Post-Approval Commercialization Workshop

April 28, 2019
Washington, D.C.
Washington Hilton Cabinet
# Table of Contents

- Workshop Sponsors ................................................................................................................... 2
- Program Schedule ..................................................................................................................... 4
- Co-Chair Listing ......................................................................................................................... 5
- Speaker Listing ........................................................................................................................ 5
- Speaker Bios ........................................................................................................................... 6
- Disclosure of Relevant Financial Relationships ........................................................................... 10
- Notes ....................................................................................................................................... 11
Post-Approval Commercialization Workshop Sponsor

The American Society of Gene & Cell Therapy is honored to acknowledge the following organization for their support of the Post-Approval Commercialization Workshop:

REGENXBIO®
Leader in AAV Gene Therapy

Seeking to improve lives through the curative potential of gene therapy.

Learn more at REGENXBIO.com

© 2019 REGENXBIO Inc.
Program

10 AM – 10:30 AM

Modernizing Gene Therapy Oversight and the Role of the RAC
SPEAKER: Jessica Tucker, PhD

10:30 AM – 12 PM

The Role of the Patient Panel
MODERATOR: Susanne Warner, MD
SPEAKER: Celia Witten, MD PhD
PANELISTS: Ronald Bartek, MA
          Sue Kahn, MBA
          Maria Kefalas, PhD
          Celia Witten, MD PhD

12 PM – 1 PM

Lunch

1 PM – 2:30 PM

Post Approval Change Management and Overall Impact on Commercialization
MODERATOR: Keith Wonnacott, PhD
SPEAKERS: Kristen Harrington-Smith, MBA
          Ramjay Vatsan, PhD
          Anne-Virginie Eggimann, MSc
          Joann Parker, MS

2:30 PM – 3 PM

Coffee break

3 PM – 6 PM

Achieving Sustainable Patient and Market Access
MODERATOR: Philip Reilly, MD, JD
SPEAKERS: Tim Hunt, JD
          William Smith, PhD
          Jeremy Allen, MPA
          Jugna Shah, MPH
          Rachel Pryor, MSW
Co-Chairs

Adora Ndu, PharmD, JD
BioMarin Pharmaceutical Inc.
Novato, CA

John Tomtishen
Legend Biotech
Piscataway, NJ

Jessica Voss, PhD
Northwestern Feinberg School of Medicine
Chicago, IL

Speakers

Jeremy Allen, MPA
Spark Therapeutics
Philadelphia, PA

Ronald Bartek, MA
Friedreich’s Ataxia Research Alliance
Springfield, VA

Anne-Virginie Eggimann, MSc
bluebird bio
Cambridge, MA

Kristen Harrington-Smith, MBA
Novartis Pharmaceuticals Corporation
New York, NY

Tim Hunt, JD
Editas Medicine
Cambridge, MA

Sue Kahn, MBA
National Tay-Sachs & Allied Diseases Association
Brighton, MA

Maria Kefalas, PhD
The Calliope Joy Foundation
Bala Cynwyd, PA

Joann Parker, MS
Pfizer Inc
Andover, MA

Rachel Pryor, MSW
Virginia Department of Medical Assistance Services
Richmond, VA

Philip Reilly, MD, JD
Third Rock Ventures
Boston, MA

Jugna Shah, MPH
Nimitt Consulting, Inc.
Spicer, MN

William Smith, PhD
Pioneer Institute
Boston, MA

Jessica Tucker, PhD
National Institutes of Health
Bethesda, MD

Ramjay Vatsan, PhD
Food and Drug Administration
Rockville, MA

Susanne Warner, MD
City of Hope
Duarte, CA

Celia Witten, MD PhD
Food and Drug Administration
Silver Spring, MD

Keith Wonnacott, PhD
Pfizer Inc
Gaithersburg, MD
Speaker Bios

Jeremy Allen, MPA
Jeremy Allen joined Spark Therapeutics in March 2017 as its Head of Government Affairs. In this role, Jeremy oversees development and execution of Spark's government affairs strategy at the federal, state, and international level. Prior to joining Spark, Jeremy spent six years at America's Health Insurance Plans managing its Federal Affairs department. As AHIP's senior lobbyist, Jeremy had responsibility for developing and executing AHIP's federal advocacy efforts. During this time he was twice named one of The Hill newspaper’s “Top Lobbyists.” Previously, Jeremy lobbied for two pharmaceutical companies and spent nearly three years working on Capitol Hill for both the House Energy and Commerce Committee and Rep. Mike Bilirakis. Jeremy began his career as a Presidential Management Intern with the Centers for Disease Control and Prevention. Jeremy received his B.S. in industrial and labor relations and M.P.A. from Cornell University. He lives with his wife Ashley and two children in Washington, DC.

Ronald Bartek, MA
Co-founder/President, Friedreich's Ataxia Research Alliance; Board of Directors/past Chairman, National Organization for Rare Disorders; Board of Directors, Alliance for a Stronger FDA and Alliance for Regenerative Medicine; Member, NIH/NCATS National Advisory Council; Vice Chair, NIH/NCATS Cures Acceleration Network Review Board; member, FDA/CTTI Patient Engagement Collaborative; 4-year member, NIH National Advisory Neurological Disorders and Stroke Council; former partner/president of business development/government affairs firm; twenty years in federal executive and legislative branches in defense, foreign policy and intelligence, (six years on House Armed Services Committee staff; four years at U.S. State Department, one year on U.S. Delegation to Intermediate-Range Nuclear Forces Treaty talks, Geneva; six years as CIA analyst, including a year as Intelligence Community representative to U.S. arms control committees); after graduation from United States Military Academy, West Point, four years as Army officer, as company commander in Korea and Infantry and Military Intelligence officer in Vietnam; Master’s Degree. Russian Area Studies - Georgetown University.

Anne-Virginie Eggimann, MSc
Anne-Virginie Eggimann, M.Sc. joined bluebird bio, Inc. to lead the Regulatory Science function in 2011. In her role as Senior Vice President, she is responsible for global regulatory strategy and focuses on innovative pathways to accelerate the development of bluebird bio's gene therapy products for the treatment of rare diseases and oncology indications. Prior to joining bluebird bio, Anne-Virginie was an Executive Director at Voisin Consulting, leading projects involving the design and implementation of regulatory strategies for medicinal products, with a particular focus on rare diseases, cancer and advanced therapies. Her experience spans from early development through commercialization, including lead roles on the registration of several orphan drugs, and regulatory expertise on both sides of the Atlantic. Anne-Virginie holds a Master of Science in Environmental Health Sciences from the UCLA School of Public Health, as well as a B.S. in Chemical Engineering from the California Institute of Technology.

Kristen Harrington-Smith, MBA
A highly successful pharmaceutical executive and patient advocate with 20 years’ experience, Kristen has worked across many therapeutic areas including Oncology, Neuroscience, Cardiovascular and Osteoporosis. Head of the Novartis US CAR-T Franchise, Kristen leads development and commercialization of the first ever immuno-cellular treatment for pediatric acute lymphoblastic leukemia and adults with diffuse large B-cell lymphoma.

Tim Hunt, JD
Mr. Hunt is senior vice president of corporate affairs at Editas Medicine and the chair of the ASGCT Government Relations Committee. Prior to joining Editas Medicine, Tim served as senior vice president of public affairs for Cubist Pharmaceuticals, before the company was acquired by Merck in 2015. Before joining Cubist, Tim spent several years at Biogen Idec, where he most recently served as vice president of public affairs, overseeing global communications. Previously, Tim also served in government affairs for GlaxoSmithKline, as well as for former Massachusetts Governor, William F. Weld. Tim received his JD from the Columbus School of Law at the Catholic University of America and his BA from Boston College.
Speaker Bios – continued

**Sue Kahn, MBA**
Sue Kahn joined National Tay-Sachs & Allied Diseases Association (NTSAD) as Executive Director in 2007. The mission of NTSAD is to lead the fight to treat and cure Tay-Sachs, Canavan and related genetic diseases and to support affected families and individuals in leading fuller lives. To advance NTSAD’s research and overall mission, Sue draws on her prior work experience at Genzyme Genetics and Chiron Diagnostics. Sue was inspired to join the patient advocacy and non-profit world through a family connection and volunteer work for Community Consulting Teams. Sue is on the Board of BioPontis Alliance, an international nonprofit focusing on advancing science into potential treatments for rare neurological diseases. Sue has an MBA from the Amos Tuck School of Business Administration at Dartmouth College and a bachelor’s degree in Applied Mathematics–Economics from Brown University.

**Maria Kefalas, PhD**
Maria Kefalas is a Professor in the Department of Sociology at Saint Joseph’s University. She is also the co-founder and executive director of The Calliope Joy Foundation, a Philadelphia-based 501(c)3 that advocates for gene therapy to treat leukodystrophies and lysosomal storage disorders. A 2018 recipient of the NORD Rare Impact Award for her work to establish the nation’s first Leukodystrophy Center of Excellence at the Children’s Hospital of Philadelphia, she has spoken at the NIH and NORD. Her advocacy has been featured on CBS Sunday Morning with Jane Pauley, STAT, and Slate. She is finishing a memoir about her daughter Cal’s diagnosis with metachromatic leukodystrophy titled Investing in Miracles (Beacon Press, forthcoming).

**Joann Parker, MS**
Joann M. Parker is a Senior Director at Pfizer Inc. where she is a Team Leader in Regulatory CMC leading the gene therapy and biologics portfolio. She has 25 years’ experience in the pharmaceutical industry with 20 years in the CMC regulatory function. Joann has extensive experience across the product life cycle from investigational through post approval for US and global registrations over a range of modalities including antibody drug conjugates, monoclonal antibodies, gene and cell therapies, devices and small molecules. Joann holds a B.S. in Pharmacy from Purdue University and a M.S. in Pharmaceutics from Temple University.

**Rachel Pryor, MSW**
Rachel Pryor is the Deputy Director of Administration for the Virginia Department of Medical Assistance Services, the agency that administers the Medicaid program in Virginia for more than 1 million individuals. As part of Virginia Medicaid’s executive management team, she directly supervises a team of more than 100 to carry forward the Commonwealth’s mission to provide accessible, quality health coverage, and oversees all regulatory and policy functions for the agency.

Prior to assuming her current role in January, Rachel was the Senior Health Policy Advisor on the Democratic Staff of the Energy and Commerce Committee in the U.S. House of Representatives. In this role, Rachel managed a broad legislative portfolio for the Committee that includes Medicaid & CHIP, Medicare Parts C & D, Long-Term Care issues, and the 340B program.

Previous roles include Senior Policy Advisor for the Senate Special Committee on Aging under Chairman Bill Nelson (D-FL); senior aide for health policy issues to Senator Richard Blumenthal (D-CT); and work for Senator John D. Rockefeller IV (D-WV).

Rachel was recognized by POLITICO in 2014 as an Emerging Health Care Leader and has been recognized by numerous associations. Prior to Capitol Hill, Rachel has worked at Ascension Health and the United States Department of Health and Human Services. Rachel has a Masters in Social Work from the University of Maryland with a dual Clinical/Policy focus, and will be awarded a Juris Doctor from Georgetown University Law Center in May, 2019.
Speaker Bios – continued

**Philip Reilly, MD, JD**
Philip R. Reilly (Cornell AB ’69, Columbia JD ’73, Yale MD ’81) is a venture partner at Third Rock Ventures in Boston, Massachusetts. Trained in internal medicine and clinical genetics, he specializes in starting and launching companies to develop breakthrough therapies for heretofore untreatable genetic disorders. He has been a founder of Voyager Therapeutics and Fulcrum Therapeutics, and helped to found bluebird bio, Inc., serving as its Chief Medical Officer in 2010 – 2012. Among other roles, Dr. Reilly was CEO and chairman of the board of Interleukin Genetics, Inc.; the Executive Director of the Eunice Kennedy Shriver Center; and an assistant professor at Harvard Medical School. He has served on numerous boards, including for Edimer Pharmaceuticals, Lotus Tissue Repair, Inc., and the American Society for Human Genetics. He has served as a trustee of Cornell University; twice as president of the American Society of Law, Medicine, and Ethics; and as a founding fellow of the American College of Medical Genetics. He is currently a member of the Board of Overseers of Weill Cornell Medical College and a member of the Advisory Council to the BU School of Public Health. He is the author of seven books about human genetics and more than 100 articles, many about public policy issues in genetics.

**Jugna Shah, MPH**
Jugna Shah is a nationally recognized expert with over 20 years of experience in health care policy, finance, and regulatory systems. She is the President and Founder of Nimitt Consulting Inc. and since 2001, has been providing education on regulatory initiatives and policies that affect health care service delivery, patient access, and compliance, as well as regulatory advocacy that has resulted in operationally simpler payment systems with more appropriate reimbursement for providers. In particular, Nimitt has specialized in addressing patient access issues, and billing and reimbursement challenges faced by manufacturers, vendors, and providers who are bringing innovative new therapies to the marketplace including cell and gene therapies such as CAR-T. Ms. Shah’s areas of expertise include understanding Medicare payment systems and rate-setting process, medical and radiation oncology coding, billing, coverage, and reimbursement, bone marrow and stem cell transplant coverage and reimbursement, pharmacy and drug coverage, and much more. Prior to founding Nimitt Consulting, Inc., Ms. Shah was a senior manager with KPMG’s Assurance-Based Advisory Services Practice, guiding providers through the clinical and financial implications of Medicare’s outpatient PPS implementation. She holds a Bachelors of Science in Biopsychology from Oberlin College, and a Masters of Public Health Policy & Administration from the University of Michigan.

**William Smith, PhD**
William S. Smith (Bill) is Visiting Fellow in Life Sciences at the Pioneer Institute, a Boston-based think tank. At Pioneer, Dr. Smith writes about public policy issues impacting the life sciences industry with particular emphasis upon the pharmaceutical industry. Dr. Smith has 25 years of experience in government and in corporate roles. He spent ten years at Pfizer Inc as Vice President of Public Affairs and Policy where he was responsible for Pfizer’s corporate strategies for the U.S. policy environment. At Pfizer, he served on the U.S. leadership team where he provided policy and government affairs counsel to the five Presidents of Pfizer’s U.S. business units. He later served as a consultant to major pharmaceutical, biotechnology and medical device companies. He also served for three years as President of a small medical device company.

His career has also included senior staff positions for the Republican House leadership on Capitol Hill, the White House, and in the Governor’s office in Massachusetts. He is also affiliated as Research Fellow and Managing Director at the Center for the Study of Statesmanship at The Catholic University of America (CUA). He earned his PhD in political philosophy (with distinction) at CUA. He holds a master’s degree in political philosophy from CUA and a bachelor’s in history from Georgetown University.

**Jessica M. Tucker, PhD**
Dr. Tucker is the Director of the Division of Biosafety, Biosecurity, and Emerging Biotechnology Policy within the Office of Science Policy (OSP) at the National Institutes of Health (NIH). In this position, she manages work on a number of policy topics that impact biomedical research, including the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules, dual use research, and emerging biotechnologies.

Previously, Dr. Tucker was a Program Director at the National Institute for Biomedical Imaging and Bioengineering (NIBIB) at NIH where she managed a program on gene and drug delivery systems and on synthetic biology for technology development. Prior to her time at NIH, she worked at the U.S. Department of Health and Human Services (HHS) in the Office of the Assistant Secretary for Preparedness and Response, which she joined initially as an American Association for the Advancement of Science (AAAS) Policy Fellow. Prior to her time at HHS, Dr. Tucker was a Visiting Assistant Professor at Stony Brook University within the Department of Technology and Society, where she conducted engineering education research and lectured within the Department of Chemical and Molecular Engineering. Dr. Tucker worked for two years in research and development in the pharmaceutical industry. She holds a B.S.E. in Chemical Engineering from Princeton University and a Ph.D. in Chemical Engineering from Carnegie Mellon University.
Speaker Bios – continued

**Ramjay Vatsan, PhD**
Dr. Ramjay Vatsan, PhD is a Team Leader in the Gene Therapy Branch in the Office of Tissues and Advanced Therapies (OTAT) in CBER/FDA. He joined CBER in 2006 and prior to that he had worked in basic and translational research at National Cancer Institute/NIH and Washington University in St. Louis. Dr. Vatsan has extensive research experience in viral and bacterial vector development, gene delivery and immunotherapy. Dr. Vatsan is an expert in the CMC aspects of Cell and Gene Therapy and has co-authored several regulatory articles and CMC guidance documents. Dr. Vatsan is an ASQ Certified Quality Auditor and a full-time CMC Master Reviewer at FDA.

**Susanne Warner, MD**
Susanne Warner is an Assistant Professor of Surgery and Immuno-Oncology at City of Hope Comprehensive Cancer Center. Dr. Warner earned her undergraduate degree from the University of Texas in Austin and her medical doctorate from Texas A&M Health Science Center College of Medicine. She pursued her training as a general surgery resident at the Mayo Clinic in Phoenix, AZ. During residency, in 2010, Dr. Warner completed a research fellowship in Dr. Yuman Fong’s laboratory at Memorial Sloan Kettering Cancer Center in New York, NY. Following residency, Dr. Warner completed a clinical fellowship in hepatopancreatobiliary and advanced gastrointestinal surgery at the University of Michigan Medical Center in Ann Arbor, MI, where she was also a clinical lecturer.

Dr. Warner is the recipient of several honors and awards, including the W. Deprez Inlow Award from the Priestly Society for excellence in resident research and a mentored research scholar grant from the American Cancer Society. To date, Dr. Warner has authored 50 peer-reviewed articles, 7 book chapters, and has presented her work at numerous national and international conferences.

Dr. Warner’s research interests include clinical applications of oncolytic viral therapies, and humanities research centered on the spiritual, emotional, and physical optimization of the perioperative patient experience.

**Celia Witten, MD, PhD**
Celia M. Witten, Ph.D., M. D. is the Deputy Director of the Center for Biologics Evaluation and Research at the Food and Drug Administration (FDA/CBER). Between 2005 and 2016 she served as the Director of the Office of Cellular, Tissue and Gene Therapy at the FDA/CBER. Between 1996 and 2005 she served as Director of the Division of General, Restorative, and Neurological Devices in the Office of Device Evaluation in the Center for Devices and Radiological Health (CDRH). Previous to FDA, she worked for over 10 years as a practicing physician at the National Rehabilitation Hospital (NRH) in Washington, D.C. Her educational background includes a B.A. earned at Princeton University (Magna Cum Laude), a Ph.D. from Stanford University, and an M.D. from the University of Miami School of Medicine. In addition to her academic achievements she is Board Certified in Physical Medicine and Rehabilitation.

**Keith Wonnacott, PhD**
Dr. Keith Wonnacott has over 15 years of regulatory experience in the field of cell and gene therapies. He joined Pfizer as an Executive Director of Regulatory Affairs in their Rare Diseases Unit in 2017. At Pfizer, Dr. Wonnacott works on regulatory policy and outreach related to cell and gene therapy. He is a member of the regulatory affairs committee for both ASGCT and ARM. He also contributes to regulatory strategy on gene therapies to treat rare diseases. Dr. Wonnacott made the move to Pfizer after spending the previous 3 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 BLA leading to the FDA approval for Kymriah (tisagenlecleucel). 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 received his Ph.D. 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>Ronald Bartek, MA</td>
<td>Pfizer, Inc.: one-time consulting fee</td>
</tr>
<tr>
<td>Maria Kefalas, PhD</td>
<td>Spark Therapeutics: Unrestricted grants for foundation</td>
</tr>
<tr>
<td></td>
<td>Orchard Therapeutics: Unrestricted grants for foundation</td>
</tr>
<tr>
<td></td>
<td>bluebird bio: Unrestricted grants for foundation</td>
</tr>
<tr>
<td>Joann Parker, MS</td>
<td>Pfizer, Inc.: salary</td>
</tr>
<tr>
<td>Anne-Virginie Eggimann, MSc</td>
<td>bluebird bio, Inc.: salary, stock</td>
</tr>
<tr>
<td>Tim Hunt, JD</td>
<td>Editas Medicine: salary, ownership interest</td>
</tr>
<tr>
<td>Jugna Shah, MPH</td>
<td>Novartis: consulting fee</td>
</tr>
<tr>
<td></td>
<td>Miltenyi Biotec: consulting fee</td>
</tr>
<tr>
<td></td>
<td>Nohla Therapeutics: consulting fee</td>
</tr>
<tr>
<td></td>
<td>Actinium Pharmaceuticals: consulting fee</td>
</tr>
<tr>
<td></td>
<td>Bellicum Pharmaceuticals: consulting fee</td>
</tr>
<tr>
<td>Susanne Warner, MD</td>
<td>Coloplast: consulting fee and honoraria</td>
</tr>
<tr>
<td></td>
<td>Olympus: consulting fee and honoraria</td>
</tr>
<tr>
<td></td>
<td>AMS: consulting fee and honoraria</td>
</tr>
<tr>
<td></td>
<td>Wolf: consulting fee and honoraria</td>
</tr>
<tr>
<td>Philip Reilly, MD, JD</td>
<td>Third Rock Venture: Salary</td>
</tr>
<tr>
<td></td>
<td>bluebird bio: Stock</td>
</tr>
<tr>
<td></td>
<td>Voyager Therapeutics: options, stock</td>
</tr>
</tbody>
</table>