FOOD AND DRUG ADMINISTRATION (FDA)
Center for Drug Evaluation and Research (CDER)

Meeting of the Pediatric Oncology Subcommittee of the
Oncologic Drugs Advisory Committee (pedsODAC)
May 11-12, 2021

AGENDA

On May 11, 2021, the subcommittee will discuss the development and successful implementation of the Pediatric Patient-Reported Outcomes Version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE) as a tool for eliciting the patient's voice in oncology clinical trials to more accurately determine tolerability and toxicity of drugs under investigation. The subcommittee will also address the challenges of capturing this type of data across the age spectrum of the pediatric population and possible generalizability of the data. It will consider approaches to address concerns about excluding the patient voice of young children deemed incapable of self-reporting. The subcommittee will also focus on approaches to investigators and commercial sponsors to use the Pediatric PRO-CTCAE in toxicity assessment moving forward.

Day 1: May 11, 2021

10:00 a.m.	Call to Order	**Alberto S. Pappo, MD** Chairperson, pedsODAC
10:05 a.m.	Introduction of Subcommittee and Conflict of Interest Statement	**She-Chia Chen, PharmD** Designated Federal Officer, ODAC
10:10 a.m.	Introductory Remarks	**Gregory Reaman, MD** Associate Director Pediatric Oncology Oncology Center of Excellence (OCE) Office of the Commissioner (OC) Associate Director Pediatric Oncology Office of Oncologic Diseases (OOD) Office of New Drugs (OND), CDER, FDA
10:15 a.m.	**FDA PRESENTATIONS**	
	FDA Perspective: Patient Self-Reporting in the Evaluation of Cancer Drug Tolerability	**Elizabeth S. Duke, MD** Medical Officer Division of Oncology 2 OOD, OND, CDER, FDA
	Patient-Reported Outcomes (PROs) in Pediatric Cancer Registration Trials – An FDA Perspective	**Meena N. Murugappan, PharmD, MPH** Research Fellow OCE, OC, FDA
10:50 a.m.	Clarifying Questions	

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AGENDA (cont.)

11:00 a.m.	**SPEAKER PRESENTATION**	
	Patient Self-Reporting and Cancer Drug Tolerability: Lessons Learned from the Adult Experience with PRO-CTCAE	**Lori Minasian, MD** Deputy Director Division of Cancer Prevention National Cancer Institute (NCI) National Institutes of Health
11:15 a.m.	**FDA PRESENTATION**	
	FDA's Project Patient Voice: Let the Children be Heard	**Vishal Bhatnagar, MD** Associate Director for Patient Outcomes OCE, OC, FDA
11:30 a.m.	**GUEST SPEAKER PRESENTATIONS**	
	Rationale for the Development of Pediatric PRO-CTCAE	**Pamela S. Hinds, PhD, RN, FAAN** Executive Director Department of Nursing Science, Professional Practice, and Quality Outcomes Research Integrity Officer Children's National Hospital Professor of Pediatrics School of Medicine and Health Sciences George Washington University
	Design and Evaluation of the Pediatric Patient-Reported Outcomes Version of the Common Terminology Criteria for Adverse Events (Ped-PRO-CTCAE™) System	**Bryce B. Reeve, PhD** Director, Center for Health Measurement Professor, Population Health Sciences Professor, Pediatrics Duke University School of Medicine
12:00 p.m.	Clarifying Questions	
12:20 p.m.	**LUNCH**	

PEDIATRIC PRO-CTCAE: FUTURE DIRECTIONS BEYOND TOLERABILITY

12:50 p.m.	**GUEST SPEAKER PRESENTATIONS**	
	Pediatric PRO-CTCAE: Future Directions Advancing Supportive Care Strategies - PROs	**Lillian Sung, MD, PhD, FRCPC** Professor, Department of Paediatrics Senior Scientist, Research Institute Canada Research Chair in Pediatric Oncology Supportive Care The Hospital for Sick Children Toronto, Canada
	Facilitating Survivorship Care and Research	**Tara O. Henderson, MD, MPH** Professor of Pediatrics Interim Chief, Pediatric Hematology, Oncology and Stem Cell Transplantation Director, Childhood, Adolescent and Young Adult Survivorship Center Comer Children's Hospital University of Chicago
	Incorporating Pediatric PRO-CTCAE in the National Clinical Trials Network (NCTN) Clinical Trials	**Douglas S. Hawkins, MD** Professor of Pediatrics University of Washington Chair, Children's Oncology Group Seattle Children's Hospital
1:35 p.m.	Clarifying Questions	
1:50 p.m.	**BREAK**	
2:00 p.m.	**OPEN PUBLIC HEARING**	
2:30 p.m.	Questions to the Subcommittee and Subcommittee Discussion	
3:00 p.m.	Wrap-Up	**Elizabeth Duke, MD**
3:10 p.m.	Closing Remarks	**Gregory Reaman, MD**
3:15 p.m.	**ADJOURNMENT**	

On May 12, 2021, the subcommittee will discuss real-world evidence (RWE) for regulatory use in pediatrics, real-world data (RWD) resources, and RWD and RWE to advance pediatric safety assessments of oncology drug products in children within the context of the FDA framework for RWE. Potential data sources and publicly available platforms, including those made possible through the development and implementation of the National Cancer Institute's Childhood Cancer Data Initiative, will be discussed. The potential for use of data sources to construct external controls to evaluate effectiveness of investigational products will be considered given the frequent dependence on single-arm studies due to extremely small study populations, now exaggerated by molecularly defined subtypes of the rare cancer types that occur in children.

Day 2: May 12, 2021

12:00 p.m.	Call to Order	**Alberto S. Pappo, MD** Chairperson, pedsODAC
12:05 p.m.	Introduction of Subcommittee and Conflict of Interest Statement	**She-Chia Chen, PharmD** Designated Federal Officer, ODAC
12:10 p.m.	Introductory Remarks	**Gregory Reaman, MD** Associate Director Pediatric Oncology Oncology Center of Excellence (OCE) Office of the Commissioner (OC) Associate Director Pediatric Oncology Office of Oncologic Diseases (OOD) Office of New Drugs (OND), CDER, FDA

REAL WORLD EVIDENCE (RWE) FOR REGULATORY USE IN PEDIATRICS

12:15 p.m. **FDA PRESENTATIONS**

The FDA Real World Evidence (RWE) Framework and Considerations for Use in Regulatory Decision-Making	**Jacqueline Corrigan-Curay, JD, MD** Director, Office of Medical Policy Acting Deputy Center Director for Operations, CDER, FDA
Designing External Controls Using Real World Data for Pediatric Cancer Drug Development	**Donna R. Rivera, PharmD, MSc** Associate Director for Pharmacoepidemiology OCE, OC, FDA

FDA PRESENTATIONS (CONT.)

Statistical Considerations for External Controls in Pediatric Trials	**Pallavi Mishra-Kalyani, PhD** Lead Mathematical Statistician Division of Biometrics V Office of Biostatistics Office of Translational Sciences CDER, FDA

12:45 p.m. Clarifying Questions

PEDIATRIC REAL WORLD DATA (RWD) RESOURCES

1:15 p.m. **SPEAKER PRESENTATIONS**

Childhood Cancer Data Initiative	**James H. Doroshow, MD** Deputy Director for Clinical and Translational Research, NCI National Institutes of Health (NIH) Director, Division of Cancer Treatment and Diagnosis, NCI, NIH
The NCI Childhood Cancer Data Initiative (CCDI) and RWD/RWE Resources for Pediatric Oncology	**Malcolm A. Smith, MD, PhD** Associate Branch Chief for Pediatrics Clinical Investigations Branch Cancer Therapy Evaluation Program Division of Cancer Treatment and Diagnosis (DCTD), NCI, NIH

2:15 p.m. Clarifying Questions

RWE IN EVALUATING PEDIATRIC DRUG SAFETY AND INFORMING RESEARCH STRATEGIES

2:30 p.m. **FDA PRESENTATION**

Real World Evidence (RWE) to Assess Pediatric Medical Product Safety	**Ann W. McMahon, MD, MS, FISPE** Deputy Director of Science Office of Pediatric Therapeutics Office of Clinical Policy and Programs OC, FDA

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2:40 p.m.	**GUEST SPEAKER PRESENTATIONS**	
	Using RWD and RWE to Evaluate Pediatric Cancer Drug Safety	**Bruce Carleton, BPharm, PharmD, FCP, FISPE** Professor of Pediatrics, Medical Genetics, Population & Public Health University of British Columbia Chair, Division of Translational Therapeutics, Department of Pediatrics Faculty of Medicine Director, Pharmaceutical Outcomes Programme, BC Children's Hospital Senior Clinician Scientist BC Children's Hospital Research Institute Vancouver, Canada
	Informing Pediatric Clinical Research Strategies and Drug Development Through RWE	**Douglas S. Hawkins, MD** Professor of Pediatrics University of Washington Seattle Children's Research Institute Chair, Children's Oncology Group
3:00 p.m.	Clarifying Questions	
3:15 p.m.	**BREAK**	
3:30 p.m.	**OPEN PUBLIC HEARING**	
4:00 p.m.	Questions to the Committee and Committee Discussion	
4:45 p.m.	**Closing Remarks**	**Gregory Reaman, MD**
5:00 p.m.	**ADJOURNMENT**	