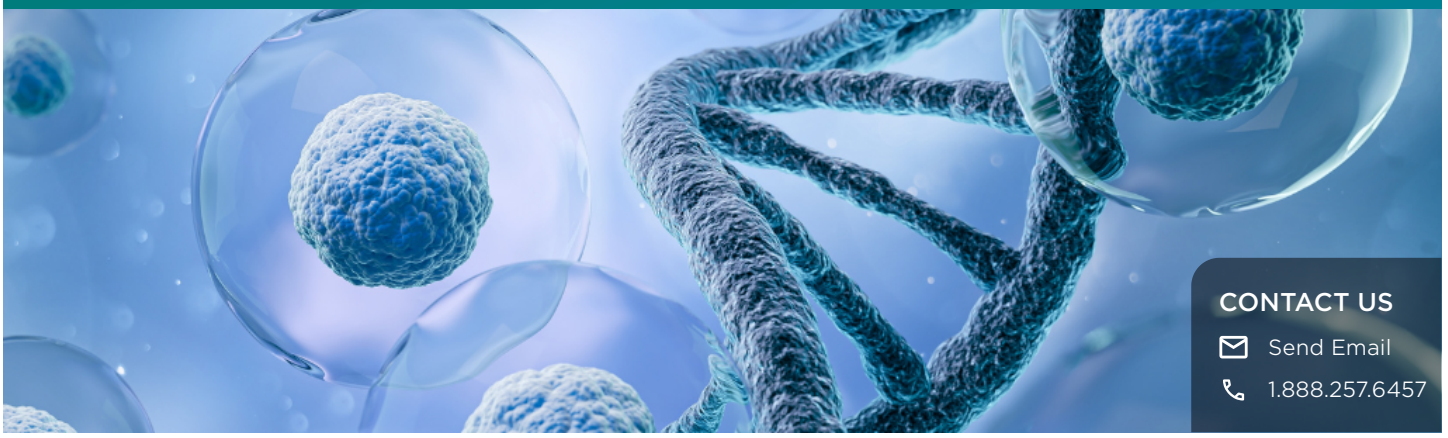
Walter E. Washington Convention Center

Jun 15, 2025 7:15 AM - Jun 15, 2025 4:30 PM

801 Allen Y. Lew Place, NW, Washington, DC 20001-3614, USA

Cell and Gene Therapy Summit

Join multi-stakeholder dialogue, identifying the barriers to progress and charting the best path forward in global regulatory harmonization for cell and gene therapies.



Print Agenda

Day 1 Jun 15, 2025

7:15 AM — 8:00 AM

Registration and Continental Breakfast

8:00 AM — 8:10 AM

Welcome and Opening Remarks from the Program Chairs: The Importance of Global Harmonization in Cell and Gene Therapies

Welcome and Opening Remarks from the Program Chairs: The Importance of Global Harmonization in Cell and Gene Therapies

Session Chair(s)

Raju Kucherlapati

Paul C. Cabot Professor of Genetics and Professor of Medicine
Harvard Medical School, United States

Maria Vassileva, PhD

Chief Science and Regulatory Officer
DIA, United States

Dr. Vassileva has decades of experience with managing complex multi-stakeholder biomedical research programs. She spent most of her career in the nonprofit sector, leading the Science Team at the Arthritis Foundation, and working at the Foundation for NIH and the American Association for the Advancement of Science. She was also on the leadership teams of two health research organizations, serving as project director on multiple government contracts. Her areas of expertise include musculoskeletal, metabolic, immunity and inflammation disorders, as well as patient engagement. She received her PhD in Biochemistry and Cell Biology from Johns Hopkins.

8:10 AM — 8:40 AM

Session 1: An Overview of the Evolution and Impact of Cell and Gene Therapy Field

Genome editing is perhaps one of the most significant advancements in biomedical sciences over the past two to three decades. Since scientists' first attempts at genome editing in the 1970s and 1980s, the field has rapidly evolved, particularly with the introduction of CRISPR in 2009 — a more agile and cost-effective technology compared to earlier genome editing methods. These advancements have revolutionized medicine by offering pioneering potential treatment for genetic disorders, rare diseases, and certain types of cancer. With the development of novel delivery systems, advancements in manufacturing scalability, and next-generation gene editing tools, the field of cell and gene therapy (CGT) has helped shift experimental treatments to real-world clinical applications. However, challenges persist in these therapies, including safety concerns, ethical considerations, regulatory complexities and commercialization hurdles.

This session will overview the history of CGT and highlight the scientific breakthroughs that were made along the way amid variable regulatory landscapes and summarize the opportunity for impact on patients in many different therapeutic areas, including oncology, rare disease, Type 1 diabetes, etc. Attendees will be encouraged to contemplate on the future of CGTs and their impact on patient care.

Learning Objective :

- Describe the role of CGTs in treating genetic disorders, rare diseases, and cancers
- Learn about how different regulatory landscapes have shaped CGT adoption and current challenges in CGT development
- Summarize the evolution of genome editing and its impact on CGTs

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Speaker(s)

Panelist

John Tisdale, MD

Senior Investigator, Cellular and Molecular Therapeutics, NHLBI
National Institutes of Health (NIH) , United States

8:40 AM — 10:00 AM

Session 2: Panel Discussion on the Challenges of Global Development for Cell and Gene Therapies

Global regulatory frameworks have evolved in tandem with the rapid advancements in cell and gene therapies. This session will review the latest regulatory guidance documents from global regulatory agencies and identify strategies that have been used to address key challenges in the field, such as protecting patient safety and product efficacy. Given the need for efficiency in a fast-moving landscape yet a balance of regulatory rigor, panelists will draw attention to how existing regulatory frameworks have adapted to the increase of cell and gene therapies and recent opportunities for global harmonization of regulatory processes. The session will highlight examples of international collaboration, how approval pathways have been streamlined, and regulatory trends that are molding the future of cell and gene therapy.

Learning Objective :

- Differentiate global harmonization efforts and understand the role of international collaboration in approval pathways

- Evaluate the impact of recent global regulatory guidance on cell and gene therapy development
- Identify key regulatory challenges in balancing safety, efficacy, and efficiency

Session Chair(s)

Raju Kucherlapati

Paul C. Cabot Professor of Genetics and Professor of Medicine
Harvard Medical School, United States

Speaker(s)

Speaker

Renata Miranda Parca, AHIP

Health regulatory specialist
ANVISA, Brazil

I hold a degree in Biology from the University of Brasília, where I also completed my master's degree. I work as a health specialist at Anvisa since 2005, in the office of blood, tissues, cells, and advanced therapy medicinal products.

Speaker

Yasuhiro Kishioka, PhD

Review Director, Office of Cellular and Tissue-based Products
Pharmaceuticals and Medical Devices Agency (PMDA), Japan

Dr. Kishioka is a Review Director of the Office of Cellular and Tissue-based Products, PMDA. In this role, he is responsible for the assessment of regenerative medical products (gene/cell therapy products). Since joining PMDA in 2008, Dr. Kishioka has been involved in various international activities including ICMRA (collaborative assessment pilot under PQKMS), ICH (Q12 and M4Q(R2)), WHO, APEC, and IPRP (Biosimilar WG). He also served as ICH Technical Coordinator (2019-2020) and MHLW/PMDA international liaison official at EMA (2020-2022). He obtained a Ph.D. from Hokkaido University in Meat Science with emphasis in Molecular Biology.

Speaker

Nicole Verdun, MD

Director, Office of Therapeutic Products, CBER
FDA, United States

Dr. Verdun joined FDA in 2012, first in the Office of Hematology Oncology Products as a medical officer and a liaison for sickle cell therapeutics and anticoagulants, and then Therapeutic Biosimilars. She was appointed as the Deputy Director of the Office of Blood Research and Review in the Center for Biologics Evaluation and Research (CBER) in October 2016 and was promoted to Office Director in 2018. In 2023, Dr. Verdun was selected as the Super Office Director of the Office of Therapeutic Products, overseeing 6 Offices dedicated to the regulation and approval of Cell

and Gene therapies in the United States. She overseas both a research and regulatory portfolio in CBER. She is also on staff at Children's National Medical Center.

Speaker

Christine Ho, RPh

Director, Advanced Therapy Products Branch
Health Sciences Authority, Singapore, Singapore

Speaker

Julian Beach

Interim Executive Director, Healthcare Quality and Access
Medicines and Healthcare Products Regulatory Agency (MHRA), United Kingdom

Speaker

Evdokia Korakianiti, PhD, MSc

Head of Quality and Safety of Medicines
European Medicines Agency, Netherlands

Evdokia joined the Agency in 2002. Since 2020, she is leading the Quality and Safety Department, which includes Quality, GxP Compliance, Referrals and PhV. She is the EMA lead of the EU Network's strategic priority to facilitate the uptake of advanced manufacturing approaches. She is also representing the Agency at the ICMRA PQKMS core group. She has trained as a pharmacist in the School of Pharmacy of the University of Athens and has received a M.Sc. and a Ph.D. in Pharm. Technology from the same Faculty.

10:00 AM — 10:15 AM

Refreshment and Networking Break

10:15 AM — 10:45 AM

Session 3: Cells as Living Medicines: A New Approach to Treat Type 1 Diabetes

Dr. Melton will talk about his application of developmental biology to understand and change the course of diabetes. In his lab, he has developed methods to make hundreds of millions of functional beta cells from human stem cells. Come hear about the innovative clinical trial of a potential cell therapy for T1D.

Session Chair(s)

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DIA, United States

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Speaker(s)

Speaker

Doug Melton, PhD

Distinguished Research Fellow
Vertex, United States

Dr. Melton is a distinguished research fellow at Vertex Pharmaceuticals where initial studies of people with type 1 diabetes who have received stem cell-derived islet cells (SC-islets) transplanted together with immunosuppressive drugs have shown promising results. Previously he served as the Director of the JDRF Center of Excellence in New England, a research collaboration with investigators (past and present) from Harvard and UMass Chan Medical Schools, The Jackson Laboratory, Joslin Diabetes Center, the Dana Farber Cancer Institute, and Boston Children's Hospital. The Center is currently led by David Harlan, MD, co-director of the UMass Chan DCOE.

10:45 AM — 12:05 PM

Session 4: Case Studies: Discussing the Challenges and Opportunities of Cell and Gene Therapy Development in Different Therapeutic Areas

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Speaker(s)

Case Study 1: The Development of CAR T to Serve Patients Needs

Lana Shiu, MD

Global Head of Regulatory Affairs
Kite Pharma (Gilead), United States

Case Study 2: Gene Therapy for Pediatric Hematology Disorders

Alessandro Aiuti, MD, PhD

Deputy Director
San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Hospital, Italy

12:05 PM — 12:25 PM

Session 5: Platform Technologies: An Approach to Development and Regulation

This discussion will explore genome editing as a platform technology in cell and gene therapy, learn about recent advancements in precision medicine, and assess its broader therapeutic applications and role in regenerative medicine. Attendees will gain insights into the challenges faced when scaling these therapies, regulatory and manufacturing considerations, and are encouraged to share their own experience and strategies for ensuring safety, efficacy, and accessibility.

Learning Objective :

- Learn how genome editing functions as a platform technology in cell and gene therapy development
- Differentiate key regulatory and manufacturing considerations for scaling genome-edited therapies
- Discuss the potential challenges and opportunities in integrating genome editing into regenerative medicine

Session Chair(s)

Representative Invited

DIA, Switzerland

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Speaker(s)

Speaker

Michael Lehmicke, MSc

Senior Vice President, Science and Industry Affairs
Alliance for Regenerative Medicine, United States

Michael has over 20 years of R&D experience in biomaterials, medical devices and regenerative medicine. He has led product development teams for class II devices, human cell and tissue-based products, and drug/device combination products. He is a creator and an inventor with multiple U.S. patents to his name. Michael has a MSc in /Biomedical Engineering, with a focus on tissue engineering, from Drexel University. Michaels areas of expertise include cell-based tissue engineering, bioceramics, biodegradable polymers, project management, strategic pipeline development, and business development.

12:25 PM — 12:40 PM

Session 6: Enabling Innovation: Regulatory and Cross-Sector Strategies from the AMP BGTC to the Future of Cell Therapies

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Session Chair(s)

Kira Gillett, MS

Program Manager, Science Partnerships
Foundation for the National Institutes of Health, United States

Kira Gillett is the Program Manager of Rare Diseases, Translation Science at the Foundation for the National Institutes of Health (FNIH). She has more than 12 years of experience in the scientific industry, including over 7 years managing complex projects and programs in the cell and gene therapy field. Her prior roles include scientific and project management positions at contract research organizations, supporting nutritional chemistry, GMP small molecule testing, and large molecule initiatives. Kira holds a B.A. in Biology and an M.S. in Biotechnology from the University of Wisconsin–Madison.

Speaker(s)

12:40 PM — 1:40 PM

Networking Luncheon

1:40 PM — 3:50 PM

Session 7: Roundtable Discussions

In Session 6, some of the most pressing challenges in advancing cell and gene therapies will be on the table, as global experts engage in three focused roundtables covering regulatory, access, and chemistry, manufacturing, and controls (CMC) issues that are shaping the CGT landscape.

The first roundtable will explore how regulatory frameworks can evolve to keep pace with rapid scientific advancements in cell and gene therapies, support innovation, and enable global regulatory convergence. The second roundtable will focus on addressing access barriers in developing countries, highlighting strategies to ensure that patients with urgent and unmet medical needs can benefit from newly approved cell and gene therapies. The third roundtable will center on CMC challenges in cell and gene therapy production, with a focus on scalability, quality control, and evolving regulatory standards across global markets.

Attendees will have the opportunity to engage directly with thought leaders, exchange perspectives, and contribute to identifying collaborative solutions that can accelerate the development and global delivery of safe, effective, and accessible cell and gene therapies.

Learning Objective :

- Examine regulatory, access, and manufacturing challenges in advancing CGTs
- Evaluate global strategies to improve patient access and regulatory alignment
- Understand key CMC considerations impacting CGT scalability and quality
- Consider collaborative approaches to support global delivery of CGTs

Session Chair(s)

Maria Vassileva, PhD

Chief Science and Regulatory Officer
DIA, United States

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Speaker(s)

Topic 1: CMC Hurdles with Cell and Gene Therapy Manufacturing

Lesbeth Caridad Rodriguez, MS

Director, Regulatory Affairs Policy and Science
Bayer, United States

Lesbeth Rodriguez, Director, Regulator Policy and Science at Bayer, is a regulatory policy leader specializing in advanced therapies and Chemistry, Manufacturing, and Controls (CMC) across all modalities. She represented PhRMA in the ICH Q13 Implementation Working Group and currently represents PhRMA in the ICH Cell and Gene Therapy Discussion Group. In addition, she is the Co-Chair of BIO Regenerative Medicine Committee and a member of the ARM US Policy Advisory Group driving harmonization efforts, regulatory efficiency, and policies that enhance patient access.

Topic 1: CMC Hurdles with Cell and Gene Therapy Manufacturing - Moderators

Tamei Elliott, MS

Director, Global Scientific Content
DIA, United States

Tamei Elliott, MS, serves as the Associate Director of Scientific Programs for the Americas region at DIA. In this pivotal role, she is responsible for identifying and prioritizing content areas and topics crucial to DIA constituents. Tamei assesses the implications of significant regulatory and health policy changes, seamlessly integrating relevant content into the development and advancement of DIA conferences and courses. Her responsibilities extend to overseeing content development and strategy within the Americas region.

Topic 1: CMC Hurdles with Cell and Gene Therapy Manufacturing - Moderators

Sandra Blumenrath, PhD, MS

Executive Editor, Scientific Publications & Senior Scientific Program Manager
DIA, United States

Dr. Sandra Blumenrath is the Executive Editor of Scientific Publications & Sr. Scientific Program Manager at DIA, where she oversees the organization's publications portfolio and leads research projects in rare neurological disorders. She collaborates with regulators, industry leaders, non-profits, academia, and patient advocacy groups. Dr. Blumenrath holds a Ph.D. in Biology from the University of Maryland, College Park, and an M.S. from the University of Copenhagen, Denmark. With international experience working across the U.S., Denmark, and Germany, she specializes in science communication, open science advocacy, strategic publication management, and interdisciplinary collaboration to share scientific knowledge and advance global health.

Topic 2: Regulatory Challenges: Ideas of how to best harmonize regulations globally and adapt existing regulatory processes in the context of application to cell and gene therapies

Monica Veldman

Director, Global Regulatory Policy
Alliance for Regenerative Medicine, United States

Monica Veldman is the Director of Global Regulatory Policy at the Alliance for Regenerative Medicine, where she leads U.S. and EU regulatory strategy through ARM's advisory groups. She focuses on proactive influence, convening expert stakeholders to shape and respond to CGT policy developments across FDA, EMA, and MHRA. She also leads the development of regulatory comment letters and policy positions, driving coordinated advocacy across ARM's regulatory, scientific, and public affairs teams. Before joining ARM, Monica was Associate Director of Regulatory Policy at Genentech, where she led policy initiatives and strategic communication across CGT, DCTs, inclusive research, and PDUFA implementation.

Topic 2: Regulatory Challenges - Moderators

Radha Goolabsingh

Global Regulatory Strategist
DIA, United States

Topic 2: Regulatory Challenges - Moderators

Sara Torgal, MPharm

Global Regulatory Policy Lead
DIA, Switzerland

Sara is currently Senior Manager, Scientific Programmes at DIA. In the EMEA region, she is responsible for engaging with external stakeholders and advancing the scientific content strategy by creating opportunities to integrate scientific and regulatory changes of interest in DIA initiatives. Additionally, she is responsible for the regional patient engagement and learning design initiatives, being the liason for the Middle East and SEE regions. Previously, she was

Public Health Promotion Projects Manager at the Portuguese Pharmaceutical Society. Sara is a Master of Pharmacy since 2015 and a Soft skills Trainer since 2012, having delivered over 300h of Training internationally primarily focused on creating impactful interactions.

Topic 2: Regulatory Challenges - Moderators

Ralf Herold, DrMed, MD

Head of Regulatory Science and Academia Workstream
European Medicines Agency, Netherlands

Ralf Herold MD PhD is the head of EMA's Regulatory Science and Academia Workstream, part of the Regulatory Science & Innovation Taskforce, coordinating research and engagement with researchers and developers from the academic sector and not-for-profit organisations. Previously, he worked on cancer and paediatric medicines as well as horizon scanning and foresight at the EMA. He was the Pediatric development leader of Bayer AG Regulatory affairs and an ICH E11A expert group member. At Charité – Universitätsmedizin Berlin, he obtained an experimental research PhD, board certified in paediatric and adolescent medicine, trained in paediatric oncology and haematology, and managed a national research network.

Topic 3: Ensuring Timely Access to Approved Therapies in Developing Countries: Addressing Patient Needs and Unmet Public Health Challenges

James Wabby, MHS

Global Head, Regulatory Affairs, Emerging Technologies and Combination Products
AbbVie, United States

James Wabby is the Global Head, Regulatory Affairs - (CoE) Emerging Technologies, Devices, and Combination Products at AbbVie in Irvine, California. He has 25 years of experience in increasing quality compliance and regulatory affairs responsibilities within the GxP regulated environment pertaining to Nutritional, Cosmetic, Branded Pharmaceutical, Generic, Biologic, Medical Device and Combination Product areas. James received his undergraduate and graduate degrees from Duquesne University and received his Health Care Compliance Certificate from Seton Hall University Law School. He is a member of various regulatory and quality work groups including RAPS, OCRA and the ASQ Orange Empire Section.

Topic 3: Ensuring Timely Access to Approved Therapies in Developing Countries: Addressing Patient Needs and Unmet Public Health Challenges - Moderators

Maria Paula Bautista Acelas, MSc

Senior Scientific Project Manager
DIA, United States

Maria Paula offers expert scientific content guidance and project management support for DIA's global consortium initiatives and specialty meetings. She is dedicated to ensuring the development and delivery of impactful, patient-

centric scientific content that generates evidence to facilitate the integration of innovation in medical product development. She brings experience in public health, patient engagement, and research management. She holds a Master of Science in Health Care Management from Marymount University and a Bachelor of Science in Microbiology and Bioanalysis from Universidad Industrial de Santander, Colombia.

Topic 3: Ensuring Timely Access to Approved Therapies in Developing Countries: Addressing Patient Needs and Unmet Public Health Challenges - Moderators

Luiz Correa

Associate Director, Scientific Programs - EMEAI
DIA, Switzerland

3:50 PM — 4:20 PM

Session 8: Report-Outs on Recommendations

Each table will summarize their discussion, 10 minutes each.

Session Chair(s)

Representative Invited

DIA, Switzerland

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4:20 PM — 4:30 PM

Closing Remarks

Closing Remarks

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DIA, United States

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Raju Kucherlapati

Paul C. Cabot Professor of Genetics and Professor of Medicine
Harvard Medical School, United States

4:30 PM — 4:30 PM

Summit Adjourns