Cell and Gene Therapy Products Europe 2025

Schedule

Thursday, 23 October, 2025

06:30-08:30 PURO Restaurant

Mövenpick Breakfast Buffet

Presentation type: IP - In Person
CGTP Europe Symposium

Breakfast is included 23-24 October for attendees staying at the Mövenpick Hotel. Conference attendees not registered as hotel guests must arrange their own breakfast.

Breakfast is available in the PURO Restaurant until 10:00 AM.

07:15-08:30 Foyer Ballroom (2nd Basement Level)

Registration

Presentation type: IP - In Person
CGTP Europe Symposium

Registration is open until 17:00.

08:30-08:40 The Ballroom (2nd Basement Level)

CASSS Welcome & CGTP Europe 2025 Introduction

Pepijn Burgers
Presentation type: IP - In Person
CGTP Europe Symposium

08:40-10:45 The Ballroom (2nd Basement Level)

Plenary Session 1 - Matrix Approach to Control Potency of Cell and Gene Therapy Modalities

Pepijn Burgers, Andrea Challand
Presentation type: IP - In Person
CGTP Europe Symposium

The control strategy for potency of cell and gene therapy (CGT) products is a fundamental yet challenging aspect of product development as the mechanism of action (MoA) for these modalities can be complex.

In this session we want to demonstrate matrix strategies which tackle important aspects (e.g., correlation between assays and limited number of batches/data) for potency control. We will explore how a combination of release assays and supplementary characterization assays can demonstrate consistent product quality, confirm the mechanism of action (MoA), and ultimately ensure a clinically efficacious product. Throughout development this matrix develops with it being clearly defined upon commercial application. Additionally, considerations for specification setting strategies (e.g., limited number of batches, high variability, patient centricity) will be discussed.

To conclude, this session aims to foster a comprehensive discussion and understanding on approaches for potency control matrix strategies and specification setting to ensure successful development and commercialization.

Session Speakers:

Potency Assurance in Cell Therapy: Bridging Theory and Practice
Matthias Heemskerk, *Galapagos NV*

Potency Strategy for a Novel In Vivo Lentiviral Vector Drug Product
Ruth Chenault, *Umoja Bipharma*

Cell-Based LC-MS Potency Assay for Individualized mRNA Immunotherapy Autogene Cevumeran
Aline Eisinger, *BioNTech SE*

Additional Panelists:

Andreea Barbu, Swedish Medical Products Agency

Marcel Hoefnagel, *Medicines Evaluation Board (MEB)*

10:45-11:10 Foyer Ballroom (2nd Basement Level)

Networking Break

Presentation type: IP - In Person
CGTP Europe Symposium

11:10-12:55 The Ballroom (2nd Basement Level)

Plenary Session 2 - Navigating the Complexities of Data Management and Application in Cell and Gene Therapy

Pepijn Burgers, Petra Minder
Presentation type: IP - In Person
CGTP Europe Symposium

In the rapidly evolving field of cell and gene therapy (CGT), the management and application of complex data, commonly referred to as 'Big Data', has become a critical component in advancing therapeutic development. Considering, for example, the more complex modalities and analytical tools, advances in manufacturing processes such as digital twins, continuously more data will be generated. This vast amount of data must be properly stored, processed, analyzed, and validated to help ensure the consistency, safety, and efficacy of each product. Three main challenges surface with the generation of these data: (1) how to make all equipment digital and linked, (2) how to store the data and make it easily accessible and (3) how to apply all those data.

This session will discuss the challenges of managing the large and heterogeneous data pool created during development and the commercial lifecycle of CGT products, types of machine learning/AI tools which digest those data, the current regulatory expectation and the aspects of digitalization.

Session Speakers:

Single-Cell Sequencing in Cell and Gene Therapy: Opportunities and Challenges for QC and Regulation
Mauro Muraro, *Single Cell Discoveries BV*

End-to-End Digital Twins for CGT Processes
Thomas Zahel, *Körber Pharma Austria GmbH*

From Data to Cure: How AIDPATH Powers CAR T Cell Therapy with Big Data, Analytics, and Continual Improvement
Menno Brandjes, *ORTEC Logiqcare*

Additional Panelists:

Marcel Hoefnagel, *Medicines Evaluation Board (MEB)*
Petra Minder, *Swissmedic.*

12:55-13:55 Topaz 1 (2nd Floor)

Lunch

Presentation type: IP - In Person
CGTP Europe Symposium

13:55-15:40 The Ballroom (2nd Basement Level)

Plenary Session 3 - Comparability: Challenges and Case Studies

Andreea Barbu, Markus Haindl
Presentation type: IP - In Person
CGTP Europe Symposium

In the rapidly evolving landscape of Advanced Therapy Medicinal Products (ATMPs), maintaining product comparability throughout development phases and following manufacturing changes across the product life cycle is crucial for ensuring quality, safety, and efficacy. Despite the early clinical success of cell and gene therapies, comparability remains a persistent challenge due to the poorly characterized nature of many ATMPs. This session is designed as a venue for industry experts, regulators, and stakeholders to share insights, foster collaborations, and contribute to the development of standardized approaches that ensure the safety and efficacy of cell and gene therapy products.

The session will feature presentations from the CGT industry and host interactive panel discussions to address challenges related to conventional comparability strategies for cell and gene therapy products. Topics for discussion may include the identification of critical quality attributes and process parameters for various ATMP products, risk assessment strategies for CMC changes, and the application of analytical methods for comprehensive comparability evaluation.

Finally, the session will conclude with an expert panel of regulators and industry representatives to discuss comparability challenges, regulatory expectations, and strategies for overcoming these hurdles through an open and productive dialogue.

Session Speakers:

Adapting Comparability Principles to Enable Continuity During the Lifecycle of ATMPs
Kathleen Francissen, *Genentech, a Member of the Roche Group*

Few Batches, Big Questions: Navigating AAV Analytical Comparability
Sonya Schermann, *Ascend Ltd.*

Comparability for Autologous Cell Therapy Products – Challenges and Learnings
Olga Sarnowska, *Bristol-Myers Squibb Company*

Additional Panelist:

Matthias Renner, *Paul-Ehrlich-Institut*

15:40-16:00 Foyer Ballroom (2nd Basement Level)

Networking Break

Presentation type: IP - In Person
CGTP Europe Symposium

16:00-17:05 The Ballroom (2nd Basement Level)

Plenary Session 4 - Controlling the Genetic Stability of Cell-based Products

Christiane Niederlaender, Maria Rathmann Sørensen
Presentation type: IP - In Person
CGTP Europe Symposium

Risk of genetic instability and aberrations has long been a concern for regulators in relation to cell-based products. These risks have historically been considered more significant for stem cells and cells genetically modified with integrating viruses. The increasing use of genome editing tools has now increased complexity of these issues with the potential presence of many off-target genetic events that require complex approaches for detection.

This session will critically evaluate the CMC risks related to genetic instability of cell-based products and discuss regulatory expectations for the control of genomic variations. We will also discuss the suite of analytical approaches currently available and how the different methodologies can complement each other. Furthermore, we will take a technical deep dive into approaches for risk assessment of genomic integrity and compare various molecular biological and bioinformatical tools.

Session Speakers:

Evaluating Sequencing Strategies for ATMP Genomic Risk Assessment
Rafaella Buzatu, *MEB - Medicines Evaluation Board*

Additional Panelist:

Matthias Heemskerk, *Galapagos NV*

17:05-18:35 Floor 2 Foyer

CGTP Europe 2025 Welcome Reception

Presentation type: IP - In Person
CGTP Europe Symposium

Join us in the Floor 2 foyer to celebrate the start of CGTP Europe 2025! Mix and mingle with attendee while enjoying delicious Swiss cuisine.

Friday, 24 October, 2025

06:30-09:00 PURO Restaurant

Mövenpick Breakfast Buffet

Presentation type: IP - In Person
CGTP Europe Symposium

Breakfast is included 23-24 October for attendees staying at the Mövenpick Hotel. Conference attendees not registered as hotel guests must arrange their own breakfast.

Breakfast is available in the PURO Restaurant until 10:00 AM.

08:00-09:00 Foyer Ballroom (2nd Basement Level)

Registration

Presentation type: IP - In Person
CGTP Europe Symposium

Registration is open until 16:00.

09:00-09:05 The Ballroom (2nd Basement Level)

Welcome Day 2 & Keynote Introduction

Christiane Niederlaender
Presentation type: IP - In Person
CGTP Europe Symposium

09:05-10:10 The Ballroom (2nd Basement Level)

Keynote Presentation

Christiane Niederlaender
Presentation type: IP - In Person
CGTP Europe Symposium

Genetic engineering of hematopoietic stem cells (HSC) with lentiviral vectors has been providing substantial benefit to growing numbers of patients affected by primary immunodeficiencies, hemoglobinopathies and storage disorders. Long-term follow up shows stable hematopoietic reconstitution by high numbers of corrected HSC without signs of clonal expansion or exhaustion. Precise engineering by gene editing may further improve the reach and safety of HSC gene therapy by achieving in situ gene correction or targeted transgene integration. Homology-driven editing, however, remains limiting in long-term HSC and the genetic outcome at target sites heterogenous and, for some by-products, potentially genotoxic. Template delivery by Integrase-defective lentiviral vectors rather than AAV6 and the use of lipid nanoparticles instead of electroporation may increase safety and efficiency of the procedure. Coupling selection for the intended edit and purging adverse outcomes may provide a preferred path towards clinical application of this currently unique modality enabling long-range edits. On the other hand, the emergence of base and prime editors that bypass the requirement for DNA double-strand breaks (DSB) allows editing single/few mutant nucleotides with limited activation of DNA damage response. We have shown, however, that DSBs are significantly lowered but not abrogated. Moreover, the expression of constitutive deaminase domains within the editors may impact the mutagenic load of treated cells. While these potentially genotoxic outcomes can be mitigated by optimizing expression and culture conditions, they should be better investigated and monitored in emerging clinical applications. Overall, our work should advance HSC gene therapy by a combination of transformative approaches leveraging on precision genetic engineering while alleviating the morbidity of the procedure, broadening application to several diseases and patients worldwide.

Keynote Speaker: Luigi Naldini, *San Raffaele Telethon Institute for Gene Therapy*

10:10-10:30 Foyer Ballroom (2nd Basement Level)

Networking Break

Presentation type: IP - In Person
CGTP Europe Symposium

10:30-12:15 The Ballroom (2nd Basement Level)

Plenary Session 5 - Enhancing AAV Gene Therapy Vectors: Advanced Analytical Strategies for Comprehensive Characterization

Andreas Maccani, Iris van Koppen
Presentation type: IP - In Person
CGTP Europe Symposium

While a standard set of critical quality attributes (CQAs) has been established for adeno-associated virus (AAV) gene therapy vectors, achieving a truly comprehensive characterization remains a challenge. Key aspects, such as the heterogeneity of recombinant AAV genomes and the impact of post-translational modifications (PTMs), are not yet fully understood, posing potential risks to efficacy and safety.

This session will explore state-of-the-art analytical approaches for the in-depth characterization of AAV vectors. We will examine the strengths and limitations of these techniques and discuss their role in enhancing process development, product characterization, and control strategies.

By fostering a multidisciplinary dialogue, this session aims to bridge the gap between scientific innovation, industrial application, and regulatory perspectives. Attendees will gain valuable insights into how advanced analytical tools can enhance the safety, efficacy, and quality of AAV-based gene therapies.

Session Speakers:

Characterising Viral Vectors for Gene Therapy Deliver Using Mass Spectrometry on Different Levels
Jonathan Bones, *The National Institute for Bioprocessing Research and Training and University College Dublin, Ireland*

Cutting-Edge Analytical Strategies for Ensuring rAAV Genome Integrity and Identity
Christoph Gstöttner, *Roche Diagnostics GmbH*

Biophysical Characterization and Control of AAV Using Multi-Attribute Methods
Angus Hibbins, *Johnson & Johnson Innovative Medicine*

Additional Panelists:

Joseph DeCourcey, Health Products Regulatory Authority, Ireland

Ilona Reischl, *Austrian Agency for Health and Food Safety GmbH (AGES)*

12:15-13:15 Topaz 1 (2nd Floor)

Lunch

Presentation type: IP - In Person
CGTP Europe Symposium

13:15-15:00 The Ballroom (2nd Basement Level)

Plenary Session 6 - Regulatory Considerations in Cell and Gene Therapy: Navigating through Challenges, Advancements and Global Convergence

Kowid Ho, Petra Minder
Presentation type: IP - In Person
CGTP Europe Symposium

Cell and gene therapies (CGTs) represent a transformative shift in modern medicine, offering potential cures for previously untreatable genetic disorders, cancers, and other conditions. The regulatory landscape for CGTs is complex and globally evolving, posing both significant challenges and unique opportunities for all stakeholders. This session will provide an overview of the current regulatory framework governing CGTs, with a focus on harmonization activities of international guidelines.

In this session, we will hear on global efforts made to support cell and gene therapy products to enable timely and safe access of effective therapies to patients. It will include regulatory updates and forward-looking discussion on the future of CGT regulation, emphasizing the need for innovative policies that balance patient access with robust quality, safety and efficacy standards.

Panelists:

Julia Djonova, *Swissmedic*

Veronika Jekerle, *European Medicines Agency (EMA)*

Olga Kolag-Robin, *European Pharmacopoeia Department, EDQM*

Ilona Resichl, *Austrian Agency for Health and Food Safety GmbH (AGES)*

Wala Turkistani, *Saudi Food and Drug Authority (SFDA)*

15:00-15:10 The Ballroom (2nd Basement Level)

Closing Remarks & Invitation to CGTP Europe 2026

Christiane Niederlaender
Presentation type: IP - In Person
CGTP Europe Symposium

15:10-15:25 Foyer Ballroom (2nd Basement Level)

Farewell Break

Presentation type: IP - In Person
CGTP Europe Symposium

We invite you to pick up some light refreshments to take with you as you leave. Thank you for being part of the inaugural CGTP Europe Symposium!