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**
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-Gene 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
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