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Cell & Gene Therapy Products Symposium
Scientific Program Summary

MONDAY, JUNE 10, 2019

07:15 – 08:30 **Continental Breakfast** in the Regency Ballroom Foyer

07:15 – 17:00 **Registration** in the Regency Ballroom Foyer

08:30 – 08:45 **CASSS Welcome** in the Regency Ballroom
Wassim Nashabeh, *F. Hoffmann-La Roche Ltd.*

08:45 – 09:00 **Cell & Gene Therapy Products (CGTP) 2019 Introduction**
in the Regency Ballroom
Bruce Thompson, *Lyell Immunopharma*
Andrew Weiskopf, *Biogen*

Keynote Presentation in the Regency Ballroom
Introduction By: Bruce Thompson, *Lyell Immunopharma*

09:00 – 10:00 **FDA's Approach to the Development of Cell and Gene Therapy Products**
Celia Witten, Deputy Director, *CBER, FDA, Silver Spring, MD USA*

10:00 – 10:30 **Networking Break** - Visit the Exhibits in the Regency Ballroom Foyer

Managing Accelerated Development: Collaborative Regulatory Pathways and CMC Experiences in Bringing Cell and Gene Therapies to Patients
Plenary Session in the Regency Ballroom
Session Chairs: Michael Chang, *PTC Therapeutics* and
Ingrid Markovic, *Genentech, a Member of the Roche Group*

10:30 – 10:35 **Introduction**

10:35 – 10:55 **Facilitating Expedited Development of Advanced Therapy Products**
Steven Oh, *CBER, FDA, Silver Spring, MD USA*

10:55 – 11:15 **Managing Accelerated Development: Industry Experience**
Yoko Momonoi, *Celgene Corporation, Summit, NJ USA*

11:15 – 11:35 **Breaking the Traditional CMC Development Pathway**
Alexandra Beumer Sassi, *Voisin Consulting Life Sciences, Cambridge, MA USA*

MONDAY, JUNE 10 *continued*

11:35 – 12:05 **Panel Discussion – Questions and Answers**
Alexandra Beumer Sassi, *Voisin Consulting Life Sciences*
Steven Oh, *CBER, FDA*
Tracy Burton, *Health Canada*
Stacey Ma, *Sana Biotechnology*
Yoko Momonoi, *Celgene Corporation*

12:05 – 13:35 **Hosted Lunch**

Cell Therapy - Analytical Characterization and CQA Assessments
Plenary Session in the Regency Ballroom
Session Chairs: Francis Poulin, *Sanofi* and Melanie Tellers, *Tmunity Therapeutics, Inc.*

13:35 – 13:40 **Introduction**

13:40 – 14:00 **Roadmap to CQAs and Analytical Control Strategy for Cell Therapies**
Tam Soden, *Kite, A Gilead Company, Santa Monica, CA USA*

14:00 – 14:20 **Combination Allogeneic Cell Therapies – Unique Considerations for CMC and Analytical**
Devyn Smith, *Sigilon Therapeutics, Cambridge, MA USA*

14:20 – 14:40 **Lessons Learned from Potency Assays for β-thalassemia and Sickle Cell Disease Autologous Gene Therapy Drug Products**
Ilya Shestopalov, *bluebird bio, Inc., Cambridge, MA USA*

14:40 – 15:10 **Panel Discussion – Questions and Answers**
Ilya Shestopalov, *bluebird bio, Inc.*
Devyn Smith, *Sigilon Therapeutics*
Tam Soden, *Kite, A Gilead Company*

15:10 –15:40 **Networking Break** – Visit the Exhibits in the Regency Ballroom Foyer

MONDAY, JUNE 10 *continued*

Gene Therapy Analytics: Challenges and Opportunities
Plenary Session in the the Regency Ballroom
Session Chairs: Svetlana Bergelson, *Biogen* and Heidi Zhang, *Juno, A Celgene Company*

15:40 – 15:45 **Introduction**

15:45 – 16:05 **TBD**
Angela Whatley, *CBER, FDA, Silver Spring, MD USA (invited)*

16:05 – 16:25 **Orthogonal Approaches to Quantify Critical Quality Attributes for AAV Gene Therapy Products**
Carl Co, *Biogen, Cambridge, MD USA*

16:25 – 16:45 **Analytical Characterization of a Complex Product: Lentiviral Vectors**
Julia Deuel, *bluebird bio, Inc., Cambridge, MA USA*

16:45 – 17:15 **Panel Discussion – Questions and Answers**
Carl Co, *Biogen*
Julia Deuel, *bluebird bio, Inc.*
Wilhelm Herok, *BASG-Federal Office for Safety in Health Care*
Angela Whatley, *CBER, FDA*

17:15 – 18:45 **Networking Reception** in the Regency Ballroom Foyer

18:45 **Adjourn Day One**

TUESDAY, JUNE 11, 2019

07:30 – 08:30 **Continental Breakfast** in the Regency Ballroom Foyer

08:00 – 17:00 **Registration** in the Regency Ballroom Foyer

Gene Editing Technology-based Medicines
Plenary Session in the Regency Ballroom
Session Chairs: Margarida Menezes Ferreira, *INFARMED-National Authority of Medicines and Health Products* and Jiwen Zhang, *Passage Bio*

08:30 – 08:35 **Introduction**

08:35 – 08:55 **Regulatory CMC Aspects of Zinc Finger Nuclease Genome Editing**
Rob McCombie, *Sangamo Therapeutics, Inc., Brisbane, CA USA*

08:55 –09:15 **CRISPR Gene Editing of T-Lymphocytes to Improve Cancer Adoptive Immunotherapy**
Yangbing Zhao, *University of Pennsylvania, Philadelphia, PA USA*

09:15 – 09:35 **Beyond Genetic Knock-out: Designing Smarter, Safer and More Efficient Allogeneic UCART Product Candidates for Patients**
Alexandre Juillerat, *Cellectis, New York, NY*

09:35 – 10:05 **Panel Discussion – Questions and Answers**
Alexandre Juillerat, *Cellectis*
Rob McCombie, *Sangamo Therapeutics, Inc.*
Yangbing Zhao, *University of Pennsylvania*

10:05 – 10:35 **Networking Break** – Visit the Exhibits in the Regency Ballroom Foyer

Custom-made Oncology Products: The Challenges and Opportunities of Developing Individualized Neoantigen-specific Therapies
Plenary Session in the Regency Ballroom
Session Chairs: Kathleen Francissen, Genentech, *a Member of the Roche Group* and Christiane Niederlaender, *MHRA-Medicines and Healthcare Products Regulatory Agency*

10:35 – 10:40 **Introduction**

10:40 – 11:00 **FDA's Regulatory Perspective on Individualized Neoantigen-specific Cancer Vaccines**
Syed Husain, *CBER, FDA, Silver Spring, MD USA*

TUESDAY, JUNE 11 *continued*

11:00 – 11:20 **Individualized Neoantigen-specific Therapies: Enabling Scientific and Technical Advances in Target Selection While Ensuring Quality and Patient Safety**
Richard Bourgon, *Genentech, a Member of the Roche Group*, South San Francisco, CA USA

11:20 – 11:40 **Optimizing the RECON® Algorithm for Designing and Manufacturing a Personalized Neoantigen Vaccine Product**
Joel Greshock, *Neon Therapeutics, Cambridge, MA USA*

11:40 – 12:10 **Panel Discussion – Questions and Answers**
Richard Bourgon, *Genentech, a Member of the Roche Group*
Syed Husain, *CBER, FDA*
Joel Greshock, *Neon Therapeutics*

12:15 – 13:45 **Hosted Lunch**

Challenges Facing Late Stage Process Development, Process Performance Qualification and Commercialization of Cell and Gene Therapy Products
Plenary Session in the Regency Ballroom
Session Chairs: Diane Blumenthal, *Spark Therapeutics* and Allison Wolf, *Eli Lilly and Company*

13:45 – 13:50 **Introduction**

13:50 – 14:10 **Can a Platform Approach Address CGT-specific Challenges during Validation and Commercialization?**
Yogesh Waghmare, *bluebird Bio Inc., Cambridge, MA USA*

14:10 – 14:30 **Are We There Yet? Readying AAV processes for Validation & Commercialization**
Patrick Bastek, *Pfizer, Inc., Rockville, MD USA*

14:30 – 14:50 **Variance Components Analysis for Autologous Therapy Process Development**
Roland Ashton, *Juno Therapeutics, A Celgene Company, Seattle, WA USA*

14:50 – 15:20 **Panel Discussion – Questions and Answers**
Roland Ashton, *Juno Therapeutics, A Celgene Company*
Patrick Bastek, *Pfizer, Inc.*
Angela Whatley, *CBER, FDA*
Yogesh Waghmare, *bluebird bio, Inc.*

TUESDAY, JUNE 11 *continued*

15:20 – 15:50 **Networking Break -** Visit the Exhibits in the Regency Ballroom Foyer

Facility Design Considerations for Cell and Gene Therapy Products
Plenary Session in the Regency Ballroom
Session Chairs: Cynthia Riggins, *Autolus Inc.* and Bryan Silvey, *Kite, A Gilead Company*

15:50 – 15:55 **Introduction**

15:55 – 16:15 **FDA Perspective on Commercial Facility Design for Cell and Gene Therapy Products**
Lily Koo, *CBER, FDA, Silver Spring, MD USA*

16:15 – 16:35 **Integrated Design of Facilities and Operations for Cell Therapies**
Aaron Vernon, *Autolus Inc., Rockville, MD USA*

16:35 – 16:55 **Design Considerations for ATMP Manufacturing Premises from the EU Point of View**
Annie Rietveld, *Rietveld Biofarma Consultancy, Utrecht, Netherlands*

16:55 – 17:25 **Panel Discussion – Questions and Answers**
Lily Koo, *CBER, FDA*
Annie Rietveld, *Rietveld Biofarma Consultancy*
Aaron Vernon, *Autolus Inc.*

17:30 **Adjourn Day Two**

WEDNESDAY, JUNE 12, 2019

07:30 – 08:30 **Continental Breakfast** in the Regency Ballroom Foyer

08:00 – 13:00 **Registration** in the the Regency Ballroom Foyer

Cell and Gene Therapy Comparability and Change Control Considerations
Plenary Session in the Regency Ballroom
Session Chairs: Heli Suila, *Finnish Medicines Agency* and Keith Wonnacott, *Pfizer, Inc.*

08:30 – 08:35 **Introduction**

08:35 – 08:55 **Comparability Approach for an Individualized Product**
Andreas Kuhn, *BioNTechRNA Pharmaceuticals GmbH, Mainz, Germany*

08:55 – 09:15 **Discussions on Our Comparability Approaches**
Zhenhong Li, *REGENXBIO Inc., Rockville, MD USA*

09:15 – 09:35 **Defining Comparability for Cell and Gene Therapy Products**
Mark Galbraith, *Spark Therapeutics, Inc., Philadelphia, PA USA*

09:35 – 10:05 **Panel Discussion – Questions and Answers**
Mark Galbraith, *Spark Therapeutics, Inc.*
Andreas Kuhn, *BioNTechRNA Pharmaceuticals GmbH*
Zhenhong Li, *REGENXBIO Inc.*

10:05 – 10:35 **Networking Break -** Visit the Exhibits in the Regency Ballroom Foyer

Regulatory Updates from Across the Globe
Plenary Session in the Regency Ballroom
Session Chairs: Bruce Thompson, *Lyell Immunopharma* and Andrew Weiskopf, *Biogen*

10:40 – 10:50 **Introduction**

10:50 – 11:00 **Regulatory Updates for Human Cell Therapy Products: A FDA Perspective**
Melanie Eacho, *CBER, FDA, Silver Spring, MD USA*

11:00 – 11:10 **Regulatory Update for Cell and Gene Therapies – A Canadian Perspective**
Christopher Storbeck, *Health Canada, Ottawa, Canada*

11:10 – 11:20 **Regulation of ATMPs in the UK – "With or Without EU"**
Christiane Niederlaender, *MHRA-Medicines and Healthcare Products Regulatory Agency, London, United Kingdom*

WEDNESDAY, JUNE 12 *continued*

11:20 – 11:30	**Recent Developments in ATMP Regulation in Europe** Marcel Hoefnagel, *Medicines Evaluation Board, Netherlands*
11:30 – 11:40	**Regulation on Regenerative Medicine in Taiwan** Wen Yi Hung, *Taiwan Food and Drug Administration (TFDA), Taiwan*
11:45 – 12:45	**Panel Discussion – Questions and Answers** Melanie Eacho, *CBER, FDA* Marcel Hoefnagel, *Medicines Evaluation Board* Wen Yi Hung, *Taiwan Food and Drug Administration (TFDA)* Margarida Menezes Ferreira, *INFARMED-National Authority of Medicines and Health Products* Christiane Niederlaender, *MHRA-Medicines and Healthcare Products Regulatory Agency* Christopher Storbeck, *Health Canada*
12:45 – 13:00	**Closing Remarks and Invitation to CGTP 2020** Heidi Zhang, *Juno Therapeutics, A Celgene Company*
13:00	**Adjournment**

Managing Accelerated Development: Collaborative Regulatory Pathways and CMC Experiences in Bringing Cell and Gene Therapies to Patients

Session Chairs: Michael Chang, *PTC Therapeutics* and Ingrid Markovic, *Genentech, a Member of the Roche Group*

This session will focus on the new and unique challenges in accelerating Chemistry, Manufacturing and Controls (CMC) development to bring life-saving cell and gene therapies to patients faster. Available global regulatory pathways have adapted to address the need to facilitate and expedite the overall development and review to address unmet medical needs in the treatment of serious or life-threatening conditions such as FDA Expedited Programs for Serious Conditions, EU EMA PRIority MEdicines (PRIME), and Japan PMDA SAKIGAKE to name a few. Opportunities for increased interactions and collaborations with agencies have evolved with availability of programs like the FDA Regenerative Medicine Advanced Therapy (RMAT) Designation and FDA INitial Targeted Engagement for Regulatory Advice on CBER ProducTs (INTERACT). With the extraordinary increase in the number of companies developing diverse types of cell and gene therapy products, both similar CMC challenges with more common technology platforms exist with novel CMC challenges specific to cell and gene therapy products. Regulators and industry experts will present their experiences in available regulatory pathways for expedited CMC development and its application to cell and gene therapy products, unique cell and gene therapy CMC challenges in bringing products to patients faster, and lessons learned in the post-approval space.

Cell Therapy – Analytical Characterization and CQA Assessments

Session Chairs: Francis Poulin, *Sanofi* and Melanie Tellers, *Tmunity Therapeutics, Inc.*

Analytical characterization is essential during the development of cell therapy products. It can be used during process development, to define appropriate storage conditions, to assess the stability of the product and process intermediates, and eventually to release the product to the clinic. Cell therapy products are complex, which is reflected in the variety of analytics used to evaluate product quality. Multiple challenges exist including defining Critical Quality Attributes, limitations of materials due to small batch sizes and limited standard methods and reference materials that can be used to appropriately characterize these complex products. Even well-defined tests, such as compendial tests used for biologics, may not be directly applicable to cell therapy products due to material requirements. This session will focus on various aspects of analytical characterization for cell therapy products: analytical development, potency assay development and CQA identification and assessment.

Gene Therapy Analytics: Challenges and Opportunities

Session Chairs: Svetlana Bergelson, *Biogen* and Heidi Zhang, *Juno Therapeutics, A Celgene Company*

Analytical characterization plays an essential role in the development of gene therapy products, from informing process development, determining storage conditions, assessing stability, to guiding the development of control strategy and enabling the release of safe and efficacious products. The complexity of gene therapy products is reflected in the wide range of methods used to assess product quality. Multiple challenges exist, including Critical Quality Attributes identification, limitation of relevant retain material due to small batch DP size, limited tool box of standard methods, and lack of appropriate reference material that can be used to analyze these complex products. Even well-defined tests, such as compendial methods used for biologics, may not be directly applicable to gene therapy products due to the large amount of material required. In this session, we will explore different aspects of analytical development for gene therapy products:

- Analytical challenges;
- Identification of critical quality attributes;
- Advanced methods or novel technologies enabling characterization and comparability;
- New approaches enabling batch release and stability testing.

Gene Editing Technology-based Medicines

Session Chairs: Margarida Menezes Ferreira, *INFARMED-National Authority of Medicines and Health Products* and Jiwen Zhang, *Passage Bio*

Genome editing of human genome using site specific nucleases e.g. ZFN, TALENS and CRISPR/Cas9 is a new pharmaceutical paradigm. Site-specific DNA nucleases can be used ex-vivo to modify human cells or for in-vivo genome editing.

This gene editing session, will discuss these different approaches and the intrinsic difficulties in terms of manufacturing and formulation, associated with the product itself whether protein or mRNA and their targeted delivery. Speakers are invited to discuss relevant characterisation tools and release parameters aiming to ensure a state of control for their approaches.

For each gene technology presented, speakers should also address how far quality assessment can address safety aspects of gene editing, namely related to the off-target effects and inherent DNA repair mechanisms. What are the current methodologies to characterize off-target incidence and potential effects in-vitro, and when is tumorigenicity and in-vivo assessment needed? These topics should be discussed.

Custom-made Oncology Products: The Challenges and Opportunities of Developing Individualized Neoantigen-specific Therapies

Session Chairs: Kathleen Francissen, *Genentech, a Member of the Roche Group* and Christiane Niederlaender, *MHRA-Medicines and Healthcare Products Regulatory Agency*

The emerging field of neoantigen-specific immunotherapies (NeST) is based on identifying mutations unique to a tumor in order to train the immune system to attack the cancer. The final products may take one of several forms, including messenger RNA, peptides, or T cell-based therapies. Next Generation Sequencing (NGS) technology and rapid computing capabilities have made possible the development of individualized neoantigen-specific immunotherapies (iNeST) where each patient receives a custom-made product that is tailored to the mutated peptide sequences present in his or her tumor(s). These iNeST products involve an innovative approach to drug design, where bioinformatics algorithms play a crucial role. Ultimately, the composition and sequence of the medicinal product differs from one patient to the next, taking into account their mutation profile, expression profile, and other factors. In this session, we will discuss key challenges and opportunities to enabling the safe and effective use of neoantigen-specific therapeutic products.

Challenges Facing Late Stage Process Development, Process Performance Qualification and Commercialization of Cell and Gene Therapy Products

Session Chair: Diane Blumenthal, *Spark Therapeutics, Inc.* and Allison Wolf, *Eli Lilly and Company*

The maturing of Cell and Gene Therapy products provides an opportunity to serve patients with options for treatment where none have previously existed. In the past few years, several cell and gene therapy products have gained regulatory approval in the US and EU. The number of products in clinical development in the US continues to increase and many of these products have potential for accelerated regulatory pathways. Manufacturers of cell and gene therapy products must tackle technological challenges under the pressure of short timelines resulting from streamlined clinical development. This session will focus on the key challenges facing CMC professionals as product development programs move the into the later stages of process development and scale-up, process performance qualification and ultimately commercialization.

Facility Design Considerations for Cell and Gene Therapy Products

Session Chairs: Cynthia Riggins, *Autolus Inc.* and Bryan Silvey, *Kite, A Gilead Company*

As the Cell and Gene Therapy pipeline becomes a reality for patients, facilities that are required to manufacture these products for clinical and commercial use likewise are coming on-line. With product safety and manufacturing efficiency as top priorities for the industry, this session looks to provide industry and regulator insight into unique aspects of these facilities. To enable manufacturing of this diverse range of products, it is important to have facility design that enables new technologies, flexible manufacturing with automation, modular design, and scale up/out. C> CMC requirements of these facilities with respect to process flow, cross-contamination controls and validation are key attributes to the design process.

Cell and Gene Therapy Comparability and Change Control Considerations

Session Chairs: Heli Suila, *Finnish Medicines Agency* and Keith Wonnacott, *Pfizer, Inc.*

One of the most frequent questions asked in scientific advice meetings is related to the suitability of the comparability program to support the introduction of changes in the manufacture of advanced therapy medicinal products. These changes are desirable to improve process and product quality and quality control. While changes are a normal part of development, the level of flexibility acceptable in early development is progressively reduced from the non-clinical stage to the pivotal clinical trial stage. Data generation should be appropriate to the stage of development and the extent of the change being made. Comparability remains an important tool required to support changes after marketing authorization where the process and the product are expected to be well defined and appropriately controlled. As such, developing a robust comparability strategy should be initiated early in development. A robust strategy supported by an extensive analytical and experimental program will help to minimize the need for additional non-clinical or even clinical data.

The present session presents challenging comparability issues raised with cell and gene therapy products and the many parameters to be considered as relevant for product quality. Cell based products are very complex in terms of their composition and dynamic nature, with the manufacturing process depending on the combination of multiple biologically active reagents. Gene therapy products are also dependent on complex starting materials and dynamic systems for their production. This session will discuss approaches and examples of how to develop a robust comparability strategy and considerations for successful comparability studies.

Regulatory Updates from Across the Globe

Session Chairs: Bruce Thompson, *Lyell Immunopharma* and Andrew Weiskopf, *Biogen*

Given the recent unprecedented increase in the development of new Cell and Gene therapies, the regulatory environment has been afire with new modalities and concepts, access to new patient populations and previously un-targetable diseases, accelerated development needs, and record requests for new investigational studies. In response, the global regulatory community has become increasingly engaged in these new modalities and have provided new guidance's, greater access to discuss development planning, and a willingness to be strong partners in bringing these therapies to patients.

We have gathered a fantastic group of regulatory leaders from the United States, Canada, Europe and other regions to discuss ongoing at their respective agencies. Each of our regulatory professionals will spend a few minutes highlighting what's on their "hot topics" list and then provide feedback and discussion in a regulatory roundtable discussion.