#NIHGTTmtg

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)

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: #NIHGTTmtg*

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**