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 #3: February 6 – 7, 2024: Committee Agenda

TUESDAY, FEBRUARY 6, 2024

CLOSED SESSION (8:30 – 9:30 AM ET) – COMMITTEE MEMBERS ONLY

9:45—10:00 am **BREAK**

OPEN SESSION (10:00 AM – 2:30 PM ET)

10:00–10:05 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

10:05–11:00 am **Trial Design for Rare Disease Drug Development**

LONGITUDINAL TRIAL DESIGN
Tiina Urv
Director, Rare Disease Clinical Trial Network
National Center for Advancing Translational Sciences
National Institutes of Health

MASTER PROTOCOLS
NICOLE MAYER HAMBLETT
Associate Professor of Pediatrics & Adjunct Associate Professor of Biostatistics, University of Washington
Co-Executive Director, Cystic Fibrosis Therapeutics Development Network Coordinating Center

EXTERNAL CONTROLS
WILLIAM MAIER
Vice President, Rare Diseases
ICON plc

Committee Discussion (30 min)

11:00 am – 12:00pm **Considerations for Pediatric Trials**

INDUSTRY PERSPECTIVE
THOMAS MILLER

Global Head of the Acute, Chronic, and Pediatric Disease Nucleus
Bayer, Pharmaceutical Division

CAREGIVER PERSPECTIVE
KARA BERASI
CEO, Haystack Project
Vice Chair of Board of Directors, CDG Care (Congenital Disorders of Glycosylation)

REGULATORY SCIENCE PERSPECTIVE
FLORENCE BOURGEOIS
Associate Professor of Pediatrics, Harvard Medical School
Co-Director, Harvard-MIT Center for Regulatory Science
Director, Pediatric Therapeutics and Regulatory Science Initiative, Boston Children's Hospital

Committee Discussion (30 min)

12:00–1:00 pm **LUNCH BREAK**

1:00-2:00 pm **Use of "Supplemental" Data**

USE OF AGGREGATE DATA
KLAUS ROMERO
Chief Executive Officer & Chief Science Officer
Critical Path Institute

EXPANDED ACCESS PROGRAMS
ALISON BATEMAN-HOUSE
Assistant Professor, Department of Population Health
New York University Grossman School of Medicine

PATIENT REGISTRIES AND NATURAL HISTORY DATA
EDWARD NEILAN
Chief Medical and Scientific Officer
National Organization for Rare Disorders

Committee Discussion (30 min)

2:00 pm **ADJOURN OPEN SESSION**

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

5:30 pm **ADJOURN DAY 1**

WEDNESDAY, FEBRUARY 7, 2024

CLOSED SESSION (8:30 – 9:30 AM ET) – COMMITTEE MEMBERS ONLY

9:15–9:30 am **BREAK**

OPEN SESSION (9:30 AM – 12:15 PM ET)

9:30–9:35 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

9:35–10:30 am **Novel Methodologies**

ANALYSIS METHODS – BAYESIAN METHODS AND SMART DESIGN
KELLEY KIDWELL
Professor of Biostatistics
University of Michigan

ANALYSIS METHODS – CAUSAL INFERENCE
XABIER GARCIA DE ALBINEZ MARTINEZ
Director of Epidemiology, RTI Health Solutions
Visiting Scientist, Department of Epidemiology, Harvard T.H. Chan School of Public Health

MODEL-INFORMED DRUG DEVELOPMENT
Hao Zhu
Division Director
Division of Pharmacometrics, Office of Combination Products, Office of Translational Sciences
Center for Drug Evaluation and Research
US Food and Drug Administration

Committee Discussion (30 min)

10:30–10:45 am ***BREAK***

10:45–
12:00 pm **Flexibilities Applied at FDA**

EMILY FREILICH
Division Director of Division of Neurology I
Center for Drug Evaluation and Research
US Food and Drug Administration

RACHAEL ANATOL
Deputy Director of Office of Therapeutic Products
Center for Biologics Evaluation and Research
US Food and Drug Administration

MARTHA DONOGHUE
Associate Director of Pediatric Oncology and Rare Cancers
Oncology Center of Excellence
US Food and Drug Administration

12:00–12:15pm **Public Comment**

12:15 pm ***ADJOURN OPEN SESSION***

CLOSED SESSION (1:00 – 5:00 PM ET) – COMMITTEE MEMBERS ONLY

5:00 pm **ADJOURN MEETING**