CGTP Summit 2026

Schedule

Monday, 8 June, 2026

07:00-08:20 Foyer A-C

Continental Breakfast

Presentation type: IP - In Person
CGTP Summit
Breakfast will be available until 09:00 Eastern

07:00-08:20 Foyer A-C

Registration

Presentation type: IP - In Person
CGTP Summit

08:20-08:30 Salons A-C

CASSS Welcome & CGTP Summit 2026 Introduction

Kevin Okimura
Presentation type: LS - Live Streamed
CGTP Summit
Specification setting for advanced therapy medicinal products (ATMPs) remains one of the most persistent and complex challenges across the development lifecycle, particularly in the context of accelerated development timelines, inherent variability, evolving potency strategies, and limited manufacturing experience. Traditional specification frameworks are often poorly suited for non-well-characterized, individualized, or patient-specific therapies, creating uncertainty around clinical relevance, regulatory expectations, and licensure readiness.

This summit convenes senior industry leaders and regulators from multiple global health authorities to examine practical strategies for defining, evolving, and defending ATMP specifications across the product lifecycle. The sessions will address challenges specific to ATMP modalities, illustrated through case studies, best practices, and thought-provoking panel discussions. The program concludes with a global regulatory panel, where insights will be shared regarding the current and evolving regulatory framework governing ATMP specifications.

Through focused session presentations and open industry–regulatory dialogue, the summit will address key challenges in ATMP specifications, with the goal of enabling a shared understanding and globally informed, fit-for-purpose approaches to support robust ATMP specifications.

08:30-10:20 Salons A-C

Session I: Challenges for ATMP Specifications - Part I

Allen Callaway, Sarmitha Sathiamoorthy, Christopher Storbeck
Presentation type: LS - Live Streamed
CGTP Summit

Specification setting for Advanced Therapy Medicinal Products (ATMPs) is built on the principles of ICH Q6B: Specifications for Biotechnological/Biological Products. The challenge for ATMP developers is the integration of regulatory principles, scientific understanding, pre-defined specifications by regulators, and platform experience for a wide variety of therapeutic ATMP modalities. As ATMP modalities become increasingly complex and diverse, sponsors face growing difficulty defining specifications that are development-stage appropriate, and adaptable across ATMP modalities and platforms.

This session will begin with an introduction to the fundamentals of specification setting for ATMPs. Speakers will discuss how traditional concepts such as critical quality attributes, acceptance criteria, and control strategies can be applied—and appropriately adapted—to advanced modalities. The discussion will then examine practical approaches to building specifications across three major advanced modality categories—cell therapies, viral vector-based gene therapies, and nucleic acid-based products—highlighting both commonalities and modality-specific challenges.

The session will then explore how platform and prior knowledge can be effectively leveraged to inform specification development. Drawing on experience from existing products and related platforms, strategies for extrapolating quality attributes, setting initial acceptance criteria, and managing uncertainty while maintaining regulatory flexibility will be explored.

Finally, the session will address decision-making around differentiating release methods (with defined acceptance criteria) from characterization methods. Speakers will discuss how to determine which quality attributes must be controlled at release versus those better suited for characterization, platform understanding, or lifecycle management, and how this distinction evolves across development stages. Data and evaluation criteria needed to support these decisions will be discussed, including assay performance, attribute relevance, risk assessment, and links to clinical and non-clinical outcomes. The importance of cross-functional collaboration—between clinical, non-clinical, and analytical stakeholders—will be emphasized as a key enabler of robust and defensible specification strategies.

Session Speakers:

Regulatory Perspectives on Establishing Specifications for Cell and Gene Therapy Products: From Speculation to Specification
Graeme Price, *CBER, FDA*

Evergreen Specification Setting Practices
Julia O'Neil, *Direxa Consulting*

Lifecycle Aligned Specifications: Ensuring Quality and Consistency in Cell and Gene Therapy Development
Tiffany Lucas, *Eliquent Life Sciences*

Additional Panelist:

Christopher Storbeck, *Health Canada*

10:20-10:50 Foyer A-C

Networking Break

Presentation type: IP - In Person
CGTP Summit

10:50-12:40 Salons A-C

Session II: Challenges for ATMP Specifications - Part II

Ilya Shestopalov, Gang Wang
Presentation type: LS - Live Streamed
CGTP Summit

Building on the foundational principles of ATMP specification setting discussed in Part 1, this second session will focus on advanced and emerging challenges that arise in rare disease, personalized, and accelerated development programs. These scenarios increasingly define the cell and gene therapy landscape and require specification strategies that balance scientific rigor, operational feasibility, and regulatory expectations under significant program and process uncertainty.

The session will begin by addressing specification settings for rare and ultra-rare programs, where the first GMP-manufactured lot may serve as both first-in-human and pivotal material. Speakers will discuss how phase-appropriate specifications can be established when product knowledge and material availability is minimal, while still enabling late-stage control and characterization expectations. Approaches for prospective planning, data generation, and risk management will be explored to support this dual-use reality.

Next, the session will examine the unique challenges of personalized therapies, where each manufactured batch corresponds to a single patient. Discussion will focus on how companies define, justify, and evolve specifications based not only on QTPP and clinical data, but also on manufacturing and process capability. Strategies for interpreting and aggregating data in an “n=1” paradigm will be shared, along with practical models for collaboration between CMC and clinical teams to contextualize product quality against patient outcomes.

Finally, the session will explore specification challenges in accelerated development pathways. Case-based discussions will consider early-phase programs with strong expectations of efficacy, as well as programs entering Phase 2/3 with compelling clinical data. Topics will include identifying critical quality data needed to support late-stage development and approval, opportunities for post-approval learning, and managing the risk of overly tight commercial specifications driven by limited batch numbers or small patient populations during clinical development.

Session Speakers:

Rethinking Product Release Specifications for Autologous CAR T Cell Therapy
Kedar Dave, *Bristol-Myers Squibb Company*

Rethinking Specification Setting in Autologous Cell and Gene Therapies: From Variability to Global Alignment
Shilpa Suravajhala, *Vertex Pharmaceuticals Incorporated*

Between Scylla and Charybdis: Navigating Specification Setting for Rare and Ultra Rare Disease Programs
Joshua Kidder, *Ultragenyx Pharmaceutical Inc*

Additional Panelist:

John Schiel, *Advanced Research Projects Agency for Health (ARPA-H)*

12:40-14:00 Brookside A&B (Lower Level)

Lunch

Presentation type: IP - In Person
CGTP Summit

14:00-15:15 Salons A-C

Session III: Considerations When Setting Specifications for Autologous CAR T-cell Products

Kathleen Francissen, Marcel Hoefnagel
Presentation type: LS - Live Streamed
CGTP Summit

There is a growing body of knowledge about autologous chimeric antigen receptor (CAR) T-cell products, which have been on the market since 2017 to treat patients with hematologic malignancies. Each batch of an autologous CAR T-cell product is obviously manufactured for a specific patient beginning with the patient's own cells. Release specifications are set as usual for clinical and commercial batches to ensure their quality, safety, and efficacy. However, there are special considerations when setting acceptance criteria for autologous CAR-T cell products due to factors such as patient-to-patient variability of the starting cells.

A significant percentage of batches fail to meet the acceptance criteria for one or more release tests. Such out-of-specification (OOS) results are typically obtained for one test, such as cell viability or potency, which are affected by the health status of each patient. There is published clinical evidence showing that batches with, for example, lower viability than the acceptance criteria, are still functional in patients and can lead to positive clinical outcomes.

While the use of OOS batches to treat patients is normally prohibited, it can be made possible in exceptional circumstances with an autologous product for a life-threatening condition through an evaluation by the treating physician and the manufacturer. This situation is sufficiently common that there are guidance documents issued by MHRA and EMA. Based on the growing body of data and experience, we will discuss the approach to setting acceptance criteria for autologous cell-based products.

References (Click Reference Links Below to Open):

Kato, K. et al., Clinical outcomes of Japanese patients treated with out-of-specification tisagenlecleucel in a phase 3b trial, *Cytotherapy (2025) 27:938-943*

Dulobdas, V. *et al.,* Risk factors for CAR T-cell manufacturing failure and patient outcomes in large B-cell lymphoma: a report from the UK National CAR T Panel, *Blood Cancer Journal (2025) 15:30 ; https://doi.org/10.1038/s41408-025-01225-9*

Worel, N. et al., CAR-T cell manufacturing failures and out-of-specification products in the real-world setting: A survey from the EBMT cellular therapy and immunobiology working party, (letter to editor) *Bone Marrow Transplantation; https://doi.org/10.1038/s41409-025-02623-0 (www.nature.com/bmt)*

Rossoff, J. et al., Out-of-specification tisagenlecleucel does not compromise safety or efficacy in pediatric acute lymphoblastic leukemia (letter to editor)

Chong, E.A., Levine, B.L., et al. CAR T cell viability release testing and clinical outcomes: is there a lower limit? Blood. 2019 Sep 25;134(21):1873–1875. doi: 10.1182/blood.2019002258, https://pmc.ncbi.nlm.nih.gov/articles/PMC6872962/

Additional Panelists:

Kelly Kral, *Genetix Biotherapeutics Inc.*

Scott Nichols, *Kite, A Gilead Company*

15:15-15:45 Foyer A-C

Networking Break

Presentation type: IP - In Person
CGTP Summit

15:45-17:05 Salons A-C

Session IV: Global Regulatory Panel on Specifications

Michael Molony, Kevin Okimura
Presentation type: LS - Live Streamed
CGTP Summit

This global panel will discuss how different regions approach the establishment and evolution of Advanced Therapy Medicinal Product (ATMP) specifications, including expectations for flexibility, scientific justification, and lifecycle management. The discussion will highlight common challenges, areas of alignment, and opportunities for continued global convergence.

Featured Panelists:

Marcel Hoefnagel, *MEB - Medicines Evaluation Board, Netherlands*

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

Elizabeth Lessey-Morillon, *CBER, FDA*

Christopher Storbeck, *Health Canada*

17:05-17:15 Salons A-C

Closing Remarks & Invitation to CGTP Summit 2027

Kevin Okimura
Presentation type: LS - Live Streamed
CGTP Summit

17:15-18:30 Brookside A&B (Lower Level)

CGTP Summit Networking Reception

Presentation type: IP - In Person
CGTP Summit

Mix and mingle with fellow attendees to celebrate the completion of the CGTP 2026 Summit!