

ADMINISTRATIVE EXPERIENCE:

2001 to Present:	Medical Director, Vannie E. Cook Jr. Cancer and Hematology Oncology Clinic. McAllen, Texas
2008 – Present:	Laboratory Director, Vannie E. Cook Jr. Children's Cancer and Hematology Laboratory
2006 to present	Board of Managers, Doctors Hospital at Renaissance McAllen, Texas
2005-2006	Chief of Pediatrics, Doctors Hospital at Renaissance McAllen, Texas
1997 – 2001:	Assistant Director, Cook Children's Specialty Clinic Midland, Texas
Oct 1996 – Jan 1997	Pediatric Chief Resident, Texas A&M College of Medicine and Scott & White Memorial Hospital
1993 -1994	Hematology/Oncology Chief Fellow at Southwestern Medical School and Children's Medical Center, Dallas, Texas
Jan – Dec 1985	Director of Birth Control Committee "Centro Integrado de Salud San Rafael de Puntarenas," Costa Rica
Jan – Dec 1985	Director of Health Clinic "Centro Penal de San Lucas" Puntarenas, Costa Rica
LANGUAGES:	Fluent in Spanish and English

AWARDS AND RECOGNITION:

2013	Hyundai Hope on Wheels Scholar
2011	Hyundai Hope on Wheels Scholar

2010	Robert Wood Johnson Foundation Community Health Leaders 2009-2010 Nominee
1992	Special recognition for outstanding performance as in-house fellow by the Pediatric Hematology/ Oncology Program Director
1989	Academic Crown as a distinguished student, "Universidad Autonoma de Centro America"
1987	Special recognition for outstanding performance as resident in charge of the outpatient clinic by the Outpatient Clinic Director; Children's National Hospital, San Jose, Costa Rica
1985	Doctor in Medical Science, "***Cum Laude Probatus"***, San Jose, Costa Rica
1984	Conclusion of Medical and Surgical Studies Certificates, ***"Cum Laude Probatus"***, San Jose, Costa, Rica
1984	Licentiate Degree in Medical Science, ***"Cum Laude Probatus",*** San Jose, Costa Rica
1983	Bachelor's Degree in Medical Science, ***"Summa Cum Laude Probatus"***, San Jose, Costa Rica

TEACHING ACTIVITIES AND PRESENTATIONS:

Sept 2009	Valley Baptist Medical Center, Pediatric Grand Rounds, "Childhood Hemangiomas…When to Worry?", Harlingen, Texas
Jun 2000	Chemotherapy Workshop for Nurses, Speaker
1997 – 1999	Department of Pediatrics; Academic Appointment, Texas Tech University, Odessa, Texas
Oct 1998	14th Annual William D. Furst, M.D.; Pediatric and Primary Care Update- Management of Cancer in Children and Adolescents
Sept 1993	Coagulation Symposium; "Presentation of Study Cases", Dallas, Texas

1988 –1989	Assistant Professor of Pediatrics; "Escuela Autonoma de Ciencias Medicas de Centro America, affiliated to the Universidad Autonoma de Centro America"

PUBLICATIONS:

BOOK CHAPTERS AND REVIEW ARTICLES

1. **Bernini, J.C.** Severe Anemia. In Levin D, Morris FC. Eds: Essentials of Pediatric Intensive Care. 2ed. New York: Quality Medical Publisher Inc. Chapter 50, page 492-7

2. **Bernini, J.C.** Diagnosis and Management of Chronic Neutropenia During Childhood. Pediatric Clinics of North America. 1996; 43:773-91

ORIGINAL MANUSCRIPTS

1. Fort D, **Bernini JC**, Johnson A, Cochran C, Buchanan GR. Splenic Rupture in hemophilia. *American Journal Pediatric Hematology Oncology* 1994; 16:255-9

2. **Bernini JC**, Buchanan GR, Ashcraft J. Hypoprothrombinemia and severe hemorrhage associated with a lupus anticoagulant. *Journal Pediatrics* 1993; 123:937-9

3. Mustafa M, Sandler E, **Bernini JC**, Aquino V. Amphotericin B Colloidal Dispersion therapy for invasive mycosis: Report of successful therapy in two pediatric patients. *Pediatric Infectious Diseases Journal* 1994; 12(4):328

4. **Bernini JC**, Fort DW, Pritchard M, Rogers BB, Winick NJ. Adjuvant chemotherapy for treatment of unresectable and metastatic angiomatoid malignant fibrous histiocytoma. *Cancer* 1994;74:962-4

5. **Bernini JC**, Carrillo JM, Quesada E. High dose intravenous Methyl-prednisolone therapy for patients with Diamond-Blackfan anemia refractory to conventional doses of steroids. *Journal of Pediatrics* 1995; 127:654-659

6. **Bernini JC**, Timmons CF, Sandler E. Acute basophilic leukemia in a child: Anaphylactoid reaction following intravenous vincristine administration, *Cancer* 1995;75:110-14

7. **Bernini JC**, Wooley R, and Buchanan GR. Low-alternate day doses of rhG-CSF in the treatment of childhood chronic neutropenia. *Journal of Pediatrics* 1996; 129:551-8

8. **Bernini JC**, Fort DW, Griener JC, Kane BJ, Chappell WB, Kamen B. The

use of Aminophylline for Methotrexate-induced Neurotoxicity: Evidence for Adenosine Receptor Blockade. Lancet 1995; 345:544-47

9. **Bernini, JC**, Mustafa MM, Sutor LJ, Buchanan GB. Fatal immune-mediated hemolysis induce by ceftriaxone in a child with sickle cell anemia. *Journal of Pediatrics* 1995: 126;813-815

10. Lowichik L, **Bernini JC**, Tonk V, Ansari MQ, Rollins N, Winick N, Timmons CF. Relapse of precursor B-cell acute lymphoblastic leukemia as an isolated central nervous system mass lesion 9 years after original diagnosis. *Medical and Pediatric Oncology* 1996; 26:129-134

11. **Bernini JC,** Zora RR, Sandler ES, Reisch JS, Quinn CT, Buchanan GR. Beneficial effect of intravenous dexamethasone in children with mild to moderately severe acute chest syndrome complicating sickle cell disease. Blood 1998;92:3082-3089

12. Oral megadose methylprednisolone therapy for refractory Diamond-Blackfan anemia. George R. Buchanan, M.D for the International Diamond-Blackfan Anemia Study Group. Journal of Pediatric Hematology Oncology 2001; 23: 353

13. Murray JC, **Bernini JC**, Bijou HL, Rossmann SN, Mahoney,Jr DH, Morad AB. Infantile cytomegalovirus-associated autoimmune hemolytic anemia. Journal of Pediatric Hematology Oncology 2001; 23:318-20.

ABSTRACTS

1. **Bernini JC**, Faingezitch I. Hospital acquired urinary tract infection.

   L National Pediatric Meeting (presented). San Jose, Costa Rica. 1983

2. **Bernini JC**, Faingezitch I. Hospital acquired urinary tract infection (presented). First National Meeting of Prevention and Control of Hospital Acquired Infections, San Jose, Costa Rica 1984.

3. **Bernini JC**, Mustafa MM, Winick NJ, Nicholson M, McHard K, Buchanan GR. Evaluation of attenuated live measles vaccine in children with cancer (presented). Program/preceding of the American Society of Clinical Oncology 1994;13:438

4. **Bernini JC**, Mustafa MM, Winick NJ, Nicholson M, McHard K, Buchanan

   GR. Evaluation of attenuated live measles vaccine in children with cancer (presented). Pediatric Research 1994; 35(4):174a

5. **Bernini JC,** Harrison L, Wooley R, and Buchanan GR. Low-alternate day doses of rhG-CSF in the treatment of childhood chronic neutropenia

(presented). American Society of Pediatric Hematology/Oncology, Official Program and Scientific Proceedings 1994;3:14

6. **Bernini JC**, Mustafa MM, Winick NJ, Nicholson M, McHard K, Buchanan GR. Evaluation of attenuated live measles vaccine in children with cancer (presented). Pediatric Research 1994;35(4):174a

7. **Bernini JC**, Buchanan GR. Hamorragia grave causada por deficiencia de protrombina en asociacion con un anticuagulante de tipo lupico: Respuesta a corticoesteroides (presented). Revista Iberoamericana de Trombosis y Hemostasis 1993;VI(2):186

8. **Bernini JC**, Buchanan GR. Severe hemorrhage due to prothrombin Deficiency associated with a lupus-type anticoagulant: Response to Corticosteroid therapy. Pediatric research 1993

9. Mustafa MM, Carlson LR, Weitman SD, Fort DW, **Bernini JC**, Winick NJ, Katz JA, Sandler ES, Rogers ZR, McCracken GH, and Buchanan GR. Cefepime vs. Ceftazidime in the empiric treatment of febrile neutropenic children with malignancy (presented). 32nd ICAAC, Anaheim, CA

10. **Bernini JC**, Carrillo JM, Quesada E, Buchanan GR. Evaluation of high dose intravenous methylprednisolone for patients with Diamond-Blackfan anemia (DBA) refractory to conventional doses of steroids (presented). Blood 1993;80(10),Suppl 1:282a

11. **Bernini JC,** Fort DW, Griener JC, Kane BJ, Chappell WB, Kamen B. The Use of Aminophylline for Methotrexate-induced Neurotoxicity: Evidence for Adenosine Receptor Blockade. Proceedings. 86th Annual

    Meeting American Association for Cancer Research, Toronto, Canada 1995;36:1718a Suppl

12. **Bernini JC**, Tkaczewski I, Rogers ZR, Sandler E, Reisch JS, Buchanan GR. Dexamethasone therapy for children with acute chest syndrome (ACS) complicating sickle cell disease (SCD): A randomized, double-blind, placebo-controlled pilot study (presented). American Society of Hematology. 37th Annual Meeting December. 1995;86:142a Suppl

13. Rogers ZR, Dale JC, **Bernini JC**, Reisch JS, Prim PA, Buchanan GR. Dexamethasone shortens the duration of painful events requiring hospitalization in children with sickle cell disease: Results of a randomized, double-blind, placebo-controlled trial (presented). American Society of Hematology. 37th Annual Meeting December. 1995;86:250aSuppl

14. Griffin T, Bowman P, Sanders J, Friedman D, Murray J, Eames G, Shah A,

Bernini JC. Favorable results of an intensive protocol for advanced-stage T-cell lymphoblastic lymphoma and acute lymphoblastic leukemia in Childhood. Proc Inter Soc Paediatr Oncol & Am Soc Pediatr Hematol/Oncol, Montreal, Sept 13-18, 1999.

EDITORIAL REQUESTS:

1995 *The Pediatric Infectious Disease Journal,* chosen manuscript reviewer

1997 American Board of Pediatrics, Sub board of Pediatric Hematology/ Oncology, Invited Question Writer for Certifying Examination

2000 *British Journal of Hematology,* chosen manuscript reviewer

PROFESSIONAL REFERENCES:

David Poplack, MD,
Director, Texas Children's Cancer and Hematology Center
Elise C. Young Professor of Pediatric Oncology
Head, Hematology-Oncology Section, Department of Pediatrics, Baylor College of Medicine
Deputy Director, Dan L. Duncan Cancer Center, Baylor College of Medicine Texas 6621 Fannin St.
Houston, TX 77030

Paul Bowman, MD, Medical Director Hematology/Oncology
Cook Children's Medical Center
801 Seventh Ave
Ft. Worth, Texas 76104

George R. Buchanan, MD, Medical Director
UT Southwestern Medical Center
5323 Harry Hines Blvd
Dallas, Texas 75235

CURRICULUM VITAE

STANTON C. GOLDMAN, M.D.

October 20, 2016

BIOGRAPHICAL:

Name:	Stanton Carl Goldman, M.D.
Spouse:	Ellen Sher, M.D.
Birth Place:	Bronx, New York
Citizenship:	U.S. Citizen
Office Address:	Medical City Dallas Hospital 7777 Forest Lane Suite D-400 Dallas, Texas 75230
Telephone:	972-566-6647
Fax:	972-566-6496

APPOINTMENTS:

1998 - Present	Texas Oncology, P.A. Pediatric Hematology/Oncology And Stem Cell Transplantation
2015 – Present	Medical Director Medical City Children's Hospital Dallas, Texas
1996 - 1998	Attending Physician Division of Pediatric Hematology/Oncology Pediatric Subspecialty Faculty Children's Hospital of Orange County
2002 - 2009	Chairman, Cancer Committee Medical City Dallas Hospital
2010 - 2011	Chairman Department of Pediatrics Medical City Dallas Hospital Dallas, Texas
2012 - 2013	President – Medical Staff (1200+ physicians) Medical City Dallas Hospital Dallas, Texas

EDUCATION AND TRAINING:

1984 - 1990	B.A./M.D. Boston University and Boston University School of Medicine, Boston, MA (Six-Year Medical Program)

1990 - 1991	Internship in Pediatrics Children's National Medical Center Washington, DC
1991 - 1993	Residency in Pediatrics Children's National Medical Center Washington, DC
1993 - 1994	Senior Clinical Fellow, Pediatric Hematology/Oncology Johns Hopkins University Medical Center Baltimore, MD
1994 (summer)	Participant in the "Molecular Biology In Clinical Oncology: A Workshop" Aspen, CO
1994 - 1996	Postdoctoral Research Fellow in the lab of Michael Kastan, M.D., Ph.D.

CERTIFICATION AND LICENSURE:

1990 - Present	National Board of Medical Examiners
1993 - Present	American Board of Pediatrics
1996 - Present	American Board of Pediatrics Hematology/Oncology Sub-Board

HONORS:

1988	Recipient Summer Research Grant, American Cancer Society
1989	Exchange Fellow, Hebrew University School of Medicine Hadassah Hospital Jerusalem, Israel
1990	Magna Cum Laude
2002 – 2016	Elected among the best pediatric specialists in Dallas each year in issues of *D Magazine*.

SOCIETIES AND MEMBERSHIPS:

1986 - 1990	Massachusetts Medical Society
1990 - Present	American Academy of Pediatrics

1994 - 2000	American Association for Cancer Research, Associate Member
1998 - Present	American Society of Clinical Oncology
2001 - Present	American Society of Pediatric Hematology/Oncology

PUBLICATIONS:

Articles

1. **Goldman SC**, Chen C-Y, Lansing TJ, Gilmer TM, Kastan MB: The p53 signal transduction pathway is intact in human neuroblastoma despite cytoplasmic localization. **American Journal of Pathology** 148:1381-1385, 1996.

2. Metcalfe SM, Canman CE, Milner J, Morris RE, **Goldman SC**, Kastan MB: Rapamycin and p53 act on different pathways to induce G1 arrest in mammalian cells. **Oncogene** 15:1635-1642, 1997.

3. **Goldman S**, Ellis R, Dhar VJ, Cairo MS: Rationale and potential use of cytokines in the prevention and treatment of neonatal sepsis. **Clinics in Perinatology**. 25(3):699-710, 1998.

4. Bracho F, **Goldman S**, Cairo MS: Potential use of granulocyte colony-stimulating factor and granulocyte-macrophage colony-stimulating factor in neonates. **Curr Opin Hematol** 5(3):215-220, 1998.

5. Abu-Ghosh A, **Goldman S**, Slone V, van de Ven C, Suen Y, Murphy L, Sender L, Cairo MS: Immunological reconstitution and correlation of circulating serum inflammatory mediators/cytokines with the incidence of acute graft-versus-host disease during the first 100 days following unrelated umbilical cord blood transplantation. **Bone Marrow Transplantation**. 24:535-544, 1999.

6. Weinthal, J, **Goldman S**, Lenarsky C: Case Report: Successful treatment of relapsed Burkitt's lymphoma using unrelated cord blood transplantation as consolidation therapy. **Bone Marrow Transplantation**, 25:1311-1313, 2000.

7. **Goldman SC**, Holcenberg JS, Finklestein JZ, Hutchinson R, Kreissman S, Johnson FL, Tou C, Harvey E, Morris E, Cairo MS: A randomized comparison between rasburicase and allopurinol in children with lymphoma or leukemia at high risk of tumor lysis. **Blood** 97(10):2998-3003, 2001.

8. **Goldman SC**, Bracho F, Davenport V, Slack R, Areman E, Shen V, Lenarsky C, Weinthal J, Hughes R, Cairo MS: A feasibility study of Interleukin-11 and G-CSF after myelosuppressive chemotherapy to mobilize peripheral blood stem cells from heavily pretreated patients. **Journal of Pediatric Hematology/Oncology** 23(5):300-305, 2001.

9. Abu-Gosh AM, Krailo MD, **Goldman SC**, Slack RS, Davenport V, Morris E, Laver JH, Reaman GH, Cairo MS: Ifosfamide, carboplatin and etoposide in children with poor-risk relapsed Wilms tumor: a Children's Cancer Group Report. **Annals of Oncology** 13:460-469, 2002.

10. **Goldman SC**, Morris E, Pui CH, Stouch B, Cairo, M: Recombinant urate oxidase (Rasburicase) provides excellent control of the uric acid (UA) and prevention of acute renal failure (ARF) in pediatric patients (pts) with advanced B-cell (Burkitt's) lymphoma/leukemia (BLL). 1st International Symposium of Childhood and Adolescent Non-Hodgkin's Lymphoma, New York, NY. **Journal of Pediatric Hematology/Oncology** 25(4): 27p, S7, 2003.

11. Harris, DT, **Goldman S**: Cord blood banking: state of the science. **Contemporary Ob/Gyn** July (Suppl.): 4-11, 2003.

12. **Goldman, S**: Rasburicase: potential role in managing tumor lysis in patients with hematological malignancies. **Expert Review of Anticancer Therapy**, 3(4): 429-433, 2003.

13. Navolanic PM, Pui CH, Larson RA, Bishop MR, Pearce TE, Cairo MS, **Goldman SC**, Jeha SC, Shanholtz CB, Leonard JP, McCubrey JA: Elitek-rasburicase: an effective means to prevent and treat hyperuricemia associated with tumor lysis syndrome, a Meeting Report, Dallas, Texas, January 2002. **Leukemia** 17(3):499-514, 2003.

14. **Goldman SC**, Weinthal J, Lenarsky C: Management of Hyperuricemia Due to Acute Lymphoblastic Leukemia in a Pediatric Patient. **Case Studies in Oncology**, pp. 3-5, May 2004.

15. Cairo MS, Davenport V, Bessmertny O, **Goldman SC**, Berg SL, Kreissman SG, Laver J, Shen V, Secola R, van de Ven C, Reaman GH: Phase I/II dose escalation study of recombinant human interleukin-11 following ifosfamide, carboplatin, and etoposide in children, adolescents, and young adults with solid tumours or lymphoma: a clinical, haematological and biological study. **Br J Haematol** 128(1):49-58, 2005.

16. Rakheja D, **Goldman S**, Wilson K, Lenarsky C, Weinthal J, Schultz R: Translocation (4;19)(q35;q13.1) associated primitive round cell sarcoma: report of a case and review of the literature. **Pediatr Dev Pathol.** 2007 Jul 16;1.

17. Kershenovich A, Price AV, Koral K, **Goldman S**, Swift DM: Failure to treat obstructive hydrocephalus with endoscopic third ventriculostomy in a patient with neurodegenerative Langerhans cell histiocytosis. **J Neurosurg Pediatr.** 2008 Nov;2(5):304-9.

18. Candrilli S, Bell T, Irish W, Morris E, **Goldman S**, Cairo MS: A comparison of inpatient length of stay and costs among patients with hematologic malignancies (excluding Hodgkin disease) associated with and without acute renal failure. **Clin Lymphoma Myeloma** 2008 Feb;8(1):44-51.

19. Cairo M, **Goldman SC**: Monoclonal antibody therapy in childhood and

adolescent non-Hodgkin lymphoma. **Am Soc Clin Oncol Ed Book**. 403-407, 2010.

20. Allen CE, Flores R, Rauch R, Dauser R, Murray JC, **Goldman S**, et al: Neurodegenerative central nervous system Langerhans cell histiocytosis and coincident hydrocephalus treated with vincristine/cytosine arabinoside. **Pediatr Blood Cancer** 2010; 54:416-423.

21. Cairo MS, Coiffier B, Reiter A, Younes A, **Goldman SC**, et al: Recommendations for the evaluation of risk and prophylaxis of tumour lysis syndrome (TLS) in adults and children with malignant diseases: an expert TLS panel consensus. **Br J Haematol** 2010 May;149(4):578-86.

22. Shiramizu B, **Goldman S**, Kusao I, Agsalda M, Lynch J, Smith L, Harrison L, Morris E, Gross TG, Sanger W, Perkins S, Cairo MS: Minimal disease assessment in the treatment of children and adolescents with intermediate-risk (Stage III/IV) B-cell non-Hodgkin lymphoma: a Children's Oncology Group report. **Br J Haematol**. 2011 Jun;153(6):758-63. doi: 10.1111/j.1365-2141.2011.08681.x.

23. Cairo MS, Sposto R, Gerrard M, Auperin A, **Goldman SC**, Harrison L, Pinkerton R, Rapheal M, McCarthy K, Perkins SL, Patte C: Advanced stage, increase3d lactate dehydrogenase, and primary site, but not adolescent age (> 15 years), are associated with an increased risk of treatment failure in children and adolescents with mature B-cell non-Hodgkin's lymphoma: results of the FAB LMB 96 study. **J Clin Oncol** 2012 Feb 1;30(4):387-93.

24. **S Goldman**, L Smith, JR Anderson, S Perkins, et al: Rituximab and FAB/LMB 96 chemotherapy in children with stage III/IV B-cell non-Hodgkin lymphoma: a Children's Oncology Group report. **Leukemia** (3 September 2012) | doi:10.1038/leu.2012.255.

25. Gerrard, Mary, Waxman IM, Sposto R, Auperin A, Perkins SL, **Goldman S**, et.al: Outcome and pathologic classification of children and adolescents with mediastinal large B-cell lymphoma treated with FAB/LMB96 mature B-NHL therapy. **Blood**, 10 January 2013; Vol 121(2):278-285.

26. Barth MJ, **Goldman S**, Smith L, Perkins S, Shiramizu B, Gross TG, Harrison L, Sanger W, Geyer MB, Guilino-Roth L, Cairo MS: Rituximab pharmacokinetics in children and adolescents with de novo intermediate and advanced mature B-cell lymphoma/leukemia: a Children's Oncology Group report. **Br J Haematol**. 2013 Jun 27. doi: 10.1111/bjh.12434.

Abstracts

1. **Goldman SC**, Chen C-Y, Kastan MB: Localization and function of p53 protein in human neuroblastoma. **Proceedings of the American Association for Cancer Research**, Vol 36:610, March 1995.

2. **Goldman SC**, Kastan MB: A human neuroblastoma cell line overexpresses the

murine double minute 2 (MDM2) oncoprotein. **Proceedings of the American Society of Pediatric Hematology/Oncology**, 1995.

3. **Goldman SC**, Kastan MB: Wild-type p53 protein function is necessary and sufficient for irradiation induced differentiation in human neuroblastoma. Johns Hopkins Oncology Center, "Cancer Center Fellow Research Day", June 1996.

4. **Goldman SC**, Sweetman R, Suen Y, Murphy L, van de Ven C, Sender L, Slone V, Cairo MS: A high incidence of severe (Grade III) acute graft vs. host disease (AGVHD) following unrelated cord blood transplants (UCBT): HLA mismatching, but not serum soluble IL-2 receptor levels are predictive of AGVHD following UCBT. **Blood** Vol. 88 (10) 1:422a, 1996.

5. Slone V, Abu-Ghosh A, **Goldman SC**, Murphy L, Sender LS, van de Ven C, Cairo MS: Delayed platelet, but comparable myeloid engraftment following unrelated cord blood transplantation (UCBT): Decreased megakaryocyte lineage (CD-34+/CD-41+) stem cells in cord blood. **Blood** Vol. 88 (10)1:114a, 1996.

6. **Goldman SC**, Slone V, van de Van C, Joubran J, Suen Y, Murphy L, Sender L, Cairo MS: Delayed platelet reconstitution and severe (Grade III) acute graft vs. host disease (AGVHD) are major obstacles to unrelated cord blood transplant (UCBT): CD34+/CD41+ subset, but not soluble IL-2R level is a useful predictor following UCBT. **Proceedings of the American Society of Clinical Oncology** 16:110a, 1997.

7. **Goldman SC**, Davenport V, Reaman G, Laver J, Kreissman S, Blazer B, Berg S, Kaye J, Patterson F, Cairo MS: Combination of rhIL-11 + G-CSF enhances platelet and myeloid recovery following Ifosfamide, Carboplatin, and Etoposide (ICE) chemotherapy in children with solid tumors: rhIL-11 is well tolerated at double the adult recommended dose. **Experimental Hematology** (25)8:786a, 1997.

8. **Goldman S**, Lenarsky C, Weinthal J: TBI/VP-16 with ATG is sufficient for sustained engraftment following alternative donor mismatched transplantation in children with high risk leukemia and lymphoma. **Blood**, 92:10, 356b, 1998.

9. Weinthal J, Moss T, Chen A, **Goldman S**, Wingard J, Moreb J, Gilman A, Lenarsky C: A quantitative immunocytologic (ICC) assay for detecting ewing's sarcoma (ES) cells in marrow and stem cell products. **ASCO Proceedings** 18:561a, 1999.

10. Weinthal J, **Goldman S**, Lenarsky C. Successful salvage therapy for relapsed Burkitt's lymphoma with unrelated cord blood transplantation (UCBT). Presented as poster at the **1999 Pan-Pacific Lymphoma Conference** held in Kauai, Hawaii and sponsored by the University of Nebraska Medical Center. July 1999.

11. **Goldman SG**, Munoz L, Lenarsky C, Weinthal J: TBI/VP-16 with ATG is sufficient for sustained engraftment following mismatched unrelated cord blood transplantation in children with high risk leukemia and lymphoma. Presented as a poster at the **Fifth Annual Marrow Transplantation in Children Conference**, Hilton Head, South Carolina, February 2000.

12. **Goldman S**, Stafford C, Weinthal J, Kerr R, White C, Savin M, Brooks B, Rosenfeld CS, Lenarsky C: Older adolescents vary greatly from children in their route of referral to the pediatric oncologist and national trials. Presented as a poster at the 36th Annual ASCO Meeting, New Orleans, Louisiana, May 2000. **ASCO Proc**, 1766:450a, 2000.

13. Klingele H, Massey E, Bailey T, Lenarsky C, **Goldman S**, Weinthal J: Monoclonal antibody therapy in pediatrics: Initial experiences with Rituximab. Presented as a poster at the **Annual Association of Pediatric Oncology Nurses Meeting**, Orlando, Florida, September 2000.

14. Weinthal JA, **Goldman SG**, Rosenfeld CS, Hooker M, Henderson D, Lenarsky C: Early and Late Infectious complications following unrelated cord blood transplantation (UCBT). **Blood** 98(11):5224a, 2001.

15. **Goldman S**, Pui CH, Baruchel A, Leverger G, Morris E, Cairo MS: Rasburicase significantly reduces urica acid and creatinine levels in patients with Burkitt's lymphoma and B-ALL at risk for tumor lysis syndrome. **Ann Onc** 13(2):606, 2002.

16. Cairo MS, Casciano R, Morris E, Arikian S, Stern L, **Goldman S**, Doyle J: Uric acid level is a significant prognostic factor in the development of tumor lysis syndrome and renal events in non-Hodgkin's lymphoma in patients admitted for inpatient chemotherapy. **Ann Onc** 13(2):624, 2002.

17. **Goldman S**, Lynch JC, Harrison L, Cairo MS: A prospective trial of prophylaxis and treatment (P+T) acute tumor lysis syndrome (ATLS) with rasburicase (R) and nonalkaline hydration in children and adolescents (C+A) with intermediate- (Group B) and high-risk (Group C) mature B-NHL: A Children's Oncology Group report. Presented as a poster at the **American Society of Clinical Oncology Conference**, Chicago, Illinois, June 2010.

18. Cairo MS, Lynch JC, Harrison L, Perkins SL, Shiramizu B, Gross TG, Sanger W, **Goldman S**: Safety, kinetics, and outcome following rituximab (R) in combination with FAB chemotherapy in children and adolescents (C+A) with stage III/IV (Group B) and BM+/CNS+ (Group C) mature B-NHL: A Children's Oncology Group report. Presented as a poster at the **American Society of Clinical Oncology Conference**, Chicago, Illinois, June 2010 (9536a).

19. Shiramizu B, **Goldman S**, Kusao I, Agsalda M, Lynch JC, Harrison L, Gross TG, Sanger W, Perkins SL, Cairo MS: Use of immunoglobulin heavy chain primer pools to assess minimal residual disease/persistent disease (MRD/PD) in children and adolescents with mature B-cell non-Hodgkin lymphoma (B-NHL): A Children's Oncology Group report. Presented as a poster at the **American Society of Clinical Oncology Conference**, Chicago, Illinois, June 2010 (9527a).

20. Day, N.S., Ayello, J., Miles, R.R., Perkins, S., Lim, M.S., Waxman, I., van de Ven, C., Sanger, W.G., Harrison, L., **Goldman, S.**, and Cairo, M.S. Comparative genomic identification of unique signaling pathways and targets in pediatric

Burkitt lymphoma (PBL). Presented, 11th International Conference on Malignant Lymphoma, 2011. Annals of Oncology 2011; 22 (supp 4):#185.

21. Day, N.S., Ayello, J., Lim, M.S., Perkins, S., Miles, R.R., Van de Ven, C., Sanger, S.G., Harrison, L., **Goldman, S.**, Cairo, M.S. Dleu1 sirna gene knockdown in pediatric Burkitt lymphoma (PBL) is associated with a significant decrease in drug induced apoptosis: implication of DLEU1 as a tumor suppressor gene. Annals of Oncology 2011; 22 (supp 4):#385.

22. Barth, M., **Goldman, S.**, Zhi, J., Smith, L., Harrison, L., Perkins, S.L., Shiramizu, B., Gross, T., Sanger, W., and Cairo, M.S. Safety and pharmacokinetics (PK) of rituximab (R) in combination with FAB chemotherapy in children and adolescents (C+A) with stage III/IV mature B-NHL: A Children's Oncology Group Report. Presented, 11th International Conference on Malignant Lymphoma, 2011. Annals of Oncology 2011; 22 (supp 4):#090.

23. Shiramizu, B., **Goldman, S.**, Smith, L., Harrison, L., Van de Ven, C., Gross, T., Sanger, W., Perkins, S., and Cairo, M.S. Minimal disseminated disease/residual disease in children and adolescents with mature B-147 cell non-Hodgkin lymphoma (B-NHL) may Impact the risk of relapse: A Children's Oncology Group Report. Presented, 11th International Conference on Malignant Lymphoma, 2011. Annals of Oncology 2011; 22 (supp 4):#097.

24. **Goldman, S.**, Smith, L., Perkins, S., Shiramizu, B., Gross, T., Sanger, W., Harrison, L., and Cairo, M.S. Outcome and patterns of failure following combined FAB chemotherapy and rituximab in children and adolescents with stage III/IV, BM+ and/or CNS+ mature B-NHL: A Children's Oncology Report. Presented, 11th International Conference on Malignant Lymphoma, 2011. Annals of Oncology 2011; 22 (supp 4):#274.

25. Galardy, P.J., **Goldman, S.**, Harrison, L., Perkins, S., and Cairo, M. Rasburicase (Ras) is safe and effective in the prevention and treatment of acute tumor lysis syndrome (ATLS) in children and adolescence undergoing reduction therapy for mature B-cell lymphoma (MBL): A report from the Children's Oncology Group. Presented, 11th International Conference on Malignant Lymphoma, 2011. Annals of Oncology 2011; 22 (supp 4):#280.

26. Nelson, M., **Goldman, S.C.**, Perkins, S.L., Harrison, L., Cairo, M.S. Sanger, W.G. Isolated *MYC* rearrangement by cytogenetics/FISH in children and adolescents (C & A) with stage III/IV B-NHL BM+ and/or CNS+ may have an improved outcomes following immunochemotherapy: A report from the Children's Oncology Group. Presented, 11th International Conference on Malignant Lymphoma, 2011. Annals of Oncology 2011; 22 (supp 4):#271.

27. Frazer, J.K., **Goldman, S.C.**, Harrison, L., Perkins, S.L., Cairo, M.S. Excellent outcome in CNS-positive pediatric Burkitt lymphoma and other mature B-NHL treated with combined chemo-immunotherapy (rituximab + FAB Group C) without CNS radiation: A Children's Oncology Group report. Presented, 11th International Conference on Malignant Lymphoma, 2011. Annals of Oncology 2011; 22 (supp 4):#278.

28. **Goldman, S.**, Smith, L., Perkins, S., Shiramizu, B., Gross, T., Sanger, W., Harrison, L., and Cairo, M.S. Outcome and pharmacokinetic (PK) analysis of adding rituximab to FAB chemotherapy in children and adolescents with advanced mature B-NHL/leukemia: a Children's Oncology group report. Presented, 43rd Congress of the International Society for Paediatric Oncology 2011. Pediatric Blood Cancer 2011; 57 (5):736, #O122.

29. **Goldman, S.**, Galardy, P.J., Smith, L., Perkins, S., Shiramizu, B., Gross, T., Sanger, W., Harrison, L., and Cairo, M.S. The efficacy of rasburicase and rituximab combined with FAB chemotherapy in children and adolescents with newly diagnosed stage III/IV BM+ and CNS+ mature B-NHL: A Childrens Oncology Group report. Presented, American Society of Hematology, 2011. Blood 118 (21):1161, #2702.

30. Cairo, M.S., Day, N., **Goldman, S.**, Sanger, W.G., Harrison, L., Lim, M.S., Miles, R.R., and Perkins, S.L. Genomic pathways and potential therapeutic targets in pediatric Burkitt lymphoma (PBL): a Children's Oncology Group Report. Presented, American Society of Hematology, 2011 American Society of Hematology, 2011. Blood 118 (21):690, #1587.

31. Hochberg, J.C., Galardy, P.J., **Goldman, S.**, Smith, L., and Cairo, M.S. The efficacy of rasburicase during reduction COP chemotherapy for the prevention of tumor lysis syndrome (TLS) in children and adolescents with newly diagnosed stage III/IV, BM_ and NS mature B-NHL: a Children's Oncology Group 150 Report. Presented, American Society of Pediatric Hematology Oncology, 2012. Pediatric Blood & Cancer 2012 58 (7):1041, #571.

32. Frazer, J.K., **Goldman, S.C.**, Smith, L., Harrison, L., Perkins, S.L., Cairo, M.S. Efficacy of rituximab plus FAB group C chemotherapy without CNS radiation in CNS-positive pediatric Burkitt lymphoma/leukemia: a report from the Children's Oncology Group. Presented, American Society of Clinical Oncology 2012 Annual Meeting. J Clin Oncol 30, 2012 (suppl; abstr 9501).

33. Frazer, J.K., **Goldman, S.C.**, Smith, L., Harrison, L., Perkins, S.L., Cairo, M.S. Improved outcomes in CNS-positive pediatric Burkitt lymphoma/leukemia using rituximab plus FAB group C1 chemotherapy without CNS radiation: a Children's Oncology report. Presented, Fourth International Symposium on Childhood, Adolescent and Young Adult Non-Hodgkin's Lymphoma, 2012. British Journal of Haematology 2012; 159 (Supp. 1):3, #5.

34. Barth, M., **Goldman, S.**, Zhi, J., Smith, L., Harrison, L., Perkins, S.L., Shiramizu, B., Gross, T., Sanger, W., Cairo, M.S. Safety and pharmacokinetics of rituximab in combination with FAB chemotherapy in children and adolescents with Stage III/IV mature B-NHL and B-cell leukemia +/- CNS disease: a Children's Oncology group report. Presented, Fourth International Symposium on Childhood, Adolescent and Young Adult Non-Hodgkin's Lymphoma, 2012. British Journal of Haematology 2012; 159 (Supp. 1):23 #40.

35. **Goldman, S.**, Smith, L., Anderson, J.R., Perkins, S., Harrison, L., Shiramizu, B., Sanger, W., Barth, M., Cairo, M.S. Outcome of advanced stage (III/IV) intermediate-risk patients with mature B-NHL using rituximab plus FAB group B4

chemotherapy: a Children's Oncology Group report. Presented, Fourth International Symposium on Childhood, Adolescent and Young Adult Non-Hodgkin's Lymphoma, 2012. British Journal of Haematology 2012; 159 (Supp. 1):24 #42.

36. Hochberg, J.C., Galardy, P.J., **Goldman, S.**, Perkins, S., Cairo, M.S. The efficacy of rasburicase during COP reduction for the prevention of TLS in children and adolescents with newly diagnosed advanced stage mature B-NHL: a Children's Oncology Group report.. Presented, Fourth International Symposium on Childhood, Adolescent and Young Adult Non-Hodgkin's Lymphoma, 2012. British Journal of Haematology 2012; 159 (Supp. 1):26 #46.

37. Galardy, P., Shiramizu, B., **Goldman, S.**, Hochberg, J., Perkins, S., Smith, L., Cairo, M. Feasibility and efficacy of immunochemotherapy for the treatment of mature B-cell leukemia: a report from the Children's Oncology Group. Presented, Fourth International Symposium on Childhood, Adolescent and Young Adult Non-Hodgkin's Lymphoma, 2012. British Journal of Haematology 2012; 159 (Supp. 1):44 #79.

38. Shiramizu, B., **Goldman, S.**, Smith, L., Harrison, L., Van de Ven, C., Gross, T., Sanger, W., Perkins, S., Cairo, M. Does minimal residual disease (MRD) predict relapse in children with advance B-NHL treated with chemoimmunotherapy: a Children's Oncology Group report. Presented, Fourth International Symposium on Childhood, Adolescent and Young Adult Non-Hodgkin's Lymphoma, 2012. British Journal of Haematology 2012; 159 (Supp. 1):53 #101.

39. **Goldman, S.**, Hochberg, J., Harrison, L., Kallis, M., Barth, M., Galardy, P.., Miles, R., Sanger, W., Shiramizu, B., Lim, M., Perkins, S.L., Hermiston, M., Frazer, J.K., Cairo, M.S. Reduced burde of oncologic therapy in advanced B-cell lymphoma (REBOOT ABLY) in children, adolescents and young adults with CD20+ mature B-cell lymphoma. Presented, Fourth International Symposium on Childhood, Adolescent and Young Adult Non-Hodgkin's Lymphoma, 2012. British Journal of Haematology 2012; 159 (Supp. 1):60 #118.

40. Barth, M.J., **Goldman, S.**, Smith, L., Perkins, S., Shiramizu, B., Gross, T.G., Harrison, L., Sanger, W., Geyer, M.B., Giulino-Roth, L., and Cairo, M.S. Rituximab pharmacokinetics in children and adolescents with de novo intermediate and advanced mature B-cell lymphoma/leukemia: A Children's Oncology Group (COG) report. Presented, American Society of Clinical Oncology. Journal of Clinical Oncology 2013;suppl, #3055.

41. Galardy, P.J., Hochberg, J., Perkins, S.L., **Goldman, S.**, Cairo, M.S. Rasburicase in the prevention of laboratory/clinical tumour lysis syndrome in children with advanced mature B-NHL: a Children's Oncology Group Report. British Journal of Haematology 2013 Nov; 163(3):365-72. doi:10.1111/bjh.12542. Epub 2013 Sep 6.

42. O'Connell, T.O., Yin, C., Barth, M., Miles, R., Ayello, J., Harrison, L., van de Ven, C., Galardy, P., **Goldman, S.C.**, Lim, M.S., Hermiston, M., McAllister-Lucas, L., Roth, L.G., Perkins, S., Lee, S., and Cairo, M.S. Ibrutinib (PCI-32765), a selective and covalent inhibitor of Bruton's tyrosine kinase, significantly inhibitis

cell proliferation and enhances apoptosis in Burkitt lymphoma (BL) and primary mediastinal B-cell lymphoma (PMBL): Ibrutinib may be a potential adjuvant therapy in the treatment of BL and/or PBML. Submitted, International Society of Pediatric Oncology (SIOP) Meeting 2014.

43. Yin, C., O'Connell, T., Ayello, J., Harrison, L., van de Ven, C., Barth, M., Miles, R., Galardy, P., **Goldman, S.C.**, Lim, M.S., Hermiston, M., McAllister-Lucas, L., Roth, L.G., Perkins, S., Lee, S., and Cairo, M.S. Ibrutinib, a selective and irreversible inhibitor of Bruton's tyrosine kinase, significantly inhibits cell proliferation and induces apoptosis in primary mediastinal B-cell lymphoma (PMBL): Ibrutinib may be a future targeted agent in combination therapy in patients with PMBL. Submitted, ASH Meeting on Lymphoma Biology, 2014.

44. Yin, C., O'Connell, T., Ayello, J., Harrison, L., van de Ven, C., Barth, M., Miles, R., Galardy, P., **Goldman, S.C.**, Lim, M.S., Hermiston, M., McAllister-Lucas, L., Roth, L.G., Perkins, S., Lee, S., and Cairo, M.S. Ibrutinib significantly alters cell proliferation and programmed cell death in Burkitt lymphoma (BL): Ibrutinib may be a potential adjuvant agent in the treatment of BL. Submitted, ASH Meeting on Lymphoma Biology, 2014.

Textbooks

1. Hochberg JC, **Goldman SC**. TLS and other emergencies. In **Hematological Malignancies in Children, Adolescents, and Young Adults**, MS Cairo, SL Perkins, Ed., World Scientific Publishing Co. Pte. Ltd., New Jersey. pp. 557-573, 2012.

2. Miles R, Galardy P, Giulino-Roth LG, **Goldman S**, and Cairo MS. Childhood and adolescent B-cell lymphoma: Bench to bedside and back to bench. In **Lost in Translation: Barriers to Incentives for Translational Research in Biomedical Sciences**, Srivastava R, Maksymowicz W, and Lopaczynski W, eds. World Scientific Publishing Co., Singapore, 2014, 2014, 247-286. ISBN 9789814489065.

BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors.
Follow this format for each person. **DO NOT EXCEED FIVE PAGES.**

NAME: M. MEAGHAN GRANGER, MD

eRA COMMONS USER NAME (credential, e.g., agency login):

POSITION TITLE: DIRECTOR, NEUROBLASTOMA PROGRAM, PEDIATRIC HEMATOLOGY/ONCOLOGY & STEM CELL TRANSPLANTATION

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable. Add/delete rows as necessary.)*

INSTITUTION AND LOCATION	DEGREE *(if applicable)*	Completion Date MM/YYYY	FIELD OF STUDY
Hendrix College, Conway, AR	B.A.	1988-1992	Biology
University of Arkansas Medical Sciences	M.D.	1992-1996	Medicine
Vanderbilt University	Resident	1996-1999	Pediatrics
Northwestern University	Fellow	1999-2000	Hematology/Oncology
University of Texas - Southwestern	Fellow	2000-2003	Hematology/Oncology

A: Personal Statement

My professional work has focused over the past 13 years on clinical care and clinical research primarily regarding pediatric patients with high-risk neuroblastoma and retinoblastoma. For the last 7 years, my clinical research has centered on myeloablative therapy for high-risk neuroblastoma through the Children's Oncology Group (COG). I have been an active member of the Neuroblastoma Committee and serve on the Neuroblastoma Steering Committee and the High-Risk Neuroblastoma Task Force. I have been a study chair for a COG group-wide clinical trial that is now successfully completed and being analyzed. I have served as the COG Principal Investigator for my local institution since 2008. My mission has been to facilitate access of patients with high-risk neuroblastoma to novel agents and through that I lead the NANT clinical trials project at Cook Children's. Cook Children's has contributed significantly to enrollment on NANT studies and is one of the top-enrolling institutions within the consortium. I am involved in protocol development on the NANT Scientific Research Council in development of Phase I and II studies for advanced neuroblastoma. I also have an interest in study conduct and serve as the Audit Chair for NANT and am a long time member of my local IRB. I hope to expand our delivery of novel agents to patients with all types of pediatric cancer through participation in the Phase I consortium.

B. Positions and Honors

Children's Oncology Group, Institution Principal Investigator, August 2007 - present
Committee Participation:

- Stem Cell Transplant
- Retinoblastoma, ARET0321
- Neuroblastoma, ANBL12P1, Study Chair, "Pilot Study Using Myeloablative Busulfan/Melphalan (BuMel) Consolidation Following Induction Chemotherapy for Patients with Newly Diagnosed High-Risk Neuroblastoma

- NBL Steering Committee member, 2012 – present

NANT Consortium Principal Investigator, August 2008 - present

- Study Co-Chair NANT 2011-01, "Randomized Phase II "pick the winner" trial comparing single-agent ^{131}I-MIBG vs. vincristine/Irinotecan/^{131}I-MIBG vs. vorinostat/^{131}I-MIBG for patients with relapsed or refractory neuroblastoma"
- NANT Consortium Audit Committee Co-Chair, June 2009 - present

Cook Children's Medical Center Activities:

- IRB Member, 2007 to present
- Tissue Committee, 2003 - 2010
- Graduate Medical Education Committee, 2008 - present
- Experience the Mission participant, 2007 - present
- Research Development Committee, 2003 – 2005

C: Contribution to Science

1. Myeloablative therapy for neuroblastoma has shown to improve overall and event-free survival. In this paper we studied the use of a tandem myeloablative preparative regimen for high-risk neuroblastoma in order to assess whether this more intensive regimen was feasible. The toxicity profile was comparable and the study was deemed feasible. This study laid the ground for a subsequent COG study which randomized tandem transplant to single transplant and found tandem transplant to have superior outcomes.

   Granger M, Grupp SA, Kletzel M, Naranjo A, London WB, Diller L. "Feasibility of a tandem autologous peripheral blood stem cell transplant regimen for high risk neuroblastoma in a cooperative group setting: a Pediatric Oncology Group study: a report from the Children's Oncology Group. Pediatr Blood Cancer. 2012 Nov; 59 (5):902-7. doi: 10.1002/pbc.24207. Epub 2012 Jun 28

   Granger MM, Yanik G, Naranjo A, et al: Myeloablative busulfan/melphalan (BuMel) consolidation following induction chemotherapy for patients with high-risk neuroblastoma: A Children's Oncology Group (COG) study. American Society of Clinical Oncology Annual Meeting 34 suppl; abstr 10528, 2016

2. In addition to the work above. myeloablative doses of 131I-MIBG have been successful in high-risk neuroblastoma. I developed an 131I-MIBG program at my institution and have continued to contribute knowledge to this field through our multidisciplinary program.

   Sarah French, Steven DuBois, Biljana Horn, Meaghan Granger, Randall Hawkins, Amy Pass, Ellen Plummer,Katherine Matthay. "^{131}I-MIBG followed by Consolidation with Busulfan, Melphalan and Autologous Stem Cell Transplantation for Refractory Neuroblastoma" Pediatric Blood and Cancer. 2013 May; 60(5):879-84. doi: 10.1002/pbc.24351. Epub 2012 Sep 28

   Miguel de la Guardia, Steven McCammon, Karen Nielson, Meaghan Granger. "Administration of ^{131} I Metaiodobenzylguanidine Using the Peristaltic Infusion Pump Method." J. Nucl. Med. Technol. 2014 Jun;42(2):109-13. doi: 10.2967/jnmt. 114. 139832. Epub 2014 May 5

   DuBois SG, Granger, MM, Matthay K. et al. Phase I Study of Vorinostat as a Radiation Sensitizer with 131I-Metaiodobenzylguandine (131I-MIBG) for Patients with Relapsed or Refractory Neuroblastoma. Clin Cancer Res. 2015 Jun 15;21(12):2715-21. doi: 10.1158/1078-0432.CCR-14-3240. Epub 2015 Feb 18.

3. Novel agents for high-risk neuroblastoma.

Michael Pranzatelli, MD., Elizabeth D. Tate, C-FNP, MN, Steven J. Verhulst , PhD, Salvatore Bertalone, MD, Deepika Bhatla, MD, Meaghan Granger, MD, Joseph Lebowizc, MD, Sharon Lockhart, MD, Joseph M. Wiley, MD, "Pediatric Dosing of Rituximab Revisited: Serum Concentrations in Opsoclonus-Myoclonus Syndrome." J Pediatr Hematol Oncol. 2010 Jul; 32(5):e167-72. doi: 10.1097/MPH. 0b013e3181cf0726

Hematology Oncol J Pediatr Hematol Oncol. 2010 Jul; 32(5):e167-72. doi: 10.1097/MPH. 0b013e3181cf0726

Barry James Maurer, Julia Lynne Glade Bender, Min Hee Kang, Judith Villablanca, Denice Wei, Susan G. Groshen, Shenping Yang, Scarlett Czarnecki, Meaghan P. Granger, Howard M. Katzenstein, Brian D. Weiss, Katherine K.Matthay, C. Patrick Reynolds, Araz Marachelian. "Fenretinide (4-HPR)Lym-X-Sorb (LXS) oral powder plus ketoconazole in patients with high-risk (HR) recurrent or resistant neuroblastoma: A New Approach to Neuroblastoma Therapy (NANT) Consortium trial. J Clin Oncol 32:5s, 2014(suppl; abstr 10071)

Quantifying Expression of Five Neuroblastoma-Associated Genes in Bone Marrow (BM) and Blood of Patients with Refractory or Relapsed Neuroblastoma (NB) Improves Assessment of Disease Status and of Disease Progression Risk. A New Approaches to Neuroblastoma Araz Marachelia, Granger, Robert C, Seeger, Poster presented at ANR cologne conference 2014

Olivia M. Padovan-Merhar, Pichai Raman, Irina Ostrovnaya, Karthik Kalletia, Kaitlyn R. Rubnitz, Eric M. Sanford, Siraj M. Ali, Vincent A. Miller, Yael P. Mosse, Meaghan P. Granger, Brian Weiss, John M. Maris, Shakeel Modak. "Enrichment of targetable mutations in the relapsed neuroblastoma genome." PLOS Genetics - Accepted for publication December 2016

4. Early research

M.M. Petty, A. Cmelak, MD., Johnson, R. Sinatra, and M.T. Jennings. Chapter in Textbook of Uncommon Cancer.Edited by D. Raghaven, et al. 1999 John Wiley and Sons Ltd.

Meaghan P. Granger, Woodring E. Wright, Jerry W. Shay. Telomerase in Cancer and Aging. *Critical Reviews in Hematology/Oncology,* 41(1): 29-40, 2002.

D: Research Support

2009-2016	Neuroblastoma "Walk for a Cure" Cumulative, over $1,000,000
2009-2014	New Year of Hope - The Caleb Larson Foundation; $65,000
2014	Wipe Out Kids Cancer, Cancer Research Scholar; $75,000
2011	Wipe Out Kids Cancer, Cancer Research Scholar; $75,000
2010	Hyundai Hope on Wheels, Cancer Research Scholar; $40,000
2010	WOKC, Spirit of Little Mo Award; Health and Medical Achievement Award
2009	St. Baldrick's Foundation Infrastructure Grant. Neuroblastoma Program Development; $50,000
2009	Cancer Research Foundation of North Texas. "Lipoprotein Nanoparticles: An Innovative Drug Delivery System for Neuroblastoma." $15,000
2002	Children's Medical Center Clinical Research Targeted Grant: *Telomere Sensitivity to Oxidative Stress in Down Syndrome.* January 2002-December 2002
2002-2004	NIH Pediatric Research Loan Repayment Program, *Telomere Inhibitors in Combination with Chemotherapy in Human Solid Tumors,* July 2002 – June 2004

BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors.
Follow this format for each person. **DO NOT EXCEED FIVE PAGES.**

NAME: Harrod, Virginia L.

eRA COMMONS USER NAME (credential, e.g., agency login):

POSITION TITLE: Director, Pediatric Neuro-Oncology, Children's Blood and Cancer Center

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable. Add/delete rows as necessary.)*

INSTITUTION AND LOCATION	DEGREE *(if applicable)*	Completion Date MM/YYYY	FIELD OF STUDY
The University of Texas, Austin, Texas	BS	12/1992	Chemistry
MD Anderson Cancer Center, The University of Texas Health Science Center at Houston, Graduate School of Biomedical Sciences, Houston, Texas	PhD	08/1998	Pharmacology and Toxicology
The University of Texas Medical School, Houston, Texas	MD	06/2001	Medicine
Children's Hospital of Austin, Austin Medical Education Programs, Austin, Texas		06/2004	Internship and Residency in Pediatrics
St. Jude Children's Research Hospital, Memphis, Tennessee		06/2007	Fellowship in Pediatric Hematology Oncology

A. Personal Statement

I have always had a strong interest in oncology research, completing my PhD at MD Anderson Cancer Center and hoping to find cures for this devastating disease. After working in pre-clinical research, I knew I wanted to expand my experience to bring the latest developments directly to the patients, which led to my decision to attend medical school.

After completing my fellowship training at St. Jude Children's Research Hospital, I joined The Children's Blood and Cancer Center at Dell Children's Medical Center (DCMC). During my first few years at DCMC, I served as medical director of the Survivorship Clinic. The pediatric cancer population is often overlooked, but as we learned more about late effects, it became obvious that although these patients were surviving, they were not thriving. As medical director, I was intimately involved in the development and implementation of a research project looking at exercise intervention in this patient population. The program and results were highly successful and have been presented at national meetings. The program continues at our center and children are healthier because of it. We have also been able to collaborate with MD Anderson Cancer Center and Texas Children's Hospital with the research project, Passport for Care, which is a web-based survivorship system that guides cancer survivors and their healthcare providers in making lifelong healthcare decisions and anticipate late effects of their particular treatments to maximize their quality of life.

My experience with the Survivorship Clinic allowed me to gain experience in leadership, which led me to my current position as chief of Pediatric Neuro-Oncology. More patients are being cured of disease than ever before, however, there are still many patients, especially in neuro-oncology, whose survival chances

are dismal even at the time of diagnosis. This is an area of research still looking for the elusive cure. I have been intimately involved with the conception, growth and development of the Neuro-Oncology Program here at Dell Children's Medical Center. We have developed an interdisciplinary team of surgeons, radiologists, radiation oncologists, pathologists and oncologists to provide the highest level of care to our patients with brain tumors. With this program development, we have been able to successfully collaborate with the Texas Oklahoma Pediatric Neuro-Oncology Consortium (TOPNOC) and the Neuroblastoma Medulloblastoma Translational Research Consortium (NMTRC). These collaborations allow us to develop and participate in research trials that directly benefit our patients here at home without travelling to outside institutions.

B. Positions and Honors

Positions and Employement

2001 – 2004	Pediatric Resident, Children's Hospital of Austin, Austin Medical Education Programs, Austin, Texas
2004 – 2007	Hematology Oncology Clinical Fellow, St. Jude Children's Research Hospital, Memphis, Tennessee
2007 – present	Physician, Dell Children's Medical Center of Central Texas, Austin, Texas
2008 – 2011	Medical Director, LIVE**STRONG** Survivorship Center, Dell Children's Medical Center of Central Texas, Austin, Texas
2011 – present	Chief of Neuro-Oncology, Children's Blood and Cancer Center, Dell Children's Medical Center of Central Texas, Austin, Texas

Professional Memberships

2001 – present	American Academy of Pediatrics
2004 – present	American Society of Hematology
2004 – present	American Society of Pediatric Hematology Oncology
2007 – present	Children's Oncology Group
2013 – present	Society of International Pediatric Oncology

Professional Reviewer

2014	Abstract Reviewer – International Society of Pediatric Neuro-Oncology meeting
2015 – present	Journal Reviewer – Pediatric Neurosurgery

Committee Memberships and Positions

Austin Medical Education Programs
- President, Resident's Association, 2002-2004
- Vice President, Resident's Association, 2001-2002
- Member, Transitional Program Committee 2002-2004
- Member, Competency Review Board, 2003-2004
- Academic Curriculum Committee, 2002-2004
- Recruiting Committee, 2002-2004

St. Jude Children's Research Hospital
- Resident Liaison, LeBonheur Children's Medical Center, 2005-2007
- Organizer, Biostatistics-Oncology Journal Club, 2005-2007
- Member, Postdoctoral Executive Council, 2005-2006

Dell Children's Medical Center, Children's Blood and Cancer Center
- Performance Improvement Chair, Dell Children's Pediatric Cancer Committee, 2008-2010
- Medical Director, LIVE**STRONG** Survivorship Center, 2008-2011
- Specially for Children Finance Committee member, 2006-2012
- Member, Medical Performance Evaluation Council – 2013 - 2015
- Chief of Neuro-Oncology - 2011-present

Certifications

2005 – 2015 Board Certification, American Board of Pediatrics
2009 – present Board Certification, American Board of Pediatrics – Pediatric Hematology/Oncology

Honors and Awards

President's Council of Advisors on Science and Technology, 1991
Golden Key National Honorary Society, 1992
American Legion Auxiliary Fellow, 1995-1998
Outstanding Resident of the Year, 2003-2004
Paul Harris Fellow Recipient, 2004
Young Investigator's Award-Hemophilia and Thrombosis Research Society, April 2007
Lemuel Diggs Endowed Fellowship in Sickle Cell Research Recipient, 2005-2007
Hyundai Hope on Wheels Pediatric Cancer Research Award: 2008, 2010, 2011, 2012
Killeen Independent School District Distinguished Alumni, 2015

.

C. Contribution to Science

Most of my career has been in the development of programs focused on patient care . I am very proud of the fact that survivors of pediatric cancers are able to receive care here locally with the development of the Survivorship Center at DCMC. Prior to that development, patients were typically lost to follow up care, despite the fact that they are at extremely high risk for health related issues secondary to their exposures to medications as children.

Currently, with the development of the Neuro-Oncology Program and associated collaborations, we continue to increase the amount of research done for patients with brain tumors. Our program's ongoing research collaborations are examining genomic profiling and development of personalized patient care in treating cancer.

My past research areas focused on hematology, and one of the most impactful studies was our efforts on treating thrombosis with low doses of thrombolytic therapy.

Leary SE, Harrod VL, de Alarcon PA, Reiss UM. Low dose systemic thrombolytic therapy for deep vein thrombosis in pediatric patients*. J Pediatr Hematol Oncol*. 2010 Mar; 32(2):97-102.

Harrod VL, Howard T, Abboud M, Hankins M, Lobo C, Ware RE. Chemical and Functional Analysis of Generic Hydroxyurea Formulations. *Pediatr Hematol Oncol*. Jun;25(5):423-9, 2008.

Harrod VL, Howard TA, Zimmerman SA, Dertinger SD, Ware RE. Quantitative Analysis of Howell-Jolly Bodies in Patients with Sickle Cell Disease. *Experimental Hematology* 35 (2):179-183, 2007.

Cherif A, Wallace S, Yang DJ, Newman RA, Harrod VL, Nornoo A, Inqoue T, Kim CG, Kuang L-R, Kim EE, Podoloff DA. Development of new markers for hypoxic cells: [^{131}I]iodomisonidazole and [^{131}I]iodoerythronitromisonidazole. *Journal of Drug Targeting* 4:31-39, 1996.

D. Research Support

1. **Texas Oklahoma Pediatric Neuro-Oncology Consortium** (variable funding depending on patients enrolled)
   a. Epidemiologic Studies of Childhood Cancers
   Project goals: Investigate epidemiological characteristics of patients with pediatric brain tumors to identify risk factors of cancer development and outcomes.
   Role/Responsibility: Primary Investigator

b. Molecular Characterization of Pediatric Brain Tumors

Project goals: Comprehensively characterize the genetic alterations occurring in pediatric brain tumors to determine if the role of the alterations in tumor development and progression and to identify targets for novel diagnostics and therapeutics.
Role/Responsibility: Primary Investigator

2. **Neuroblastoma Medullloblastoma Translational Research Consortium** (variable funding depending on patients enrolled)
Role: Primary Investigator

a. NMTRC-012: Pediatric Precision Laboratory Advanced Neuroblastoma Therapy

Project goals: Characterize molecular targets in newly diagnosed neuroblastoma patients to identify and implement patient specific medications in the treatment protocol to improve outcomes for patients.
Role/Responsibility: Primary Investigator

b. NMTRC-014: DFMO utilization patients with Relapsed/Refractory Neuroblastoma

Project goals: Utilization of DFMO as a maintenance phase therapy for high risk neuroblastoma to minimize the relapse rate and increase survival.
Role/Responsibility: Primary Investigator

BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME	POSITION TITLE
Hartman, Lisa Louise Rubin	Assistant Professor of Pediatrics, Hematology/Oncology
eRA COMMONS USER NAME (credential, e.g., agency login) LRHARTMAN	

EDUCATION/TRAINING *(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)*

INSTITUTION AND LOCATION	DEGREE *(if applicable)*	MM/YY	FIELD OF STUDY
Pomona College, Claremont, CA	B.A.	05/01	Neuroscience
University of Texas Medical Branch, Galveston	M.D.	06/05	
Albert Einstein College of Medicine, Bronx, NY	Residency	06/08	Pediatrics
University of California, San Diego, La Jolla, CA	Fellowship	09/12	Pediatric Hematology/Oncology
University of California, San Diego, La Jolla, CA	M.A.S.	12/12	Clinical Research

A. Positions and Honors

Positions and Employment

2005-2008 Resident, Division of Social Pediatrics, Montefiore Medical Center, Bronx, NY
2008-2009 Staff Physician, Acute Kids Urgent Care, Dallas, TX
2009 Volunteer Physician and Medical Staff Director, Heart to Heart International, Guatemala
2009-2012 Fellow, Division of Pediatric Hematology/Oncology, University of California, San Diego
2010-2012 Post-doctoral Scholar, Cancer Therapeutics Training Program, University of California, San Diego
2012- Assistant Professor, Pediatrics, Hematology/Oncology, Texas Tech University Health Sciences Center, El Paso Children's Hospital
2013- Pediatric Residency Hematology/Oncology Rotation Director, Department of Pediatrics, Texas Tech University Health Sciences Center, El Paso Children's Hospital

Other Experience and Professional Memberships

2002-2012 Member, American Academy of Pediatrics
2009- Member, American Society of Pediatric Hematology/Oncology
2009-2012 Member (training), Children's Oncology Group
2009-2012 Member, UCSD Graduate Medical Education Committee
2009-2011 Medical Staff Physician Volunteer, Hemophilia and Oncology Resident Camps, Julian, CA
2010- Member, American Association of Cancer Research
2010-2012 Member, Pediatric Hematology/Oncology Fellowship Recruitment Committee
2013- Medical Staff Physician Volunteer, Oncology Resident Camp, Cloudcroft, New Mexico
2013- Medical Staff Physician Volunteer, Camp Young Judaea, Wimberley, Texas
2013- Member, Pediatric Resident Clinical Competency Committee, Texas Tech University Health Sciences Center
2014- Member, Institutional PI, Voting Body, Children's Oncology Group
2014- Member, Women in Medicine and Science (WIMS), Texas Tech University Health Sciences Center
2015- Member, Pediatric Research Committee, El Paso Children's Hopsital

2016- Member, Cancer Prevention & Research Institute of Texas (CPRIT) Advisory Committee on Childhood Cancer

B. Peer-reviewed Publications

Hartman LL, Crawford JR, Makale MT, Milburn M, Joshi S, Salazar AM, Hasenauer B, Vandenberg SR, MacDonald TJ, Durden DL. Pediatric Phase II Trials of Poly0ICLC in the Management of Newly Diagnosed and Recurrent Brain Tumors. *Journal of Pediatric Hematology/Oncology*, Volume 36(6), August 2014. http://www.ncbi.nlm.nih.gov/pubmed/24309609

Abstract/Posters

2012 A Phase II Trial of Poly-ICLC in the Management of Newly Diagnosed and Recurrent Pediatric Brain Tumors, AACR National Meeting

2013 Integration of a Personalized Molecular Targeted Therapy into the Multimodal Treatment of Refractory Early Childhood sPNET, AACR Pediatric Cancer at the Crossroads Meeting

C. Other Information Relevant to the current application

1. Children's Oncology Group Institutional PI for 20 open and enrolling clinical trials at El Paso Children's Hospital, 2014- ongoing
2. "A Phase II Trial of Poly-ICLC in the Management of Recurrent or Progressive Pediatric Low Grade Gliomas." Clinical Trial at Rady Children's Hospital/UCSD, 2010-2012.
3. "A Phase Ib Study of Sorafenib with Vinblastine in Children with Recurrent or Refractory High Risk Neuroblastoma." Clinical Trial at Rady Children's Hospital/UCSD, 2010-2012.
4. Testing of novel combinations of therapeutic agents in neuroblastoma tissue culture cell lines and *in vivo* mouse models. Research in the Durden Laboratory, Moores Cancer Center/UCSD, 2010-2012.
5. CPRIT Molecularly Targeted Therapy for Soft Tissue Sarcoma in Texas-Biospecimen Banking Protocol (Leavey, Patrick) 3/1/15-8/30/2017, Institutional PI at Texas Tech University Health Sciences Center El Paso
6. 5 T32 CA121938-03 (Howell, Stephen) 07/01/2006 - 06/30/2011,10/1/2011- NIH-NCI NCI UCSD Cancer Center Training Program in Drug Development - *Cancer Therapeutics Training (CT^2) Program.* Ruth L. Kirschstein National Research Service Award (NRSA)
   Major goals: The goal of this training program is to train post-doctoral scientists and physician scientists for careers in academic cancer research, with an emphasis on translational research targeted at developmental therapeutics. Role: Post-doctoral Scholar, 2010 - 2012

Personal Statement

I am an assistant professor of pediatrics in the division of hematology/oncology at Texas Tech University Health Sciences Center at El Paso (TTUHSC) and the El Paso Children's Hospital. My fellowship research as part of the Cancer Therapeutics Training (CT^2) Program at the University of California, San Diego focused on the development of novel cancer therapeutics for refractory pediatric solid tumor patients. Conducted in the laboratory of Donald Durden, MD, PhD, I tested targeted therapeutic agents in preclinical pediatric tumor models with the goal of developing new phase I and II clinical trials for refractory solid tumors.

As a fellow, I wrote and handled the day-to-day operations of a Phase 1b clinical trial of vinblastine with dose escalation of Sorafenib, a multitargeted tyrosine kinase inhibitor, in patients with recurrent or refractory solid tumors and a Phase II clinical trial of Poly-ICLC in pediatric recurrent low grade gliomas. I also performed biomarker analysis to correlate tumor response with activation states of key proteins involved in the signaling pathways of tumor growth and cell survival in patients treated with these therapy regimens. My research also used genomic analysis of tumor tissue in order to explore signaling pathways to guide tumor response to future targeted therapies. A second aspect of my research involved preclinical *in vitro* and *in vivo* testing of novel drug combinations aimed to identify potential therapies for patients with recurrent high risk solid tumors, especially focusing on PI-3 kinase inhibitors for pediatric clinical use.

Following completion of my pediatric hematology/oncology fellowship and CT^2 training, I started my career as a clinician and clinical trialist at an academic medical center, Texas Tech University Health Sciences Center at El Paso, where I have been focusing my efforts to care for patients in this unique international border city. I have completed my Master's in Clinical Research as well as graduated from the Faculty Development Course at TTUHSC. My research interests include clinical research trials and novel therapeutics for pediatric oncology patients. I am the institutional PI for a CPRIT statewide soft tissue sarcoma banking protocol. I have embraced the role of Principal Investigator for our institution in Children's Oncology Group in order to advance the care for our local patients and contribute to the growing body of pediatric oncology research. I am especially interested in the health disparities seen in our Hispanic patients along the border region and am excited to collaborate on the Individualized Tumor Analysis Center of Texas (INPACT) and the CPRIT-sponsored Adolescent and Childhood Cancer Epidemiology and Susceptibility Service (ACCESS) grants.

November 22, 2016

CURRICULUM VITAE

NAME: BARKAT HOODA, MD

PRESENT POSITION AND ADDRESS:
Director, Division of Hematology Oncology &
Associate Professor of Pediatrics
The University of Texas Medical Branch
301 University Boulevard
Galveston, Texas 77555-0361

BIOGRAPHICAL:
Permanent Residence United States

EDUCATION:

NEURO-ONCOL FELLOWSHIP 07/1999 - 02/2000	Fellow Pediatric Neuro-oncology & Clinical Instructor, Department of Pediatrics New York University School of Medicine New York, New York
FELLOWSHIP 07/1996 - 06/1999	Fellow, Pediatric Hematology Oncology Brown University School of Medicine Hasbro Children's Hospital Providence, Rhode Island
RESIDENCY 07/1993 - 06/1996	Resident, Department of Pediatrics University of Florida College of Medicine Shands Children's Hospital Gainesville, Florida
INTERNSHIP 11/1991 – 10/1992	Rotating Internship Department of Pediatrics (6 months) and OBGYN (6 months) Aga Khan University Hospital Karachi, Sind, Pakistan
MEDICAL SCHOOL 09/1986 – 08/1991	Bachelor of Medicine & Bachelor of Surgery (MBBS) Aga Khan University Hospital Karachi, Sind, Pakistan

US BOARD CERTIFICATIONS:

03/30/2009 - 12/31/2016	American Boards of Pediatric Hematology Oncology
10/09/1996 -12/31/2012	American Board of Pediatrics

CRRENT LICENSURE/S:

05/01/2013 – 05/31/2017	Texas State Medical Board License, P5993
07/09/2010 – 05/31/2017	Texas Department of Public Safety (DPS), 60174009
08/28/2009 – 10/31/2018	Drug Enforcement Administration (DEA), FH1623912

PAST LICENSURE/S:

07/01/2012 – 04/30/2013	Texas State Medical Board License, FT43972
07/01/2011 – 07/01/2012	Texas State Medical Board License, FT43482
07/01/2010 – 07/01/2011	Texas State Medical Board License, FT43093
08/01/2009 - 08/31/2012	New York State Education Department

GRANTS/HONORS:

10/26/16	Nominated as Lead Pediatric Hematologist Oncologist *** Cancer Prevention and Research Institute of Texas (CPRIT) Advisory Committee on Childhood Cancer (ACCC) Austin, Texas
09/14/15	Presidential Citation from Dr David L Callender Completion of Tenure as Faculty Senator University of Texas Medical Branch at Galveston Galveston, Texas
06/19/15	Presidential Citation from Dr David L Callender Completion of 5 years of dedicated service University of Texas Medical Branch at Galveston Galveston, Texas
12/2014	Nomination for John P. McGovern Academy of Oslerian Medicine Excellence in Clinical Teaching Award University of Texas Medical Branch at Galveston Galveston, Texas
06/1999	Faculty Fellow of the Year Award, (US $50,000) Children Brain Tumor Foundation Inc., New York New York University School of Medicine New York, New York

PROFESSIONAL WORK HISTORY AND TEACHING EXPERIENCE:
(Academic & Non-academic Appointments)

ASSOCIATE PROFESSOR OF PEDIATRICS 09/01/14 – Present	Associate Professor of Pediatrics & Director, Division of Pediatric Hematology Oncology University of Texas Medical Branch at Galveston Galveston, Texas
ADJUNCT ASSOCIATE PROFESSOR OF PEDIATRICS 07/01/14 – 06/30/17	Adjunct Associate Professor of Pediatrics Baylor College of Medicine One Baylor Plaza Houston, Texas
CLINICAL ASSOCIATE PROFESSOR OF PEDIATRICS 07/19/10 – 08/31/14	Clinical Associate Professor of Pediatrics & Director, Division of Pediatric Hematology Oncology University of Texas Medical Branch at Galveston Galveston, Texas
GENERAL PEDIATRICIAN 10/2009 – 06/2010	Private Practice in General Pediatrics& Adolescent Medicine Southern Tier Pediatrics Jamestown, New York
CONSULTANCY IN KINGDOM OF SAUDI ARABIA II 01/2008 – 08/2009	Consultant Pediatric Hematologist Oncologist & Neuro-oncologist JCIA accredited Saad Specialist Hospital Al-Khobar, Kingdom of Saudi Arabia (KSA)
CONSULTANCY IN KINGDOM OF SAUDI ARABIA I 05/2006 – 12/2007	Consultant Pediatric Oncologist & Neuro-oncologist in the MOH King Fahad Specialist Hospital Dammam, Kingdom of Saudi Arabia (KSA)
ASSISTANT PROFESSSOR 05/2004 – 05/2006	Senior Assistant Professor of Pediatrics & Consultant Hematologist Oncologist and Neuro-oncologist Aga Khan University (AKU) Hospital (AKUH) & School of Medicine Karachi, Pakistan
SENIOR LECTURER 03/2000 – 04/2004	Consultant Pediatric Oncologist & Clinical Senior Lecturer Wellington Children's Hospital & Wellington School of Medicine University of Otago Wellington, New Zealand

NB: PLEASE NOTE THAT SENIOR LECTURER IS A BRITISH/EUROPEAN EQUIVALENT OF A FACULTY APPOINTMENT AS AN ASSISTANT PROFESSOR IN THE US SYSTEM

RESEARCH ACTIVITIES:

ACADEMIC GOALS	To serve in the Administration/Dean's Office for Academic &/or Clinical Affairs & Actively participate in UTMB in its collaboration with MD Anderson Cancer Center – Long Term Full Tenured Professorship by 2018 – Mid term To serve in the Institutional Review Board (IRB) – Short term (Ongoing) To serve as SOM Senator in UT Faculty Senate – (Tenures Completed)
AREAS OF CLINICAL INTEREST	Children's Oncology Group (COG) & Clinical Trials Childhood Leukemia, Neuro-oncology, Solid Tumors, Hematology & Supportive Care including Transfusion Safety & Blood Management Program
AREAS OF TEACHING INTEREST	Graduate & Post Graduate Medical Education Medical Curriculum Development Professional Communication & Risk Management Nursing and Nurse Practitioner's Training and Teaching
PRINCIPAL INVESTIGATOR II 07/2010 – Present	Principal Investigator Designate for Clinical Trials & COG Membership Pediatric Oncology Service University of Texas Medical Branch Galveston, Texas
PRINCIPAL INVESTIGATOR I 08/2002 – 04/2004	Principal Investigator for Clinical Trials & COG Membership Central Regions Pediatric Oncology Service Wellington Children's Hospital Grant through New Zealand Ministry of Health & Wellington Cancer Center Wellington, New Zealand

COMMITTEE RESPONSIBILITIES:
NB: International Career

INTERNATIONAL RECOGNITION

2006	Listed as Lead Pediatric Oncologist for Wellington, New Zealand in the 2006 edition of Principles & Practice of Pediatric Oncology by Pizzo & Poplack Wellington, New Zealand

NATIONAL

10/26/16	Nominated as Lead Pediatric Hematologist Oncologist *** Cancer Prevention and Research Institute of Texas (CPRIT) Advisory Committee on Childhood Cancer (ACCC) Austin, Texas
07/2005	General Secretary Nominee and Founding Member of the Pakistan Society of Pediatric Oncology (PSPO) Pearl Continental Hotel Karachi, Pakistan
08/2000 – 04/2004	Wellington representative in the National Pediatric Oncology Steering Group (POSG), Wellington, New Zealand Co Author of Service Specification Document Co-Director of Pediatric Oncology Protocol Development and Supportive Care work-stream Project
08/2000 – 04/2004	Projects participated for POSG included National Child Cancer Registry National Pediatric Oncology Updates and Telepediatrics Portfolio at the Ministry of Health Level New Zealand
07/1999 - 02/2000	Children's Brain Tumor Foundation 2009 Faculty Fellow of the Year Award & Clinical Instructor, Department of Pediatrics Experience included High Dose Chemotherapy and Autologous Stem Cell Transplantation New York University School of Medicine New York, New York

DEPARTMENTAL

11/09//2016	*Participated as* Core Faculty of Department of Pediatrics*** Meeting with the ACGME delegation visiting UTMB University of Texas Medical Branch Galveston, Texas
02/2016-Present	*Member,* Chair's committee for Collaboration of UT Health Pediatric oncology services with MD Anderson Cancer Center University of Texas Medical Branch Galveston, Texas

2012-2016	Author of Pediatric Hematology Oncology Guidelines for the UTMB Residents & Students – 7th Revision in 2016 Division of Pediatric Hematology Oncology University of Texas Medical Branch at Galveston Galveston, Texas
07/2013-06/2016	*Mentor,* PL1/2/3 Resident (Shajeer Noorudeen) School of Medicine University of Texas Medical Branch Galveston, Texas
07/19/2010 - Present	Director, Division of Pediatric Hematology Oncology Service & Associate Professor, Department of Pediatrics University of Texas Medical Branch at Galveston Galveston, Texas
01/12/2012 - Present	Founding Chair, Pediatric Hematology Oncology Tumor Board Division of Pediatric Hematology Oncology University of Texas Medical Branch at Galveston Galveston, Texas
3/2006	Appointed as Acting Chief of Pediatric Oncology Service Aga Khan University (AKU) Hospital & School of Medicine Karachi, Pakistan
2005	Appointed as Co-director of Pediatric Hematology Oncology Fellowship Aga Khan University (AKU) Hospital & School of Medicine Karachi, Pakistan
2004	Author of Pediatric Hematology Oncology Manual for the Residents Wellington Cancer Center Wellington, New Zealand Principal Investigator of the grant from Child Cancer Foundation to establish Pediatric Oncology Research section in the Clinical Research Unit of Wellington Cancer Centre, Total grant award NZ $20,000/yr. Wellington, New Zealand

SOM & HOSPITAL

11/03/2016	Author of Elective Course description (PEDU-4007) for MS3 wishing to do Clinical Rotation in the Division of Hematology Oncology School of Medicine University of Texas Medical Branch at Galveston Galveston, Texas

02/15/2016	Author of Elective Course description (PEDU-4053) for MS4 wishing to do Acting Internship (AI) in the Division of Hematology Oncology School of Medicine University of Texas Medical Branch at Galveston Galveston, Texas
01/29/2016-Present	*Interviewer,* 3+4 BS/MD program University of Texas Medical Branch Galveston, Texas
03/12/2015	Participated as Director of *Heme/Onc Subspecialty in the Institutional Review of General Pediatrics* Program by External Reviewers University of Texas Medical Branch Galveston, Texas
05/01/2015 - 05/30/2015	*Mentor,* MS1 Shadowing (Jessica Michalak MS1) School of Medicine University of Texas Medical Branch Galveston, Texas
09/01/2013 – 08/31/2015	*Senator*, *SOM representative in UT System Faculty Senate* University of Texas Medical Branch Galveston, Texas
09/01/2012 – Present	*Member, Institutional Review Board # 1* Human Subjects Protection Program University of Texas Medical Branch Galveston, Texas
01/24/2013 – 01/24/2014	*Physician Opinion Leader in the Strategic Health Care Group* UTMB Blood Management/Transfusion Safety Officers Program Meeting University of Texas Medical Branch Galveston, Texas
10/2012-Present	*Interviewer,* Pediatric Residency Training Program University of Texas Medical Branch Galveston, Texas
07/2012-Present	*Mentor,* Design a Case School of Medicine University of Texas Medical Branch Galveston, Texas
04/19/2012	*Internal Expert in the Institutional Review of Radiation Oncology* Program University of Texas Medical Branch Galveston, Texas

01/2012-Present	*Chair* of the Institutional Pediatric Oncology Tumor Board University of Texas Medical Branch Galveston, Texas
12/2010-Present	*Member,* Institutional Hematopathology Lymphoma Tumor Board University of Texas Medical Branch Galveston, Texas
11/2010-Present	*Interviewer*, Pediatric Faculty Recruits University of Texas Medical Branch Galveston, Texas
10/2010-Present	*Member*, Interviewing panel for Medical School Admissions University of Texas Medical Branch Galveston, Texas
10/2010-Present	*Reviewer & Teacher*, History taking and Physical Examination School of Medicine University of Texas Medical Branch Galveston, Texas
08/2010 - Present	*Member and Auditor, Hospital Transfusion Committee* University of Texas Medical Branch Galveston, Texas
07/2010-Present	*Member, Institutional Neuro Oncology Tumor Board* University of Texas Medical Branch Galveston, Texas
2006	*Member,* CEO led delegation from King Fahad Specialist Hospital that visited major medical facilities in three different cities of Pakistan
2006	*Pediatric oncologist representative,* Oncology Task Force formed for the creation of brand new Cancer Centre opened in 2006 Aga Khan University Hospital (AKUH) Karachi, Pakistan
2004 - 2006	*Departmental Director* for two years for Quality Assurance/JCIA/ & ISO 9001 related activities that contributed towards JCIA certification of the AKUH
2006	*Author,* End of Life Care Policy for the AKUH for JCIA accreditation purpose Aga Khan University Hospital (AKUH) Karachi, Pakistan

2006	*Participant* in the video program on AKUH's clinical policies & protocols shown to the entire faculty in the auditorium in preparation for the successful JCIA accreditation survey Aga Khan University Hospital (AKUH) Karachi, Pakistan
2005	*National radio talk show guest* on 'Child Cancer' representing AKUH Member, Examination and Promotion Committee of AKUH Mentor, MSc (Nursing) students for clinical and research training at the AKUH
	Certified Problem Based Learning (PBL) *facilitator*; prepared PBL cases and conducted many sessions for the 4th year medical students & received excellent evaluations at AKUH
	Interviewer for the medical student /internship/residency and fellowship program applicants at AKUH

ADVOCACY FOR CHILD HEALTH & OTHER ACTIVITIES

09/01/2014	Wrote an article in the September 2014 issue of CHANGE magazine, 'Diagnosing and Treating Childhood Cancer – Good news and Encouraging Outcomes' Bay Area Houston, Texas
06/01/2013	Wrote an article in the June 2013 issue of CHANGE magazine, 'Children with Blood disorders have high hopes for long term Health' Bay Area Houston, Texas
08/26/2004	Delivered a talk to GP's of Karachi on early detection of Child Cancer Continuing Medical Education Series Lecture Aga khan University School of Medicine Aga Khan University Hospital (AKUH) Karachi, Pakistan
07/03/2004	Delivered keynote speech to the general public of Karachi on Pediatric Cancers in the presence of news media representatives Aga Khan University Auditorium Aga Khan University Hospital (AKUH) Karachi, Pakistan
03/2000-/04/2004	Member Health Professionals Subcommittee National Child Cancer Foundation (CCF) Central Division Wellington, New Zealand

09/04/2003	Organized and Chaired Grand Rounds at the Wellington School of Medicine Auditorium Wellington, New Zealand Invited guests included Dr Giulio D' Angio, Editor-in-Chief of the prestigious Medical & Pediatric Oncology Journal Visit funded by CCF
2005-2006	Chief, website development committee Department of Pediatrics Aga Khan University Karachi, Pakistan

TEACHING & OTHER RESPONSIBILITIES AT UTMB:

School of Medicine (SOM)	Associate Professor of Pediatrics & Director Division of Hematology Oncology Chair, Monthly Divisional Meetings including Tumor Board Member, Faculty Senate & its Medical/Clinical Affairs Committee Member and reviewer, UTMB IRB # 1 Member, UTMB Transfusion Committee/Blood Management Program DHM Lecturer, UTMB School of Medicine MS1 Shadowing Mentor
Mentees/Advisees/Trainees	Director of Curriculum and Clerkship rotations in Pediatric Hematology Oncology for Residents (PL1, PL2 & PL3) Director of Curriculum and Clerkship rotations in Pediatric Hematology Oncology for Medical Students (MS1, MS3 & MS4) Mentor for one PL3 Resident's research project – poster presented by Dr Melethil in the Resident research meeting in June 2014 Mentor for 1 MS3 Student's research project, 2013-14 Mentor for 2 MS4 Student's Design a Case project, 2013-14 Mentor for 1 MS1 Student's summer shadowing, 2015 Lecturer for Residents (PL1, PL2 & PL3) and Nurse Practitioners DHM Lecturer for UTMB SOM MS1 courses

CLINICAL RESPONSIBILITIES

Division Director for Clinical Hematology & Oncology
Daily clinics, 10 half-day sessions at UTMB Pediatrics Specialty Center at Bay Colony in League City & 1 half-day session at University Health Clinics Primary Health Care Pavilion in Galveston in 14 day block
Inpatient Service coverage 1 in 2 at alternate week intervals
24 hour on-call service coverage 1 in 2 at alternate week intervals

CONTINUING MEDICAL EDUCATION

06/17/2016 – 06/18/2016	65th Pediatrics by the Gulf Annual Conference on Advances in Pediatrics Galveston, TX
10/30/2014 – 11/1/2014	UAE Cancer Congress 2014 'Promoting Excellence in Oncology' Dubai, United Arab Emirates
06/20/2014	Delivered talk on Rationale Use of Blood Products Pediatrics by the Gulf: 63rd Annual Conference on Advances in Pediatrics University of Texas Medical Branch Galveston, Texas
02/28/2014 – 03/01/2014	Pediatric Neuro-Oncology Symposium - In Memory of Dr. Marnie Rose MD Anderson Cancer Center Houston, TX
06/14/2013 – 06/15/2013	62nd Pediatrics by the Gulf Annual Conference on Advances in Pediatrics Galveston, TX
05/31/2013 – 06/01/2013	18th Annual PGME Conference Social Accountability in Health Professions Education The Aga Khan University Auditorium Karachi, PK
01/18/2013 – 01/18/2013	UTMB Connect Charge Capture for Providers Faculty EPIC (electronic medical records) course Galveston, TX
10/03/2012 – 10/03/2012	Faculty E/M Coding Course (1995 Guidelines) TrailBlazer Challenger Corporation & E/M University Memphis, TN

10/03/2012 – 10/03/2012	Emergency Department E/M Services (1995 Guidelines) TrailBlazer Challenger Corporation & E/M University Memphis, TN
07/23/2012 – 07/23/2012	Rational Physician Coding for E/M Services (1995 Guidelines) TrailBlazer Challenger Corporation & E/M University Memphis, TN
05/09/2012 – 05/12/2012	25th Annual Meeting American Society of Pediatric Hematology Oncology (ASPHO) New Orleans, LA
09/13/2011 – 09/17/2011	COG Fall Meeting Children's Oncology Group (COG) Atlanta, GA
09/09/2011 – 09/09/2011	Texas Pediatric Neuro-oncology Consortium (TOPNOC) Meeting Texas Children's Hospital Houston, TX
09/21/2010 – 09/24/2010	COG Fall Meeting & Pediatric Bone Marrow Transplant Symposium Children's Oncology Group (COG) Dallas, Texas
02/02/2009 – 02/08/2009	State of the Art Review of Pediatric Hematology Oncology The American Society of Pediatric Hematology Oncology Dallas, Texas
10/22/2008 – 10/26/2008	6th SIUT International Conference of Urology, Nephrology and Transplantation Sind Institute of Technology & Transplantation Karachi, Pakistan
11/07/2007 – 11/07/2007	Regional Conference on Update on the Management of Immunocompromised host King Fahad Specialist Hospital Dammam, Saudi Arabia
06/11/2007-06/12/2007	Regional Seminar on Medical Ethics King Fahad Specialist Hospital Dammam, Saudi Arabia
03/19/2007 – 03/22/2007	International Oncology Conferences on Recent Advances in Clinical Oncology United Arab Emirates University Al Ain, United Arab Emirates

09/02/2006 – 09/07/2006	International Society of Blood Transfusion 29th International Congress Cape Town, South Africa
09/19/2005 – 09/24//2005	International Society of Pediatric Oncology 37th Annual Congress Vancouver, Canada
03/01/2004 – 03/03/2004	Teenage Cancer trust Third International Conference on Cancer & the Adolescent Royal College of Physicians London, England
06/19/2003 – 06/22/2003	Australia and New Zealand Children's Cancer Study Group 17th Annual Scientific Meeting Sydney, Australia
03/05/2003 – 03/08/2003	Methods in New Drug Development 2nd Pediatric Oncology Conference Rome, Italy
06/09/2002 – 06/12/2002	The International symposium of Pediatric Neuro-oncology 10th Annual Meeting London, England
10/09/2001 – 10/12/2001	International Society of Pediatric Oncology 31st Annual Congress Brisbane, Australia
07/07/2001 – 07/12/2001	International Society of Thrombosis and Hemostasis 28th Annual Congress Paris, France
10/04/2000 – 10/07/2000	International Society of Pediatric Oncology 32nd Annual Congress Amsterdam, Netherlands
11/19/1999 – 11/21/1999	Thalamic and Hypothalamic tumors of Childhood Post Graduate Symposium at Beth Israel Medical Centre New York, New York
05/15/1999 – 05/18/1999	American Society of Clinical Oncology 35th Annual Meeting & Satellite Symposia Atlanta, Georgia

11/12/1998 – 11/15/1998	Society for Neuro-oncology 3rd Annual Meeting *Invited for oral presentation of Fellowship Research Project* San Francisco, California
12/05/1997 – 12/09/1997	American Society of Hematology Annual Meeting San Diego, California
11/14/1996	Thalassemia Intermedia Conference Children's Hospital of Boston Boston, Massachusetts

PAST & CURRENT MEMBERSHIP IN SCIENTIFIC SOCIETIES/PROFESSIONAL ORGANIZATIONS:

2011-Present	Texas Pediatric Neuro-oncology Consortium (TOPNOC)
2009	American Society of Pediatric Hematology Oncology
2002	International Society of Pediatric Oncology (SIOP)
2000	Australasian New Zealand Children Cancer Study Group (ANZCCSG)
1999	American Society of Clinical Oncology (ASCO)
1999	American Society of Hematology (ASH)
1999	Fellow of the American Academy of Pediatrics (FAAP)

PUBLICATIONS:

A - PEER REVIEWED JOURNALS

Hooda, B.S., Lalani, G., Fadoo, Z., & Billoo, G.: Implantable Port Devices Are Catheters of Choice for Administration of Chemotherapy in Pediatric Oncology Patients—A Clinical Experience in Pakistan. Annals of the New York Academy of Sciences, September 2008, 1138: 43-46

Above abstract presented in the International Oncology Conferences on Recent Advances in Clinical Oncology United Arab Emirates University Al Ain: 03/19-22/2007

B – NON-PEER REVIEWED JOURNALS

Hooda, B.S., Finlay, J.L.: Recent advances in the diagnosis and treatment of central nervous system germ cell tumors. Current Opinion in Neurology, 12 (6), December 1999, pp 693-696

C - ABSTRACTS PRESENTATION

06/20/2014	Pediatrics by the Gulf: 63rd Annual Conference on Advances in Pediatrics Poster Presentation: Shajitha University of Texas Medical Branch Galveston, Texas
07/2005	Juvenile Myelomonocytic Leukemia (JMML) undergoes remission with AML based therapy in combination with Cis-retinoic acid – Case report and review of literature. Abstract presented in 1st International Pediatric Oncology Conference Karachi, Pakistan
2004	Precursor B cell Acute Lymphoblastic Leukemia/lymphoma presenting as Eosinophillia: Diagnostic utility of flow cytometric immunophenotyping A D'souza, B Hooda et al submitted at Wellington Cancer Center Meeting
1999	Chronic Interferon exposure alters sensitivity to DNA damage in the Human Glioblastoma Cell line T 98G; Correlation with intracellular NAD+ Pools. *Neuro-Oncology* 1999, 1(1): S26. Accepted for oral presentation at the Society for Neuro-oncology 3Rrd Annual Meeting at San Francisco, CA, USA
1994	ALL presenting as Leukoerythroblastosis; Case studies and review of literature Presented at Annual Science Day University of Florida Gainesville, Florida, USA

D - MAGAZINES/NEWS ARTICLES

09/01/2014	Wrote an article in the September 2014 issue of CHANGE magazine, 'Diagnosing and Treating Childhood Cancer – Good news and Encouraging Outcomes' Bay Area Houston, Texas
06/01/2013	Wrote an article in the June 2013 issue of CHANGE magazine, 'Children with Blood disorders have high hopes for long term Health' Bay Area Houston, Texas

E - UNPUBLISHED CASE REPORT

2013	Koshy, N., Elayappen, A., & Hooda, B. S.: Rhabdomyolysis in a Patient Presenting with Sickle Cell Crisis: A Case Report and Review

F – INVITED TALKS/CHAIR CONFERENCES

09/08/2016	Malignant Bone Tumors PL-3 Resident's teaching session for Board Review University of Texas Medical Branch Galveston, Texas
07/10/2015	Heme Onc Emergencies PL-1 Resident's Teaching session University of Texas Medical Branch Galveston, Texas
08/27/2015	Solid Tumors & Neuroblastoma PL-2 Resident's Teaching session University of Texas Medical Branch Galveston, Texas
04/24/2015	Disorders of Leukocytes PL-3 Resident's teaching session University of Texas Medical Branch Galveston, Texas

03/31/2015	Lymphoma and related disorders DHM Lecture 22 for MS 2 University of Texas Medical Branch School of Medicine Galveston, Texas
03/30/2015	Leukemia and related disorders DHM Lecture 21 for MS 2 University of Texas Medical Branch School of Medicine Galveston, Texas
11/21/2014	Neuroblastoma Review PL-2 Resident's teaching session University of Texas Medical Branch Galveston, Texas
06/20/2014	Rationale Use of Blood Products Pediatrics by the Gulf: 63rd Annual Conference on Advances in Pediatrics University of Texas Medical Branch Galveston, Texas
01/17/2014	Leukocytes disorders PL-3 Resident's teaching session University of Texas Medical Branch Galveston, Texas
01/01/2013	Solid Tumor/s in Pediatric Oncology - Neuroblastoma PL-2 Resident's teaching session University of Texas Medical Branch Galveston, Texas
12/12/2012	Common Presentation and Management in Pediatric Oncology (School Health Nursing Perspective) Region 5 Education Service Center (ESC) School Health Nursing Network & Transportation Safety Beaumont, Texas
01/27/2012	Signs, Symptoms and Differential in Pediatric Hematology Resident's teaching Session University of Texas Medical Branch Galveston, Texas
11/17/2011	Synopsis of Pediatric Hematology Pediatric Nurse Practitioner's session University of Texas Medical Branch Galveston, Texas

02/11/2005	Chair, Pediatric Malignant Hematology Session, 7th National Hematology Oncology Conference of Pakistan Society of Hematology Karachi, Pakistan
02/11/2005	Member of the Expert Panel Brain Tumor Update (Gliomas) Section of Neurosurgery & Oncology of Aga Khan University Karachi, Pakistan
01/15/2005	T-cell Non-Hodgkin's Lymphoma – Prognostic factors and Treatment strategies National Symposium on Lymphoma Management B T Institute of Health Sciences & Blood Diseases Centre Karachi, Pakistan
05/18/2005	Bone Marrow Failure Pediatric nursing refresher course in Hematology Oncology Aga Khan University Karachi, Pakistan
11/11/2004	Aplastic Anemia Pediatric nursing refresher course in Hematology Oncology Aga Khan University Karachi, Pakistan

G - KEYNOTE ADDRESS

11/07/2007	Immunocompromised Host in Pediatric Oncology First Regional Conference on Update on the Management of Immunocompromised Host King Fahad Specialist Hospital Dammam, Saudi Arabia
07/08/2005	Latest advances in Chemotherapy for Brain Tumors in children First International Pediatric Oncology Conference, Pakistan Society of Pediatric Oncology Karachi, Pakistan
05/08/2005	Stem Cell Transplantation in Thalassemia – coverage on national media Fatmid Blood Foundation International Thalassemia Day Karachi, Pakistan

01/30/2005 — Bone Marrow Transplantation in Pediatric Practice
First national symposium of the National Institute of Child Health (NICH)
Karachi, Pakistan

H – GRAND ROUNDS

10/14/2016 — Presented: *The Limping Child – Differential Diagnosis of Limp in a child including both malignant and non malignant conditions*
Department of Pediatrics at University of Texas Medical Branch
Galveston, Texas

10/23/2015 — Presented: *Burkitt's Lymphoma presenting as Adenoidal Mass & Pancreatitis*
Department of Pediatrics at University of Texas Medical Branch
Galveston, Texas

02/14/2014 — Presented: *Blood Component Transfusions in Pediatric Medicine*;
Department of Pediatrics at University of Texas Medical Branch
Galveston, Texas

08/24/2012 — Presented: *Brain Tumors of Childhood and Infancy; an overview, management of PNETs and current state of the art including gene microarray based Classification*
Department of Pediatrics at University of Texas Medical Branch
Galveston, Texas

02/11/2011 — Presented: *Oncologic Emergency on New Year; A multi-disciplinary approach to care in Pediatric Hematology Oncology at UTMB*
Department of Pediatrics at University of Texas Medical Branch
Galveston, Texas

REFERENCES — Available upon request

REVISED: November 22, 2016

Julie L. Luke, DNP, RN, CPNP, CPHON
4410 Medical Dr., Suite 500
San Antonio, TX 78229
210-575-6240 (office)
210-274-5027 (cell)
Julie.Luke@MHShealth.com

EDUCATION

Doctorate of Nursing Practice (DNP) Aug 2012
University of Alabama, Tuscaloosa, AL

Master of Science Nursing (PNP) May 2007
University of Texas Health Science Center, San Antonio, TX

Bachelor of Science Nursing (BSN) May 1981
University of Texas Health Science Center, San Antonio, TX

PROFESSIONAL EXPERIENCE

Sep 2014 – current: **Pediatric Nurse Practitioner**
Pediatric Hematology/Oncology Team
Pediatric Specialists of Texas
Methodist Children's Hospital, San Antonio, TX

Aug 2007 – Sep 2014: **Pediatric Nurse Practitioner**
Pediatric Bone Marrow Transplant Team
Texas Transplant Physician Group
Methodist Children's Hospital, San Antonio, TX

Jan 1998 – Jul 2007: Staff Nurse V
Pediatric Hematology/ Oncology/Bone Marrow Transplant Inpatient Unit
Methodist Children's Hospital, San Antonio, TX

Dec 1992 – Dec 1997: Charge Nurse
Adolescent/Young Adult Medical/Surgical/Oncology Unit
Children's Hospital, Birmingham, AL

Apr 1992 – Dec 1992: Staff Nurse, Pediatrics
Sacred Heart Children's Hospital, Pensacola, FL

Feb 1984 – Apr 1992: homemaker/child development
Mar 1982 – Feb 1984: Staff Nurse, Pediatrics
USAF- Wilford Hall Medical Center, San Antonio, TX
Jun 1981- Mar 1982: Staff Nurse, Pediatrics
St. Joseph Hospital, Bryan, TX

PROFESSIONAL CERTIFICATIONS

Certified PNP Pediatric Nursing Certification Board, 2007- current
Certified Pediatric Hematology Oncology Nurse (CPHON) 2000 – current
Certified Instructor – APHON Chemotherapy/Biotherapy Provider Course, 2007- current
Chemotherapy/Biotherapy Provider (APHON), 1993 - current
Pediatric Advanced Life Support (PALS), 1995 – current

PROFESSIONAL LICENSURE

Registered Nurse, TX # 256206
Advanced Practice Registered Nurse, TX # AP115972
Prescriptive Authorization, TX # 7773
DEA Controlled Substance Registration, # ML1710640
NPI # 1861685448

PROFESSIONAL MEMBERSHIPS

Association of Pediatric Hematology/Oncology Nurses (APHON) 1993 - current
National Association of Pediatric Nurse Practitioners (NAPNAP) 2005 – current
American Society of Pediatric Hematology/Oncology (ASPHO) 2016 - current

PROFESSIONAL ACTIVITIES

Treasurer, San Antonio APHON Chapter 2013- current
President, San Antonio APHON Chapter 2007-2010
President, San Antonio NAPNAP Chapter 2010-2011
Secretary, San Antonio NAPNAP Chapter 2011-2012
Planning Committee San Antonio APHON Chapter Annual Conference, 2004 – current
Instructor – APHON Foundations of Pediatric Hematology/Oncology Nursing and Bone Marrow Transplant Nursing, 2007 - current
Speaker – APHON 2007 National Conference – Viral Infectious Complications Post Pediatric Hematopoietic Stem Cell Transplant
Speaker – APHON 2010 National Conference – Reduced Intensity Hematopoietic Stem Cell Transplant for Children with Hemoglobinopathies
Poster Presentation – University of Alabama System, Joint DNP Intensive, Mar 28-30, 2012, Immunization of Pediatric Hematopoietic Stem Cell Recipients: A Review of the Literature
Publications – Clinical Practice Implications of Immunizations after Pediatric Bone Marrow Transplant: A Literature Review (2012) Journal of Pediatric Oncology Nursing, 30, 7-17. doi: 10.1177/1043454212462069
Participant – ONCC CPHON Item Writing Workshop May 17-19, 2013
Content Contributor – Living Now Special Issue for Parents (post-transplant newsletter series) published by Be The Match, Sep 2013
Manuscript Reviewer - Journal of Pediatric Oncology Nursing and Journal of Transcultural Nursing

CURRICULUM VITAE
Cindy L. Schwartz, MD, MPH

PRESENT TITLE AND AFFILIATION

Primary Appointment

Edgar Lewis Curtis, Savilla Elizabeth Curtis, and Eleanor Lewis Curtis Distinguished Professorship in Pediatric Cancer, Department of Pediatrics Patient Care, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX

Director of Clinical and Translational Research, Department of Pediatrics Patient Care, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX

Director, Pediatric Phase I, Department of Pediatrics Patient Care, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX

Section Chief for Non-neural solid tumors, Department of Pediatrics Patient Care, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX

Dual/Joint/Adjunct Appointment

Professor, Department of Investigational Cancer Therapeutics, The University of Texas MD Anderson Cancer Center, Houston, TX

CITIZENSHIP

United States

OFFICE ADDRESS

The University of Texas MD Anderson Cancer Center
1515 Holcombe Blvd
Unit Number: 0087
Houston, TX 77030
Room Number: B8.4308
Phone: 713-745-3145
Fax: 713-794-5042
Email: clschwartz@mdanderson.org

EDUCATION

Degree-Granting Education

Yale College, New Haven, CT, BS, 1975, Bachelor of Science in Chemistry

UMDNJ Rutgers Medical School, Piscataway, NJ. 1977 (transferred to Brown)

Brown University Program in Medicine, Providence, RI, MD, 1979, Doctor of Medicine

Harvard School of Public Health, Boston, MA, MPH, 2011, Master of Public Health

Postgraduate Training

Residency, Pediatric, Johns Hopkins University School of Medicine, Baltimore, MD, 1979-1982

Fellowship, Pediatric Hematology/Oncology, Johns Hopkins University School of Medicine, Baltimore, MD, 1982-1985

Public Leadership, Leading Teams Workshop, Center for Public Leadership, Harvard Kennedy School, Cambridge, MA, 2011-2011

Leadership, Faculty Leadership Academy, MDACC, Houston, TX, 2015-2016

Program for Chairs of Clinical Services, Harvard School of Public Healthl. January 2017.

CREDENTIALS

Board Certification

American Board of Pediatrics, 1985

American Board of Pediatrics-Hematology/Oncology, 1994-2015, Recertification Date: 2008

Licensures

Active

Medical License, TX, 01296, 8/2014-7/2016

Inactive

MD, D28219, 8/1982-1987

NY, 165175-1, 1/1986-12/1994

Temporary, MA, 412T, 1993-1995

MD, D28219, 1994-9/2005

MA, 81701, 1995-2000

RI, MD11815, 5/2005-6/2014

Temporary, TX, 44360, 9/2013-9/2014

EXPERIENCE/SERVICE

Academic Appointments

Assistant in Oncology, Division of Pediatrics Oncology, Department of Oncology and Pediatrics, Johns Hopkins University School of Medicine, Baltimore, MD, 7/1983-11/1985

Assistant Professor, Department of Pediatrics, Division of Pediatric Hematology/Oncology, University of Rochester, Rochester, NY, 11/1985-6/1992

Associate Professor, Department of Pediatrics, Division of Pediatrics Oncology, University of Rochester, Rochester, NY, 7/1992-4/1994

Associate Professor, Department of Oncology and Pediatrics, Division of Pediatric Oncology, Johns Hopkins University School of Medicine, Baltimore, MD, 4/1994-8/2005

Professor, Department of Pediatrics, Division of Pediatrics Hematology/Oncology, Brown University, Providence, RI, 8/2005-9/2013

Professor, Department of Pediatrics and Investigational Cancer Therapeutics, The University of Texas MD Anderson Cancer Center, Houston, TX, 9/2013-present

Administrative Appointments/Responsibilities

Medical Director, Long Term Survivors Clinic, Pediatric Hematology/Oncology, University of Rochester Medical Center/Strong Memorial Hospital, Rochester, NY, 12/1988-4/1994

Clinical Director of Pediatric Hematology/Oncology, University of Rochester Medical Center, Strong Memorial Hospital, Rochester, NY, 1/1990-4/1994

Acting Division Director, University of Rochester, Rochester, NY, 4/1993-4/1994

Associate Director of Clinical Programs in Pediatric Oncology, Johns Hopkins University School of Medicine, Baltimore, MD, 4/1994-9/2003

Director, Long-Term Program in Pediatric Oncology, Johns Hopkins University School of Medicine, Baltimore, MD, 4/1994-8/2005

Associate Director of Clinical Research, Pediatric Oncology, Johns Hopkins University School of Medicine, Baltimore, MD, 9/2003-8/2005

Director, Division of Pediatric Hematology/Oncology, Brown University, Providence, RI, 8/2005-9/2013

Director, CHAMPS Survivorship Program, Providence, RI, 1/2006-9/2013

Member, Consortium for New England Childhood Cancer Services, Providence, RI, 2007-2013

Director of Clinical and Translational Research, Department of Pediatrics Patient Care, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX, 2013-present

Director, Pediatric Phase I, Department of Pediatrics Patient Care, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX, 2013-present

Deputy Division Head for Clinical and Translational Research, Department of Pediatrics Patient Care, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX, 9/2013-present.

Section Chief for Non-neural solid tumors, Department of Pediatrics Patient Care, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX, 1/2015-present

Division Head *ad interim*, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX 2/2015-12/2016

Department Chair *ad interim*, Division of Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX 2/2015-12/2016.

Director, Adolescent and Young Adult Oncology Program Development, The University of Texas MD Anderson Cancer Center, Houston, TX 2016 - present.

Other Appointments/Responsibilities

Principal Investigator, University of Rochester, Pediatric Oncology Group, Rochester, RI, 1992-1994

Principal Investigator, Johns Hopkins University, Pediatric Oncology Group, Baltimore, MD, 1994-2000

Chair - Hodgkin Disease Committee, Pediatric Oncology Group (POG), St. Louis, MO, 1999-2001

Steering Committee, COG Bone Tumor, Children's Oncology Group (COG), Arcadia, CA, 2000-present

Steering Committee, COG Voting Body, Children's Oncology Group (COG), Arcadia, CA, 2001-2003

Principal Investigator (Hopkins), Children's Oncology Group, Arcadia, CA, 2001-2005

Principal Investigator (Brown University), Children's Oncology Group (COG), Arcadia, CA, 2001-2005

Chair - Hodgkin Lymphoma Committee, Children's Oncology Group (COG), Arcadia, CA, 2001-2011

Scientific Chairs Committee, Children's Oncology Group (COG), Arcadia, CA, 2001-2011

Steering Committee, COG Hodgkin Committee, Children's Oncology Group, Arcadia, CA, 2001-present

Member, Advisory Board, NCI PDQ Pediatric Cancer Treatment Advisory Board, Washington, DC, 2002-2011

Scientific Advisory Board, Childhood Cancer Survivor Study (CCSS), Memphis, TN, 6/2004-6/2011

Principal Investigator (RIH/Brown), Children's Oncology Group (COG), St. Louis, MO, 2005-2013

Member, Data Safety Monitoring Board, Columbia University/Babies' Hospital Pediatric Hematology/Oncology/BMT, New York, NY, 2006-2008.

Principal Investigator (Brown University), Dana Farber ALL Consortium, Providence, RI, 2007-2013

Chair, Research Committee, Consortium for New England Childhood Cancer Survivors, Providence, RI, 2008-2013

Steering Committee and Founding Member, Consortium for New England Childhood Cancer Survivors, Providence, RI, 2008-2013

Expert Panel on Radiation Oncology - Hodgkin Lymphoma, American College of Radiology Appropriateness Criteria Committee, Radiation Oncology Panel, Reston, VA, 2009-2013.

COG Executive Committee, Children's Oncology Group (COG), Arcadia, CA, 2010-2013.

Steering Committee, Hematology Oncology Discipline, Children's Oncology Group (COG), Arcadia, CA, 2013-present

DSMB member, St. Jude Children's Hospital, Memphis, TN, 2015-present

Chair, Data Safety Monitoring Board, NANT- Novel Approaches to Neuroblastoma Therapy, Los Angeles, CA, 2002-present.

Endowed Positions

Alan G. Hassenfeld Professor of Pediatric Oncology, Warren Alpert School of Medicine, Department of Pediatrics at Brown University, Providence, RI, 8/2006-9/2013

Edgar Lewis Curtis, Savilla Elizabeth Curtis, and Eleanor Lewis Curtis Distinguished Professorship in Pediatric Cancer, The University of Texas MD Anderson Cancer Center, Houston, TX, 6/2015-present

Consultantships

N/A

Military or Other Governmental Service

N/A

Institutional Committee Activities

University of Rochester - Dept.t of Pediatrics Education Committee, Member, 1988-1990

University of Rochester - Dept. of Pediatrics Intern Selection Committee, Member, 1988-1994

University of Rochester - Dept. of Pediatrics Quality Assurance Committee, Member, 1989-1993

University of Rochester Cancer Center Protocol Review Board, Member, 1989-1994

University of Rochester - Dept. of Pediatrics Division Chiefs Committee, Member, 1993-1994

Johns Hopkins Oncology Center Quality Assurance Committee, Member, 1994-1995

Johns Hopkins Oncology Center Medical Services Committee, Member, 1994-1996

Johns Hopkins Oncology Center Clinical Chiefs Committee, Member, 1994-1998

Johns Hopkins Oncology Center Clinical Research Committee, Member, 1994-2005

Johns Hopkins University - Pediatrics Clinical Leaders Committee, Member, 1995-1996

Johns Hopkins Oncology Center Greenspring Subcommittee, Member, 1995-1997

Johns Hopkins University - Pediatric Oncology Task Force, Member, 1995-1997

Johns Hopkins Oncology Center Clinical Research Advisory Committee, Member, 1995-1998

Johns Hopkins Hospital, Pediatrics at Home Board of Directors, Member, 1995-2001

Johns Hopkins University - Pediatric Residency Review Committee, Member, 1995-2005

Johns Hopkins Oncology Center Clinical Investigator Subcommittee, Chair, 1996-1997

Johns Hopkins University - Pediatric Oncology Tumor Board Coordinator, Member, 1996-1998

Johns Hopkins Hospital/Pediatric Hospice Medical Advisory Committee, Member, 1996-2001

Johns Hopkins Hospital Cancer Committee, Member, 1996-2005

Johns Hopkins University - Pediatric Oncology Clinical Research Committee Coordinator, Member, 1996-2005

Johns Hopkins University School of Medicine- Admissions Committee, Member, 1997-1999

Johns Hopkins Hospital/Sub-committee Internal Relations of Clinical Practice Association, Member, 1999-2000

Johns Hopkins Hospital Children's Center Operations Committee, Member, 1999-2001

Johns Hopkins Oncology Center Clinical Research Executive Committee, Member, 1999-2005

Johns Hopkins Oncology Center Clinical Practice Committee, Member, 1999-2005

Johns Hopkins Oncology Center Executive Clinical Trials Unit, Member, 2002-2005

Johns Hopkins Oncology Center Physician Assistant/ Nurse Practitioner Council, Member, 2003-2005

Johns Hopkins University - Pediatric Oncology Core Clinical Care Committee, Member, 2003-2005

Rhode Island Hospital/Cancer Committee, Member, 2005-2013

Warren Alpert Medical School of Brown University - Search Committee for Pediatric Hematologist/Oncologist, Member, 2006-2007

Rhode Island Hospital/Chemotherapy Safety Initiative Group, Member, 2006-2013

Rhode Island Hospital/BMT/CON Group, Member, 2006-2013

Rhode Island Hospital/Oncology Sub-Council, Member, 2006-2013

Rhode Island Hospital/Oncology Council, Member, 2007-2013

Warren Alpert Medical School of Brown University - Brown University Oncology Group (BRUOG), Member, 2007-2013

Warren Alpert Medical School of Brown University - Search Committee for Pediatric Pulmonologist, Chair, 2008-2011

Warren Alpert Medical School of Brown University-Committee on Medical Faculty Appointments, Member, 2008-2011

Warren Alpert Medical School of Brown University - Pediatric Promotion Committee, Member, 2009-2013

Warren Alpert Medical School of Brown University - Search Committee for Chief of Pathology, RIH, Member, 2010-2011

Rhode Island Hospital/Department of Pediatrics Research Task Force, Member, 2011-2013

Warren Alpert Medical School of Brown University - Search Committee for Chair of Dermatology, Member, 2012-2013

Warren Alpert Medical School of Brown University - Search Committee for Pediatric Hematologist/Oncologist, Member, 2013

MDACC Children's Cancer Hospital Executive Leadership Committee, Member, 2013-2014

MDACC Pediatrics Clinical Research Operations, Member, 2013-2014

Pediatrics Women's Faculty Committee, Member, 2013-2014

MDACC/TCH Joint Sarcoma Committee, Member, 2013-present

Clinical Research Committee #3, Member, 2014-present

Vice Provost Advisory Council (VPAC), Member, 2014-present

Divisional Council of Gender Inclusiveness (DCGI), Member, 2014-present

MDACC Shared Governance Committee, Member, 2015-present

MDACC Clinical Department Chairs, Member, 2015-present

MDACC Division Head Committee, Member, 2015-present

Adolescent and Young Adult Program Planning Group, Director, 2015-present

Executive Clinical Operations Team, Member, 2015-present

PRS Executive Council, Member, 2015-present

MDACC Pediatric Advisory Committee for Clinical Trials, Chair, 2015-present

MDACC Pediatric POTIS Board, Chair, 2015

Clinical Research Advisory Committee, Member, 4/2016- present

HONORS AND AWARDS

National Merit Finalist, 1971

Sigma XI, Medical Science Society, 1979

American Cancer Society Clinical Fellowship, 1983-1984

Recognition Award, Pediatric Oncology Group, 1999

America's Top Doctors, 2001-2016

America's Top Doctors For Cancer, 2005-2016

Lifespan Executive Mentorship Program, 2010

Recognition Award, Tomorrow Fund, 2010

Women Making Strides Award, Ronald McDonald House of Providence, RI, 2010

Best Clinical Trial Finalist, International Society of Paediatric Oncology (SIOP), 2011

Recognition Award - Hodgkin Lymphoma Chair 2001-2011, Children's Oncology Group (COG), 2011

Top 211 Docs in Houston. Houston Chronicle's MedCity Magazine, 2016

RESEARCH

Grants and Contracts

Funded

Co-Investigator, Dexrazoxane Follow-up Study: Revisiting POG 9404/9425/9426, St. Baldrick's Consortium Research Grant, 2012-2017, $50,000 ($10,000/year)

Co-Principal Investigator, 1%, Modulating FAS expression to improve outcome for patients with refractory osteosarcoma, GAC, PI - Schwartz CL and Harrison D, 4/1/2016-3/31/2017, $50,000 ($50,000/year)

Co-Investigator, Immunomodulatory effects of nutritional intervention in malnourished pediatric oncology patients, Pediatric Clinical Innovator Award, PI - Fogelsong J

Pending

Co-Investigator, 5%, Dexrazoxane and Prevention of Anthracycline-Related Cardiomyopathy, NIH/NCI, PI - Eric Chow

Co-Investigator, 5%, Fgl-2 targeted therapy for reversing multi-modality immune suppression, NIH/NCI,, NIH/NCI, PI - Shulin Li

Other

N/A

Completed

Chair COG Hodgkin Lymphoma Committee
Principal Investigator (Hopkins, RI Hospital) 28%, Children's Oncology Group, U10CA098543, NIH/NCI, 1/1/2003-10/2013, $27,161,785 ($125,239/year)

Co-Investigator and Study Vice-Chair, 5%, HEALTH OUTCOMES FOR HODGKIN DISEASE SURVIVORS, 5R01CA106750-03, NIH/NCI, PI - D. Friedman, 6/2004-6/2008, $1,551,968 ($45,463/year).

Co-Investigator, Optimizing Long-term Outcome for Hodgkin Lymphoma Patients: Modeled Reduction in Late Effects (MORALE), Canadian Institutes of Health Research, PI - Hodgson, 3/1/2010-3/1/2011, $89,557

PI-RIH,Dana Farber Cancer Institute – ALL Consortium, PI -Stephen Sallan,
3% 2007-9/30/2013 ($18,000/year).

Principal Investigator (Clinical - Project 1), 5%, SPORE in Lymphoma Project 1 EBV and Hodgkin's Disease, 1P50CA096888, NIH/NCI, PI - Ambinder, 7/1/2002-6/30/2007, $13,279,453 ($330,489/year).

Principal Investigator, Neurobehavioral Outcome after CNS Prophylaxis without RT in ALL, NIH 5M01RR000052-430965, NIH, 12/1/2003-11/30/2004, $24,000 ($470/year)

Principal Investigator, Movement Restriction Fatigue in Cancer Survivors, RR000052-430965, NIH/NCI, 12/1/2002-11/30/2003, $149,500 ($149,500/year)

Chair, 7%, Pediatric Oncology Group, Hodgkin's Disease Committee Chair Funding, NIH NCI/2 U10 CA 288 76 17, NIH/NCI, 1/1/2000-12/31/2002, $27,285 ($20,988/year)

Co-Investigator, 5%, Behavioral Medicine Approaches to Pediatric Acute Pain, 5ROL HD35528, Pediatric Oncology Group, PI - Keith Slifer, PhD, 1/1/1998-3/31/2001, $24,188 ($8,031/year)

Principal Investigator, 20%, Evaluation of Risk for Myocardial Ischemia in Survivors of Hodgkin's Disease Treated with Mediastinal Radiation, CA 0697335S1, NIH/NCI, 5/1/1997-4/30/1999, $292,870 ($146,435/year)

Principal Investigator, 18%, Pediatric Oncology Group U10 Award (Johns Hopkins), NIH NCI/2 U10 CA 28476, NIH/NCI, 4/15/1994-2/28/2002, $1,058,532 ($219,600/year)

Principal Investigator, Pediatric Oncology Group Phase I grant at Johns Hopkins, Agreement #0600-370-C352JHPX, NIH/NCI, 7/1/1999-6/30/2002 ($10,000/year)

PI at Hopkins, Phase 1 POG trial, M01RR000052, NIH/NCI, 12/1/1997-9/30/1999, $40,735 ($20,649/year)

PI (U. of Rochester 1993-94), The Biology and Treatment of Human Leukemia and Lymphoma, CA-34183-11, NIH/NCI, PI - Stephen Sallan, MD (DFCI), 8/1/1983-12/31/1994 ($16,347/year)

Principal Investigator, MRI assessment of Marrow Response to ALL Therapy, IN-18-31 ACS Institutional Award, 1/1/1989-12/31/1989, $7,500 ($7,500/year)

Principal Investigator, The Use of Cyclosporin-A to Overcome Multiple Drug Resistance, Cancer Action, 7/1/1992-6/30/1994, $4,500 ($4,500/year)

Principal Investigator, Clinical Investigation of MDR-1 and Late Effects in Childhood Cancer, CA06973, Cancer Center Support Grant (NIH CORE), 5/1/1993-4/30/1996, $50,000 ($50,000/year)

Principal Investigator, 15%, Phase I: Samarium-153 for recurrent or metastatic osteosarcoma, Cytogen, 2005-2006

Principal Investigator, Pediatric Oncology Infrastructure support, Cure Kids Cancer Radiothon, 2009-9/30/2013, $225,000 ($75,000/year)

Principal Investigator, Pediatric Oncology Infrastructure support, Cure Kids Cancer Radiothon, 2009-9/30/2013, $225,000 ($75,000/year)

Co-Investigator, Perspectives Regarding Fertility in Female Adolescent and Young Adult Childhood Cancer Survivors, Cure Kids Cancer Radiothon, PI - P. Rao, 2011-2012, $6,000 ($6,000/year)

Co-Investigator, 2%, Telephone-based Counseling Intervention for Primary Caregivers, R21-141313, NIH/NCI, PI - Debra Friedman, MD, 9/1/2011-9/30/2013, $417,423 ($6,114/year)

Principal Investigator, Pilot: Regional Promotion of Healthy Behaviors in Adolescent Cancer Survivors, Cure Kids Cancer Radiothon, 2012-2013, $35,000 ($35,000/year)

Principal Investigator, A Research Infrastructure for Adolescent Survivorship in New England, St. Baldrick's Foundation Infrastructure Grant, 2012-2013, $49,729 ($49,729/year)

Principal Investigator, Long term survivorship program, Hyundai Research Grant, 2011-2012, $100,000 ($100,000/year).

Protocols

Funded

Collaborator, Late Effects of Treatment of Hodgkin's Disease - A Pediatric Oncology Group Non-Therapeutic Study, POG8828, 1988-2002, NCI/POG

Collaborator, A case control study of Hodgkin’s disease in childhood., POG8829, PI - S. Grufferman, 1988-2003, NCI POG

Principal Investigator, MRI assessment of Marrow Response to ALL Therapy, IN-18-31, 1989, $7,200 ($7,200/year), ACS Institutional Award

Specific, The Use of Cyclosporin-A to Overcome Multiple Drug Resistance, 1992-1994, $4,500 ($4,500/year), Cancer Action

Study Chairman, The use of cyclosporine A to overcome multidrug resistance in osteosarcoma, POG9357, 1993-1997, NIC/POG

Study Chairman, Trial of ADR, CDDP and MTX with and Without Ifos, with and without MTP-PE for Treatment of Osteogenic Sarcoma, POG9351, 1993-1997, NCI POG

Principal Investigator (Hopkins), A Randomized, Double-Blind, Open Label, Trial of AmBisome Liposomal, 1994-1996, $131,000, Pfizer

Study Chairman, Treatment of Children with Recurrent or Refractory Hodgkin's Disease, Wilms' Tumor, Ewing's Sarcoma, Rhabdomyosarcoma or Other Soft Tissue Sarcomas with Cyclosporine-A, Actinomycin-D, Vincristine - A Pediatric Oncology Group Phase II Study, POG 9227, 1995-1998, NCI/POG

Collaborator, Etoposide/Ifosfamide + G-CSF in the Treatment of Newly Diagnosed Metastatic Osteosarcoma or Unresectable Osteosarcoma. A Pediatric Oncology Group Phase II Study, P9450, PI - A. Goorin, 1995-1999, NCI POG

Principal Investigator (Hopkins), Phase I Study to Determine the Safety and Pharmacokinetics of FK463 in febrile neutropenic pediatric patients, 98-0-043 M680301-2068, 1995-2001, $111,500, Fujisawa

Study Chairman, Advanced Stage Hodgkins Disease - A Pediatric Oncology Group Phase III Study. Dose dense, response based therapy using ABVE-PC, P9425, 1997-2001, NCI/POG

Principal Investigator (Hopkins), A Randomized, Open Label, Comparative, Multicenter Trial of Voriconazole vs. AmBisome for Empirical Antifungal Therapy in Immunocompromised Patients with Persistent Fever and Neutropenia, 97-N-0042, 1998-1999 ($102,599/year), Pfizer

Principal Investigator (Hopkins), Open, Intravenous Multiple Dose, Multi-Center Study to Investigate the Pharmocokinetics, Safety and Toleratipn of Voriconazone in Children Ages 2-12 years, A15010076045 M6813012127, 1998-2000, $5,182, Pfizer

Principal Investigator (Hopkins), Prospectively randomized double blind comparative multi-center study to evaluate efficacy and safety of Nyotran vs. amphotericin B for empiric antifungal treatment in neutropenic patients, 1999-2000, Aronex

Principal Investigator (Hopkins), Phase I Study to Determine the Safety and Pharmacokinetics of FK463 in febrile neutropenic pediatric patients, 98-0-043 M6813012105, 1999-2001, $242,900 ($63,463/year), Fujisawa

Study Chairman, Protocol for Patients with Newly-Diagnosed, Non-metastatic Osteosarcoma: A Pilot Study. Intensified therapy with ifosfamide/ etoposide or high cumulative dose doxorubicin with dexrazoxane., P9754, 1999-2002, NCI/POG grant

Collaborator, Osteosarcoma Biology Protocol: Companion to Group-Wide Therapeutic Studies, POG 9851, PI - R. Gorlick, 1999-2008, NCI POG/COG

Principal Investigator, Phase I/II Trial of Samarium in High-Risk Osteogenic Sarcoma, 2000-2002, $73,582 ($73,582/year), Berlix

Principal Investigator (Hopkins), Phase 1B Study of the Safety, Tolerance, and Pharmacokinetics of Oral Posaconazole in Immunocompromised Children with Neutropenia, MK0991, 2000, $6,115 ($6,115/year), Merck

Principal Investigator (Hopkins), MK-0091 (Caspofungin) Pediatric PK Study, MK-0091, 2001-2002, Merck

HL Committee Chair and Study Committee Member, A Pilot Study of Re-Induction Chemotherapy with Ifosfamide, and Vinorelbine (IV) in Children with Refractory/Relapsed Hodgkin Disease, AHOD00P1, 2001-2006, NCI COG

Study Vice-Chair, Treatment of Children with Newly-Diagnosed Low Stage Lymphocyte Predominant Hodgkin Disease (LPHD) 1/06-11/10.AHOD0031: A Phase III Study of Dose-Intensive, Response-Based Chemotherapy and Radiation Therapy for Children and Adolescents with Newly Diagnosed Intermediate Risk Hodgkin Disease, AHOD03P1, PI - Appel B, 2002-2009, NCI COG

Committee Chair and Study Committee member, A Phase II/III Study of Immunomodulation After High Dose Myeloablative Therapy with Autologous Stem Cell Rescue for Refractory/Relapsed Hodgkin Disease, AHOD0121, PI - Chen A., 2003-2007, NCI COG

Committee Chair and Study Committee investigator, A Phase II Study of Weekly Gemcitabine and Vinorelbine in Children with Recurrent or Refractory Hodgkin Disease., AHOD0321, PI - Cole P, 2004-2007, NCI COG

Study Vice Chair, Health-Related Outcomes for Hodgkin Disease Survivors, ALTE04N!, PI - Friedman, 2004-2011, NCI COG

Committee Chair and study investigator, Phase III Study for the Treatment of Children and Adolescents with Newly Diagnosed Low-Risk Hodgkin Disease, AHOD 0431, PI - Keller F, 2006-2009, NCI COG

Committee Chair and Study Investigator, Hodgkin Disease Banking Study, AHOD04B!, 2006-present, NCI COG

Principal Investigator (Hopkins), A Phase 1/Phase 2 Study of CP-751,871 in Patients with relapsed and/or Refractory Ewing's Sarcoma Family of Tumors, A4021020, 2007, Pfizer

Study Chairman, The role of MGMT in Pediatric Hodgkin Lymphoma and Chemotherapy Induced Toxicities, AHOD13B2, 2013-2014

Collaborator (MDACC), Phase I Dose Escalation of Monthly Intravenous Ra-223 dichloride in Osteosarcoma, 2013-00037360JW, PI - Subbiah, 2013, Bayer Healthcare AG

COG Study Investigator and MDACC PI, A Randomized Phase III Study of Brentuximab vedotin (Bv) for Newly Diagnosed Classical Hodgkin Lymphoma (cHL)., AHOD1331, PI - Schwartz, 2015-present, NCI COG

Collaborator (MDACC), Phase I open label, dose escalation trial to determine the MTD, safety, PK and efficacy of afatinib monotherapy in children aged 2 years to <18 years with recurrent/refractory neuroectodermal tumours, rhabdomyosarcoma and/or other solid, 00051236, PI - D. Harrison, 2016, Boehringer Ingelheim

Collaborator (MDACC), Study of the Safety and Pharmacokinetics of anti-PD-L1 antibody (MPDL3280A) in pediatric and young adult patients with previously treated solid tumors, 2015-00051260, 2016, Memorial Sloan-Kettering Cancer Center

Unfunded

Collaborator, Longitudinal Biospecimen Acquisition for All Tumor Types for Adaptive Patient Oriented Longitudinal Learning and Optimization (APOLLO), 2015-, PI - Thomas Buchholz, 2016

Co-Principal Investigator, Pre-surgical Supervised Exercise for Bone Cancer Patients, Pending, PI - Lewis V, Schwartz CL, 2016

Patents and Technology Licenses

Patents

N/A

Technology Licenses

N/A

Grant Reviewer/Service on Study Sections

Review Panel/Children's Cancer Survivor Study, NCI-E, Ad-hoc Committee Member, 1998-1999

Review Panel/Site Visit of Children's Cancer Group, NCI-H, Ad-hoc Review Committee Member, 1998

NCI-D, Ad-Hoc Committee Member, NIH, Site Reviewer, 1999-2000

Review Panel/Intergroup, Rhabdomyosarcoma Study-Late Effects Program, NCI-E, As-hoc Review Committee Member, 1999

PDQ Pediatric Cancer Treatment Advisory Board, NCI, Member, 2002-2014

Development Steering Committee for Late Effects Toxicity Criteria: Pediatric Representative, NCI/CTEP, Ad-hoc Review Committee Member, 2002

Special Emphasis Panel/Initial Review Group CONC, NIH, Member, 2004-2005

Pediatric Oncologic Drug Advising Committee, NCI, Ad Hoc Member, 2005-2010

Lymphoma Leukemia Spore, NCI, Ad Hoc Member, 2008

NCI Lymphoma, Leukemia, Lung, GI Spore, NCI, Ad Hoc Member, 2009

Cancer Center Support Grant, P30 Site Reviewer, Eppley Cancer Center, University of Nebraska Medical Center, NIH, Member, 2010

NCI Lymphoma, Leukemia, Lung, GI Spore, NCI, Ad Hoc Member, 2012

Special Emphasis Panel, "Research Answers to NCI's Provocative Questions", NCI, Member, 2012

NCTN Network - Lead Academic Participating Site Reviewer, NCI, Member, 2013

PUBLICATIONS

Peer-Reviewed Original Research Articles

1. **Schwartz CL**, Bender KS, Burke PJ, Kan JS, Civin CI. QT interval prolongation and cardiac dysrhythmia in a patient receiving amsacrine. Cancer Treat Rep 68(7-8):1043-4, Jul-Aug, 7/1984. PMID: 6547639.
2. Noga SJ, Donnenberg AD, **Schwartz CL**, Strauss LC, Civin CI, Santos GW. Development of a simplified counterflow centrifugation elutriation procedure for depletion of lymphocytes from human bone marrow. Transplantation 41(2):220-9, 2/1986. PMID: 2935979.
3. Noga SJ, Cremo CA, Duff SC, **Schwartz CL**, Melaragno A, Civin CI, Donnenberg AD. Large scale separation of human bone marrow by counterflow centrifugation elutriation. J Immunol Methods 92(2):211-8, 9/1986. PMID: 2944969.
4. Noga SJ, **Schwartz CL**, Civin CI, Loken M, Donnenberg AD. Rapid separation of whole human bone marrow aspirates by counterflow centrifugation elutriation. Transplantation 43(3):438-40, 3/1987. PMID: 3547800.
5. **Schwartz CL**, Minniti CP, Harwood P, Na S, Banquerigo ML, Strauss LC, Kurtzberg J, Smith SD, Civin CI. Elimination of clonogenic malignant human T cells using monoclonal antibodies in combination with 2'-deoxycoformycin. J Clin Oncol 5(12):1900-11, 12/1987. PMID: 3500279.
6. Henrickson KJ, Powell KR, **Schwartz CL**. A dilute solution of vancomycin and heparin retains antibacterial and anticoagulant activities. J Infect Dis 157(3):600-1, 3/1988. PMID: 3343531.
7. **Schwartz CL**, Miller NR, Wharam MD, Leventhal BG. The optic nerve as the site of initial relapse in childhood acute lymphoblastic leukemia. Cancer 63(8):1616-20, 4/1989. PMID: 2635874.
8. **Schwartz CL**. Creating life on the plateau: Reproductive potential in survivors of childhood Hodgkin's disease. Int J Rad Onc Biol Phys(19):1099-1100, 1990.
9. **Schwartz CL**, Henrickson KJ, Roghmann K, Powell K. Prevention of bacteremia attributed to luminal colonization of tunneled central venous catheter with vancomycin susceptible organisms. J Clin Oncol(8):1591-1597, 1990.
10. Wang N, Cedrone E, Dry J, Skuse GR, **Schwartz CL**, Terryberry S. Transposition of the oncogene ets-1 in t(11;19) translocation in acute leukemia. Cancer Genet Cytogenet(50):199-205, 1990.
11. Tulikangas P, Hobbie W, **Schwartz CL**. Cyclophosphamide treatment for childhood leukemia: Effects on reproductive function. J Univ Rochester Med Center, 1991.
12. **Schwartz CL**, Hobbie WL, Truesdell S, Constine LC, Clark EB. Corrected QT interval prolongation in anthracycline-treated survivors of childhood cancer. J Clin Oncol 11(10):1906-10, 10/1993. PMID: 8410117.
13. Rosier RN, Teot LA, Hicks DG, **Schwartz C**, O'Keefe RJ, Puzas JE. Multiple drug resistance in osteosarcoma. Iowa Orthop J 15:66-73, 1995. PMCID: PMC2329065.
14. O'Reilly R, Link M, Fletcher B, Gebhardt M, Krance R, Meyers P, Neff J, **Schwartz CL**. CCN pediatric osteosarcoma practice guidelines. Oncology 10(112):1799-806, 1996.

15. Korones DN, Weidner S, **Schwartz CL**. Intermittent high-dose deferoxamine for patients with iron overload and sickle anemia. Int J Pediatr Hematol/Oncol(3):53-6, 1996.
16. Dome JS, **Schwartz CL.** Osteosarcoma. Cancer Treat Res 92:215-51, 1997. PMID: 9494763.
17. Leung W, Chen AR, Klann RC, Moss TJ, Davis JE, Noga SJ, Cohen KJ, Friedman AD, Small D, **Schwartz CL,** Borowitz MJ, Wharam MD, Paidas CN, Long CA, Karandis S, McMannis JD, Kastan MB, Civin CI. Frequent detection of tumor cells in hematopoietic grafts in Neuroblastoma and Ewing's sarcoma. Bone Marrow Transplantation:971-9, 1998.
18. Walsh TJ, Finberg R, Arndt C, Hiemenz J, **Schwartz CL**, Bodensteiner D, Pappas P, Seibel N, Greenberg RN, Dummer S, Schuster M, Holcenberg JS. A Randomized, Double-Blind, Multicenter Trial of Liposomal Amphotericin B versus Conventional Amphotericin B for Empirical Antifungal Therapy of Persistently Febrile Neutropenic Patients. N Engl J Med(11):794-771, 1999.
19. Spunt SL, Lobe TE, Pappo AS, Parham DM, Wharam MD, Arndt C, Anderson JR, Crist WM, Paidas C, Wiener E, Andrassy RJ, **Schwartz CL**. Rhabdomyosarcoma of the Biliary Tract in Children and Adolescents - The Intergroup Rhabdomyosarcoma Study Group Experience (1972-98). J. Pediatr Surg(35):309-321, 2000.
20. Lipton JM, Federman N, Khabbaze Y, **Schwartz CL,** Hilliard LM, Clark JI, Vlachos A, Diamond-Black Anemia Registry. Osteogenic sarcoma associated with Diamond-Blackfan anemia: a report from the Diamond-Blackfan Anemia Registry. J Pediatr Hematol Oncol 23(1):39-44, 1/2001. PMID: 11196268.
21. **Schwartz CL**, Thompson EB, Gelber RD, Young ML, Chilton D, Cohen HJ, Sallan SE. Improved response with higher corticosteroid dose in children with acute lymphoblastic leukemia. J Clin Oncol 19(4):1040-6, 2/2001. PMID: 11181667.
22. Meij P, Vervoort MB, Bloemena E, Schouten TE, **Schwartz C,** Grufferman S, Ambinder RF, Middeldorp JM. Antibody responses to Epstein-Barr virus-encoded latent membrane protein-1 (LMP1) and expression of LMP1 in juvenile Hodgkin's disease. J Med Virol 68(3):370-7, 11/2002. PMID: 12226824.
23. Friedman AM, Sengul H, Lehman H, **Schwartz CL**, Goodman S. Do basic laboratory tests or clinical observations predict bleeding in thrombocytopenic oncology patients? Transfusion Medicine Reviews (16):34-45, 2002.
24. Goorin A, Harris M, Bernstein M, Ferguson W, Devidas M, Siegal GP, Gebhardt MC, **Schwartz CL,** Link M. Grier HE Successful phase II trial of etoposide and high dose ifosfamide in newly diagnosed metastatic osteosarcoma: A Pediatric Oncology Group trial (POG). J Clin Oncol (20):426-433, 2002.
25. Adams MJ, Lipshultz SE, **Schwartz C**, Fajardo LF, Coen V, Constine LS. Radiation-associated cardiovascular disease: manifestations and management. Semin Radiat Oncol 13(3):346-56, 7/2003. PMID: 12903022.
26. Gorlick R, Anderson P, Andrule I, Arndt C, Beardsley Peter G, Bernstein M, Bridge J, Cheung N, Dome J, Ebb D, Gardner T, Gebhardt M, Holcombe G, Hansen M, Healey J, Helman L, Hock J, Houghton J, Houghton P, Huvos A, Chand K, Kieran M, Kleinerman E, Ladanyi M, Lau C, Malkin D, Neyssa M, Meltzer P, Meyers P, Schofield D, **Schwartz CL**, Smith M, Toretsky J, Tsokos M, Wexler L, Wigginton J, Withrow S, Schoenfeldt M, Anderson B. Biology of Childhood Osteogenic Sarcoma and Potential Targets for Therapeutic Development. Clin Cancer Research (9):5422-53, 2003.
27. Welsh J, Thurman S, Borzillary S, **Schwartz CL**, Howard S. Samarium-153 Ethylenediaminetetramethylene Phosphonic Acid for Recurrent Metastatic Osteosarcoma. lin Adv Hematol Oncol(1), 2003.
28. **Schwartz CL**. Health status of adult long-term survivors of childhood cancer: a report from the Childhood Cancer Survivor Study. J Pediatr 144(3):407-8, 3/2004. PMID: 15032224.
29. Meyers PA, **Schwartz CL**, Krailo M, Kleinerman ES, Betcher D, Bernstein ML, Conrad E, Ferguson W, Gebhardt M, Goorin AM, Harris MB, Healey J, Huvos A, Link M, Montebello J, Nadel H, Nieder M, Sato J, Siegal G, Weiner M, Wells R, Wold L, Womer R, Grier H. Osteosarcoma: a randomized, prospective trial of the addition of ifosfamide and/or muramyl

tripeptide to cisplatin, doxorubicin, and high-dose methotrexate. J Clin Oncol 23(9):2004-11, 3/2005. PMID: 15774791.
30. **Schwartz CL.** Special issues in pediatric Hodgkin's disease. Eur J Haematol Suppl(66):55-62, 7/2005. PMID: 16007870.
31. Seibel NL, **Schwartz C**, Arrieta A, Flynn P, Shad A, Albano E, Keirns J, Lau WM, Facklam DP, Buell DN, Walsh TJ. Safety, tolerability, and pharmacokinetics of Micafungin (FK463) in febrile neutropenic pediatric patients. Antimicrob Agents Chemother 49(8):3317-24, 8/2005. PMCID: PMC1196271.
32. Walsh TJ, Adamson PC, Seibel NL, Flynn PM, Neely MN, **Schwartz C**, Shad A, Kaplan SL, Roden MM, Stone JA, Miller A, Bradshaw SK, Li SX, Sable CA, Kartsonis NA. Pharmacokinetics, safety, and tolerability of caspofungin in children and adolescents. Antimicrob Agents Chemother 49(11):4536-45, 11/2005. PMCID: PMC1280172.
33. Wimmer RS, Chauvenet AR, London WB, Villaluna D, de Alarcon PA, **Schwartz CL**. APE chemotherapy for children with relapsed Hodgkin disease: a Pediatric Oncology Group trial. Pediatr Blood Cancer 46(3):320-4, 3/2006. PMID: 16200630.
34. Kung FH, **Schwartz CL**, Ferree CR, London WB, Ternberg JL, Behm FG, Wharam MD, Falletta JM, de Alarcon P, Chauvenet AR, Children's Oncology Group. POG 8625: a randomized trial comparing chemotherapy with chemoradiotherapy for children and adolescents with Stages I, IIA, IIIA1 Hodgkin Disease: a report from the Children's Oncology Group. J Pediatr Hematol Oncol 28(6):362-8, 6/2006. PMID: 16794504.
35. Oeffinger KC, Mertens AC, Sklar CA, Kawashima T, Hudson MM, Meadows AT, Friedman DL, Marina N, Hobbie W, Kadan-Lottick NS, **Schwartz CL**, Leisenring W, Robison LL, Childhood Cancer Survivor Study. Chronic health conditions in adult survivors of childhood cancer. N Engl J Med 355(15):1572-82, 10/2006. PMID: 17035650.
36. Tebbi CK, London WB, Friedman D, Villaluna D, De Alarcon PA, Constine LS, Mendenhall NP, Sposto R, Chauvenet A, **Schwartz CL**. Dexrazoxane-associated risk for acute myeloid leukemia/myelodysplastic syndrome and other secondary malignancies in pediatric Hodgkin's disease. J Clin Oncol 25(5):493-500, 2/2007. PMID: 17290056.
37. Ehrlich PF, Friedman DL, **Schwartz CL**, Children Oncology Group Hodgkin Lymphoma study section. Monitoring diagnostic accuracy and complications. A report from the Children's Oncology Group Hodgkin lymphoma study. J Pediatr Surg 42(5):788-91, 5/2007. PMID: 17502184.
38. **Schwartz CL**, Gorlick R, Teot L, Krailo M, Chen Z, Goorin A, Grier HE, Bernstein ML, Meyers P, Children's Oncology Group. Multiple drug resistance in osteogenic sarcoma: INT0133 from the Children's Oncology Group. J Clin Oncol 25(15):2057-62, 5/2007. PMID: 17513810.
39. Mahone EM, Prahme MC, Ruble K, Mostofsky SH, **Schwartz CL**. Motor and perceptual timing deficits among survivors of childhood leukemia. J Pediatr Psychol 32(8):918-25, 9/2007. e-Pub 5/2007. PMID: 17522113.
40. Hope WW, Seibel NL, **Schwartz CL**, Arrieta A, Flynn P, Shad A, Albano E, Keirns JJ, Buell DN, Gumbo T, Drusano GL, Walsh TJ. Population pharmacokinetics of micafungin in pediatric patients and implications for antifungal dosing. Antimicrob Agents Chemother 51(10):3714-9, 10/2007. e-Pub 7/2007. PMCID: PMC2043253.
41. Terlou A, Ruble K, Stapert AF, Chang HC, Rowe PC, **Schwartz CL**. Orthostatic intolerance in survivors of childhood cancer. Eur J Cancer 43(18):2685-90, 12/2007. e-Pub 8/2007. PMID: 17689953.
42. Carver J, Shapiro C, Ng A, Jacobs L, **Schwartz C**, et al. American Society of Clinical Oncology Clinical Evidence Review on the Ongoing Care of Adult Cancer Survivors: Cardiac and Pulmonary Late Effects. J Clin Oncol 25(25):3991-4008, 2007.
43. Meyers PA, **Schwartz CL,** Krailo MD, Healey JH, Bernstein ML, Betcher D, Ferguson WS, Gebhardt MC, Goorin AM, Harris M, Kleinerman E, Link MP, Nadel H, Nieder M, Siegal GP, Weiner MA, Wells RJ, Womer RB, Grier HE, Children's Oncology Group. Osteosarcoma: the addition of muramyl tripeptide to chemotherapy improves overall survival--a report from the Children's Oncology Group. J Clin Oncol 26(4):633-8, 2/2008. PMID: 18235123.

44. Williams KM, Higman MA, Chen AR, **Schwartz CL**, Wharam M, Colombani P, Arceci RJ. Successful treatment of a child with late-onset T-cell post-transplant lymphoproliferative disorder/lymphoma. Pediatr Blood Cancer 50(3):667-70, 3/2008. PMID: 17318876.
45. Lucas KG, **Schwartz C**, Kaplan J. Allogeneic stem cell transplantation in a patient with relapsed Ewing sarcoma. Pediatr Blood Cancer 51(1):142-4, 7/2008. PMID: 18266223.
46. Constine LS, Tarbell N, Hudson MM, **Schwartz C**, Fisher SG, Muhs AG, Basu SK, Kun LE, Ng A, Mauch P, Sandhu A, Culakova E, Lyman G, Mendenhall N. Subsequent malignancies in children treated for Hodgkin's disease: associations with gender and radiation dose. Int J Radiat Oncol Biol Phys 72(1):24-33, 9/2008. PMCID: PMC3531236.
47. Basu SK, **Schwartz C**, Fisher SG, Hudson MM, Tarbell N, Muhs A, Marcus KJ, Mendenhall N, Mauch P, Kun LE, Constine LS. Unilateral and bilateral breast cancer in women surviving pediatric Hodgkin's disease. Int J Radiat Oncol Biol Phys 72(1):34-40, 9/2008. PMCID: PMC3532026.
48. Trask CL, Welch JJ, Manley P, Jelalian E, **Schwartz CL**. Parental needs for information related to neurocognitive late effects from pediatric cancer and its treatment. Pediatr Blood Cancer 52(2):273-9, 2/2009. PMID: 18985741.
49. Cole PD, **Schwartz CL**, Drachtman RA, de Alarcon PA, Chen L, Trippett TM. Phase II study of weekly gemcitabine and vinorelbine for children with recurrent or refractory Hodgkin's disease: a children's oncology group report. J Clin Oncol 27(9):1456-61, 3/2009. e-Pub 2/2009. PMCID: PMC2668553.
50. Loeb DM, Garrett-Mayer E, Hobbs RF, Prideaux AR, Sgouros G, Shokek O, Wharam MD, Scott T, **Schwartz CL.** Dose-finding study of 153Sm-EDTMP in patients with poor-prognosis osteosarcoma. Cancer 115(11):2514-22, 6/2009. PMCID: PMC2974628.
51. **Schwartz CL**, Constine LS, Villaluna D, London WB, Hutchison RE, Sposto R, Lipshultz SE, Turner CS, deAlarcon PA, Chauvenet A. A risk-adapted, response-based approach using ABVE-PC for children and adolescents with intermediate- and high-risk Hodgkin lymphoma: the results of P9425. Blood 114(10):2051-9, 9/2009. e-Pub 7/2009. PMCID: PMC2744567.
52. Kelly MJ, Vivier PM, Panken TM, **Schwartz CL**. Bacteremia in febrile nonneutropenic pediatric oncology patients. Pediatr Blood Cancer 54(1):83-7, 1/2010. PMID: 19774640.
53. Silverman LB, Supko JG, Stevenson KE, Woodward C, Vrooman LM, Neuberg DS, Asselin BL, Athale UH, Clavell L, Cole PD, Kelly KM, Laverdière C, Michon B, Schorin M, **Schwartz CL**, O'Brien JE, Cohen HJ, Sallan SE. Intravenous PEG-asparaginase during remission induction in children and adolescents with newly diagnosed acute lymphoblastic leukemia. Blood 115(7):1351-3, 2/2010. e-Pub 12/2009. PMCID: PMC2826760.
54. Silverman LB, Stevenson KE, O'Brien JE, Asselin BL, Barr RD, Clavell L, Cole PD, Kelly KM, Laverdiere C, Michon B, Schorin MA, **Schwartz CL**, O'Holleran EW, Neuberg DS, Cohen HJ, Sallan SE. Long-term results of Dana-Farber Cancer Institute ALL Consortium protocols for children with newly diagnosed acute lymphoblastic leukemia (1985-2000). Leukemia 24(2):320-34, 2/2010. e-Pub 12/2009. PMCID: PMC2820141.
55. Ng A, Constine LS, Advani R, Das P, Flowers C, Friedberg J, Hodgson DC, **Schwartz CL,** Wilder RB, Wilson LD, Yunes MJ. ACR Appropriateness Criteria: follow-up of Hodgkin's lymphoma. Curr Probl Cancer 34(3):211-27, May-Jun, 5/2010. PMID: 20541059.
56. Fitzgerald TJ, Bishop-Jodoin M, Cicchetti MG, Hanusik R, Kessel S, Laurie F, McCarten KM, Moni J, Pieters RS, Rosen N, Ulin K, Urie M, Chauvenet AR, Constine LS, Deye J, Vikram B, Friedman D, Marcus RB, Mendenhall NP, Williams JL, Purdy J, Saltz J, **Schwartz CL,** White KS, Wolden S. Quality of radiotherapy reporting in randomized controlled trials of Hodgkin's lymphoma and non-Hodgkin's lymphoma: in regard to Bekelman and Yahalom (Int J Radiat Oncol Biol Phys 2009;73:492-498). Int J Radiat Oncol Biol Phys 77(1):315-6, 5/2010. PMCID: PMC2908913.
57. Cole PD, McCarten KM, Drachtman RA, Alarcon Pd, Chen L, Trippett TM, **Schwartz CL**. Early [¹ F]fluorodeoxyglucose positron emission tomography-based response evaluation after treatment with gemcitabine and vinorelbine for refractory Hodgkin disease: a children's oncology group report. Pediatr Hematol Oncol 27(8):650-7, 11/2010. PMCID: PMC3083978.

58. Loeb DM, Hobbs RF, Okoli A, Chen AR, Cho S, Srinivasan S, Sgouros G, Shokek O, Wharam MD, Scott T, **Schwartz CL**. Tandem dosing of samarium-153 ethylenediamine tetramethylene phosphoric acid with stem cell support for patients with high-risk osteosarcoma. Cancer 116(23):5470-8, 12/2010. e-Pub 8/2010. PMCID: PMC2991401.
59. Das P, Ng A, Constine LS, Advani R, Flowers C, Friedberg J, Hodgson DC, **Schwartz CL**, Wilder RB, Wilson LD, Yunes MJ. ACR Appropriateness Criteria® on Hodgkin's lymphoma-unfavorable clinical stage I and II. J Am Coll Radiol 8(5):302-8, 5/2011. PMID: 21531305.
60. Vrooman LM, Neuberg DS, Stevenson KE, Asselin BL, Athale UH, Clavell L, Cole PD, Kelly KM, Larsen EC, Laverdière C, Michon B, Schorin M, **Schwartz CL**, Cohen HJ, Lipshultz SE, Silverman LB, Sallan SE. The low incidence of secondary acute myelogenous leukemia in children and adolescents treated with dexrazoxane for acute lymphoblastic leukemia: a report from the Dana-Farber Cancer Institute ALL Consortium. Eur J Cancer 47(9):1373-9, 6/2011. e-Pub 4/2011. PMCID: PMC3736806.
61. Kenney LB, Bradeen H, Kadan-Lottick NS, Diller L, Homans A, **Schwartz CL.** The current status of follow-up services for childhood cancer survivors, are we meeting goals and expectations: a report from the Consortium for New England Childhood Cancer Survivors. Pediatr Blood Cancer 57(6):1062-6, 12/2011. e-Pub 1/2011. PMID: 21910208.
62. Voss SD, Chen L, Constine LS, Chauvenet A, Fitzgerald TJ, Kaste SC, Slovis T, **Schwartz CL**. Surveillance computed tomography imaging and detection of relapse in intermediate- and advanced-stage pediatric Hodgkin's lymphoma: a report from the Children's Oncology Group. J Clin Oncol 30(21):2635-40, 7/2012. e-Pub 6/2012. PMID: 22689804.
63. Janeway KA, Barkauskas DA, Krailo MD, Meyers PA, **Schwartz CL**, Ebb DH, Seibel NL, Grier HE, Gorlick R, Marina N. Outcome for adolescent and young adult patients with osteosarcoma: a report from the Children's Oncology Group. Cancer 118(18):4597-605, 9/2012. e-Pub 1/2012. PMCID: PMC4008337.
64. Tebbi CK, Mendenhall NP, London WB, Williams JL, Hutchison RE, Fitzgerald TJ, de Alarcón PA, **Schwartz C**, Chauvenet A. Response-dependent and reduced treatment in lower risk Hodgkin lymphoma in children and adolescents, results of P9426: a report from the Children's Oncology Group. Pediatr Blood Cancer 59(7):1259-65, 12/2012. e-Pub 8/2012. PMCID: PMC3468662.
65. Terezakis SA, Metzger ML, Hodgson DC, **Schwartz CL**, Advani R, Flowers CR, Hoppe BS, Ng A, Roberts KB, Shapiro R, Wilder RB, Yunes MJ, Constine LS. ACR Appropriateness Criteria Pediatric Hodgkin Lymphoma. Pediatr Blood Cancer 61(7):1305-12, 7/2014. e-Pub 2/2014. PMID: 24616347.
66. Gorlick S, Barkauskas DA, Krailo M, Piperdi S, Sowers R, Gill J, Geller D, Randall RL, Janeway K, **Schwartz C,** Grier H, Meyers PA, Gorlick R, Bernstein M, Marina N. HER-2 expression is not prognostic in osteosarcoma; a Children's Oncology Group prospective biology study. Pediatr Blood Cancer 61(9):1558-64, 9/2014. e-Pub 4/2014. PMCID: PMC4288578.
67. Friedman DL, Chen L, Wolden S, Buxton A, McCarten K, FitzGerald TJ, Kessel S, De Alarcon PA, Chen AR, Kobrinsky N, Ehrlich P, Hutchison RE, Constine LS, **Schwartz CL**. Dose-Intensive Response-Based Chemotherapy and Radiation Therapy for Children and Adolescents With Newly Diagnosed Intermediate-Risk Hodgkin Lymphoma: A Report From the Children's Oncology Group Study AHOD0031. J Clin Oncol 32(32):3651-8, 11/2014. e-Pub 10/2014. PMCID: PMC4220044.
68. Trippett TM, **Schwartz CL**, Guillerman RP, Gamis AS, Gardner S, Hogan S, London WB, Chen L, de Alarcon P. Ifosfamide and vinorelbine is an effective reinduction regimen in children with refractory/relapsed Hodgkin lymphoma, AHOD00P1: A children's oncology group report. Pediatr Blood Cancer 62(1):60-4, 1/2015. e-Pub 10/2014. PMCID: PMC4465390.
69. Dharmarajan KV, Friedman DL, FitzGerald TJ, McCarten KM, Constine LS, Chen L, Kessel SK, Iandoli M, Laurie F, **Schwartz CL**, Wolden SL. Radiotherapy Quality Assurance Report From Children's Oncology Group AHOD0031. Int J Radiat Oncol Biol Phys 91(5):1065-71, 4/2015. e-Pub 2/2015. PMID: 25670539.

70. Harrison DJ, **Schwartz C**. Survivorship. J Surg Oncol 111(5):648-55, 4/2015. e-Pub 12/2014. PMID: 25557722.
71. Dharmarajan KV, Friedman DL, **Schwartz CL**, Chen L, FitzGerald TJ, McCarten KM, Kessel SK, Iandoli M, Constine LS, Wolden SL. Patterns of Relapse From a Phase III Study of Response-Based Therapy for Intermediate-Risk Hodgkin Lymphoma (AHOD0031): A Report From the Children's Oncology Group. Int J Radiat Oncol Biol Phys 92(1):60-6, 5/2015. e-Pub 12/2014. PMCID: PMC4395527.
72. Horton TM, Drachtman RA, Chen L, Cole PD, McCarten K, Voss S, Guillerman RP, Buxton A, Howard SC, Hogan SM, Sheehan AM, López-Terrada D, Mrazek MD, Agrawal N, Wu MF, Liu H, De Alarcon PA, Trippet TM, **Schwartz CL**. A phase 2 study of bortezomib in combination with ifosfamide/vinorelbine in paediatric patients and young adults with refractory/recurrent Hodgkin lymphoma: a Children's Oncology Group study. Br J Haematol 170(1):118-22, 7/2015. e-Pub 4/2015. PMCID: PMC4478135.
73. Chow EJ, Asselin BL, **Schwartz CL**, Doody DR, Leisenring WM, Aggarwal S, Baker KS, Bhatia S, Constine LS, Freyer DR, Lipshultz SE, Armenian SH. Late Mortality After Dexrazoxane Treatment: A Report From the Children's Oncology Group. J Clin Oncol 33(24):2639-45, 8/2015. e-Pub 5/2015. PMCID: PMC4534526.
74. Macpherson CF, Hooke MC, Friedman DL, Campbell K, Withycombe J, **Schwartz CL**, Kelly K, Meza J. Exercise and Fatigue in Adolescent and Young Adult Survivors of Hodgkin Lymphoma: A Report from the Children's Oncology Group. J Adolesc Young Adult Oncol 4(3):137-140, 9/2015. PMCID: PMC4575513.
75. **Schwartz CL**, Wexler LH, Krailo MD, Teot LA, Devidas M, Steinherz LJ, Goorin AM, Gebhardt MC, Healey JH, Sato JK, Meyers PA, Grier HE, Bernstein ML, Lipshultz SE. Intensified Chemotherapy With Dexrazoxane Cardioprotection in Newly Diagnosed Nonmetastatic Osteosarcoma: A Report From the Children's Oncology Group. Pediatr Blood Cancer 63(1):54-61, 1/2016. e-Pub 9/2015. PMID: 26398490.
76. Appel BE, Chen L, Hutchison RE, Hodgson DC, Ehrlich P, Constine LS, **Schwartz CL**. Minimal Treatment of Low-risk Pediatric Lymphocyte Predominant Hodgkin Lymphoma: A report from the Children's Oncology Group. J Clin Oncol 34(10); 2372-9. 7/2016 PMID: 27185849.
77. Bishop MW, Chang YC, Krailo MD, Meyers PA, Provisor AJ, **Schwartz CL**, Marina NM, Teot LA, Gebhardt MC, Gorlick R, Janeway KA, Chou AJ. Assessing the Prognostic Significance of Histologic Response in Osteosarcoma: A Comparison of Outcomes on CCG-782 and INT0133-A Report From the Children's Oncology Group Bone Tumor Committee. Pediatr Blood Cancer 63(10);1 737-43. 10/2016. PMID: 27128693.
78. Marina N, Smeland S, Bielack S, Bernstein M, Jovic G, Krailo M, Hook J, Arndt C, vanden Berg, H, Brennan B, Brichard B, Brown KLB, Butterfass-Bahloul T, Calaminus G, Erikkson M, Gebhardt M, Gelderblom H, Gerss J, Goldsby R, Goorin A, Gorlick R, Grier HE, Hale JP, Sundby Hall K, Hardes J, Helmke K, Hogendoorn PCW, Isakoff M, Janeway KA, Jurgens H, Kager L, Kuhne T, Lau C, Leavey P, Lessnick SL, Mascarenhas L, Meyers PA, Mottl H, Nathrath M, Papai Z, Randall L, Reichardt P, Renard MMB, Hawkins DS, Safwat SS, **Schwartz C**, Stevens MCG, Strauss S, Teot L, Werner M, Sydes M. Randomized Comparison of MAPIE vs MAP in patients with a Poor Response to pre-operative chemotherapy for newly-diagnosed high-grade osteosarcoma: results from the EURAMOS-1 trial. Lancet Oncology 17(10),1396-1408. 10/2016. PMID: 27569442.
79. Charpentier A-M,Friedman DL, Wolden S, **Schwartz C**, Gill B, Hodgson D. Predictive Factor Analysis of Response-adapted Treatment for Chemotherapy-sensitive Pediatric Hodgkin Lymphoma: a Report from Children's Oncology Group Trial AHOD0031. Int J Radiation Oncology Biology Physics 96(5):943-950, 12/2016. PMID: 27869096.**Schwartz CL**, Chen L, McCarten K, Wolden S, Constine LS, Hutchison RE, deAlarcon PA, Keller FG, Kelly KM, Trippet TA, Voss SD, Friedman DL. Childhood Hodgkin International Prognostic Score (CHIPS) Predicts EFS in Hodgkin Lymphoma. Pediatr Blood Cancer. e-Pub 10/2016. PMID: 27786406.

80. Kaul S, Avila J, Mutambudzi M, Russell H, Kirchhoff AC, **Schwartz CL**. Mental Distress and Healthcare Utilization among Survivors of Adolescent and Young Adult Cancer: A Cross-Sectional Analysis of the National Health Interview Survey. Cancer. e-Pub 11/2016. PMID: 27859009.
81. **Schwartz CL**, Chen L, McCarten K, Wolden S, Constine LS, Hutchison RE, de Alarcon PA, Keller FG, Kelly KM, Trippet TA, Voss SD, Friedman DL.Childhood Hodgkin International Prognostic Score (CHIPS) Predicts event-free survival in Hodgkin Lymphoma: A Report from the Children's Oncology Group. Pediatr Blood Cancer. 2016 Oct 27. doi: 10.1002/pbc.26278. [Epub ahead of print].
82. Fernandez KS, **Schwartz CL**, Chen l, Chauvenet A, Constine LS, deAlarcon PA. Outcome of Adolescents and Young Adults Compared to Children with Hodgkin Lymphoma Treated with Response-Based Chemotherapy on Pediatric Protocols: Children's Oncology Group Report. J Clin Oncol, Submitted 2016.
83. Welch JG, **Schwartz CL**, Higman M, Chen L, Buxton A, Kanakry JA, Kahwash SB, Hutchison RE, Friedman DL, Ambinder RF. Epstein-Barr virus DNA in serum as an early prognostic marker in children and adolescents with Hodgkin lymphoma. Blood Advances. Re-submitted, 2016.
84. Keller FG, Castellino SM, Chen L, Pei Q, Voss SD, McCarten KM, Senn SL, Buxton A, Constine LS, **Schwartz CL**. Results of the AHOD0431 Trial of Response Adapted Therapy and a Salvage Strategy for Limited-Stage Classical Hodgkin Lymphoma: A Report from the Children's Oncology Group, J Clin Oncol, Resubmitted 2016.

Invited Articles

1. **Schwartz CL**, Cohen HJ. Preleukemic syndromes and other syndromes predisposing to leukemia. Pediatr Clin NA 35:854-871, 1988.
2. Hobbie WL, **Schwartz CL**. Endocrine late effects among survivors of cancer. Seminars in Oncol Nursing 5:14-21, 1989.
3. **Schwartz CL**. Late effects of treatment in long-term survivors of cancer. Cancer Treat Rev 21(4):355-66, 1995.
4. **Schwartz CL.** Long-term survivors of childhood cancer: the late effects of therapy. Oncologist 4:45-54, 1999.
5. **Schwartz CL.** The management of Hodgkin disease in the young child. Curr Opin Pediatr 15(1):10-6, 2/2003. PMID: 12544266.
6. **Schwartz CL**. Prognostic factors in pediatric Hodgkin disease. Curr Oncol Rep 5:498-504, 2003.
7. **Schwartz CL**. The Management of Hodgkin disease in the Young Child. Current Opinions in Oncology 15(1):10-16, 2003.
8. Mauz-Körholz C, Metzger ML, Kelly KM, **Schwartz CL**, Castellanos ME, Dieckmann K, Kluge R, Körholz D. Pediatric Hodgkin Lymphoma. Journal of Clinical Oncology 33(27):211-225, 1/2015. e-Pub . PMID: 26304892.
9. **Schwartz CL.** Very Late Outcomes of Wilms Tumor Survivors: British Childhood Cancer Study. Journal of Clinical Oncology 34(15):Podcast, 5/2016.

Editorials

1. **Schwartz CL.** Health status of childhood cancer survivors: cure is more than the eradication of cancer. JAMA 290(12):1641-3, 9/2003. PMID: 14506124.
2. **Schwartz, CL**. Health status of adult long-term survivors of childhood cancer: a report from the Childhood Cancer Survivor Study. J Pediatr 144(3):407-408, 2004.

3. **Schwartz, CL**. Curing Hodgkin Disease: Too much of a good thing. Current Controversies in Oncology, ASCO News and Forum:21-22, 4/2007.

Other Articles

N/A

Abstracts (partial listing of last 5 years)

1. **Schwartz CL**, Friedman DL, McCarten K, Wolden S L, Voss S, Constine LS. Chen L. Early PET and CT as predictors of outcome in pediatric Hodgkin lymphoma, Society International Pediatric Oncology. Pediatric Blood Cancer 57(5):75, 2011.
2. Castellino C, keller F, Dunphy C, Nachman J, Constine L, Chen L, **Schwartz C**. Mixed Cellularity Histology Confers Favorable Prognosis in Children and Adolescents with Low Risk Hodgkin Lymphoma (HL). 1st International Symposium on Childhood, Adolescent, and Young Adult Hodgkin Lymphoma, 2011.
3. **Schwartz CL,** Friedman DL, McCarten K, Wolden SL, Voss S, Constine LS, Chen L. Predictors of Early Response and Event Free Survival in Hodgkin Lymphoma (HL): PET vs CT Imaging. Proceedings ASCO, 29: 505s, 2011.
4. Fernandez KS, Chen L, **Schwartz C**, Chauvenet A, deAlarcon PA. Survival in Adolescents and Young Adults with Hodgkin Lymphoma Treated with Response-Based Chemotherapy on P9425 and P9426 Protocols: A Report From the Children's Oncology Group. Blood 118:91, 2011.
5. **Schwartz C**, Chen L, Constine L, Wolden S, Keller FG, Kelly KM, Friedman DL. The Childhood Hodgkin International Prognostic Score (CHIPS) for Predicting Event Free Survival in Pediatric and Adolescent Hodgkin Lymphoma. Blood 118:3649, 2011.
6. Appel B, Chen L, Hutchison RE, Hodgson DC, Ehrlich P, Constine LC, **Schwartz CL.** 18F-FDG (FDG) PET Five-Point Visual and Quantitative SUV-Based Assessment and Prognosis in Pediatric Hodgkin Lymphoma (HL). ASH, 2012.
7. **Schwartz CL**, Kaplan J, Chen L, Hutchison R, Friedman D. Albumin: A Predictor of EFS in MC Hodgkin Lymphoma. Platform Presentation, SIOP, 2012.
8. Gorlick R, Barkauskas D, Krailo M, Piperdi S, Sowers R, Gorlick S, Gill J, Geller D, Randall L, Janeway K, **Schwartz C,** Grier H, Meyers P, Bernstein M, Marina N. HER2 Expression is Not Prognostic in Osteosarcoma. CTOS, 2012.
9. Kopp L, Bernstein ML, **Schwartz CL**, Ebb D, Krailo MD, Frier HE, Meyers PA,k Wexler LH, Marina N, Womer R, Janeway KA, Gorlick RG, Lipshultz SE. The Effects of Dexrazoxane on Cardiac Function and Secondary Malignant Neoplasms in Patients with Osteosarcoma (OS). Proceedings ASCO, 30: 606s, 2012.
10. Appel B, Ehrlich P, Chen L, Hutchison RE, Hodgson DC, Constine LS, **Schwartz CL.** Treatment of Pediatric Stage IA Lymphocyte-Predominant Hodgkin Lymphoma with Surgical Resection Alone: A COG Report. Proceedings ASCO, 2012.
11. **Schwartz CL**, Hirway P, Ader J, Bradeen HA, Grewal SS, Huang MS, Kadan-Lottick N, Usmani N, Kenney LB. Barriers to Survivorship Care - CONNECCS. Proceedings ASCO, 2013.
12. Cho SY, McCarten KM, Chen Lu, Friedman DL, Wolden SL, Karolczuk K, **Schwartz CL**, Kelly KM. ^{18}F-FDG (FDG) PET Five-Point Visual and Quantitative SUV-BasedAssessment and Prognosis in Pediatric Hodgkin Lymphoma (HL). A Preliminary Retrospective Analysis of Children's Oncology Group (COG) AHOD0031. ASH Poster Presentation, December 2012.
13. Appel B, Chen L, Hutchison RE, Hodgson DC, Ehrlich P, Constine LC, **Schwartz CL**. Treatment of pediatric lymphocyte predominant Hodgkin lymphoma (LPHL): A report from the Children's Oncology Group,. Proceedings ASCO, 2013.
14. Charpentier A, Friedman D, Wolden S, **Schwartz C,** Gill B, Sykes J, Albert-Green A, constine L, Kelly K, Hodgson D. Predictive Factor Analysis of Response-adapted Treatment for Chemotherapy-sensitive Pediatric Hodgkin Lymphoma: a Report from Children's Oncology Group Trial AHOD0031 , 2014.

15. Castellino SM , Keller F, Voss S, Cho S, Constine L, Homson JT, Dunphy C, McCarten K, Chen L, **Schwartz CL.** Outcomes and Patterns of Failure in Children/Adolescents with Low Risk Hodgkin Lymphoma (HL) Who are FDG-PET (PET3) Positive After AVPC Therapy. ISCAYAHL, 5/2015.
16. Subbiah V, Anderson P, W. Huh, Hess K, Ravi V, Fernandez JG, Lui A, Daw N, Somaiah N, Kappadath C, Ravissini G, Ludwig J, ChawlaS, Benjamin RS, Patel S, **Schwartz CL**, Hong DS, Rohren E. Safety, Tolerability, and Feasibility of Alpha Particle Radium-223 Dichloride (223RaCl2) in High Risk Osteosarcoma: Phase I Dose Escalation Trial. Connective Tissue Oncology Society, 10/2015.
17. Moonat HR, Wang WL, Kenneth R. Hess, **Schwartz CL**, Moise M, Patel S, Benjamin RS, Lewis VO, Lazar A, Daw NC. Small Cell Osteosarcoma: The MD Anderson Cancer Center Experience. Connective Tissue Oncology Society, 10/2015.
18. Spaker-Perlman HL, Barkauskas DA, Meyers PA; **Schwartz CL,** Ebb DH, Doski JJ, Krailo MD, Gorlick R, Janeway KA. Survivial After Recurrence in Osteosarcoma: A Report from the Chidlren's Oncology Group. Connective Tissue Oncology Society, 10/2015.
19. Appel B, Chen L, Hutchison RE, Hodgson DC, Ehrlich P, Constine LC, **Schwartz CL**. 18F-FDG (FDG) PET Five-Point Visual and Quantitative SUV-Based Assessment and Prognosis in Pediatric Hodgkin Lymphoma (HL), A Preliminary Retrospective Analysis of Children's Oncology Group (COG) AHOD0031. Blood (ASH poster) Supplement, 2015.
20. Henderson TO, Parson SK, Wroblewski K, Chen L, Hong F, Smith SM, McNeer J, Advani R, Gascoyne RD, Constine L, Horning SJ, Bartlett NL, MD, Shah BD, MD, Connors JM, Leonard JP, Kahl BS, Kelly KM, **Schwartz CL**, Friedman DL,Gordon Li, Evens AM. Outcomes in Adolescents and Young Adults (AYA) with Hodgkin Lymphoma (HL) Treat on US Cooperative Group Protocols: An Adult Intergroup (E2496) and Children's Oncology Group (COG AHOD0031). Blood (ASH poster) Supplement, 2015.
21. Kelly KM, MD, Cole PD, Chen L, Roberts KB, Hodgson DC, McCarten K, Steve Y Cho SY, **Schwartz C.** Phase III Study of Response Adapted Therapy for the Treatment of children with Newly Diagnosed Very HIgh Risk Hodgkin Lymphoma (Stages IIIB/IVB) (AHOD0831): A Report from the Children's Oncology Group. Blood, 2015.
22. Mottok A, Johnston RL, Chan FC, Scott DW, Friedman DL, **Schwartz CL**, Kelly K, Terzah M. Horton TM.Steidl C. Prediction of Primary Treatment Outcome Using Gene Expression Profiling of Pre-Treatment Biopsies Obtained from Childhood and Adolescent Hodgkin Lymphoma Patients. Blood Supplement(126), 12/2015.
23. Shaw A, Schadler K, **Schwartz CL**, Kleinerman E. Exercise Preconditioning Mitigates Doxorubicin Uptake in Cardiac Tissue. Pediatric Blood and Cancer 63(S1):S66 (#4010), 5/2016.
24. Lewis B, Kresta K, Shaw A, Robert R, Maetzold B, Valderrama R, Harman N, Cion I, Franklin Q Morse E, Skillman D, **Schwartz CL**, Tewari P. Totally Excited about Moving, Mobility and Exercise: a Multidisciplinary Effort to Help Increase Mobility for Children, Adolescents and Young Adults During their Inpatient Admission. Pediatric Blood and Cancer 63(S1):S9, 5/2016.
25. Chow EF, Doody DR, Armenian SH, Aggarwal S, Baker KS, Bhatia S, Blythe NA, Constine LS, Freyer DR, Kopp LM, Leisenring WM, Sasake N, Vrooman LM, Asselin BL, **Schwartz CL**, Lipshultz SE. Effect of Dexrazoxane on Heart Function Among LongTerm Leukemia and Lymphoma: A Report from the Children's Oncology Group (COG). ASH oral presentation 2016. In Press.
26. Giulino-Roth L, Pei Q, Buxton A, Wolden S, Constine LS, Kelly K, **Schwartz CL,** Friedman DL. Subsequent Malignant Neoplasms Among Children and Adolescents with Hodgkin Lymphoma Treated with Response-Adapted Therapy: A Report From The Children's Oncology Group Study AHOD0031. ASH poster 2016. In Press.
27. Moonat HR, Roxas M, Huh WW, Herzog CE, Piha-Paul S, Daw NC, Rytting M, Mote E, Ward EN, Amin Y, Meric-Bernstam F, **Schwartz CL**, Subbiah V. Phase I evaluation of Everolimus (mTOR inhibitor) in combination with Vandetanib (multikinase inhibitor of EGFR, VEGFR, and

RET) in children, adolescents and young adults with advanced solid tumors. Cancer Research 76(5 Supplement) (#A48), 3/2016.

28. Mottok A, Johnston RL, Chan FC, Scott DW, Friedman DL, **Schwartz CL**, Kelly KM, Horton TM.Steidl C. Prediction of primary treatment response and outcome using digital gene expression. 10th International Symposium of Hodgkin Lymphoma, Cologne, Germany. October 2016.
29. Kelly KM, Cole PD, Chen L, Roberts KB, Hodgson DC, McCarten K, Cho S, **Schwartz CL**. Phase III Study of Response Adapted Therapy for the Treatment of Children with Newly Diagnosed Very High Risk Hodgkin Lymphoma (Stages IIIB/IVB) (AHOD0831): A Report from the Children's Oncology Group. 10th International Symposium of Hodgkin Lymphoma, Cologne, Germany. October 2016.
30. **Schwartz CL,** Wolden S, Constine LC, Chen L, Friedman DL. CHIPS as a predictor of outcome in radiated vs. unradiated HL. 10th International Symposium of Hodgkin Lymphoma, Cologne, Germany. October 2016.

Book Chapters

1. **Schwartz CL**, Cohen HJ. Myeloproliferative and myelodysplastic syndromes. In: Principles and Practice of Pediatric Oncology. Ed(s) Pizzo PA, Poplack DG. JB Lippincott Company: Philadelphia, PA, 397-413, 1989.
2. **Schwartz CL.** Myelodysplastic and myeloproliferative disorders in children. In: Hematology: Basic Principles and Practice. Ed(s) Hoffman R, Benz EJ, Shattil S, Furie B, Cohen HJ. Churchill Livingstone: New York, 889-896, 1991.
3. **Schwartz CL**. Cancers in Childhood. In: Pediatric Primary Care. Ed(s) Hoekelman R. Mosby: St. Louis, MO, 1157-1179, 1992.
4. **Schwartz, CL**. Retinoblastoma. In: Merck Manual, 16th Edition. Ed(s) Berkow R. Merck, Sharp & Dohme Research Laboratories: Rahway, New Jersey, 2205-2206, 1992.
5. **Schwartz CL**, Cohen HJ. Myeloproliferative and myelodysplastic syndromes. In: Principles and Practice of Pediatric Oncology, 2n edition. Ed(s) Pizzo PA, Poplack DG. JB Lippincott Company: Philadelphia, PA, 519-535, 1993.
6. **Schwartz CL**, Constine LC, Putnam TC, Cohen HJ. Pediatric solid tumors. In: Clinical Oncology, A Multidisciplinary Approach, 7th edition. Ed(s) Saunders WB, 251-298, 1993.
7. **Schwartz CL**, Truesdell SA, Clark EB. The Use of the QTc in screening for anthracycline related cardiotoxicity. In: Cardiac toxicity cardiomyopathy after Cancer Therapy. Ed(s) Bricker GT, D'Angio GJ, Green DM. John Wiley and Sons: New York, 103-108, 1993.
8. **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Algorithms of Late Effects by Disease. In: Survivors of Childhood Cancer - Assessment and Management. Ed(s) Schwartz CL, Hobbie WL, Constine LC, Ruccione KS. Mosby Yearbook: Missouri, 7-20, 1994.
9. Truesdell S, **Schwartz CL**, Clark E. Cardiovascular Effects of Therapy. In: Survivors of Childhood Cancer - Assessment and Management. Ed(s) Schwartz CL, Hobbie WL, Constine LC, Ruccione KS. Mosby Yearbook, Inc.: Missouri, 159-176, 1994.
10. Constine LS, Hobbie WL, **Schwartz CL**. Facilitated Assessment of Chronic Treatment Effects by Symptom and Organ Systems. In: Survivors of Childhood Cancer - Assessment and Management. Ed(s) **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Mosby Yearbook, Inc.: Missouri, 21-80, 1994.
11. Constine LS, Hobbie WL, **Schwartz CL**. Facilitated Assessment of Chronic Treatment Effects by Symptom and Organ Systems. In: Survivors of Childhood Cancer - Assessment and Management. Ed(s) **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Mosby Yearbook, Inc.: Missouri, 7-20, 1994.
12. McDonald S, Rubin P, **Schwartz CL**. Pulmonary Effects of Antineoplastic Therapy. In: Survivors of Childhood Cancer - Assessment and Management. Ed(s) **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Mosby Yearbook, Inc.: Missouri, 177-196, 1994.

13. Schwartz CL, Hobbie WL, Ruccione K, Constine LS. The Establishment of the follow-up Clinic. In: Survivors of Childhood Cancer - Assessment and Management. Ed(s) **Schwartz CL,** Hobbie WL, Ruccione K, Constine LS. Mosby Yearbook, Inc.: Missouri, 367-389, 1994.
14. Constine LS, Schwartz CL. The Thyroid Gland. In: Survivors of Childhood Cancer - Assessment and Management. Ed(s) **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Mosby Yearbook, Inc: Missouri, 151-158, 1994.
15. **Schwartz, CL.** Myelodysplastic and myeloproliferative disorders in children. In: Hematology: Basic Principles and Practice, 2nd edition. Ed(s) Benz EJ, Cohen HJ, Furie B, Hoffman R, Sharril S. Churchill Livingstone: New York, 1184-1192, 1995.
16. **Schwartz CL**. Cancers in Childhood. In: Pediatric Primary Care, 3rd Edition. Ed(s) Hoekelman R. Mosby Yearbook, Inc: Missouri, 1217-1238, 1997.
17. Korones DN, **Schwartz CL**. Myeloproliferative and myelodysplastic syndromes. In: Principles and Practice of Pediatric Oncology, 3rd Edition. Ed(s) Oldham KT, Colombani PM, Foglia RP. Lippincott-Raven: Philadelphia, 537-48, 1997.
18. **Schwartz CL**, Cohen HJ. Myeloproliferative and myelodysplastic syndromes. In: Principles and Practice of Pediatric Oncology, 3rd Edition. Ed(s) PA Pizzo, Poplack DG. JB Lippincott Company: Philadelphia, 505-521, 1997.
19. Korones DN, **Schwartz CL**. Principles of Oncology. In: Surgery of Infants and Children. Ed(s) Oldham KT, Colombani PM, Foglia RP. Lippincott-Raven: Philadelphia, 537-48, 1997.
20. Chauvenet, A, **Schwartz CL**, Weiner M. Hodgkin's Disease in Children and Adolescents. In: Cancer Medicine, 5th Edition. Ed(s) Holland, Frei. B.C. Decker Inc, 2140-2150, 2000.
21. Schwartz, CL. Myelodysplastic and myeloproliferative disorders in children. In: Hematology: Basic Principles and Practice. Ed(s) Hoffman R, Benz EJ, Shattil SJ, Furie B, Cohen HJ, Silberstein LE, McGlave P. Churchill Livingstone: New York, 1205-1212, 2000.
22. Constine LC, Paidas C, **Schwartz CL**, Korones DN. Pediatric solid tumors. In: Clinical Oncology, A Multidisciplinary Approach, 8th Edition. Ed(s) Rubin P. WB Saunders, 336-404, 2001.
23. Ruble K, **Schwartz C**. Sports for the Child with Cancer. In: " Educating the Child with Cancer: A Guide for Parents & Teachers, Nancy Keen. Candlelighters Childhood Cancer Foundation, 2003.
24. Loeb D, Castleberry R, **Schwartz CL**. Myelodysplastic and myeloproliferative disorders in children. In: Hematology: Basic Principles and Practice. Ed(s) Hoffman R, Benz EJ, Shattil SJ, Furie B, Cohen HJ, Silberstein LE, McFlave P. Churchill Livingstone: New York, 1297-1306, 2004.
25. Simpson L, Baird K, Korones DN, **Schwartz CL**. Principles of Oncology. In: Surgery of Infants and Children. Ed(s) Foglia RP. Lippincott-Raven: Philadelphia, 2004.
26. **Schwartz CL**, Hobbie, WL, Constine, L. Algorithms of Late Effects by Disease. In: Survivors of Childhood and Adolescent Cancer – A Multidisciplinary Approach. Ed(s) Schwartz CL, Hobbie WL, Constine LC, Ruccione KS. Springer-Verlag & Co, 5-16, 2005.
27. **Schwartz CL**, Hobbie, WL, Constine, L. Facilitating Assessment of Late Effects by Organ System. In: Survivors of Childhood and Adolescent Cancer – A Multidisciplinary Approach. Ed(s) **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Springer-Verlag & Co, 17-34, 2005.
28. Biswas T, Constine LS, **Schwartz CL**. The Thyroid Gland. In: Survivors of Childhood and Adolescent Cancer – A Multidisciplinary Approach. Ed(s) **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Springer-Verlag & Co: Heidelberg, Germany, 125-132, 2005.
29. Hudson MM, Schwartz C, Constine LS. Treatment of Pediatric Hodgkin Lymphoma. In: Pediatric Lymphomas. Ed(s) Weinstein HJ, Hudson MM, Link MP. Springer: Heudekberg, Germany, 35-66, 2007.
30. Hinkle AS, **Schwartz CL**. Cancers in Childhood. In: Textbook of Pediatric Care. Ed(s) McInerny TK. American Academy of Pediatrics: Elk Grove Village, IL, 1873-1902, 2009.

31. **Schwartz CL**. Early Response-based Therapy for Children with Hodgkin Lymphoma: A Surrogate for Using Biology to Effect Cure and Minimize Toxicity. In: ASCO Educational Book, 2010.
32. Ruble K, **Schwartz C**. Physical Activity. In: Educating the Child with Cancer: A Guide for Parents & Teachers. Ed(s) Keen N. American Childhood Cancer Organization, 2011.
33. **Schwartz CL**. Hodgkin Lymphoma (Commentary). In: Evidence Based Pediatric Oncology, 3rd Edition. Ed(s) Pinkerton R, Shankar A, Matthay KK. Wiley-Blackwell, 105-108, 2013.

Books (edited and written)

1. **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Ed(s) Schwartz CL, Hobbie WL, Constine LC, Ruccione KS. Survivors of Childhood Cancer - Assessment and Management. Mosby Yearbook, Inc: Missouri, 1994.
2. **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Ed(s) Schwartz CL, Hobbie WL, Constine LC, Ruccione KS. Survivors of Childhood and Adolescent Cancer – A Multidisciplinary Approach. Springer-Verlag & Co.: Heidelberg, 2005.
3. **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Ed(s) **Schwartz CL**, Hobbie WL, Constine LC, Ruccione KS. Survivors of Childhood and Adolescent Cancer – A Multidisciplinary Approach, 3rd Edition. Springer-Verlag & Co: Heidelberg. In Press.

Letters to the Editor

N/A

Manuals, Teaching Aids, Other Teaching Publications

N/A

Other Publications

N/A

EDITORIAL AND REVIEW ACTIVITIES

Editor/Service on Editorial Board(s)

Editorial Board, Pediatric Blood and Cancer, 2004-2008

Associate Editor, Journal of Pediatric Hematology Oncology, 2013-present

Editorial Board, Cardio-Oncology, 2015-2017

Member of Editorial Review Board

Member, Journal of Clinical Oncology, 1997-1999

Member, Journal of Clinical Oncology, 2003-2006

Journal Reviewer

Cancer, 1989 - present

American Journal of Pediatric Hematology/Oncology, 1990-present

Journal of Clinical Oncology, 1990-present

International Journal of Radiation Oncology and Biological Physics, 1991-5

New England Journal of Medicine, 1996- present

Archives of Pediatric & Adolescent Medicine, 1997-

Journal National Cancer Institute, 1998

International Journal of Pediatric Hematology/Oncology, 2000

Mayo Clinic Procedures, 2002

Journal of American Medical Association (JAMA), 2003

Pediatric Blood and Cancer, 2004-present

Supportive Care in Cancer, 2012

Annals of Oncology, 2014

Pediatric Blood and Cancer, 2010-present

Other Editorial and Review Activities

N/A

TEACHING

Teaching Within Current Institution - The University of Texas MD Anderson Cancer Center (GSBS)

Formal Teaching

Courses Taught

Instructor, Ethics in Clinical Trials Research, The University of Texas MD Anderson Cancer Center (GSBS), Course Number: GS21 1102

Fall, 11/2014

Training Programs

N/A

Other Formal Teaching

Lecturer, Fellow Lecture - Hodgkin Lymphoma, survivorship, bone tumors, Course Hours: 1/yr

2014-present

Lecturer, MDAC Pediatric Resident conference on Solid Tumors, Course Hours: 3

2014-present

Supervisory Teaching

Committees

Advisory Committees

Mentor, Clinical research fellowship, Angela Shaw, 2015-present

Supervisory Committees

Masters in Clinical Investigation (Weill-Cornell), Douglas Harrison, 2012-2016.

Examining Committees

N/A

Direct Supervision

Undergraduate and Allied Health Students

N/A

Medical Students

N/A

Graduate Students

N/A

Postdoctoral Research Fellows

N/A

Clinical Residents and Fellows

N/A

Other Supervisory Teaching

N/A

Teaching Outside Current Institution

Formal Teaching

Courses Taught

Pediatric Oncology Fellowship Lectures, Johns Hopkins

1994-2005

Moderator, Annual Pediatric Trends, Johns Hopkins University Office of Continuing Medical Education, Baltimore, MD
1995-1999

Instructor, Causes of Childhood Cancer, 25th Annual Pediatric Trends, Johns Hopkins University Office of Continuing Medical Education
1997

Coordinator, Instructor and Section Leader, 2nd year Pathophysiology Course, Pediatric Leukemia, Johns Hopkins University
1997-2000

Pediatric Leukemia, Johns Hopkins University
1998-1999

Coordinator and Instructor, Lecture Series for Pediatric Residents, Johns Hopkins University School of Medicine
2000-2005

Instructor, Quality of Life in Survivors of Childhood Cancer, 32nd Annual Pediatric Trends, Johns Hopkins University Office of Continuing Medical Education
2004

Pediatric Oncology Fellowship Lectures, RIH/Brown University
2005-2013

Instructor, Pediatric Noon Conference, Hodgkins Lymphoma Survivors of Childhood Cancer, Brown University
2006-2013

Training Programs
N/A

Other Formal Teaching
N/A

Supervisory Teaching

Committees

Advisory Committees

MPH Project Committee, Brown University, Kaplan, Joel, DO, 2005-2007

MPH Project Committee, Brown University, Kelly, Michael, 2007-2009

MPH Project Committee, Brown University, Piquet, Nicole, 2008-2010

Research Committee Member, Brown University, Samkari, Ayman, 2009-2011

MPH Project Committee, Brown University, Sauer, Nadine, 2011-2013

Supervisory Committees
N/A

Examining Committees
N/A

Direct Supervision

Undergraduate and Allied Health Students

Clinical Research Mentor, Brown University, Salvemini, Philomena, 1998

Clinical Research Mentor, Brown University, Aders, Jeremy, 2010-2012

Medical Students

Clinical Research Mentor, Johns Hopkins University, Harris, Jennifer, MD, 1996-1998

Clinical Research Mentor, Johns Hopkins University, Brian Cornblatt, MD, 1997

Clinical Research Mentor, Johns Hopkins University, Czudowska, Monica, MD, 2001

Clinical Research Mentor, Johns Hopkins University, Stapert, A, MD, 2005

Clinical Research Mentor, Johns Hopkins University, Terlou, A, 2005

Graduate Students

Clinical Research Mentor, Johns Hopkins University, Bydon, Mohamed, 2002-2003

Clinical Research Mentor, Brown University, Wilhelm, Charlotte, 2006-2007

Postdoctoral Research Fellows

N/A

Clinical Residents and Fellows

Clinical Investigation Mentor-Fellow, Johns Hopkins University, Dome, Jeffrey, MD, 1994-1996

Clinical Investigation Mentor-Fellow, Johns Hopkins University, Leung, Wing-Hang, MD, 1994-1996

Clinical Research Mentor-Fellow, Johns Hopkins University, Friedmann, Allison, MD, 1996-1997

Clinical Investigation Mentor-Fellow, Johns Hopkins University, Spunt, Sheri, MD, 1996-1997

Clinical Investigation Mentor-Fellow, Johns Hopkins University, Lowe, Elizabeth, MD, 1997-1999

Clinical Research Mentor-Fellow, Johns Hopkins University, Fitzpatrick, Lorna, 1998-2000

Clinical Research Mentor-Fellow, Brown University, Kelly, Michael, MD, 2007-2009

Clinical Research Mentor-Fellow, Brown University, Piquet, Niccole, MD, 2008-2010

Clinical Investigation Mentor-Residency, Brown University, Rao, Pooja, 2011-2012

Clinical Research Mentor-Fellow, Brown University, Sauer, Nadine, 2011-2013

Other Supervisory Teaching

Lecturer, Clinical research, Brown University, Pediatric Hematology Oncology, 2009-2013

Instructor, Pediatric Hematology/Oncology Outpatient service, Brown University, Medical students, residents, fellows

Instructor, Pediatric Hematology Oncology Inpatient service, Brown University, Residents and Medical Students

CONFERENCES AND SYMPOSIA

Organization of Conferences/Symposia (Include chairing session)

Johns Hopkins University, Progress in Hematologic Malignancy, BMT and Pediatric, Baltimore, MD, Conference Chair, 1995

National Cancer Institute (NCI) Office of Cancer Survivorship, Bethesda, MD, Moderator, 1998

1st Annual Conference, Johns Hopkins Hodgkin's Cohort, Survival After Hodgkin's Disease, Baltimore, MD, Conference Chair, 1999

Clinical Trials in Pediatric Oncology, International Society of Paediatric Oncology (SIOP) and American Society of Pediatric Hematology/Oncology (ASHPO), Montreal, Canada, Co-Chair, 1999

2nd Annual Conference, Johns Hopkins Hodgkin's Cohort, Baltimore, MD, Conference Chair, 2000

American Society of Pediatric Hematology/Oncology (ASHPO), Bone Tumor Symposium, Minneapolis, MN, Chair, 2000

American Society of Pediatric Hematology/Oncology (ASHPO), Lymphoma Symposium, Baltimore, MD, Chair, 2002

International Workshop on Prognostic Factors in Pediatric Hodgkin Lymphoma, Paris, France, Co-Chair, 2004

8th International Symposium on Hodgkin Lymphoma, Cologne, Germany, Chair, Workshop on Pediatric Hodgkin Lymphoma, 2010

American Society of Clinical Oncology (ASCO), Educational Session, Response Based Therapy: A Personalized Approach To Pediatric Hodgkin Lymphoma, Chicago, IL, Chair, 2010

2nd International Symposium on Childhood, Adolescent and Young Adult Hodgkin Lymphoma, Arlington, VA, Co-Chair, Frontline Therapy of Hodgkin Lymphoma, 2011

American Society of Clinical Oncology (ASCO), What is the Way Forward in Osteosarcoma?, Chicago, IL, Discussant, 2013

American Society of Clinical Oncology (ASCO), Late Cardiopulmonary Complications of Cancer Therapy, Chicago, IL, Discussant, 2014

Prognostic Factors in the Hodgkin's Lymphoma – COG studies, Berlin, Germany, Co-Chair, 2014

GAP Conference. Pediatric Co-Chair, Brazil, Sao Paulo, Brazil, 4/27/2016

Presentations at National or International Conferences

Invited

Schwartz CL, Henrickson, KJ, Powell K. Prevention of intraluminal catheter infections by vancomycin, Society for Pediatric Research, Birmingham, AL, 1989

Schwartz CL, Truesdell S, Clark EB. The use of the electrocardiogram in screening patients for anthracycline related cardiomyopathy, 2nd International Conference on Long-Term Complications of Treatment of Children and Adolescents with Cancer, Buffalo, NY, 6/1992

Plenary Session: "Multiple Drug Resistance in Osteosarcoma and Refractory Solid Tumors, Pediatric Oncology Group (POG) Annual Meeting, Chicago, IL, 1993

Schwartz CL, Hicks D, Weiner M, Rosier R. The use of CSAV to overcome drug resistance, Platform Presentation, American Society of Clinical Oncology (ASCO), Chicago, IL, 1993

Survival After Hodgkin's Disease, 1st Annual Survivors' Conference, Johns Hopkins Hodgkin's Cohort, Johns Hopkins University, Baltimore, MD, 1998

The Later Late Effects of Childhood Cancer Therapy, Research Issues in Cancer Survivorship, National Cancer Institute (NCI) Office of Cancer Survivorship, Bethesda, MD, 1998

Ruble, K Schwartz, C. Formation of a Hodgkin's Cohort to Conduct Research and Educate Survivors, Cancer Survivorship: Research Challenges and Opportunities for the new Millennium, Bethesda, MD, 1999

Survival after Hodgkin's Disease, 2nd Annual Survivors' Conference, Johns Hopkins Hodgkin's Cohort, Johns Hopkins University, Baltimore, MD, 1999

Anthracycline Cardiotoxicity, International Conference about Cardiotoxicity, Instituto De Oncologia Pediatrica, Sao Paulo, Brazil, 2000

Issues of Cancer Survivorship for the Adolescent, Society for Adolescent Medicine Annual Meeting, Philadelphia, PA, 2000

Pediatric Hodgkin's Disease, American Society for Therapeutic Radiology and Oncology (ASTRO) 43rd Annual Meeting, San Francisco, CA, 2001

Care Of Survivors of Childhood Cancer: The Role of Survivorship Clinics, Institute of Medicine: National Cancer Policy Board, Bethesda, MD, 2002

Late-effects endpoints of COG clinical trials, Late Effects of Hodgkin's Disease Symposium, Children's Oncology Group Meeting (COG), Jacksonville, FL, 2002

New approaches to Pediatric Hodgkin's Disease, Lymphoma Symposium, American Society of Pediatric Hematology-Oncology (ASPHO), Baltimore, MD, 2002

Schwartz, CL. Pediatric Survivorship, National Cancer Policy Board, Washington, DC, 2002

Treatment of Childhood Hodgkin's Disease, 10th Hematologic Malignancies: 100 years of the Reed-Sternberg Cell, Johns Hopkins University, Baltimore, MD, 2002

Common Terminology Criteria for Adverse Events, Long Term Follow Up Care for Pediatric Cancer Survivors, National Cancer Institute (NCI), Rockville, MD, 2003

Pediatric Lymphocyte Predominant Hodgkin Disease: U. S. approach, 6th International Symposium on Hodgkin's Lymphoma, Cologne, Germany, 2004

Schwartz CL. Osteosarcoma: Overcoming Barriers to Cure, Israel Pediatric Hematology-Oncology Society, Tiberias, Israel, 2005

When More is Less: Dose Dense, Response Based Therapy of Pediatric Hodgkin Disease, Israel Pediatric Hematology-Oncology Society, Tiberias, Israel, 2005

Schwartz CL, Wexler LH, Devidas M, Teot LA, Goorin A, Grier H, Gebhard M, Steinherz L, Sato J, Healey J, Lipshultz S, Miser J, Womer R, P Meyers P,* Bernstein M. Non-Metastatic Osteosarcoma: Response based augmentation of therapy, Connective Tissue Oncology Society Annual Meeting (CTOS), Venice, Italy, 11/2006

Classical Hodgkin Disease: The U.S. Pediatric Approach, 7th international Hodgkin`s Symposium at Cologne, Cologne, Germany, 2007

Early Response-based HL therapies of the COG, 8th International Symposium on Hodgkin Lymphoma, Cologne, Germany, 2010

Optimizing Chemotherapy: Response and Biology, American Society of Children's Oncology (ASCO), Chicago, IL, 2010

Response Based Treatment of Hodgkin's Lymphoma: Improving Efficacy and Reducing Long Term Toxicity, Chemotherapy Foundation Symposium XXVIII, New York, NY, 2010

Current Management of Pediatric Hodgkin Lymphoma in the US, 1st International Symposium on Childhood, Adolescent and Young Adult Hodgkin Lymphoma, Arlington, VA, 2011

Schwartz CL, Friedman DL, McCarten K, Wolden S L, Voss S, Constine LS. Chen L. Predictors of early response and event free survival in Hodgkin lymphoma (HL): PET vs. CT imaging, American Society of Clinical Oncology (ASCO) Platform Presentation, Lymphoma session, Chicago, IL, 2011

New Approach to Stratification in Hodgkin Lymphoma: The Childhood Hodgkin's International Prognostic Score, Children's Oncology Group Annual Meeting (COG), Hodgkin Committee, 3/2012

Hodgkin Lymphoma. New Paradigms improve upon Older Therapy, Pediatrics, The University of Texas MD Anderson Cancer Center, Houston, TX, 1/2013

What's next in Osteosarcoma?, American Society of Clinical Oncology (ASCO), Chicago, IL, 6/23/2013

Pediatric Hodgkin Lymphoma: New Paradigms, XVII Meeting Association Hemato-Oncologica Pediatric Centro American (AHOPCA), Santo Domingo, Dominican Republic, 2/2014

The Childhood Hodgkin International Prognostic Score (CHIPS) Predicting EFS in Pediatric and Adolescent Hodgkin Lymphoma, 2nd International Symposium on Childhood, Adolescent and Young Adult Hodgkin Lymphoma, Berlin, Germany, 5/2014

Schwartz, C. Life after Childhood Cancer: When the Eradication of Disease is not Enough, 9th International Cancer Conference in Trinity College Dublin, Dublin, Ireland, 9/18/2014

Schwartz, C. Childhood Cancer Survivorship: Is Cure Enough?, 4th annual conference Medical Oncology and Hematology 2014: Multidisciplinary Approaches that Improve Coordination of Care., 4th annual conference Medical Oncology and Hematology 2014: The University of Texas MD Anderson cancer Center, Houston, TX, 10/16/2014

Anthracycline Cardiotoxicity in Children, Cardio-Oncology Grand Rounds, MDACC, Houston, TX, 12/1/2015

Schwartz CL. Hodgkin Lymphoma: US approach. The past decade and the future., GAP Brazil, Sao Paulo, Brazil, 4/27/2016

Schwartz CL. II SYMPOSIUM OF PHARMACY IN PEDIATRIC ONCOLOGY: Cardiotoxicity, XV Congresso Brasileiro de Oncologia Pediatrica- SOBOPE, Rio de Janeiro, Brazil, 11/17/2016

Schwartz CL. Satellite Symposium: Strategies of Cardioprotection during intensified chemotherapy, Zodiac, XV Congresso Brasileiro de Oncologia Pediatrica- SOBOPE, Rio de Janeiro, Brazil, 11/18/2016

Seminar Invitations from Other Institutions

Secondary Malignancy, Pediatric Trends, Johns Hopkins University, Baltimore, MD, 1983

Osteosarcoma, Pediatric Trends, Johns Hopkins University, Baltimore, MD, 1984

Cancer Chemotherapy , Medical-Dental Inter-relationship Series, University of Rochester School of Medicine and Dentistry, Rochester, NY, 1986

Leukemia: New Approaches to New and Old Problems, Grand Rounds, University of Rochester School of Medicine, Rochester, NY, 1986

Osteosarcoma, Oncology Center Grand Rounds, University of Rochester, Rochester, NY, 1986

Anemia In Neonates, Regional Postgraduate CME Program, University of Rochester, Rochester, NY, 1987

Down Syndrome and Megakaryoblastic Leukemia, Hematology Research Conference, University of Rochester, Rochester, NY, 1987

Long Term Survivors of Childhood Cancer, Pediatric Grand Rounds, University of Rochester School of Medicine, Rochester, NY, 1990

Long Term Survivors of Childhood Cancer, Radiation Oncology Grand Rounds, University of Rochester, Rochester, NY, 1990

Multiple Drug Resistance in Pediatric Malignancy, Johns Hopkins Oncology Center, Baltimore, MD, 1993

Obstacles to the Cure of Osteosarcoma, Brown University School of Medicine, Providence, RI, 1993

Long Term Survivors of Childhood Cancer, Pediatric Grand Rounds, Rochester General Hospital, Rochester, NY, 1994

Long Term Survivors of Childhood Cancer, Fairfax Hospital, Fairfax, VA, 1997

Long Term Survivors of Childhood Cancer, Pediatric Oncology Branch, National Cancer Institute (NCI), Bethesda, MD, 1997

Long Term Survivors of Childhood Cancer, East Baltimore Medical Center, Baltimore, MD, 1997

Meet the Professor Session: Late Effects of Therapy of Childhood Cancer,, American Society of Clinical Oncology (ASCO), Denver, CO, 1997

Surgery Education Session: Late Effects of Chemotherapy, Pediatric Oncology Group (POG) Spring Meeting, Chicago, IL, 1997

Overcoming Obstacles to Cure in Osteosarcoma, Duke University School of Medicine, Pediatrics, Durham, NC, 1998

Long Term Survivors of Childhood Cancer, Paul C. Gaffney Visiting Professorship,, Children's Hospital of Pittsburgh, Pittsburgh, PA, 1999

Long-Term Effects of Hodgkin's Disease, Children's Hospital of Philadelphia, Philadelphia, PA, 1999

Overcoming Obstacles to Cure in Osteosarcoma, University of California at San Francisco, Pediatrics, San Francisco, CA, 1999

Late Effects of Childhood Cancer, Georgetown University Medical Center, Washington, DC, 2000

Upfront Treatment of Hodgkin's Disease, University of Virginia, Pediatrics, Charlottesville, VA, 2002

Clinical Trial Progress and Challenges: Hodgkin Disease, Clinical Oncology Group (COG) Nursing Challenges, Dallas, TX, 2003

New Approaches to Pediatric Hodgkin Disease: Reducing the Long Term Consequences of Therapy, Leukemia Lymphoma Society, New Orleans, LA, 2003

Novel Approaches to Pediatric Hodgkin Disease, Pediatric Grand Rounds, Brown University School of Medicine, Rhode Island Hospital, Providence, RI, 2003

Childhood Cancer Survivors, Program in Pediatrics, Colby College, Colby College, ME, 2007

Hoofbeats of Zebras – Presentations of Childhood Cancer Program in Pediatrics, Colby College, Colby College, ME, 2007

Late Effects of Treatment, Welcome Back conference, Leukemia and Lymphoma Society, Providence, RI, 2007

Pediatric Hodgkin Disease Meet-the-Expert., American Society of Hematology (ASH), Atlanta, GA, 2007

Pediatric Hodgkin Lymphoma, University of New Mexico Cancer Center, Phoenix, NM, 2008

Barriers to Survivorship Care, New England Childhood Cancer Survivors in Transition, Children's Oncology Group (COG), Bedford, NH, 2009

Hodgkin Lymphoma Committee: Advances of the Decade, Children's Oncology Group (COG) Annual Meeting, Hodgkin Committee, Los Angeles, CA, 2011

Optimizing Therapy for Hodgkin Lymphoma: Early Response and Biology, The Research Institute, Nationwide Children's Hospital, Columbus, OH, 2011

Predictors of Response in Hodgkin Lymphoma, Harvard School of Public Health, Boston, MA, 2011

Response based therapy of Hodgkin Lymphoma, Oncology Grand Rounds, Children's Hospital of Los Angeles, Los Angeles, CA, 2011

Update: Barriers to Care in ALL, Consortium of New England Childhood Cancer Survivors, Dartmouth Hitchcock Medical Center, Manchester, NH, 10/2012

New Paradigms in the Treatment of Pediatric Hodgkin Lymphoma, Pediatric HSCT, Dana Farber Cancer Institute, Boston, MA, 3/2013

Pediatric Hodgkin Lymphoma: Evolving Paradigms of Care, Pediatric Grand Rounds/ Pediatric Seminars, Johns Hopkins Children's Center Grand Rounds, Pediatrics, Baltimore, MD, 5/2013

Hodgkin Lymphoma: Changing Paradigms for Cure, University of Illinois, Pediatrics, Peoria, IL, 12/10/2015

Hodgkin Lymphoma: US approach The past decade and the future, Hospital de Cancer Infantojuvenil de Barretos, Pediatrics, Barretos, Brazil, 4/29/2016

Osteosarcoma: What is the way forward?, Hospital de Cancer Infantojuvenil de Barretos, Barretos, Brazil, 4/29/2016

Lectureships and Visiting Professorships

Childhood Cancer Survivorship, Paul C. Gaffney Lectureship, Children's Hospital of Pittsburgh, 1999

Other Presentations at State and Local Conferences

White Cell Disorders in Children, Lab Technologist Curricula, University of Rochester, Rochester, NY, 1987

Children with Cancer, Cancer Action, Inc., Rochester, NY, 1988, 1989, 1991.

Childhood Cancer, Corning Hospital, Corning, NY, 1994

Life on the Plateau - Long Term Survivors of Childhood Cancer, Grand Rounds, St. Agnes Hospital, Baltimore, MD, 1998

Looking Forward: Survivors of Childhood Cancer, Childhood Cancer Survivorship: Creating Partners for Living. A conference for parents, adult survivors of childhood cancer, education and health professionals, Warwick, RI, 2006

Schwartz, CL. Late effects of pediatric transplantation, Celebrating a Second Chance at Life Survivorship Symposium, InfoNet Survivorship Symposium, Burlington, MA, 3/21/2010

Schwartz CL. Epigenetics of Childhood Leukemia, RI Hospital, Pediatrics, Providence, RI, 1/2013

Childhood Cancer Survivorship: Life after Cure, Symposium for Cancer Survivorship, Rhode Island Hospital, Providence, RI, 3/2013

Schwartz CL. Pediatric Hodgkin Lymphoma, Pediatric Nursing, Nursing, Houston, TX, 2014

PROFESSIONAL MEMBERSHIPS/ACTIVITIES

Professional Society Activities, with Offices Held

National and International

Children's Oncology Group, Arcadia, CA
- Member, 2000-present
- Steering Committee Member, Voting Body, 2001-2003
- PI, Johns Hopkins University, 2001-2005
- Developmental Therapeutic Committee, Member, 2001-2006
- Osteosarcoma Biology Committee, 2001-2006
- Chair, Hodgkin Disease Committee, 2001-2011
- Member, Scientific Chairs Committee, 2001-2011

Member, Voting Body, 2001-2013
Steering Committee, Bone Tumor Committee, 2001-present
Steering Committee, Hodgkin Committee, 2001-present
Adolescent/Young Adult Steering Committee, 2002-2010
Survivorship Transition Sub-Committee, 2003-2005
PI, RIH/Brown University, 2005-2013
Member, Executive Committee, 2010-2013
Steering Committee, Hematology Oncology Discipline, 2013-present
Member, Institutional Performance Monitoring Committee, 2014-present

American Society of Clinical Oncology
Member, 1989-present
Publications Committee, 1999-2001
Patients Living with Cancer Advisory Board (Hodgkin's disease), 2003-2011
Expert Panel on Long-Term Medical Care for Adult Cancer Survivors, 2005-2007
CME Committee, 2005-2009

American Society of Pediatric Hematology/Oncology
Member, 1988-present
Program Committee, 1999-2002
Nominating Committee, 2009-2012
Chair, Nominating Committee, 2011-2012

Dana Farber ALL Consortium
Member, 2008-2013

American Pediatric Society
Member, 2007-present

National Cancer Center Network (NCCN)
Pediatric Committee, 1995-2001
Osteosarcoma Subcommittee, 1999-2001
Adolescent Young Adult Committee, 2015- present

Pediatric Oncology Group, Chicago, IL
Committee Member, Hodgkin's Disease, 1989-2000
Principal Investigator: University of Rochester, 1992-1994
Committee Member, Sarcoma, 1994-2000
Principal Investigator: Johns Hopkins, 1994-2000
Committee Member, Sarcoma Intergroup/Transition Committee, 1997-2000
Chair, POG Hodgkin's Committee, 1999-2000
Co-Chair, Hodgkin's Transition Committee, 1999-2000

Children’s Study Cancer Group
Children’s Study Cancer Group, 1986-1988

American Cancer Society, New York State
Upstate New York Pediatric Oncology Group, 1990-1994

Women in Cancer Research
Member, 1992-1999

Children's Cancer Foundation, Baltimore, MD
Medical Advisory Committee, 1998-2003

Israel Children's Cancer Foundation, Lawrence, NY
Member, Medical Advisory Board, 1998-present

American Society of Hematology

Member, 2003

Tomorrow Fund, Providence, RI
Executive Board Member and Program Education Committee, 2006-2013

Local/State
N/A

UNIQUE ACTIVITIES
N/A

DATE OF LAST CV UPDATE
10/7/2016

CURRICULUM VITAE

I. GENERAL BIOGRAPHICAL INFORMATION

A. Personal

Name: Sheila Thampi, MD
Citizenship: USA

Education

2001-2005	University of California, Riverside	BS	Biomedical Sciences
2004-2008	University of California, Los Angeles	MD	
2008-2011	University of California, Davis	Resident	Pediatrics
2011-2014	University of California, San Francisco	Fellow	Hematology/Oncology
2012-2013	University of California, San Francisco	Certificate	Clinical Research

B. Academic Appointment

8/2014-present Assistant Professor Pediatrics. Division of Pediatric Hematology and Oncology. Baylor College of Medicine, Children's Hospital San Antonio, San Antonio, Texas.

C. Other information

Certification

2010	Neonatal Resuscitation Program
2010	Medical Licensure, State Board of California
2011	Pediatric Advanced Life Support
2011	Board Certification Pediatrics, American Board of Pediatrics
2014	Basic Life Support
2014	Medical Licensure, State Board of Texas
2015	Board Certification Hematology/Oncology, American Board of Pediatrics

II. RESEARCH INFORMATION

A. Research Support

PAST

2011 Children's Miracle Network Resident Mentor Award. Validation of a Human Leukemia Mouse Model Using Next Generation RNA Sequencing
Role: Perform RNA extraction

2012-2014

T32 CA128583
NIH/NCI
Research Training in Childhood Cancer
Objective: This program supports 4 physician/scientist trainees per year who are conducting research relevant to pediatric oncology.
Role: Trainee

B. Fellowship Research Projects

1. UCSF outcomes with Intra-arterial Melphalan Therapy, retrospective chart review.
2. Osteosarcoma and Regional lymph node involvement, SEER database review.
3. Pilot study for children with advanced intraocular retinoblastoma, Early phase clinical research grant from the UCSF cancer center.
4. Extraskeletal Osteosarcoma, SEER database review.

C. National Scientific Participation

Membership of Professional Organizations:

2007-present	American Academy of Pediatrics
2011-present	Children's Oncology Group
2013-present	American Society of Pediatric Hematology/Oncology
2013-present	American Society of Clinical Oncology
2014-2016	San Antonio Pediatric Society

D. Publications

PEER REVIEWED PUBLICATIONS

1. **Thampi S**, Salmi D, Imashuku S, Ducore J, Satake N. Thrombotic Thrombocytopenic Purpura in a Child with Systemic Lupus Erythematosus. *J Pediatr Hematol Oncol*. 2011; 33: 221-223.

2. **Thampi S**, Hetts SW, Cooke D, Stewart P, Robbins E, Banerjee A, DuBois SG, Char D, Halbach V, Matthay K. Superselective intra-arterial melphalan therapy for newly diagnosed and refractory retinoblastoma: results from a single institution. *Clinical Ophthalmology*. 2013; 7: 981-989.

3. **Thampi S**, Matthay KK, Goldsby R, DuBois SG. Adverse Impact of Regional Lymph Node Involvement in Osteosarcoma. *Eur J Cancer*. 2013;49(16):3471-3476. [Epub 2013 Jul 15]

4. **Thampi S**, Matthay KK, Boscardin WJ, Goldsby R, DuBois SG. Clinical Features and Outcomes Differ between Skeletal and Extraskeletal Osteosarcoma. *Sarcoma. 2014:* 902620. doi: 10.1155/2014/902620. Epub 2014 Sep 9.

5. McCallin T, **Thampi S**, Eighmy S, Griffin T, Sorrel A. Acute Upper Extremity Edema in a 10-Year-Old Girl as Presenting Symptom of a Rare Cancer. *Pediatric Emergency Care*. Submitted for review.

ABSTRACTS/POSTER PRESENTATIONS

1. Sataki N, **Thampi S**, Tepper C, Chang A, Zhou P, McLaughlin B, McGee J, and Nolta JA. Validation of a Human Acute Lymphoblastic Leukemia Mouse Model Using Next Generation RNA Sequencing. Presented at the 53rd American Society of Hematology Annual Meeting; Dec 2011.

2. **Thampi S**, Matthay K, Goldsby R, DuBois S. Adverse Impact of Regional Lymph Node Involvement in Osteosarcoma. Presented at the American Society of Pediatric Hematology and Oncology annual meeting; Miami FL, April 2013.

3. **Thampi S**, Facchino J, Matthay K, Horn B, Dvorak C. Eculizumab for the Treatment of Hematopoietic Stem Cell Transplant (HSCT) Associated Thrombotic Microangiopathy (TA-TMA). Presented at the Pediatric Blood and Marrow Transplant Consortium/American Society of Pediatric Hematology and Oncology combined annual meeting; Miami FL, April 2013.

4. **Thampi S**, Matthay K, Boscardin JW, Goldsby R, DuBois S. Clinical Features, Outcomes and Prognostic Factors in patients with Extraskeletal Osteosarcoma. Presented at the American Society of Clinical Oncology annual meeting; Chicago, IL, June 2014.

5. Basaldua K, Sedillo D, **Thampi S**. What are the odds: Viral suppression vs. Leukemia. Presented at the Pediatric Hospital Medicine Conference; San Antonio, TX, July 2015.

6. McCallin T, **Thampi S**, Eighmy S, Griffin T, Sorrel A. Acute Upper Extremity Edema as a Presenting Symptom of a Rare Cancer. Presented at the Children's Hospital of San Antonio Research Symposium; San Antonio, TX, Sept 2016

BOOKS AND CHAPTERS

1. **Thampi S**, DuBois SG. Osteosarcoma. In: Cabana MD Editor (Ed). The 5-Minute Pediatric Consult Standard. 7th ed. Philadelphia, PA: Lippincott Williams & Wilkins; 2015.

2. **Thampi S**, Stewart P. Retinoblastoma. In: Cabana MD Editor (Ed). The 5-Minute Pediatric Consult Standard. 7th ed. Philadelphia, PA: Lippincott Williams & Wilkins; 2015.

3. **Thampi S**, DuBois SG. Osteosarcoma. In: Cabana MD Editor (Ed). The 5- Minute Pediatric Consult Standard. 8th ed. Philadelphia, PA: Lippincott Williams & Wilkins; Submitted for review.

4. **Thampi S**, Damato, B. Retinoblastoma. In: Cabana MD Editor (Ed). The 5-Minute Pediatric Consult Standard. 8th ed. Philadelphia, PA: Lippincott Williams & Wilkins; Submitted for review.

III. TEACHING

A. Lectures

Informal Teaching

2014- present	Bedside teaching and informal teaching sessions with medical students and residents.

Mentoring Activities

2011-present	Supervision and mentoring of residents and medical students when on service
2015-present	Resident mentor for Crystal Cuellar

Formal Teaching/Presentations at UCSF

2011-2014	Pediatric Housestaff Lecture Series
7/2011	"Myelodysplastic Syndrome" Pediatric Bone Marrow Transplant Conference
9/21/11	"Coagulopathy" UCSF Coagulation Conference
12/2011	"Recurrent Venous Thromboembolism" Pediatric Hematology and Oncology Mortality and Morbidity Conference
1/12/12	"Coagulopathy in an infant" Non-Malignant Hematology Conference
1/25/12	"A bleeding Diathesis Unexplained?" UCSF Coagulation Conference
3/5/12	"Perioperative Management when on Anticoagulation" Pediatric Hematology and Oncology Division Conference
3/20/12	"Hurler's and Hunters: Outcomes with Transplant" Pediatric Bone Marrow Transplant Conference
4/16/12	"Pediatric CML" Multi-specialty Case Conference
6/12/12	"Pediatric CML" Pediatric Bone Marrow Transplant Conference
7/2012	"Retinoblastoma" Pediatric Hematology and Oncology Division Journal Club
1/29/13	"Clinical Investigation of Pediatric Solid Tumors" Pediatric Hematology and Oncology Division Conference
3/25/13	"Morbidity from Chemotherapy" Pediatric Hematology and Oncology Mortality and Morbidity Conference
4/8/13	"Cixutumumab and temsirolimus for patients with bone and soft-tissue sarcoma: a multicenter, open-label, phase 2 trial" Phase 1 Journal Club
8/26/13	"Hepatoblastoma" Pediatric Hematology and Oncology Division Journal Club
10/7/13	"Clinical investigations in retinoblastoma and osteosarcoma" Pediatric Hematology and Oncology Division Conference
10/14/13	"Phase 1 Pharmacokinetic and Pharmacodynamic Study of Pazopanib in Children with Soft Tissue Sarcoma and Other Refractory Solid Tumors: A Children's Oncology Group Phase 1 Consortium Report" Phase 1 Journal Club

Formal Teaching/Presentations at Children's Hospital of San Antonio

10/15/14	"Abdominal Mass" Multidisciplinary Team meeting
4/1/15	"Solid tumors" Multidisciplinary Team meeting
11/11/15	"Metastatic Neuroblastoma" Multidisciplinary Team meeting
12/16/15	"More Solid Tumors" Multidisciplinary Team meeting
2/4/16	"2 neoplasms, 1 patient" PICU team meeting
2/24/16	"Chemo allergy" Multidisciplinary Team meeting
3/16/16	"MPNST" Multidisciplinary Team meeting
8/26/16	"Platelets, Pica, and PTT: A review of the evidence behind common practices in pediatric Hematology" Children's Hospital of San Antonio Grand Rounds

9/14/16	"Langerhans's Cell Histiocytosis" Multidisciplinary Team meeting
11/11/16	Janus Rounds speaker
1/18/17	"Desmoid Tumors" Multidisciplinary Team meeting

INVITED PRESENTATIONS

NATIONAL

2011 American Society of Hematology Meeting, poster presentation
2013 American Society of Pediatric Hematology/Oncology Meeting, poster presentation
2013 Pediatric Blood and Marrow Transplant Consortium Meeting, poster presentation
2014 American Society of Clinical Oncology, poster presentation
2015 Pediatric Hospital Medicine Meeting, poster presentation

REGIONAL AND OTHER INVITED PRESENTATIONS

2009 Tumor Board, Stanford University, Palo Alto, CA, oral slide presentation
2011 UCD Pediatric Grand Rounds, Sacramento, CA, oral slide presentation
2013 Pediatric Fellows Research Day (UCSF), poster presentation
2014 Pediatric Fellows Research Day (UCSF), oral slide presentation
2015 Pediatric Board Review course speaker (San Antonio, TX), oral slide presentation
2016 Children's Hospital of San Antonio Research Symposium, poster presentation
2016 Pediatric Grand Rounds Children's Hospital of San Antonio, oral slide presentation
2016 Pediatric Board Review course speaker (San Antonio, TX), oral slide presentation

IV. MEDICAL AND SERVICE INFORMATION

PROFESSIONAL ACTIVITIES

Summary of Clinical Activities:

As an attending in the Children's Hospital of San Antonio I have accumulated a variety of patients in both hematology and oncology. Every 5 weeks I care for the inpatient service in addition to managing both inpatient and outpatient consults and cross cover for the bone marrow transplant unit. During outpatient clinic I see a variety of follow up and new patients. During inpatient service and for my primary patients I have performed numerous lumbar punctures and bone marrow evaluations.

UNIVERSITY AND PUBLIC SERVICE

Public Service

2004	Camp Okizu (cancer camp), camp counselor
2005	Sandals Church, Sunday school teacher
2005-2006	Student Run Homeless Clinic, medical student
2004-2006	Tar Wars, teacher for elementary school kids about negative effects of smoking
2014-2015	Community Bible Church, children's ministry volunteer

University Service

2007-2008	UCLA Doctoring 4: Tutor
2008-2011	UCD Community & Physicians Together: Member
2009-2011	UCD Pediatric Palliative Care Committee: Member
2010-2011	UCD Curriculum and Evaluation Committee: Member
2011-2014	UCSF Pediatric Fellows' Leadership and Advocacy Group (FLAG): Member
2011-2012	Revision of orientation and "survival guide" for incoming Pediatric Hematology and Oncology fellows
2012- 2014	UCSF Phase 1 group: Member
2012	Coordinated and facilitated a 1 week Pediatric Hematology and Oncology orientation
2013	Arranged the fellow procedure schedule
2013-2014	UCSF Retinoblastoma Multidisciplinary Group: coordinator and member
8/2014-2015	Children's Hospital Association Collaboration: member
8/2014-2016	Ethics committee: member
2/2015-2016	FMEA Chemotherapy Administration Committee: member
2/2015-present	Chemotherapy Task Force: member
3/2015-present	Children's Hospital Association Texas Sepsis initiative: member
7/2015-present	Children's Oncology Group Cancer Control Responsible Individual
9/2016	Intern Selection Committee
10/26/16	CPRIT Advisory Committee on Childhood Cancers

February 2017 Oversight Committee
Internal Audit Status Report
As of January 31, 2017

Weaver and Tidwell, LLP (Weaver) is the outsourced internal auditor of the Cancer Prevention Research Institute of Texas (CPRIT). The Weaver engagement team is led by Alyssa Martin, Partner; Daniel Graves, Sr. Manager; and Adam Wright, Manager.

2017 Internal Audit Plan and Schedule

Weaver plans to perform the following internal audits in FY 2017.

NEW INTERNAL AUDITS		
Internal Audit	**Description**	**Timing**
Training	Internal Audit will include an evaluation of risks and internal controls in place related to CPRIT's Training practices. Employee Technical Training, Oversight Committee Training, Employee Compliance and Ethics Training, and Grantee Training and Onboarding. Fieldwork has been completed. An exit meeting with Management is scheduled for February 6, 2017.	January 2017
Internal Agency Compliance	Internal Audit will include an evaluation of risks and internal controls in place related to CPRIT's Internal Agency Compliance practices. Activities to be evaluated will include Disclosures, Ethics Policy and Compliance and Code of Conduct. A planning meeting with Management was held on January 31, 2017. Fieldwork is scheduled to begin on February 13, 2017.	February 2017
Pre-Award Grant Management	Internal Audit will include an evaluation of risks and internal controls in place related to CPRIT's Pre-Award Grant Management process. Activities to be evaluated will include the RFA Review Process, Conflicts of Interest, Peer Review (including travel coordination), and Grant Application Approval.	March 2017
Procurement and P-Cards	Internal Audit will include an evaluation of risks and internal controls in place related to the CPRIT's Procurement practices. Activities to be evaluated will include Purchase Orders, Bidding and Awards, Contract Negotiation and Approval, Vendor Management and Selection, Vendor Acceptance, Vendor Set-up, P-card Program, P-card Purchases, Central Travel Card, and Employee Travel Cards.	May and June 2017

Weaver will perform follow-up procedures for the following audits in 2017.

FOLLOW-UP PROCEDURES		
Follow-Up	**Description**	**Timing**
IT Security	Internal Audit will perform follow-up procedures on the 11 findings from the 2016 Internal Audit to ensure corrective action has been taken. A planning meeting with Management was held on January 26, 2017. Fieldwork is scheduled to begin on February 6, 2017.	February 2017
Revenue	Internal Audit will perform follow-up procedures on the two findings from the 2016 Internal Audit to ensure corrective action has been taken.	May 2017
Commodity and Service Contracts	Internal Audit will perform follow-up procedures on the five findings from the 2016 Internal Audit to ensure corrective action has been taken.	May 2017
Cash Management	Internal Audit will perform follow-up procedures on the one finding from the 2016 Internal Audit to ensure corrective action has been taken.	May 2017

Additional engagements will be performed at the request of management or the Oversight Committee.

Alyssa G. Martin, CPA, MBA, Internal Auditor
Executive Partner
Weaver and Tidwell L.L.P

Cancer Prevention and Research Institute of Texas
Internal Audit of Training Programs
Internal Audit Risk Coverage
January 2017

Scope: The audit focused on the Training Programs as they are deployed to employees, the Oversight Committee and grant recipients. Key functions and sub-processes within the Training Programs process that were reviewed include:

- Employee Technical Training
- Oversight Committee Training
- Employee Compliance and Ethics Training
- Grantee Training and Onboarding

The audit did not include grantee compliance monitoring, internal compliance programs, human resource requirements, or information technology security related to training records and programs.

Monitored Risks

Training Programs		
Process Area	**Risks Monitored**	
Employee Technical Training	**1**	Employee training needs are not identified and tracked
	2	Employee certification and continuing education requirements are not met
	3	Training plans do not address all necessary courses
	4	Attendance is not recorded and monitored
	5	Training content does not address technical needs
	6	Training budgets are not appropriate
Oversight Committee Training	**7**	New Oversight Committee members do not receive onboarding training
	8	Oversight Committee members are not aware of governance responsibilities
	9	Oversight Committee members are not notified of legislative and compliance updates
	10	Oversight Committee members do not receive contract oversight training
Employee Compliance and Ethics Training	**11**	Employees do not receive onboarding training
	12	Employees do not receive training related to the Code of Conduct and Ethics
	13	New compliance requirements are not identified and communicated to employees
	14	Employees do not receive updated ethics training
	15	Employees do not receive policy compliance and civil rights training
Grantee Training and Onboarding	**16**	Grantees do not receive onboarding training
	17	Grantees do not receive required compliane training
	18	Grantee attendance is not tracked and monitored
	19	Grantee training content does not address all necessary compliance topics
	20	Appropriate representatives from the grantee organiation do not attend required training

Cancer Prevention and Research Institute of Texas
Schedule of Audits, Status, and Findings Summary
As of January 31, 2017

					Open Findings				Closed Findings				Total Findings				
Audit	**Fiscal Year**	**Status/Timing**	**Report Date**	**Report Rating**	**High**	**Mod**	**Low**	**Total**	**High**	**Mod**	**Low**	**Total**	**High**	**Mod**	**Low**	**Total**	
Fiscal Year 2015																	
Grant Management	2015	Complete	July 27, 2015	Satisfactory	-	8	1	9					-	8	1	9	Note A
Expenditures Internal Audit	2015	Complete	August 24, 2015	Strong	-	-	2	2					-	-	2	2	Note B
2014 Governance and IT Follow-Up	2015	Complete	August 14, 2015	Satisfactory				9				7	-	1	1	2	Note C, Note E
2014 Grantee Monitoring Follow-Up	2015	Complete	July 31, 2015	Satisfactory				14				11	1	-	2	3	Notes C, D
Fiscal Year 2015 Subtotal					**-**	**8**	**3**	**34**	**-**	**-**	**-**	**18**	**1**	**9**	**6**	**16**	
Fiscal Year 2016																	
Commodity and Service Contracts Internal Audit	2016	Complete	May 13, 2016	Satisfactory	-	3	2	5	-	-	-	-	-	3	2	5	
Revenue Internal Audit	2016	Complete	July 8, 2016	Strong	-	-	2	2	-	-	-	-	-	-	2	2	
Information Security Internal Audit	2016	Complete	August 3, 2016														
Cash Management Internal Audit	2016	Complete	August 12, 2016	Strong		1		1					-	1	-	1	
2015 Grant Management Follow-Up	2016	Complete	June 9, 2016	Strong	-	8	1	9	-	8	1	9	-	-	-	-	Note A
2015 Information Technology Follow-Up	2016	Complete	N/A	N/A	-	1	1	2	-	1	1	2	-	-	-	-	Note E
Fiscal Year 2016 Subtotal					**-**	**13**	**6**	**19**	**-**	**9**	**2**	**11**	**-**	**4**	**4**	**8**	
Fiscal Year 2017																	
Training Internal Audit	2017	January 2017			Fieldwork is complete. Exit meeting is scheduled for February 6, 2017.												
Internal Agency Compliance	2017	February 2017			Fieldwork is scheduled to begin February 13, 2017.												
Pre-award Grant Management	2017	March 2017															
Procurement and P-Card Internal Audit	2017	May 2017															
2016 Information Security Follow-Up	2017	February 2017			Fieldwork is scheduled to begin February 6, 2017.												
2016 Commodity and Service Contracts Follow-Up	2017	May 2017															
2016 Revenue Follow-Up	2017	May 2017															
2016 Cash Management Follow-Up	2017	May 2017															
Fiscal Year 2017 Subtotal																	
Fiscal Year 2018																	
Post Award Grant Monitoring Internal Audit	2018																
Grant Contracting Internal Audit																	
Information Technology Services Internal Audit	2018																
Communication	2018																
State Reporting	2018																
2017 Procurement Follow-Up	2018																
2017 Non-Grant Expenditures Follow-Up	2018																
2017 Training Follow-Up	2018																
2017 External Affairs Follow-Up	2018																
Fiscal Year 2018 Subtotal																	

FISCAL YEAR 2017 SUMMARY

Audit	**Fiscal Year**	**Status/Timing**	**Report Date**	**Report Rating**	**Findings**				**Closed Findings**				**Total Open Findings**				**Timing of Follow-Up Procedures by IA**
					High	**Mod**	**Low**	**Total**	**High**	**Mod**	**Low**	**Total**	**High**	**Mod**	**Low**	**Total**	
Commodity and Service Contracts Internal Audit	2016	Complete	May 13, 2016	Satisfactory	-	3	2	5	-	-	-	-	-	3	2	5	**March 2017**
Revenue Internal Audit	2016	Complete	July 8, 2016	Strong	-	-	2	2	-	-	-	-	-	-	2	2	**May 2017**
Information Security Internal Audit	2016	Complete	August 3, 2016														**June 2017**
Cash Management Internal Audit	2016	Complete	August 12, 2016	Strong	-	1	-	1	-	-	-	-	-	1	-	1	**August 2017**
Total Findings For Internal Audit Follow-Up					**-**	**4**	**4**	**8**	**-**	**-**	**-**	**-**	**-**	**4**	**4**	**8**	

Cancer Prevention and Research Institute of Texas
Schedule of Audits, Status, and Findings Summary
As of January 31, 2017

NOTES	
A	The nine findings from the 2015 Grant Management internal audit were closed as part of the 2016 Internal Audit Follow-up procedures.
B	At the conclusion of the audit, no follow-up procedures were recommended to be performed by Internal Audit based on the nature and risk rating of the findings in the report. Internal Audit has recommended that Management perform their own follow-up procedures to validate remediation has occurred. Management has agreed to report the confirmation of the remediation to the Audit Subcommittee separately.
C	The prior internal auditor did not provide risk ratings for the individual findings in the final report. Therefore the number of findings and the findings remediated are shown in total.
D	At the conclusion of the audit, follow-up procedures were recommended to be performed by CPRIT's Compliance group, which is occurring. Internal Audit does not plan to perform follow-up procedures on these open findings. Management has agreed to report the confirmation of the remediation to the Audit Subcommittee separately.
E	The 2015 Governance and IT Follow-up procedures closed all the outstanding Governance findings. We incorporated the remaining open Information Technology Services follow-up procedures into the Information Security Internal Audit. The two open findings are information security related, and the audit procedures included in the audit included the evaluation of the conditions related to the open findings. The two prior open findings have been consolidated into the 2016 IT Security Internal Audit.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

NOTE

Item 14 – Tab 11

Advisory Committee on Childhood Cancers (ACCC) Annual Report will be added to the agenda packet when it's received.

PO Box 12097 | Austin, Texas 78711 | PH: 512-463-3190 | F: 512-475-2563 | cprit.texas.gov

2016 CPRIT University Advisory Committee Annual Report
Submitted to the CPRIT Oversight Committee
January 31, 2017

CPRIT 2016 University Advisory Committee Member Roster

Mary Ann Ottinger, Ph.D.
Chair, 2016-2018
Associate Vice Chancellor
University of Houston System
Associate Vice President for Research
University of Houston

Michelle C. Barton, Ph.D.
Vice Chair, 2016-2018
Dean, Grad School Biomed Sciences
Professor, Epigenetics & Mol. Carcin.
The University of Texas MD Anderson Cancer Center

Mike Blanda, Ph.D.
Assistant Vice President for
Research and Federal Relations
Professor of Chemistry
Texas State University

Carrie L. Byington, M.D.
The Jean and Thomas McMullin Professor and Dean of Medicine
Senior Vice President Health Science Center
Vice Chancellor for Health Services
Texas A&M University

C. Kent Osborne, M.D.
Director
Duncan Cancer Center
Baylor College of Medicine

Yousif Shamoo, Ph.D.
Vice Provost for Research
Rice University

Ian Thompson, M.D.
Professor
University of Texas Health Science Center
San Antonio

New UAC Members for 2017 will include:

- Representative, Texas A&M University
- Representative, Texas Tech University
- Representative, University of North Texas Health Science Center at Fort Worth

2016 UAC Meetings, Reports and Teleconferences

- January, 2016—preparation and submission of the 2015 Annual Report by UAC
- April 25, 2016
- June 17, 2016—UAC meeting at UH
- August 5, 2016—Chair call
- October 4, 2016—UAC meeting at CPRIT Office

The CPRIT University Advisory Committee was restarted in 2014 under new leadership, with representation from Texas Institutions. Dr. Margaret Kripke, assisted by Mr. Michael Brown, re-established a high-quality grants program, fostering research excellence focused on the prevention and eradication of cancers, particularly those found in high frequency in Texas. The year of 2016 was one of transition for both the CPRIT leadership and the membership of the CPRIT University Advisory Committee (UAC). Dr. James Willson, as successor to Dr. Kripke, enabled a smooth transition with active engagement of the UAC during this process.

During 2016, meetings were held approximately quarterly with preparation of the annual report in January and full UAC meetings in April, June, and October. Our first UAC meeting with Dr. Willson focused on the overall research program; including current CPRIT Program Priorities (Oversight Committee Report), reviewing each of the specific grant mechanisms and discussion of meaningful metrics that capture how CPRIT impacts the citizens of Texas. Further, the UAC considered current grant mechanisms in the context of selected metrics and if areas of unmet needs are being addressed. A summary of UAC discussions and recommendations is provided below:

1. UAC Perspectives on 2016 CPRIT Initiatives

- CPRIT is a critical catalyst, providing a laser focus on i) understanding the underlying mechanisms, genomic and environmental triggers that stimulate the onset and progression of cancer and cancer-associated disease, ii) the efficacy of interventions, and iii) the testing of highly promising technologies and products.
- Recruitment grants continue to be a highly effective mechanism for recruiting talented cancer researchers to Texas Institutions. As in previous years, the UAC highly endorses funding this mechanism and strongly supports continued funding for recruitment grants. These faculty hires offer highly innovative and cutting edge contributions that aggressively drive progress towards combating cancer and inspire teams of researchers and trainees in Texas Institutions, as a legacy of CPRIT support in the State of Texas for years to come.
- The UAC appreciates the interactive approach of the CPRIT Office with the cancer research community. For example, in response to the low success rate for awards to the IIRA for Computational Biology, Dr. Kripke consulted with the Chairs of the Scientific Review Panels and applicants, held a meeting with investigators at MD

Anderson and presented webinar as strategies to improve applicants' success rate. Dr. Willson attends meetings of the Scientific Review Panels and is available to help interpret reviews. This extremely valuable communication path enables cancer researchers to gain valuable insights into key priority areas and express any concerns, as well as providing transparency in the review process.

- Submission rates were robust over FY16, indicative of a high level of engagement with the cancer research community; funding rates varied with mechanism with an overall rate of 11% for individual investigator awards, indicating a high level of competition, similar to that encountered by PIs at federal agencies.
- The MIRA mechanism has been put on pause at this time. While this mechanism has been important in promoting cooperative research; this is a resource-intensive mechanism. As such, the UAC is in agreement with the decision to defer announcing this mechanism until FY 18.

UAC Recommendations:

- Support for innovative clinical trials that incorporate translational studies involving biological/molecular correlates is needed to provide insight into optimizing treatment efficacy and accelerating production of effective treatments. The UAC is enthusiastic about considering funding in this arena.
- Promote greater linkage between the Product Development Program and the research community, thereby enhancing the effectiveness of the Early Translational Research Awards. Similar linkage between the Prevention Program and research community is encouraged.
- A new RFA was released in January 2017, focusing on translational research, specifically clinical trials of new treatment strategies and particularly those with biomarker or other biologic correlative studies. This RFA addresses an unmet need with tremendous potential, particularly because it also includes molecular studies on tissues, already accrued from prior trials, which will help illuminate mechanism of action or resistance pathways. This RFA partners well with the Early Translational Research Program. Outcomes from these projects should be carefully monitored and are predicted to have high potential for impact.

2. UAC Recommendations on CPRIT Outcome Metrics

- This year marked the halfway point in authorized funding for CPRIT. The UAC reviewed the documented impacts and suggests that additional metrics include publications, overall data on treatment efficacies, economic impacts with successful interventions and education, as well as other potential measures.
- Outreach to additional institutions and stakeholders across Texas is proving effective and this effort should be encouraged in order to continue to bring a spectrum of Texas Institutions across the state into the conversation and actively involved. Metrics including publications, new grants linked to CPRIT funding, trainee accomplishments, rankings of Texas Universities, and other recognitions remain important metrics. Highlight the accomplishments of CPRIT Recruited Faculty; they have made critical contributions both within their Institutions as well as on a national and international scale. All these efforts in addition to the

outstanding cancer researchers already working in Texas emphasize the breadth and depth of our programs and our rapid forward progress.

- Impacts on underserved populations should be considered a key component of determining the efficacy of our translational science; this is critical to many areas in Texas, both in urban and rural regions.
- Collaborations through core facilities across Texas Institutions remain an important aspect in optimizing reach and inclusiveness. Partnerships emerging by collaborating on technologies, available through core facilities, provide opportunities that may be otherwise unavailable: i) utilizing specialized technologies with experts, and ii) fostering quantum leaps forward in research that underpins translational science. Metrics on these relationships enable assessment of overall impact and demonstrate a statewide spirit of cooperation with a shared goal of curing cancer.
- Educating stakeholders remains critical; publications and write-ups are needed that highlight outcomes of translating research into applications that impact patient survival and reduce unintended side effects of treatments.

3. UAC Comments and Recommendations on Program Progress

- As noted in the Fall UAC meeting, the CPRIT Academic Research Office has been interactive with the cancer research community during 2016 through a number of information sessions at Texas Institutions, workshops and visits to cancer researchers. Researchers are very appreciative of these opportunities, especially to obtain more guidance and to determine which funding across CPRIT's portfolio best fits their research interests.
- The UAC enthusiastically supports programs in prevention and early detection research; computational biology and analytic methods; childhood cancers; and intractable cancers with particular emphasis on population disparities and cancers of significance in Texas (e.g. lung, liver, and cervical cancers). Finally, it is critically important to add to the life sciences infrastructure in the State of Texas.
- As such, The Oversight Committee Priorities listed below are in full agreement with UAC priorities.
    - Recruitment of outstanding cancer researchers to Texas
    - Investment in core facilities
    - A broad range of innovative, investigator-initiated research projects
    - Prevention and early detection
    - Computational biology and analytic methods
    - Childhood cancers
    - Population disparities and cancers of importance in Texas
- It is important to have continued attention to factors; environmental, economic, community and lifestyle that influence and potentially mitigate or prevent the onset and progression of cancer and cancer-related disease.
- The CPRIT Academic Research RFA Prospective Time Line is reasonable and provides for sound fiscal management.
- The graphic showing the Recruitment impact illustrates the reach of the program and rapid gains engendered through this funding mechanism; supporting the strong continuation of this mechanism.

Recruitment Impact

	CPRIT Scholars Recruited to Texas		Extramural Funding to Date
First Time Tenure Track	71		$73,000,000
Rising Stars	15		$36,000,000
Established Investigators	27		$73,000,000
Total	**113**		**$182,000,000**

Accolades for CPRIT Scholars:

- ✓ 8 members National Academies of Sciences; Medicine; Engineering
- ✓ 2 Members Howard Hughes Medical Institute
- ✓ 2 National Cancer Institute Outstanding Investigators
- ✓ 2015 Lasker DeBakey Clinical Medical Research Award
- ✓ 2015 American Society of Clinical Oncology's Gianni Bonadonna Breast Cancer Award
- ✓ 2016 American Society of Clinical Oncology Distinguished Achievement Award
- ✓ 2016 O'Donnell Award in Medicine, Academy of Medicine, Engineering and Science of Texas

"UCSD loses 3 star scientists to Rice University" ***The San Diego Star Union-Tribune 5/11/11***

"In a time of declining federal investment in biomedical research, the resources from CPRIT will make it possible to do things in Texas that are difficult or impossible to do in most other places".
Sean Morrison and the Texas Brain Gain J Clin Invest. **2012;12:424.**

"The Battle for Biomedical Supremacy …Much of the frenzy has been driven by the outsize role of Texas""
August 29, 2015, ***NY Times Editorial 8/29/15***

CANCER PREVENTION & RESEARCH INSTITUTE OF TEXAS

MEMORANDUM

To:	**OVERSIGHT COMMITTEE MEMBERS**
From:	**KRISTEN PAULING DOYLE, GENERAL COUNSEL** **CAMERON L. ECKEL, STAFF ATTORNEY**
Subject:	**CHAPTER 703 RULE CHANGES PROPOSED FOR FINAL ADOPTION**
Date:	**FEBRUARY 8, 2017**

Summary and Recommendation

The Board Governance Subcommittee recommends that the Oversight Committee adopt the proposed administrative rule changes to Chapter 703 as originally considered at the November 2016 meeting. Once the Oversight Committee approves the final orders adopting the rule changes, CPRIT will submit the amendments to the Secretary of State and the changes will be considered final and effective 20 days later.

Discussion

State law requires an agency to set policy using a rulemaking process, which includes an opportunity for public comment on proposed rules and rule changes before the agency formally adopts the policy. The first rule amendment removes a reference to OMB Circular A-133 that has been superseded. The second rule amendments explains that a request to carry forward unspent grant funds from one project year to the next requires Institute approval if the amount of the unexpended budget line item balance is 25% or more of the line item amount for the year.

CPRIT published the proposed rules in the December 23rd edition of the *Texas Register*, as well as solicited public comment via CPRIT's website.

The Board Governance Subcommittee met on February 2nd to review the public comments and final orders with CPRIT's General Counsel. The Subcommittee recommends the Oversight Committee approve the final orders adopting the proposed rule changes.

Next Steps

After the Oversight Committee adopts the proposed rule changes, CPRIT will submit the final order to the Secretary of State. The rule changes become effective 20 days after the date CPRIT files the order with the Secretary of State.

TITLE 25. HEALTH SERVICES

PART 11. CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

CHAPTER 703. Grants for Cancer Prevention and Research

The Cancer Prevention and Research Institute of Texas ("CPRIT" or "the Institute") adopts the amendments to § 703.13(e) regarding guidance for government auditing standards and to § 703.25 regarding approval of a grantee's request to carry forward unspent project year funds into the following project year. The proposed amendments were published in the December 23, 2016, issue of the *Texas Register* (41 TexReg 10064).

Reasoned Justification

The proposed amendment to § 703.13(e)(4) replaces a reference to OMB Circular A-133 that has been superseded. The change clarifies guidance for government auditing standards. The proposed amendment to § 703.25 explains that a request to carry forward unspent grant funds from one project year to the next requires Institute approval if the amount of the unexpended budget line item balance is 25% or more of the line item amount for the year. Section 703.25 already allows grantees to requests a carry forward of unspent funds. This change aligns § 703.25 with the Institute's current practice. All other requirements regarding carry forward requests remain the same in § 703.25.

Summary of Public Comments and Staff Recommendation

CPRIT received no public comments regarding the proposed amendments.

The rule changes are adopted under the authority of the Texas Health and Safety Code Annotated, §§ 102.108 and 102.251, which provides the Institute with broad rule-making authority to administer the chapter, including rules for awarding grants.

Certification

The Institute hereby certifies that the adoption has been reviewed by legal counsel and found to be a valid exercise of the agency's legal authority.

To be filed with the Office of Secretary of State on February 17, 2017.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

To:	**OVERSIGHT COMMITTEE MEMBERS**
From:	**KRISTEN PAULING DOYLE, GENERAL COUNSEL** **CAMERON L. ECKEL, STAFF ATTORNEY**
Subject:	**SUMMARY OF PROPOSED RULE CHANGES TO BE PROPOSED FEBRUARY 2017**
Date:	**FEBRUARY 8, 2017**

Summary and Recommendation

The Board Governance Subcommittee recommends that the Oversight Committee approve the six proposed administrative rule changes. The proposed changes affect Texas Administrative Code Chapters 701 and 703. After approval, CPRIT will publish the proposed changes in the *Texas Register* for public comment.

Discussion

CPRIT's administrative rules set policy guiding CPRIT's grant review and grant contracting processes. State law requires agencies to set policy using a rulemaking process, which includes an opportunity for the public to comment on proposed rules and rule changes before the agency adopts the final policy.

The Board Governance subcommittee met on February 2nd to discuss the proposed rule changes with legal staff. A summary of each proposed rule change is attached. The subcommittee voted to recommend approval and publication to the Oversight Committee.

Next Steps

Once approved by the Oversight Committee, CPRIT will publish the proposed rules in the *Texas Register*. The publication date begins the 30-day period soliciting public comment. CPRIT staff will post the proposed rules on CPRIT's website and announce the opportunity for public comment via the CPRIT electronic list serve. CPRIT legal staff will summarize all public comments for the Oversight Committee's consideration when approving the final rule changes in May.

Proposed Rule Amendments – February 2017

§ 701.3 Definitions

Proposed Change - § 701.3(57) is amended to correct the explanation of a relationship by second-degree consanguinity. Second-degree consanguinity does not include aunts, uncles, nieces, or nephews. The amendment also updates the characterization of affinity for clarity.

§ 703.5 Scientific Research and Prevention Programs Committees

Proposed Change - § 703.5(g) is amended to expand the existing one-year restriction against a peer review panel member providing professional services in excess of $5000 to grantees reviewed by the member's panel. The restriction now applies to all grant applicants reviewed by the member's panel.

§ 703.6 Grant Review Process

Proposed Change – § 703.6(e)(4)(B) is amended to reflect that the Product Development Review Council decides the applications moving forward to due diligence. The Review Council decision is based on the applications recommended for due diligence by the Peer Review Panel(s).

§ 703.10 Awarding Grants by Contract

Proposed Change - § 703.10(c)(25) is added to the items included in the CPRIT grant award contract. The addition requires the grantee to provide certification of approval by the Institutional Animal Care and Use Committee (IAUCUC) or Institutional Review Board (IRB), if applicable, prior to receiving CPRIT funds. The amendment also requires the grantee to update the certification(s) annually.

§ 703.11 Requirement to Demonstrate Available Funds for Cancer Research Grants

Proposed Change - § 703.11(a)(4) is added to clarify that CPRIT may require a grantee to commit to a matching funds obligation that is greater than the 2:1 ratio set by the statute. Research grantees are required to contribute $1 for every $2 CPRIT pays toward the project. The Product Development Subcommittee recently recommended a policy change for Oversight Committee consideration to increase the amount contributed by a Product Development grantee who has previously received a CPRIT award. If the Oversight Committee approves the policy change, this rule amendment is necessary to carry out the new requirement. CPRIT will notify grant applicants of the increased matching fund commitment via the request for applications.

§ 703.24 Financial Status Reports

Proposed Change - § 703.24(a) is amended to add a new paragraph (4) that sets a deadline for grantees to submit supporting documentation associated with the quarterly financial status report (FSR). Occasionally, supporting documentation will be deficient or incorrect. CPRIT may request additional information and leave the FSR submission open while waiting for the additional information (rather than disapproving the FSR). The proposed amendment sets a uniform deadline for the grantee to submit the requested documentation. Failure to submit the documentation within 21 days will result in an automatic disapproval of the FSR.

TITLE 25. HEALTH SERVICES

PART 11. CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

CHAPTER 701. Policies and Procedures

The Cancer Prevention and Research Institute of Texas (Institute) proposes an amendment to § 701.3 to clarify the definition of relative within the second degree by consanguinity or affinity.

Background and Justification

The proposed amendment to § 701.3 removes the examples of "uncle, aunt, niece, or nephew" from the list of examples of relatives related within the second degree by consanguinity or affinity. The rule references Texas Government Code §§ 573.021-.025 and within that statute, the above examples are relatives within the third degree of consanguinity or affinity.

Fiscal Note

Kristen Pauling Doyle, General Counsel for the Cancer Prevention and Research Institute of Texas, has determined that for the first five-year period the rule changes are in effect, there will be no foreseeable implications relating to costs or revenues for state or local government because of enforcing or administering the rules.

Public Benefit and Costs

Ms. Doyle has determined that for each year of the first five years the rule changes are in effect the public benefit anticipated because of enforcing the rules will be clarification of policies and procedures the Institute will follow to implement its statutory duties.

Small Business and Micro-business Impact Analysis

Ms. Doyle has determined that the rule changes shall not have an effect on small businesses or on micro businesses.

Written comments on the proposed rule changes may be submitted to Ms. Kristen Pauling Doyle, General Counsel, Cancer Prevention and Research Institute of Texas, P. O. Box 12097, Austin, Texas 78711 no later than April 3, 2017. Parties filing comments are asked to indicate whether or not they support the rule revisions proposed by the Institute and, if a change is requested, to provide specific text proposed to be included in the rule. Comments may be submitted electronically to kdoyle@cprit.texas.gov. Comments may be submitted by facsimile transmission to 512/475-2563.

Statutory Authority

The rule changes are proposed under the authority of the Texas Health and Safety Code Annotated, § 102.108 which provides the Institute with broad rule-making authority to administer the chapter. Kristen Pauling Doyle, the Institute's General Counsel, has reviewed the proposed amendments and certifies the proposal to be within the Institute's authority to adopt.

There is no other statute, article or code that is affected by these rules.

701.3 Definitions

The following words and terms, when used in this chapter, shall have the following meanings, unless the context clearly indicates otherwise.

(1) Advisory Committee--a committee of experts, including practitioners and patient advocates, created by the Oversight Committee to advise the Oversight Committee on issues related to cancer.

(2) Allowable Cost--a cost that is reasonable, necessary for the proper and efficient performance and administration of the project, and allocable to the project.

(3) Annual Public Report--the report issued by the Institute pursuant to Texas Health and Safety Code §102.052 outlining Institute activities, including Grant Awards, research accomplishments, future Program directions, compliance, and Conflicts of Interest actions.

(4) Authorized Expense--cost items including honoraria, salaries and benefits, consumable supplies, other operating expenses, contracted research and development, capital equipment, construction or renovation of state or private facilities, travel, and conference fees and expenses.

(5) Approved Budget--the financial expenditure plan for the Grant Award, including revisions approved by the Institute and permissible revisions made by the Grant Recipient. The Approved Budget may be shown by Project Year and detailed budget categories.

(6) Authorized Signing Official (ASO)--the individual, including designated alternates, named by the Grant Applicant, who is authorized to act for the Grant Applicant or Grant Recipient in submitting the Grant Application and executing the Grant Contract and associated documents or requests.

(7) Bylaws--the rules established by the Oversight Committee to provide a framework for its operation, management, and governance.

(8) Cancer Prevention--a reduction in the risk of developing cancer, including early detection, control and/or mitigation of the incidence, disability, mortality, or post-diagnosis effects of cancer.

(9) Cancer Prevention and Control Program--effective strategies and interventions for preventing and controlling cancer designed to reduce the incidence and mortality of cancer and to enhance the quality of life of those affected by cancer.

(10) Cancer Prevention and Research Fund--the dedicated account in the general revenue fund consisting of legislative appropriations, gifts, grants, other donations, and earned interest.

(11) Cancer Research--research into the prevention, causes, detection, treatments, and cures for all types of cancer in humans, including basic mechanistic studies, pre-clinical studies, animal model studies, translational research, and clinical research to develop preventative measures, therapies, protocols, medical pharmaceuticals, medical devices or procedures for the detection, treatment, cure or substantial mitigation of all types of cancer and its effects in humans.

(12) Chief Compliance Officer--the individual employed by the Institute to monitor and report to the Oversight Committee regarding compliance with the Institute's statute and administrative rules. The term may also apply to an individual designated by the Chief Compliance Officer to fulfill the duty or duties described herein, unless the context clearly indicates otherwise.

(13) Chief Executive Officer--the individual hired by the Oversight Committee to perform duties required by the Institute's Statute or designated by the Oversight Committee. The term may apply to an individual designated by the Chief Executive Officer to fulfill the duty or duties described herein, unless the context clearly indicates otherwise.

(14) Chief Prevention Officer--the individual hired by the Chief Executive Officer to oversee the Institute's Cancer Prevention program, including the Grant Review Process, and to assist the Chief Executive Officer in collaborative outreach to further Cancer Research and Cancer Prevention. The term may also apply to an individual designated by the Chief Prevention Officer to fulfill the duty or duties described herein, unless the context clearly indicates otherwise.

(15) Chief Product Development Officer--the individual hired by Chief Executive Officer to oversee the Institute's Product Development program for drugs, biologicals, diagnostics, or devices arising from Cancer Research, including the Grant Review Process, and to assist the Chief Executive Officer in collaborative outreach to further Cancer Research and Cancer Prevention. The term may apply to an individual designated by the Chief Product Development Officer to fulfill the duty or duties described herein, unless the context clearly indicates otherwise.

(16) Chief Scientific Officer--the individual hired by the Chief Executive Officer to oversee the Institute's Cancer Research program, including the Grant Review Process, and to assist the Chief Executive Officer in collaborative outreach to further Cancer Research and Cancer Prevention. The term may apply to an individual designated by the Chief Scientific Officer to fulfill the duty or duties described herein, unless the context clearly indicates otherwise.

(17) Code of Conduct and Ethics--the code adopted by the Oversight Committee pursuant to Texas Health and Safety Code §102.109 to provide guidance related to the ethical conduct expected of Oversight Committee Members, Program Integration Committee Members, and Institute Employees.

(18) Compliance Program--a process to assess and ensure compliance by the Oversight Committee Members and Institute Employees with applicable laws, rules, and policies, including matters of ethics and standards of conduct, financial reporting, internal accounting controls, and auditing.

(19) Conflict(s) of Interest--a financial, professional, or personal interest held by the individual or the individual's Relative that is contrary to the individual's obligation and duty to act for the benefit of the Institute.

(20) Encumbered Funds--funds that are designated by a Grant Recipient for a specific purpose.

(21) Financial Status Report--form used to report all Grant Award related financial expenditures incurred in implementation of the Grant Award. This form may also be referred to as "FSR" or "Form 269-A."

(22) Grant Applicant--the public or private institution of higher education, as defined by §61.003, Texas Education Code, research institution, government organization, non-governmental organization, non-profit organization, other public entity, private company, individual, or consortia, including any combination of the aforementioned, that submits a Grant Application to the Institute. Unless otherwise indicated, this term includes the Principal Investigator or Program Director.

(23) Grant Application--the written proposal submitted by a Grant Applicant to the Institute in the form required by the Institute that, if successful, will result in a Grant Award.

(24) Grant Award--funding, including a direct company investment, awarded by the Institute pursuant to a Grant Contract providing money to the Grant Recipient to carry out the Cancer Research or Cancer Prevention project in accordance with rules, regulations, and guidance provided by the Institute.

(25) Grant Contract--the legal agreement executed by the Grant Recipient and the Institute setting forth the terms and conditions for the Cancer Research or Cancer Prevention Grant Award approved by the Oversight Committee.

(26) Grant Management System--the electronic interactive system used by the Institute to exchange, record, and store Grant Application and Grant Award information.

(27) Grant Mechanism--the specific Grant Award type.

(28) Grant Program--the functional area in which the Institute makes Grant Awards, including research, prevention and product development.

(29) Grant Progress Report--the required report submitted by the Grant Recipient at least annually and at the close of the grant award describing the activities undertaken to achieve the goals and objectives of the funded project and including information, data and program metrics. Unless the context clearly indicates otherwise, the Grant Progress Report also includes other required reports such as a Historically Underutilized Business and Texas Supplier form, a single audit determination form, an inventory report, a single audit determination form, a revenue sharing form, and any other reports or forms designated by the Institute.

(30) Grant Recipient--the entire legal entity responsible for the performance or administration of the Grant Award pursuant to the Grant Contract. Unless otherwise indicated, this term includes the Principal Investigator, Program Director, or Company Representative.

(31) Grant Review Cycle--the period that begins on the day that the Request for Applications is released for a particular Grant Mechanism and ends on the day that the Oversight Committee takes action on the Grant Award recommendations.

(32) Grant Review Process--the Institute's processes for Peer Review, Program Review and Oversight Committee approval of Grant Applications.

(33) Indirect Costs--the expenses of doing business that are not readily identified with a particular Grant Award, Grant Contract, project, function, or activity, but are necessary for the general operation of the Grant Recipient or the performance of the Grant Recipient's activities.

(34) Institute--the Cancer Prevention and Research Institute of Texas or CPRIT.

(35) Institute Employee--any individual employed by the Institute, including any individual performing duties for the Institute pursuant to a contract of employment. Unless otherwise indicated, the term does not include an individual providing services to the Institute pursuant to a services contract.

(36) Intellectual Property Rights--any and all of the following and all rights in, arising out of, or associated therewith, but only to the extent resulting from the Grant Award:

(A) The United States and foreign patents and utility models and applications therefore and all reissues, divisions, re-examinations, renewals, extensions, provisionals, continuations and such claims of continuations-in-part as are entitled to claim priority to the aforesaid patents or patent applications, and equivalent or similar rights anywhere in the world in Inventions and discoveries;

(B) All trade secrets and rights in know-how and proprietary information;

(C) All copyrights, whether registered or unregistered, and applications therefore, and all other rights corresponding thereto throughout the world excluding scholarly and academic works such as professional articles and presentations, lab notebooks, and original medical records; and

(D) All mask works, mask work registrations and applications therefore, and any equivalent or similar rights in semiconductor masks, layouts, architectures or topography.

(37) Invention--any method, device, process or discovery that is conceived and/or reduced to practice, whether patentable or not, by the Grant Recipient in the performance of work funded by the Grant Award.

(38) License Agreement--an understanding by which an owner of Technology and associated Intellectual Property Rights grants any right to make, use, develop, sell, offer to sell, import, or otherwise exploit the Technology or Intellectual Property Rights in exchange for consideration.

(39) Matching Funds--the Grant Recipient's Encumbered Funds equal to one-half of the Grant Award available and not yet expended that are dedicated to the research that is the subject of the Grant Award. For public and private institutions of higher education, this includes the dollar amount equivalent to the difference between the indirect cost rate authorized by the federal government for research grants awarded to the Grant Recipient and the five percent (5%) Indirect Cost limit imposed by §102.203(c), Texas Health and Safety Code.

(40) Numerical Ranking Score--the score given to a Grant Application by the Review Council that is substantially based on the final Overall Evaluation Score submitted by the Peer Review Panel, but also signifies the Review Council's view related to how well the Grant Application achieves program priorities set by the Oversight Committee, the overall Program portfolio balance, and any other criteria described in the Request for Applications.

(41) Overall Evaluation Score--the score given to a Grant Application during the Peer Review Panel review that signifies the reviewers' overall impression of the Grant Application. Typically it is the average of the scores assigned by two or more Peer Review Panel members.

(42) Oversight Committee--the Institute's governing body, composed of the nine individuals appointed by the Governor, Lieutenant Governor, and the Speaker of the House of Representatives.

(43) Oversight Committee Member--any person appointed to and serving on the Oversight Committee.

(44) Patient Advocate--a trained individual who meets the qualifications set by the Institute and is appointed to a Scientific Research and Prevention Programs Committee to specifically represent the interests of cancer patients as part of the Peer Review of Grant Applications assigned to the individual's committee.

(45) Peer Review--the review process performed by Scientific Research and Prevention Programs Committee members and used by the Institute to provide guidance and recommendations to the Program Integration Committee and the Oversight Committee in making decisions for Grant Awards. The process involves the consistent application of standards and procedures to produce a fair, equitable, and objective evaluation of scientific and technical merit, as well as other relevant aspects of the Grant Application. When used herein, the term applies individually or collectively, as the context may indicate, to the following review process(es): Preliminary Evaluation, Individual Evaluation by Primary Reviewers, Peer Review Panel discussion and Review Council prioritization.

(46) Peer Review Panel--a group of Scientific Research and Prevention Programs Committee members conducting Peer Review of assigned Grant Applications.

(47) Prevention Review Council--the group of Scientific Research and Prevention Programs Committee members designated as the chairpersons of the Peer Review Panels that review Cancer Prevention program Grant Applications. This group includes the Review Council chairperson.

(48) Primary Reviewer--a Scientific Research and Prevention Programs Committee member responsible for individually evaluating all components of the Grant Application, critiquing the merits according to explicit criteria published in the Request for Applications, and providing an individual Overall Evaluation Score that conveys the general impression of the Grant Application's merit.

(49) Principal Investigator, Program Director, or Company Representative--the single individual designated by the Grant Applicant or Grant Recipient to have the appropriate level of authority and responsibility to direct the project to be supported by the Grant Award.

(50) Product Development Review Council--the group of Scientific Research and Prevention Programs Committee Members designated as the chairpersons of the Peer Review Panels that review Grant Applications for the development of drugs, drugs, biologicals, diagnostics, or devices arising from earlier-stage Cancer Research. This group includes the Review Council chairperson.

(51) Product Development Prospects--the potential for development of products, services, or infrastructure to support Cancer Research efforts, including but not limited to pre-clinical, clinical, manufacturing, and scale up activities.

(52) Program Income--income from fees for services performed, from the use or rental of real or personal property acquired with Grant Award funds, and from the sale of commodities or items fabricated under the Grant Contract. Except as otherwise provided, Program Income does not include rebates, credits, discounts, refunds, etc. or the interest earned on any of these items. Interest otherwise earned in excess of $250 on Grant Award funds is considered Program Income.

(53) Program Integration Committee--the group composed of the Chief Executive Officer, the Chief Scientific Officer, the Chief Product Development Officer, the Commissioner of State Health Services, and the Chief Prevention Officer that is responsible for submitting to the Oversight Committee the list of Grant Applications the Program Integration Committee recommends for Grant Awards.

(54) Project Results--all outcomes of a Grant Award, including publications, knowledge gained, additional funding generated, and any and all Technology and associated Intellectual Property Rights.

(55) Project Year--the intervals of time (usually 12 months each) into which a Grant Award is divided for budgetary, funding, and reporting purposes. The effective date of the Grant Contract is the first day of the first Project Year.

(56) Real Property--land, including land improvements, structures and appurtenances thereto, excluding movable machinery and equipment.

(57) Relative--a person related within the second degree by consanguinity or affinity determined in accordance with §§573.021 - 573.025, Texas Government Code. For purposes of this definition:

(A) examples of an individual within the second degree by consanguinity are a child, grandchild, parent, grandparent, brother, sister, ~~uncle, aunt, niece, or nephew~~;

(B) a husband and wife are related to each other in the first degree of affinity. For other relationship by affinity, the degree of relationship is the same as the degree of the underlying relationship by consanguinity. [~~examples of an individual within the second degree by affinity are a spouse, a person related to a spouse within the second degree by consanguinity, or a spouse of such a person;~~]

(C) an individual adopted into a family is considered a Relative on the same basis as a natural born family member; and

(D) an individual is considered a spouse even if the marriage has been dissolved by death or divorce if there are surviving children of that marriage.

(58) Request for Applications--the invitation released by the Institute seeking the submission of Grant Applications for a particular Grant Mechanism. It provides information relevant to the Grant Award to be funded, including funding amount, Grant Review Process information, evaluation criteria, and required Grant Application components.

(59) Review Council--the term used to generally refer to one or more of the Prevention Review Council, the Product Development Review Council, or Scientific Review Council.

(60) Scientific Research and Prevention Programs Committee--a group of experts in the field of Cancer Research, Cancer Prevention or Product Development, including trained Patient Advocates, appointed by the Chief Executive Officer and approved by the Oversight Committee for the purpose of conducting Peer Review of Grants Applications and recommending Grant Awards. A Peer Review Panel is a Scientific Research and Prevention Programs Committee, as is a Review Council.

(61) Scientific Research and Prevention Programs Committee Member--an individual appointed by the Chief Executive Officer and approved by the Oversight Committee to serve on a Scientific Research and Prevention Programs Committee. Peer Review Panel Members are Scientific Research and Prevention Programs Committee Members, as are Review Council Members.

(62) Scientific Review Council--the group of Scientific Research and Prevention Programs Committee Members designated as the chairpersons of the Peer Review Panels that review Cancer Research Grant Applications. This group includes the Review Council chairperson.

(63) Scope of Work--the goals and objectives of the Cancer Research or Cancer Prevention project, including the timeline and milestones to be achieved.

(64) Senior Member or Key Personnel--the Principal Investigator, Project Director or Company Representative and other individuals who contribute to the scientific development or execution of a project in a substantive, measurable way, whether or not the individuals receive salary or compensation under the Grant Award.

(65) Technology--any and all of the following resulting or arising from work funded by the Grant Award:

(A) Inventions;

(B) Third-Party Information, including but not limited to data, trade secrets and know-how;

(C) databases, compilations and collections of data;

(D) tools, methods and processes; and

(E) works of authorship, excluding all scholarly works, but including, without limitation, computer programs, source code and executable code, whether embodied in software, firmware or otherwise, documentation, files, records, data and mask works; and all instantiations of the foregoing in any form and embodied in any form, including but not limited to therapeutics, drugs, drug delivery systems, drug formulations, devices, diagnostics, biomarkers, reagents and research tools.

(66) Texas Cancer Plan--a coordinated, prioritized, and actionable framework that helps to guide statewide efforts to fight the human and economic burden of cancer in Texas.

(67) Third-Party Information--generally, all trade secrets, proprietary information, know-how and non-public business information disclosed to the Institute by Grant Applicant, Grant Recipient, or other individual external to the Institute.

(68) Tobacco--all forms of tobacco products, including but not limited to cigarettes, cigars, pipes, water pipes (hookah), bidis, kreteks, electronic cigarettes, smokeless tobacco, snuff and chewing tobacco.

TITLE 25. HEALTH SERVICES

PART 11. CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

CHAPTER 703. Grants for Cancer Prevention and Research

The Cancer Prevention and Research Institute of Texas (Institute) proposes an amendment to §§ 703.5, 703.6, 703.10, 703.11, and 701.24. The proposed changes affect conflicts of interest prohibitions of Scientific Research and Prevention Program Committee Members, Product Development Review Council role in due diligence, grant contract terms regarding certification of approval by the Institutional Animal Care and Use Committee (IACUC) or Institutional Review Board (IRB), the matching fund requirements of Academic Research and Product Development Research grantees, and disapproval of Financial Status Reports for untimely supporting documentation.

Background and Justification

The proposed amendment to § 703.5(g) prohibits a Scientific Research and Prevention Programs Committee Member from providing professional services to a grant applicant that results in compensation of more than $5,000. The restriction would be in place for one year beginning from the due date of the Grant Application or the effective date of the Grant Award, whichever is later.

The proposed amendment to § 703.6(c)(4)(B) reflects that the Product Development Review Council decides the applications moving forward in the review process for due diligence. The Review Council's decision is based on the Grant Applications recommended by the Peer Review Panel(s).

The proposed amendment to § 703.10(c) would require grant recipients to provide to the Institute certification of approval by the Institutional Animal Care and Use Committee (IACUC) or Institutional Review Board (IRB), as appropriate, if the grant project includes the use of vertebrate animals. The certification must be provided before contract funds are disbursed and annually as recertified.

The proposed amendment to § 703.11(a) clarifies that the Institute may require a research grant recipient to demonstrate a matching funds obligation greater than one-half of the grant award amount. In the event that the Institute increases the matching funds obligation, the proposed language requires the Institute to include the obligation in the Request for Applications. Texas Health and Safety Code § 102.0255 requires research grant recipients to dedicate an amount of matching funds equal to one-half of the grant award.

The proposed amendment to § 703.24 allows the Institute to disapprove a Financial Status Report (FSR) if a Grant Recipient does not timely respond to a written request by the Institute for more information or backup documentation. If the Institute submits a request in writing for more information or backup documentation regarding an FSR, the Grant Recipient has 21 days to respond to the request. If there is no response by the Grant Recipient within that timeframe,

the FSR will be disapproved. The proposed amendment is not intended to restrict the Institute's ability to disapprove an FSR or to extend the Grant Recipient's FSR due date.

Fiscal Note

Kristen Pauling Doyle, General Counsel for the Cancer Prevention and Research Institute of Texas, has determined that for the first five-year period the rule changes are in effect there will be no foreseeable implications relating to costs or revenues for state or local government as a result of enforcing or administering the rules.

Public Benefit and Costs

Ms. Doyle has determined that for each year of the first five years the rule changes are in effect the public benefit anticipated as a result of enforcing the rules will be clarification of policies and procedures the Institute will follow to implement its statutory duties.

Small Business and Micro-business Impact Analysis

Ms. Doyle has determined that the rule changes shall not have an effect on small businesses or on micro businesses.

Written comments on the proposed rule changes may be submitted to Ms. Kristen Pauling Doyle, General Counsel, Cancer Prevention and Research Institute of Texas, P. O. Box 12097, Austin, Texas 78711 no later than April 3, 2017. Parties filing comments are asked to indicate whether or not they support the rule revisions proposed by the Institute and, if a change is requested, to provide specific text proposed to be included in the rule. Comments may be submitted electronically to kdoyle@cprit.texas.gov. Comments may be submitted by facsimile transmission to 512/475-2563.

Statutory Authority

The rule changes are proposed under the authority of the Texas Health and Safety Code Annotated, §§ 102.108 and 102.251, which provide the Institute with broad rule-making authority to administer the chapter and to issue rules regarding the procedures for awarding grants. Kristen Pauling Doyle, the Institute's General Counsel, has reviewed the proposed amendments and certifies the proposal to be within the Institute's authority to adopt.

There is no other statute, article or code that is affected by these rules.

RULE §703.5 Scientific Research and Prevention Programs Committees

(a) The Oversight Committee shall establish Scientific Research and Prevention Programs Committees for the purpose of conducting Peer Review of Grant Applications submitted to the Institute. Such Peer Review activities may include post award evaluation of Grant Progress Reports. The Chief Executive Officer, with approval by simple majority of the Oversight Committee, is responsible for appointing experts in the fields of Cancer Research, Prevention life science Product Development, and patient advocacy to serve as Scientific Research and Prevention Programs Committee members for terms designated by the Chief Executive Officer.

(b) The Chief Executive Officer may provisionally appoint an individual as a Scientific Research and Prevention Programs Committee Member until such time that the individual can be considered for approval by the Oversight Committee. The provisional appointee may participate in the Peer Review Process prior to a vote of the Oversight Committee on the appointment so long as the appointment is considered at the next regular Oversight Committee meeting.

(c) A Scientific Research and Prevention Programs Committee Member is responsible for conducting Peer Review of the Grant Applications assigned to the individual member's Peer Review Panel.

(d) A Scientific Research and Prevention Programs Committee Member may receive an honorarium in accordance with the policy described in Chapter 701, §701.15 of this title (relating to the Scientific Research and Prevention Programs Committee Honoraria Policy).

(e) A member of a Scientific Research and Prevention Programs Committee is prohibited from attempting to use the committee member's official position to influence a decision to approve or award a grant or contract to the committee member's employer.

(f) A member of a Scientific Research and Prevention Programs Committee must comply with the requirements set forth in Chapter 702 of this title (relating to Institute Standards on Ethics and Conflicts, Including the Acceptance of Gifts and Donations to the Institute) and Chapter 102, Texas Health and Safety Code.

(g) The Scientific Research and Prevention Programs Committee Member shall not provide professional services for compensation exceeding $5,000 to any Grant ~~Recipient~~ Applicant that was reviewed by the Scientific Research and Prevention Programs Committee Member's Peer Review Panel.

(1) The term of this restriction is for a period of one year from the due date of the Grant Application or the effective date of the Grant Award, whichever is later, unless waived by a vote of the Oversight Committee.

(2) For purposes of this restriction, "professional services" do not include those services for which an honorarium is paid; however, honoraria exceeding $5,000 paid to a Scientific Research and Prevention Programs Committee Member by a Grant ~~Recipient~~ Applicant while the individual is serving as a Committee Member shall be reported within 30 days to the Institute's Chief Executive Officer.

(3) Even if a payment to a Scientific Research and Prevention Programs Committee Member is not otherwise prohibited, a Grant Recipient shall not pay a Scientific Research and Prevention Programs Committee Member with Grant Award funds.

(h) An individual that serves as a Scientific Research and Prevention Programs Committee Member may not concurrently serve on the Board of Directors or other governing board of a Grant Recipient or of a foundation or similar organization affiliated with the entity. This prohibition lasts so long as the Grant Recipient receives Grant Award funds or the Scientific Research and Prevention Programs Committee Member receives an honorarium from the Institute, whichever ends first.

(i) The Scientific Research and Prevention Programs Committee Member shall not use non-public Third-Party Information or knowledge of non-public decisions related to Grant Applicants, gained by virtue of the individual's participation in the Institute's Peer Review Process, to make an investment or take some other action resulting in a financial benefit to the individual or the individual's employer.

(j) A violation of any requirement of this section may result in the removal of the Scientific Research and Prevention Programs Committee Member from further participation in the Institute's Peer Review Process.

(k) The Institute shall provide on the Institute's Internet website a register of the individuals appointed as Scientific Research and Prevention Programs Committee Members, including provisional members. The register may list the Scientific Research and Prevention Programs Committee members by Peer Review Panel. For the purpose of identifying undisclosed Conflicts of Interest, a Grant Applicant may be notified of the Peer Review Panel to which the Grant Application has been assigned.

(l) The Chief Executive Officer shall ensure that at least one Patient Advocate is appointed to each Peer Review Panel. To be considered for a Patient Advocate appointment by the Chief Executive Officer as a Scientific Research and Prevention Programs Committee Member, an applicant must:

(1) Represent an organization or other community of people;

(2) Demonstrate prior community involvement or other work on behalf of cancer patients;

(3) Possess good communication and writing skills, including the ability to analyze information and make judgments with consideration of patient impact;

(4) Express interest in and fundamental knowledge of the medical research process, including basic and translational scientific research and prevention concepts;

(5) Reside outside of the state of Texas;

(6) Have science-based training. This training requirement shall be considered fulfilled if the Patient Advocate has:

(A) attended a science-based training program from the American Association for Cancer Research Survivor-Scientist Program, American Society of Clinical Oncology Research Review Sessions for Patient Advocates, Research Advocacy Network Advocate Institute or National Breast Cancer Coalition Project LEAD no more than three years prior to appointment to the Institute's Scientific Research and Prevention Programs Committee; or

(B) participated in at least one full cycle of grant review conducted by the Institute, National Institutes of Health, Department of Defense Congressionally Directed Medical Research Programs, Federal Drug Administration or Patient-Centered Outcomes

Research Institute no more than three years prior to appointment to the Institute's Scientific Research and Prevention Programs Committee.

(m) An individual interested in a Patient Advocate appointment shall submit an application, in a format specified by the Institute that includes at least the following information:

(1) Dates of service on a peer review panel within the past three years, or dates of attendance at advocate training programs within the past three years as documentation of the fulfillment of the science-based training program requirement;

(2) Current resume or curriculum vitae;

(3) A letter of recommendation from a community-based organization and a personal statement on advocacy and education if the applicant has attended a training program but not yet served on a peer review panel

RULE §703.6 Grant Review Process

(a) For all Grant Applications that are not administratively withdrawn by the Institute for noncompliance or otherwise withdrawn by the Grant Applicant, the Institute shall use a two-stage Peer Review process.

(1) The Peer Review process, as described herein, is used to identify and recommend meritorious Cancer Research projects, including those projects with Cancer Research Product Development prospects, and evidence-based Cancer Prevention and Control projects for Grant Award consideration by the Program Integration Committee and the Oversight Committee.

(2) Peer Review will be conducted pursuant to the requirements set forth in Chapter 702 of this title (relating to Institute Standards on Ethics and Conflicts, Including the Acceptance of Gifts and Donations to the Institute) and Chapter 102, Texas Health and Safety Code.

(b) The two stages of the Peer Review Process used by the Institute are:

(1) Evaluation of Grant Applications by Peer Review Panels; and

(2) Prioritization of Grant Applications by the Prevention Review Council, the Product Development Review Council, or the Scientific Review Council, as may be appropriate for the Grant Program.

(c) Except as described in subsection (e) of this section, the Peer Review Panel evaluation process encompasses the following actions, which will be consistently applied:

(1) The Institute distributes all Grant Applications submitted for a particular Grant Mechanism to one or more Peer Review Panels.

(2) The Peer Review Panel chairperson assigns each Grant Application to no less than two panel members that serve as the Primary Reviewers for the Grant Application. Assignments are made based upon the expertise and background of the Primary Reviewer in relation to the Grant Application.

(3) The Primary Reviewer is responsible for individually evaluating all components of the Grant Application, critiquing the merits according to explicit criteria published in the Request for Applications, and providing an individual Overall Evaluation Score that conveys the Primary Reviewer's general impression of the Grant Application's merit. The Primary Reviewers' individual Overall Evaluation Scores are averaged together to produce a single initial Overall Evaluation Score for the Grant Application.

(4) The Peer Review Panel meets to discuss the Grant Applications assigned to the Peer Review Panel. If there is insufficient time to discuss all Grant Applications, the Peer Review Panel chairperson determines the Grant Applications to be discussed by the panel. The chairperson's decision is based largely on the Grant Application's initial Overall Evaluation Score; however a Peer Review Panel member may request that a Grant Application be discussed by the Peer Review Panel.

(A) If a Grant Application is not discussed by the Peer Review Panel, then the initial Overall Evaluation Score serves as the final Overall Evaluation Score for the Grant Application. The Grant Application is not considered further during the Grant Review Cycle.

(B) If a Grant Application is discussed by the Peer Review Panel, each Peer Review Panel member submits a score for the Grant Application based on the panel member's general impression of the Grant

Application's merit and accounting for the explicit criteria published in the Request for Applications. The submitted scores are averaged together to produce the final Overall Evaluation Score for the Grant Application.

(i) The panel chairperson participates in the discussion but does not score Grant Applications.

(ii) A Primary Reviewer has the option to revise his or her score for the Grant Application after panel discussion or to keep the same score submitted during the initial review.

(C) If the Peer Review Panel recommends changes to the Grant Award funds amount requested by the Grant Applicant or to the goals and objectives or timeline for the proposed project, then the recommended changes and explanation shall be recorded at the time the final Overall Evaluation Score is set.

(5) At the conclusion of the Peer Review Panel evaluation, the Peer Review Panel chairperson submits to the appropriate Review Council a list of Grant Applications discussed by the panel ranked in order by the final Overall Evaluation Score. Any changes to the Grant Award funding amount or to the project goals and objectives or timeline recommended by the Peer Review Panel shall be provided to the Review Council at that time.

(d) The Review Council's prioritization process for Grant Award recommendations encompasses the following actions, which will be consistently applied:

(1) The Review Council prioritizes the Grant Application recommendations across all the Peer Review Panels by assigning a Numerical Ranking Score to each Grant Application that was discussed by a Peer Review Panel. The Numerical Ranking Score is substantially based on the final Overall Evaluation Score submitted by the Peer Review Panel, but also takes into consideration how well the Grant Application achieves program priorities set by the Oversight Committee, the overall Program portfolio balance, and any other criteria described in the Request for Applications.

(2) The Review Council's recommendations are submitted simultaneously to the presiding officers of the Program Integration Committee and Oversight Committee. The recommendations, listed in order by Numerical Ranking Score shall include:

(A) An explanation describing how the Grant Application meets the Review Council's standards for Grant Award funding;

(B) The final Overall Evaluation Score assigned to the Grant Application by the Peer Review Panel, including an explanation for ranking one or more Grant Applications ahead of another Grant Application with a more favorable final Overall Evaluation Score; and

(C) The specified amount of the Grant Award funding for each Grant Application, including an explanation for recommended changes to the Grant Award funding amount or to the goals and objectives or timeline.

(3) A Grant Award recommendation is not final until the Review Council formally submits the recommendation to the presiding officers of the Program Integration Committee and the Oversight Committee. The Program Integration Committee, and, if appropriate, the Oversight Committee must

make a final decision on the Grant Award recommendation in the same state fiscal year that the Review Council submits its final recommendation.

(e) Circumstances relevant to a particular Grant Mechanism or to a Grant Review Cycle may justify changes to the dual-stage Peer Review process described in subsections (c) and (d) of this section. Peer Review process changes the Institute may implement are described in this subsection. The list is not intended to be exhaustive. Any material changes to the Peer Review process, including those listed in this subsection, shall be described in the Request for Applications or communicated to all Grant Applicants.

(1) The Institute may use a preliminary evaluation process if the volume of Grant Applications submitted pursuant to a specific Request for Applications is such that timely review may be impeded. The preliminary evaluation will be conducted after Grant Applications are assigned to Peer Review Panels but prior to the initial review described in subsection (c) of this section. The preliminary evaluation encompasses the following actions:

(A) The criteria and the specific Grant Application components used for the preliminary evaluation shall be stated in the Request for Applications;

(B) No less than two Peer Review Panel members are assigned to conduct the preliminary evaluation for a Grant Application and provide a preliminary score that conveys the general impression of the Grant Application's merit pursuant to the specified criteria; and

(C) The Peer Review Panel chairperson is responsible for determining the Grant Applications that move forward to initial review as described in subsection (c) of this section. The decision will be based upon preliminary evaluation scores. A Grant Application that does not move forward to initial review will not be considered further and the average of the preliminary evaluation scores received becomes the final Overall Evaluation Score for the Grant Application.

(2) The Institute shall assign all Grant Applications submitted for recruitment of researchers and clinicians to the Scientific Review Council.

(A) The Scientific Review Council members review all components of the Grant Application, evaluate the merits according to explicit criteria published in the Request for Applications, and, after discussion by the Review Council members, provide an individual Overall Evaluation Score that conveys the Review Council member's recommendation related to the proposed recruitment.

(B) The individual Overall Evaluation Scores are averaged together for a final Overall Evaluation Score for the Application.

(C) If more than one recruitment Grant Application is reviewed by the Scientific Review Council during the Grant Review Cycle, then the Scientific Review Council shall assign a Numerical Ranking Score to each Grant Application to convey its prioritization ranking.

(D) If the Scientific Review Council recommends a change to the Grant Award funds requested by the Grant Application, then the recommended change and explanation shall be recorded at the time the final Overall Evaluation Score is set.

(E) The Scientific Review Council's recommendations shall be provided to the presiding officer of the Program Integration Committee and to the Oversight Committee pursuant to the process described in subsection (d) of this section.

(3) The Institute may assign continuation Grant Applications to the appropriate Review Council.

(A) The Review Council members review all components of the Grant Application, evaluate the merits according to explicit criteria published in the Request for Applications, and, after discussion by the Review Council members, provide an individual Overall Evaluation Score that conveys the Review Council member's recommendation related to the progress and continued funding.

(B) The individual Overall Evaluation Scores are averaged together for a final Overall Evaluation Score for the Application.

(C) If more than one continuation Grant Application is reviewed by the Review Council during the Grant Review Cycle, then the Review Council shall assign a Numerical Ranking Score to each continuation Grant Application to convey its prioritization ranking.

(D) If the Review Council recommends a change to the Grant Award funds or to the scope of work or timeline requested by the continuation Grant Application, then the recommended change and explanation shall be recorded at the time the final Overall Evaluation Score is set.

(E) The Review Council's recommendations shall be provided to the presiding officer of the Program Integration Committee and to the Oversight Committee pursuant to the process described in subsection (d) of this section.

(4) The Institute's Peer Review process described in subsections (c) and (d) of this section may include the following additional process steps for Product Development of Cancer Research Grant Applications:

(A) A Grant Applicant may be invited to deliver an in-person presentation to the Peer Review Panel. The Product Development Review Council chairperson is responsible for deciding which Grant Applicants will make in-person presentations. The decision is based upon the initial Overall Evaluation Scores of the primary reviewers following a discussion with Peer Review Panel members, as well as explicit criteria published in the Request for Applications.

(i) Peer Review Panel members may submit questions to be addressed by the Grant Applicant at the in-person presentation.

(ii) A Grant Application that is not presented in-person will not be considered further. The average of the primary reviewers' initial Overall Evaluation Scores will be the final Overall Evaluation Score for the Grant Application.

(iii) Following the in-person presentation, each Peer Review Panel member submits a score for the Grant Application based on the panel member's general impression of the Grant Application's merit and accounting for the explicit criteria published in the Request for Applications. The submitted scores are averaged together to produce the final Overall Evaluation Score for the Grant Application.

(B) A Grant Application may undergo business operations and management due diligence review and an intellectual property review conducted by third parties. The Peer Review Panel submits a list of applications recommended for due diligence review to the Product Development Review Council. The

Product Development Review Council decides which Grant Applications submitted by the Peer Review Panel will undergo business operations and management due diligence and intellectual property review. The decision is based upon the Grant Application's final Overall Evaluation Score, but also takes into consideration how well the Grant Application achieves program priorities set by the Oversight Committee, the overall Program portfolio balance, and any other criteria described in the Request for Applications. A Grant Application that is not recommended for due diligence and intellectual property review will not be considered further.

(C) After receipt of the business operations and management due diligence and intellectual property reviews for a Grant Application, the Product Development Review Council and the Primary Reviewers meet to determine whether to recommend the Grant Application for a Grant Award based upon the information set forth in the due diligence and intellectual property reviews. The Product Development Review Council may recommend changes to the Grant Award budget and goals and objectives or timeline

(D) The Product Development Review Council assigns a Numerical Ranking Score to each Grant Application recommended for a Grant Award.

(f) Institute Employees and Oversight Committee members may attend Peer Review Panel and Review Council meetings. If an Institute Employee or an Oversight Committee member attends a Peer Review Panel meeting or a Review Council meeting, the attendance shall be recorded and the Institute Employee or Oversight Committee member shall certify in writing compliance with the Institute's Conflict of Interest rules. The Institute Employee's and Oversight Committee member's attendance at the Peer Review Panel meeting or Review Council meeting is subject to the following restrictions:

(1) Unless waived pursuant to the process described in Chapter 702, §702.17 of this title (relating to Exceptional Circumstances Requiring Participation), Institute Employees and Oversight Committee members shall not be present for any discussion, vote, or other action taken related to a Grant Applicant if the Institute Employee or Oversight Committee member has a Conflict of Interest with that Grant Applicant; and

(2) The Institute Employee or Oversight Committee member shall not participate in a discussion of the merits, vote, or other action taken related to a Grant Application, except to answer technical or administrative questions unrelated to the merits of the Grant Application and to provide input on the Institute's Grant Review Process.

(g) The Institute's Chief Compliance Officer shall observe meetings of the Peer Review Panel and Review Council where Grant Applications are discussed.

(1) The Chief Compliance Officer shall document that the Institute's Grant Review Process is consistently followed, including observance of the Institute's established Conflict of Interest rules and that participation by Institute employees, if any, is limited to providing input on the Institute's Grant Review Process and responding to committee questions unrelated to the merits of the Grant Application. Institute Program staff shall not participate in a discussion of the merits, vote, or any other action taken related to a Grant Application.

(2) The Chief Compliance Officer shall report to the Oversight Committee prior to a vote on the award recommendations specifying issues, if any, that are inconsistent with the Institute's established Grant Review Process.

(3) Nothing herein shall prevent the Institute from contracting with an independent third party to serve as a neutral observer of meetings of the Peer Review Panel and/or the Review Council where Grant Applications are discussed and to assume the reporting responsibilities of the Chief Compliance Officer described in this subsection. In the event that the independent third party observes the meeting of the Peer Review Panel and/or the Review Council, then the independent third party reviewer shall issue a report to the Chief Compliance Officer specifying issues, if any, that are inconsistent with the Institute's established Grant Review Process.

(h) Excepting a finding of an undisclosed Conflict of Interest as set forth in §703.9 of this chapter (relating to Limitation on Review of Grant Process), the Review Council's decision to not include a Grant Application on the prioritized list of Grant Applications submitted to the Program Integration Committee and the Oversight Committee is final. A Grant Application not included on the prioritized list created by the Review Council shall not be considered further during the Grant Review Cycle.

(i) At the time that the Peer Review Panel or the Review Council concludes its tasks for the Grant Review Cycle, each member shall certify in writing that the member complied with the Institute's Conflict of Interest rules. An Institute Employee or an Oversight Committee member attending one or more Peer Review Panel meetings during the Grant Review Cycle shall certify compliance with the Institute's Conflict of Interest rules.

(j) The Institute shall retain a review record for a Grant Application submitted to the Institute, even if the Grant Application did not receive a Grant Award. Such records will be retained by the Institute's electronic Grant Management System. The records retained by the Institute must include the following information:

(1) The final Overall Evaluation Score and Numerical Ranking Score, if applicable, assigned to the Grant Application;

(2) The specified amount of the Grant Award funding for the Grant Application, including an explanation for recommended changes to the Grant Award funding amount or to the goals and objectives or timeline;

(3) The Scientific Research and Prevention Programs Committee that reviewed the Grant Application;

(4) Conflicts of Interest, if any, with the Grant Application identified by a member of the Scientific Research and Prevention Programs Committee, the Review Council, the Program Integration Committee, or the Oversight Committee; and

(5) Documentation of steps taken to recuse any member or members from the Grant Review Process because of disclosed Conflicts of Interest.

(k) For purposes of this rule, a Peer Review Panel chairperson or a Review Council chairperson that is unable to carry out his or her assigned duties due to a Conflict of Interest with regard to one or more Grant Applications or for any other reason may designate a co-chairperson from among the appointed

Scientific Research and Prevention Programs committee members to fulfill the chairperson role. Such designation shall be recorded in writing and include the specific time and extent of the designation

RULE §703.10 Awarding Grants by Contract

(a) The Oversight Committee shall negotiate on behalf of the state regarding the awarding of grant funds and enter into a written contract with the Grant Recipient.

(b) The Oversight Committee may delegate Grant Contract negotiation duties to the Chief Executive Officer and the General Counsel for the Institute. The Chief Executive Officer may enter into a written contract with the Grant Recipient on behalf of the Oversight Committee.

(c) The Grant Contract shall include the following provisions:

(1) If any portion of the Grant Contract has been approved by the Oversight Committee to be used to build a capital improvement, the Grant Contract shall specify that:

(A) The state retains a lien or other interest in the capital improvement in proportion to the percentage of the Grant Award amount used to pay for the capital improvement; and

(B) If the capital improvement is sold, then the Grant Recipient agrees to repay to the state the Grant Award used to pay for the capital improvement, with interest, and share with the state a proportionate amount of any profit realized from the sale;

(2) Terms relating to Intellectual Property Rights and the sharing with the Institute of revenues generated by the sale, license, or other conveyance of such Project Results consistent with the standards established by this chapter;

(3) Terms relating to publication of materials created with Grant Award funds or related to the Cancer Research or Cancer Prevention project that is the subject of the Grant Award, including an acknowledgement of Institute funding and copyright ownership, if applicable;

(4) Repayment terms, including interest rates, to be enforced if the Grant Recipient has not used Grant Award funds for the purposes for which the Grant Award was intended;

(5) A statement that the Institute does not assume responsibility for the conduct of the Cancer Research or Cancer Prevention project, and that the conduct of the project and activities of all investigators are under the scope and direction of the Grant Recipient;

(6) A statement that the Cancer Research or Cancer Prevention project is conducted with full consideration for the ethical and medical implications of the project and that the project will comply with all federal and state laws regarding the conduct of the Cancer Research or Prevention project;

(7) Terms related to the Standards established by the Oversight Committee in Chapter 701 of this title (relating to Policies and Procedures) to ensure that Grant Recipients, to the extent reasonably possible, demonstrate good faith effort to purchase goods and services for the Grant Award project from suppliers in this state and from historically underutilized businesses as defined by Chapter 2161, Texas Government Code, and any other state law;

(8) An agreement by the Grant Recipient to submit to regular inspection reviews of the Grant Award project by Institute staff during normal business hours and upon reasonable notice to ensure compliance with the terms of the Grant Contract and continued merit of the project;

(9) An agreement by the Grant Recipient to submit Grant Progress Reports to the Institute on a schedule specified by the Grant Contract that include information on a grant-by-grant basis quantifying the amount of additional research funding, if any, secured as a result of Institute funding;

(10) An agreement that, to the extent possible, the Grant Recipient will evaluate whether any new or expanded preclinical testing, clinical trials, Product Development, or manufacturing of any real or intellectual property resulting from the award can be conducted in this state, including the establishment of facilities to meet this purpose;

(11) An agreement that the Grant Recipient will abide by the Uniform Grant Management Standards (UGMS) adopted by the Governor's Office, if applicable unless one or more standards conflicts with a provision of the Grant Contract, Chapter 102, Texas Health and Safety Code, or the Institute's administrative rules. Such interpretation of the Institute rules and UGMS shall be made by the Institute;

(12) An agreement that the Grant Recipient is under a continuing obligation to notify the Institute of any adverse conditions that materially impact milestones and objectives included in the Grant Contract;

(13) An agreement that the design, conduct, and reporting of the Cancer Research or Prevention project will not be biased by conflicting financial interest of the Grant Recipient or any individuals associated with the Grant Award. This duty is fulfilled by certifying that an appropriate written, enforced Conflict of Interest policy governs the Grant Recipient.

(14) An agreement regarding the amount, schedule, and requirements for payment of Grant Award funds, if such advance payments are approved by the Oversight Committee in accordance with this chapter. Notwithstanding the foregoing, the Institute may require that up to ten percent of the final tranche of funds approved for the Grant Award must be expended on a reimbursement basis. Such reimbursement payment shall not be made until close out documents described in this section and required by the Grant Contract have been submitted and approved by the Institute;

(15) An agreement to provide quarterly Financial Status Reports and supporting documentation for expenses submitted for reimbursement or, if appropriate, to demonstrate how advanced funds were expended;

(16) A statement certifying that, as of June 14, 2013, the Grant Recipient has not made and will not make a contribution, during the term of the Grant Contract, to the Institute or to any foundation established specifically to support the Institute;

(17) A statement specifying the agreed effective date of the Grant Contract and the period in which the Grant Award funds must be spent. If the effective date specified in the Grant Contract is different from the date the Grant Contract is signed by both parties, then the effective date shall control;

(18) A statement providing for reimbursement with Grant Award funds of expenses made prior to the effective date of the Grant Contract at the discretion of the Institute. Pre-contract reimbursement shall be made only in the event that:

(A) The expenses are allowable pursuant to the terms of the Grant Contract;

(B) The request is made in writing by the Grant Recipient and approved by the Chief Executive Officer; and

(C) The expenses to be reimbursed were incurred on or after the date the Grant Award recommendation was approved by the Oversight Committee.

(19) Requirements for closing out the Grant Contract at the termination date, including the submission of a Financial Status Report, a final Grant Progress Report, a equipment inventory, a HUB and Texas Business report, a revenue sharing form, a single audit determination report form and a list of contractual terms that extend beyond the termination date;

(20) A certification of dedicated Matching Funds equal to one-half of the amount of the Research Grant Award that includes the name of the Research Grant Award to which the matching funds are to be dedicated, as specified in Section §703.11 of this chapter (relating to Requirement to Demonstrate Available Funds for Cancer Research Grants);

(21) The project deliverables as described by the Grant Application and stated in the Scope of Work for the Grant Contract reflecting modifications, if any, approved during the Peer Review process or during Grant Contract negotiation; and

(22) An agreement that the Grant Recipient shall notify the Institute and seek approval for a change in effort for any of the Senior Members or Key Personnel of the research or prevention team listed on the Grant Application.

(23) An agreement that the Grant Recipient is legally responsible for the integrity of the fiscal and programmatic management of the organization.

(24) An agreement that the Grant Recipient is responsible for the actions of its employees and other research collaborators, including third parties, involved in the project. The Grant Recipient is responsible for enforcing its standards of conduct, taking appropriate action on individual infractions, and, in the case of financial conflict of interest, informing the Institute if the infraction is related to a Grant Award.

(25) The certification date of approval by the Institutional Animal Care and Use Committee (IAUCUC) and/or the Institutional Review Board (IRB), as appropriate, if vertebrate animals and/or human subjects are included in the proposed research. Such certification, if applicable, is required before funding can occur. The Grant Recipient must provide annual recertification to the Institute.

(d) The Grant Recipient's failure to comply with the terms and conditions of the Grant Contract may result in termination of the Grant Contract pursuant to the process prescribed in the Grant Contract and trigger repayment of the Grant Award funds.

RULE §703.11 Requirement to Demonstrate Available Funds for Cancer Research Grants

(a) Prior to the disbursement of Grant Award funds, the Grant Recipient of a Cancer Research Grant Award shall demonstrate that the Grant Recipient has an amount of Encumbered Funds equal to at least one-half of the Grant Award available and not yet expended that are dedicated to the research that is the subject of the Grant Award.

(1) The Grant Recipient's written certification of Matching Funds, as described in this section, shall be included in the Grant Contract.

(2) A Grant Recipient of a multiyear Grant Award may certify Matching Funds on a year-by-year basis for the amount of Award Funds to be distributed for the Project Year based upon the Approved Budget.

(3) A Grant Recipient receiving multiple Grant Awards may provide certification at the institutional level.

(4) Nothing herein restricts the Institute from requiring the Grant Recipient to demonstrate an amount of Encumbered Funds greater than one-half of the Grant Award available and not yet expended that are dedicated to the research that is the subject of the Grant Award. To the extent that a greater Matching Funds amount will be required, the Institute shall include the requirement in the Request for Applications and in the Grant Contract.

(b) For purposes of the certification required by subsection (a) of this section, a Grant Recipient that is a public or private institution of higher education, as defined by §61.003, Texas Education Code, may credit toward the Grant Recipient's Matching Funds obligation the dollar amount equivalent to the difference between the indirect cost rate authorized by the federal government for research grants awarded to the Grant Recipient and the five percent (5%) Indirect Cost limit imposed by §102.203(c), Texas Health and Safety Code, subject to the following requirements:

(1) The Grant Recipient shall file certification with the Institute documenting the federal indirect cost rate authorized for research grants awarded to the Grant Recipient;

(2) To the extent that the Grant Recipient's Matching Funds credit does not equal or exceed one-half of the Grant Award funds to be distributed for the Project Year, then the Grant Recipient's Matching Funds certification shall demonstrate that a combination of the dollar amount equivalent credit and the funds to be dedicated to the Grant Award project as described in subsection (c) of this section is available and sufficient to meet or exceed the Matching Fund requirement;

(3) Calculation of the portion of federal indirect cost rate credit associated with subcontracted work performed for the Grant Recipient shall be in accordance with the Grant Recipient's established internal policy; and

(4) If the Grant Recipient's federal indirect cost rate changes less than six months following the anniversary of the Effective Date of the Grant Contract, then the Grant Recipient may use the new federal indirect cost rate for the purpose of calculating the Grant Recipient's Matching Funds credit for the entirety of the Project Year.

(c) For purposes of the certification required by subsection (a) of this section, Encumbered Funds must be spent directly on the Grant Project or spent on closely related work that supports, extends, or facilitates the Grant Project and may include:

(1) Federal funds, including, but not limited to, American Recovery and Reinvestment Act of 2009 funds, and the fair market value of drug development support provided to the recipient by the National Cancer Institute or other similar programs;

(2) State of Texas funds;

(3) funds of other states;

(4) Non-governmental funds, including private funds, foundation grants, gifts and donations;

(5) Unrecovered Indirect Costs not to exceed ten percent (10%) of the Grant Award amount, subject to the following conditions:

(A) These costs are not otherwise charged against the Grant Award as the five percent (5%) indirect funds amount allowed under §703.12(c) of this chapter (relating to Limitation on Use of Funds);

(B) The Grant Recipient must have a documented federal indirect cost rate or an indirect cost rate certified by an independent accounting firm; and

(C) The Grant Recipient is not a public or private institution of higher education as defined by §61.003 of the Texas Education Code.

(6) Funds contributed by a subcontractor or subawardee and spent on the Grant Project, so long as the subcontractor's or subawardee's portion of otherwise allowable Matching Funds for a Project Year may not exceed the percentage of the total Grant Funds paid to the subcontractor or subawardee for the same Project Year.

(d) For purposes of the certification required by subsection (a) of this section, the following items do not qualify as Encumbered Funds:

(1) In-kind costs;

(2) Volunteer services furnished to the Grant Recipient;

(3) Noncash contributions;

(4) Income earned by the Grant Recipient that is not available at the time of Grant Award;

(5) Pre-existing real estate of the Grant Recipient including building, facilities and land;

(6) Deferred giving such as a charitable remainder annuity trust, a charitable remainder unitrust, or a pooled income fund; or

(7) Other items as may be determined by the Oversight Committee.

(e) To the extent that a Grant Recipient of a multiyear Grant Award elects to certify Matching Funds on a Project Year basis, the failure to provide certification of Encumbered Funds at the appropriate time for

each Project Year may serve as grounds for suspending reimbursement or advancement of Grant Funds for project costs or terminating the Grant Contract.

(f) In no event shall Grant Award funds for a Project Year be advanced or reimbursed, as may be appropriate for the Grant Award and specified in the Grant Contract, until the certification required by subsection (a) of this section is filed and approved by the Institute.

(g) No later than 30 days following the due date of the FSR reflecting expenses incurred during the last quarter of the Grant Recipient's Project Year, the Grant Recipient shall file a form with the Institute reporting the amount of Matching Funds spent for the preceding Project Year.

(h) If the Grant Recipient failed to expend Matching Funds equal to one-half of the actual amount of Grant Award funds distributed to the Grant Recipient for the same Project Year the Institute shall:

(1) Carry forward and add to the Matching Fund requirement for the next Project Year the dollar amount equal to the deficiency between the actual amount of Grant Award funds distributed and the actual Matching Funds expended, so long as the deficiency is equal to or less than twenty percent (20%) of the total Matching Funds required for the same period and the Grant Recipient has not previously had a Matching Funds deficiency for the project;

(2) Suspend distributing Grant Award funds for the project to the Grant Recipient if the deficiency between the actual amount of Grant Funds distributed and the Matching Funds expended is greater than twenty percent (20%) but less than fifty percent (50%) of the total Matching Funds required for the period.

(A) The Grant Recipient will have no less than eight months from the anniversary of the Grant Contract's effective date to demonstrate that it has expended Encumbered Funds sufficient to fulfill the Matching Funds deficiency for the project.

(B) If the Grant Recipient fails to fulfill the Matching Funds deficiency within the specified period, then the Grant Contract shall be considered in default and the Institute may proceed with terminating the Grant Award pursuant to the process established in the Grant Contract;

(3) Declare the Grant Contract in default if the deficiency between the actual amount of Grant Award funds distributed and the Matching Funds expended is greater than fifty percent (50%) of the total Matching Funds required for the period. The Institute may proceed with terminating the Grant Award pursuant to the process established in the Grant Contract; or

(4) Take appropriate action, including withholding reimbursement, requiring repayment of the deficiency, or terminating the Grant Contract if a deficiency exists between the actual amount of Grant Award funds distributed and the Matching Funds expended and it is the last year of the Grant Contract;

(i) Nothing herein shall preclude the Institute from taking action other than described in subsection (h) of this section based upon the specific reasons for the deficiency. To the extent that other action not described herein is taken by the Institute, such action shall be documented in writing and included in Grant Contract records. The options described in subsection (h)(1) and (2) of this section may be used by the Grant Recipient only one time for the particular project. A second deficiency of any amount shall be considered an event of default and the Institute may proceed with terminating the Grant Award pursuant to the process established in the Grant Contract.

(j) The Grant Recipient shall maintain adequate documentation supporting the source and use of the Matching Funds reported in the certification required by subsection (a) of this section. The Institute shall conduct an annual review of the documentation supporting the source and use of Matching Funds reported in the required certification for a risk-identified sample of Grant Recipients. Based upon the results of the sample, the Institute may elect to expand the review of supporting documentation to other Grant Recipients. Nothing herein restricts the authority of the Institute to review supporting documentation for one or more Grant Recipients or to conduct a review of Matching Funds documentation more frequently 703.12. Limitation on Use of Funds

703.24 Financial Status Reports

(a) Grant Recipients shall report expenditures to be reimbursed with Grant Award funds on the quarterly Financial Status Report form.

(1) Expenditures shall be reported by budget category consistent with the Grant Recipient's Approved Budget.

(2) All expenditures must be supported with appropriate documentation showing that the costs were incurred and paid. A Grant Recipient that is a public or private institution of higher education as defined by §61.003, Texas Education Code is not required to submit supporting documentation for an individual expense totaling less than $750 in the "supplies" or "other" budget categories.

(3) The Financial Status Report and supporting documentation must be submitted via the Grant Management System, unless the Grant Recipient is specifically directed in writing by the Institute to submit or provide it in another manner.

(4) The Institute may request in writing that a Grant Recipient provide more information or correct a deficiency in the supporting documentation for a Financial Status Report. If a Grant Recipient does not submit the requested information within 21 days after the request is submitted, the Financial Status Report will be disapproved by the Institute.

(A) Nothing herein restricts the Institute from disapproving the FSR without asking for additional information or prior to the submission of additional information.

(B) Nothing herein extends the FSR due date.

(45) The requirement to report and timely submit quarterly Financial Status Reports applies to all Grant Recipients, regardless of whether Grant Award funds are disbursed by reimbursement or in advance of incurring costs.

(b) Quarterly Financial Status Reports shall be submitted to the Institute within 90 days of the end of the state fiscal quarter (based upon a September 1 - August 31 fiscal year). The Institute shall review expenditures and supporting documents to determine whether expenses charged to the Grant Award are:

(1) Allowable, allocable, reasonable, necessary, and consistently applied regardless of the source of funds; and

(2) Adequately supported with documentation such as cost reports, receipts, third party invoices for expenses, or payroll information.

(c) Except as provided herein, the Grant Recipient waives the right to reimbursement of project costs incurred during the reporting period if the Financial Status Report for that quarter is not submitted to the Institute within 30 days of the Financial Status Report due date. Waiver of reimbursement of project costs incurred during the reporting period also applies to Grant Recipients that have received advancement of Grant Award funds.

(1) For purposes of this rule, the "Financial Status Report due date" is 90 days following the end of the state fiscal quarter.

(2) The Chief Executive Officer may approve a Grant Recipient's request to defer submission of the reimbursement request for the current fiscal quarter until the next fiscal quarter if, on or before the original Financial Status Report due date, the Grant Recipient submits a written explanation for the Grant Recipient's inability to complete a timely submission of the Financial Status Report.

(3) A Grant Recipient may appeal the waiver of its right to reimbursement of project costs.

(A) The appeal shall be in writing, provide good cause for failing to submit the Financial Status Report within 30 days of the Financial Status Report due date, and be submitted via the Grant Management System.

(B) The Chief Executive Officer may approve the appeal for good cause. The decision by the Chief Executive Officer to approve or deny the grant recipient's appeal shall be in writing and available to the Grant Recipient via the Grant Management System.

(C) The Chief Executive Officer's decision to approve or deny the Grant Recipient's appeal is final, unless the Grant Recipient timely seeks reconsideration of the Chief Executive Officer's decision by the Oversight Committee.

(D) The Grant Recipient may request that the Oversight Committee reconsider the Chief Executive Officer's decision regarding the Grant Recipient's appeal. The request for reconsideration shall be in writing and submitted to the Chief Executive Officer within 10 days of the date that the Chief Executive Officer notifies the Grant Recipient of the decision regarding the appeal as noted in subparagraph (C) of this paragraph.

(E) The Chief Executive Officer shall notify the Oversight Committee in writing of the decision to approve or deny the Grant Recipient's appeal. The notice should provide justification for the Chief Executive Officer's decision. In the event that the Grant Recipient timely seeks reconsideration of the Chief Executive Officer's decision, the Chief Executive Officer shall provide the Grant Recipient's written request to the Oversight Committee at the same time.

(F) The Grant Recipient's request for reconsideration is deemed denied unless three or more Oversight Committee members request that the Chief Executive Officer add the Grant Recipient's request for reconsideration to the agenda for action at the next regular Oversight Committee meeting. The decision made by the Oversight Committee is final.

(G) If the Grant Recipient's appeal is approved by the Chief Executive Officer or the Oversight Committee, the Grant Recipient shall report the project costs and provide supporting documentation for the costs incurred during the reporting period covered by the appeal on the next available financial status report to be filed by the Grant Recipient.

(H) Approval of the waiver appeal does not connote approval of the expenditures; the expenditures and supporting documentation shall be reviewed according to subsection (b) of this section.

(I) This subsection applies to any waivers of the Grant Recipient's reimbursement decided by the Institute on or after September 1, 2015.

(4) Notwithstanding subsection (c) of this section, in the event that the Grant Recipient and Institute execute the Grant Contract after the effective date of the Grant Contract, the Chief Program Officer may approve additional time for the Grant Recipient to prepare and submit the outstanding Financial Status Report(s). The approval shall be in writing and maintained in the Grants Management System. The Chief Program Officer's approval may cover more than one Financial Status Report and more than one fiscal quarter.

(5) In order to receive disbursement of grant funds, the most recently due Financial Status Report must be approved by the Institute.

CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

MEMORANDUM

To:	**OVERSIGHT COMMITTEE MEMBERS**
From:	**HEIDI MCCONNELL, CHIEF OPERATING OFFICER**
Subject:	**CHIEF OPERATING OFFICER REPORT**
Date:	**FEBRUARY 6, 2017**

CPRIT Financial Overview for FY 2017, Quarter 1

FY 2017, Quarter 1 Operating Budget

CPRIT expended or obligated approximately $600,000 in Indirect Administration during the year. The agency has also expended or obligated $9.1 million in Grant Review and Award Operations. The obligations reflected in the Professional Fees and Services category are primarily for service contracts for pre- and post-award grants management support services, legal due diligence services, business and regulatory due diligence services, and compliance and audit services.

During this quarter, the agency collected $15,862 in revenue sharing payments.

FY 2017, Quarter 1 Performance Measure Report

CPRIT reported on its two key quarterly performance measures to the Legislative Budget Board. It met or exceeded the prevention measure but did not meet performance on the product development measure on company relocations to Texas because no company recipients of a CPRIT grant award relocated to Texas during the reporting period from September through November 2016.

Debt Issuance History

CPRIT has issued $116.9 million in commercial paper notes since the beginning of FY 2017 through the Texas Public Finance Authority (TPFA). These debt issuances include approximately $7.4 million for agency operating costs and $1.5 million for the transfer to the Department of State Health Services for Texas Cancer Registry operations. The remaining $108 million has been and is being used to make reimbursement payments due to grant recipients for award expenses.

Financial Audit for the Year Ending August 31, 2016

The 2016 financial audit performed by McConnell & Jones, LLP was completed on December 5, 2016, with no audit findings. The audit was reviewed with the Audit Subcommittee at a specially called meeting on December 13, 2016. The report was submitted to the Comptroller of Public Accounts and the State Auditor's Office.

Cancer Prevention and Research Institute of Texas
Quarterly Financial Report
As of November 30, 2016

Indirect Administration (B.1.1.)

		2017 Appropriated	2017 Budgeted	% of Total Budget	Actual Expenditures & Grant Encumbrances (FYTD)	Remaining Budget	Percent Expended	Estimated Expenditures (YTD)	Lapse/Overspent
1001	Salaries and Wages	$ 1,432,617	$ 1,417,617		$ 305,915	1,111,702	22%	$ 305,915	$ 1,111,702
1002	Other Personnel Costs	52,785	67,785		25,823	41,962	38%	25,823	41,962
2001	Professional Fees and Services	807,317	807,317		158,554	648,763	20%	158,554	648,763
2003	Consumable Supplies	27,584	27,584		3,747	23,837	14%	3,747	23,837
2004	Utilities	58,577	58,577		536	58,041	1%	536	58,041
2005	Travel	45,000	45,000		8,292	36,708	18%	8,292	36,708
2006	Rent-Building	-	18,408		18,408	0	0%	18,408	0
2007	Rent-Machine and Other	32,172	32,172		4,568	27,604	14%	4,568	27,604
2009	Other Operating Expenses	574,600	556,192		102,031	454,161	18%	102,031	454,161
	Subtotal - Indirect Administration (B.1.1.)	**$ 3,030,652**	**$ 3,030,652**	**1.02%**	**$ 627,874**	**$ 2,402,778**	**21%**	**$ 627,874**	**$ 2,402,778**

Grant Review and Award Operations (A.1.3.)

		2017 Appropriated	2017 Budgeted	% of Total Budget	Actual Expenditures & Grant Encumbrances (FYTD)	Remaining Budget	Percent Expended	Estimated Expenditures (YTD)	Lapse/Overspent
1001	Salaries and Wages	$ 2,730,580	2,681,233		$ 750,198	$ 1,931,035	28%	$ 750,198	$ 1,931,035
1002	Other Personnel Costs	3,856	53,203		58,285	(5,083)	0%	58,285	(5,083)
2001	Professional Fees and Services	10,809,493	10,809,493		8,272,659	2,536,834	77%	8,272,659	2,536,834
2003	Consumable Supplies	-	-		-	-	0%	-	-
2004	Utilities		10,000		824	9,176	8%	824	9,176
2005	Travel	65,000	65,000		15,501	49,499	24%	15,501	49,499
2009	Other Operating Expenses	201,297	191,297		37,909	153,388	20%	37,909	153,388
	Conference		-		-	-	#DIV/0!	-	-
	Subtotal - Grant Operations (A.1.3.)	**$ 13,810,226**	**$ 13,810,226**	**4.65%**	**$ 9,135,377**	**$ 4,674,849**	**66%**	**$ 9,135,377**	**$ 4,674,849**

Grants

		2017 Appropriated	2017 Budgeted	% of Total Budget	Actual Expenditures & Grant Encumbrances (FYTD)	Remaining Budget	Percent Expended	Estimated Expenditures (YTD)	Lapse/Overspent
4000	Grants - Prevention (A.1.2)	$ 28,334,312	$ 28,334,312		$ -	$ 28,334,312	0%	$ -	$ 28,334,312
4000	Grants - Research (A.1.1.)	251,780,562	$ 251,780,562		-	$ 251,780,562	0%	-	251,780,562
	Subtotal - Grants	**$ 280,114,874**	**$ 280,114,874**	**94.33%**	**$ -**	**$ 280,114,874**	**0%**	**$ -**	**$ 280,114,874**
	Grand Totals	**$ 296,955,752**	**$ 296,955,752**	**100.00%**	**$ 9,763,250**	**$ 287,192,502**	**3%**	**$ 9,763,250**	**$ 287,192,502**

Cancer Prevention and Research Institute of Texas
Cancer Prevention and Research Institute Fund Account - 5136
As of November 30, 2016

	11/01/2016 thru 11/30/2016	AY 17 Year to Date as of 11/30/2016
Beginning Balance : 11/01/2016		**$ 600,506**
Increases:		
(1)	$ -	$ -
(2)	-	
Total Increases	**$ -**	**$ 600,506.00**
Reductions:		
Expenditures - Appropriated	$ -	$ -
	$ -	$ -
	$ -	$ -
Total Reductions	**$ -**	**$ -**
Ending Balance, 11/30/2016		**$ 600,506.00**

Note: (1) The Institute received a settlement from the Texas Cancer Coalition (TCC). This amount represents the final distribution and transfer of all funds ($303,877) from the TCC which ceased operations in May 2013. These funds are in the State Treasury but are not appropriated to CPRIT. The beginning balance reflects the transfer of all TCC funds.

Cancer Prevention and Research Institute of Texas

License Plate Trust Fund Account - 0802

As of November 30, 2016

	11/01/2016 thru 11/30/2016	AY 17 Year to Date as of 11/30/2016
Beginning Balance : 11/01/2016		**$ -**
Increases:		
(1) License Plate Revenue Received	$ 617.82	$ 2,465.78
Total Increases	**$ 617.82**	**$ 2,465.78**
Reductions:		
Expenditures - Appropriated	$ -	$ -
	-	-
	-	-
Total Reductions	**$ -**	**$ -**
Ending Balance, 11/30/2016		**$ 2,465.78**

Note:

Cancer Prevention and Research Institute of Texas

Appropriated Receipts - 666

As of November 30, 2016

		11/01/2016 thru 11/30/2016	AY 17 Year to Date as of 11/30/2016
Beginning Balance : 11/01/2016			**$ 96,416.49**
Increases:			
(1)	Product Development Application Fees Received	$ -	$ -
(2)	Appropriated Receipts applied to payments	$ -	$ -
(3)	Conference Registration Fees	$ -	$ -
(4)	Conference Registration Fees-Credit Card	$ -	$ -
Total Increases		**$ -**	**$ -**
Reductions:			
	Conference Expenditures - Appropriated	$ -	$ -
	Credit Card Fees Expended		$ -
		$ -	$ -
Total Reductions		**$ -**	**$ -**
Ending Balance, 11/30/2016			**$ 96,416.49**

Begin balance is $76,000 for application fees and $20,416.49 for conference fees

Cancer Prevention and Research Institute of Texas

General Revenue Fund Account - 0001

As of November 30, 2016

		11/01/2016 thru 11/30/2016	AY 17 Year to Date as of 11/30/2016
Beginning Balance : 11/01/2016			$ -
Increases:			
(1)	Revenue Sharing / Royalties	$ 11,861.81	$ 15,861.81
Total Increases		**$ 11,861.81**	**$ 15,861.81**
Reductions:			
	Expenditures - Appropriated	$ -	$ -
	Sweep Account	$ (11,861.81)	$ (15,861.81)
		$ -	$ -
Total Reductions		**$ (11,861.81)**	**$ (15,861.81)**
Ending Balance, 11/30/2016			**$ -**

Note:

Cancer Prevention and Research Institute of Texas
FY 2017, Quarter 1 Performance Measure Report

Measure	Targeted Performance	QTR 1	QTR 2	QTR 3	QTR 4	Sum of QTRs	% of Mandate Attained
Number of People Served by Institute Funded Prevention and Control Activities	800,000	175,441				175,441	14.26%
Number of Entities Relocating to TX for Cancer Research Related Projects	2.00	0.00				0.00	0.00%
Annual Age-adjusted Cancer Mortality Rate	152.5	N/A	N/A	N/A	N/A		0.00%
Number of Published Articles on CPRIT-Funded Research Projects	450	N/A	N/A	N/A	N/A		0.00%
Number of New Jobs Created and Maintained	315	N/A	N/A	N/A	N/A		0.00%

Variance Explanations

Number of Entities Relocating to TX for Cancer Research Related Projects
This output is dependent on the number of companies applying for CPRIT Company Relocation Awards that can successfully advance through CPRIT's rigorous review and evaluation process, receive an award and actually relocate operations to Texas.

CPRIT Commercial Paper and G.O. Bond Issuance

Fiscal Year	Amount Appropriated	Dated Issued	Amount Issued	Amount Issued for Fiscal Year	Commercial Paper or GO Bond Issuance	Series	Comments	Interest Rate
2010	$ 225,000,000	September 9, 2009	$ 9,100,000		Commercial Paper Notes	Series A, Taxable		
2010		September 9, 2009	$ 3,600,000		Commercial Paper Notes	Series B, Tax-Exempt	Defeased with cash July 2011	
2010		March 12, 2010	$ 63,800,000		Commercial Paper Notes	Series A, Taxable		
2010		August 26, 2010	$ 148,500,000		Commercial Paper Notes	Series A, Taxable		
				$ 225,000,000				
2011	$ 225,000,000	September 7, 2010	$ 11,800,000		Commercial Paper Notes	Series A, Taxable		
2011		August 10, 2011	$ 50,775,000		G.O. Bonds	Taxable Series 2011	Par amount of new money	Fixed Rate Bonds All-In-True Interest Cost 4.0144%
2011		August 10, 2011	$ 232,045,000		G.O. Bonds (Refunding Bonds)	Taxable Series 2011	Par amount of refunding; Refunded $233.2M of GOCP CPRIT Series A (9/9/09, 3/12/09, 8/26/09, 9/7/10)	Fixed Rate Bonds All-In-True Interest Cost 4.0144%
				$ 62,575,000				
2012	$ 300,000,000	September 7, 2011	$ 3,200,000		Commercial Paper Notes	Series A, Taxable		
2012		December 8, 2011	$ 3,200,000		Commercial Paper Notes	Series A, Taxable		
2012		March 2, 2012	$ 12,300,000		Commercial Paper Notes	Series A, Taxable		
2012		June 21, 2012	$ 15,000,000		Commercial Paper Notes	Series A, Taxable		
2012		August 16, 2012	$ 42,000,000		Commercial Paper Notes	Series A, Taxable		
				$ 75,700,000				
2013	$ 300,000,000	September 6, 2012	$ 9,600,000		Commercial Paper Notes	Series A, Taxable		
2013		May 16,2013	$ 13,400,000		Commercial Paper Notes	Series A, Taxable		
				$ 23,000,000				
2014	$ 300,000,000	November 25, 2013	$ 55,200,000		Commercial Paper Notes	Series A, Taxable		
2014		March 13, 2014	$ 47,000,000		Commercial Paper Notes	Series A, Taxable		
2014		June 17, 2014	$ 60,300,000		Commercial Paper Notes	Series A, Taxable		
2014		July 8, 2014	$ 233,280,000		G.O. Bonds (Refunding Bonds)	Taxable Series 2014	Par amount of refunding; Refunded $237.88M of GOCP CPRIT Series A	Fixed Rate Bonds All-In-True Interest Cost 3.327184%
				$ 162,500,000				
2015	$ 300,000,000	November 5, 2014	$ 57,600,000		Commercial Paper Notes	Series A, Taxable		
2015		April 29, 2014	$ 112,000,000		Commercial Paper Notes	Series A, Taxable		
2015		June 26, 2015	$ 75,000,000		Commercial Paper Notes	Series A, Taxable		
				$ 244,600,000				

CPRIT Commercial Paper and G.O. Bond Issuance

Fiscal Year	Amount Appropriated	Dated Issued	Amount Issued	Amount Issued for Fiscal Year	Commercial Paper or GO Bond Issuance	Series	Comments	Interest Rate
2016	$ 300,000,000	September 22, 2015	$ 55,400,000		Commercial Paper Notes	Series A, Taxable		
2016		October 29, 2015	$ 300,000,000		G.O. Bonds (Refunding Bonds)	Taxable Series 2015C	Par amount of refunding; Refunded $300M of GOCP CPRIT Series A	Fixed Rate Bonds All-In-True Interest Cost 3.299867%
2016		October 29, 2015	$ 69,800,000		G.O. Bonds	Taxable Series 2015C	Disbursed to CPRIT January 2016	Fixed Rate Bonds All-In-True Interest Cost 3.299867%
2016		May 16, 2016	$ 92,100,000		Commercial Paper Notes	Series A, Taxable		
2016		August 29, 2016	$ 60,000,000		Commercial Paper Notes	Series A, Taxable		
				$ 277,300,000				
2017	$300,000,000	October 19, 2016	$ 58,000,000		Commercial Paper Notes	Series A, Taxable		
		January 5, 2017	$ 58,900,000		Commercial Paper Notes	Series A, Taxable		
				$ 116,900,000				
TOTAL ISSUED TO DATE				**$ 1,187,575,000**				

Oversight Committee Meetings and Standing Subcommittees Meetings 2018

November 2017

Sunday	Monday	Tuesday	Wednesday	Thursday	Friday	Saturday
10/29	10/30	10/31 PIC Meeting CPRIT Staff Only	1 Portal Opens	2 Board Governance	3	4
5	6 Audit	7 Prevention	8 Academic Scientific Research	9 Product Development	10 Nominations	11
12	13	14	**15 Oversight Committee Meeting**	16	17	18

February 2018

Sunday	Monday	Tuesday	Wednesday	Thursday	Friday	Saturday
4	5	6 PIC Meeting CPRIT Staff Only	7 Portal Opens	8 Board Governance	9	10
11	12 Audit	13 Prevention	14 Academic Scientific Research	15 Product Development	16 Nominations	17
18	19	20	**21 Oversight Committee Meeting**	22	23	24

May 2018

Sunday	Monday	Tuesday	Wednesday	Thursday	Friday	Saturday
4/29	4/30	1 PIC Meeting CPRIT Staff Only	2 Portal Opens	3 Board Governance	4	5
6	7 Audit	8 Prevention	9 Academic Scientific Research	10 Product Development	11 Nominations	12
13	14	15	**16 Oversight Committee Meeting**	17	18	19

August 2018

Sunday	Monday	Tuesday	Wednesday	Thursday	Friday	Saturday
7/29	7/30	7/31 PIC Meeting CPRIT Staff Only	1 Portal Opens	2 Board Governance	3	4
5	6 Audit	7 Prevention	8 Academic Scientific Research	9 Product Development	10 Nominations	11
12	13	14	**15 Oversight Committee Meeting**	16	17	18

Note: Unless the subcommittee members agree to a different time, all subcommittee meetings will begin at 10:00 a.m. with the exception of Diversity and Nominations that will begin at 10:30 a.m. Members of the Audit and Program subcommittees should allocate 1.5 hours for a meeting. All others subcommittee meetings require one hour.