



Scientific and Ethical Underpinnings of Gene Transfer/Therapy in Vulnerable Populations: Considerations Supporting Novel Treatments

March 7, 2019

Amicus Therapeutics, Inc., 1 Cedar Brook Drive, Cranbury, New Jersey

8:00 a.m. - 8:45 a.m.	Breakfast/Networking
8:45 a.m. - 9:00 a.m.	Welcome Debbie Hart , President & CEO, BioNJ Andrew E. Mulberg, M.D. , SVP, Global Regulatory Affairs, Amicus Therapeutics Stuart Peltz, Ph.D. , CEO, PTC Therapeutics
9:00 a.m. - 9:20 a.m.	The Challenges of Science Supporting Gene Therapy in Neurodegenerative Diseases Peter B. Kang, M.D. , Chief, Division of Pediatric Neurology, Professor of Pediatrics, University of Florida College of Medicine
Panel 1: Discussion of the Scientific and Clinical Development Challenges for Gene Therapy Treatment of Rare Neurodegenerative Disease	
9:20 a.m. - 9:40 a.m.	Overcoming the Technical Challenges of Gene Delivery to the CNS James Wilson, M.D., Ph.D. , Director, Gene Therapy Program, Professor and Director, Orphan Disease Center, Professor of Medicine and Pediatrics, Department of Medicine, University of Pennsylvania
9:40 a.m. - 10:00 a.m.	Approaches for Improved Protein Expression, Targeting and Stability for Novel Gene Therapies Hung Do, Ph.D. , CSO, Amicus Therapeutics, Inc.
10:00 a.m. - 10:20 a.m.	Accelerating Clinical Development: Contrast between CNS Rare Diseases With or Without Neurodegeneration Stuart Peltz, Ph.D. , CEO, PTC Therapeutics
10:20 a.m. - 10:40 a.m.	From Bench to Launch – Key Considerations for Gene Therapies in Rare Diseases Jodi Cook, Ph.D. , Head of Gene Therapy Strategy, PTC Therapeutics
10:40 a.m. - 11:15 a.m.	Panel 1 Discussion: James Wilson, M.D., Ph.D. , Director, Gene Therapy Program, Professor and Director, Orphan Disease Center, Professor of Medicine and Pediatrics, Department of Medicine University of Pennsylvania Hung Do, Ph.D. , CSO, Amicus Therapeutics Peter Kang, M.D. , Chief, Division of Pediatric Neurology, Professor of Pediatrics, University of Florida College of Medicine Stuart Peltz, Ph.D. , CEO, PTC Therapeutics Jodi Cook, Ph.D. , Head of Gene Therapy Strategy, PTC Therapeutics <i>Moderator: Allen Reha, VP, Program Management, Amicus Therapeutics</i>
11:15 a.m. - 11:30 a.m.	Networking Break
Panel 2: Discussion of the Ethical Framework for Clinical Development Plans for Gene Therapies in Vulnerable Populations	
11:30 a.m. - 11:50 a.m.	Establishing the Evidence Supporting "First-in-Human" Trials in Children Robert "Skip" Nelson, M.D., Ph.D. , Sr. Director, Pediatric Drug Development, Child Health Innovation Leadership Dept. (CHILD), Johnson & Johnson
11:50 a.m. - 12:15 p.m.	Future of Expanded Access to Unapproved Drugs for Newborns and Children Arthur Caplan, Ph.D. , Director, Division of Medical Ethics, Professor of Bioethics, Department of Population Health, NYU School of Medicine



12:15 p.m. - 12:35 p.m.	What Next? - An Ethical Analysis of Prioritizing Conditions and Populations for Developing Novel Therapies Armand Antommaria, M.D., Ph.D. , Director, Ethics Center, The Lee Ault Carter Chair of Pediatric Ethics & Professor in the Division of Hospital Medicine, University of Cincinnati
12:35 p.m. - 1:30 p.m.	Working Luncheon
1:00 p.m. - 1:20 p.m.	Ethical Issues in the Recruitment and Enrollment of Children with Neurodegenerative Disorders into Gene Transfer Studies Lainie Ross, M.D., Ph.D. , Carolyn and Matthew Bucksbaum Professor of Clinical Medical Ethics; Professor, Departments of Pediatrics, Medicine, Surgery and the College; Co-Director of the University of Chicago Institute for Translational Medicine, and Associate Director of the MacLean Center for Clinical Medical Ethics at the University of Chicago
1:20 p.m. - 2:00 p.m.	Panel 2 Discussion: Lainie Ross, M.D., Ph.D. , Carolyn and Matthew Bucksbaum Professor of Clinical Medical Ethics; Professor, Departments of Pediatrics, Medicine, Surgery and the College; Co-Director of the University of Chicago Institute for Translational Medicine, and Associate Director of the MacLean Center for Clinical Medical Ethics at the University of Chicago Armand Antommaria, M.D., Ph.D. , Director, Ethics Center, The Lee Ault Carter Chair of Pediatric Ethics & Professor in the Division of Hospital Medicine, University of Cincinnati Arthur Caplan, Ph.D. , Director, Division of Medical Ethics, Professor of Bioethics, Department of Population Health, NYU School of Medicine Robert "Skip" Nelson, M.D., Ph.D. , Sr. Director, Pediatric Drug Development, Child Health Innovation Leadership Dept. (CHILD), Johnson & Johnson <i>Moderator(s):</i> Jodi Cook, Ph.D. , Head of Gene Therapy Strategy, PTC Therapeutics Andrew E. Mulberg, M.D. , SVP, Global Regulatory Affairs, Amicus Therapeutics
2:00 p.m. - 2:20 p.m.	Making a Business of Gene Therapy Marcio Souza , COO, PTC Therapeutics
Panel 3: Significance of Patient Engagement in Meeting Patient Needs and Expectations for Gene Therapies	
2:20 p.m. - 3:30 p.m.	Panel 3 Discussion: Jayne Gershkowitz , Chief Patient Advocate, Amicus Therapeutics T. Craig Benson , Chairman & Founding Director, Beyond Batten Disease Foundation Pat Furlong , Founding President & CEO, Parent Project Muscular Dystrophy <i>Moderator(s):</i> Mary Frances Harmon , SVP, Corporate Relations, PTC Therapeutics Beth Allen , Patient and Professional Advocacy, Amicus Therapeutics
3:30 p.m. - 4:00 p.m.	Panel Summary and Next Steps <i>Moderator:</i> All Sections Leaders
4:00 p.m. - 4:15 p.m.	Closing Remarks Andrew E. Mulberg, M.D. , SVP Global Regulatory Affairs, Amicus Therapeutics Stuart Peltz, Ph.D. , CEO, PTC Therapeutics Debbie Hart , President & CEO, BioNJ