CGTP Summit 2024

Schedule

Monday, 10 June, 2024

07:15-08:30 Foyer A-C

Continental Breakfast

Presentation type: IP -In Person

Breakfast will be available until 9:00 AM Eastern

07:15-08:30 Foyer A-C

Registration

Registration is open until 17:00 Eastern in the Foyer C alcove

08:30-08:50 Salons A-C

CASSS Welcome & CGTP Summit 2024 Introduction

Session Chairs: Deep Shah
Presentation type: Live Streamed
CGTP Summit Oral

Advancing Comparability Understanding of Cell-based Medicinal Products

Despite early success of adoptive cell therapies and AAV-based gene therapies, it is quite evident that bringing such advanced medicines to the market has proven to be significantly challenging, most importantly due to CMC concerns. Amongst many challenges faced by CGT developers, comparability has continuously emerged as a recurring and inevitable hurdle due to the poorly understood and characterized nature of CGT products. During the first edition of the CGTP Summit in 2023, comparability considerations and current industry practices were discussed focusing on AAV-based gene therapies and CAR T-cell therapies. Reflecting on past success, the summit will continue the discussions on comparability with a focus on cell-based therapies.

Cell-based medicinal products are inherently one of the most complex therapeutic modalities developed so far. Due to live cells and inherent variability from where they are sourced and stage of development, cell-based medicinal products have proven to be difficult to characterize and standardize with currently available analytical methodologies. This summit will showcase presentations from the CGT industry and hold interactive panel discussions to tackle some of the challenges seen with conventional comparability strategies for cell-based products. In this summit, we will focus on somatic cell products and cell-based gene therapy products.

Finally, this summit will feature an expert regulatory panel comprised of regulators from key regulatory agencies. To that effect, this summit will discuss the recently issued FDA draft guidance on manufacturing changes and comparability for human cell and gene therapy products. The purpose of this panel is to openly discuss the common comparability challenges, to engage in a productive dialogue about mitigating those challenges and opportunities for harmonization between regulatory agencies.

08:50-10:15 Salons A-C

Session I - Autologous Cell-based Therapies

Session Chairs: Bita Badiei, Rob McCombie, Deep Shah
Presentation type: Live Streamed
CGTP Summit Oral

Autologous Cell-Based Medicinal Products

Comparison of new manufacturing processes against established ones is crucial for ensuring the quality, safety, and efficacy of biotherapeutic products. The assessment of comparability for Chemistry, Manufacturing, and Controls (CMC) changes becomes particularly paramount in the pharmaceutical industry when transitioning from earlier to newer production methods. Autologous cell-based medicinal products (referred to autologous products herein), derived from a patient's own cells, present unique challenges when it comes to evaluating changes in CMC parameters due to their inherent variability and intricacies in manufacturing.

This abstract highlights the significance of an autologous session dedicated to discussing comparability assessment strategies for CMC changes in the context of evolving manufacturing processes. Such sessions serve as platforms for industry experts, regulators, and stakeholders to collaborate, share insights, and develop standardized approaches that ensure the safety and efficacy of autologous products.

This session's main objectives include outlining best practices for characterizing the critical quality attributes (CQAs) of autologous products, establishing robust comparability protocols, and addressing regulatory expectations for CMC changes in autologous therapies. Participants will delve into the complexities of autologous product manufacturing and explore analytical methodologies that can effectively capture the nuances of these personalized treatments.

Key topics to be covered during the session may include the identification of critical process parameters (CPPs) for autologous products, risk assessment strategies for CMC changes, and the utilization of advanced analytical techniques such as mass spectrometry, next-generation sequencing, and bioinformatics for comprehensive comparability evaluation. Case studies illustrating successful comparability assessments for autologous products will be presented to showcase real-world applications of the discussed methodologies.

In conclusion, the autologous session focusing on comparability assessment for CMC changes represents a critical initiative in advancing the field of personalized medicine. By fostering collaboration, innovation, and regulatory alignment, this session aims to drive the development of robust CMC strategies that support the continued advancement and commercialization of autologous products while upholding stringent quality and safety standards.

Session Speakers:

Comparability Considerations for In-Licensing an Early-Stage Academic Program
Annie Chiu, *CARGO Therapeutics, Inc.*

Comparability Study Considerations in the Development and Approval of AMTAGVI™ (Lifileucel)
Arvind Natarajan, *IOVANCE Biotherapeutics, Inc.*

Approaches for Establishing Comparability for Cell Therapy Products
Nitin Agarwal, *Kite Pharma*

Analytical Comparability for Autologous CAR-T Products
Hai Yue, *Bristol-Myers Squibb Company*

10:15-10:45 Foyer A-C

Networking Break

Presentation type: IP -In Person

10:45-12:00 Salons A-C

Session I - Panel Discussion - Questions & Answers

Session Chairs: Bita Badiei, Rob McCombie, Deep Shah
Presentation type: Live Streamed
CGTP Summit Oral

Additional Panelists:

Tal Salz, *Dark Horse Consulting*

12:00-13:00 Brookside A&B (Lower Level)

Lunch

Presentation type: IP -In Person

13:00-14:05 Salons A-C

Session II - Allogeneic Cell-based Therapies

Session Chairs: Diane Blumenthal, Margarida Menezes Ferreira, KR Poudel
Presentation type: Live Streamed
CGTP Summit Oral

Allogeneic Cell-based Therapies

Allogeneic cell therapy products are long sought for expanding the success of cell based therapeutic approaches. Off the shelf products, manufacturable in relatively large quantities could significantly increase access for patients while reducing the cost of manufacturing for companies. The complexities of commercially manufacturing allogeneic cell products remain challenging. Manufacturing changes are needed inevitable for the continuous process improvement. Allogeneic cell therapy has the added challenge of the need to periodically replace the donor starting materials. This is due to limitations in batch size based on the expansion of a single donor and hence requiring robust comparability evaluations. In this session, we will discuss the complexities of implementing a comparability strategy during manufacturing for allogeneic cell-based products. Presentations will be based on case studies in the clinical and commercial space involving healthy donor and induced pluripotent stem cells (iPSC) derived products. Panel discussions will center around phase appropriate strategies to implementing changes required to optimize manufacturing and donor replacement for allogeneic products.

Session Speakers:

Analytical Control Strategy and Multi-Level Comparability for Genome Editing Components for Cell Therapy Products
Julien Camperi, *Genentech, a Member of the Roche Group*

Regulatory Strategies & Comparability Assessment For Allogenic Cell Therapy Products With Variable Donor Starting Material
Sangeetha Prakash, *Takeda Pharmaceutical Company Limited*

Case Study in Comparability for an iPSC-Derived, Genome-Edited Cell Therapy Product
Jennifer Dashnau, *Century Therapeutics, Inc.*

14:05-14:35 Foyer A-C

Networking Break

Presentation type: IP -In Person

14:35-15:50 Salons A-C

Session II: Panel Discussion - Questions & Answers

Session Chairs: Diane Blumenthal, Margarida Menezes Ferreira, KR Poudel
Presentation type: Live Streamed
CGTP Summit Oral

Additional Panelist:

Barbara Bonamassa, *Italian Medicines Agency*

15:50-16:00 Foyer A-C

Mini Break

16:00-17:30 Salons A-C

Featured Session - Comparability Regulatory Panel

Session Chairs: Bita Badiei, Margarida Menezes Ferreira, Deep Shah
Presentation type: Live Streamed
CGTP Summit Oral

Engaging Global Regulators in Discussion on Comparability for Cell-based Medicinal Products: Industry Challenges and a Path Towards Harmonized Solutions

In a rapidly evolving landscape of cell-based medicinal products, ensuring product comparability during manufacturing changes is a critical aspect of maintaining product quality, safety, and efficacy at any stage of product development. To gain insights and perspectives from global regulatory bodies on this topic, a regulatory panel is organized inviting key regulators such as the Food and Drug Administration (FDA), Pharmaceuticals and Medical Devices Agency (PMDA), Health Canada and Swedish Medical Products Agency. The primary objective of this panel is to openly discuss common comparability challenges seen by regulators, engage in a productive dialogue on mitigating these challenges, and explore opportunities for regulatory harmonization.

The panel will focus on key challenges witnessed by global regulatory bodies in assessing comparability studies for cell-based medicinal products, expectations from global regulatory bodies including considerations for establishing comparability, managing manufacturing changes, and ensuring product quality and safety. Regulators will also share their perspectives on the newly issued FDA draft guidance on comparability for cell and gene therapy products, highlighting areas of consensus and divergence with their own regulatory frameworks.

One of the key discussion points would be recognition of the need for greater harmonization and convergence among regulatory agencies to support the development and approval of cell-based medicinal products on a global scale. By aligning their expectations and requirements, regulators can enhance the predictability and consistency of regulatory reviews, reduce duplication of efforts for manufacturers, and ultimately expedite patient access to innovative therapies.

Overall, this panel aims to provide a valuable platform for regulators, industry stakeholders, and experts to come together, exchange insights, and collaborate toward a more harmonized regulatory approach for understanding comparability for cell-based medicinal products. The insights and recommendations generated through this dialogue have the potential to shape future regulatory guidance and facilitate the development and commercialization of advanced therapies for patients worldwide.

Panelists:

Andreea Barbu, Swedish Medical Products Agency

Yoshiaki Maruyama, *PMDA*

Christopher Storbeck, *Health Canada*

Andrew Timmons, *CBER, FDA*

17:30-17:45 Salons A-C

Closing Remarks & Invitation to CGTP Summit 2025

Session Chairs: Deep Shah
Presentation type: Live Streamed
CGTP Summit Oral

17:45-19:00 Brookside A&B (Lower Level)

CGTP Summit Networking Reception

Presentation type: IP -In Person

Mix and mingle with fellow attendees to celebrate the completion of the CGTP 2024 Summit!