# TABLE OF CONTENTS

<table>
<thead>
<tr>
<th>Section</th>
<th>Page</th>
</tr>
</thead>
<tbody>
<tr>
<td>Program Schedule</td>
<td>2</td>
</tr>
<tr>
<td>Co-Chairs</td>
<td>3</td>
</tr>
<tr>
<td>Speakers</td>
<td>3</td>
</tr>
<tr>
<td>Speaker Biographies</td>
<td>4</td>
</tr>
<tr>
<td>Disclosure of Relevant Financial Relationships</td>
<td>6</td>
</tr>
</tbody>
</table>
PROGRAM SCHEDULE

All times are listed in Eastern Daylight Time (EDT UTC -4).

1:00 PM - 5:00 PM

Room: 302
CO-CHAIRS: Maria L. Escolar, MD and Timothy J. Miller, PhD

1:00 PM - 1:25 PM
Strategies and Details for Clinical Development of a Gene Therapy in an Academic Setting
Dolan Sondhi, PhD

1:25 PM - 1:50 PM
Planning for Manufacturing Success Early in Gene Therapy Development
Michelle Berg

1:50 PM - 2:15 PM
Viral Vector Production for Early Phase Clinical Study
Stephen Kaminsky, PhD

2:15 PM - 2:40 PM
Developing Potency Assays from Preclinical to Clinical Gene Therapy
Mark Johnson

2:40 PM – 3:10 PM
Break

3:10 PM - 3:35 PM
Gene Therapy for Ganglioside Storage Diseases – Preclinical Development to Clinical Trials
Doug Martin, PhD

3:35 PM - 4:05 PM
Lessons Learned Preparing for Phase 3 Gene/Cell Therapy Clinical Trials
John Maslowski, PhD

4:05 PM - 4:35 PM
Successful Clinical Endpoints in Gene/Cell Therapies
Angela Schulz, MD, PhD

4:35 PM - 5:00 PM
Regulatory Writing—Pre-IND to IND
Neil Hackett, PhD

Eastern Time Zone (EDT UTC -4)
CO-CHAIRS

Maria L. Escolar, MD
University of Pittsburgh Medical Center
Pittsburgh, PA

Timothy J. Miller, PhD
Cleveland, OH

SPEAKERS

Michelle Berg
Aldevron
Fargo, ND

Stephen Kaminsky, PhD
Weill Medical College of Cornell University
New York, NY

Neil Hackett, PhD
Independent Consultant
Santa Monica, CA

Doug Martin, PhD
Auburn University
Auburn, AL

Mark Johnson
Northern Biomedical Research
Spring Lake, MI

John Maslowski, PhD
Castle Creek Biosciences, Inc.
Exton, PA

Angela Schulz, MD, PhD
University Medical Center Hamburg-Eppendorf
Children’s Hospital
Hamburg, Germany

Dolan Sondhi, PhD
Weill Medical College of Cornell University
New York, NY
Michelle Berg
Michelle Berg brings over 20 years of experience in the biotechnology sector, 10 years of which have been spent in executive leadership roles. In her current position as President of Aldevron’s GMP Nucleic Acids Business Unit, Berg oversees Aldevron’s strategy to provide GMP plasmids and mRNA for gene editing, gene therapy, and cell therapy applications. She works closely with the company’s operational team to meet the requirements of these growing fields, while supporting the clinical and commercial efforts of Aldevron’s clients. Aldevron’s GMP plasmid facility, located in Fargo, ND, is the largest in the world. Berg earned a Bachelor of Science in Biotechnology from North Dakota State University (NDSU). She is a contributing author and speaker on patient-focused programming, rare disease advocacy, and accessible education on genetic medicines. In addition to her roles with Abeona and Aldevron, she has performed research on behalf of the Department of Plant Sciences at NDSU.

Neil Hackett, PhD
Neil Hackett, PhD is an independent consultant based in Santa Monica, CA with extensive experience in pre-clinical and translational programs for gene therapy. He currently works with research groups and foundations to navigate stem cell and gene therapy projects through pre-IND to IND. This includes project management, advising on proof-of-concept and toxicity studies, vector manufacturing and regulatory writing. His background includes professorial appointments at Vanderbilt University and Weill-Cornell Medical College resulting in over 100 academic publications. Previous assignments include participating in design, qualification and management of a successful GMP facility for viral vector manufacturing and as co-investigator in multiple gene therapy clinical trials for cardiac, pulmonary and neurological indications.

Mark Johnson
Mark Johnson is the Vice President of Safety Assessment of Northern Biomedical Research and co-founder and board member of Northern Biomolecular Services, bringing over 25 years’ experience in the development and evaluation of gene therapies, regenerative medicines and medical devices. Previously, Mark served as a Senior Study Director and Senior Director of Surgery, Ophthalmology and Cell and Molecular Biology for Charles River Laboratories. In his role with Charles River/ MPI Research, Mark was responsible for the overall management of these business lines. During his tenure, he was responsible for the conduct of several hundred GLP and Non-GLP studies in support of IND, NDA, IDE and PMA for new drugs, biologics, gene therapies, cell therapies, and medical devices. Mark holds a Master’s Degree in Neuroscience.

Stephen Kaminsky, PhD
Dr. Kaminsky is a Professor of Research in Genetic Medicine and an Associate Director of the Belfer Gene Therapy Core Facility. He has a PhD in Molecular Biophysics and Biochemistry from Yale and a BS in Engineering Physics from Cornell. Dr. Kaminsky directs multiple projects ranging from cancer therapeutics, metabolic disorders such as alpha-1 antitrypsin, and gene therapies for the eye and heart to vaccines for addictive drugs. He contributes expertise to preclinical development, safety and toxicology study design, GMP manufacture, facility design and lab management. Dr. Kaminsky has prior experience designing and developing several candidate prophylactic and therapeutic HIV vaccines as well as a candidate prostate cancer therapeutic vaccine, each of which went to a clinical trial. Dr. Kaminsky participated in the design of the Weill Cornell Gene Therapy Core Facility, created the GMP production laboratory and established all of the underlying processes and controls and is a member of the project team that developed gene therapies to the CNS for Batten disease and Alzheimer’s, both from idea through the clinic trial. Dr. Kaminsky has authored more than fourteen FDA regulatory submissions with application to diagnostics, vaccines and therapeutics; he has sat on the NIH grant review panels for HIV vaccine program projects (HIVRAD), Partnerships for Development of Vaccine Technologies and co-chaired an FDA panel on peptide-based vaccines products.

Douglas R. Martin, PhD
Douglas R. Martin, PhD, is a Professor of Anatomy, Physiology & Pharmacology and the Scott-Ritchey Research Center at Auburn University’s College of Veterinary Medicine. He has 25 years’ experience with developing therapies for fatal neurologic disorders such as Tay-Sachs disease, Sandhoff disease and GM1 gangliosidosis. He has been inspired by heroic families and patients such as Porter Heatherly, who died of GM1 gangliosidosis at four years of age. With colleagues at the University of Massachusetts Medical School and others, Dr. Martin’s lab demonstrated the profound therapeutic effect of AAV vectors in large animal models, dramatically improving quality of life and lifespan to >5 times that of untreated animals. Their results led to the first-in-human gene therapy clinical trials for patients. Dr. Martin has served on numerous review panels for the National Institutes of Health and international foundations, and has been recognized with honors such as the Above and Beyond Award from the National Tay-Sachs & Allied Diseases Association and the 2020 Southeastern Conference Faculty Achievement Award for Auburn University. He has two beautiful, adult step-daughters who reside in Phoenix and New York City, and he lives with his lovely wife (and numerous pets) in Opelika, Alabama.
John Maslowski, PhD

John Maslowski is Chief Executive Officer of Castle Creek Biosciences, Inc. Previously, he served as President and Chief Executive Officer of Fibrocell Science, Inc., leading the company through a commercialization agreement and eventual acquisition by Castle Creek Holdings, Inc. in 2019. He also served as a member of Fibrocell’s Board of Directors. Formerly, Mr. Maslowski was Fibrocell’s Senior Vice President, Scientific Affairs and Vice President, Operations, driving the U.S. FDA approval of LAVIV® (azficel-T), an autologous fibroblast cell therapy for improving the appearance of nasolabial folds in adults. Prior to Fibrocell, Mr. Maslowski held various positions at Wyeth Pharmaceuticals (now Pfizer), Merck & Co., and Teva Pharmaceutical Industries. He earned a BS in biology from Ursinus College and an MS in microbiology from Villanova University. Mr. Maslowski currently serves on the Board of Directors of the Alliance for Regenerative Medicine and is Chairman of the Board of Falcon Therapeutics, a private Durham, NC company focused on the development of cell therapy products for oncology.

Angela Schulz, MD, PhD

Angela Schulz has been Head of the Research Group for Childhood Neurodegenerative Disease at the University Medical Centre Hamburg-Eppendorf, Germany, since 2015. Dr. Schulz received her medical degree from the Albert-Ludwigs University of Freiburg, Germany, in 2000. After a 1-year medical sub-internship at the University of Tampa, Florida, US, she completed her junior (2001-2003) and senior residency (2006-2011) at the Hamburg University Medical Centre Children’s Hospital. Between her two residencies, Dr. Schulz was a post-doctoral research fellow at the Department of Paediatric Neurology at Duke University Medical Center, Durham, North Carolina, US. She obtained board certification in paediatrics and clinical specialisation in paediatric palliative care medicine in 2011. Dr. Schulz’s research interests are paediatrics, neurology and neurodegeneration. In view of this, she has investigated rare paediatric neurodegenerative diseases, especially lysosomal storage diseases (LSDs) such as NCL. She is currently principal investigator of several clinical studies in the field of rare diseases, including studies on intraventricular enzyme replacement therapy (ERT) in patients with CLN2 disease and intrathecal ERT in patients with mucopolysaccharidosis (MPS) type IIIA. She is also work package lead for the European Horizon 2020 project, BATCure, the National NCL Research consortium, NCL2TREAT, and previously served as a coordinator for the European FP7 project, DEM-CHILD, between 2011 and 2014. Dr. Schulz received a research award from the German Society for Paediatric Neurology in 2005 and has authored and co-authored many peer-reviewed publications, as well as contributing to the patent application on a method for diagnosis of NCL.

Dolan Sondhi, PhD

Dr. Sondhi is a Professor of Research in Genetic Medicine and an Associate Director of the Belfer Gene Therapy Core Facility. She received her post-doctoral training at Rockefeller University, her PhD in Chemistry from Brown University, Rhode Island, and her BS in Chemistry from St. Stephens College, Delhi University, India. As a research scientist in genetic medicine, she has over two decades of experience in gene therapy, translational medicine and manufacturing of gene therapy products. Her special focus is on developing therapies for rare genetic diseases and those diseases that significantly impact the brain such as lysosomal storage diseases that affect the central nervous system and other neurodegenerative diseases such Alzheimer’s disease and chronic traumatic encephalopathy. She has expertise in all aspects of clinical development in the academic setting from choosing a disease target, preliminary proof of concept studies, IND enabling pre-clinical efficacy studies, safety/toxicology studies, cGMP manufacturing, regulatory submissions, design and conduct of early phase clinical studies and data analysis. She has project managed three gene therapy clinical trials all the way from concept to first in human clinical study. In addition, as the Associate Director of a gene therapy manufacturing facility where they make viral vectors for pre-clinical research, toxicology studies, and GMP manufacturing for clinical studies, she is also familiar with the product requirements at various stages of development.
## DISCLOSURE OF RELEVANT FINANCIAL RELATIONSHIPS

<table>
<thead>
<tr>
<th>Name</th>
<th>Relationships</th>
</tr>
</thead>
<tbody>
<tr>
<td>Maria L. Escolar, MD</td>
<td>PassageBio, Consulting Fees, Consulting</td>
</tr>
<tr>
<td>Neil Hackett, PhD</td>
<td>Lexeo Therapeutics, Salary, Employee; Après Therapeutics, Consulting Fee, Independent Consultant</td>
</tr>
<tr>
<td>Mark Johnson</td>
<td>Northern Biomedical Research, Salary and Ownership Interest, Vice President of Safety Assessment; Northern Biomedical Research, Ownership Interest, Board Member</td>
</tr>
<tr>
<td>Stephen Kaminsky, PhD</td>
<td>LEXEO Therapeutics, Consulting, ownership interest, intellectual property rights, Consulting, research; Xylocor Therapeutics, intellectual property rights, Intellectual property; Adverum, intellectual property rights, Intellectual property</td>
</tr>
<tr>
<td>John Maslowski, PhD</td>
<td>Castle Creek Biosciences, Salary, CEO</td>
</tr>
<tr>
<td>Angela Schulz, MD, PhD</td>
<td>BioMarin, Consulting fee, Consulting; Regenx Bio, Honoraria, Teaching; Takeda, Honoraria, Teaching; Neurogene, Consulting fee, Consulting; Polaryx, Honoraria, Teaching; Amicus, Honoraria, Teaching</td>
</tr>
<tr>
<td>Dolan Sondhi, PhD</td>
<td>LEXEO Therapeutics, Consulting, intellectual property rights, Consulting, research; Xylocor Therapeutics, intellectual property rights, Intellectual property; Adverum biotechnologies, intellectual property rights, Intellectual property</td>
</tr>
</tbody>
</table>