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-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

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**