Cell and Gene Therapy Products 2025

Schedule

Tuesday, 10 June, 2025

07:00-08:30 Foyer A-E

Continental Breakfast

CGTP Symposium

Breakfast will be available until 9:00 AM Eastern

07:00-08:30 Foyer A-E

Registration

CGTP Symposium

Registration is open until 17:00 Eastern.

08:30-08:45 Salon D

CASSS Welcome & CGTP 2025 Introduction

Alexandra Beumer Sassi, Raj Poudel
Live Streamed
CGTP Symposium

Session Chairs: Alexandra Beumer Sassi, *AstraZeneca* and Raj Poudel, *Mammoth Biosciences, Inc.*

08:45-09:55 Salon D

Keynote Presentation | Developing and Deploying N-of-1 Gene-Editing Therapies

Alexandra Beumer Sassi, Raj Poudel
Live Streamed
CGTP Symposium

Session Chairs: Alexandra Beumer Sassi, *AstraZeneca* and Raj Poudel, *Mammoth Biosciences, Inc.*

Keynote Speaker:
Dr. Kiran Musunuru, *University of Pennsylvania*

We recently diagnosed a newborn with a severe Mendelian disease of metabolism, CPS1 deficiency, and then designed, derisked, manufactured, and administered a CRISPR-based gene editor to the infant with full regulatory oversight by the US FDA CBER OTP within 6 months of diagnosis. The patient received three sequential doses of the non-viral drug product, with early clinical endpoints being suggestive of treatment-related benefit. This effort was made possible by an unprecedented partnership involving many institutions across academia and industry, including Children's Hospital of Philadelphia, Perelman School of Medicine at the University of Pennsylvania, UC Berkeley's Innovative Genomics Institute, two Danaher Corporation operating companies (Integrated DNA Technologies, Aldevron), and Acuitas Therapeutics. The innovation across all facets of the underlying problem space to get to this outcome provides a "cookbook" for CRISPR-treatments-on-demand, which we hope to extend beyond a handful of individual N-of-1 cases to a robust platform that will enable the development and deployment of personalized gene-editing treatments to a large number of patients affected by any of a broad spectrum of rare diseases.

09:55-10:45 Foyer A-E

Networking Break

IP -In Person
CGTP Symposium

09:55-10:45 Brookside A&B (Lower Level)

First Time Attendee Networking Event

IP -In Person
CGTP Symposium

First-time attendees are invited to mingle and meet others within the CASSS community

10:45-12:35 Salon D

Plenary Session 1 - Gene Editing

Michael Molony, Isabella Palazzolo, Marcos Timon
Live Streamed
CGTP Symposium

Session Chairs: Michael Molony, *Insmed Incorporated,* Isabella Palazzolo, *Intellia Therapeutics, Inc.,* and Marcos Timon, *Spanish Agency of Medicines and Medical Products*

This session will provide a comprehensive exploration of the current landscape of genome and epigenome editing technologies, including CRISPR/Cas9, Zinc Finger Proteins, Large Serine Integrases, and other emerging editing mechanisms. We will delve into the significant potential these technologies hold for treating genetic diseases, while also addressing the critical ethical and regulatory considerations surrounding their application in human clinical trials.

Key Discussion Points:

Recent Breakthroughs: Highlighting the latest advancements in gene editing technologies and their transformative potential for therapeutic applications.

Epigenetic Therapies: Examining how these technologies can be harnessed for epigenetic therapies, including an in-depth discussion of any concerns related to patient safety.

CMC Challenges: Addressing the chemistry, manufacturing, and controls (CMC) challenges encountered throughout the clinical development phases, such as strategies for targeting delivery of in vivo therapies to new organs/tissues, and manufacturing and control challenges for multiple gene editions.

Ex Vivo Gene Therapy: Showcasing success stories of genome editing applications in ex vivo gene therapy, with a focus on their journey from research to clinical trials.

Impurity Characterization: Providing insights into the progress made in characterizing impurities related to genome editing for complex products, and its implications for product safety and efficacy.

This presentation aims to stimulate a comprehensive discussion among researchers, clinicians, and regulators on the responsible advancement of genome editing technologies in the clinical setting. By addressing both the opportunities and challenges, this session will contribute to the ongoing dialogue on how to safely and effectively integrate these cutting-edge technologies into therapeutic practices.

Session Speakers:

Challenges in Getting Genome Editing Medicines into the Clinic
Phillip Ramsey, *Sangamo Therapuetics, Inc.*

Advancing Analytical Development for Prime Editing Therapeutics: A Platform-Based Approach
Yune Kunes, *Prime Medicine, Inc.*

Splicing the Dots: Potency Assurance Strategy for RNA-Editing Gene Therapies
John Kerwin, *Ascidian Therapeutics, Inc.*

Session Panelists:

Kiran Musunuru, *University of Pennsylvania*
Marcos Timón, *AEMPS*

12:35-14:00 Foyer A-E

Hosted Lunch

IP -In Person
CGTP Symposium

Lunch provided in conjunction with the technical seminar talk presented by Cygnus Technologies

13:00-13:45 Salon D

Analytics for Process-related Impurities in Viral Vector Manufacturing | Presented by Cygnus Technologies, LLC

Live Streamed
CGTP Symposium
Sponsors: Cygnus Technologies

Session Speaker:

Alla Zilberman, *Cygnus Technologies*

14:00-15:05 Salon D

Plenary Session 2 - Fireside Chat: Non-Profit/Alliance Updates

Diane Blumenthal, Barbara Bonamassa
Live Streamed
CGTP Symposium

Session Chairs: Diane Blumenthal, *Dianthus Biopharma Consulting, LLC* and Barbara Bonamassa, *Italian Medicines Agency*

Bringing ATMPs to patients presents drug development challenges that are unique, particularly in the rare and ultra-rare disease setting. Indeed, from a business perspective, the target patient population is quite small and the yearly incidence of new patients who present with the disease are even smaller. As a result, there is limited interest from industry in taking on products. From a technical perspective, the manufacturing scale is typically small in volume, resulting in limited availability of product to complete the extensive studies needed to support a robust CMC filing. Planning larger batch sizes or smaller batches is often cost prohibitive. The analytical demand for the development of robust QC assays and to analyze all the process development work required for a manufacturing control strategy can be daunting.

Academic institution and Foundations are a major contributor to the development of ATMPs as key sources of ideas and basic research and, as such, are picking up the slate. However, they can be ill equipped financially, and limited with resources to bring these medicines beyond Phase 1/2 and into late stage and commercial development. Furthermore, navigating regulatory requirements can be challenging for them. In addition, the business model and mission statement for these types of institutions are not in line with commercial product development even if they are selling them for modest to no profit.

This session will explore how non-profit entities are navigating these challenges in hope of bringing these important therapies to patients in desperate need.

Session Panelists:
Gonzalo Calvo, *Hospital Clinic of Barcelona*
Steven Gray, *University of Texas Southwestern Medical Center*
Sean Russell, *Fondazione Telethon ETS*
Ashley Winslow, *Odlyia Therapeutics*

15:05-15:35 Foyer A-E

Networking Break

IP -In Person
CGTP Symposium

15:35-16:45 Salon D

Flash Poster Talks - Session 1

Isabella Palazzolo
Live Streamed
CGTP Flash Poster Talks

Session Chair: Isabella Palazzolo, *Intellia Therapeutics, Inc.*

NEW to CGTP 2025!

The flash poster talks session will include brief poster presentations with the opportunity for questions. Posters will also be featured on the virtual poster gallery that is available for all attendees to view 24/7 during the Symposium.

Poster Presenters:

Analysis of CMC Information Requests for Cell and Gene Therapy Submissions
Jennifer Woods, *VCLS*

From Complexity to Simplicity – Phase-Appropriate Development of a QC- Friendly Gene Therapy Potency Assay
Rajeev Boregowda, *Sanofi*

Anticipating the Exceptional Release Bottleneck
Jessica Eisenstatt and Yoonji Preville, *AstraZeneca*

15:35-16:45 White Oak (Lower Level)

Roundtable Discussions - Session 1

IP -In Person
CGTP Roundtables

There will be 10 roundtable topics available to join on a first come, first-serve basis.

Table 1 - Potency Assurance Strategies for Cell and Gene Therapies

Table 2 - Donor Selection Criteria Across ICH Regions for Cells Used as Starting Material for ATMP Manufacture

Table 3 - Non-traditional Approaches to Comparability

Table 4 - Multiplex Approaches to Analytical Platforms for CGT Release

Table 5 - Phase Appropriate Engineering Run Approaches (i.e. IND enabling runs for filing and stability)

Table 6 - Solutions for Personalized Treatment Challenges

Table 7 - GMO Environmental Risk Assessment Applications

Table 8 - Use of New Rapid Sterility Testing Methods - Successes and Challenges

Table 9 - Target Product Profiles and Quality Target Product Profiles: Product Development with the End in Mind

Table 10 - Multigenerational Workforce

16:45-18:45 Veranda

CGTP 2025 Welcome Reception

IP -In Person
CGTP Symposium

Join us on the outdoor veranda to celebrate the start of CGTP 2025!

Wednesday, 11 June, 2025

07:15-08:15 Foyer A-E

Registration

CGTP Symposium

Registration is open until 17:00 Eastern.

07:15-08:15 Foyer A-E

Continental Breakfast

IP -In Person
CGTP Symposium

Breakfast will be available until 9:00 AM Eastern

08:15-09:00 Salon D

Breakfast Chat: FDA START Pilot Program – A Participant Perspective

Alexandra Beumer Sassi
Live Streamed
CGTP Symposium

Session Chair: Alexandra Beumer Sassi, *AstraZeneca*

The discussion on the START (Support for Clinical Trials Advancing Rare Disease Therapeutics) Pilot FDA Program will feature Dr. Becky Schweighardt, Chief Scientific Officer at Grace Science, LLC, and Dr. Ellery Mangas, Vice President Regulatory at Neurogene Inc. Both companies have been granted participation in the START program. This session will delve into the FDA's initiative aimed at addressing the unique hurdles of developing therapies for rare diseases, such as small patient populations, limited natural history data, and the need for innovative trial designs.

Grace Science is developing GS-100, a gene therapy to treat NGLY1 deficiency, a rare autosomal recessive disease that devastates the central nervous system. GS-100 is an AAV9 single-stranded viral vector that encodes the full length human NGLY1 protein.

Neurogene is developing NGN-401 an investigational AAV9 gene therapy aimed as a one-time treatment for Rett syndrome. It is the first clinical candidate to deliver the full-length human MECP2 gene under the control of Neurogene's EXACT technology.

Dr. Schweighardt and Dr. Mangas will share their firsthand experience with the START program, offering insights into how the tailored regulatory guidance and collaborative framework have supported their efforts to develop groundbreaking treatments. Panelists will explore the program's role in fostering patient-centric approaches, leveraging real-world evidence, and enabling the use of adaptive and innovative trial designs to accelerate therapeutic development without compromising safety or efficacy.

The discussion will also focus on how the START program can serve as a blueprint for advancing rare disease drug development on a global scale, emphasizing the importance of collaboration between regulators, industry, and patient advocacy groups. Attendees will gain a deeper understanding of the program's objectives, its impact on companies such as Grace Science and Neurogene, and the broader implications for addressing unmet medical needs in rare disease communities. This session promises to provide actionable insights and inspiration for stakeholders committed to driving innovation in the rare disease space.

Featured Panelists:

Ellery Mangas, *Neurogene Inc.*

Becky Schweighardt, *Grace Science LLC*

09:00-09:05

Transition Time

09:05-10:50 Salons A-C

Parallel Session 3 - Advancing Stem Cell Therapy Development: Overcoming Challenges and Expanding Horizons

Francois Gianelli, Deep Shah, Christopher Storbeck
Live Streamed
CGTP Symposium

Session Chairs: Francois Gianelli, *TreeFrog Therapeutics,* Deep Shah, *Sana Biotechnology,* and Christopher Storbeck, *Health Canada*

The field of Cell and Gene Therapy has witnessed tremendous growth in recent years with the approval of many CAR T cell and AAV-based Gene therapy products. Stem cell therapeutic products on the other hand, with their tremendous potential for treatment of many diseases due to their inherent properties of self renewal, differentiation potential and tissue regeneration and repair, have been lagging behind in their development. This may be in part be due to numerous challenges regarding their therapeutic use in the clinic, including the risk of immunological rejection, tumorigenesis, and achieving precise control of their characteristics.

The recent approval of a stem cell-based Gene editing product for Sickle Cell Disease and Beta Thalassemia may signal the beginnings of an upward trend for advancement of stem cell therapeutic products. This trend will be explored in this session with a look to promising products emerging in the stem cell therapeutic space.

Session Speakers:

Ensuring Product Comparability in Cell Therapy: A Risk-Based Approach to Managing Multifactorial Changes
Patty Sachamitr, *BlueRock Therapeutics LP*

Transparency-Driven Partnerships for Shared Success – iPSC Journey
Pedro Castanho Vaz, *Catalent, Inc*

Introduction to CASGEVY®, The First CRISPR-Cas9 Based Commercially Approved Therapy For SCD and TDT
Irina Kadiyala, *Vertex Pharmaceuticals Incorporated*

Additional Panelist:

Kathleen Francissen, *Genentech, a Member of the Roche Group*

09:05-10:50 Salon D

Parallel Session 4 - Development and Characterization of Viral Vectors

JR Dobbins, Bryan Silvey, Seshu Tyagarajan
Live Streamed
CGTP Symposium

Session Chairs: JR Dobbins, *Eli Lilly and Company*, Bryan Silvey, *GxP/CMC Principle in Cell Therapy,* and Seshu Tyagarajan*, Candel Therapeutics*

Viral vectors (VV) are emerging as a transformative gene therapies modality for treating a variety of genetic disorders. A multitude of VV containing products have progressed into clinical development, and a subset have become commercial products. As a result of this progression, substantial advances have been made in the chemistry, manufacturing, and control (CMC) of VV.

This session will provide insight into advances in CMC with a focus on novel VV modalities, leveraging platforms to accelerate and streamline development, phase appropriate innovative analytical testing and characterization approaches, and associated regulatory considerations. Attendees will have the opportunity to hear from industry and Health authority experts through presentations focused on case studies, practical application examples and participate in an interactive panel discussion. Attending this session will provide insight into the current industry and regulatory advances and trends with the use of VV in gene therapies.

Session Speakers:

Advanced Sequencing Approaches for Comprehensive AAV Vector Characterization
Will Arnold, *ElevateBio*

Cell Line Engineering for in Vitro AAV Potency Assays
Hsiang Chen Chou, *Biogen*

Integrating Process and Analytics: A Prerequisite to Streamlining the Production of Viral Vectors
Amitabha Deb, *CellGene Consulting LLC*

10:50-11:20 Foyer A-E

Networking Break

IP -In Person
CGTP Symposium

11:20-12:30 Salon D

Flash Poster Talks - Session 2

Raj Poudel
Live Streamed
CGTP Flash Poster Talks

Session Chair: Raj Poudel, *Mammoth Biosciences, Inc.*

NEW to CGTP 2025!

The flash poster talks session will include brief poster presentations with the opportunity for questions. Posters will also be featured on the virtual poster gallery that is available for all attendees to view 24/7 during the Symposium.

Poster Presenters:

Comprehensive AAV Characterisation Using Orthogonal Methodologies for Empty/full Capsid Analysis and Genome Integrity
Colin Guy, *Labcorp*

A Single, Ready-to-Use Kit for Fast, Comprehensive Analysis of Both Plasmid Topological Isoforms and Linear Sizing on a Multi-Capillary Electrophoresis Platform
Tingting Li and Henry Kang, *SCIEX*

Investigating the Aggregation, Genome Release, and Self-Interactions of Adeno-Associated Virus Formulations
Lily Motabar, University of Delaware

11:20-12:30 White Oak (Lower Level)

Roundtable Discussions - Session 2

IP -In Person
CGTP Roundtables

There will be 10 roundtable topics available to join on a first come, first-serve basis.

Table 1 - Potency Assurance Strategies

Table 2 - Donor Selection Criteria Across ICH Regions for Cells Used as Starting Material for ATMP Manufacture

Table 3 - Non-traditional Approaches to Comparability

Table 4 - Multiplex Approaches to Analytical Platforms for CGT Release

Table 5 - Phase Appropriate Engineering Run Approaches (i.e. IND enabling runs for filing and stability)

Table 6 - Setting Specifications for Autologous CAR T Cell Products Given the Inherent Patient to Patient Variability of the Apheresis Starting Material

Table 7 - Cost drivers (in CMC)

Table 8 - Establishing a Robust Supply Chain

Table 9 - Challenges and Best Practices for Partnering Between Academia and Industry

Table 10 - Global Health Equity in Cell and Gene Therapy

12:30-13:55 Foyer A-E

Lunch

IP -In Person
CGTP Symposium

Lunch provided in conjunction with Special Vendor Lunch Panel Talk

12:50-13:40 Salons A-C

Reducing COGM in Cell & Gene Therapy - The CDMO Perspective (Sponsored Lunch Session)

Live Streamed
CGTP Symposium
Sponsors: Kincell Bio, Viralgen, LumaCyte, Inc.

Session Chair: Raj Poudel, *Mammoth Biosciences, Inc.*

High manufacturing costs remain a major challenge in cell and gene therapy (CGT), limiting patient access and commercial success. Join leading CDMO's for a lunch session on strategies to reduce the cost of goods manufactured (COGM) while maintaining quality and compliance.

A panel of experts will discuss process optimization, scalable platforms, automation, and strategic sourcing to improve efficiency and financial sustainability. Additionally, learn how some CDMOs are dedicating manufacturing slots to rare disease therapies, supporting innovation and patient access.

This session is ideal for biotech leaders and decision-makers looking to drive affordability, profitability, and impact in CGT manufacturing.

Panelists:

Renée Hart, *LumaCyte, Inc.*
Andy Holt, *Viralgen*
Bruce Thompson, *Kincell Bio*

Sponsored By:
Kincell Bio, LumaCyte, Inc., and Viralgen

13:55-15:40 Salon D

Plenary Session 5 - Platform Development

Marcel Hoefnagel, Kevin Okimura, Cynthia Riggins
Live Streamed
CGTP Symposium

Session Chairs: Marcel Hoefnagel, *Medicines Evaluation Board,* Kevin Okimura, *Orca Bio,* and Cynthia Riggins, *ElevateBio, LLC*

Cell and gene therapies hold immense potential to transform clinical outcomes for patients with previously untreatable conditions. However, the individualized nature of process and analytical development introduces significant challenges, including increased time, resource demands, and complexity. Leveraging platform technologies offers a promising pathway to address these barriers, enabling broader clinical realization of these therapies. As demonstrated by last year's draft guidance from FDA on the platform technology designation program, health authorities are working to provide a framework for advancing programs via use of platforms. However, maturation of the understanding and experience of platform development in action both within the US and worldwide would provide benefit.

This plenary session will delve into the evolving landscape of platform development, highlighting strategies and real-world case studies that illustrate its application in process and analytical development. Join us to explore how these innovations can drive efficiency and scalability in the field of cell and gene therapy.

Session Speakers:

Considerations in Development, Characterization, and Commercialization of Platform Processes
Stephen Madaras, *Miltenyi Biotec*

Accelerating Access to Life-Saving Cell Therapies
John Tomtishen, *Cellares*

Beyond "One Disease at a Time" : Genetic Therapy Platforms for Rare Monogenic Disease
PJ Brooks, *National Center for Advancing Translational Sciences (NCATS), NIH*

Additional Panelists:

Andreea Barbu, *Swedish Medical Products Agency*

Kathleen Francissen, *Genentech, a Member of the Roche Group*

15:40-16:10 Foyer A-E

Networking Break

IP -In Person
CGTP Symposium

16:10-17:15 Salon D

Plenary Session 6 - Fireside Chat: Investing in CGT

Bruce Thompson
Live Streamed
CGTP Symposium

Session Chair: Bruce Thompson, *Kincell Bio*

Please join us for an exciting fireside chat about the state of the startup and funding environments for Cell and Gene Therapies. We will discuss various topics, including company creation, innovative approaches to financing and fundraising, effective CMC development in constrained funding environments, and what large pharma and biotechs are looking for in partnerships. Our panelists have a wealth of experience in moving products through development, starting new companies, venture capital financing, and identifying innovative technologies and unmet medical indications needing novel approaches to get treatments to patients.

Panelists:

Amanda Placone, *Lilly Ventures*

Stephen Sofen, *Sofen Consulting LLC*

Sonia Vallabh, *The Broad Institute of MIT and Harvard*

17:15-18:30 Foyer A-E

CGTP 2025 Exhibitor Reception

IP -In Person
CGTP Symposium

Join us in the Foyer to mix and mingle with our exhibitor partners!

Thursday, 12 June, 2025

07:30-08:30 Foyer A-E

Continental Breakfast

IP -In Person
CGTP Symposium

Breakfast will be available until 9:00 AM Eastern

08:00-08:30 Foyer A-E

Registration

CGTP Symposium

Registration is open until 12:00 Eastern.

08:30-10:20 Salon D

Parallel Session 7 - Optimizing the Analytical Testing Panel

Svetlana Bergelson, Seshu Tyagarajan
Live Streamed
CGTP Symposium

Session Chairs: Svetlana Bergelson, *Biogen* and Seshu Tyagarajan, *Candel Therapeutics*

Analytical characterization is essential for developing cell and gene therapy (CGT) products. It guides process development, determines storage conditions, assesses stability, and ultimately supports product release for patient use. The complexity of CGT products necessitates a wide range of quality assessment methods, often resulting in multiple assays for release and stability testing.

This session will focus on strategies to optimize these testing panels by leveraging different approaches to assess Critical Quality Attributes (CQAs). One strategy involves adopting new technologies to develop superior, validatable methods that correlate with existing orthogonal assays. Another approach uses a matrix of methods for enhanced product characterization, allowing a focus on the most suitable methods for advancing the product. For autologous cell therapies, maximizing sampling efficiency, minimizing sample volume, and reducing testing turnaround time are critical considerations for release testing panels.

As the CGT field expands, embracing these varied approaches will be crucial for efficiently delivering these transformative therapies to patients.

Session Speakers:

Optimizing Analytical Release Testing Through Reduced Volume and Turn-around Times
Michael Giffin, *ElevateBio*

A Matrix Approach to Characterize a Stem Cell-Derived Cell Product During Early Development
Maya Srinivas, *BlueRock Therapeutics LP*

Is the Vehicle Empty or Full?— Mass Photometry for AAV Capsid E/F Assessment in the GMP Space
Xue (Shelly) Li, *Regeneron Pharmaceuticals Inc.*

Additional Panelist:

Christopher Storbeck, *Health Canada*

08:30-10:20 Salons A-C

Parallel Session 8 - Non-Traditional Modes of Delivery for Ex-vivo and In-vivo Gene Therapies

Rob McCombie, Raj Poudel, Zenobia Taraporewala
Live Streamed
CGTP Symposium

Session Chairs: Rob McCombie, *Avidity Biosciences,* Raj Poudel, *Mammoth Biosciences, Inc.,* and Zenobia Taraporewala, *BioMarin Pharmaceutical Inc.*

The first wave of cell and gene therapies (CGT) developed, mostly, with viral vectors such as adeno-associated virus (AAV), adenovirus, lentivirus and retrovirus were transformative in treating many intractable diseases. Yet, the widespread adoption of these novel therapies has been limited by the challenges associated with viral delivery systems. Notably, challenges due to limited packaging capacity, safety concerns with administration of high doses and genotoxicity, efficacy concerns with inability to redose due to seroconversion, manufacturing complexities requiring product-specific optimizations, copurifying impurities impacting safety and potency, and high production costs limiting scalability and clinical application.

Non-viral delivery methods, initially developed for the delivery of plasmid DNA, are being adopted for in vivo or ex vivo delivery of RNA and genome editing (GE) components using bespoke lipid nanoparticles (LNPs) and physical delivery methods like electroporation and sonoporation. As a result, the scope of treatable conditions with CGT products has greatly expanded with non-viral delivery approaches that are being tailored to target hepatic and extrahepatic sites such as lung. By overcoming the limitations of viral delivery, non-viral methods are poised to drive the development of the "NextGen" CGTs.

This session will examine the challenges and discuss the potential solutions encountered during CMC development of in vivo and ex vivo non-viral mediated CGTs.

Session Speaker:

Lipid Nanoparticles-Points to Consider For Non-Viral Delivery of RNA-based Therapeutics
Mo Heidaran, *Cellx Consulting*

INS1203: RNA-End Joining Technology Enables A Dual AAV Approach for ABCA4 Gene Replacement in Stargardt Disease
Kathryn Hilde, *Insmed Incorporated*

CRISPR Genome Editing Components Used for Ex-Vivo Genome-Editing of Allogeneic Cell Therapy Products: Their Clearance and Their Effects
Alex Chialastri, *Century Therapeutics*

Additional Panelist:

Kathleen Francissen, *Genentech, a Member of the Roche Group*

10:20-10:50 Foyer A-E

Networking Break

IP -In Person
CGTP Symposium

10:50-12:35 Salon D

Plenary Session 9 - Global Regulatory Panel

Alexandra Beumer Sassi, Andrew Weiskopf
Live Streamed
CGTP Symposium

Session Chairs: Alexandra Beumer Sassi, *AstraZeneca* and Andrew Weiskopf, *Sana Therapeutics*

Over the past decade, we have witnessed the potential of cell and gene therapies beginning to be realized, as cutting-edge science and novel modalities have begun to reach patients worldwide. The efforts of global health authorities have been critical to making these transformational therapies possible. Thanks in no small part to their investment in building capability and capacity in these new technologies, health authorities reviewed an unprecedented number of cell and gene therapy products last year. Between 2023 and 2024, we have also seen more than a dozen cell and gene therapy product approvals for a wide array of diseases granted across the US, Canada, EU, UK, Japan, China, and several Middle Eastern countries.

As the field continues to advance, with increasingly diverse and complex products making their way into and through clinical development, how do global health regulators plan to keep up the momentum? In this session, we will hear from regulatory leaders from health authorities across the world about the work being done to support cell and gene therapy products and ensure timely and safe access of effective therapies to patients.

Panelists:

Barbara Bonamassa, *Italian Medicines Agency (AIFA)*

Marcel Hoefnagel, *Medicines Evaluation Board (MEB)*

Ingrid Markovic, *Independent Expert*

Atsushi Nishikawa, *Pharmaceuticals and Medical Devices Agency (PMDA)*

Christopher Storbeck, *Health Canada*

Marcos Timón, *AEMPS*

12:35-12:45 Salon D

Closing Remarks and Invitation to CGTP 2026

Raj Poudel
Live Streamed
CGTP Symposium

Session Chair: Raj Poudel, *Mammoth Biosciences, Inc.*