



CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS

Advisory Committee on Childhood Cancer

Committee Annual Report

February 15, 2023

Presented By: Richard Gorlick, MD

Chair, ACCC

Division Head and Chair, Pediatrics, MD Anderson

H. Grant Taylor, M.D., W.W. Sutow, M.D., and Margaret P. Sullivan, M.D. Distinguished Chair in Pediatrics

Department Chair *ad interim*, Sarcoma Medical Oncology, Division of Cancer Medicine



THE UNIVERSITY OF TEXAS

MD Anderson ~~Cancer~~ Center
Children's Cancer Hospital®

Outline of Presentation

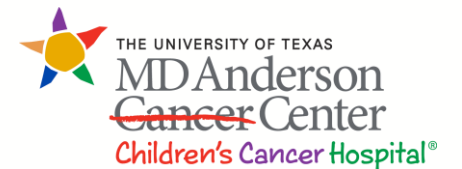
- **Childhood Cancer Summary**
- **Summary of CPRIT Pediatric Achievements**
- **ACCC Recent Steps**
 - Membership/Researchers Roundup
- **ACCC Strategy**
 - Short term goals
 - Vision for future
- **Summary/Next Steps**



Childhood Cancer



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Childhood Cancer: Statement of the Problem

- **Leading cause of childhood death from disease***
 - 14,000 new cases per year in US
 - 1 in every 330 Americans develops cancer before age 20
- **Childhood cancer survivors represent a growing “problem”**
 - 1 in 750 20-year-olds alive in the US today is a survivor of childhood cancer
- **More effective treatments are still needed**
 - Cure rates of patients with many types of malignancies remain poor including metastatic sarcomas, high risk neuroblastoma, acute myeloid leukemia and brain stem gliomas
 - The prognosis of these malignancies has shown limited to no improvement in the last few decades



Adolescent & Young Adult Cancer (AYA)

- 90,000 AYAs (15-39 yrs) diagnosed with cancer each year in the US
 - 10% of AYAs with cancer are diagnosed and treated in Texas
- Prevalence of cancer subtypes differ between AYAs, older adults, and children
 - Biology of childhood, AYA and adult cancers differs for many malignancies
- >100,000 childhood and AYA survivors live in Texas
- 5-yr Overall Survival - 85%, however, limited improvement in cure rates for many AYA diagnoses (sarcoma, CNS tumors, early onset CRC, breast cancer)
- Few studies focused on AYA short and long-term survival and quality of life
- TX is uniquely positioned to be leader in AYA cancer research

<https://seer.cancer.gov/statfacts/html/aya.html>

Smith et al. JCO 2010

Isakoff et al. JCO 2015

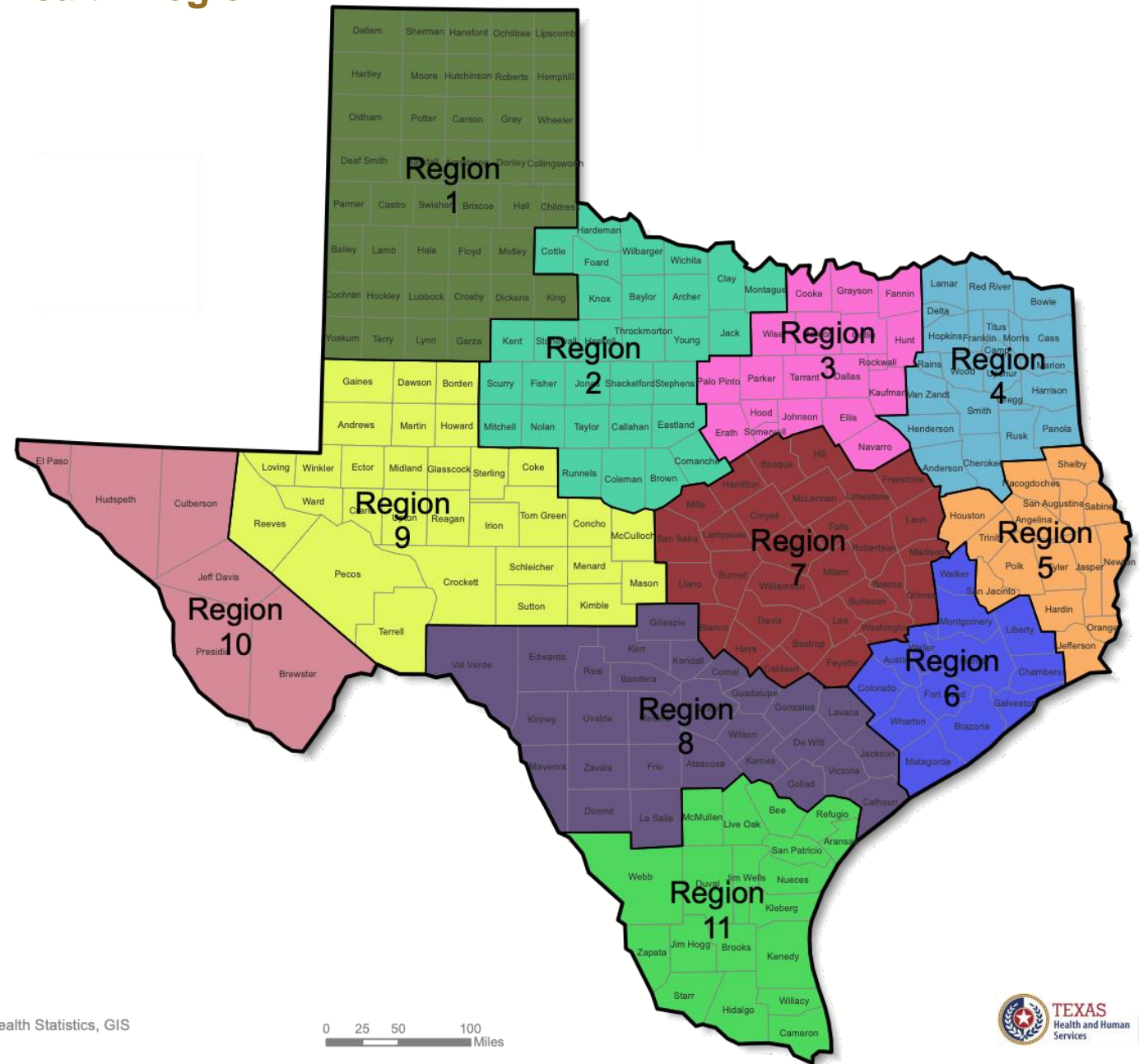


Incidence and Five-Year Survival by Public Health Region

Using data from TCR 1995-2018

PHR	Incidence, per M	5-Yr Survival
1	174.1	84.0%
2	173	80.7%
3	181.5	83.0%
4	174	82.9%
5	176.7	83.4%
6	190.1	82.2%
7	179.1	82.7%
8	185.9	81.0%
9	170.3	83.6%
10	212.8	79.6%
11	185.8	79.7%
TX	184.2	82.1%

Red cells are worse than state values.



Summary

- Survival & outcomes of patients with childhood cancer needs improvement
- Second largest state population of childhood cancer patients is in Texas
- AYAs with cancer represent an additional population with critical needs
- Survival rate of children with cancer in Texas similar to national average
- Pediatric cancer incidence and survival varies by public health regions in Texas
- Both improvements in outcomes across the state as well as in specifically areas where they are less favorable is needed



Summary of CPRIT Pediatric Accomplishments



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CPRIT Funds Important Childhood Cancer Research

- New research projects tackling big problems
 - Neuroblastoma and Rhabdomyosarcoma, Reynolds, Texas Tech HSC
 - Burkitt's Lymphoma – Green, MDA
 - High-grade Gliomas – Hu, MDA
 - Medulloblastoma – Taylor, BCM
 - Role of Germline BRCA Pathway Mutations – Plon, BCM
 - Rhabdomyosarcoma – Grow, UTSouthwestern
 - Leukemias – Song, BCM
 - Screening in Children and Adolescents Genetically at High Risk of Pediatric Cancer – Tomlinson, UT HSC San Antonio
 - AI-Based Education and Communication about HPV in Children and Adolescents, Tao, UT HSC Houston



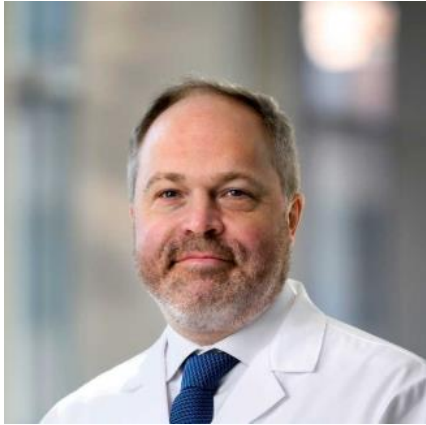
Core Facilities Support Awards create new resources

Provides financial support for a wide variety of projects relevant to cancer research in Texas, including for pediatric specific projects such as:

Title	PI	Institution	Award Year
The Adolescent and Childhood Cancer Epidemiology and Susceptibility Service (ACCESS) for Texas	Michael Scheurer, PhD, MPH	Baylor College of Medicine	2021
Center for Innovative Drug Discovery Enhancement of a Shared Cancer Resource for South Texas	Stanton McHardy, Ph.D.	The University of Texas Health Science Center at San Antonio	2022
Patient-Derived Xenograft and Advanced In Vivo Models (PDX-AIM) Core Facility of Texas	Michael Lewis, Ph.D	Baylor College of Medicine	2022
West Texas Pharmacology Core	Min Kang, PharmD	Texas Tech University Health Sciences Center	2022
Advanced Protein Therapeutics Core	Jennifer Maynard, Ph.D	The University of Texas at Austin	2022
Texas Pediatric Cancer Testing (TPCT) Core	Peter J. Houghton, PhD	The University of Texas Health Science Center at San Antonio	2022



CPRIT Recruits New Childhood Cancer Researchers to Texas



Michael D. Taylor, M.D., Ph.D.

- CPRIT Established Investigator
- Recruited to Baylor College of Medicine from Cincinnati Children's
- Pediatric neurosurgeon and leader in the international neurooncology community



Edward Grow, Ph.D.

- CPRIT First-Time, Tenure Track Faculty Member
- Recruited to UTSW from the University of Utah
- discovered a novel DNA damage response involving p53 activation; rhabdomyosarcoma



Julea Vlassakis, PhD, BA

- CPRIT First-Time, Tenure Track Faculty Member
- Recruited to Rice University from the University of California, Berkeley
- Expert in single-cell & single molecule analysis of proteins & interactions with macromolecules.



Yuan Pan, PhD

- CPRIT First-Time, Tenure Track Faculty Member
- Recruited to MD Anderson from Stanford University
- Role of neuronal activity in childhood cancer



Many thanks to CPRIT Leadership

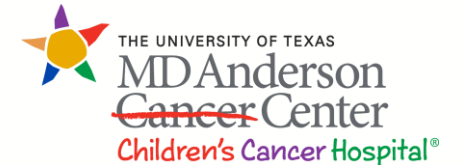
- Incredible engagement in issues facing children with cancer
 - Michelle Le Beau
 - Wayne Roberts
 - Mahendra Patel
 - Patty Moore



Advisory Committee on Childhood Cancer Recent Steps



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CPRIT ACCC Organization

Leadership

Richard Gorlick, M.D., Chair
Donald (Will) Parsons, M.D., Ph.D., Vice-Chair

Members

Karen Albritton, M.D.; Carl E. Allen, M.D.; Mohamad Al-Rahawan, M.D., MPH; Greg Aune, M.D., Ph.D., FAAP; Juan Carlos Bernini, M.D.; Smita Bhaskaran, M.D.; Tim Culliver; Stan Goldman, M.D.; Meaghan Granger, M.D.; Barkat Hooda, M.D.; Eugenie Kleinerman M.D.; Andrew Y Koh, M.D.; Annette Leslie; Julie Luke, CPNP; Virginia Harod, M.D.; C Patrick Reynolds, M.D., Ph.D.; Stephen X. Skapek, M.D.; Lisa Tichenor; Gail Tomlinson, M.D., Ph.D.

SC1: AYA

Karen Albritton
(Leader),
Michael Roth,
Chibuzo O'Suoji
(Members)

SC2: Brain Tumors

Donald Parsons
(Leader), Daniel
Bowers, Holly
Lindsay
(Members)

SC3: Cell Therapy

Andrew Koh
(Leader), Kris
Mahadeo, Robin
Parihar, Samuel
John, Matthew
Campbell,
Meena Hegde
(Members)

SC4: Epidemiology

Philip Lupo
(Leader), Paul
Scheet, Sandi
Pruitt, Michael
Scheurer,
Michael Roth
(Members)

SC5: Frontiers

Smita
Bhaskaran,
Mohamad Al-
Rahawan (Co-
Leaders), Lisa
Thicenor,
Shannon Cohn
(Members),
Richard Gorlick
(Advisor)

SC6: Genetic Predisposition & Risk

Gail Tomlinson
(Leader), Laura
Klesse (Member)

SC7: Leukemia / Lymphoma

Carl Allen
(Leader), Rachel
Rau (Member)

SC8: Solid Tumors

Nino Rainusso
(Leader),
Jessica
Naiditch, Dinesh
Rakheja,
Gabriel Axelrud,
Lorimar
Ramirez
(Members)

SC9: Survivorship

Greg Aune
(Leader),
Barbara Jones,
Monica
Gramatges,
Chibuzo
O'Suoji, Michael
Roth (Members)



The Cancer Prevention and Research Institute of Texas
The Carson Leslie Foundation proudly present Researchers' RoundUp
July 24-26, 2022

THANKS TO THE GENEROUS SUPPORT OF OUR WRANGLERS (aka sponsors)



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PHILANTHROPIES

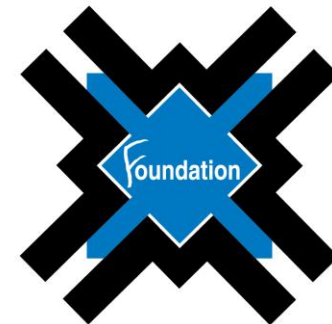


helping kids fight cancer



PEGASUS
PARK
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what would Willie want



www.QuadW.org

RESEARCHERS
ROUNDUP

together, helping kids fight cancer



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Advisory Committee on Childhood Cancer Strategy



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Continue Childhood Cancer Core Facilities Support Awards

- Impact to date
 - 18 Cores, \$104M follow-on funds, 135 publications, 2 patents, 3 clinical trials with 443 patients enrolled.
 - New, shareable childhood cancer models
 - Texas Pediatric PDX facility (Houghton, UTHSCSA)
 - PDX-AIM (Lewis, BCM)
 - Cancer Animal Facility (Trasti, TTUHSC)
 - New capacity for data storage and sharing
 - Pediatric Cancer Data Core (Xie, UTSW)
 - Pediatric Solid Tumors Comprehensive Data Core (Gorlick, UTMDACC)
 - ACCESS for Texas (Scheurer, BCM)
- Recommendations
 - Specific calls for CFSA proposals focused on childhood cancer
 - Ensure impact extends beyond local institutions
 - Enlist ACCC to help prioritize Core Facilities
 - Invite competitive renewal of high-performing Cores that fill state-wide needs



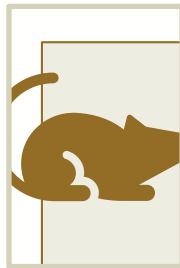
Potential Short-Term Goals/Suggestions



Enhanced website for
Core information
dissemination



Patient Derived
Xenograft
sharing/homogenization



Preclinical
testing/product
development integration



Data harmonization and
sharing



RFAs should require
use of cores

A publication disseminating the results of the RR as a means of sharing the strategy










Strategy for advancing childhood cancer care in Texas will address the following issues

- The population is not well defined, particularly where they receive their treatment with this even more profound for AYA patients
- Variation in incidence across Texas is not understood with demographic details and environmental exposures not captured
- At many sites there is a lack of critical infrastructure necessary to provide information to registries, collect biological materials or participate in clinical trials
- Insufficient numbers of pediatric cancer-focused Population Scientists and Implementation Scientists



Consider creating funding mechanisms which would accrue patients throughout Texas

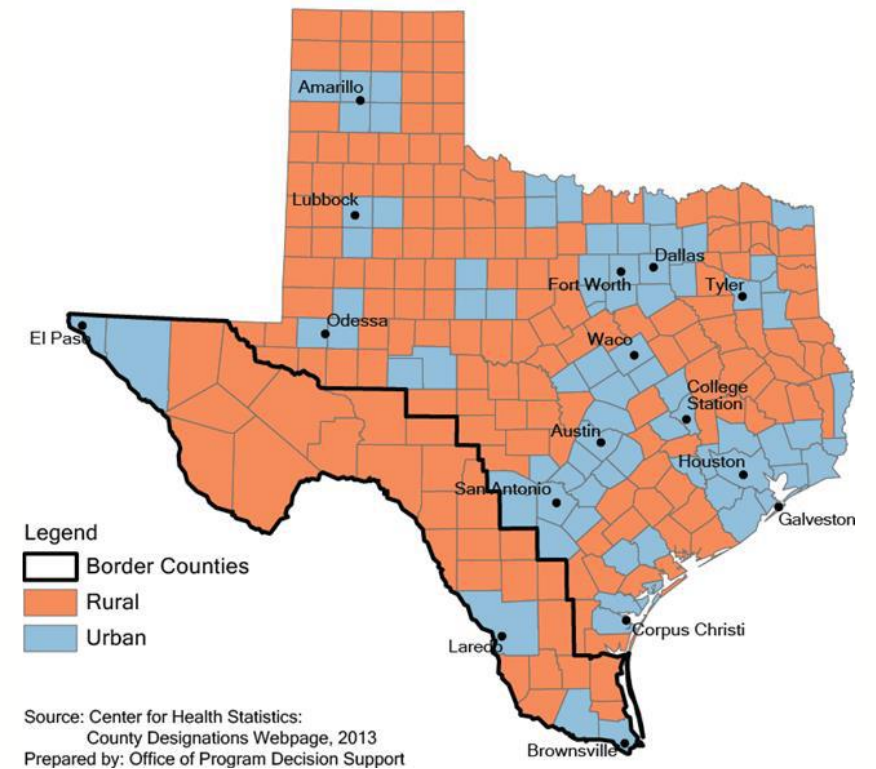
						
Design population-based studies	Study sub-groups	Utilize Implementation Science to maximize engagement	Provide interventions	Expand reach via technology	Include underserved populations	Provide services not often available outside large academic centers



The RFPs will help define Childhood Cancer throughout Texas

- Identify primary, secondary and tertiary **referral pattern** of new/relapsed patients
- Assess current **clinical trial accrual rate**
- Obtaining data on the **capabilities** of rural pediatric oncology centers to conduct clinical trials
- Identifying **Infrastructure that is lacking** and much needed to improve those capabilities

2013 Rural, Urban, and Border County Designations in Texas



CPRIT Recruits New Childhood Cancer Researchers to Texas

- Consider specifically requesting applications for new researchers focused on Childhood Cancer topics that are critical needs.
- Areas of high priority include pediatric cancer population scientists and implementation scientists



Summary/Next Steps



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ACCC Recommendations to CPRIT

- Critical to continue Pediatric Applications particularly Core Grants but also research grants and Recruitment Awards
- As Immediate Opportunities:
 - Enhanced website for Core information dissemination
 - Model sharing with treatment development structure
 - Data harmonization/sharing
 - Enhanced ability to participate in Cores as well as encouragement of utilization
 - Prepare manuscript describing ACCC strategy and outcomes for publication in a scientific journal



ACCC Recommendations to CPRIT

- Longer-Term Opportunities:
 - Utilize the Cores along with a new RFP to:
 - Enhance collaboration across Texas with smaller programs
 - Facilitate data sharing across the state
 - Increase access to trials
 - Implement best practices and care throughout Texas
 - Conduct population-based studies
 - Propose a common infrastructure for pediatric cancer research in Texas



Summary

- The ACCC applauds Texans for the forward-thinking development of CPRIT and supporting its visionary leadership that continues to embrace childhood cancer research.
- CPRIT has supported remarkable innovation and scientific breakthroughs benefitting children with cancer in Texas. Continued support of pediatric targeted proposals remains critical.



Thank you to the entire CPRIT Advisory Committee on Childhood Cancers for their engagement, support and commitment to our mission!



Q&A

Richard Gorlick, MD
Chair, CPRIT Advisory Committee on Childhood Cancers



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