Cell and Gene Therapy Products 2026

Schedule

Tuesday, 9 June, 2026

07:15-08:30 Foyer A-D

Breakfast

Presentation type: IP - In Person
CGTP Symposium

Breakfast will be available until 09:00 Eastern

07:15-08:30 Foyer A-D

Registration

Presentation type: IP - In Person
CGTP Symposium

Registration is open until 17:00 Eastern.

08:30-08:45 Salon D

CASSS Welcome & CGTP 2026 Introduction

Raj Poudel
Presentation type: LS - Live Streamed
CGTP Symposium

08:45-09:55 Salon D

Keynote Presentation - From Innovation to Translation to Patients: The Future of Genetically Engineered CAR T Cell Therapies

Raj Poudel, Andrew Weiskopf
Presentation type: LS - Live Streamed
CGTP Symposium

Since the 1990s, gene-modified T cells have progressed from early clinical exploration to approved therapies that deliver durable responses in otherwise refractory disease. Chimeric antigen receptor (CAR) T cells, which recognize targets independent of HLA, have demonstrated sustained complete remissions in B cell leukemias and lymphomas, establishing a new therapeutic paradigm.

As the field expands beyond CD19 and hematologic malignancies, next-generation designs are addressing key limitations in potency, persistence, and safety. These include engineered control mechanisms, resistance to tumor-mediated immunosuppression, and incorporation of cytokine or signaling modules to enhance in vivo activity. Parallel advances in manufacturing are shortening production timelines and improving consistency.

Despite this progress, cell and gene therapies remain fundamentally different from conventional drugs. Defining critical quality attributes, establishing phase-appropriate specifications, and linking product characteristics to clinical performance continue to challenge traditional regulatory frameworks. Dose, potency, and pharmacokinetics must be reconsidered for a living, proliferating therapeutic.

Broadening patient access will depend not only on scientific innovation but also on scalable, robust manufacturing systems, automation, analytics, and global regulatory alignment.

Keynote Speaker:
Bruce Levine, *University of Pennsylvania*

09:55-10:45 Foyer A-D

Networking Break

Presentation type: IP - In Person
CGTP Symposium

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

First Time Attendee Networking Event

Presentation type: IP - In Person

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

10:45-12:35 Salon D

Plenary Session 1 - Navigating Comparability Challenges in Cell and Gene Therapy Development

JR Dobbins, Ilona Reischl, Cynthia Riggins, Seshu Tyagarajan
Presentation type: LS - Live Streamed
CGTP Symposium

The evolving landscape of cell and gene therapy (CGT) development presents unique comparability challenges that extend beyond those encountered with traditional biopharmaceutical products. This session will explore scenarios where conventional comparability approaches must be adapted because of accelerated clinical timelines, the complexity of CGT products and processes, and / or the scarcity of materials.

Speakers will share strategies for comparability assessments for process changes, facility transfers, and method transitions when analytical sample retention is limited or impossible. The session will also provide insight into innovative risk-based statistical and scientific approaches to overcome small sample sizes, including considerations for leveraging prior knowledge, platform experience, and historical data to inform and strengthen comparability assessments.

By attending this session, attendees will have the opportunity to hear dialogue between industry and regulators on pragmatic, science-driven comparability strategies that maintain product quality and patient safety while enabling the efficient advancement of these transformative therapies to patients.

Session Speakers:

Manufacturing Changes and Comparability for Cell and Gene Therapy Products
Andrew Timmons, *CBER, FDA*

Risk Based Analytical Comparability for am AAV Gene Therapy After an Early Manufacturing Site Change
Sam Sathiamoorthy, *AspireBio Consulting*

Progressive Analytical Comparability Strategy for an Autologous CAR-T Cell Therapy Product Across Multiple Process Changes and Manufacturing Sites
Houman Dehghani, *Cabaletta Bio, Inc.*

Additional Panelist:

Christopher Storbeck, *Health Canada*

12:35-13:50 Foyer A-D

Hosted Lunch

Presentation type: IP - In Person
CGTP Symposium

Lunch provided in conjunction with the technical seminar talk.

12:50-13:35 Salons A-C

Facilitating the Transition to Late-Phase Gene Therapy Production | Presented by Catalent, Inc.

Presentation type: LS - Live Streamed
CGTP Symposium

Session Speaker:

Robert Levendosky, *Catalent, Inc.*

13:50-15:35 Salon D

Parallel Session 2 - Leveraging Prior Knowledge in Early Development of ATMP's

Diane Blumenthal, Barbara Bonamassa, Francois Gianelli, Marcel Hoefnagel
Presentation type: LS - Live Streamed
CGTP Symposium

Although Prior Knowledge is not formally defined, it is an established term reported in several ICH and various EMA and FDA guidelines. The term Prior Knowledge refers to an established tool used for informing decisions during pharmaceutical development and lifecycle management. In such settings, Prior Knowledge can account for internal knowledge from a company's proprietary development and manufacturing experience (e.g., historical experience based on similar compounds, products and processes, including data modelling, application of 'platform technologies', knowledge from previous filings), external knowledge such as reference to scientific and technical publications, the application of established scientific principles. Knowledge also exists within regulatory agencies, and experience derived from such knowledge may inform regulators' assessments.

Leveraging prior knowledge offers an opportunity to support risk-based development strategies. Structured use of Prior Knowledge can inform key early development decisions for ATMPs. As an example, making use of Prior Knowledge in regulatory applications, to support manufacturing and control strategies, may be justifiable in certain circumstances and may compensate for a deferral of certain data on a risk-proportionate basis. While Prior Knowledge can be applied to all stages of control strategy development, its application may be of particular relevance to significantly accelerate the start of the (first) clinical trials particularly in case of medicinal products developed in the context of unmet medical need, rare disease and accelerated pathway settings, which represent frequent development scenarios for ATMPs.

In this session, we will hear from relevant Stakeholders about their experience in the application/assessment of Prior Knowledge in the ATMPs space.

Session Speakers:

Considerations for Leveraging Prior Knowledge for Cell and Gene Therapy Products
Stella Lee, *CBER, FDA*

Designing a Modular CMC Platform for Lentiviral Gene Therapies
Diego Castellaro, *Fondazione Telethon ETS*

Modeling Strategies to Accelerate and De-Risk Viral Vector Development
Sandra Aedo, *Johnson and Johnson Innovative Medicines*

Additional Panelist:

Lauren Kokai, *CBER, FDA*

13:50-15:35 Salons A-C

Parallel Session 3 - Characterization and Quality Expectations for mRNA in Various Applications

Taro Fujimori, May Guo, Ilya Shestopalov
Presentation type: LS - Live Streamed
CGTP Symposium

Messenger RNA (mRNA) has emerged as a transformative therapeutic modality, with established success in prophylactic vaccines and rapidly expanding potential in gene therapy applications, including mRNA-encoded gene editing enzymes. As the scope of mRNA-based medicines broadens, so too do the expectations for robust characterization strategies and fit-for-purpose quality attributes across diverse clinical contexts.

This session will explore evolving quality paradigms for mRNA drug substance and lipid nanoparticle (LNP) excipient components, highlighting similarities and key distinctions between vaccine and gene therapy applications. The session will address critical quality attributes (CQAs) of mRNA therapies, including identity, integrity, purity, sequence fidelity, capping, poly(A) tail characteristics, and residual impurities arising from in vitro transcription and downstream processing. Emphasis will be placed on how application-specific factors—such as administration path, duration of expression, and target tissue—inform analytical development, acceptance criteria, and control strategies. Attention will be given to mRNA used to encode gene editing enzymes, where transient expression, off-target risk, and long-term safety considerations may drive different quality expectations compared to vaccines.

In addition, the session will examine quality considerations for LNP excipients, including ionizable lipids, helper lipids, cholesterol, and targeting lipids. Topics will include lipid composition analysis, impurity profiles, degradation pathways, and the impact of excipient variability on potency, biodistribution, and safety. Strategies for defining and controlling excipient quality across development stages will be discussed, along with emerging expectations from regulatory authorities.

Through perspectives spanning analytical science, process development, and regulatory strategy, this session aims to provide a practical framework for aligning characterization approaches and quality expectations with intended clinical use. Attendees will gain insight into how to design risk-based, application-appropriate quality strategies that support innovation while ensuring patient safety and product consistency across the growing landscape of mRNA therapeutics.

Session Speakers:

From Insight to Specification: Defining which Assays to Place on Release vs Characterization in mRNA Therapeutics
Elisabeth Boucher, *Tessera Therapeutics*

Addressing Challenges in the Development and Quality Control of Individualized mRNA Products
Simon Klenk, *BioNTech SE*

Characterization of Targeted Lipid Nanoparticles for mRNA Delivery to Extrahepatic Tissues
Liping Zhou, *AstraZeneca*

Additional Panelist:

Anurag Sharma, *CBER, FDA*

15:35-16:05 Foyer A-D

Networking Break

Presentation type: IP - In Person
CGTP Symposium

16:05-17:15 White Oak (Lower Level)

Roundtable Discussions - Session 1

Presentation type: IP - In Person
CGTP Roundtables

There will be 8 roundtable topics available to join on a first come, first-serve basis.

Table 1 - Unorthodox Approaches to Stability Programs for Small Batch-size Cell and Gene Therapies

Table 2 - How Has AI Changed the Way We Work? What Role Will AI and ML Play in Personalized Cell/Gene Therapies?

Table 3 - Cost Drivers (in CMC)

Table 4 - What are the Most Critical Bottlenecks in CGT Manufacturing, and How Are We Solving Them?

Table 5 - Nontraditional Process Validation Approaches

Table 6 - Development of Phase Appropriate Specifications

Table 7 - Non-traditional Approaches to Comparability

Table 8 - Target Product Profiles and Quality Target Product Profiles: Product Development with the End in Mind

16:05-17:00 Salons A-C

Poster Talks & Panel | Session 1

Jennifer Woods
Presentation type: LS - Live Streamed
CGTP Poster Talk & Panel

The poster talks & panel session will include brief poster presentations with the opportunity for questions. Posters will be available in-person in Foyer A as well as featured on the virtual poster gallery that is available for all attendees to view 24/7 during the Symposium.

Poster Session Presenters:

A Quantitative Nuclear Translocation Assay for the Functional Characterization of Recombinant AAV
Prerana Pathak, *Biogen*

Development of a GMP-compliant Mass Photometry Platform Method for AAV Capsid Population Analysis
Long Zhang, *Sanofi*

Revamp, Replace, or Revolutionize? Strategic Decisions in Potency Assay Evolution through Research and CMC
Stephanie Whipple, *Sanofi*

Adoption of Synthetic Cell Mimics Enable Accelerated Method Transfer and PPQ
Justin De La Cruz, *Cellares*

Advancing Global Submissions Through a One Dossier Operating Model: Concept, Implementation and Early Outcomes
Jen Pappas, *AstraZeneca*

17:15-18:00 Veranda

Spotlight Social

Presentation type: IP - In Person
CGTP Symposium

Join us on the outdoor veranda to celebrate the start of CGTP 2026. Enjoy small bites and drinks before the Spotlight Talk.

18:00-18:50 Salon D

Spotlight Talk - From Innovation to Platform: Advancing the Future of mRNA Medicines

Alexandra Beumer Sassi, Taro Fujimori
Presentation type: LS - Live Streamed
CGTP Symposium

Spotlight Speaker:

Bijoyita Roy, *Moderna, Inc.*

Wednesday, 10 June, 2026

07:15-08:15 Foyer A-D

Breakfast

Presentation type: IP - In Person
CGTP Symposium

Breakfast will be available until 09:00 Eastern

07:15-08:15 Foyer A-D

Registration

CGTP Symposium

Registration is open until 17:00 Eastern.

08:15-09:00 Salon D

Breakfast Chat - FDA Pilot Program: CDRP

Alexandra Beumer Sassi, Zenobia Taraporewala
Presentation type: LS - Live Streamed
CGTP Symposium

The FDA Chemistry, Manufacturing, and Controls Development and Readiness Pilot (CDRP) Program supports earlier dialogue on CMC readiness for cell and gene therapy (CGT) products. Aligned with this year's CGTP Symposium theme, "Innovation to Impact: Next Generation CGTs," this breakfast chat will explore how the program may help sponsors navigate complex manufacturing and regulatory expectations.

The session brings together three perspectives across the regulatory ecosystem: two industry leaders, Dr. Isabella Pallazollo from Intellia and Dr. Natalie Ward from Bristol Myers Squibb, and a regulator, Dr. Ramjay Vatsan from the U.S. Food and Drug Administration. Discussion during this breakfast chat will address the goals of the CDRP Program, its role in surfacing critical CMC questions earlier in development, and other similar Agency efforts, specifically, FDA's Precheck Program that allows for early communication with the agency to ensure facility readiness and reduce downstream risks when planning onshoring of manufacturing for critical medicine.

For CGT sponsors, manufacturing strategy is closely tied to product development strategy. This discussion will examine how the CDRP Program can support engagement on product and manufacturing readiness, and where it may add value for sponsors managing analytical, comparability, supply chain, and timeline challenges. Industry panelists will share practical perspectives on assessing participation, preparing internally, and aligning cross-functional teams for engagement. Their insights will complement public FDA learnings with real-world considerations on the program's value and limitations. By combining FDA and industry perspectives, this session will give attendees a practical understanding of how the CDRP Program may influence CGT CMC development strategies and regulatory engagement.

Session Panelists:

Isabella Palazzolo, *Intellia Therapeutics, Inc.*

Natalie Ward, *Bristol-Myers Squibb Company*

Ramjay Vatsan, *CBER, FDA*

09:00-10:50 Salon D

Plenary Session 4 - Gene and Epigenome Editing: Mechanisms Driving Early-Phase Therapies

Isabella Palazzolo, Raj Poudel, Max Tejada
Presentation type: LS - Live Streamed
CGTP Symposium

This session provides a comprehensive overview of genome modulating platforms, spanning established ex vivo approaches to emerging in vivo strategies. We will explore the evolution from early CAR-T successes in hematologic malignancies to cutting-edge applications in autoimmune disorders, inherited diseases, and gene expression modulation through epigenetic modifications.

Through case studies in sickle cell disease, β-thalassemia, hemophilia, and systemic lupus erythematosus, participants will gain insights into critical CMC considerations—including manufacturing scalability, product characterization, and delivery system optimization. Nonclinical lessons learned will address biodistribution studies, off-target assessment strategies, and immunogenicity prediction.

The session will highlight both triumphs and setbacks, examining how challenges in delivery efficiency, immune responses, and manufacturing complexity have shaped development strategies. Attendees will leave with actionable knowledge to advance their own genome editing programs while navigating the unique regulatory and technical landscapes of this transformative therapeutic class.

Session Speakers:

CMC Considerations for CRISPR and Next-Gen Technologies, Including ElevateBio's LETI-101 as a Case Study
April Sena, *ElevateBio*

Developing First-in-Class In Vivo Epigenome Editing for Chronic Hepatitis B: Accelerated CMC Development Strategies
Tyler Goodwin, *Tune Therapeutics*

Driving Concurrent US and EU Approvals for Gene Therapies Using a Reg-CMC Roadmap
Rajiv Gangurde, *Parexel International*

Additional Panelist:
Luis Santos, *Prime Medicine, Inc.*

10:50-11:20 Foyer A-D

Networking Break

Presentation type: IP - In Person
CGTP Symposium

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

Roundtable Discussions - Session 2

Presentation type: IP - In Person
CGTP Roundtables

There will be 8 roundtable topics available to join on a first come, first-serve basis.

Table 1 - Unorthodox Approaches to Stability Programs for Small Batch-size Cell and Gene Therapies

Table 2 - How Has AI Changed the Way We Work? What Role Will AI and ML Play in Personalized Cell/Gene Therapies?

Table 3 - Cost Drivers (in CMC)

Table 4 - What are the Most Critical Bottlenecks in CGT Manufacturing, and How Are We Solving Them?

Table 5 - Nontraditional Process Validation Approaches

Table 6 - Development of Phase Appropriate Specifications

Table 7 - Early Phase vs. Late Phase Comparability Analysis After CMC Changes

Table 8 - How are we maximizing the leveraging of prior knowledge and platform processes?

11:20-12:10 Salons A-C

Poster Talks & Panel | Session 2

Bryan Silvey
Presentation type: LS - Live Streamed
CGTP Poster Talk & Panel

The poster talks & panel session will include brief poster presentations with the opportunity for questions. Posters will be available in-person in Foyer A as well as featured on the virtual poster gallery that is available for all attendees to view 24/7 during the Symposium.

Poster Session Presenters:

Manufacturing of mRNA by Fed-Batch In-Vitro Transcription: Optimization of Production Costs, Space-Time Yield & Product Quality
May Guo, *Novaribo LLC*

Plasmid-Free mRNA Synthesis on a Reusable Solid-Phase IVT Platform
Su Hui Catherine Teo, *Avantor, Inc.*

Development of an NGS-Based Method for Characterization of gRNA Sequence Impurities
Athea Vichas, *Bristol-Myers Squibb Company*

Navigating the "DS vs. Critical Component" Continuum for Gene Editing Tools: Lessons from Autologous Cell Therapy Program Lifecycle Management
Leakhena Som, *AstraZeneca*

12:30-13:45 Foyer A-D

Hosted Lunch

Presentation type: IP - In Person
CGTP Symposium

Lunch provided in conjunction with the Lunch with the Experts panel session.

12:45-13:35 Salons A-C

Lunch with the Experts: CDMO Insights on Reducing COGM for CGT Products

Raj Poudel, Bryan Silvey
Presentation type: LS - Live Streamed
CGTP Symposium
Panel Support:: AspireBio Consulting, Franklin Biolabs, Cellares, ElevateBio

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.

Session Panelists:

Kenneth Yancey, *Franklin Biolabs*

Ossama Eissa, *Cellares*

Katie Shannon, *ElevateBio*

Robert van den Heuvel, *AspireBio Consulting*

This panel is made possible with support from AspireBio Consulting, Cellares, ElevateBio, and Franklin Biolabs

13:45-15:30 Salon D

Parallel Session 5 - Innovative Process Performance Qualification Strategies Supporting Accelerated Programs

Kevin Okimura, Deep Shah, Seshu Tyagarajan
Presentation type: LS - Live Streamed
CGTP Symposium

Accelerated programs within advanced therapeutic modalities increasingly require Process Performance Qualification (PPQ) decisions to be made with limited batch history, constrained material availability, and rapidly evolving process knowledge. This session will bring together sponsors and regulatory perspectives to explore innovative, risk-based PPQ strategies that enable timely licensure while maintaining robust assurance of quality.

Discussion will include aligning PPQ with process characterization and control strategy maturity, integrating continued process verification (CPV) and lifecycle validation concepts, leveraging prior knowledge and platform approaches, and effectively communicating benefit-risk and justification to health authorities. Participants will leave with practical frameworks and examples to strengthen PPQ readiness under accelerated timelines.

Session Speakers:

PPQ-As-You-Go: Accumulating CMC Validation Evidence in Step with Clinical Data in Rare Genetic Disease Programs
Chris Bell, *Prime Medicine, Inc.*

Holistic Thinking for PPQ Strategy: Lessons Learned and Future Opportunities
Mary Rodley, *Medius Consulting, LLC*

PPQ Under Pressure: Designing Practical Validation Strategies for CGT Programs
Javier Cardenas, *Eliquent Life Sciences*

Additional Panelists:

Karin Knudson, *CBER, FDA*

Marissa Roush, *Mammoth Biosciences, Inc.*

13:45-15:30 Salons A-C

Parallel Session 6 - Control Strategies for Gene Editing Across Platforms

Michael Molony, Christopher Storbeck, Max Tejada, Marcos Timon, Sarah Wassmer
Presentation type: LS - Live Streamed
CGTP Symposium

As gene editing transitions from revolutionary proof-of-concept to clinical practice, the industry faces critical challenges in harmonizing control strategies across diverse modalities. This session will examine the evolving landscape of manufacturing and safety controls for next-generation platforms, including base editing, prime editing, and a multitude of gene editing systems.

Participants will discuss the integration of quality monitoring and manufacturing controls to maximize product quality and mitigate genotoxicity and off-target risks.

Points to Consider:

- Technical Precision: Benchmarking control strategies for reducing typical product quality concerns such as purity, potency, specificity, genotoxicity.
- Regulatory Harmonization: Navigating global compliance frameworks and multi-jurisdictional clinical trials.
- Analytical Innovations: Challenges with implementing newer technologies such as high-throughput proteomics and NGS-enabled analytics in a GMP environment to assess product quality.
- Manufacturing Concerns: Strategies for balancing rigorous CMC (Chemistry, Manufacturing, and Controls) with the speed required for accelerated programs.

This session brings together developers, regulatory experts, and analytical scientists to define the roadmap for safe and efficacious gene editing products.

Session Speakers:

From Molecule to Function: Holistic Control Strategies for CAR T Starting Materials
Julien Camperi, *Genentech, a Member of the Roche Group*

Development of an Off-Target Strategy for a Novel Small Nuclease
Maggie Bobbin, *Mammoth Biosciences, Inc.*

QC Assays With Unique Precision and Clinical Relevancy: Logical Strategies From Advanced Therapy Developers
George Dorfman, *Mission Bio*

Additional Panelist:

Jacob Bitterman, *CBER, FDA*

15:30-16:10 Foyer A-D

Networking Break

Presentation type: IP - In Person
CGTP Symposium

15:30-16:10 Foyer A

Poster Showcase with Presenters

Presentation type: IP - In Person
CGTP Symposium

Attendees are invited to visit Foyer A to view physical poster presentations and engage directly with presenters. This session provides an opportunity to ask questions, discuss research findings, and network with colleagues.

16:10-17:10 Salon D

Plenary Session 7 - Fireside Chat: ICH Harmonization efforts for ATMPs

Alexandra Beumer Sassi, Kathleen Francissen
Presentation type: LS - Live Streamed
CGTP Symposium

ICH has begun to address the unique regulatory considerations for advanced therapy medicinal products (ATMPs). The first ICH guideline to specifically address ATMPs was ICH S12 Nonclinical Biodistribution Considerations for Gene Therapy Products, which was adopted in March 2023. Since then, the expert working groups (EWG) for the ICH Q1 R1 (revision to guidelines for stability of drug substance and drug product) and for the ICH Q6 R1 (revision to guidelines for specifications) are preparing ATMP Annexes.

In addition, the Cell and Gene Therapy Discussion Group (CGTDG) formed in October 2023 as a technical discussion forum for issues related to ICH harmonization efforts in the ATMP field. The CGTDG fulfilled its remit and issued 'Recommendations with regard to future ATMP related guidance', which was endorsed by the ICH Management Committee in November 2025 and posted on the ICH website (ich.org). The CGTDG took a broad interdisciplinary view of the CGT field and gathered regional ATMP-specific guidelines that totaled over 300 documents, which showed the level of maturity and expanse of the field as well as the need for harmonized global regulatory guidance. The CGTDG also prepared high level principles that describe special considerations for ATMPs; while this was not scientific guidance, it provided the basis for what could be guidance in the future.

The recommendations are the following: (1) ATMP Annex to ICH Q5E Comparability; (2) new ICH E guideline on long term follow up (LTFU) for ATMPs; (3) ATMP Annex to ICH Q11 to address control strategy and material quality and related expectations; (4) new ICH S guideline on nonclinical evaluation of ATMPs; (5) annex to ICH E8 on considerations for early/exploratory clinical trials evaluating ATMPs; (6) Annex to ICH Q5A(R2) on viral safety for cell-based therapeutics; (7) Illustrative examples for ICH Q2 and Q14 to include case studies of unconventional analytical methods. Among the CGTDG recommendations, the ICH Q5E annex was endorsed by the ICH MC and this much-needed harmonization effort is expected to start in early 2026.

Session Panelist:

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

17:10-18:25 Foyer A-D

CGTP 2026 Exhibitor Reception

Presentation type: IP - In Person
CGTP Symposium

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

Thursday, 11 June, 2026

07:30-08:00 Foyer A-D

Hosted Breakfast

Presentation type: IP - In Person
CGTP Symposium

Breakfast available in conjunction with the technical seminar

Breakfast will be available until 09:00 Eastern

07:30-08:00 Foyer A-D

Registration

CGTP Symposium

Registration is open until 12:00 Eastern.

08:00-08:30 Salon D

Host Cell Protein Analytics and Viral Vector Manufacturing for Cell and Gene Therapies | Presented by Cygnus Technologies, LLC

Presentation type: LS - Live Streamed
CGTP Symposium

Session Speaker:

Alla Zilberman, *Cygnus Technologies*

08:30-08:40 Salon D

Transition Time

CGTP Symposium

08:40-10:30 Salon D

Plenary Session 8 - Next-Generation RNA Modalities: CMC and Regulatory Strategies for Clinical Translation

John Kerwin, Andreas Kuhn, Zenobia Taraporewala
Presentation type: LS - Live Streamed
CGTP Symposium

The therapeutic horizon for genetic medicines is rapidly expanding through next-generation RNA modalities, such as self-amplifying RNA (saRNA), trans-amplifying RNA (transRNA), and circular RNA (circRNA). These RNA modalities offer potential for the development of a broad range of transformative products, from gene, base, or prime editing to immunology and immuno-oncology therapeutics. This session focuses on the Chemistry, Manufacturing, and Controls (CMC) and regulatory strategies necessary to transition these RNA modalities from bench to bedside.

To move beyond the laboratory, these complex RNA-containing modalities require specialized manufacturing frameworks that prioritize structural integrity and purity across all unit operations. This involves navigating product development, manufacturing and controls from R&D to a phase appropriate GMP-compliant production processes while addressing unique product quality, stability, and delivery needs—such as encapsulation into lipid nanoparticles (LNPs) or other innovative systems—to ensure product potency and preserve the intended mechanism of action.

Key discussion topics include:

1. Process Performance and Yield Optimization: Addressing the unique manufacturing yields and purification requirements for structurally distinct RNA classes; examples would include optimization of cell-free DNA synthesis, downstream steps for removal of common impurities such as dsRNA, and assembly/formulation of the product/delivery vehicle (from components/intermediates).

2. Product Quality and Analytics: Method and development approaches to ensure the potency, purity, and identity of novel RNA species.

3. Integrated Formulation and Fill-Finish: Overcoming the challenges of encapsulating diverse RNA species into innovative gene delivery systems, such as lipid nanoparticles (LNPs) or polymeric solutions, while monitoring encapsulation efficiency, RNA stability and particle properties through in-process analytics.

4. CMC Risk Management and Regulatory Readiness: Utilizing formal risk assessments to define Quality Target Product Profiles (QTPPs) and Critical Quality Attributes (CQAs) necessary to establish a robust, IND-enabling CMC package.

Session Speakers:

Using a Risk-Based Approach to RNA Lipid Nanoparticle Development
Jeff Atkinson, *Orna Therapeutics, Inc.*

Adventures in Making Extreme mRNA Molecules
Aaron Larsen, *Crystal NAX, Inc.*

Genetic Medicines and Individualized Manufacturing for Everyone
John Schiel, *Advanced Research Projects Agency for Health (ARPA-H)*

Additional Panelist:

Brian Stultz, *CBER, FDA*

10:30-10:55 Foyer A-D

Networking Break

Presentation type: IP - In Person
CGTP Symposium

10:55-12:35 Salon D

Plenary Session 9 - Global Regulatory Panel

Kathleen Francissen, Andrew Weiskopf
Presentation type: LS - Live Streamed
CGTP Symposium

Join regulatory leaders from around the globe for an interactive dialogue on the evolution of regulatory oversight for cell and gene therapy products. As the advanced therapy medicinal product (ATMP) field has matured, more than 300 regional guidelines have emerged worldwide, alongside a growing number of approved products that continue to expand regulatory experience and scientific understanding. With this progress, regulatory agencies and sponsors alike have continued to gain valuable insights into the unique challenges posed by these modalities.

This session will explore the regulators' perspectives on key CMC learnings from early development to commercialization, including manufacturing scalability, lifecycle management, comparability following manufacturing process changes, and quality issues encountered pre and post approval. Regulators will discuss how experience has informed the use of fit-for-purpose, risk based, and product specific CMC approaches, and where regulatory expectations have evolved as development paradigms and manufacturing technologies have advanced. We will also consider regulatory best practices for autologous and other patient specific ATMPs, as well as expectations for managing donor derived starting materials, inherent biological variability, and characterization of complex cell and tissue based products across the manufacturing lifecycle.

Additionally, each of our guest panelists will share their respective agency's key priorities in the ATMP space. Our discussion will also highlight opportunities for greater international regulatory collaboration and voluntary convergence, recognizing these efforts as important enablers of future harmonization, including emerging ICH initiatives addressing ATMP specific regulatory considerations.

Featured Panelists:

Barbara Bonamassa, *AIFA - Italian Medicines Agency*

Yasuhiro Kiskioka, *PMDA - Pharmaceuticals and Medical Devices Agency*

Laura Ricles, *CBER, FDA*

Sarah Wassmer, *Health Canada*

12:35-12:45 Salon D

Closing Remarks and Invitation to CGTP 2027

Andrew Weiskopf
Presentation type: LS - Live Streamed
CGTP Symposium