Cell and Gene Therapy Products 2023 / CGTP Summit 2023

Schedule

Monday, 26 June, 2023

07:30-08:30 Foyer A-C

Continental Breakfast

Breakfast will be available until 9:00 AM Eastern

07:30-08:30 Foyer A-C

Registration

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

08:30-09:00 Salons A-C

CASSS Welcome & CGTP Summit 2023 Introduction

Session Chairs: Kathleen Francissen
Presentation type: H - Hybrid
CGTP Summit Oral

Comparability assessments are necessary for life cycle management of all biological products, including cell and gene therapies (CGT), and are conducted to ensure that manufacturing changes do not adversely impact product quality, safety, or efficacy. The complexity and diversity of CGT product modalities can pose considerable challenges to the usual approaches, which are guided by the principles in ICH Q5E. In this summit, we will discuss a range of CGT modalities and some new concepts in comparability required for certain CGT products.

09:00-10:40 Salons A-C

Session 1: Comparability Considerations for Viral Vector-Based Gene Therapies

Session Chairs: Diane Blumenthal, Leslie Nash, Zenobia Taraporewala
Presentation type: H - Hybrid
CGTP Summit Oral

Viral vector-based gene therapy products are the fastest growing segment of the gene therapy field, with adeno-associated virus (AAV)-based vectors dominating the landscape. The possibility of a one-time injection, and a relatively low-risk safety profile, increased demand for application of AAV-based vectors for the treatment of rare, serious, and life-threatening diseases. As sponsors scale-up and scale-out to meet the increasing demands and implement changes to optimize manufacturing process productivity and performance, it is imperative to effectively design and carry out comparability studies.

A well thought out comparability study is critical for demonstrating that the product attributes indicative of quality, safety and efficacy are similar pre and post change. Data derived from a robust comparability study also ensures that the preclinical and clinical data derived pre-change can be leveraged for future clinical studies and/or for supporting licensure without the need for repeat studies with post change product to demonstrate comparability. Implementing manufacturing changes to meet the demands of clinical supply and commercialization has presented challenges when evaluating comparability of the pre- and post-change product. For example, transition from an adherent to a suspension process may increase output while simultaneously impacting process- and product-related impurities. Limitations in the current understanding of how structure impacts function thus limits our ability to know which product quality attributes are important when making manufacturing changes. Inadequate comparability studies can lead to delays and misalignment of the clinical and CMC development and sometimes to the stalling of promising clinical programs.

This session will include case studies that illustrate the challenges and the strategies used for assessing comparability at different stages of development of viral vector-based gene therapies, and a discussion of the key considerations in the design of comparability studies.

Session Speakers:

Case Study: Comparability between AAV manufactured by Triple-plasmid transfection/HEK293 and Double-baculovirus/Sf9 Processes
Garrett Daniels, *Prevail Therapeutics*

Analytical Comparability – Evolution of Analytical Methods & Case Study for Late-Stage Change
Phillip Ramsey, *Sangamo Therapeutics*

De-Risking Analytical Comparability for an AAV Manufacturing Process Change in Late Development
Taro Fujimori, *Ultragenyx*

Assessing Comparability: It's More Than Just Numbers
Julia O'Neill, *Direxa Consulting*

10:40-11:10 Foyer A-C

Networking Break

11:10-12:25 Salons A-C

Session 1: Panel Discussion - Questions & Answers

Presentation type: H - Hybrid
CGTP Summit Oral

Additional Panelists:

Anurag Sharma, *CBER, FDA*

12:25-13:55

Lunch

13:55-15:10 Salons A-C

Session 2: Comparability Considerations for Cell-Based Therapies

Session Chairs: Barbara Bonamassa, KR Poudel, Deep Shah
Presentation type: H - Hybrid
CGTP Summit Oral

Comparability assessments for cell-based therapies are challenging due to complex manufacturing processes, inherent variability of cell starting materials, and evolving understanding of product quality attributes and analytical technologies. Many of these products are made-to-order for a specific patient, and materials are limited for any type of analytical evaluation. Early generation of the first autologous cell therapy products applied fast-to-market CMC approaches to expedite the delivery of life-saving treatments to patients.

As the focus of the field shifts towards optimized manufacturing, better understanding of CQAs, and superior product performance, well designed comparability strategies can streamline cell therapy product development, reduce the costs of manufacturing, and provide faster access to these important medicines. Limited characterization and understanding of the relationship between the product and its function further complicate the ability to rigorously compare pre- and post-change product.

In this session, comparability strategies used with commercial and investigational cellular therapy products will be presented as case studies. In addition, industry stakeholders and regulators will engage in discussions to address uncertainties in comparability including planning comparability studies during early and late stages of drug development, and challenges involved in showing clinical relevance. The relevance of the lessons learned to newer generations of autologous and allogeneic CAR-T cell products will be discussed. Discussions will also include gene editing and healthy donor considerations to maximize product and process consistency while ensuring product quality, safety, and efficacy.

Session Speakers:

Manufacturing Changes and Comparability for Human Cellular and Gene Therapy Products
Elizabeth Lessey-Morillon, *CBER, FDA*

Statistical Perspectives on Analytical Comparability Studies for Autologous Cell-based Therapies
Kedar Dave, *Bristol-Myers Squibb Company*

Challenges and Considerations for Allogeneic CAR T Therapy Comparability Studies
Mark DiMartino, *Allogene Therapeutics*

15:10-15:40 Foyer A-C

Networking Break

15:40-17:20 Salons A-C

Session 2: Panel Discussion - Questions & Answers

Presentation type: H - Hybrid
CGTP Summit Oral

Additional Panelists:

Margarida Menezes Ferreira, *Retired, INFARMED - National Authority of Medicines and Health Products, I.P.*

17:20-17:35 Salons A-C

Closing Remarks & Invitation to CGTP Summit 2024

Session Chairs: Kathleen Francissen
Presentation type: H - Hybrid
CGTP Summit Oral

17:35-19:05 Brookside A&B

CGTP Summit Networking Reception

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

Tuesday, 27 June, 2023

07:30-08:30 Foyer A-D

Continental Breakfast

Breakfast will be available until 9:00 AM Eastern

07:30-08:30 Foyer A-C

Registration

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

08:30-09:00 Salon D

CASSS Welcome & CGTP 2023 Introduction

Session Chairs: Svetlana Bergelson, Rob McCombie
Presentation type: H - Hybrid
CGTP Symposium Oral

09:00-10:00 Salon D

Keynote Presentation

Session Chairs: Kathleen Francissen, Rob McCombie
Presentation type: H - Hybrid
CGTP Symposium Oral

Keynote Speaker:

Gene Therapy Drug Development for Ultra-Rare Disease: Challenges & Opportunities
Becky Schweighardt, *Grace Science LLC*

10:00-10:45 Foyer A-D

Networking Break

10:00-10:45 Brookside A&B

New Member Networking Event

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

10:45-12:20 Salon D

Parallel Session 1: CMC Challenges with Ultra Rare Diseases

Session Chairs: Diane Blumenthal, Rob McCombie
Presentation type: H - Hybrid
CGTP Symposium Oral

Bringing treatment to those afflicted with an ultra-rare disease is complex. These products are encumbered with challenges ranging from high development costs with a limited ROI, to issues with executing and interpreting clinical trials with unusually small patient populations. Furthermore, the cost to generate the quantities of product required to support an approvable CMC package can be prohibitive for many organizations.

This session will address ways in which companies have tackled the CMC and regulatory challenges when bringing these life changing therapies to patients in need.

Session Speakers:

Manufacturing Challenges Limiting the Access to ATMPs
Ralf Altenburger, *F. Hoffmann-La Roche Ltd.*

Development of a Single Patient CRISPR Therapeutic
Richard Horgan, *Cure Rare Disease*

Additional Panelists:

Emmanuel Adu-Gyamfi, *CBER, FDA*
Becky Schweighardt, *Grace Science LLC*

10:45-12:20 Salons A-C

Parallel Session 2: Product Quality Testing for Individualized Medicines

Session Chairs: Svetlana Bergelson, Andreas Kuhn
Presentation type: H - Hybrid
CGTP Symposium Oral

With the significant progress in the development of individualized medicines, several issues with respect to their routine quality testing have emerged. As a new batch has to be tested for each patient, an extensive set of quality attributes to be analyzed and elaborate testing methods can be a challenge on both number of samples that are needed and the time that is required for testing. The latter is especially true for verifying the sterility of the drug product. As batches for individualized medicines cannot be stocked and the medical needs often require fast treatment of the patients, standard sterility testing can become the limiting factor for the success of an individualized therapy. In addition, it has to be ensured that the assays will function independent of the patient-specific aspects of the final product.

In this session, we will discuss key challenges and opportunities with respect to product quality testing for Individualized medicines to enable the successful development of such therapies.

Session Speakers:

USP Evolving Position on Use of Rapid Microbial Methods
Huiping Tu, *United States Pharmacopeia*

Defining Microbial Control Strategies for Cell-free and Cell-based Individualized ATMP's
Friedrich von Witzingerode, *Genentech, a Member of the Roche Group*

Delivering Next Generation Cell Therapy Manufacturing Faster without Compromising Quality
Scott Nichols, *Kite, A Gilead Company*

Additional Panelists:

Elvira Argus, *CBER, FDA*
Bryan Silvey, *A2 Biotherapeutics, Inc.*

12:20-14:20 Foyer A-D

Lunch

Lunch provided in conjunction with the technical seminar talk

12:45-13:45 Salon D

Technical Seminar presented by MilliporeSigma

Presentation type: H - Hybrid
CGTP Technical Seminar

Ensuring the Virus Safety Profile of Your Product - Expectations and Recommendations From the Latest Update to ICH Q5A
Rebecca Bova, *MilliporeSigma*
Kathryn Martin Remington, *MilliporeSigma*

14:20-15:55 Salon D

Parallel Session 3: Lifecycle Approaches to Potency Assay Selection

Session Chairs: Bryan Silvey, Max Tejada
Presentation type: H - Hybrid
CGTP Symposium Oral

Potency testing is a critical part of the assessment and release of cell therapy products. These functional assays provide quantitative assessments of the biological activity of a cell or gene therapy product that are associated with that product's in vivo mechanism(s) of action (MOA). Current state-of-the-art analytical methods still rely mainly on in vitro assays including immune-assays, cell-based proliferation, cytokine release as well as cytotoxicity assays and involve complex technology such as flow cytometry. In some cases, these assays may lack the required robustness, simplicity, sensitivity and/or throughput required to function well in the QC GMP environment. In other cases these assays reflect an average measurement that represents the entirety of a sample and do not provide information regarding the function associated with individual cell phenotypes. Next generation potency assays are needed which are easy to use, robust and provide a more comprehensive evaluation of the quality attributes of the Drug Substance (i.e. Viral Vector, plasmid) and Drug Product.

This session will involve case studies highlighting innovative approaches being used to develop potency assays in support of programs from early phase to late phase and commercialization. Assay design strategies focused on reflecting a cell therapy product's complex MOA, while balancing this with attributes that are important for a method to function effectively to perform QC release and stability testing will also be included. Points to consider for critical reagents used to support potency assays for cell/gene products as well as viral vector will also be discussed.

Session Speakers:

Rethink the Potency Paradigm for Gene Therapy Products
Xiaohui Lu, Ultragenyx

Success Story: Luxturna Potency Assay Development to Validation
Ravindra Kumar, *Spark Therapeutics*

Unleashing the Power: Assessing CAR T Cell Therapy Potency and Maximizing Life Cycle Management
Seema Bansal, *Bristol-Myers Squibb*

Additional Panelists:

Andrew Byrnes, *CBER, FDA*

14:20-15:55 Salons A-C

Parallel Session 4: Considerations for CGTP Delivery Device Development

Session Chairs: Ilona Reischl, Jiwen Zhang
Presentation type: H - Hybrid
CGTP Symposium Oral

Many cell and gene therapy products use special delivery devices. Generally, there are two approaches for development and commercialization. 1: specify a device brand in the label to be used with the cell or gene therapy products. Under this approach, the sponsor is developing a drug/device combination product, need to navigate complex, often rather different, regulatory pathways in different countries, regions. 2: not to specify a brand but only describe device characteristics so that end users can source the device on their own to the defined characteristics. Under this approach, the sponsor needs to consider data required to support drug compatibility with potentially a class of device products. The US FDA has issued a guidance in 2019 on CGTP delivery devices. In the EU, regulatory requirements differ between integral, non-integral co-packaged, or not co-packaged (e.g. referenced) medical devices. In addition, distinct legislative frameworks for medicinal products and medical devices require joint input of respective experts.

This session will use case studies to explore and discuss each approach, share lessons learned during early and late stage CGTP development on managing regulatory procedures, overcoming regulatory hurdles.

Session Speakers:

The Regulatory Interface of ATMPs and Medical Devices in Europe
Ilona Reischl, *Austrian Medicines and Medical Devices Agency*

U.S. FDA's Perspective on Delivery Devices Used to Administer Cell and Gene Therapy Products
Laura Ricles, *CBER, FDA*

What Device Engineers Can Teach You About CGTP Delivery Devices
Saran Baskaran, *AstraZeneca*
Samir Shah, *AstraZeneca*

15:55-16:25 Foyer A-D

Networking Break

15:55-16:25 Brookside A&B

Margaritas & Mobile App

Join us for a refreshing margarita and learn more about the CASSS mobile app during the afternoon networking break!

16:25-17:25 White Oak

Roundtable Discussions - Session 1

Presentation type: IP -In Person
CGTP Roundtable

Table Topics:

1. Potency Assays

2. Donor Profile for Allogeneic Cells

3. Comparability for Cell and Gene Therapy Products

4. Process Development of a Cell or Gene Therapy - Speed to a Phase 1 FIH Clinical Supply - Best Practices or Lessons Learned

5. Stability Testing

6. Tech Transfer of a Phase 1 FIH Manufacturing Process - Process Development to the Manufacturing 'Floor'/QC Lab.- 'The Good and Bad - What Makes for Success?

7. Building Comparability Strategies in Rare Diseases (Very Few Batches)

8. Phase Appropriate Validations

9. CQA Risk Assessments for Cell and Gene Therapies

11. Donor profiling for Allogeneic Products

12. Bridges Strategies for Manufacturing and CQA Assay Changes Moving Phase 1 to Phase 2 Focus–from Phase1/2 to Pivotal

13. The Challenges and Opportunities with Viral Vector CMOs - Best Practices in an Effective CMC Relationship

17:25-17:30 Veranda

Adjourn Day 1 - Thank you!

Join us on the Veranda for the CGTP 2023 Welcome Reception!

17:30-19:30 Veranda

CGTP 2023 Welcome Reception

Join us on the outdoor veranda to celebrate the start of CGTP 2023! Enjoy live music from the Grasso Brothers while enjoying summer BBQ-inspired food and drinks.

Wednesday, 28 June, 2023

07:30-08:30 Foyer A-D

Continental Breakfast

Breakfast will be available until 9:00 AM Eastern

08:00-08:30 Foyer A-C

Registration

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

08:30-10:10 Salon D

Plenary Session 5: Challenges with Incoming Materials - Raw, Starting, Ancillary, etc.

Session Chairs: Vandana Chauhan, Cynthia Riggins
Presentation type: H - Hybrid
CGTP Symposium Oral

Raw material selection decisions early in the development of ATMPs are crucial and can impact the final product quality and ultimately patient safety. Therefore, it is imperative that developers employ a risk-based approach when selecting and qualifying materials to establish the necessary controls to ensure process robustness and safety of the product.

Material classification is key to a successful implementation of a risk-based approach. The same material (e.g. plasmids) may be classified differently according to its use in different products and thus warrant a different control strategy. Classification of materials should be considered early in development with input from regulators as appropriate. Current guidance from many regulatory and standards bodies including FDA, EU, ICH, PIC/S, USP, and Ph. Eur., provide a foundation though some inconsistencies and use of alternate terminology do exist.

Control strategies for materials should be commensurate to their classification and, therefore, their potential to affect critical quality attributes of the product. Suppliers are an extension of an ATMP developer’s manufacturing process and the rigor of suppliers’ qualification activities and controls can impact the manufacturing process and the product. As suppliers are often unfamiliar with therapeutic manufacturing requirements (e.g. the need to minimize or eliminate the use of animal-derived materials),a partnership mindset by both parties to ensure transparency and joint mitigation of risks is crucial.

Session Speakers:

Material Considerations for The Manufacture of Pluripotent Stem Cell Derived Products
Saran Karumbayaram, *CBER, FDA*

Challenges and Perspectives in the Manufacture of Synthetic sgRNA used in Cell and Gene Therapy
Joe Guiles, *Agilent Technologies Inc.*

Material Qualification from a CDMO Perspective: A Phase Appropriate Approach to Material and Component Risk Control
Athenesia Faggins, *WuXi Advanced Therapies*

10:10-10:40 Foyer A-D

Networking Break

10:40-12:15 Salon D

Parallel Session 6: Allogeneic Cell-based Therapies: Next Frontier in Advanced Therapies

Session Chairs: Alexandra Beumer Sassi, Kevin Okimura, Deep Shah
Presentation type: H - Hybrid
CGTP Symposium Oral

Allogeneic cell therapies have a potential to revolutionize life-saving Cell and Gene therapy (CGT) field by removing the bottlenecks created by long scheduling delays, lengthy manufacturing times and potential product failures of autologous cell therapies. Despite some early data showing promising clinical results, allogeneic cell therapies have not yet achieved the clinical benchmark set by their predecessors. Some of the challenges unique to allogeneic cell therapies include limited understanding of donor characterization and variability, diminished persistence of allogeneic cells in patients, rapidly evolving need for novel techniques such as gene-editing approaches, and lack of suitable analytical techniques. As already seen in the first generation of autologous cell therapies, the Achilles heel of the commercialization of allogeneic cell therapies remains the CMC challenges. While the industry and regulators have collaborated immensely to advance the CGT field by bringing a number of autologous cell therapy products to the market, similar efforts are warranted for allogeneic cell therapies as the industry is experiencing a shift towards an allogeneic approach.

In this session, we will hear from the industry stakeholders and regulators on the challenges and opportunities in allogeneic cell therapies. Speaker presentations will be followed by a panel discussion and live Q&A with panel members representing both industry and regulators.

Session Speakers:

Donor Qualification and Procurement of Allogeneic Cellular Starting Material: A Global Regulatory Perspective
Jared Schuster, *National Marrow Donor Program*

Allogenic Cell and Gene Therapy Production, Opportunities and Challenges in the Use of iPSCs
Francois Gianelli, *TreeFrog Therapeutics*

CMC Considerations for Manufacturing Allogeneic CAR T Products
Athena Wong, *Sana Biotechnology*

Additional Panelists:

Pankaj Mandal, *CBER, FDA*

10:40-12:15 Salons A-C

Parallel Session 7: Unique Challenges of Stability Testing for Cell and Gene Therapy Products

Session Chairs: Madhu Siluveru, Bruce Thompson
Presentation type: H - Hybrid
CGTP Symposium Oral

Cell and Gene Therapy programs are progressing from early academic settings to commercial manufacturing at increasingly faster pace and in greater numbers. These therapies are complex, difficult to manufacture in large quantities, and involve many bespoke technologies to make, test and release. Stability of these products is important to determine, and demonstrate the quality, safety and efficacy throughout the product shelf life which can vary widely between cell therapy products (e.g., autologous T cell products which are dosed immediately) and some gene therapies (e.g., AAV or LVV products that can be manufactured for inventory and require longer shelf storage stability).

In this session, we will explore challenges in the design, execution and interpretation of stability studies for Cell and Gene therapy products. Additional topics include addressing short shelf life, in-use stability, product heterogeneity, lack of standardized protocols and harmonized health authority requirements and expectations and challenges with sample collection and storage.

Session Speakers:

Stability Challenges for Gene Therapy (GTx) Programs
Bingli Yan, *Pfizer Inc.*

Minimizing Volume Requirements for Stability Studies of Viral Vector-Based Gene Therapy Products
Ying Xu, *Sanofi*

Critical Quality Attributes, Stability-Indicating Test Methods and Cell-based Products: Untangling the Gordian Knot
Don Fink, *Dark Horse Consulting Group, Inc.*

Additional Panelists:

Julia O'Neill, *Direxa Consulting*

12:15-14:15 Foyer A-D

Lunch

Lunch provided in conjunction with the technical seminar talk

12:40-13:40 Salon D

Technical Seminar presented by Cygnus Technologies

Presentation type: H - Hybrid
CGTP Technical Seminar

Analytics for Process-related Impurities in Viral Vector Manufacturing
Alla Zilberman, *Cygnus Technologies, Inc.*

14:15-15:15 White Oak

Roundtable Discussions - Session 2

Presentation type: IP -In Person
CGTP Roundtable

Table Topics:

1. Potency Assays

2. Process Development of a Cell or Gene Therapy - Speed to a Phase 1 FIH Clinical Supply - Best Practices or Lessons Learned

3. Comparability for Cell and Gene Therapy Products

4. Product Quality Testing for Individualized Products

5. Stability Testing

6. Introduction of Viral Clearance Into Some Gene Therapy Products With the New ICH Q5A (R2) Version

7. Building Comparability Strategies in Rare Diseases (Very Few Batches)

8. Phase Appropriate Validations

9. CQA Risk Assessments for Cell and Gene Therapies

10. NGS for Adventitious Agent Testing

11. Reference Standard Challenges for Different Modalities

12. Product Quality for Individualized Testing

13. DE&I: Unconscious Bias

15:15-15:45 Foyer A-D

Networking Break

15:45-17:20 Salon D

Parallel Session 8: Approaches to Decentralized Manufacturing

Session Chairs: Barbara Bonamassa, Christiane Niederlaender, Marcos Timon
Presentation type: H - Hybrid
CGTP Symposium Oral

Decentralized manufacturing involves the use of manufacturing units that can be deployed to multiple locations and in cases, to sites close to the patients. This new manufacturing concept can bring benefits over more traditional, central manufacturing, allowing, for instance, a production adaptable to demand or a more agile and flexible reaction to public health emergencies. In the case of cell and gene therapies, many are highly personalized or have very short shelf-lives, features that could benefit from being manufactured close to the patients.

Decentralized manufacturing presents new regulatory challenges since it requires a shift away from existing frameworks that are designed to meet the regulatory expectations for large-scale centralized manufacture.

This session will bring together regulators and manufacturers with the objective of reviewing the different decentralized manufacturing approaches (e.g. distributed vs point of care) and identify some of the regulatory adaptations needed. The session will also discuss proposals to fulfil manufacturing and control challenges on a decentralized scenario, such as comparability between (potentially) many manufacturing sites, or carrying out complex testing (e.g. potency).

Session Speakers:

Decentralised and Point of Care Manufacturing - A UK Perspective
Ian Rees, *Retired, MHRA*

Distributed Manufacturing for Cell Therapies: Regulatory Challenges and Solutions
Gülbengü Yüksel, *Tigen Pharma SA*

Attaining Worldwide Standardization Within A Decentralized Framework
Chaya Mazouz, *Orgenesis Inc.*

Additional Panelists:

Johnny Lam, *CBER, FDA*
Marcos Timón, *AEMPS*

15:45-17:20 Salons A-C

Parallel Session 9: Genetic Characterization Technologies for Product Quality, Safety and Identity

Session Chairs: Marcel Hoefnagel, Andrew Weiskopf
Presentation type: H - Hybrid
CGTP Symposium Oral

The further advance of techniques for genetic modification also necessitates development of novel and more comprehensive genetic characterization tools to identify intended and unintended genetic changes in (subpopulations of) genetically modified cells. But the proliferation of these tools poses both opportunities and challenges for those developing cell and gene therapy products. The selection of global and/or targeted genomic analysis methods requires developers to consider the questions they wish to answer and the intended purposes behind the testing.

Is the analysis intended as a one-time highly-detailed survey of a gene-modified product, or will it be used for lot release testing? For viral vector products, what methods are best to confirm transgene identity and integrity? For gene-edited cells, how does one approach assessment of on-target versus off-target edits? For induced pluripotent stem cells (iPSCs), how does one detect genetic variants? What is the role for sequencing methods in testing of critical components and starting materials? How can bioinformatics tools be qualified in order to reliably use the large datasets generated by methods such as Next Generation Sequencing (NGS)? What are the strengths and limitations of the tools available to us, and how do more traditional methods such as karyotyping fit into the control strategy for cellular therapies?

In this session, we will explore the varied technologies available for genetic characterization and how they can be applied to ensure product quality and patient safety.

Session Speakers:

Characterization of VivoVec: A Surface-Engineered Lentiviral Vector Platform That Generates CAR T Cells in Vivo
Richard Rogers, *Umoja Biopharma*

Control of Chromosome Integrity in the Routine Manufacturing of Allogeneic CAR T Cell Therapies
Kimberly Davis, *Sana Biotechnology*

INDUCE-seq: Ensuring the Safe Development of Cell and Gene Therapies by Gene Editing
Simon Reed, *Cardiff University*

Additional Panelists:

Anna Kwilas, *CBER, FDA*

17:20-18:50 Foyer A-D

CGTP 2023 Exhibitor Reception

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

Thursday, 29 June, 2023

07:30-08:30 Foyer A-D

Continental Breakfast

Breakfast will be available until 9:00 AM Eastern

08:00-08:30 Foyer A-C

Registration

Registration is open until the conclusion of the program in the Foyer C alcove

08:30-10:10 Salon D

Parallel Session 10: Platform Development Approaches for AAV Gene Therapy Products

Session Chairs: Christopher Storbeck, Allison Wolf
Presentation type: H - Hybrid
CGTP Symposium Oral

With the recent approval of several Adeno-Associated Virus (AAV)-based gene therapies, the application of AAV gene therapy approaches to the treatment of human disease is expanding. AAV is emerging as the vector of choice for gene therapy based on a developing understanding of numerous features of its utility, including, but not limited to its' safety, versatility, durability, and manufacturing approaches.

As this knowledge base expands, a given AAV quality target product profile (i.e., specific AAV serotype for targeting a specific organ or cell types) may lend itself to being the basis for a platform approach to product development for targeting specific monogenic diseases impacting similar target organs or cell types.

The advantages to this approach can be to leverage prior knowledge from preclinical studies and manufacturing process development, including an understanding of product critical quality attributes, critical process parameters for process steps, including those assessed for virus clearance, analytical methods and other elements of the control strategy, to create synergies for other products in development. This synergistic platform approach to AAV development is essential to help reduce costs, streamline development, and ultimately deliver these products to the patients who need them more efficiently.

This session will highlight different approaches taken by industry, CMOs and other organizations and illustrate their experience, including the benefits achieved with the application of platform approaches to AAV product development.

Session Speakers:

Establishing Analytical Tools for an AAV Platform
Melissa Clague, *Eli Lilly and Company*

Assembling Complex Lego Blocks - Building AAV Manufacturing Platforms
John Kerwin, *National Resilience, Inc.*

NIH Programs Supporting the Development of AAV as a Therapeutic Platform
PJ Brooks, *NCATS, NIH*

Additional Panelists:

Christiane Niederlaender, *Parexel International*

08:30-10:10 Salons A-C

Parallel Session 11: Progress in Gene Editing

Session Chairs: Margarida Menezes Ferreira, Robin Wesselschmidt, Heidi Zhang
Presentation type: H - Hybrid
CGTP Symposium Oral

Gene editing holds the promise to revolutionize medicine due to its potential to correct or disrupt target DNA sequences with a putative long-lasting effect. To realize the promise of these revolutionary medicines, appropriate quality control is needed to ensure targeting specificity to avoid unwanted off-target or on-target effects, such as translocation and integrations, and to address challenges related to formulation and delivery of a biologically active product.

In this session, we will discuss different technologies that lead to the improved specificity and control strategies to ensure product quality and safety. Examples of in vivo and ex vivo gene editing approaches that reached the clinic will be presented. Progress in innovative approaches, such as base editing, prime editing, and the emerging epigenetic editing technologies will be covered.

Session Speakers:

Regulatory Perspectives on Gene Therapies Incorporating Human Somatic Genome Editing
Zhaohui Ye, *CBER, FDA*

Analytical Challenges with Gene Regulation and Gene Editing In-vivo and Ex vivo Gene Therapies
Michael Moloney, *Sangamo Therapeutics*

Epigenetic Editing: New Frontier for Genetic Medicines
Raj Poudel, *Tune Therapeutics*

Additional Panelists:

Barbara Bonamassa, *AIFA*
Premanjali Lahiri, *Graphite Bio*

10:10-10:40 Foyer A-D

Networking Break

10:40-12:15 Salon D

Plenary Session 12: Updates in the Regulatory Landscape for Cell & Gene Therapy Products

Session Chairs: Kathleen Francissen, Zenobia Taraporewala
Presentation type: H - Hybrid
CGTP Symposium Oral

Several cell and gene therapy products have received marketing approval from health authorities and, with that, a more complete picture is now emerging about the regulatory CMC requirements to commercialize these products. In the past couple of years, AAV-based gene therapies, autologous cell-based gene therapies (e.g., CAR-T cell products), and tissue-based products have received marketing authorization. During this period, many guidelines were issued by regulatory agencies to address CMC and clinical considerations for development of cell and gene therapies and additional stakeholder support mechanisms were introduced to provide early feedback for product development and manufacturing, such as FDA-CBER's INTERACT and CATT meetings, respectively, and EMA's PRIME designation.

In this session, we will engage regulators and hear their perspectives on how these initiatives can be leveraged towards successful clinical development of cell and gene therapy products. The session will provide a forum for regulators and developers to highlight the CMC challenges and to share in the learnings from successful development of this diverse array of innovative medicines; specifically, in the strategies that can be applied going forward in a pragmatic and risk-based manner for manufacturing high quality, potent and safe CGT products.

Panelists:

Jounghee Baek, *NIFDS, South Korea MFDS*
Andrew Byrnes, *CBER, FDA*
Ilona Reischl, *Austrian Medicines and Medical Devices Agency*

12:15-12:30 Salon D

Closing Remarks and Invitation to CGTP 2024

Session Chairs: Kathleen Francissen
Presentation type: H - Hybrid
CGTP Symposium Oral