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Commercialization II Workshop: Value, Evidence, and Reimbursement

PROGRAM SCHEDULE

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

1:00 PM - 5:00 PM

ROOM: 304 & 306

CO-CHAIRS: Diane Berry, PhD and Philip R. Reilly, MD, JD

1:00 PM - 1:20 PM
Adapting Value Assessment for Gene Therapies
Sarah Emond, MPP

1:20 PM - 1:40 PM
Valuing Transformative Therapies
Janet Lynch Lambert, MBA

1:40 PM - 2:00 PM
Considerations for Valuing Gene Therapies for Market Viability in Europe
Allison Dupuy, PhD

2:00 PM - 2:30 PM
FDA Evidence Requirements and Determination of Treatment Population for Gene Therapies
Peter Marks, MD, PhD

2:30 PM - 3:00 PM
Panel Discussion

3:00 PM - 3:30 PM
Break

3:30 PM - 3:50 PM
A Provider Perspective on Coverage and Reimbursement of CAR T-Cell Therapy
Navneet Majhail, MD, MBBS, MS

3:50 PM - 4:05 PM
A Public Payer Perspective on Coverage and Reimbursement
John M. O’Brien, PharmD, MPH

4:05 PM - 4:25 PM
Payer Perspectives on Coverage and Reimbursement
Stephanie Farnia, MPH

4:25 PM - 5:00 PM
Panel Discussion

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

Diane Berry, PhD
Sarepta Therapeutics
Cambridge, MA

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

SPEAKERS

Allison Dupuy, PhD
Simon-Kucher & Partners
San Francisco, CA

Sarah Emond, MPP
Institute for Clinical and Economic Review (ICER)
Boston, MA

Stephanie Farnia, MPH
Blue Cross Blue Shield Association
Chicago, IL

Janet Lynch Lambert, MBA
Alliance for Regenerative Medicine
Washington, DC

Navneet Majhail, MD, MBBS, MS
Cleveland Clinic
Cleveland, OH

Peter Marks, MD, PhD
Food & Drug Administration
Silver Spring, MD

John M. O’Brien, PharmD, MPH
JMOB & Associates, LLC, Leonard D. Schaeffer Center for Health Policy & Economics
Sarasota, FL
Allison Dupuy, PhD
Dr. Dupuy is a Managing Partner and US Head of Healthcare and Life Sciences at Simon-Kucher & Partners. She is a member of the global Pharma and Biotech practice and splits her time between Simon-Kucher’s offices in Boston and San Francisco. In her work at Simon-Kucher, she focuses on strategic pharmaceutical marketing, value-to-customer, market entry strategies, pricing and market access strategies, innovative pricing strategies, alternative access solutions, licensing and valuation, market forecasting, and value communication (including global value story development and price-value communication). She is a trusted advisor of leading pharmaceutical and biotech companies across a wide range of therapeutic areas in the US, Europe, and Asia and has extensive experience in the launch and commercialization of cell and gene therapies.

Sarah Emond, MPP
With over 20 years of experience in the business and policy of health care, Sarah leads the strategic operations of the Institute for Clinical and Economic Review, a leading non-profit health policy research organization, as Executive Vice President and Chief Operating Officer. In that role, she is responsible for overseeing ICER’s public programs, stakeholder engagement, and finances. Prior to joining ICER, Sarah spent time as a communications consultant, with six years in the corporate communications and investor relations department at a commercial-stage biopharmaceutical company, and several years with a health care communications firm. Sarah began her health care career in clinical research at Beth Israel Deaconess Medical Center in Boston. A graduate of the Heller School for Social Policy and Management at Brandeis University, Sarah holds a Master of Public Policy degree with a concentration in health policy. Sarah also received a bachelor’s degree in biological sciences from Smith College. Sarah speaks frequently at national conferences on the topics of prescription drug pricing policy, comparative effectiveness research, and value-based health care. Sarah recently learned to ski and is proud that she hasn’t broken any bones doing so, and has completed seven half-marathons since she took up long-distance running in 2013.

Stephanie Farnia, MPH
Stephanie Farnia currently serves as the Director, Gene Therapy and Cellular Immunotherapy in the Office of Clinical Affairs at the Blue Cross Blue Shield Association. Ms. Farnia is responsible for providing strategic leadership and direction on advanced therapies to BCBS Companies, including clinical pipeline management, network strategies, value-based payment models and strategic engagement with external stakeholders.

Ms. Farnia has previously served in payment policy and strategic leadership roles for the American Society for Transplantation and Cellular Therapy and the National Marrow Donor Program/Be The Match. Ms. Farnia holds a Master’s degree in Public Health Policy and Administration from the University of Minnesota, where she was a Judd Fellow.

Janet Lynch Lambert, MBA
Janet Lynch Lambert joined ARM in 2017 as the organization’s first CEO. With more than 25 years in public and private sector management, Janet is an experienced government relations and business professional with an extensive record of accomplishment. Janet most recently served as the Acting Head of Engagement for the All of Us Research Program at the National Institutes of Health and as head of the Outreach Office in the Office of the NIH Director. Prior to joining NIH, she was Vice President of Government Relations and head of the Washington office of Life Technologies, aiding the company in its growth from $300 million in annual sales to more than $3 billion. Prior to Life Technologies, Janet held leadership positions in government relations, marketing and business development at large and small life science organizations, including GE and InforMax. Her experience also includes legislative and staff leadership positions in the U.S. Senate and House of Representatives. Janet received her MBA in International Business from Georgetown University and her BA in Political Science from Stanford University. She lives in the Washington, D.C. area with her husband and two daughters.
Navneet Majhail, MD, MBBS, MS
Dr Navneet Majhail is the Director of the Blood & Marrow Transplant Program at the Taussig Cancer Institute, Cleveland Clinic and Professor at the Cleveland Clinic Lerner College of Medicine. He has received medical training at the Government Medical College in Chandigarh (India), the All India Institute of Medical Sciences in New Delhi (India), the Cleveland Clinic Foundation in Cleveland, and the University of Minnesota in Minneapolis. After completing his fellowship training in Hematology-Oncology, Dr Majhail joined the University of Minnesota as Assistant Professor with their Blood and Marrow Transplant Program. Subsequently, and prior to joining the Cleveland Clinic, he was Medical Director, Health Services Research with the National Marrow Donor Program and an Adjunct Associate Professor of Medicine with the University of Minnesota. Dr Majhail is board certified in Internal Medicine and Hematology and he specializes in the care of adult patients receiving blood and marrow transplantation. His research focuses on health services and health policy issues in blood and marrow transplantation and quality of life and late effects in transplant survivors.

John O’Brien, PharmD
John O’Brien is a pharmacist, researcher and consultant who most recently served as Senior Advisor to the Secretary at the U.S. Department of Health and Human Services. Dr. O’Brien led policy development on multiple Administration priorities, including improving the affordability and access of prescription drugs. He also served as Deputy Assistant Secretary for Health Policy, and a key advisor to the Secretary for health policy research, development, and analysis related to Medicare, Medicaid, and private health insurance. Dr. O’Brien is a certified health insurance executive, held senior policy positions in the life sciences and managed care industries, was a career official at CMS during the Obama Administration, and served as a health policy fellow in the U.S. Senate. He also holds a number of academic appointments, a master’s degree in public health from the Johns Hopkins Bloomberg School of Public Health, a doctor of pharmacy degree from Nova Southeastern University, and studied pharmacy and public policy at the University of Florida.

Peter Marks, MD, PhD
Peter Marks received his graduate degree in cell and molecular biology and his medical degree at New York University and completed Internal Medicine residency and Hematology-Medical Oncology training at Brigham and Women’s Hospital in Boston. He has worked in academic settings teaching and caring for patients and in industry on drug development. He joined the FDA in 2012 as Deputy Center Director for CBER and became Center Director in January 2016.
## DISCLOSURE OF RELEVANT FINANCIAL RELATIONSHIPS

<table>
<thead>
<tr>
<th>Name</th>
<th>Disclosure</th>
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<tbody>
<tr>
<td>Stephanie Farnia</td>
<td>BCBS Association, Salary, Employment; Nimitt Consulting, Consulting Fee, Limited consulting on FFS Inpatient Medicare issues</td>
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