JUNE 17—DAY 3: Regulatory and Equitable Access

12:00 p.m.  Welcome Remarks
Diana Bianchi, M.D., Director, Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), National Institutes of Health (NIH)

12:10 p.m.  Day 3 Overview
Robert Green, M.D., M.P.H., Professor of Medicine, Harvard
Tippi MacKenzie, M.D., Professor of Surgery, University of California, San Francisco

12:25 p.m.  Bioethics, Regulatory, and Equity Panel
This session will be focused on the ethical implications of gene-targeted therapies, regulatory challenges and pathways to gene-targeted therapies, and barriers to equitable access to gene-targeted therapies.

Moderator:
•  Jill Morris, Ph.D., Program Director, Division of Neuroscience, National Institute of Neurological Disorders and Stroke (NINDS), NIH

Panelists:
•  R. Alta Charo, J.D., Professor of Law and Bioethics, University of Wisconsin at Madison
•  Peter Marks, M.D., Ph.D., Director, Center for Biologics Evaluation and Research (CBER), Food and Drug Administration (FDA)
•  Vence Bonham, J.D., Senior Advisor to the NHGRI Director on Genomics and Health Disparities, National Human Genome Research Institute (NHGRI), NIH

1:35 p.m.  Lessons from the Frontlines: Patient Advocacy for MLD Gene Replacement Therapy
Maria Kefalas, Ph.D., Author, Advocate, and Philanthropist, The Calliope Joy Foundation and Cure MLD

1:50 p.m.  Genomics England
Professor Sir Mark Caulfield, M.D., FRCP, FESC, FPharm, FBHS, FMedSci, Chief Scientist, Genomics England

2:15 p.m.  Break
2:10 p.m. **Working Group 3 Presentations**

**Introduction**
Lily J. Lou, M.D., Professor of Clinical Pediatrics; Director of Government Relations, Department of Pediatrics, University of Illinois, Chicago

R. Rodney Howell, M.D., FAAP, FACMG, Emeritus Professor; Chairman of Pediatrics; Emeritus Member, Hussman Institute for Human Genomics, University of Miami Miller School of Medicine

- Equitable Access to Therapies
  - Sara F. Goldkind, M.D., M.A., Research and Clinical Bioethics Consultant, Goldkind Consulting, LLC
  
  - R. Rodney Howell, M.D., FAAP, FACMG, Emeritus Professor; Chairman of Pediatrics; Emeritus Member, Hussman Institute for Human Genomics, University of Miami Miller School of Medicine

- Federal Regulations/Universal Screening/Identification
  - Aaron Goldenberg, Ph.D., Vice-Chair and Associate Professor, Department of Bioethics, Case Western Reserve University School of Medicine
  
  - Lily J. Lou, M.D., Professor of Clinical Pediatrics; Director of Government Relations, Department of Pediatrics, University of Illinois, Chicago
  
  - Annie Kennedy, Chief of Policy and Advocacy, EveryLife Foundation for Rare Diseases

- Funding Innovations/Incentives
  - Patricia Deverka, M.D., Executive Director, Deverka Consulting, LLC

3:25 p.m. **Questions and Open Discussion**

Moderator:

- Jill Morris, Ph.D., Program Director, Division of Neuroscience, NINDS, NIH

*To submit questions or comments:*

1. Email ORDR@nih.gov
2. Send Live Feedback via Videocast
3. Tweet: #NIHGTIImtg

4:15 p.m. **Closing Remarks**

Tiina K. Urv, Ph.D., Program Director, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Science (NCATS), NIH

Robert Green, M.D., M.P.H., Professor of Medicine, Harvard

4:30 p.m. **Adjourn**