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Pre-Approval Commercialization Workshop: Clinical Path Toward Commercialization Sponsor

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

REGENXBIO®
# Program

## 10 AM – 10:30 AM

**Patient Perspective Keynote**

*SPEAKER:* Mark Skinner, JD

## 10:30 AM – 12 PM

**Industry Perspective on CMC Challenges for Cell and Gene Modified Cell Therapy Products**

*SPEAKERS:* Yoko Momonoi, MS  
Jason Hamilton, PhD  
Katy Spink, PhD  
*Panel Discussion Moderated by John Tomtishen*

## 12 PM – 1 PM

**Lunch**

## 1 PM – 2:30 PM

**Global Regulatory Update: Outlook in 2019 – Policy Approaches and Considerations in Gene Therapy**

*SPEAKERS:* Peter Marks, MD, PhD  
Martina Schussler-Lenz, MD  
*Panel Discussion Moderated by Adora Ndu, PharmD, JD*

*PANELISTS:* Peter Marks, MD, PhD  
Martina Schussler-Lenz, MD  
Manuela Corti, PT, PhD  
Debra Segal

## 2:30 PM – 3 PM

**Coffee break**

## 3 PM – 4:30 PM

**Manufacturing Challenges during Late Phase Development of Gene Therapy Products**

*CHAIR:* Eduard Ayuso, DVM, PhD  
*SPEAKERS:* Xiaobin Victor Lu, PhD  
Richard Snyder, PhD  
Kim Warren, PhD  
Pratik Jaluria, PhD

## 4:30 PM – 6 PM

**Hindrance to Commercializing AAV Product Candidates**

*CHAIR:* H. Trent Spencer, PhD  
*SPEAKERS:* Harrison Brown, PhD  
Fraser Wright, PhD  
Hank Fuchs, MD
Pre-Approval Commercialization Workshop: Clinical Path Toward Commercialization

Co-Chairs

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

John Tomtishen
Legend Biotech
Piscataway, NJ

Isabelle Riviere, PhD
Memorial Sloan-Kettering Cancer Center
New York, NY

Speakers

Eduard Ayuso, DVM, PhD
INSERM, University of Nantes
Nantes, France

Harrison Brown, PhD
Odylia Therapeutics
Atlanta, GA

Manuela Corti, PhD
University of Florida
Gainesville, FL

Hank Fuchs, MD
BioMarin Pharmaceutical Inc.
Novato, CA

Jason Hamilton, PhD
Legend Biotech
Piscataway, NJ

Pratik Jaluria, PhD
Adverum Biotechnologies
Menlo Park, CA

Xiaobin Victor Lu, PhD
Innovative Cellular Therapeutics
Rockville, MD

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

Yoko Momonoi, MS
Celgene
Summit, NJ

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

Martina Schussler-Lenz, MD
Paul-Ehrlich Institute
Langen, Germany

Debra Segal
Solid Biosciences
Cambridge, MA

Mark Skinner, JD
Institute for Policy Advancement Ltd
Washington, DC

Richard Snyder, PhD
Brammer Bio
Alachua, FL

H. Trent Spencer, PhD
Emory University School of Medicine
Atlanta, GA

Katy Spink, PhD
Dark Horse Consulting
Campbell, CA

John Tomtishen
Legend Biotech
Piscataway, NJ

Kim Warren, PhD
AVROBIO
Cambridge, MA

Fraser Wright, PhD
Axovant Gene Therapies
New York, NY
Speaker Bios

**Eduard Ayuso, DVM, PhD**

Eduard Ayuso obtained his degree in Veterinary Medicine in 2001 and his PhD in Biochemistry and Molecular Biology in 2006 obtained both at the Autonomous University of Barcelona. Dr. Ayuso is an expert in several viral vector platforms for gene therapy, such as adenoviral vectors, helper-dependent adenoviral vectors and adeno-associated vectors. He has made significant contributions to the field of in vivo gene transfer in small and large animal models particularly in metabolic diseases. His training in viral vectors included stays at the University of Ulm (Germany), the International Centre for Genetic Engineering and Biotechnology (Trieste, Italy) and the Children’s Hospital of Philadelphia (USA). Also, Dr. Ayuso has participated in several academic research programs in partnership with pharmaceutical and biotechnology companies. He is the scientific director of the translational vector core at the University of Nantes and belongs to the scientific advisory board of the Veterinary School of Nantes. Dr. Ayuso publication track includes >40 peer-reviewed articles and 6 patents. In 2013, he moved to the Institut National de la Santé et de la Recherche Médicale (INSERM, UMR1089) in Nantes (France) and obtained a tenure position to lead the “Innovative Vectorology” team with the main mission of improving the efficacy and safety of recombinant adeno-associated vectors for clinical gene transfer. He is the vice-president of the French Gene and Cell Therapy Society, member of the “translational science and drug product development committee” of the American Society of Gene and Cell therapy and is a key contributor of the AAV Reference Standard Material Working Group.

**Manuela Corti, PT, PhD**

Dr. Manuela Corti is currently an Assistant Professor at the University of Florida in the Child Health Research Institute and Powell Gene Therapy Center. Dr. Corti is a clinical scientist engaged in translational research focusing on understanding the contribution of neurological impairment in neuromuscular disorders by combining expertise in clinical assessment with novel therapies that rely on correcting the fundamental genetic defect. Her specific research is dedicated at developing AAV gene therapy programs for neuromuscular diseases and immunomodulation strategies to a) prevent immune responses against the AAV capsid and the transgene, and b) allow for AAV administration in pre-existing immunity. Her research interests also include outcome measures and clinical trial readiness for neuromuscular diseases such as Pompe disease, Friedreich's Ataxia and Duchenne Muscular Dystrophy.

**Harrison Brown, PhD**

Harrison Brown joined Odylia Therapeutics in 2017, where he has served as the Vice President of Operations and Chief Scientific Officer. Through Odylia, he has been working to redefine the pre-clinical and clinical landscape surrounding the development of clinically oriented AAV vectors with the goal of finding a commercially viable path to commercialization for AAV vectors designed to treat ultra-rare disorders. Prior to joining Odylia, his research focused on the development of synthetic methods of enhancing gene therapy delivery and expression of clinically-oriented AAV vectors. He received his PhD in pharmacology from Emory University.

**Hank Fuchs, MD**

Henry J. Fuchs, MD, joined BioMarin in March 2009 and currently serves as our President of Worldwide Research & Development. From December 2009 to October 2016, Dr. Fuchs served as our Executive Vice President and Chief Medical Officer. From March 2009 to December 2009, Dr. Fuchs served as our Senior Vice President and Chief Medical Officer. From September 2005 until December 2008, Dr. Fuchs served as Executive Vice President and Chief Medical Officer for Onyx Pharmaceuticals, a biopharmaceutical company. Dr. Fuchs served as Chief Executive Officer of Ardea Biosciences, Inc. from January 2003 until June 2005. Dr. Fuchs first joined Ardea Biosciences, Inc. as Vice President, Clinical Affairs in October 1996 and was appointed President and Chief Operating Officer in November 2001. From 1987 to 1996, Dr. Fuchs held various positions at Genentech, Inc. where, among other responsibilities, he led the clinical program that resulted in the approval of Pulmozyme, a therapeutic for cystic fibrosis. Dr. Fuchs was also responsible for the Phase III development program that led to the approval of Herceptin to treat metastatic breast cancer. Dr. Fuchs received an MD degree from George Washington University and a B.A. degree in biochemical sciences from Harvard University. Dr. Fuchs is currently a director of Mirati Therapeutics, a public biopharmaceutical company, and Genomic Health, Inc., a public molecular diagnostics company.
Speaker Bios – continued

**Jason Hamilton, PhD**
Dr. Jason Hamilton is currently Senior Director of Manufacturing and Development for Legend Biotech. Jason has more than 15 years of academic and industry experience in the development of cellular therapeutics. Prior to joining Legend Biotech, Jason was Technical Project Leader at Novartis, where he was a key contributor to the CMC development and global commercial filings for the first FDA-licensed CAR-T therapy (Kymriah™). Earlier in his career, Jason served as Program Head of Transplantation and Inflammation at Athersys Inc., where he led technical development of an allogeneic cell therapy product for the treatment of both Multiple Sclerosis and GvHD. Jason holds a B.S. in Neuroscience from the University of Rochester and a PhD in Neuroscience from the University of Rochester School of Medicine, with postdoctoral training at University of California, Irvine.

**Pratik Jaluria, PhD**
Pratik Jaluria is the executive director of the process development & manufacturing group at Adverum. Prior to this position, he worked for several organizations including Alexion Pharmaceuticals and Five Prime Therapeutics focused on bringing biologics to market for a variety of serious and life-threatening diseases. Earlier in his career, Dr. Jaluria worked in vaccine development at Wyeth and VaxInnate, focusing on novel therapies for infectious diseases. Dr. Jaluria is the author of numerous peer-reviewed journal articles and other publications. In addition, he is a co-inventor on several patents related to targeted gene expression and manufacturing processes. Dr. Jaluria received a Ph.D. in Biomolecular Engineering from Johns Hopkins University and B.S. in Biochemical Engineering from Rutgers University. Dr. Jaluria was awarded an Intramural Training Fellowship allowing him to conduct research at the National Institutes of Health, where he studied modulating cellular properties including growth, adhesion, and adaptation with the use of bioinformatics tools.

**Xiaobin Victor Lu, PhD**
Dr. Lu has over 18 years’ experience in product development and regulatory affairs for cellular and gene therapies. Dr. Lu is currently a Senior VP, Head of Regulatory Affairs at Innovative Cellular Therapeutics Inc. (ICT) in Rockville Maryland.

He has 10 years’ experience as a Reviewer in the Gene Therapy Branch, Division of Cellular and Gene Therapies, Office of Tissue and Advanced Therapies, Center for Biologics Evaluation and Research, US FDA with the main role of reviewing regulatory applications for cellular and gene therapy based investigational new drug (INDs) products and biologics license applications (BLAs) including CAR-T cells.

Prior to joining the FDA, Dr. Lu was a senior scientific liaison for the Vaccine Expert Committee at US Pharmacopeia (USP) for two years in charge of developing general chapters and monographs for vaccine related products.

Before joining USP, Dr. Lu served as a R & D Senior Director at ViRxSYS Corporation overseeing the product development work for first lentiviral vector-based gene therapy investigational product in human clinical trials. He completed postdoctoral training at the University of California, San Francisco (UCSF) and holds a Ph.D. from State University of New York at Buffalo in Molecular Virology. He holds a B.S. in Biochemistry from Fudan University in Shanghai, China.

**Adora Ndu, PharmD, JD**
Dr. Adora Ndu provides strategy and oversight for Global Regulatory Policy, Intelligence, Patient Engagement and Outcomes Research, as well as International Regulatory Affairs in a number of regions including LATAM, APAC and MEACIS at BioMarin Pharmaceutical, Inc. Prior to BioMarin, she served in multiple roles at the Food and Drug Administration, including as Director for FDA’s Division of Medical Policy Development where she led the development of a broad range of FDA guidances and regulations. At FDA she also held leadership roles in the Office of Prescription Drug Promotion (OPDP), and was involved in FDA’s pharmacovigilance program. Prior to FDA, Dr. Ndu was in Medical Affairs and Professional and Scientific Relations at Procter & Gamble Pharmaceuticals. Dr. Ndu received her doctor of pharmacy degree from Howard University and her JD from the University of Maryland.

**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.
Yoko Momonoi, MS
Yoko joined Regulatory CMC at Celgene in March 2018 and works on a late phase CAR-T product. Prior to Celgene, Yoko was at Novartis Pharmaceuticals where she supported the Kymriah BLA. In her role as Head of Late Stage Process Development at Novartis, she was responsible for the process characterization, comparability studies, and post-approval process development. Before joining the cell and gene therapy group at Novartis, she was in Regulatory CMC at Novartis Japan and worked on cell and gene therapy products, monoclonal antibodies, and small molecules. She started her career in biotechnology at Genentech where she worked as a process engineer.

Martina Schussler-Lenz, MD
Dr. Martina Schüssler-Lenz is the Chair of the Committee for Advanced Therapies (CAT) of the European Medicines Agency’s (EMA). The CAT is the committee responsible for evaluating the quality, safety and efficacy of marketing authorisations of cell and gene therapies (Advanced Therapy Medicinal Products, ATMPs). She is working as MD at the Paul-Ehrlich Institute in the Section Advanced Therapies and Tissue Preparations.

Debra Segal
Debra Segal is the Vice President of Regulatory Affairs at Solid Biosciences, a life science company focused solely on finding meaningful therapies for Duchenne muscular dystrophy (DMD). Deb played a key role in the IND for Solid’s investigational microdystrophin gene therapy, SGT-001. Previously, Deb was Senior Director of Regulatory Affairs at Biogen, where she led the concurrent global registration of 2 hemophilia drugs, coagulation factor IX (recombinant), Fc fusion protein and antihemophilic factor (recombinant), Fc fusion protein. In her 15+ years working in regulatory affairs at several biopharma companies, Deb has gained experience in a broad range of therapeutic areas and across all stages of development, from first-in-human studies to product approvals and product label expansions.

Mark Skinner, JD
Mark Skinner, Washington, DC, is President/CEO of the Institute for Policy Advancement Ltd, a global health company specialized in patient-centered outcomes research. He is an Assistant Professor in the Department of Health Research Methods, Evidence and Impact at McMaster University and a Senior Consultant to the Workers Compensation Research Institute. He has led both national and international patient organizations including the World Federation of Hemophilia and National Hemophilia Foundation where he currently serves on their Medical and Scientific Advisory Council. He is the principal investigator for the Patient Reported Outcomes Burdens and Experiences (PROBE) study, a global research project to enhance the direct patient voice in healthcare decision-making. He holds numerous roles as an advisor on critical blood safety and supply matters including serving on the US Health and Human Services Advisory Committee on Blood and Tissue Safety and Availability. He serves on the boards of the Institute for Clinical and Economic Review (ICER) and formerly the Patient Centered Outcomes Research Institute (PCORI) Advisory Panel on Rare Disease, where he was an inaugural member. Previously, he was Vice President State Programs at the American Insurance Association and Administrative Assistant/Chief of Staff to the Speaker of the Kansas House of Representatives. He holds degrees in Public and Business Administration from Kansas State University and JD from Washburn University School of Law.
Speaker Bios – continued

Richard Snyder, PhD
Richard O. Snyder, PhD, is the Chief Scientific Officer at Brammer Bio, a CDMO providing clinical and commercial supply of viral vectors for in vivo gene therapy and ex vivo modified-cell based therapy, along with process and analytical development, and regulatory support, enabling large pharma and biotech clients to accelerate the delivery of novel medicines to improve patients’ health. Dr. Snyder has been investigating virus biology, vector development, cGMP vector manufacturing and analytical technologies, and viral vector-mediated gene transfer for over 32 years, and was a member of teams who developed novel viral vector-based human gene therapies. Dr. Snyder was an Associate Professor of Molecular Genetics and Microbiology, and Director of Biotherapeutic Programs at the University of Florida; an Assistant Professor of Pediatrics at Harvard Medical School; and was previously employed by Cell Genesys, Somatix, Merlin, and Avigen where he was engaged in the development of gene transfer vector and vaccine technology, along with therapeutic applications. Dr. Snyder was a postdoctoral fellow at Johns Hopkins University School of Medicine, received his doctoral degree in Microbiology from The State University of New York at Stony Brook, and obtained his B.A. in Biology from Washington University in St. Louis.

H. Trent Spencer, PhD
Dr. Spencer is the Director of the Cell and Gene Therapy Program in the Aflac Cancer and Blood Disorders Center at Emory University School of Medicine, he holds the Jean R. Amos Cell and Gene Therapy Chair, is a Professor in the Department of Pediatrics, and is a co-Director of the Center for Pediatric Cellular Therapeutics at Children’s Healthcare of Atlanta. He is also the co-founder of three start-up biotechnology companies focused on developing gene therapy products and is the current president and co-founder of Expression Therapeutics, a company located in Atlanta that focuses on the development of gene therapy treatments for childhood diseases.

Katy Spink, PhD
Katy Spink, PhD joined Dark Horse as a Managing Partner in January 2018. Prior to joining Dark Horse, Dr. Spink was Chief Operating Officer of Asterias Biotherapeutics from 2013 to 2018. In this role, she led the Manufacturing, Quality, Process Development, Research, Business Development, Program Management, Facilities, and Intellectual Property functions at Asterias. Dr. Spink previously served as Senior Vice President of Alliance Management and Cell Therapy Program Operations at Geron Corporation, where she was employed in roles of increasing responsibility within the R&D and Business Development groups from 2003 through 2011. Prior to joining Geron, Dr. Spink was a management consultant at McKinsey & Company, advising clients in the biotechnology, pharmaceutical, and medical device industries on matters relating to R&D strategy, business development, and marketing. Dr. Spink graduated Magna Cum Laude and Phi Beta Kappa with a BA in Biochemistry from Rice University, and received her PhD in Cancer Biology from Stanford University.

John Tomtishen
John Tomtishen is currently employed by Legend Biotech in Piscataway, NJ. At Legend, John has oversight over Legend Biotech’s U.S. Manufacturing and Technical Operation teams and is responsible for developing a robust global CMC strategy to manufacture cell and gene therapy products, including LCAR-B38M/JNJ-68284528 in collaboration with Janssen Pharmaceuticals. Prior to joining Legend, John was employed by Novartis Pharmaceuticals within Cell and Gene Technical Development & Manufacturing in Morris Plains, NJ where he worked in a variety of CMC roles overseeing cell and gene therapy products from late stage clinical development through commercial lifecycle management. During his time at Novartis, John had an integral role in the filing and approval of the first CAR-T BLA (Kymriah™-tisagenlecleucel) for the treatment of pALL in addition to a supplemental BLA for the treatment of DLBCL.
Speaker Bios – continued

**Kim Warren, PhD**
Kim is Head of Operations for the AVROBIO, based in Cambridge, MA. AVROBIO is a clinical-stage company using lentiviral-based gene therapy and the patient’s own cells to restore normal gene function. Kim oversees all aspects of manufacturing, including coordination of CDMOs globally. Her previous experience includes 10 years at Lonza, developing a services business to support GMP manufacturing of cell and gene therapies, and heading R&D at Cambrex, developing cell-based research products. She founded and ran Poietic Technologies, acquired by Cambrex in 1999. Kim has also held research positions in pharma and biotech. She has a PhD in Comparative Physiology from Univ. of Maryland and postdoctoral training in Immunology at USUHS, Bethesda MD.

**Fraser Wright, PhD**
Dr. Wright received his PhD in 1989 from the University of Toronto (Biochemistry) for studies characterizing the interaction of complement with IgM, and completed post-doctoral studies at INSERM / CENG Grenoble, France in molecular immunology. He was awarded a CRCS/ MRC Scholarship in 1993, with faculty appointment at the University of Toronto. In 1996 he moved to industry as a Scientist at Pasteur Sanofi working on the development of cancer immunotherapies, and subsequently as Director of Development and Clinical Manufacturing at Avigen, a gene therapy company that pioneered rAAV based investigational products for hemophilia and Parkinson’s Diseases. In 2004 he returned to academia, establishing the Clinical Vector Core at the Center for Cellular and Molecular Therapeutics at Children’s Hospital of Philadelphia, gaining faculty appointment at the University of Pennsylvania Perelman School of Medicine as professor of Pathology and Laboratory Medicine. Dr. Wright has contributed to several clinical development programs in gene therapy, including those for Luxturna and Kymriah, the first approved gene therapies for genetic (RPE65 deficiency) and non-genetic (CAR-T immunotherapy) diseases, respectively, in the United States (2017). He is a Co-founder of Spark Therapeutics, served as Chief Technology Officer previously at Spark and currently at Axovant Gene Therapies, and is Principal at Wright Biologics Consulting.
# Disclosure of Relevant Financial Relationships

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<tr>
<th>Name</th>
<th>Disclosure</th>
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<tr>
<td>Eduard Ayuso, DVM, PhD</td>
<td>Consulting Fee: Esteve Pharmaceuticals</td>
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<td>Manuela Corti, PT, PhD</td>
<td>Consulting Fee and Ownership Interest: Aavanti</td>
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<td>Hank Fuchs, MD</td>
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<td>Board Compensation: Genomic Health, Inc., Mirati Therapeutics</td>
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<td>Jason Hamilton, PhD</td>
<td>Salary, Ownership and Interest: Legend Biotech</td>
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<td>Ownership and Interest: Novartis, Athersys</td>
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<td>Pratik Jaluria, PhD</td>
<td>Salary and stock: Adverum Biotechnologies</td>
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<td>Salary and stock options: Innovative Cellular Therapeutics Inc.</td>
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<td>Adora Ndu, PharmD, JD</td>
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<td>Mark Skinner, JD</td>
<td>Grants: Baxalta, Bayer, Bioverativ a Sanofi company, CSL Behring, Novo Nordisk, Roche, Sobi</td>
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<td>Debra Segal</td>
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<td>Salary and Equity: Brammer Bio</td>
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<td>H. Trent Spencer, PhD</td>
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Notes