Clinical Trials Training I Workshop

MAY 11, 2020

ASGCT
23rd ANNUAL MEETING

BOSTON
MAY 12-15 • 2020
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**PROGRAM SCHEDULE**

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

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<tr>
<th>Time</th>
<th>Session</th>
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| 8:00 AM - 12:00 PM | ROOM: BALLROOM A  
CO-CHAIRS: Thomas Conlon, PhD and H. Trent Spencer, PhD |  
8:00 AM - 8:30 AM | Development and Implementation of Lentivirus-Mediated Gene Therapy for Fabry Disease | Jeffrey A. Medin, PhD          |
| 8:30 AM - 9:00 AM | Obtaining Trial Funding and the Ingredients for a Strong IND | Barry J. Byrne, MD, PhD         |
| 9:00 AM - 9:30 AM | Considerations for Technology Transfer                                                      | Miguel Sena-Esteves, PhD        |
| 9:30 AM - 10:00 AM | The Winding Path from an Academic Lab to a Commercial IND | Christopher B. Doering, PhD     |
| 10:00 AM - 10:30 AM | Break                                                                                       |                                 |
| 10:30 AM - 11:00 AM | Technology Transfer Considerations for Cell and Gene Therapy with a Focus on Documentation to Support an IND | Carmella Moody, PhD             |
| 11:00 AM - 11:30 AM | A Practical Guide to Preclinical Studies                                                     | Kirsten E. Coleman              |
| 11:30 AM - 12:00 PM | Clinical Translation of rAAV Vectors                                                         | Terence R. Flotte, MD           |
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CO-CHAIRS

Thomas Conlon, PhD
CR Scientific and Compliance Consulting and
The Michelson Found Animals Foundation
Culver City, CA

H. Trent Spencer, PhD
Emory University School of Medicine
Atlanta, GA

Barry J. Byrne, MD, PhD
University of Florida College of Medicine
Gainesville, FL

Kirsten E. Coleman
University of Florida College of Medicine
Gainesville, FL

Christopher B. Doering, PhD
Emory University School of Medicine
Atlanta, GA

Terence R. Flotte, MD
University of Massachusetts Medical School
Worcester, MA

Jeffrey A. Medin, PhD
Medical College of Wisconsin
Milwaukee, WI

Carmella Moody, PhD
RTI International
Research Triangle Park, NC

Miguel Sena-Esteves, PhD
University of Massachusetts Medical School
Worcester, MA

SPEAKERS
Barry J. Byrne, MD, PhD

Dr. Barry Byrne is a clinician scientist who studies a variety of rare diseases with the specific goal of developing therapies for inherited muscle disease. His focus as a pediatric cardiologist is on conditions that lead to skeletal muscle weakness and abnormalities in heart and respiratory function. His group has made significant contributions to the understanding and treatment of Pompe disease. They develop new therapies using adeno-associated virus mediated gene therapy to restore cardiac and skeletal muscle function in DMD, Friedreich’s Ataxia, Pompe, and other inherited neuromuscular diseases.

Kirsten Coleman

Kirsten Coleman is the Associate Director of the University of Florida Powell Gene Therapy Center Toxicology Core. She obtained both her BS in microbiology and MBA degrees from the University of Florida. After completing her undergraduate honors thesis in Dr. Terence Flotte’s laboratory in 2004, she joined the Toxicology Core to participate in the first-in-human trial of adeno-associated virus (AAV) 2 for the treatment of alpha-1 antitrypsin deficiency. Since then, she has participated in bench to bedside research for multiple disorders utilizing the AAV gene therapy platform. Kirsten’s current responsibilities include designing and coordinating numerous aspects of preclinical and clinical testing, as well as overseeing that the Toxicology Core adheres to FDA Good Laboratory Practice (GLP) 21 CFR Part 58 regulations. Her research focuses on the testing of cell and gene therapy vectors to determine their biodistribution, immunogenicity, pathology and efficacy. Along with the team at UF, she has successfully developed FDA-approved investigational new drug applications for the treatment of a wide range of musculoskeletal, metabolic, ocular and neurological diseases.

Christopher Doering, PhD

Christopher B. Doering, PhD is a Professor of Pediatrics in the Aflac Cancer and Blood Disorders Center, Department of Pediatrics, Emory University School of Medicine and Co-founder/Chief Scientific Officer of Expression Therapeutics, LLC. As an experienced molecular biologist, experimental hematologist/oncologist and gene therapist with more than 20 years of professional experience, his scientific interests center on the development of gene therapy and protein engineering strategies for genetic diseases and cancers. Current areas of research involve the development of pharmaceutically inspired coagulation factors, general approaches to viral vector genome engineering, and cancer gene therapy approaches involving genetically engineered innate immune cells as well as exotic chimeric antigen receptors. His graduate and postgraduate training in genetics and hematolology/oncology departments, respectively, focused on discovery and manipulation of the mechanisms regulating the expression of disease-related human genes and proteins. He has been continuously funded by NIH and serves or has served as PI/MPI on 20 NIH awards (R01, R21, R41, R42, R43, R44 and R56), as well as Project and Core Leader on two U54 Center awards. He also recently completed a full term as Chairperson of the NHLBI MTI (K99/R00- Mentored Transition to Independence) Study Section and currently serves as Chairperson for the Maximizing the Scientific Value of the NHLBI Biorepository Scientific Opportunities for Exploratory Research Study Section. Within the past few years, his lab has published articles in Nature Biotechnology, Nature Communications, Molecular Therapy, Human Gene Therapy, Blood, Oncoimmunology, Molecular Therapy Oncolytics, Cytotherapy, and the Journal of Thrombosis and Hemostasis. Dr. Doering is an inventor on several patents for bioengineered proteins as well as cell and gene therapies.
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**Terence Flotte, MD**

Dr. Flotte currently is the Provost and Executive Deputy Chancellor, Celia and Isaac Haidak Professor of Medical Education, and Dean of the University of Massachusetts Medical School. An internationally known pioneer in human gene therapy, Dr. Flotte is currently investigating the use of gene therapy for genetic diseases, including alpha-1 antitrypsin deficiency and Tay-Sachs disease. In 1995, he led the team at Johns Hopkins that became the first to use the apparently harmless adeno-associated virus, or AAV, as a vehicle to deliver corrective genes to targeted sites in the body, including the damaged airways of adults with cystic fibrosis. He is the author of more than 250 scholarly papers and his research has been funded by the National Institutes of Health and the Alpha One Foundation. Since 2015, he has also been Editor-in-Chief of his field’s oldest journal family, Human Gene Therapy.

**Jeffrey Medin, PhD**

Dr. Jeffrey A. Medin received his BSc from the University of Wisconsin-Parkside and his PhD in Biochemistry from the University of Kentucky. He then trained at the National Institutes of Health in Bethesda, MD, working on gene transcription and gene transfer/therapy. Following that he was appointed as an Assistant Professor of Medicine at the University of Illinois-Chicago. In 2001, Dr. Medin relocated to Toronto, Canada, where he rose through ranks to become a Senior Scientist at the University Health Network and a Full Professor in the Department of Medical Biophysics at the Medical College of Wisconsin in Milwaukee, WI. He is also Vice Chair of Research Innovation for the MCW Department of Pediatrics and Director of the MCW Vector Production Facility. Dr. Medin is one of two Academic Founders of AVROBIO, Inc., of Boston, MA. He is a member of the SAB of Rapa Therapeutics and an editorial board member of Biomedicines. Dr. Medin is the Principal Investigator of an ongoing clinical gene therapy trial in Canada targeting correction of autologous CD34+ hematopoietic cells for amelioration of Fabry disease and a Co-Investigator on another ongoing clinical trial that involves IL-12 gene transfer into blasts to treat AML. Dr. Medin holds multiple filed and issued patents, has published more than 150 peer-reviewed scientific papers, has edited an immunotherapy book, and presented at more than 150 invited conferences and workshops.

**Carmella Moody, PhD**

Carmella S. Moody, PhD is a Regulatory Program Director at RTI; she has more than 30 years of experience in development of small molecules, large molecules (biologic), and combination products with emphasis in regulatory affairs, project management, and quality assurance. Her specific expertise is in CMC (Quality) Regulatory Affairs. She is trained as an industrial microbiologist where her major skill set is development and large-scale manufacture of bacterial and cell-based products. Experience includes leading activities for submission of US and international regulatory dossiers as eCTDs, including cell and gene therapy INDs, and defining regulatory strategy for global regulatory programs for FDA, EMA, PMDA, and Health Canada. Additionally, she has extensive experience as the contact for regulatory agencies and developing strategies and training teams for global regulatory agency meetings including pre-IND, End of Phase 1, End of Phase 2 and Pre-NDA and Pre-MAA. Dr. Moody received her PhD in Microbiology from North Carolina State University and completed a post-doctoral program in pharmacology at Duke University.

**Miguel Sena-Esteves, PhD**

Miguel Sena-Esteves, PhD, is an associate professor of neurology and a member of the Horae Gene Therapy Center. Miguel Sena-Esteves received his PhD in biomedical sciences from the University of Porto, Portugal, where he started his work in gene therapy. His laboratory at UMMMS focuses on the development of adeno-associated virus (AAV) based gene therapies for lysosomal storage diseases, Huntington’s disease, ALS, FSHD and brain tumors. In addition, his laboratory develops novel AAV capsids to improve targeting, distribution and potent transduction of target tissues in these diseases, with a particular focus on the central nervous system. Dr. Sena-Esteves leads the Tay-Sachs Gene Therapy Consortium with the focus to develop an AAV gene therapy and conduct the first-in-human clinical trial. He is also leading the pre-clinical development efforts for a GM1-gangliosidosis AAV gene therapy in collaboration with Lysogene. In 2011 he received the Outstanding New Investigator Award from the American Society of Gene & Cell Therapy (ASGCT) for his many contributions to the field of gene and cell therapy. He has authored 88+ peer-reviewed publications and is inventor on several patent applications.
# DISCLOSURE OF RELEVANT FINANCIAL RELATIONSHIPS

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<tr>
<th>Name</th>
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<tr>
<td>Christopher B Doering, PhD</td>
<td>Expression Therapeutics, LLC, ownership interest, research funding, royalty, Founder, Inventor</td>
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<tr>
<td>Terence R Flotte, MD</td>
<td>Trizell Holding, S.A., Consulting Fees, Scientific Advisory Board Member</td>
</tr>
<tr>
<td>Jeffrey A Medin, PhD</td>
<td>AVROBIO, Inc, Stocks, Honorarium, Travel, Scientific Co-Founder; Speaker; Rapa Therapeutics, Honorarium, SAB Member</td>
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