Gene-Targeted Therapies: Early Diagnosis and Equitable Delivery

June 3 from 12-4:30 p.m. EDT (The Who, What, and When)
June 10 from 12-4:30 p.m. EDT (Infrastructure and Mechanics)
June 17 from 12-4:30 p.m. EDT (Regulatory and Equitable Access)

Draft as of 6/1/2021

JUNE 17—DAY 3: Regulatory and Equitable Access

12:00 p.m. ET  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., MPH, Professor of Medicine, Harvard
Tippi MacKenzie, M.D., Professor of Surgery, University of California, San Francisco

12:15 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

1:20 p.m.  Patient Story
Maria Kefalas, Ph.D., Author, Advocate, and Philanthropist, The Calliope Joy Foundation and Cure MLD

1:35 p.m.  England’s Program
Professor Sir Mark Caulfield, MD, FRCP, FESC, FPharm, FBHS, FMedSci, Chief Scientist, Genomics England

1:50 p.m.  Break
2:00 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
- Funding Innovations/Incentives
- Federal Regulations/ Universal Screening/Identification
- Partnerships and Resources

3:40 p.m. **Questions and Open Discussion**

To submit questions or comments:
1. **Email** ORDR@nih.gov
2. **Send Live Feedback via Videocast**
3. **Tweet**: #NIHGTTmtg

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

*Robert Green, M.D., MPH*, Professor of Medicine, Harvard

*Tippi MacKenzie, M.D.*, Professor of Surgery, University of California, San Francisco

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