The Growing Promise of Gene Therapy Approaches to Rare Diseases

Day 1: August 20, 2018

8:00 a.m. **Registration**

8:30 a.m. **Welcome and Overview for Day 1**
Wilson Bryan, MD, Director, Office of Tissues and Advanced Therapies (OTAT), Center for Biologics Evaluation and Research (CBER), Food and Drug Administration (FDA) & Anne Pariser, MD, Director, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

8:40 a.m. **NCATS/NIH Remarks**
Christopher Austin, MD, Director, NCATS, NIH

8:50 a.m. **CBER/FDA Remarks**
Peter Marks, MD, Director, CBER, FDA

9:00 a.m. **ASGCT Remarks**
Barry Byrne, MD PhD, Director UF Powell Gene Therapy Center, Professor Pediatrics and Molecular Genetics & Microbiology, University of Florida Health

9:10 a.m. **Session 1: Pre-Clinical Development**
This session will discuss pre-clinical work of delivery systems (such as 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**, MD, NIH Eunice Kennedy Shriver National Institute for Child Health and Human Development (NICHD)

Session Co-Chair: **Charles Venditti**, MD PhD, NIH National Human Genome Research Institute (NHGRI)

Speakers/Panelists: **Jay Chiorini**, PhD, NIH National Institute of Dental and Craniofacial Research (NIDCR)
Leonela Amoasii, PhD, UT Southwestern
Harry Malech, MD, NIH National Institute of Allergy and Infectious Diseases (NIAID)
Iwen Wu, PhD, CBER FDA

10:40 a.m. **Break**

11:00 a.m. **Session 2: Clinical Development from Bench-to-Bedside**
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**, MD, Director of Center for Gene Therapy, Nationwide Children's Hospital
Session Co-Chair: **Terence R. Flotte**, MD, Professor, Dean of the School of Medicine, Provost and Executive Deputy Chancellor, University of Massachusetts

Speakers/Panelists: **Chester Whitley**, MD PhD, Professor, Department of Pediatrics, and Experimental and Clinical Pharmacology, University of Minnesota
Doug Martin, PhD, Professor, Department of Anatomy, Physiology and Pharmacology, Auburn University
Florian Eichler, MD, Director, Center for Rare Neurological Diseases, Massachusetts General Hospital and Associate Professor of Neurology, Harvard Medical School
Theresa Chen, PhD, CBER FDA
Tejashri Purohit-Sheth, MD, CBER FDA

12:30 p.m. **Lunch** *(on your own)*

1:30 p.m. **Session 3: Clinical Development Logistics and Practical Considerations**
This session will examine issues in trial design, international collaborations, and long-term surveillance.

Session Chair: **Marshall L. Summar**, MD, Children's National Medical Center
Session Co-Chair: TBD

Speakers/Panelists: **Lei Xu**, MD, Branch Chief of General Medicine, CBER FDA
TBD

3:00 p.m. **Break**

3:30 p.m. **Session 4: Quality and Manufacturing**
This session will discuss issues related to manufacturing, GMP standards, and scalability; discussing the transfer from academic to large facility settings and identifying the gaps.

Session Chair: **R. Jude Samulski**, PhD, Professor, Department of Pharmacology University of North Carolina School of Medicine
Session Co-Chair: **Andrew Byrnes**, PhD, CBER FDA

Speakers/Panelists: **Ken Cornetta**, MD, Professor of Clinical Medical & Molecular Genetics, Indiana University of Medicine

Jaysson Eicholtz, MS, Director of GMP Operations, Nationwide Children's Hospital
Josh Grieger, PhD, Chief Technology Officer, Asklepios BioPharmaceutical Inc.
Richard Snyder, PhD, Chief Scientific Officer, Brammer Bio
Denise Gavin, PhD, CBER FDA

5:00 p.m. **Adjournment for Day 1**

Day 2: August 21, 2018

8:30 a.m. **Welcome and Overview for Day 2**
Wilson Bryan, MD and Anne Pariser, MD

8:40 a.m. **Session 5: Partnerships and Transitions**
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**, PhD, VP Regulatory Affairs, RegenxBio
Session Co-Chair: **Erik Lium**, PhD, Senior Vice President, Mount Sinai Innovation Partners

Speakers/Panelists: **Brian Halak**, PhD, CEO, Windmill Therapeutics, Inc.
R. Scott McIvor, PhD, Professor of Genetics, Cell Biology and Development, Center for Genome Engineering, University of Minnesota
Nora N. Yang, PhD, Senior Scientist, Therapeutics for Rare and Neglected Diseases, Director, Portfolio Management and Strategic Operations Division of Pre-Clinical Innovation, NCATS, NIH
Debra Miller, CEO and Founder CureDuchenne

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

10:30 a.m. **Session 6: Business Models and Patient Access**
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**, MD, President and Head of Research and Development, Spark Therapeutics
Session Co-Chair: **Marlene Haffner**, MD MPH, CEO Haffner Associates

Speaker/Panelists: **Eric Auger**, Partner/Chair of Partnership Committee, Putnam Associates
Greg Daniel, PhD MPH RPh, Deputy Director and Clinical Professor, Duke-Margolis Center for Health Policy
Dawn Rotellini, SVP for Program Development, National Hemophilia Foundation
Kristin Smedley, President, Curing Retinal Blindness Foundation
Ilan Irony, MD, Acting Director, FDA Office of Orphan Products Development
Raj Puri, MD PhD, Director, Division of Cellular and Gene Therapies CBER FDA

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

1:00 p.m. **Session 7: "No Disease left behind; No patient left behind" and/or "Into the Future: Gene Therapy as a Precision Medicine Tool"**
This session will discuss use cases and ideas for innovative methods and frameworks that could help bring access to gene therapies to more patients with diseases on the long list of conditions with unmet need. This session will also look towards the future of gene therapy approaches for more common, or polygenic diseases.

Session Co-Chairs: **Barry Byrne**, MD PhD, Director UF Powell Gene Therapy Center, Professor Pediatrics and Molecular Genetics & Microbiology, University of Florida Health
PJ Brooks, PhD, Program Director, Division of Clinical Innovation, NCATS, NIH

Speakers/Panelists: **Barry Byrne**, MD PhD, Director UF Powell Gene Therapy Center, Professor Pediatrics and Molecular Genetics & Microbiology, University of Florida Health
Steven Gray, PhD, Assistant Professor, Department of Ophthalmology, University of North Carolina Gene Therapy Center
Sharon Hesterlee, PhD, Director Gene Therapy, Pfizer

3:00 p.m. **Open floor discussion with Q&A for panelists**

4:00 p.m. **Adjournment for Day 2**