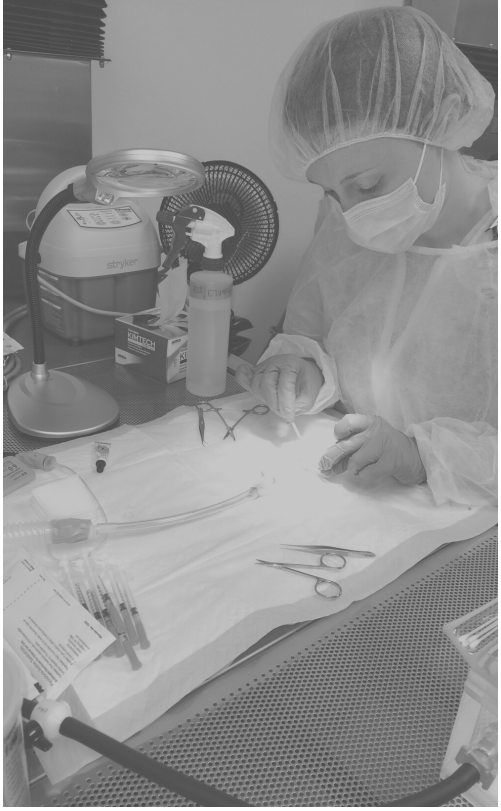

ASGCT 2021

Monday, May 10

10 a.m. - 2 p.m. ET

Recent Developments in Global Regulation of Gene Therapies

In collaboration with the European Society of Gene and Cell Therapy

EUROPEAN SOCIETY OF GENE & CELL THERAPY

Table of Contents

Sponsors

The American Society of Gene & Cell Therapy is honored to acknowledge the following organizations for their support of Recent Developments in Global Regulation of Gene Therapies:

DARK HORSE CONSULTING

Recent Developments in Global Regulation of Gene Therapies

Co-Chairs: Keith Wonnacott, Ph.D., Daniela Drago, Ph.D., RAC, and Michela Gabaldo

Session 1: Regulatory Convergence in GMO Requirements and Environmental Risk Assessment

10 - 10:15 a.m.
Recent Efforts Toward Harmonizing and Streamlining GMO Requirements
Tomáš Boráň, M.D., State Institute for Drug Control (SUKL), Czech Republic

10:15 - 10:30 a.m.
Advocacy for Exemption of ATMPs from GMO Requirements
Stuart Beattie, Ph.D., Biogen

10:30 - 11 a.m.
Panel Discussion
Moderator: Alessandro Aiuti, M.D., Ph.D., San Raffaele Telethon Institute

- Stuart Beattie, Ph.D., Biogen
- Tomáš Boráň, M.D., SUKL, Czech Republic
- Michael Havert, Ph.D., bluebird bio
- Gentaro Tajima, Pfizer

Recent Developments in Global Regulation of Gene Therapies

Session 2: Hot Topics and Emerging Trends in the Regulation of Gene Therapies

11 - 11:30 a.m.
Moderated by Daniela Drago, Ph.D., RAC

- Wilson Bryan, M.D., FDA CBER, Office of Tissues and Advanced Therapies
- Martina Schüssler-Lenz, M.D., Paul-Ehrlich Institute, EMA Committee for Advanced Therapies

Session 3: Industry Experience in the Global Development of Gene Therapies

11:45 a.m. - 12 p.m.
Keith Wonnacott, Ph.D., Pfizer

12 - 12:15 p.m.
Michael Ladd, PharmD., Orchard Therapeutics

12:15 - 12:30 p.m.
Katie Picone, PharmD., Novartis

12:30 - 1 p.m.
Panel Discussion
Moderator: Dan Takefman, Ph.D., Takefman Gene Therapy Advisors LLC

Recent Developments in Global Regulation of Gene Therapies

Session 4: Use of Regulatory Reliance for Regenerative Medicines/Advanced Therapies

1 - 1:15 p.m.
Use of Reliance-Based Regulatory Pathways
Murray Lumpkin, M.D., The Bill and Melinda Gates Foundation

1:15 - 1:30 p.m.
Use of Regulatory Reliance for Regenerative Medicines/Advanced Therapies
Lawrence Liberti, Ph.D., Centre for Innovation in Regulatory Science

1:30 - 1:45 p.m.
Access Work-Sharing: An Innovative Model of International Regulatory Collaboration
Michael Shum, Australian Therapeutic Goods Administration

1:45 - 2 p.m.
Panel Discussion
Moderator: Daniela Drago, Ph.D., RAC

Alessandro Aiuti, M.D., Ph.D.
San Raffaele Telethon Institute

Alessandro Aiuti is Deputy Director, Clinical Research Coordinator, Head of Unit on Pathogenesis and Therapy of PID and Head of Pediatric Clinical Research Unit of SR-Tiget, Milan; Director of the Pediatric Immunohematology Unit, San Raffaele Hospital, Milan; full Professor of Pediatrics, and Director of the Residency Program of Pediatrics at Vita-Salute San Raffaele University, Milan, Italy. He is M.D., specialized in Immunology, and Ph.D. in Molecular and Cell Biology. In 1998 he obtained the National Board in Hematology. His main interests are hematology, immunology, and pediatrics, particularly in the study of PID. His main domains of research are HSC gene therapy for genetic diseases (PI of 5 clinical trials: ADA-SCID, WAS, beta thalassemia, MLD and MPS1), clonal dynamics of hematopoiesis, genetics and pathogenesis of primary immunodeficiencies. He has published more than 200 papers in international peer reviewed journal including Science, Nature Medicine, New England Journal of Medicine, Lancet, Journal of Experimental Medicine, Blood.

Stuart Beattie, Ph.D.
Biogen

Stuart has over 20 years' experience in the field of cell and gene therapy. Following a post-doc and fellowship with Dr. Terry Flotte at the University of Florida and UMass Medical School, as the field of gene therapy has developed, Stuart took up positions within industry, moving from research to the development of AAV-based programs. Stuart is a Regulatory CMC Gene Therapy Clinical Lead for early and later phase programs at Biogen. Stuart is a member of the EFPIA and ARM, where the majority of the advocacy efforts to exempt ATMPs from EU regulation have stemmed from.

Tomáš Boráň, M.D.

State Institute for Drug Control (SUKL), Czech Republic

2000-2008: Research Assistant, Institute of Experimental Medicine ASCR, Department of Teratology, Czech Republic; 2008-2017: Clinical Trial Assessor, State Institute for Drug Control, Czech Republic; 2009-2017: Lecturer, 3rd Medical Faculty, Dpt. of Embryology and Teratology, Charles University, Prague, Czech Republic; 2017-present: Director of the Marketing Authorisation Section, State Institute for Drug Control, Czech Republic; Expertise in Regulatory Science, Histology and Embryology, Advanced Therapy Medicinal Products, Nanomedicines, Clinical Trials and GMO; Alternate member of the Committee for Advanced Therapies (CAT) EMA; CZ representative of EU Innovation Network; External member of the Czech Committee for GMO.

Wilson Bryan, M.D.
FDA CBER, Office of Tissues and Advanced Therapies

Wilson Bryan is a neurologist who graduated from the University of Chicago Pritzker School of Medicine. He served on the neurology faculty of the University of Texas Southwestern Medical School for 13 years. He has been an investigator on clinical trials in cerebrovascular disease and neuromuscular disorders, particularly amyotrophic lateral sclerosis. Dr. Bryan joined the United States Food and Drug Administration (FDA) in 2000, and now serves as Director of the Office of Tissues and Advanced Therapies (OTAT) in the Center for Biologics Evaluation and Research (CBER).

Daniela Drago, Ph.D., RAC
Biogen

Dr. Daniela Drago is Senior Director Regulatory Sciences at Biogen. Before joining Biogen, she was an Assistant/Associate Professor at George Washington University's School of Medicine and Health Sciences. During her career, Dr. Drago has held senior positions in global regulatory and medical affairs at F. Hoffman-La Roche, Vifor Pharma, and Bausch & Lomb. She has significant experience in the development of small molecules, biologics, gene therapies, and combination products in rare and common disease areas, including oncology, ophthalmology, and neurology. Currently, she serves on the Regulatory Affairs Certification's Board of Directors, the Executive Committee of the American Course on Drug Development and Regulatory Science (ACDRS), and the Regulatory Affairs Committee of the American Society of Gene and Cell Therapy (ASGCT). She is a fellow of the Regulatory Affairs Professional Society (FRAPS) and of The Organisation for Professionals in Regulatory Affairs (FTOPRA). In recognition of her achievements, Dr. Drago received several awards, including the TOPRA Award for Regulatory Excellence and the RAPS Community Leadership Award. She received her Ph.D. in chemistry from the Swiss Federal Institute of Technology (ETH Zurich, Switzerland).

Michela Gabaldo
Fondazione Telethon

Michela Gabaldo is Head, Alliance Management & Regulatory Affairs at Fondazione Telethon in Milan (Italy). Within this role she is supporting the 2 internal Scientific Institutes in Italy, SR-TIGET (San Raffaele-Telethon Institute for gene therapy in Milan) and TIGEM (in Naples) in the progression of gene therapy projects (ex-vivo and in-vivo) for rare genetic disease at different stages of development spanning from preclinical up to patient access. These programs are developed both internally and in partnership with relevant pharmaceutical industries or through start-up. Within this role she has been actively involved in the development and access of the ex-vivo gene therapy registered worldwide, Strimvelis in 2016 and in the Libmeldy program who has reached recently the EU approval. Before joining Telethon in 2011 she has been working in the pharma industry for GSK in Clinical Operations and CMC Regulatory Affairs areas for New Chemical Entities (NCE) development. After graduation in Pharmaceutical Chemistry and Technology, she granted a master's degree in Regulatory Affairs and Market Access for NCEs and Biopharmaceutical. Winner of 2017 edition of Top RA Award in the Future category, Michela offers up to date knowledge of the most recent regulations in ATMPs and Orphans. She is currently a member of the IRDIRC (International Rare Disease Research Consortium) Therapies Scientific Committee.

Michael Havert, Ph.D.
bluebird bio

Mike started as a Senior Director of Regulatory CMC at bluebird in February 2019. Before coming to bluebird, Mike worked in the FDA Gene Therapy Branch (GTB) for 15 years. Mike has long been interested in studying viruses, and this interest fits well with the diverse group of technologies that have historically been used in gene therapy applications. As CMC Reviewer in GTB he assessed how products were made and tested, the science supporting their development and use in clinical studies, and potential impacts on patient safety and environmental exposure. Mike's recent accomplishments at FDA include serving as BLA Review Chair for one of the three GTB BLA approvals in 2017, championing the release of new GT guidance documents and representing FDA in efforts to harmonize global GT regulation. Prior to joining FDA, Mike received a Ph.D. from the University of Wisconsin, conducted postdoctoral fellowships in virology at JHU (Sindbis virus) and NIH (HBV/HCV) and taught biology at a local Community College in Virginia.

Michael Ladd, PharmD.
Orchard Therapeutics

Since January 2021, Dr. Michael Ladd currently serves as VP of Regulatory Science at Orchard Therapeutics. Prior, he was with Sanofi as their Senior Director and Global Regulatory Affairs Leader. His expertise lies in global regulatory strategies relating to health authority interactions, registrational study designs, and clinical medical affairs. Dr. Ladd received a B.S. in Pharmacy at the University of Connecticut before obtaining his Doctorate in Pharmacy from the University of Colorado in 2008.

Lawrence Liberti, Ph.D.
Centre for Innovation in Regulatory Science

Dr. Liberti has worked in the fields of pharmaceutical regulatory affairs, communications, and clinical R&D for the past four decades. He began his career at Wyeth Laboratories working in product development, then as a regulatory writer in clinical R&D, and manager of safety surveillance in medical affairs. He served as the editorial director for the North American operations of ADIS international after which he founded PIA Ltd, a company specializing in regulatory writing and consulting; he co-founded Astrolabe Analytica under which he helped develop, patent and commercialize the Astrolabe Message Mapping System. From 2009 to 2019 served as the Executive Director of CIRS (the Centre for Innovation in Regulatory Science, Ltd, forming part of Clarivate Analytics). In 2019 he transitioned to Head of Regulatory Collaborations for CIRS; he retired from CIRS in February 2021 and now conducts research and teaches at Temple University and Utrecht University. He has been actively involved in promulgating best practices with regard to reliance and facilitated regulatory pathways. Dr. Liberti received his doctorate in International Regulatory Policy through the WHO Collaborating Centre for Pharmaceutical Policy and Regulation based in the Utrecht Institute for Pharmaceutical Sciences, Utrecht University, the Netherlands, where his research centered on expedited regulatory pathways with applicability in the emerging markets. He received both his Bachelor of Science in pharmacy and master's degree in pharmacognosy from the Philadelphia College of Pharmacy and Science (now the University of the Sciences in Philadelphia). He attained the status of Regulatory Affairs Certified (RAC) with the Regulatory Affairs Professional Society and was awarded an honorary Doctor of Science degree by his alma mater. He is a Fellow of the American Medical Writers Association and is a recipient of their Golden Apple award for excellence in teaching.

Murray Lumpkin, M.D., M.Sc.
The Bill and Melinda Gates Foundation

Murray ("Mac") Lumpkin became Deputy Director - Regulatory Affairs and Lead for Global Regulatory Systems Initiatives at the Bill and Melinda Gates Foundation in January 2014. In this capacity, he leads the Foundation's strategic initiatives around global regulatory systems. These initiatives currently include the Foundation's regulatory systems optimization work with partners such as: (1) the World Health Organization and other multinational organizations, (2) regional and other regulatory harmonization/alignment initiatives, and (3) national and regional medical products regulatory authorities. These initiatives are focused on working with these and other partners to make more efficient and effective (without sacrificing product quality or safety) the regulatory processes through which products must pass in order to be developed, legally marketed, and overseen appropriately after marketing authorization in low- and middle-income countries. Dr. Lumpkin retired from the U.S. Food and Drug Administration in January 2014 after just over 24 years as a part of that organization. From 2011 to 2014, he was the Commissioner's Senior Advisor and Representative for Global Issues. From 2001-2011, he was responsible for the policy development and operational aspects of the FDA's international activities, most recently as Deputy Commissioner for International and Special Programs (2005-2011) during which, under his leadership, FDA's foreign posts were established and FDA's confidentiality arrangements and in-depth working relationships and harmonization activities with its foreign counterpart agencies were designed and implemented. From 1993-2000, he was the Deputy Center Director (Review Management) of FDA's Center for Drug Evaluation and Research (CDER), and from 1989-1993, he was the Director of CDER's Division of Anti-infective Drug Products.

Katie Picone, PharmD.
Novartis

Katie is currently working at Novartis Gene Therapies as the Executive Director of Global Regulatory Affairs. Prior, she was the head of the Asian Pacific branch of Regulatory Affairs before beginning her current role in June of 2020. Katie had previously earned a bachelor's degree in Chemistry from Villanova and gained her Doctorate in Pharmacy from the University of Buffalo, immediately followed by a two-year fellowship position at Rutgers. Her concentration in clinical research has aided her prior position at Merck, as well as her current role at Novartis.

Martina Schüssler-Lenz, M.D.

Paul-Ehrlich-Institut, EMA Committee for Advanced Therapies

In 2020, Dr. Martina Schüssler-Lenz was elected on her second mandate as 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).
Dr. Schüssler-Lenz received her medical degree at Mainz University, Germany. She is board certified in internal medicine, worked many years in Hematology/Medical Oncology and was a research fellow at Memorial Sloan Kettering Cancer Center and at the Instituto Municipal de Investigacion Medica in Barcelona, Spain, before she joined pharmaceutical industry for clinical drug development projects in haemato-oncology. In 2005 she joined the Paul-Ehrlich-Institut, the German Federal Agency for Vaccines and Biomedicines, and has been a member of the EMA Committee for Advanced Therapies for many years before she was elected as its chairperson in February 2017.

Michael Shum

Australian Therapeutic Goods Administration

Michael Shum is the Director of the Application and Advisory Management Section in the Prescription Medicines Authorisation Branch at the Australian Therapeutic Goods Administration. Michael joined TGA in 2008. In his current role he leads the TGA's team of regulatory project managers and coordinates TGA's participation international regulatory collaborations, in particular work-sharing through the Australia-Canada-Singapore-Switzerland-United Kingdom (ACCESS) Consortium and the US FDA's Project Orbis. In previous roles at the TGA Michael has worked in medical devices, prescription and OTC medicines regulatory reform and product evaluation.

Gentaro Tajima
Pfizer

Mr. Gentaro Tajima received his Master of Science from Shinshu University, Nagano, Japan, in 1998. He is expected to earn his Ph.D. in March 2022. He is currently a Japan Regulatory Lead and has specialized in the gene therapy field for more than 6 years at the Department of Regulatory Affairs, Pfizer R&D Japan G.K. in Tokyo, Japan.

Daniel Takefman, Ph.D.
Takefman Gene Therapy Advisors

Dan provides expert regulatory advice for the development and commercialization of cell and gene therapies. Previously Dr. Takefman was SVP and Head of Regulatory Affairs at Spark Therapeutics for five years. At Spark, Dr. Takefman supervised the submission through to approval of the FDA and EMA Luxturna (voretigene neparvovec) marketing applications. Dan also supervised the regulatory process for multiple AAV based investigation products including two additional Breakthrough Designation products: SPK-9001 for the treatment of Hemophilia B and SPK-8011 for the treatment of Hemophilia A. Dan was also the Chief of the Gene Therapy Branch within the U.S. Food and Drug Administration (FDA). He supervised the Chemistry, Manufacturing and Control (CMC) review process for all gene therapy products and for a variety of therapeutic vaccine products. Dan began his career at FDA in 1999 as a Postdoctoral Fellow and became a Staff Fellow shortly thereafter. Dr. Takefman holds a Ph.D. in microbiology from Rush University and a B.S. in microbiology from the University of Iowa.

Keith Wonnacott, Ph.D.
Pfizer

Dr. Keith Wonnacott has 20 years of regulatory experience in the field of cell and gene therapies. He currently works at Pfizer as an Executive Director of Regulatory Affairs in their Rare Diseases Unit. At Pfizer, Dr. Wonnacott leads efforts to develop regulatory strategy and policy related to cell and gene therapy. He is a member of the regulatory affairs committee for both ASGCT and ARM; he is on working groups related to advanced therapies with PhRMA, BIO, and EFPIA; and participates with other organizations that work on cell and gene therapy policy. He also contributes to regulatory strategy for AAV-based 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 Kymriah (tisagenlecleucel) BLA and coordinating CMC activities which led to its approval in 2017. 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 has published several articles and book chapters on the regulation of cellular therapies. 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.

Disclosures

A. Aiuti
Orchard Therapeutics Ltd.; Research support. OTL is sponsor of gene therapy clinical trials; P.I. of Gene Therapy clinical trials presently sponsored by OTL

D. Drago
BioGen, Inc.; Salary; Employee

L. Liberti
Temple University; Stipend; Teaching

D.M. Takefman
Abintus Bio, Inc.; Scientific advisory board member

Several cell and gene therapy companies; Paid consultant

K. Wonnacott
Pfizer; Employer