

Congressional / OSTP



2022

DIPG/Pediatric Brain Cancer

BRIEFING

117<sup>th</sup> Congress ~ H. Res. 404

2022 MOONSHOT4KIDS  
STREAMING EVENT  
Facebook.com/CCTalkRadio

## Virtual Summit

Supporting greater awareness for DIPG, Childhood Brain Cancer and the urgent, unmet medical needs of all children with cancer as presented in H. Res. 404, the *DIPG Awareness Resolution*.

**NKORE BIOTHERAPEUTICS BREAKTHROUGH CANCER TREATMENT PRESENTATION**

**Tuesday, SEPTEMBER 20, 7pm ET**

***EVENT Recording to be available on youtube and FB***

## MOONSHOT4KIDS CONGRESSIONAL / OSTP BRIEFING

Originally presented on Tuesday, May 17, 2022 at 9:00 am  
2045 Rayburn HOB, Washington, DC 20515



*‘DIPG, Pediatric Brain Cancer and the Importance of  
National Awareness in Expediting Solutions for Children,  
as presented in H. Res. 404.’*



PRESENTED BY THE CURE STARTS NOW  
&  
The CARSON LESLIE FOUNDATION

*In Collaboration with the Offices of:  
Congresswoman Debbie Dingell (D-MI-12),  
Congressman David Joyce (R-OH-14),  
and Congressman Michael McCaul (R-TX-10)*

Special Thanks to Annette Leslie, Congressman McCaul, and Keith Desserich...  
*and most especially to*

Aspen, Xavier (not pictured) Lilly, Ella, Sophie, Grace, Finley and Kami  
*and to Lisa Ward for bringing Lily, and Angel Jace*

## *~ Program ~*

### ***Special Thanks to the Childhood Cancer Community and Our Sponsors***

The Cure Starts Now      Carson Leslie Foundation

### ***Welcome & Introduction***

–Janet Demeter for DIPG Advocacy Group:

**DIPG: A Unique Opportunity for Childhood Cancer Awareness,**  
Congressional Support, and a Unifying Effort to Accelerate cures for Children and to Facilitate  
Innovation in Policy and Research to better Accommodate the Needs of Our Children  
**Blasting Off Collective Denial with A Moonshot4Kids, H. Res. 404**

### **Congressman McCaul and the Kids, 5/16 – 17 2022**

Survivors gather at Reception; Congressman McCaul accepts the Archangel of Hope Award  
Opening Remarks at Briefing  
Medulloblastoma Survivor Ella Breese  
DIPG Mom Cassie Diaz

Message from Congresswoman Debbie Dingell (MI-12)

### **I. DIPG/DMG Basic Science**

*“So close yet so far.” –translatable research and the promise of novel therapies,  
current outcomes remaining unchanged*

**Michelle Monje Deisseroth, MD PhD** - Stanford University, Palo Alto, CA  
**Adam Green, MD** -University of Colorado School of Medicine (Anschutz Campus)  
**Mark M. Souweidane, M.D., F.A.C.S., F.A.A.P.**  
*New York-Presbyterian/Weill Cornell Medical Center and  
Memorial Sloan Kettering Cancer Center*

### **II. The Investment of Parents Driving Needed Research**

*trailblazing the pathway for the Homerun Cure and the DIPG Registry*

**Keith Desserich** CEO, The Cure Starts Now  
**Lindsey M. Hoffman, DO**, Phoenix Children’s Medical Group,  
*Hematology/Oncology, and DIPG Registry*

### **III. CPRIT**

*Unparalleled State-Level Leadership for Childhood Brain Cancer*

**Gerald McDougall, Board Chairman**, Carson Leslie Foundation, Dallas TX  
**Ripley Martin, Director**, Carson Leslie Foundation, Dallas, TX  
**Michelle M. Le Beau, PhD, CSO**,  
Cancer Prevention & Research Institute of Texas (CPRIT)

### **IV. Biotech and Industry**

*A Pediatric Focus: Burden of making the case for investment, Academic collaboration;  
challenge of drug approval/use off the shelf*

**Samuel Blackman, MD PhD and Mark Kieran, MD PhD** - Day One Bio

## V. Crossroads of databases and clinical trials:

*The Importance of Increased Collaborative Research in the United States  
and Around The World*

**Adam Resnick, PhD** - Children's Hospital of Philadelphia;  
Lead, CBTN, PNOC, CCDI

**Will Parsons MD, PhD**, Texas Children's Cancer and Hematology Center,  
Baylor College of Medicine (Houston TX)

**Javad Nazarian, Ph.D, M.Sc** - PNOC, Children's University Hospital, Zurich,  
DIPG Centre of Excellence; Children's National Hospital, Washington, DC

## VI. NIH/NCI Steps Up for Childhood Cancer Research

Brigitte C. Widemann, M.D. *Chief, Pediatric Oncology Branch,  
National Cancer Institute (NCI) and Special Advisor  
to the NCI Director for Childhood Cancer*

## VII. Philanthropic Partners

**David Arons, JD (CEO)**, *National Brain Tumor Society*

*A message from **Warrior Jace Foundation**, Jennifer Thompson (North Carolina)*

## VIII. Families and Boots on the Ground in Real-Time:

*The Unreality of DIPG statistics;  
childhood brain cancers as neglected, rather than rare diseases*

**Marcelo Ramalho-Ortigao, PhD** - DIPG Advocacy Group and DIPG parent (Juliana)  
–*Scientist and non-scientist patient families, trailblazing, relying on social media*

**Grace Wethor**, DIPG Patient, Actress, Author, Brain Tumor Survivor

Nicole Puglisi, DIPG Patient, Student, Survivor

**Philip Tan, PhD** - DMG parent (Xavier)

**Ryan Agnew**, DIPG parent (Aspen)

**Congressman David Joyce (OH-14) Remarks in Support of H. Res. 404,**  
and expediting cures for our children.

Conclusion - Let us be resolved, together, for our children.

*“Given this opportunity to bring attention to a hidden population of suffering,  
we are asking you to please help us speed a cure; support H. Res. 404 and set a precedent  
of acknowledgement and caring for children facing this death sentence.  
Let's make history for Childhood Cancer Awareness now, THIS YEAR!”*

~ ~

## Featured Presentation followed by Community Discussion Q&A

*“Novel strategies to treat and prevent aggressive lethal cancers using  
super-charged Natural Killer cell immunotherapeutic”*

**Dr. Anahid Jewett** (UCLA), NKORE Biotherapeutics

**Tracy Ryan**, brain tumor parent/entrepreneur, CCO, NKORE Biotherapeutics

# MOONSHOT4KIDS CONGRESSIONAL / OSTP BRIEFING

## ~ Briefing Presenters ~

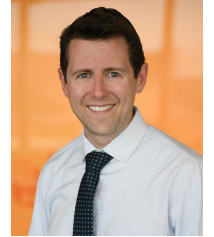


*Michelle Monje Deisseroth*

MD, PhD, Professor of Neurology and Neurological Sciences; Professor, by courtesy, of Neurosurgery, Pathology, Psychiatry and Pediatrics; Investigator, Howard Hughes Medical Institute, Stanford University

Dr. Monje is a **neuroscientist and practicing pediatric neuro-oncologist** whose research program focuses at the intersection of neuroscience and brain cancer biology. Her work has focused on neuron-glial interactions in health and oncological disease.

*Adam Green, MD*



Assistant Professor of Pediatrics | Attending Physician in Pediatric Neuro-Oncology/Oncology at Colorado University School of Medicine, and Colorado Children's Hospital, My research focuses on a specific type of cancer called high-grade glioma, an aggressive brain tumor affecting both children and adults. We focus especially on two pediatric subtypes called diffuse intrinsic pontine glioma and pediatric glioblastoma. I work in the lab to find new treatments for these tumors; we then study the treatments in laboratory models of the tumors, including mouse models, and then bring the treatments most likely to be successful to clinical trials in children.

*Mark M. Souweidane, M.D., F.A.C.S., F.A.A.P.*



Dr. Mark M. Souweidane is internationally recognized as a leader in pediatric neurosurgery, with a career focus on CNS tumors of childhood. He is the Vice Chairman of the Department of Neurological Surgery at the Weill Cornell Medical College and director of pediatric neurosurgery at the New York-Presbyterian Hospital/Weill Medical Center and Memorial-Sloan Kettering Cancer Center. He is the principal investigator (PI) for a clinical trial using convection-enhanced delivery (CED) and immuno-radiotherapy as a novel therapeutic approach for children with DIPG (NCT01502917). In 2017 Dr. Souweidane was selected by the National Cancer Institute (NCI) to serve as the Neurological Surgery Special Expert on its Brain Malignancies Steering Committee (BMSC) and was appointed to the Children's Oncology Group (COG) CNS Steering Committee.

*Keith Desserich*



Keith Desserich is a father to Elena, a 6-year-old girl diagnosed with DIPG in 2006 and the subject of the New York Times Bestselling book, "Notes Left Behind". He and his wife Brooke founded The Cure Starts Now Foundation, the largest funder of DIPG/DMG research internationally. Today the efforts of this charity are in 3 countries with 40 chapters, in partnership with over 600 warrior families and have led to research at over 109 hospitals worldwide. He also founded the DIPG/DMG Collaborative (a group of 24+ independent foundations that work cooperatively to fund research), [www.dipg.org](http://www.dipg.org), [www.medulloblastoma.org](http://www.medulloblastoma.org) and the DIPG/DMG Symposium. He is a steering committee member of the DIPG/DMG International Registry, the SIOPe DIPG Registry, the CONNECT Consortium and the Pediatric Brain Tumor Consortium. He and his wife live in Ohio with their adult daughter Grace, and youngest daughter Nina.



*Lindsey Hoffman, DO*

Lindsey Hoffman, DO, is an Assistant Professor of Pediatrics and investigator in the Morgan Adams Foundation Pediatric Brain Tumor Research Program at the University of Colorado. She did her undergraduate work at William Jewell College and earned her medical degree from Kansas City University of Medicine and Biosciences. She served as a pediatric resident at Texas Children's Hospital/Baylor College of Medicine and completed fellowship training at the University of Colorado, where she worked in the laboratory of Dr. Nicholas Foreman studying ependymoma biology. She now leads the cancer predisposition program in the Center for Cancer and Blood Disorders and is an active member of the experimental therapeutics team focused on development of clinical trials for high-risk pediatric CNS tumors at Children's Hospital Colorado.

## *Texas leads Team USA for Childhood Cancer Research*

*Gerald McDougall*



Gerry McDougall spent over 20 years as a senior partner at PricewaterhouseCoopers. He built the firm's Global Health Science consulting practice, establishing PwC as the global leader among strategy and consulting firms in the creation of strategic alliances, joint ventures and partnerships across the health care value chain. What began with a focus on basic, translational and clinical science, soon expanded to building expertise in personalized/precision medicine.

He is the Board Chairman of the Carson Leslie Foundation #cureMEDullo, and is a Member of the ASCO Foundation/Conquer Cancer, as well as a Member of the Infectious Disease Research Institute, and a Member/Exec Committee of the Multiple Myeloma Research Foundation in Boston, MA.

*Ripley Martin*



Ripley Martin is a healthcare software executive and a board director of the Carson Leslie Foundation. He is also a brain-tumor parent. A 20-year veteran of the healthcare industry, Mr. Martin serves as Senior Vice President of Life Science Partnerships and Real World Data for Loopback, a provider of Specialty Pharmacy and Clinical Analytics software. Prior to Loopback, Mr. Martin spent 10 years in executive roles with Philips Healthcare including most recently as global leader for the company's venture investing arm and served as a Principal with the Healthcare practice of the Boston Consulting Group. Mr. Martin's 11-year old son Cooper is a 5-year Medulloblastoma survivor.





Michelle M. Le Beau, Ph.D.

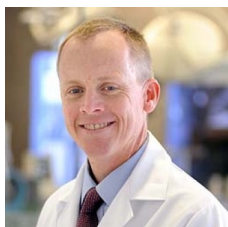
Chief Scientific Officer, Cancer Prevention and Research Institute of Texas (CPRIT)

Dr. Le Beau leads CPRIT's Academic Research Program in supporting innovative cancer research and recruiting world-class cancer researchers to Texas institutions. Her distinguished research career has focused on basic and translational research. She is recognized for her work identifying recurring chromosomal abnormalities and defining the clinical and genetic subsets of leukemia, characterizing the genetic pathways that lead to hematological malignancies, and the application of fluorescence in situ hybridization for clinical diagnostics and gene mapping.

## *Data, Collaboration, Research Infrastructure, and Time*

Adam Resnick, PhD

Director of Data Driven Discovery in Biomedicine, Children's Hospital of Philadelphia (CHOP); Scientific Chair, Children's Brain Tumor Network (CBTN), Pacific Pediatric Neuro-Oncology Consortium (PNOC) Dr. Resnick's research focuses on the cell signaling mechanisms of oncogenesis and tumor progression in brain tumors. He studies signaling cascades and alterations to elucidate the molecular and genetic underpinnings in order to develop targeted therapies. As co-director of the Center of Data-Driven Discovery in Biomedicine, he leads a multidisciplinary team building and supporting a scalable, patient-focused healthcare and educational discovery ecosystem.



Will Parsons MD, PhD

Sidney L. and Donald F. Faust Chair of Pediatric Cancer Precision Medicine, Texas Children's Hospital  
Deputy Director, Texas Children's Cancer and Hematology Center  
Co-Director, Brain Tumor Program; Co-Director, Cancer Genetics and Genomics Program;  
Director, Center for Precision Oncology  
Associate Professor, Department of Pediatrics, Section of Hematology-Oncology,  
Baylor College of Medicine

Javad Nazarian, MD, PhD

*Scientific Director*, Brain Tumor Institute at Children's National Hospital in Washington, D.C;  
*Department Head*, DMG Research Center, University Children's Hospital, Zurich Switzerland

Dr. Nazarian leads the international collaborations of PNOC, DMG-ACT in the effort to accelerate the discovery of clinically translatable therapies for the treatment of childhood brain cancers. Since inception in January 2020, the DMG-ACT team has generated data to support two new clinical trials with adaptive arms for children diagnosed with diffuse midline gliomas.



Brigitte C. Widemann, M.D.



*Chief, Pediatric Oncology Branch, National Cancer Institute (NCI), Special Advisor to the NCI Director for Childhood Cancer*

Dr. Widemann is a pediatric oncologist with the primary interest of developing effective therapies for children and adults with genetic tumor predisposition syndromes, such as neurofibromatosis type 1 (NF1), and rare solid tumors. She began her career in medicine in Germany where she completed her pediatric residency at the University of Cologne. Dr. Widemann then moved to the NIH for a pediatric hematology and oncology fellowship in the Pediatric Oncology Branch of the NCI. She conducted her research in the [Pharmacology and Experimental Therapeutics Section \(PETS\)](#), where she studied antimetabolites and had a leadership role in the clinical development of glucarpidase, a rescue agent for patients who experience renal failure after administration of high-dose methotrexate.

Samuel Blackman, MD PhD

Founder and Chief Medical Officer, Day One Biopharmaceuticals, Inc.

Dr. Blackman is a physician-scientist trained in pediatric hematology/oncology and neuro-oncology, and has led the early clinical development of more than 10 novel cancer therapeutics. Through roles at CureSearch and ACCELERATE, Dr. Blackman is an active leader in the pediatric oncology drug development community.



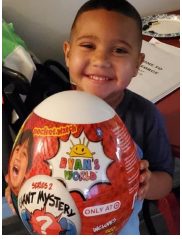
David Arons, JD (CEO),  
National Brain Tumor Society

David Arons is the Chief Executive Officer of the National Brain Tumor Society. Prior to joining NBTS, he served in leadership and external positions at the American Cancer Society in Minnesota, the Center for Lobbying in the Public Interest and at Independent Sector. As an attorney, he previously represented patients facing disabilities and serious health conditions. He is the author of several books, including *Power in Policy: A Funder's Guide to Advocacy and Civic Participation*, *Strengthening Nonprofit Advocacy*, and *A Voice for Nonprofits*. David has served on the National Cancer Institute's Council of Research Advocates and Clinical Trials Advisory Committee. In 2016, David was named to the Blue Ribbon Panel of experts selected to help advise the National Cancer Moonshot, led by former Vice President, now President Biden.

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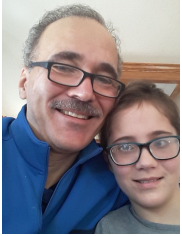
~ Briefing Presenters, continued;

Parents, Patients ~



Cassie Diaz (New York, NY)

Cassie shares about her adorable boy Jayce who at the time was struggling with the latent effects of DIPG. He succumbed in early 2021.



Marcelo Ramalho-Ortigao PhD. (Fort Collins, CO)

Dr Marcelo Ramalho-Ortigao is a scientist and professor of vector-borne and emerging infectious diseases. Dr Ortigao spent nearly 30 years in academia investigating underlying mechanisms driving the transmission of pathogens and onset of infectious diseases. Following the diagnosis of his daughter Juliana with DIPG he is now dedicated to the DIPG cause. Dr Ortigao's experience navigating health issues ranging from drug development to effective therapies and from clinical trials to patient advocacy helps bring attention to many of the needs of the DIPG community. Dr Ortigao is currently the Medical and Science Advisor of the DIPG Advocacy Group



Grace Wethor (Los Angeles, CA)

Actress, Author, Brain Tumor Survivor

*Grace continues to beat the odds, living her dreams; her tumor dormant.*



Nicole Puglisi

Clifton, NJ

Survivor, 8 yrs past diagnosis



Philip Tan, PhD (San Diego, CA)

"My name is Phil Tan and my 20-year old son Xavier was diagnosed 20 months ago. I wanted to be here in person but now I need to be with Xavier and my family for these could be his last days with us. Along his journey, he has been hospitalized 6 times, had 3 brain surgeries, and had numerous disease- and drug-induced complications. The amount of pain and suffering he has endured is unbelievable. This is a truly evil disease."

*Xavier Tan passed away 7/3/2022 surrounded by his loving family.*



Ryan and Alison Agnew (Sandy, UT)

A beautiful family, with precious Aspen (6) fighting DIPG, and her little brother. Aunt Tristan, Grandpa Bill and Grandma Kim are determined advocates.

*Aspen is currently (Sept. 2022) in hospice care.*

## Featured Scientific Presentation

NKore™ is combining proprietary-cellular and protein-based diagnostics, novel AI analytics and a natural, proprietary approach to “Super Charging” allogeneic Natural Killer Cells to deploy a new era in immuno-therapeutic medicine for the treatment of cancer in patients of all ages. At NKore, we put patients first!



Tracy Ryan (Los Angeles, CA)

### *Saving Sophie; The Mom, the Scientist & Their Journey to Cure Cancer*

Sophie Ryan was diagnosed with a low-grade brain tumor at 8 1/2 months old, devastating first time parents Tracy and Josh Ryan. Little did they know that this horrific diagnosis would lead them down a path to advance science for cancer patients everywhere, including their beautiful daughter.

Now 9-years later, Tracy has joined forces with world renowned cancer research scientist Dr. Anahid Jewett, and their two other partners Greg Brophy and Tim Brahm, to launch NKore BioTherapeutics; a Natural Killer Cell diagnostics and therapeutics company. With over 30-years of research behind their technology, this team is on a mission to bring therapies to market that fix a patient's Natural Killer Cell system, their number one defense against cancer, versus using toxic poisons to target the disease.



Dr. Anahid Jewett

Professor and Director of Tumor Immunology Laboratory  
Division of Oral Biology and Medicine  
The Jane and Jerry Weintraub Center for Reconstructive Biotechnology  
Jonsson Comprehensive Cancer Center  
UCLA School of Dentistry and Medicine  
CSO, NKORE Biotherapeutics

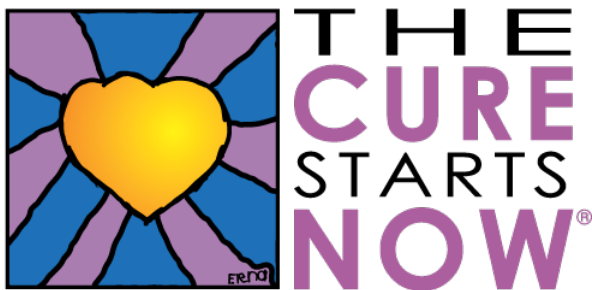


# *The Ones Who Made It All Happen*



Special Thanks to Keith Desserich, Annette Leslie and Congressman McCaul,  
and most especially to  
Aspen, Xavier (not pictured) Lilly, Ella, Sophie, Grace, Cameron and Finley...  
and to Lisa Ward for bringing Lily, and Angel Jace

*~ Thank You ~*



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*Special Thanks to:*

