Emerging Technologies for Rare Diseases: Clinical and Regulatory Case Studies and Approval Pathways
Willard Intercontinental Hotel in Washington, DC
Tuesday, September 12th, 2017

8:30am - Registration and Breakfast
9:00am - Welcome Remarks, Max Bronstein, MPP, Senior Director, Health Policy and Corporate Affairs, Audentes Therapeutics
9:05am - EveryLife Foundation Overview, Emil Kakkis, MD, PhD, President, EveryLife Foundation for Rare Diseases
9:15am – The Curative and Transformative Potential of Novel Therapies for Rare Diseases in the Age of Precision Medicine, Christopher Austin, MD, Director, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

Unique Challenges of Clinical Development for Emerging Therapies
9:30am – Sara Nochur, PhD, SVP Regulatory Affairs, Alnylam
9:50am – Timothy Miller, PhD, CEO, Abeona Therapeutics
10:10am – Nick Leschly, CEO, bluebird bio
10:30am – Break

Beyond the Clinic: Bringing Emerging Therapies to Market
10:40am – John Gray, PhD, Senior Vice President & Chief Scientific Officer, Audentes Therapeutics
11:00am – Gilmore O’Neill, Senior Vice President, Drug Innovation Units, Biogen
11:30am – Break and Networking Lunch

The Role of Patient Organizations in Advancing Emerging Therapies
12:45pm – Lori Sames, Co-Founder & CEO, Hannah’s Hope Fund

Next Generation Gene Editing
1:05pm – Harry Malech, MD, Chief, Laboratory of Host Defenses and the Genetic
Immunotherapy Section National Institute of Allergy and Infectious Diseases, NIH
1:25pm – Gerry Cox, MD, PhD, Chief Medical Officer, Editas Medicine
1:45pm – Break

**Regulatory Pathways for Rare Diseases**
2:00pm – Celia Witten, MD, PhD, Deputy Director, Center for Biologics Evaluation and Research (CBER), Food and Drug Administration (FDA)
2:20pm - Rich Moscicki, MD, Deputy Center Director for Science Operations, Center for Drug Evaluation and Research (CDER), FDA

**Reimbursement, Public Perception, and Governance of Emerging Technologies**
2:40pm – Richard Bankowitz, MD, MBA, Executive Vice President, Clinical Affairs, America’s Health Insurance Plans (AHIP)
3:00pm – Cary Funk, Associate Director of Research on Science & Society, Pew Research Center
3:20pm – Final Discussion with Key Themes and Takeaways
4:00pm – Workshop Concludes

**Thank you to our 2017 sponsors**