Welcome to FDA's Rare Disease Day 2022 Virtual Public Meeting

Webcast link:

https://fda.yorkcast.com/webcast/Play/e6545934e2e64b24a798b698d1df12521d

Meeting Agenda

Agenda for FDA Rare Disease Day 2022: Friday, March 4, 2022 (virtual)
Sharing Experiences in Rare Diseases Together

9:00-9:05am **Welcome**

- Robert M. Califf, MD, MACC, FDA Commissioner
- Sandra Retzky, DO, JD, MPH, Director, Office of Orphan Products Development (OOPD), FDA

9:05-9:10am **Meeting Overview**

Lewis Fermaglich, MD, MHA, Medical Officer, OOPD, FDA

9:10-9:55am **Panel 1: Oncology Center for Excellence (OCE)**

A Glimpse into the Reviewer's Journey Leading to Approval of Two Drugs to Treat Rare Tumors

Session Goal/Overview:
In this session, FDA reviewers will discuss and provide perspectives on their role and experiences as researchers, oncologists, and FDA reviewers during the clinical development and application review for development programs leading to approval of two new drugs, selumetinib and tebentafusp, to treat rare tumors.

Moderator: Martha Donoghue, MD, Deputy Director, Division of Oncology 2, Office of Oncologic Diseases (OOD), CDER; and Acting Associate Director for Pediatric and Rare Cancer Drug Development, OCE, Office of the Commissioner, FDA

Panelists:

- Denise Casey, MD, MS, Pediatric Oncologist and former FDA clinical reviewer
- Diana Bradford, MD, Pediatric Oncologist and Cross Disciplinary Team Leader, Division of Oncology 2, OOD, CDER, FDA
- Jamie R. Brewer, MD, Oncologist and Acting Cross Disciplinary Team Leader, Division of Oncology 3, OOD, CDER, FDA
- Elizabeth Spehalski, PhD, Pharmacologist, Division of Hematology Oncology Toxicology, OOD, CDER, FDA

9:55-10:40am **Panel 2: Center for Biologics Evaluation and Research (CBER)**

Working Together and Sharing Experiences in the Review of Gene Therapies for Neurocognitive Disorders in Children

Session Goal/Overview:
In each medical product center at FDA, reviewers and other staff with different roles and expertise are assigned and work together as a team to review each medical product. If needed, a reviewer with special expertise from another part of FDA is added to the review team. This happens frequently, particularly in reviewing certain products for rare diseases. In this panel, three expert reviewers, two from CBER and one from CDER, will discuss their experiences in working together on review teams for gene therapy products for neurocognitive diseases in children. We will hear their perspective on the importance

of collaboration in review of these complex biological products for rare pediatric diseases.

Moderator: Victor Baum, MD, Medical Officer, Division of Blood Components and Devices, Office of Blood Research and Review (OBRR), CBER, FDA

Panelists:

- Elizabeth Hart, MD, Chief, General Medicine Branch I, Division of Clinical Evaluation and Pharmacology/Toxicology, Office of Tissues and Advanced Therapies (OTAT) CBER, FDA
- Naomi Knoble, PhD, Reviewer, Division of Clinical Outcome Assessment, Office of New Drugs, Center for Drug Evaluation and Research (CDER), FDA
- Andrew Byrnes, PhD, Chief, Gene Transfer and Immunogenicity Branch, Division of Cellular and Gene Therapies, OTAT, CBER, FDA

10:40-10:50am **Break**

10:50-11:35am **Panel 3: Center for Drug Evaluation and Research (CDER)**

Amyloidosis—Our journey to enhance product development using a public-private partnership approach

Session Goal/Overview:
CDER strives to involve academia, industry, and patient groups, as early as possible, in the drug development process for rare diseases. In this session, CDER highlights a collaborative effort among diverse stakeholders in a public-private partnership to advance drug development for amyloidosis, a rare disease. The audience will hear the lessons learned that could potentially be replicated for other rare diseases.

CDER Introduction: Kerry Jo Lee, MD – Associate Director for Rare Diseases, Rare Diseases Team, CDER/Office of New Drugs (OND)/ Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine (ORPURM)/Division of Rare Diseases and Medical Genetics , FDA

Moderator: Preston Dunnmon, MD, MBA, FACP, FACC, Cardiologist and former FDA Clinical Team Leader

Panelists:

- Rosalyn Adigun, MD, PharmD, MSc – Medical Officer, CDER/OND/ Office of Cardiology, Hematology, Endocrinology and Nephrology (OCHEN) / Division of Cardiology and Nephrology, FDA
- Mathew Maurer, MD –Professor of Medicine, Arnold and Arlene Goldstein Professor of Cardiology, Columbia University Irving Medical Center
- Kristen Hsu, Executive Director of Clinical Research - Amyloidosis Research Consortium
- James Signorovitch, PhD - Managing Principal, The Analysis Group

11:35-12:20pm **Panel 4: Center for Devices and Radiological Health (CDRH)**

The CDRH approach to rare diseases and the Patient Voice. Reviewer experiences with orthopedic Humanitarian Device Exemptions (HDE)

Session Goal/Overview:
Collaboration and engaging diverse groups are integral to the evaluation of medical devices performed by staff at the Center for Devices and Radiological Health (CDRH). In this session, CDRH will discuss patient science and engagement efforts, collaborative communities, and other activities that are critical to CDRH incorporating patient and other stakeholder input into CDRH decision making. Devices intended to diagnose or treat a condition that impacts 8,000 or less people in the US are often submitted to FDA under a Humanitarian Device Exemption (HDE). We will also discuss two orthopedic examples of HDEs to illustrate how we consider benefits and risks in our evaluation of devices used to treat people living with rare conditions.

Moderator: Michelle Tarver, MD, PhD, Deputy Director, Office of Strategic Partnerships and Technology Innovation, FDA

Panelists:

Patient Experience & Medical Devices: Advancing Efforts in Rare Diseases. Perspectives from the Center for Devices and Radiological Health (CDRH)

- Michelle Tarver, MD, PhD, Deputy Director, Office of Strategic Partnerships and Technology Innovation, FDA

Our experience with an orthopedic HDE for osteoid osteoma

- CDR Michel Janda, MS, Lead reviewer and Biomedical Engineer, FDA
- David Scott, MD, Medical Officer, FDA

Our experience with an orthopedic HDE for pediatric scoliosis devices

- Eileen Cadel, PhD, Lead Reviewer and Biomedical Engineer, FDA
- Caroline Moazzam, MD, Medical Officer, FDA

12:20pm-1:00pm **Lunch**

1:00-1:10pm **Introductory Remarks for the Afternoon**
Janet Woodcock, MD, Principal Deputy Commissioner of FDA

1:10-2:10pm **Panel 5: How FDA Engages Rare Disease Patients and Advocates**

Session Goal/Overview:
Patients provide a unique perspective about their health conditions and are an important part of FDA's public health mission. Through our patient engagement programs and activities, we listen closely to patients and caregivers to help inform medical product development, regulatory decision making, clinical trial design, and patient preferences. In this session, members of the rare disease patient community (patients, caregivers, patient organizations) will share their experiences engaging with patient-oriented programs and activities at FDA.

Moderator: Wendy Slavit, MPH, Health Programs Coordinator, Office of Patient Affairs, Office of the Commissioner

Panelists:

- Aviva Rosenberg, Gaucher Community Alliance
- Marc Yale, International Pemphigus and Pemphigoid Foundation

- Julie Breneiser, Gorlin Syndrome Alliance

2:10-2:20pm **Break**

2:20-3:20pm **Panel 6: Our Future Journey**

Session Goal/Overview:
Experts from each FDA Center will spotlight initiatives aimed to improve drug development for rare diseases.

Moderator: Sandra Retzky, DO, JD, MPH, Director, OOPD

Panelists:

Rare Diseases Cures Accelerator-Data and Analytics Platform (RDCA-DAP)
- Michelle Campbell, PhD, Sr. Clinical Analyst for Stakeholder Engagement and Clinical Outcomes, Office of Neuroscience, CDER, FDA

Future Directions: CBER Perspective
- Celia M. Witten, PhD, MD, Deputy Director, CBER, FDA

Applications of Real-World Data in Drug Development for Rare Cancers
- Donna R. Rivera, PharmD, MSc, Associate Director for Pharmacoepidemiology, OCE, FDA

Health Technology and Rare Diseases
- Sara Brenner, MD, MPH, Associate Director for Medical Affairs and Chief Medical Officer for In Vitro Diagnostics, CDRH, FDA

Computational re-positioning of approved drugs to treat rare diseases
- Weida Tong, PhD, Director of the Division of Bioinformatics and Biostatics, FDA National Center for Toxicological Research (NCTR), FDA

3:20-4:20pm **Open Public Comment Period**
Teresa Rubio, Health Science Administrator, PharmD, OOPD, FDA

4:20-4:30pm **Closing Remarks**
Sandra Retzky, DO, JD, MPH, Director, OOPD, FDA