As of: 11/06/2023

NATIONAL ACADEMIES Sciences Engineering Medicine

BOARD ON HEALTH SCIENCES POLICY

Committee on Processes to Evaluate the Safety and Efficacy of Drugs for Rare Diseases in the United States and the European Union

Meeting #1: November 6 – 7, 2023: Public Agenda

Please note that the Agenda posted below may be subject to change. Changes, if any, will be posted along with the date of last update.

MONDAY, NOVEMBER 6, 2023

1:00 – 2:15 PM EST: CLOSED SESSION – COMMITTEE MEMBERS ONLY

2:15–2:30 pm ***BREAK***

2:30 – 4:30 PM: OPEN SESSION

2:30–2:35 pm **Welcome and Introductions**

JEFFREY KAHN, *Committee Chair*
Andreas C. Dracopoulos Director
Robert Henry Levi and Ryda Hecht Levi Professor of Bioethics and Public Policy
John Hopkins Berman Institute of Bioethics

2:35–3:30 pm **Sponsor Perspective and Charge to the Committee**

KERRY JO LEE
Associate Director for Rare Diseases, Division of Rare Diseases and Medicine Genetics
Office of Rare Diseases, Pediatrics, Urological, and Reproductive Medicines
Office of New Drugs
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

SANDRA RETZKY
Director, Office of Orphan Product Development
Office of the Commissioner
U.S. Food and Drug Administration

MIRANDA RAGGIO
Expedited Programs Manager, Office of Program Operation
Office of New Drugs
Center for Drugs Evaluation and Research
U.S. Food and Drug Administration

JULIENNE VAILLANCOURT
Policy Advisor and Rare Disease Laison
Office of the Director
Center for Biologics Research and Evaluation
U.S. Food and Drug Administration

KATHERINE TYNER
FDA Liaison to the European Medicines Agency, Europe Office
Office of Global Policy and Strategy
Office of the Commissioner
U.S. Food and Drug Administration

SARAH ZAIDI
Physician Liaison for Pediatric Cluster, Pediatric International Team
Office of Pediatric Therapeutics
Office of Clinical Policy and Programs
Office of the Commisssioner
U.S. Food and Drug Administration

Other Sponsor Stakeholders on Standby for Q&A

JUDITH ARCIDIACONO
International Regulatory Expert, Office of Therapeutic Products
Center for Biologics Research and Evaluation
U.S. Food and Drug Administration

ROBYN BENT
Director, Patient-Focused Drug Development Program
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

KEVIN FAIN
Senior Policy Advisor, Office of New Drug Policy
Office of New Drugs
Center for Drug Research and Evaluation
U.S. Food and Drug Administration

DIONNE L. PRICE
Deputy Director, Office of Biostatistics
Office of Translational Sciences
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

3:30–4:30 pm **Discussion with Committee**

4:30 pm **ADJOURN MEETING DAY 1**

TUESDAY, NOVEMBER 7, 2023

9:00 – 9:45 AM EST: CLOSED SESSION – COMMITTEE MEMBERS ONLY

9:45–10:00 am ***BREAK***

10:00 AM – 1:00 PM EST: OPEN SESSION

10:00 am **Welcome and Introductions**

JEFFREY KAHN, *Committee Chair*
Andreas C. Dracopoulos Director
Robert Henry Levi and Ryda Hecht Levi Professor of Bioethics and Public Policy
Johns Hopkins Berman Institute of Bioethics

10:05–10:30 am **Trends in Rare Disease Drug Product Approvals and Utilization of Regulatory Pathways**

ANNA SOMUYIWA
Head
Centre for Innovation in Regulatory Science

MAGDA BUJAR
Senior Manager, Regulatory Programme and Strategic Partnerships
Centre for Innovation in Regulatory Science

JUAN LARA
Research Analyst
Centre for Innovation in Regulatory Science

10:30–11:00 am **Discussion with Committee**

11:00–11:15 am ***BREAK***

11:15 am –12:30 pm **Perspectives from Rare Disease Organizations**

VIRGINIE HIVERT
Therapeutic Development Director
EURODIS

SAIRA SULTAN
Consultant
Haystack Project

ANNIE KENNEDY
Chief of Policy, Advocacy, and Patient Engagement
EveryLife Foundation

KARIN HOELZER
Director of Policy and Regulatory Affairs
NORD

12:30–12:45 pm **Public Comment**

Public comments will provide the committee with additional insight into key issues related to the study's statement of task. These include, but are not limited to:

- The use of regulatory flexibilities and supplementary data (e.g. natural history studies and patient registries) when evaluating the safety and efficacy of drugs for rare diseases and conditions; and
- FDA and EMA engagement of people with lived experience when developing guidance, policies and programs.

Public comments, alongside other materials stakeholders have shared, will be reviewed by the committee and may help inform committee deliberations on the statement of task**.** All comments and materials shared with the committee will be made publicly available in accordance with institutional policies. **As such, please do not send confidential or HIPAA protected information and take caution when including personally identifiable information.** Should a quote from your public comment be used word-for-word in the committee's final report, you will be contacted by study staff.

If you would like to provide a verbal comment, please limit remarks to 2-3 minutes. Requests to provide verbal public comments may submitted via the meeting registration page **here**. Public commenters will be added to the agenda based on the order in which requests are received. Please note that space is limited and not all requests may be fulfilled. **You may also submit a written public comment via email: RareDiseaseregPolicyStudy@nas.edu**.

Public comments made at meetings and submitted in writing are subject to the same institutional disclosure requirements.

12:45pm ***ADJOURN OPEN SESSION***

1:00 PM – 3:30 PM EST: CLOSED SESSION – COMMITTEE MEMBERS ONLY

3:30 pm **ADJOURN MEETING**