# CGTP Summit 2025

Schedule

## Monday, 9 June, 2025

07:00-08:20 Foyer A-C

### Continental Breakfast

IP -In Person
CGTP Summit

07:00-08:20 Foyer A-C

### Registration

CGTP Summit

08:20-08:30 Salons A-C

### CASSS Welcome & CGTP Summit 2025 Introduction

Isabella Palazzolo
Live Streamed
CGTP Summit

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

The CGTP Summit will comprehensively address CMC-related delays in the clinical development of CGT products, with a primary focus on the development of a globally acceptable potency strategy. These delays often arise from several factors, including a lack of clarity on the minimum potency requirements needed to advance into Phase 1 studies, and a lack of alignment on the strategies necessary to proceed to pivotal or late-phase studies. Additionally, the absence of global harmonization on potency requirements poses significant challenges, making it difficult for the industry to advance a unified global product.

Throughout the Summit, we aim to highlight pressing issues and provide phase-appropriate solutions. By drawing lessons from more established CGT modalities such as AAV and CAR-T, which have successfully reached commercial stages, we can better navigate the path forward for newer modalities. The Summit will also delve into the challenges specific to emerging technologies, such as genome and epigenome editing, and induced pluripotent stem cells (iPSCs), focusing on their strategies for entering early-phase clinical development.

Participants can expect a rich agenda featuring presentations from industry experts in the CGT field. These sessions will provide valuable insights and practical guidance. Moreover, the Summit will include interactive panel discussions, enhanced by the presence of regulatory representatives.

In addition to these discussions, the Summit will incorporate short case studies designed to openly discuss common potency challenges, and a regulatory panel discussion, which will feature regulators from key regulatory agencies. The goal is to foster an open dialogue that can lead to the development of innovative solutions and best practices to overcome the hurdles in CGT product development. This comprehensive approach ensures that the Summit will be a valuable platform for addressing critical issues and fostering collaboration within the CGT industry.

08:30-10:20 Salons A-C

## Session I - Building Strong Foundations: Early Phase Strategies for Potency Assays

Francois Gianelli, Elaine Peters
Live Streamed
CGTP Summit

Session Chairs: Francois Gianelli, *TreeFrog Therapeutics* and Elaine Peters, *Senior Leader in Cell and Gene Therapy Analytical Development*

Testing for potency is expected for all biological medicinal products including ATMPs. Regulators expect potency assays will reflect the product's mechanism of action (MOA) and, ideally, be predictive of the clinical response. The potency testing expectations for AAV and CAR-T products are relatively established. However, as more complex and novel modalities are being developed, there is still much to learn about what attributes are important for potency assurance.

In early phases of development, the mechanism of action of a new ATMP may not be fully understood. At early stage, developers face answering a number of questions: what should be measured for potency? What analytical methods should be used? How much characterisation data is needed? Starting multiple potency assay candidates' development early in parallel to increasing knowledge of the MOA is the ideal approach. However, considering limited resources and test materials available early-on, it remains challenging to develop a fully relevant and yet efficient potency assay for early clinical trials.

This session will highlight pragmatic potency approaches for entering the clinic with novel therapeutic modalities while paving the way for later product development.

Session Speakers:

Case Study - Challenges and Opportunities in Developing Functional Dopamine Secretion Assays for Microtissues
Lucie Manache-Alberici, *TreeFrog*

Considerations for Potency Assay Development for Gene Editing Products
Ilya Shestopalov, *Tessera Therapeutics*

Additional Panelist:

Christina Grigoriadou, *AstraZeneca*

10:20-10:50 Foyer A-C

## Networking Break

IP -In Person
CGTP Summit

10:50-12:40 Salons A-C

## Session II - Challenges and Opportunities for Potency Assays

Kaavya Maganti, Michael Molony
Live Streamed
CGTP Summit

Session Chairs: Kaavya Maganti, *Eli Lilly and Company* and Michael Molony, *Insmed Incorporated.*

Potency assays for cell and gene therapy products present unique, multi-faceted challenges due to the complex nature of these therapies. One of the primary challenges continues to remain the development of a functional potency assay that can accurately measure the biological activity of to confirm the intended therapeutic effect.

Furthermore, the rapid pace of innovation in the field of cell and gene therapy, and urgency to bring lifesaving medicines to patients, warrants potency assay development to be adaptable to new technologies and therapeutic approaches. This requires ongoing research and development and necessitates a phased and multi-attribute approach to potency assay development.

This session seeks to discuss some of the common challenges that are yet to be overcome in this unique therapeutic space and explore potential opportunities to build a path forward to keep up with clinical development timelines.

Presentations and panel for this session will discuss:

- Global regulatory expectations and streamlining testing strategies

- FDA draft guidance on Potency Assurance – Identifying potency related CQAs and phase specific expectations with limited manufacturing experience

- Potential to use platform approaches in the context of deriving a potency assurance strategy and streamlining analytical testing panels

- Matrix approaches – While matrix approaches have seen success with regulators in lieu of a functional potency assay, expectation to include an infectivity assay (TCID50) continues to remain a challenge.

- Early phase vs late phase potency assay development - transition and appropriate bridging strategies to ensure comparability

- Common challenges and ways to navigate them – use of representative cell lines (include health authority expected rationale for use of engineered cell lines), setting clinically meaningful acceptance criteria, validation strategies given limited number of batches to name a few.

Session Speakers:

Early Development of an In Vitro Relative Potency Assay for an AAV9 Gene Therapy Candidate:  Transitions from Exploratory Research to a GMP-ready Environment
Mike Jorge*, Insmed Incorporated*

Advancing Genomic Medicine: Navigating Challenges in CMC Potency Assay Development Throughout the Product Life-Cycle
Phillip Ramsey, *Sangamo Therapuetics, Inc.*

Strategy for Potency Determination of Gene Therapy Products - Overview of the BioPhorum GT Potency Strategy Workstream
Fabian Borghese, *UCB S.A.*

Additional Panelists:

Ingrid Markovic, *Independent Expert*

Bhavin Parekh, *Eli Lilly and Company*

12:40-13:40 Brookside A&B (Lower Level)

## Lunch

IP -In Person
CGTP Summit

13:40-15:15 Salons A-C

## Session III - Deep Dives into Key Topics in Cell and Gene Therapy Potency

Diane Blumenthal, Mark Galbraith, Mayumi Holly
Live Streamed
CGTP Summit

Session Chairs: Diane Blumenthal*, Dianthus Biopharma Consulting, LLC,* Mark Galbraith, *TRANSPOSON Consulting Group, LLC.,* and Mayumi Holly, *Tune Therapeutics*

Potency is a critical quality attribute that is intended to demonstrate the ability of the drug product to effect a given result and be reflective of the intended mechanism of action. As such, Potency plays a critical role in the development of cellular and gene therapies. Potency assays present analytical, CMC, and regulatory challenges for cellular and gene therapies. The biological activity, intricacies of cellular and gene therapies, and mechanism of action are often complex or not well understood, requiring development of bespoke methods, which must meet both the needs of evaluating manufacturing consistency as well as clinical efficacy. This session will dive into key challenges of potency assays facing drug developers today.

Challenges with the industry standard potency assay for AAV (TCID50) and strategies for development of new methodologies: What information is the TCID50 providing? How can TCID50 be improved to provide more meaningful and consistent data? Can TCID50 be replaced as other assays are developed and what is the strategy for replacement?

In vitro potency assays for CAR-T cell therapies: Balancing QC-friendly assays for lot release, ensuring manufacturing consistency, short turnaround time, and clinical relevance.

US FDA Draft Guidance on Potency Assurance for Cellular and Gene Therapies and Challenges with applying this guidance. How the US FDA guidance differs from guidance provided by other agencies with particular focus on guidance provided by the EMA.

Panelists:

Gaël Debauve, *UCB S.A.*

Neil Haig, *BioBrilliance, CMC Consulting*

Max Tejada, *Genentech, a Member of the Roche Group*

Keith Wonnacott, *Lexeo Therapeutics, Inc.*

15:15-15:45 Foyer A-C

## Networking Break

IP -In Person
CGTP Summit

15:45-17:15 Salons A-C

## Session IV - Navigating Potency Assays in Cell and Gene Therapy: Global Regulatory Perspectives

Michael Molony, Isabella Palazzolo
Live Streamed
CGTP Summit

Session Chairs: Michael Molony, *Insmed Incorporated.* and Isabella Palazzolo, *Intellia Therapeutics, Inc.*

This panel discussion will unite regulators from multiple countries to share their perspectives on the regulatory challenges and best practices for managing potency assays during the clinical development of Cell and Gene Therapy (CGT) products. The discussion will focus on global regulatory approaches to potency assays, including key considerations for developing a matrix approach of potency assays that can accurately measure multiple attributes of a CGT product, and reflections on what an acceptable potency matrix evolution during drug development looks like. The panel will address the challenges and opportunities in CGT product potency development, including what types of readouts are acceptable when a biological assay is not quantitative, sufficiently robust, or lacks precision, as well as reference standard bridging expectations.

A key objective of the discussion is to provide a forum for regulators to share their perspectives on global harmonization of regulatory approaches to potency assays. Regulators will share their experiences and insights on regulatory requirements and expectations for potency assays in different regions, discussing strategies for ensuring consistency and comparability between manufacturing batches through potency testing.

Through this discussion, we aim to advance the understanding of regulatory challenges and best practices for managing potency assays for CGT products, and to promote global harmonization of regulatory approaches to this complex and rapidly evolving field.

Session Panelists:

Andreea Barbu, *Swedish Medical Products Agency*

Marcel Hoefnagel, *Medicines Evaluation Board (MEB)*

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

Tal Salz, *Dark Horse Consulting Group*

Christopher Storbeck, *Health Canada*

17:15-17:25 Salons A-C

## Closing Remarks & Invitation to CGTP Summit 2026

Isabella Palazzolo
Live Streamed
CGTP Summit

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

17:25-18:40 Brookside A&B (Lower Level)

## CGTP Summit Networking Reception

IP -In Person
CGTP Summit

Mix and mingle with fellow attendees to celebrate the completion of the CGTP 2025 Summit!