

# The Growing Promise of Gene Therapy Approaches to Rare Diseases

August 20–21, 2018

National Institutes of Health • Bethesda, Maryland

## AGENDA

### Day 1 — Monday, August 20

- 8:00 a.m. Registration**
- 8:30 a.m. Welcome and Overview for Day 1**  
Wilson Bryan, M.D., Director, Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER), Food and Drug Administration (FDA)  
Anne Pariser, M.D., Director, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)
- 8:45 a.m. NCATS/NIH Remarks**  
Christopher Austin, M.D., Director, NCATS, NIH
- 9:00 a.m. CBER/FDA Remarks**  
Peter Marks, M.D., Director, CBER, FDA
- 9:15 a.m. American Society of Gene & Cell Therapy (ASGCT) Remarks**  
Barry Byrne, M.D., Ph.D., Director, University of Florida Powell Gene Therapy Center (UF PGTC); Professor, Pediatrics and Molecular Genetics and Microbiology, UFHealth
- 9:30 a.m. Session 1: Pre-Clinical Development**  
The participants in this session will discuss pre-clinical work of delivery systems (such as adeno-associated virus [AAV], lentivirus, etc.), cell-based, and gene editing approaches that led to the gene therapies currently marketed or in clinical development.
- Session Chair: **Stephen G. Kaler**, M.D., Senior Investigator, Section on Translational Neuroscience, *Eunice Kennedy Shriver* National Institute for Child Health and Human Development (NICHD), NIH
- Session Co-Chair: **Charles Venditti**, M.D., Ph.D., Medical Genomics and Metabolic Genetics Branch, National Human Genome Research Institute (NHGRI), NIH
- Speakers/Panelists: **John (Jay) Chiorini**, Ph.D., Chief, Adeno-Associated Virus Biology Section, National Institute of Dental and Craniofacial Research (NIDCR), NIH  
**Leonela Amoasii**, Ph.D., University of Texas (UT) Southwestern Medical Center  
**Harry Malech**, M.D., Chief, Genetic Immunotherapy Section National Institute of Allergy and Infectious Diseases (NIAID), NIH  
**Iwen Wu**, Ph.D., Branch Chief, Pharmacology/Toxicology Branch 2 (PTB2), Division of Clinical Evaluation and Pharmacology/Toxicology (DCEPT), OTAT, CBER, FDA
- 11:30 a.m. Lunch** (on your own)



**12:30 p.m. Session 2: Clinical Development from Bench-to-Bedside**

The participants in this session will discuss clinical development strategies including issues such as the route of administration, distribution, and integration if applicable.

Session Chair: **Jerry R. Mendell**, M.D., Director, Center for Gene Therapy, Nationwide Children's Hospital

Session Co-Chair: **Reed Clark**, Ph.D., Senior Vice President, Ultragenyx Gene Therapy

Speakers/Panelists: **Jerry R. Mendell**, M.D., Director, Center for Gene Therapy, Nationwide Children's Hospital

**Chester Whitley**, M.D., Ph.D., Professor, Department of Pediatrics and Experimental and Clinical Pharmacology, University of Minnesota

**Doug Martin**, Ph.D., Professor, Department of Anatomy, Physiology, and Pharmacology, Auburn University

**Florian Eichler**, M.D., Director, Center for Rare Neurological Diseases, Massachusetts General Hospital and Associate Professor of Neurology, Harvard Medical School

**Theresa Chen**, Ph.D., Pharmacology/Toxicology Reviewer, DCEPT, OTAT, CBER, FDA

**Tejashri Purohit-Sheth**, M.D., Director, DCEPT, OTAT, CBER, FDA

**2:30 p.m. Break**

**2:45 p.m. Session 3: Quality and Manufacturing**

The participants in this session will discuss issues related to manufacturing, good manufacturing practice (GMP) standards, and scalability, discussing the transfer from academic to large facility settings and identifying the gaps.

Session Chair: **R. Jude Samulski**, Ph.D., Professor, Department of Pharmacology, University of North Carolina School of Medicine

Session Co-Chair: **Andrew Byrnes**, Ph.D., Branch Chief, Gene Transfer and Immunogenicity Branch, CBER, FDA

Speakers/Panelists: **Ken Cornetta**, M.D., Professor, Clinical Medical and Molecular Genetics, Indiana University School of Medicine

**Jaysson Eicholtz**, M.S., Director, GMP Operations, Nationwide Children's Hospital

**Josh Grieger**, Ph.D., Chief Technology Officer, Asklepios BioPharmaceutical Inc.

**Richard Snyder**, Ph.D., Chief Scientific Officer, Brammer Bio

**Denise Gavin**, Ph.D., Branch Chief, Gene Therapy Branch, OTAT, CBER, FDA

**4:45 p.m. Adjournment**

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### Day 2 — Tuesday, August 21

**8:30 a.m. Welcome and Overview for Day 2**

Wilson Bryan, M.D., and Anne Pariser, M.D.

**8:40 a.m. Session 4: Partnerships and Transitions**

The participants in this session will discuss the hand-offs between discovery research based at academic institutions, start-up companies, biotech companies, and pharma with focus on patient engagement and frameworks for intellectual property and conflict of interest management.

Session Chair: **Lynne F. McGrath**, M.P.H., Ph.D., Regulatory Affairs Consultant-Genetic Therapy

Session Co-Chair: **Brian Halak**, Ph.D., Chief Executive Officer, WindMIL Therapeutics, Inc.

Speakers/Panelists: **Walter Kowtoniuk**, Ph.D., Principal, Third Rock Ventures  
**R. Scott McIvor**, Ph.D., Professor of Genetics, Cell Biology and Development, Center for Genome Engineering, University of Minnesota  
**Nora N. Yang**, Ph.D., Senior Scientist, Therapeutics for Rare and Neglected Diseases; Director, Portfolio Management and Strategic Operations, Division of Pre-Clinical Innovation, NCATS, NIH  
**Debra Miller**, Chief Executive Officer and Founder, CureDuchenne

**10:10 a.m. Break**

**10:30 a.m. Session 5: Orphan Designation, Patient Access, and Business Models in Gene Therapy**

The participants in this session will discuss potential topics such as increasing development efficiency, leveraging private-public partnerships, developing value-based reimbursement models, and managing patient expectations.

Session Chair: **Katherine A. High**, M.D., President and Head of Research and Development, Spark Therapeutics

Session Co-Chair: **Marlene Haffner**, M.D., M.P.H., Chief Executive Officer, Haffner Associates



Speaker/Panelists: **Ilan Irony**, M.D., Deputy Director, DCEPT, CBER, FDA  
**Dawn Rotellini**, Senior Vice President for Program Development, National Hemophilia Foundation  
**Kristin Smedley**, President, Curing Retinal Blindness Foundation  
**Eric Auger**, Partner/Chair of Partnership Committee, Putnam Associates  
**Marianne Hamilton Lopez**, Ph.D., M.P.A., Research Director, Value-Based Payment Reform, Duke-Margolis Center for Health Policy  
**Raj Puri**, M.D., Ph.D., Director, Division of Cellular and Gene Therapies, CBER, FDA

**12:00 p.m.**     **Lunch** (on your own)

**1:00 p.m.**     **Session 6: No Disease Left Behind**

The participants in this session will discuss innovative methods and frameworks that could help bring access to gene therapies for diseases affecting such small numbers of patients that standard business models are not feasible.

Session Co-Chairs: **Barry Byrne**, M.D., Ph.D., Director, UF PGTC; Professor, Pediatrics and Molecular Genetics and Microbiology, UFHealth  
**PJ Brooks**, Ph.D., Program Director, ORDR, NCATS, NIH

Speakers/Panelists: **Barry Byrne**, M.D., Ph.D., Director, UF PGTC; Professor, Pediatrics and Molecular Genetics and Microbiology, UFHealth  
**Steven Gray**, Ph.D., Associate Professor, Department of Pediatrics, UT Southwestern Medical Center  
**Keith Wonnacott**, PhD, Executive Director, Regulatory Affairs, Pfizer  
**Scott Dorfman**, President and Chief Executive Officer, Odylia Therapeutics  
**Rachel Witten**, Medical Officer, OTAT, CBER, FDA

**3:00 p.m.**     **Open Floor Discussion with Questions & Answers for Panelists**

**4:00 p.m.**     **Adjournment**