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, 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)
Harry Malech, MD, NIH National Institute of Allergy and Infectious Diseases (NIAID)
Leonela Amoasii, PhD, UT Southwestern
Iwen Wu, PhD, FDA CBER

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, Nationwide Children’s Hospital  
**Session Co-Chair:** Terence R. Flotte, MD, University of Massachusetts  
**Speakers/Panelists:** Chet Whitley, MD PhD, University of Minnesota  
Doug Martin, PhD, Auburn University  
Florian Eichler, MD, Massachusetts General/Harvard University  
Theresa Chen, PhD, FDA CBER  
Tejashri Purohit-Sheth, MD, FDA CBER  

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, FDA CBER, Branch Chief of General Medicine  
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, UNC School of Medicine  
**Session Co-Chair:** Andrew Byrnes, PhD, FDA CBER  
**Speakers/Panelists:**  
Ken Cornetta, MD, Indiana University of Medicine  
Jaysson Eicholtz, MS, Director of GMP Operations, Nationwide Children’s Hospital  
Josh Grieger, PhD, Asklepios BioPharmaceutical Inc.  
Richard Snyder, PhD, Brammer Bio  
Denise Gavin, PhD, FDA CBER  

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, Putnam Associates
Greg Daniel, PhD MPH RPh, Duke-Margolis Center for Health Policy
Gumei Liu, MD PhD, FDA Office of Orphan Products
Raj Puri, MD PhD, FDA CBER
Speaker on patient access.

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, University of Florida Health
PJ Brooks, PhD, NCATS, NIH

Speakers/Panelists: TBD

3:00 p.m. Open floor discussion with Q&A for panelists
4:00 p.m. Adjournment for Day 2