

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

Meeting #2: December 4 – 5, 2023: Committee 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, DECEMBER 4, 2023

CLOSED SESSION (9:00 – 9:45 AM ET) – COMMITTEE MEMBERS ONLY

9:45–10:00 am BREAK

OPEN SESSION (10:00 AM – 1:30 PM ET)

10:00–10:10 am Welcome and Introduction

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

10:10–11:15 am EMA Regulatory Policies for Drugs to Treat Rare Diseases and Conditions

STEFFEN THIRSTRUP
Chief Medical Officer
European Medicines Agency

Committee Discussion (30 min)

11:15 am–12:00 pm Panel Discussion: Similarities and Differences Between the FDA and EMA

STEFFEN THIRSTRUP
Chief Medical Officer
European Medicines Agency

JACQUELINE CORRIGAN-CURAY
Principal Deputy Center Director
Center for Drug Evaluation and Research
U.S. Food and Drug Administration

CELIA WITTEN
Deputy Director
Center for Biologics Evaluation and Research
U.S. Food and Drug Administration

12:00–12:15 pm BREAK

12:15–1:15 pm Industry Perspectives on the Application of Regulatory Flexibilities

LUCY VERESHCHAGINA
Senior Vice President, Science and Regulatory Advocacy
PhRMA

DIEGO ARDIGÒ
Head of R&D, Global Rare Diseases
Cheisi

E'LISSA FLORES
Director, Science and Regulatory Affairs
BIO

VICTOR MAERTENS
Government Affairs Director
EUROPE

Committee Discussion (20 min)

1:15–1:30 pm Public Comment

1:30 pm ADJOURN OPEN SESSION

CLOSED SESSION (2:30 PM – 5:00 PM ET) – COMMITTEE MEMBERS ONLY

5:00 pm ADJOURN DAY 1

TUESDAY, DECEMBER 5, 2023

CLOSED SESSION (9:00 – 9:30 AM ET) – COMMITTEE MEMBERS ONLY

9:30–10:00 am BREAK

OPEN SESSION (10 AM – 12:30 PM ET)

10:00–10:05 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

10:05–11:00 am Use of “Supplemental Data” for Regulatory Decision-Making

Case Study: Skyclarys approval for Friedreich’s ataxia (Use of natural history data)
COLIN MEYER (Formerly at Reata Pharmaceuticals Inc.)
Biogen

Case Study: Elevidys approval for Duchenne’s muscular dystrophy (Challenges of regulatory review without “supplemental data”)
HUONG HUYNH
Director, Regulatory Science
Critical Path Institute

Case Study: Relyvrio approval for ALS (Use of natural history and open label extension [active treatment extension] data)

SABRINA PAGANONI
Physician Scientist
Healey & AMG Center for ALS

Case Study: Oxbyrta approval for Sickle Cell Disease (Use of natural history data)

LAKIEA BAILEY
Executive Director
Sickle Cell Community Consortium

Committee Discussion (20 min)

11:00–11:15 am ***BREAK***

11:15 am– **Trends in Designation and Approvals of Drugs for Rare Diseases and Conditions**
12:00 pm

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

LEWIS FERAGLICH
Medical Officer, Office of Orphan Product Development
Office of the Commissioner
U.S. Food and Drug Administration

Committee Discussion (15 min)

12:00–12:30 pm **Impact of FDA and EMA Collaborative Efforts**

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

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

STEFFEN THIRSTRUP
Chief Medical Officer
European Medicines Agency

Committee Discussion (15 min)

12:30 pm ***ADJOURN OPEN SESSION***

CLOSED SESSION (1:30 – 4:00 PM ET) – COMMITTEE MEMBERS ONLY

4:00 pm ***ADJOURN MEETING***