# CELL & GENE THERAPY SUMMIT 2025

BREAKING BARRIERS IN GENE THERAPY BY INNOVATING SMARTER DELIVERY, SAFER EDITING, AND SCALABLE MANUFACTURING TO REVOLUTIONIZE PRECISION MEDICINE AND TRANSFORM PATIENT OUTCOMES WORLDWIDE.

### DATE: 12TH -13TH NOVEMBER 2025 (EST) | VIRTUAL CONGRESS

#### **KEY TOPICS ON THIS YEAR'S AGENDA INCLUDE:**

- Innovations in engineering smarter vectors are overcoming biological barriers to improve precision genetic therapy delivery, enhancing specificity, efficiency, and clinical outcomes.
- Approaches to reduce host immune responses in viral gene therapy focus on minimizing immune recognition while enhancing vector stability and delivery effectiveness.
- Explore how cutting-edge techniques improve CRISPR precision and safety by addressing off-target effects through novel Cas9 variants and advanced computational tools.
- Advancements in CAR-T and TCR-T therapies for solid tumors focus on personalized treatments, combination strategies, and overcoming challenges posed by the tumor microenvironment.
- Manufacturing Approaches for Cell-Based Therapies Focused on ensuring quality, costeffectiveness, and meeting regulatory standards in advanced bioproduction.
- Innovative clinical trial designs for gene and cell therapy utilize adaptive models that improve efficiency, maintain data integrity, and speed up development timelines.
- Addressing variability in manufacturing and clinical outcomes through quality control, risk management, and standardization to achieve reliable therapeutic results.
- Enhancing scalability, contamination control, and regulatory compliance by utilizing advanced technologies in automated and closed-system manufacturing.
- Facility designs that enable quick scaling and smooth shifts from clinical to commercial production, using modular manufacturing platforms for agile and multi-product flexibility.
- Standardizing potency assays to achieve consistent clinical outcomes through analytical strategies that provide reliable potency measurement for autologous, allogeneic, and gene-modified therapies.
- Engineering allogeneic cell therapies to create off-the-shelf solutions through scalable bioprocesses and universal donor strategies that promote global access and affordability.
- Innovations in sustainable manufacturing for cell and gene therapy focus on eco-friendly methods that enhance efficiency, safety, and reduce environmental impact in biotech production.
- Aligning research and development early with payer requirements to enhance value and prevent delays in product launch.
- Approaches that combine value-based pricing with real-world evidence integration help align therapy pricing with long-term outcomes and increase payer confidence.
- Using artificial intelligence to generate evidence and engage stakeholders, helping close reimbursement gaps and promote cooperative market access.

#### CELL & GENE THERAPY SUMMIT 2025 REDEFINING POSSIBILITIES THROUGH SMARTER DELIVERY, SAFER EDITING, AND SCALABLE INNOVATION DAY 1: 12TH NOVEMBER 2025 (EST)| VIRTUAL CONGRESS

08:50 Chairperson Opening Remarks

BREAKING BIOLOGICAL BARRIERS 09:00 Engineering Smarter Vectors To Revolutionize Precision Delivery Of Genetic Therapies With Unmatched Specificity And Clinical Impact.

- Explore the next frontier in genetic medicine: precision delivery of therapeutic payloads to specific cells and tissues.
- Discuss cutting-edge strategies in vector engineering to overcome biological delivery barriers.
- Highlight approaches that enhance specificity, efficiency, and safety in genetic therapy delivery.
- Provide insights into the rational design of nextgeneration viral and non-viral vectors.
- Showcase tailored delivery systems for a broad range of therapeutic applications.
- Emphasize translational breakthroughs bridging the gap from bench to bedside.
- Present how smarter vector systems are enabling more effective and clinically impactful gene therapies.

09:25 Questions & Discussion

#### **BEYOND IMMUNITY**

#### 09:30 REDUCING HOST RESPONSES WHILE ENHANCING DELIVERY IN VIRAL GENE THERAPY PLATFORMS

Viral gene therapy has emerged as a promising avenue for the treatment of a variety of genetic disorders. However, the success of these therapies often hinges on two critical challenges: the immune responses of the host and the efficiency of viral delivery systems. This talk will delve into innovative strategies designed to reduce host immune responses to viral vectors while simultaneously enhancing their ability to effectively deliver genetic material to targeted cells. Key areas of focus include:

- Exploration of genetic modifications and novel viral platform designs aimed at minimizing immune recognition and improving vector stability and specificity.
- Discussion on advancements in vector delivery techniques, such as tissue-specific targeting and controlled release, to increase the therapeutic efficacy of gene therapies.
- Examination of current clinical trials and preclinical research that showcase these advancements, along with a forward-looking perspective on overcoming remaining obstacles.

#### ADVANCING CRISPR PRECISION PANEL DISCUSSION 10:00 CRISPR Under the Microscope: Innovations

for Precision and Safety in Genome Editing

#### 10:00 Navigating the Precision and Pitfalls of CRISPR-Cas9: Addressing Off-Target Effects in Genome Editing.

CRISPR-Cas9 revolutionizes gene editing with unprecedented precision, but off-target effects remain a critical challenge. This talk explores strategies to enhance specificity, ensuring safer, more effective applications in medicine and biotechnology.

### 10:15 Off-Target Effects in CRISPR: A Critical Barrier to Reliable Therapeutic Applications.

This talk explores the significance of off-target effects in CRISPR technology, discussing their impact on both therapeutic interventions advancements. We'll examine current challenges, potential risks, and strategies for improving precision.

## 10:30 Unveiling Off-Target Modifications: Advancing CRISPR Safety in Medicine.

Understanding off-target modifications is crucial for enhancing CRISPR's safety. Investigating unintended modifications requires exploring the mechanisms driving off-target effects to improve precision and reduce risks.

#### 10:45 Enhancing CRISPR Precision and Safety: Strategies for Detecting and Mitigating Off-Target Effects with Novel Cas9 Variants and Computational Algorithms

This session offers an in-depth look at the latest strategies aimed at improving CRISPR technology by detecting and mitigating off-target effects. We will discuss novel Cas9 variants and computational algorithms crucial for reducing unintended genetic modifications, ensuring safer gene editing.

#### 11:00 Enhancing Precision: The Future of CRISPR Technology in Genetic Engineering

Advancements in CRISPR technology are significantly improving its accuracy, reducing off-target effects, and advancing safer genetic engineering practices. Future innovations aim to refine specificity, paving the way for broader, more reliable applications in medicine.

11:15 Interactive Discussion

11:45 Networking Refreshment Break

#### PANEL DISCUSSION ON CAR-T and TCR-T THERAPIES 12:00 Enhancing CAR-T and TCR-T Therapies in Solid Tumors: Clinical Insights on Personalization, Combination Approaches, and Translational Hurdles

- Advancing Clinical Strategies for CAR-T and TCR-T Cell Therapies in Solid Tumors: Overcoming Challenges and Enhancing Efficacy
- CAR-T and TCR-T Cell Therapies in Solid Tumors: Navigating Safety, Persistence, and Tumor Microenvironment Challenges in Clinical Trials
- Optimizing CAR-T and TCR-T Cell Therapies: Lessons from Clinical Trials and Future Directions for Solid Tumor Treatment
- Personalizing CAR-T and TCR-T Therapies for Solid Tumors: Biomarkers, Combination Strategies, and Clinical Translation Challenges

12:25 Questions & Discussion

#### OVERCOMING VARIABILITY

#### 12:30 Scalable Manufacturing Strategies For Cell-Based Therapies To Achieve Quality, Affordability, And Regulatory Excellence In Advanced Bioproduction

Scaling the production of cell-based therapies poses significant technological and economic challenges. Ensuring consistent quality across batches is difficult due to inherent product variability. This talk explores innovative strategies to enhance manufacturing scalability while maintaining rigorous quality control, addressing key hurdles in standardization, cost-efficiency, and regulatory compliance in advanced therapeutic production.

12:55 Questions & Discussion

13:00 Networking Lunch Break

#### PANEL DISCUSSION

Innovative Gene Therapy Clinical Trial Designs Improving Efficiency Data Integrity and Accelerating Development Timelines

### 14:00 Bridging Preclinical Insights with Clinical Execution for Accelerated Success in Cell and Gene Therapy Trials

This session will explore how early-stage insights can be strategically aligned with clinical trial design, regulatory pathways, and patient-centric considerations to accelerate therapeutic success. Attendees will gain perspectives on optimizing preclinical models, anticipating translational hurdles, and implementing adaptive clinical strategies that reduce risk and enhance trial efficiency. Real-world case studies will illustrate how integrated cross-functional planning can shorten development timelines and increase the likelihood of clinical impact.

#### 14:15 Early Detection and Mitigation of Operational and Clinical Threats to Ensure Success in Cell and Gene Therapy Trials through Proactive Risk-Based Quality Management

In this presentation, we'll investigate how implementing proactive, risk-based quality management strategies can identify and address operational and clinical threats early in cell and gene therapy trials. By leveraging data-driven risk assessment and mitigation techniques, sponsors and clinical teams can enhance trial integrity, patient safety, and regulatory compliance. Attendees will gain insights into practical approaches for minimizing disruptions, optimizing trial outcomes, and accelerating the development of innovative therapies in this rapidly evolving field.

#### 14:30 Collaborative Trial Networks and CRO Partnerships Driving Innovation to Enhance Efficiency and Accelerate Clinical Trial Success

This talk explores how strategic collaboration between trial networks and Contract Research Organizations (CROs) is transforming clinical research. By leveraging innovative partnerships, stakeholders can streamline processes, reduce timelines, and improve data quality. Attendees will gain insights into best practices for fostering collaboration, harnessing technology, and overcoming common challenges to accelerate clinical trial success and bring therapies to patients faster.

#### 14:45 Innovative Strategies in Gene Therapy Clinical Trial Design to Enhance Operational Efficiency and Data Integrity

This session will cover cutting-edge approaches to designing gene therapy clinical trials that optimize operational workflows while ensuring robust data integrity. Attendees will gain insights into novel trial frameworks, adaptive methodologies, and digital tools that streamline study execution and improve data accuracy. Emphasis will be placed on overcoming unique challenges in gene therapy trials, such as patient recruitment, complex endpoints, and regulatory compliance. This session aims to equip researchers, clinicians, and trial managers with practical strategies to accelerate development timelines without compromising scientific accuracy.

15:00 Interactive Discussion

15:30 Networking Refreshment Break

CLINICAL TRIALS CHALLENGES PANEL DISCUSSION Maximizing Success in Cell and Gene Trials through Eligibility Precision, Adaptive Design, Monitoring Innovation, and Analytics (15 minutes each section)

16:00 Navigating Patient Eligibility & HLA Matching in Cell and Gene Therapies: Overcoming Immune and Disease Stage Challenges to Ensure Optimal Treatment Success

16:15 Overcoming Clinical Trial Design Challenges in Rare Disease Populations: Innovative Approaches to Endpoint Definition, Statistical Significance, and Regulatory Standards in Small Patient Cohorts

16:30 **Long-Term Follow-Up in Cell and Gene Therapy:** Addressing Safety, Efficacy, and High Costs in Lifelong Monitoring for Durable Treatment Outcomes

#### 16:45 **Risk-Based Monitoring Strategies Tailored for Cell and Gene Therapy Clinical Trial Protocols**

We will unpack the challenges and strategies around insights into designing monitoring plans that prioritize critical data and processes, leverage real-time analytics, and ensure compliance while maintaining patient safety and data integrity. Case studies will highlight practical applications and regulatory expectations, providing a roadmap for successful RBM implementation in CGT clinical development.

#### 17:00 Implementing Real-Time Data Capture and Analytics in Cell and Gene Therapy Clinical Trial Operations

This session will shed light on gain insights into best practices for implementing digital platforms, overcoming integration challenges, and leveraging analytics to optimize protocol adherence, logistics, and trial outcomes. Case studies will illustrate how leading organizations are using real-time data to drive operational excellence compliance in the rapidly evolving CGT landscape.

17:15 Interactive Discussion

17:45 End of day one

### <u>Proceed to the next page for Day Two of the conference agenda.</u>

#### CELL & GENE THERAPY SUMMIT 2025 REDEFINING POSSIBILITIES THROUGH SMARTER DELIVERY, SAFER EDITING, AND SCALABLE INNOVATION DAY 2: 13TH NOVEMBER 2025 (EST)| VIRTUAL CONGRESS

08:50 Chairperson Opening Remarks

### CELL & GENE THERAPY MANUFACTURING PANEL DISCUSSION

Innovative Strategies for Overcoming Cell and Gene Therapy Manufacturing Bottlenecks to Enhance Efficiency, Consistency, and Product Quality

#### 09:00 Addressing Critical Product and Process Challenges in Cell & Gene Therapy Manufacturing

This presentation delves into innovative strategies and technologies that streamline manufacturing workflows, reduce variability, and improve batch consistency, ultimately enabling more reliable and scalable production of life-changing therapies.

#### 09:15 Advancing Fully Automated, Closed-System Manufacturing to Improve Scalability, Contamination Control, and Efficiency in Cell and Gene Therapy Production

This talk explores the integration of fully automated, closed-system technologies in CGT manufacturing. Attendees will learn how automation minimizes human error, reduces contamination risks, and supports regulatory compliance. The session will showcase case studies, discuss equipment and digital integration, and highlight how these systems enable scalability from clinical to commercial production.

#### 09:30 Overcoming Process and Scale-Up Challenges in Viral Vector Manufacturing to Enable Commercial-Ready Cell and Gene Therapies

In this session, we examine key strategies for scalable and robust viral vector manufacturing, including upstream and downstream optimization, platform technologies, and quality control. Attendees will gain insights into process intensification, automation, and regulatory expectations, with real-world case studies illustrating successful tech transfers.

#### 09:45 Modular Manufacturing Platforms for Cell Therapies: Designing Agile, Scalable Facilities to Enable Multi-Product Flexibility and Accelerate Clinical-to-Commercial Transitions.

The talk investigates how modular facility design and process platforms are transforming cell therapy manufacturing. Attendees will gain insights into implementing flexible, multi-product environments that support rapid product changeover, tech transfer, and scale-up. We'll highlight engineering strategies, facility layouts, automation integration, and regulatory considerations that reduce time-to-market while ensuring GMP compliance. Case studies will showcase real-world examples of modular cleanroom pods, closed systems, and digital infrastructure that support concurrent manufacturing of autologous and allogeneic therapies.

#### 10:00 Standardizing Potency Assays for Cell and Gene Therapies to Ensure Consistent Clinical Outcomes via Robust Analytical Strategies

In this presentation, we address the critical role of potency assays in cell and gene therapy development, focusing on analytical strategies to ensure consistency, reliability. Topics include assay design principles, qualification and validation, biological relevance, and case studies highlighting challenges in autologous, allogeneic, and gene-modified products.

#### 10:15 Explore how engineering allogeneic cell therapies enables scalable, off-the-shelf manufacturing strategies that enhance access, lower costs, and accelerate commercial readiness for global impact.

This session explores the evolving landscape of allogeneic cell therapy manufacturing, focusing on scalable bioprocesses, universal donor strategies, and gene editing technologies that enable off-the-shelf therapeutic platforms. Key topics include closed-system automation, potency and safety challenges, and how robust CMC frameworks can support affordability and global commercialization. The talk will highlight technical solutions to overcome immunogenicity, ensure consistent product quality, and meet regulatory expectations for allogeneic therapies.

#### 10:30 Redefining Manufacturing Excellence in Cell & Gene Therapy: Operational Innovations for Consistency and Scalability.

This talk will explore how the industry is redefining manufacturing excellence through operational innovation. Key topics include advanced automation strategies, datadriven process control, and quality-by-design (QbD) approaches that ensure product consistency across batches. Attendees will gain insights into best practices, real-world case studies, and the strategic integration of technology to overcome common production bottlenecks in CGT.

10:45 Interactive Discussion

11:30 Networking Refreshment Break

#### 12:00 Evaluating Scalable Approaches to Cell and Gene Therapy Manufacturing in the Context of Speed, Safety, and Sustainability

In this session, we will navigate scalable manufacturing strategies that optimize speed to market, ensure product safety and quality, and uphold principles of sustainability. We will examine current bottlenecks, emerging technologies, and innovative process designs that enable efficient, compliant, and environmentally conscious production.

12:25 Questions & Discussion

#### 12:30 Integrating cutting-edge technologies in cell and gene therapy manufacturing to optimize speed, safety, and environmental sustainability in biotech.

This presentation examines how cutting-edge technologies are revolutionizing cell and gene therapy manufacturing by enhancing production speed, ensuring patient safety, and advancing environmental sustainability. Participants will explore innovations such as advanced automation, real-time process monitoring, and eco-friendly manufacturing methods that improve efficiency while minimizing the biotech industry's environmental impact. The session will showcase actionable strategies and pioneering solutions that align with regulatory standards and streamline the transition from development to large-scale commercial production. Discover how these technology-driven advancements are shaping the future landscape of cell and gene therapy manufacturing.

12:25 Questions & Discussion

13:00 Networking Lunch Break

#### 14:00 Unlocking Scalable Innovation in Cell & Gene Therapy: Strategic Frameworks for Accelerating Market Access and Commercial Viability Across Global Biotech

This conversation centers around how to align innovation with infrastructure, streamline development-to-launch timelines, and ensure broader patient access. Attendees will gain actionable insights into building adaptable, future-ready strategies that support CGT growth across diverse markets worldwide.

14:25 Questions & Discussion

#### 14:30 Reimbursement Readiness: Building Early Market Access Strategy into R&D to Maximize Value and Minimize Launch Delays

This discussion sets out to explore how proactive planning for reimbursement during product development can streamline evidence generation, support pricing and value narratives, and reduce time-to-market. Attendees will gain insights into aligning clinical and regulatory strategies with payer expectations, optimizing health economics and outcomes research (HEOR), and fostering cross-functional collaboration to enhance commercial success. Real-world examples and best practices will illustrate how to build reimbursement readiness into the DNA of therapeutic innovation.

14:55 Questions & Discussion

15:00 Networking Refreshment Break

#### PANEL DISCUSSION

Driving Market Access in Cell and Gene Therapies Through Value-Based Pricing and Real-World Evidence Integration

#### 15:30 Unlocking Payer Value: Aligning Cell and Gene Therapy Pricing Strategies with Long-Term Outcomes and Real-World Evidence for Market Access Success

This session explores innovative strategies to align CGT pricing with long-term clinical outcomes and real-world evidence (RWE), addressing payer concerns around value, sustainability, and budget impact. Attendees will gain insights into outcome-based agreements, data-driven value assessments, and collaborative approaches to market access that can unlock payer confidence and ensure timely patient access. Learn how to position therapies for reimbursement success in a value-based healthcare environment.

#### 15:45 Automating Evidence: Can Al Bridge Access Gaps in High-Cost Advanced Therapies?

Artificial intelligence is gaining traction in predicting patient outcomes and optimizing reimbursement data. This talk assesses current use cases, potential, and readiness of AI for real-time data generation and payer engagement in cell and gene therapy markets.

#### 16:00 Strategic Stakeholder Engagement to Navigate Payer Skepticism in Cell and Gene Therapy Reimbursement

This session will explore how early, strategic engagement with key stakeholders, including payers, providers, regulators, and patient advocacy groups can help mitigate uncertainty and support more sustainable reimbursement pathways. Attendees will gain insights into best practices for evidence generation, real-world data integration, and value communication tailored to payer concerns. The session will also highlight successful models and collaborative approaches that have improved market access and reimbursement success for CGT innovators.

#### 16:15 Indication-Based Pricing: Opportunity, Risk, and Reality in Cell and Gene Therapy Expansion

Indication-based pricing offers promise, but limited implementation raises questions. Learn how policy, payer adoption, and provider reimbursement models impact its future, and what lessons can be drawn from successful pricing model.

#### 16:30 Designing Outcome-Based Agreements That Balance Payer Risk and Biotech ROI in Cell and Gene Therapies

This session explores how outcome-based agreements (OBAs) can offer a solution aligning payment with therapeutic success while balancing the financial risks for payers and ensuring sustainable returns for biotech innovators. Attendees will gain insights into structuring OBAs, real-world implementation challenges, and strategies to measure outcomes effectively in a rapidly evolving regulatory and commercial landscape.

16:45 Interactive Discussion

17:30 End of conference