Commercialization I Workshop: Opportunities and Challenges for Global Clinical Trials

MAY 11, 2020
# TABLE OF CONTENTS

- Workshop Sponsors .................................................. 2
- Program Schedule .................................................. 4
- Co-Chairs ............................................................... 5
- Speakers ............................................................... 5
- Speaker Biographies .................................................. 6
- Disclosure of Relevant Financial Relationships ................. 8
WORKSHOP SPONSORS

The American Society of Gene & Cell Therapy is honored to acknowledge the following organization for its support of the Commercialization I Workshop:

[Legend Biotech logo]
Legend Biotech is an integrated biopharmaceutical company specialized in the discovery and development of novel cell therapies, focused on hematologic malignancies, solid tumors, autoimmune and infectious diseases.

Our story is just beginning. We are already seeing success and are looking for you to help us write the next chapter. Do you have a passion for science and a commitment to improving patients' lives? We are looking for people with a depth of scientific experience and an appreciation for the rapid pace and steady change in cell therapy.

Now is a unique chance to join us and help ignite the future of cell therapy.

Join the Legend legacy.

Learn more about us:
legendbiotech.com

Visit LinkedIn for Career Opportunities

© Legend Biotech USA Inc. 2020. All Rights Reserved. 04/2020
LinkedIn logo is a registered trademark of the LinkedIn Corporation.
# PROGRAM SCHEDULE

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

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker</th>
</tr>
</thead>
<tbody>
<tr>
<td>8:00 AM – 12:00 PM</td>
<td><strong>Commercialization I: Opportunities and Challenges for Global Clinical Trials</strong></td>
<td>Kit Shaw, PhD and Keith Wonnacott, PhD</td>
</tr>
<tr>
<td>8:00 AM – 8:25 AM</td>
<td>Regulatory Considerations for the Global Development of Gene Therapy Products</td>
<td>Anita Freed, PhD</td>
</tr>
<tr>
<td>8:25 AM – 8:50 AM</td>
<td>Regulatory Pathways and Challenges to Global Commercialization of Cell and Gene Therapy Products from the CMC Perspective</td>
<td>Cindy Riggins, PhD</td>
</tr>
<tr>
<td>8:50 AM – 9:15 AM</td>
<td>Nonclinical Studies Supporting First-in-Human ZFN-Based Genomic Medicines for MPS II</td>
<td>Kathleen Meyer, MPH, PhD, DABT</td>
</tr>
<tr>
<td>9:15 AM – 9:45 AM</td>
<td>Moderated Panel Discussion</td>
<td>Kit Shaw, PhD</td>
</tr>
<tr>
<td>9:45 AM – 10:15 AM</td>
<td>Break</td>
<td></td>
</tr>
<tr>
<td>10:15 AM - 10:40 AM</td>
<td>Clinical Trial Authorization in the EU: Current State and Future Developments</td>
<td>Jan Mueller-Berghaus, MD</td>
</tr>
<tr>
<td>10:40 AM - 11:05 AM</td>
<td>International Clinical Trials to Support Marketing Approval: An FDA Perspective</td>
<td>Wilson W. Bryan, MD</td>
</tr>
<tr>
<td>11:05 AM - 11:30 AM</td>
<td>Case Study on the Need for Global Regulatory Harmonization: <em>Ex-Vivo</em> Autologous HSC Gene Therapy for a Rare Pediatric Serious Condition</td>
<td>Anne Dupraz-Poiseau, PhD</td>
</tr>
<tr>
<td>11:30 AM - 12:00 PM</td>
<td>Moderated Panel Discussion</td>
<td>Keith Wonnacott, PhD</td>
</tr>
</tbody>
</table>

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

Kit Shaw, PhD
Dana-Farber Cancer Institute
Boston, MA

Keith Wonnacott, PhD
Pfizer
Silver Spring, MD

Anne Dupraz-Poiseau, PhD
Orchard Therapeutics
London, United Kingdom

Anita Freed, PhD
Pfizer
Groton, CT

Kathleen Meyer, MPH, PhD, DABT
Sangamo Therapeutics
Kensington, CA

Jan Mueller-Berghaus
Paul Ehrlich Institute
Langen, Germany

Cindy Riggins, PhD
Autolus, Inc.
Rockville, MD

SPEAKERS
### SPEAKER BIOGRAPHIES

**Wilson Bryan, MD**

Wilson Bryan is a neurologist who graduated from the University of Chicago Pritzker School of Medicine. He served on the neurology faculty of the University of Texas Southwestern Medical School for 13 years. He has been an investigator on clinical trials in cerebrovascular disease and neuromuscular disorders, particularly amyotrophic lateral sclerosis. Dr. Bryan joined the United States Food and Drug Administration (FDA) in 2000, and now serves as Director of the Office of Tissues and Advanced Therapies (OTAT) in the FDA’s Center for Biologics Evaluation and Research (CBER).

**Anne Dupraz-Poiseau, PhD**

Anne brings 25 years of experience of tackling R&D, clinical & regulatory challenges to Orchard Therapeutics, a biopharma company developing ex vivo autologous gene therapy to address severe & life-threatening inherited disorders. Before joining ORTX, Anne was Executive VP at VCLS, where she was involved in the design & preparation of a high number of successful regulatory submissions at all stages of product development with FDA, EMA & local agencies. She assisted over 50 cell, tissue & gene therapies development programs. Bringing expertise in Advanced Therapies, Anne began her career as R&D Project Manager at Medtronic Sofamor-Danek after she obtained her PhD in biomaterials & artificial organs.

**Anita Freed, PhD**

Anita L. Freed, PhD, is a Director of Global Regulatory Affairs in the Rare Diseases Unit at Pfizer, where she is a Global and US Regulatory Lead of a late stage gene therapy program. As a part of this role, she develops and delivers global regulatory strategies, manages all regulatory aspects of projects, including the preparation and submission of correspondences and applications for regulatory agencies. Anita also acts as the direct liaison with the FDA and other ex-US Health Authorities. Prior to working with Rare Diseases, Anita was the Regulatory Lead of various early development neuroscience assets. She provided support for a dermatology program at the registration stage, which was also in preparation for a potential FDA Advisory Committee. Prior to this, Anita was a regulatory product lead in Pfizer’s Global Chemistry, Manufacturing and Controls group, and has also held various scientific/technical roles. She has a PhD from the University of Kansas, Department of Pharmaceutical Chemistry, and has been with Pfizer for 19 years.

**Kathleen E. Meyer, MPH, PhD, DABT**

Dr. Kathleen Meyer is Vice President of Nonclinical Development at Sangamo Therapeutics and leads nonclinical development of Sangamo’s zinc finger-based gene editing and regulation, gene therapy and cell therapy therapeutic candidates. Dr. Meyer oversees nonclinical safety evaluation strategy, and pharmacology/toxicology, nonclinical operations and bioanalytical sciences groups supporting nonclinical and clinical studies. She has over 20 years of industry experience in nonclinical safety evaluation and supported development of ZFN-based gene editing, small molecule, monoclonal antibody, enzyme replacement, botulinum toxin, gene and cell therapy programs. Prior to joining Sangamo, she worked in the pharmacology/toxicology group at BioMarin Pharmaceutical Inc. where she guided small molecule and biologic drug candidates through the nonclinical development process supporting clinical trials and marketing authorization for rare diseases and metabolic disorders. She led the nonclinical safety, pharmacokinetics and bioanalytical sciences group at XOMA LLC focusing on development of monoclonal antibodies for inflammatory disorders, and oncology as well as small molecule treatments for acne. Prior to this, she worked in the nonclinical safety department at Elan Pharmaceuticals developing therapeutic candidates and approved products for neurology indications. Before joining industry, she worked as a post-doctoral fellow evaluating non-viral methods of gene delivery at the University of California, San Francisco. Dr. Meyer received an AB in Physiology, a master’s degree in Public Health specializing in Toxicology and Epidemiology, and her PhD in Environmental Health Science/Toxicology from the University of California, Berkeley. Dr. Meyer is a Diplomat of the American Board of Toxicology.

**Jan Mueller-Berghaus, MD**

Jan Mueller-Berghaus is paediatrician by training and joined the Paul-Ehrlich-Institut as clinical expert in 2005. The Paul-Ehrlich-Institut is the German Federal Agency for Vaccines and Biomedicines and is actively participating in all aspects of German and European marketing authorisation as well as clinical trial authorisation. In 2011 he was elected as co-opted member of the Committee for Medicinal Products for Human Use (CHMF), and since 2017 he is a member of the Committee for Advanced Therapies (CAT). Additional professional experience includes basic research in immunology at the University of Pittsburgh (USA) and translational research in the immunotherapy of cancer (German Cancer Research Center, Heidelberg, Germany).
Cindy Riggins, PhD
Cindy Riggins is the Head of Regulatory CMC at Autolus, a biotech company developing autologous CAR T-cell therapy products. She has over 15 years of experience working in CMC Regulatory Affairs in Biologics including large molecules and advanced therapies. Cindy joined FDA/CBER in 2001 as a research post-doctoral fellow but eventually transitioned to review of investigational new drug applications for cellular products. In 2008, Cindy moved to MedImmune in the CMC Regulatory Affairs group providing global regulatory strategy and submission guidance for numerous early and late phase monoclonal antibody product development teams. In 2014, Novartis offered an opportunity to get back into development of cell and gene therapy products. Cindy was involved in the development of several different types of products but most notably, the successful development and submission of the Kymriah CAR T-Cell Product marketing applications in several countries/regions, as well as post-approval life-cycle management. Cindy joined Autolus in 2018 for the chance to build a highly skilled Cell & Gene Therapy Regulatory CMC team and develop next generation T-cell therapies.

Kit Shaw, PhD
Dr. Shaw has over 10 years’ experience in gene therapy clinical trials. She received her PhD in Microbiology from the University of Alabama Birmingham where she studied the foamy virus envelope protein. She performed post-doctoral work in the lab of Donald Kohn at Children’s Hospital Los Angeles where she engineered lentivirus vectors. She eventually moved to the University of California Los Angeles and managed several early phase gene transfer clinical trials for severe combined immunodeficiency (SCID), including regulatory and cell manufacturing activities. Dr. Shaw is currently the Technical Director of the Novel Cell Therapy Laboratory, Cell Manipulation Core Facility at Dana-Farber Cancer Center. The Novel Cell Therapy Lab develops and manufactures cellular products that are more than minimally manipulated and regulated under Investigative New Drug Applications.

Keith Wonnacott, PhD
Dr. Keith Wonnacott has over 15 years of regulatory experience in the field of cell and gene therapies. He currently works at Pfizer as an Executive Director of Regulatory Affairs in their Rare Diseases Unit. At Pfizer, Dr. Wonnacott leads efforts to develop regulatory strategy and policy related to cell and gene therapy. He is a member of the regulatory affairs committee for both ASGCT and APIM; he is on working groups related to advanced therapies with PhRMA, BIO, and EFPIA; and participates with other organizations that work on cell and gene therapy policy. He also contributes to regulatory strategy for AAV-based gene therapies to treat rare diseases. Dr. Wonnacott made the move to Pfizer after spending the previous three years with Novartis Pharmaceuticals as a Director of Regulatory Affairs in the Cell and Gene Therapy group. In that role he advised on regulatory strategy and led the team responsible for developing the CMC module for the Kymriah (tisagenlecleucel) BLA and coordinating CMC activities which led to its approval in 2017. Prior to working at Novartis, Dr. Wonnacott was the Chief of the Cellular Therapies Branch at the Center for Biologics Evaluation and Research (CBER) at the U.S. Food and Drug Administration (FDA). His branch was responsible for the CMC review for all cellular therapies including stem cells, allogeneic pancreatic islets, immunotherapies, cancer vaccines, xenotransplantation products, and tissue engineered products. His branch was also responsible for the review of medical devices used in the processing and storage of cellular products. Dr. Wonnacott has published several articles and book chapters on the regulation of cellular therapies. Dr. Wonnacott received his PhD in microbiology and immunology from the Pennsylvania State University College of Medicine, Hershey, Pennsylvania in 2001. Dr. Wonnacott completed his bachelor’s degree in microbiology at Brigham Young University, Provo, Utah in 1996.
## DISCLOSURE OF RELEVANT FINANCIAL RELATIONSHIPS

<table>
<thead>
<tr>
<th>Name</th>
<th>Disclosure</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anita Freed, PhD</td>
<td>Pfizer, employee</td>
</tr>
<tr>
<td>Kathleen Meyer, MPH, PhD, DABT</td>
<td>Sangamo Therapeutics, Salary, VP on Nonclinical Development</td>
</tr>
<tr>
<td>Cindy Riggins, PhD</td>
<td>Autolus, Inc., salary, employee</td>
</tr>
</tbody>
</table>