## FDA Public Workshop: The Future of Registries in Oncology

The purpose of this 2-day public workshop is to bring together all interested parties to discuss ongoing efforts and future directions in registry development in oncology, with a specific focus on the malignant pediatric brain tumor, Diffuse Midline Glioma (DMG)/Diffuse Intrinsic Pontine Glioma (DIPG). Day 1 will introduce relevant concepts regarding registries and common data elements, highlight successes and challenges in registry development, and review learnings from registries which have supported regulatory decision making in oncology. Day 2 will focus on the ongoing efforts to build national and international registries of patients with pediatric DMG/DIPG, with forward-looking discussions regarding registry "best practices" for this rare, life-threatening cancer. The goals of this workshop are to provide a first step in a longitudinal effort to optimize registry development in DMG/DIPG, and to encourage the collection of fit-for-use, high-quality registry data that could potentially support novel therapeutic development for patients with DMG/DIPG.

## Session 1: Wednesday, August 27, 2025

## The Future of Registries in Oncology: Best Practices for Innovation in Drug Development

| | |
|---|---|
| 10:00-10:10 am<br>(all times in EST) | **Welcome and Introduction**<br>*Elizabeth Duke, MD – Office of Oncologic Diseases (OOD), Center for Drug Evaluation and Research (CDER), FDA* |
| 10:10-10:35 am | **How can registries be used in oncology drug development and regulatory decision-making?**<br>• *Pallavi Mishra-Kalyani, PhD – Deputy Director, Division of Biometrics V, CDER, FDA*<br>• *Donna Rivera, PharmD, MSc – Former Associate Director for Pharmacoepidemiology, Oncology Center of Excellence, FDA; University of Maryland School of Pharmacy Affiliate* |
| 10:35-11:20 am | **Learning from the Past: Examples of Registries in Oncology**<br>• *Marcelo Pasquini, MD – Center for International Blood and Marrow Transplant Research (CIBMTR); Medical College of Wisconsin*<br>• *Bill Wood, MD, MPH – American Society of Hematology (ASH) Research Collaborative; University of North Carolina at Chapel Hill*<br>• *Sarah Leary, MD – Children's Oncology Group (COG); Seattle Children's Hospital*<br>• *Q&A* |
| 11:20-11:35 am | **BREAK** |

| | |
|---|---|
| 11:35-12:45 pm | **Panel Discussion: What can we learn, what are registry "best practices," and what has contributed to the success of past efforts in oncology registry development?**<br><br>Moderator: *Pallavi Mishra-Kalyani, PhD – Deputy Director, Division of Biometrics V, CDER, FDA*<br><br>Panelists:<br>• *Marcelo Pasquini, MD – Center for International Blood and Marrow Transplant Research (CIBMTR); Medical College of Wisconsin*<br>• *Bill Wood, MD – American Society of Hematology (ASH) Research Collaborative; University of North Carolina at Chapel Hill*<br>• *Sarah Leary, MD – Children's Oncology Group (COG); Seattle Children's Hospital*<br>• *Gregory Reaman, MD –Scientific Director, Childhood Cancer Data Initiative (CCDI), National Cancer Institute (NCI); Professor Emeritus of Pediatrics, George Washington University*<br>• *Kris Ann Schultz, MD – Children's Minnesota; Pleuropulmonary Blastoma (PPB)/DICER1 Registry*<br>• *Trish Anderson – Patient Advocate; Pleuropulmonary Blastoma (PPB)/DICER1 Registry*<br>• *Somak Chatterjee, PhD – Division of Biometrics V, CDER, FDA* |
| 12:45-1:00pm | **Summary and Closing**<br>*Elizabeth Duke, MD – Office of Oncologic Diseases, CDER, FDA* |

## Session 2: Thursday, August 28, 2025

## The Future of Registries in Oncology: Advancing Drug Development in Pediatric DMG/DIPG

| | |
|---|---|
| 10:00-10:10 am (all times in EST) | **Welcome and Summary of Session 1**<br>*Elizabeth Duke, MD – Office of Oncologic Diseases, CDER, FDA* |
| 10:10-10:25 am | **Clinical Overview and Unmet Need in DMG/DIPG**<br>*Maryam Fouladi, MD, MSc – Children's Oncology Group (COG) CNS Committee Chair; Nationwide Children's Hospital* |
| 10:25-11:25 am | **Current State of the Science: Registries in DMG/DIPG**<br>• *Trent Hummel, MD – International DIPG/DMG Registry; Cincinnati Children's Hospital*<br>• *Keith Desserich – SIOPE DIPG/DMG Registry; The Cure Starts Now Foundation; DIPG/DMG Collaborative*<br>• *Adam Resnick, PhD – PNOC & SJCRH Collaborative DIPG Radiogenomic Investigation; Children's Hospital of Philadelphia*<br>• *Giselle Sholler, MD, MSc – Beat Childhood Cancer Consortium; Penn State Health*<br>• *Q&A* |
| 11:25-11:35 am | **BREAK** |
| 11:35-12:45 pm | **Panel Discussion: Where are we now with registries in DMG/DIPG and what are the next steps to optimize registry development for this disease?**<br><br>Moderator: *Martha Donoghue, MD – Associate Director, Pediatric Oncology and Rare Cancers, Oncology Center of Excellence, FDA*<br><br>Panelists:<br>• *Maryam Fouladi, MD, MSc – Children's Oncology Group (COG) CNS Committee Chair; Nationwide Children's Hospital*<br>• *Trent Hummel, MD – International DIPG/DMG Registry; Cincinnati Children's Hospital*<br>• *Keith Desserich – SIOPE DIPG/DMG Registry; The Cure Starts Now Foundation; DIPG/DMG Collaborative*<br>• *Adam Resnick, PhD – PNOC & SJCRH Collaborative DIPG Radiogenomic Investigation; Children's Hospital of Philadelphia*<br>• *Giselle Sholler, MD, MSc – Beat Childhood Cancer Consortium; Penn State Health*<br>• *Arzu Onar-Thomas, PhD – COG and PBTC Statistician; St. Jude Children's Research Hospital*<br>• *Nicole Drezner, MD – Deputy Director, Division of Oncology 2, CDER, FDA* |
| 12:45-1:00pm | **Summary, Closing, and Next Steps**<br>*Elizabeth Duke, MD – Office of Oncologic Diseases, CDER, FDA* |