Cell and Gene Therapy Products 2024

Schedule

Tuesday, 11 June, 2024

06:00-07:15

WATCH NOW: Utilizing HiBiT Targeted Cell Killing Bioassays To Validate Cell Therapy and mAb-Mediated Immunotherapy Potency

This technical Seminar will be available to watch on demand 24/7 during the Symposium. Click on this session to access the presentation video.
NOTE: If prompted to login to view the video, enter your CASSS member credentials.

Presented by: Promega Corporation
Click to Watch Video

06:00-07:15

WATCH NOW: Limiting Off-Target Effects of CRISPR-Based Products- Guide RNA Sequencing by NGS

This technical Seminar will be available to watch on demand 24/7 during the Symposium. Click on this session to access the presentation video.
NOTE: If prompted to login to view the video, enter your CASSS member credentials.

Presented by: MilliporeSigma
Click to Watch Video

07:15-08:30 Foyer A-E

Continental Breakfast

Breakfast will be available until 9:00 AM Eastern

07:15-08:30 Foyer A-E

Registration

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

08:30-08:50 Salon D

CASSS Welcome & CGTP 2024 Introduction

Session Chairs: Kathleen Francissen, Andrew Weiskopf
Presentation type: Live Streamed
CGTP Symposium Oral

08:50-10:30 Salon D

Plenary Session 1 - Genetic Modification Technologies for Cell-based Gene Therapies

Session Chairs: Cynthia Riggins, Madhu Siluveru, Andrew Weiskopf
Presentation type: Live Streamed
CGTP Symposium Oral

In addition to the now well-known CAR T (chimeric antigen receptor T cells) and HSC (CD34+ hematopoietic stem cells) cell-based gene therapies, there are several other modes of gene modification as well as cell types already approved and on the horizon. CAR T and HSC genetically engineered therapeutics have typically relied upon lentiviral or gamma-retroviral vector transduction to introduce gene(s) of interest but other methods of gene modification can introduce, replace or inactivate genes in the cells of interest. Recently approved CASGEVY is the first approved therapy to rely on CRISPR/Cas9 gene-editing to modify HSCs. More complex genetically engineered cell therapies may require inclusion of multiple modes of gene modification in the same cell to ensure the desired product attributes. Challenges include effective delivery methods, effective targeting, effective cell selection and expansion, and development of appropriate analytical methods to assess more complex and novel genetic engineering approaches.

Session Speakers:

Ingenui-T, a Rapid Autologous Chimeric Antigen Receptor (CAR)-T Manufacturing Solution Using Whole Blood, for Treatment of Autoimmune Disease
Sunetra Biswas, *Kyverna Therapeutics, Inc.*

CMC Development of PM359, a Prime Edited Hematopoietic Stem Cell Therapy for the Treatment of P47phox-Deficient Chronic Granulomatous Disease (CGD)
Barrett Nehilla, *Prime Medicine, Inc.*

Enabling the Advancement of Cell Therapies with Epigenetic Editing
Nathan Yee, *Tune Therapeutics*

Additional Panelist:

Zhaohui Ye, *CBER, FDA*

10:30-11:15 Foyer A-E

Networking Break

Presentation type: IP -In Person

10:30-11:15 Brookside A&B (Lower Level)

First Time Attendee Networking Event

Presentation type: IP -In Person

First-time attendees and New Members are invited to mingle and meet others within the CASSS community

11:15-12:25 Salon D

Keynote Presentation - FDA Cell and Gene Therapy Regulatory Update

Session Chairs: Alexandra Beumer Sassi, Kathleen Francissen
Presentation type: Live Streamed
CGTP Symposium Oral

Dr. Nicole Verdun, *CBER, FDA*

Dr. Verdun (ver-done) received her undergraduate degree from Duke University and her medical degree from the University of Chicago Pritzker School of Medicine. She then completed a Pediatrics Residency at Children's Memorial Hospital-Northwestern University and a Pediatric Hematology-Oncology Fellowship at the Children's Hospital of Philadelphia (CHOP). After practicing as a hematologist with a focus on hemostasis and thrombosis, Dr. Verdun joined FDA in 2012, first in the Office of Hematology Oncology Products as a medical officer and a liaison for sickle cell therapeutics and anticoagulants, and then Therapeutic Biosimilars. She was appointed as the Deputy Director of the Office of Blood Research and Review in the Center for Biologics Evaluation and Research (CBER) in October 2016 and was promoted to Office Director in 2018. In 2023, Dr. Verdun was selected as the Super Office Director of the Office of Therapeutic Products, overseeing 6 Offices dedicated to the regulation and approval of Cell and Gene therapies in the United States. She overseas both a research and regulatory portfolio in CBER. She is also on staff at Children's National Medical Center.

12:25-13:55 Foyer A-E

Hosted Lunch

Presentation type: IP -In Person

Lunch provided in conjunction with the technical seminar talk.

Additional seating will be available on the Veranda.

12:50-13:35 Salon D

Rapid and Precise Potency Assays with Label-Free Laser Force Cytology™ | Presented by LumaCyte, Inc.

Presentation type: Live Streamed
CGTP Technical Seminar

Rapid and Precise Potency Assays with Label-Free Laser Force Cytology™

Presented by:
Colin Hebert, *University of Maryland*

13:55-15:35 Salon D

Parallel Session 2 - How to Control Off Target Genotoxicity for Genome and Epigenome Editing Medicines

Session Chairs: Isabella Palazzolo, KR Poudel, Jiwen Zhang
Presentation type: Live Streamed
CGTP Symposium Oral

Genome editing is a tool that enables a new generation of medicines by directly targeting with high precision the genetic cause of a disease. It also allows for immune evasion and prevents microenvironment responses: concepts vital to more potent, off the shelf, cell, and gene therapies. To ensure safe and efficacious administration of gene edited products to patients, minimizing unintended effects is paramount. This session will review approaches to characterize off target genotoxicity at different stages of development for different class of genome/epigenome editing medicines. Current regulatory standards will be discussed, and lessons learned may be applied to relieve bottlenecks for future therapies.

Session Speakers:

Overview of Current Genomic Analytical Tools to Enable Advancement of Investigational in vivo Genome Editing Products into Clinical Studies
Jessica Seitzer, *Intellia Therapeutics, Inc.*

Analytical Control Strategies for Ensuring Genomic Integrity in CAR-T Cell Therapies
Athea Vichas, *Bristol-Myers Squibb Company*

Off-Target Analysis of Genome Editing Products
Yongwook Choi, *CBER, FDA*

Additional Panelist:

Jennifer Dashnau, *Century Therapeutics, Inc.*

13:55-15:35 Salons A-C

Parallel Session 3 - Viral Vector-based Gene Therapy Products

Session Chairs: Svetlana Bergelson, Alexandra Beumer Sassi, Christopher Storbeck
Presentation type: Live Streamed
CGTP Symposium Oral

The field of Cell and Gene Therapy has witnessed expanding numbers of regulatory approvals in recent years. Of the approved products, a significant number are viral vector-based, either employing the viral vector for the delivery of a therapeutic transgene to cells ex vivo during the manufacturing process (e.g., LVV for modified cells), or via direct administration to the patient (e.g., AAV-based gene therapy product) to achieve a therapeutic effect. Given the rate of recent approvals, coupled with technological advances in molecular sciences, and partly driven by evolving regulatory expectations around controls, there is an evolution of product understanding, particularly in the application of analytical methods used to support process development and characterize or monitor product attributes. The aim of this session is to examine these changes in greater detail, including analytical method choices for optimal assessment of product characteristics, attribute criticality and monitoring. This may also include innovative approaches to determination of potency, including the use of surrogate metrics in the context of a potency assay matrix. A greater scientific understanding of viral vector-based Gene Therapy product attributes will improve the overall quality of these important therapeutic products.

Session Speakers:

LYFGENIA's Journey: Lessons Learned in the Development of LVV-based Cell & Gene Therapy Products
Marc d'Anjou, *bluebird bio, Inc.*

Potency Method Development, Bridging and Control Strategies for AAV Gene Therapy
Ping Carlson, *Passage Bio*

Gene Therapy Product Analytics: Potency Method Validation and Optimization
Wandong Zhang, *BioMarin Pharamceutical Inc.*

Additional Panelist:

Jessica Chery, *CBER, FDA*

15:35-16:05 Foyer A-E

Networking Break

Presentation type: IP -In Person

16:05-17:05 White Oak (Lower Level)

Roundtable Discussions - Session 1

Presentation type: IP -In Person
CGTP Roundtable

Table 1- ATMP Raw / Starting Material Risk Assessments, Control Strategy and Regulatory Expectations

Table 2 - Industry Feedback on FDA Comparability Guidance

Table 3 - FDA Draft Guidance on Potency Assurance

Table 4 - Detection of Particulates / Visual Inspection of CGT Products

Table 5 - Material Classification for Gene Editing Components

Table 6 - Creative Approaches to Stability Assessment of Frozen Products

Table 7 - Non-Viral Delivery Options for Cell-based Gene Therapy Products

Table 8 - Process Validation Approaches for Complex Modalities

Table 9 - Platform Applications Outside of AAV Gene Therapy Field

Table 10 - Review of Different Aspects of BIO's Third Annual Report on Measuring Diversity in the Biotech Industry

17:05-19:05 Veranda

CGTP 2024 Welcome Reception

Presentation type: IP -In Person

Join us on the outdoor veranda to celebrate the start of CGTP 2024!

Wednesday, 12 June, 2024

06:00-07:30

WATCH NOW: Utilizing HiBiT Targeted Cell Killing Bioassays To Validate Cell Therapy and mAb-Mediated Immunotherapy Potency

This technical Seminar will be available to watch on demand 24/7 during the Symposium. Click on this session to access the presentation video.
NOTE: If prompted to login to view the video, enter your CASSS member credentials.

Presented by: Promega Corporation
Click to Watch Video

06:00-07:30

WATCH NOW: Limiting Off-Target Effects of CRISPR-Based Products- Guide RNA Sequencing by NGS

This technical Seminar will be available to watch on demand 24/7 during the Symposium. Click on this session to access the presentation video.
NOTE: If prompted to login to view the video, enter your CASSS member credentials.

Presented by: MilliporeSigma
Click to Watch Video

07:30-08:30 Foyer A-E

Continental Breakfast

Presentation type: IP -In Person

Breakfast will be available until 9:00 AM Eastern

07:30-08:30 Foyer A-E

Registration

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

08:30-09:15 Salon D

Hot Topic Session - ICH Cell & Gene Therapy Discussion Group Updates

Session Chairs: Kevin Okimura
Presentation type: Live Streamed
CGTP Symposium Oral

The International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) brings together regulatory authorities and representatives from the pharmaceutical industry from across the globe to discuss a range of technical and scientific considerations for medicinal products and to develop ICH guidance. In this manner, ICH provides a harmonized approach to ensure that safe, effective and high-quality medicines are developed, registered, and maintained in a resource efficient manner whilst meeting high standards. As cell and gene therapy products have become ever more prevalent in the therapeutic landscape, the specific considerations applicable to such products are in need of specific recommendations for revisions to existing ICH guidelines and/or new guideline development. The ICH Cell and Gene Therapies Discussion Group (CGT DG) has been formed, and the Remit was endorsed by the ICH Management Committee in May 2023. In this session we will hear about the scope of ICH CGT DG activities and their progress toward targeted deliverables.

Session Speaker:

ICH CGT DG: Progress Toward Delivering a Strategic Roadmap for ATMPs
Kathleen Francissen, *Genentech, A Member of the Roche Group*

Additional Panelist:

Melanie Eacho, *CBER, FDA*

09:15-10:55 Salon D

Plenary Session 4 - Potency Assays for Gene Edited Products

Session Chairs: Rob McCombie, Margarida Menezes Ferreira, Marcos Timon
Presentation type: Live Streamed
CGTP Symposium Oral

Measurement of the biological activity is crucial for lot release, stability and any comparability exercise of all biological medicinal products. Potency assays should be implemented as early in clinical developments as possible because correlation with clinical performance could help in future identification of sub potent batches. However, development of an appropriate potency assay is often challenging, especially for products with complex or not well-defined mechanisms of action.
Gene editing (GE) tools are used to achieve targeted modifications of genome sequences resulting in gene inactivation, modifications or insertions at specific locations, either in vivo or ex vivo. This allows the generation of very diverse cell phenotypes that can be used in the clinic to treat a high array of diseases. Thus, developing specific potency assays for gene edited medicinal products can have additional complications.

This session will aim to review FDA's recently released draft guideline on potency assurance for cell and gene therapy products. It will also cover issues to be considered when designing a potency assay for ex vivo genome-edited products or products that are intended to edit the genome in vivo, considering the different GE tools and possible mechanisms of actions. The session will also present the particular challenges of developing a potency assay for a GE product intended to knock-out a gene.

Session Speakers:

Potency Assurance for Cellular and Gene Therapy Products
Andrew Byrnes, CBER, FDA

Potency Development for an in Vivo AAV Gene Editing Therapy
Debaditya Bhattacharya, *ElevateBio*

Approaches to Potency Assays for CRISPR Genome Editing Therapeutics
Kristy Wood, *Intellia Therapeutics, Inc.*

Additional Panelist:

Keith Wonnacott, *Lexeo Therapeutics, Inc*.

10:55-11:25 Foyer A-E

Networking Break

Presentation type: IP -In Person

11:25-12:25 White Oak (Lower Level)

Roundtable Discussions - Session 2

Presentation type: IP -In Person
CGTP Roundtable

Table 1 - ATMP Raw/starting Material Comparability and Change Management

Table 2 - Industry Feedback on FDA Comparability Guidance

Table 3 - FDA Draft Guidance on Potency Assurance

Table 4 - ICH M4Q CTD Structure: Considerations for ATMPs (Industry Experience and Challenges)

Table 5 - Facility Expansion - Scale-out (as opposed to scale up) is much more common in the CGTP space. What CMC considerations should be taken into account when scaling out for commercial approval?

Table 6 - GMO Environmental Risk Assessments and the Application Process

Table 7 - Advancing Stem Cell Therapy Development: Overcoming Challenges and Expanding Horizons

Table 8 - QbD Tools for MAbs Don't Quite Work for CGT Products

Table 9 - ATMPs and Companion Diagnostics - Development Challenges

Table 10 - Best Practices in CMC Decision-Making (Clinical Phase) by Applying Inclusive Principles in Team Build

12:25-13:40 Foyer A-E

Lunch

Presentation type: IP -In Person

Lunch provided in conjunction with the technical seminar talk

Additional seating will be available on the Veranda.

13:40-15:20 Salon D

Plenary Session 5 - Stem Cell-derived Medicinal Products

Session Chairs: Christiane Niederlaender, Kevin Okimura, Heli Suila
Presentation type: Live Streamed
CGTP Symposium Oral

Stem cell-derived medicines have seen somewhat of a renaissance over the last year, following a difficult period in terms of regulatory approval. New stem cell therapies, including many iPSC-based therapies, are now under development for a variety of indications and more traditional regenerative approaches have seen regulatory approval. Despite this, there remain difficult challenges for stem cell-derived products, including product characterization and potency assay development for mechanisms of action that often must be postulated fairly widely.
This session will present case studies by stakeholders that are successfully navigating this field, together with viewpoints from regulators.

Speaker:

Releasing Criteria and Potency Assay Development of an iPSC-Derived Neural Progenitor Cell Product Targeting CNS Diseases
Jing Fan, *Hopstem Biotechnology Inc.*

Navigating the Regulatory Path: Clinical Translation of iPSCs
Saravanan Karumbayaram, *CBER, FDA*

Cellular Engineering to Address Manufacturing and Regulatory Concerns
Todd McDevitt, *Genentech, a Member of the Roche Group*

Panelist:

Andreea Barbu, *Swedish Medical Products Agency*

15:20-15:50 Foyer A-E

Networking Break

Presentation type: IP -In Person

15:50-16:55 Salon D

Plenary Session 6 - Fireside Chat: Evolution of Cell and Gene Therapy

Session Chairs: Alexandra Beumer Sassi, Bruce Thompson
Presentation type: Live Streamed
CGTP Symposium Oral

Join us for an engaging fireside chat delving into the revolutionary landscape of cell and gene therapy. This interactive session will feature esteemed experts at the forefront of the industry, offering insights into the latest advancements, challenges, and future prospects shaping this rapidly evolving field.

From pioneering research to transformative clinical applications, our panelists will explore the profound impact of cell and gene therapies on human health. Discussions will encompass breakthrough technologies, innovative treatment modalities, regulatory considerations, and the pivotal role of collaboration in driving progress.

Through candid conversations and real-world experiences, attendees will gain invaluable perspectives on navigating the dynamic landscape of cell and gene therapy. Whether you're a seasoned professional, researcher, regulatory professional, or enthusiast, this fireside chat promises to ignite curiosity, foster connections, and inspire the next wave of innovation in biomedicine.

Panel Members:

Michael Lehmicke, *Alliance for Regenerative Medicine*

Sarah Pitluck, *SP Consulting*

Joseph Tarnowski, *CHO Plus, Inc.*

16:55-18:15 Foyer A-E

CGTP 2024 Exhibitor Reception

Presentation type: IP -In Person

Join us in the Foyer to mix and mingle with our exhibitor partners!

Thursday, 13 June, 2024

06:00-07:30

WATCH NOW: Utilizing HiBiT Targeted Cell Killing Bioassays To Validate Cell Therapy and mAb-Mediated Immunotherapy Potency

This technical Seminar will be available to watch on demand 24/7 during the Symposium. Click on this session to access the presentation video.
NOTE: If prompted to login to view the video, enter your CASSS member credentials.

Presented by: Promega Corporation
Click to Watch Video

06:00-07:30

WATCH NOW: Limiting Off-Target Effects of CRISPR-Based Products- Guide RNA Sequencing by NGS

This technical Seminar will be available to watch on demand 24/7 during the Symposium. Click on this session to access the presentation video.
NOTE: If prompted to login to view the video, enter your CASSS member credentials.

Presented by: MilliporeSigma
Click to Watch Video

07:30-08:30 Foyer A-E

Continental Breakfast

Presentation type: IP -In Person

Breakfast will be available until 9:00 AM Eastern

08:00-08:30 Foyer A-E

Registration

Registration is open until 12:00 Eastern in the Foyer C alcove

08:30-10:10 Salon D

Plenary Session 7 - Streamlining AAV Vector-based Gene Therapy Development Through the Utilization of Manufacturing and Analytical Platform Approaches

Session Chairs: Diane Blumenthal, Taro Fujimori, Zenobia Taraporewala
Presentation type: Live Streamed
CGTP Symposium Oral

Our ability to treat diseases previously untreatable through the delivery of adeno-associated virus (AAV)-based gene therapies has been a tremendous advancement in pharmaceutical development. The ability to bring promising products in the pipeline to the clinic and ultimately to the market is hampered by inefficiencies in the development of the manufacturing processes and analytical tools, and challenges with scalability. This is largely attributable to the biological complexity of the products, the optimization needed every time a new AAV serotype capsid vector is being developed for gene therapy, and limited understanding of the structure-function relationship that guides optimization of the manufacturing process and analytical testing

The diseases that necessitate the implementation of gene therapy to treat patients are often characterized by small patient populations. The number of these diseases is quite extensive leaving pharma companies faced with completing the extensive set of CMC requirements needed to support a regulatory filing for each new product. This is labor intensive and costly, often outstripping the ability of companies to recover the costs of bringing these products to market.

A platform approach can be developed for a class of products (e.g., AAV vectors, lentivirus vectors) after manufacturers have gained considerable experience, and have developed a production strategy based on similar manufacturing processes/unit operations. Platform-based approaches have been used successfully to streamline manufacturing of well characterized biologics like mAbs to reduce the CMC burden and cost.

The goal of the session is to further our understanding of how manufacturers have streamlined the development of AAV-based gene therapy products through the utilization of platform approaches. The session will include presentations from developers and CDMOs that have adopted platform approaches to decrease the CMC burden and bring promising therapies through the pipeline (in early development) to the clinic and through late phase development and commercialization to patients. The objective of the session is to share experiences, strategies and regulatory feedback that facilitated the development of platform approaches for AAV based gene therapie

Session Speakers:

Balancing Flexibility and Standardization of AAV Manufacturing Processes
Mercedes Segura, *ElevateBio*

Gaining Momentum from an AAV Gene Therapy Manufacturing Platform
Joe Peltier, *BioMarin Pharamceutical Inc.*

Shoring up Your Platform: Applying Knowledge Management and Regulatory Considerations to Gene Therapy Development
Shannon Holmes, *Ultragenyx Pharmaceutical Inc.*

Additional Panelists:

Andrew Harmon, *CBER, FDA*

10:10-10:40 Foyer A-E

Networking Break

Presentation type: IP -In Person

10:40-12:25 Salon D

Plenary Session 8 - Global Regulatory Updates and Panel Discussion

Session Chairs: Kathleen Francissen, Allison Wolf
Presentation type: Live Streamed
CGTP Symposium Oral

The cell and gene therapy field spans an enormous range of product types and technologies with the potential to provide curative treatments for a wide range of serious diseases. This tremendous potential has led to thousands of investigational treatments entering clinical development and many new technologies being applied to manufacture and control these complex products. Global health authorities have had to add capabilities and capacity to support the emergence of this diverse and complex portfolio of cell and gene therapy products.
In this session, we will hear from regulatory leaders from health authorities across the world about the work being done to support cell and gene therapy products and ensure timely and safe access of effective therapies to patients. Since many cell and gene therapy products are developed in rare diseases, the benefit of harmonized guidelines for global development is of particular relevance. Multiple activities on different levels are ongoing on the level of WHO and ICH to bring harmonized requirements and expectations for developers of these products. Initiatives are also being created to allow for coordination and work share across multiple health authorities to review a product. These efforts have the potential to bring efficiency to the review process for sponsors and health authorities allowing parallel authorization and facilitating access to patients around the world. Another opportunity that is gaining momentum with regulatory agencies and industry is the potential application of regulatory reliance mechanisms to allow for broader reach of a product into additional markets based on the robust review from a major health authority such as EMA, FDA, or PMDA. In this panel, we will discuss the activities being considered to enable these key levers such as workshare and reliance pathways that are key to maximizing the potential of these advanced therapies.

Panelists:

Yoshiaki Maruyama, *PMDA*

Steven Oh, *CBER, FDA*

Ilona Reischl, *Austrian Medicines and Medical Devices Agency*

12:25-12:40 Salon D

Closing Remarks and Invitation to CGTP 2025

Session Chairs: Alexandra Beumer Sassi
Presentation type: Live Streamed
CGTP Symposium Oral