2018 PDA Cell and Gene Therapy Conference

Advancing into Commercialization

October 23-24, 2018 | Hyatt Regency Bethesda Hotel | Bethesda, MD

As of October 10, 2018

Tuesday, October 23

7:00 a.m. – 5:30 p.m.
Registration Open

7:15 a.m. – 8:15 a.m.
Continental Breakfast

8:15 a.m. – 8:30 a.m.
Welcome and Opening Remarks from Conference Co-Chair
Michael Blackton, MBA, Vice President, Quality, CMC, *Adaptimmune, LLC*

8:30 a.m. – 10:00 a.m.
P1: Navigating the Progress and Promise of Gene Editing
Moderator: Michael Blackton, MBA, Vice President, Quality, CMC, *Adaptimmune, LLC*

Since the early 1980's, gene editing has proven to be the key enabler in the development of game changing pharmaceutical products from recombinant human growth hormone to the latest anti PD-1 monoclonal antibody products. As the science progresses, the increasing accuracy and precision of gene editing has resulted in the development of novel cell and gene therapy products. These advances present unique regulatory challenges and previously unreachable opportunities for the industry. This session will explore the regulator's perspective on gene editing, and also show how innovative gene editing strategies can be used to develop products that will potentially change how we think about drug development and biologic therapeutics in general.

8:30 a.m. – 9:00 a.m.
Regulatory Framework for Gene Therapies Incorporating Human Genome Editing: A CBER Perspective
Anna Kwilas, PhD, Product CMC Reviewer, CBER, *FDA*

9:00 a.m. – 9:30 a.m.
HLA Engineering of Pluripotent Stem Cells
Gregory Block, PhD, VP Corporate Development, *Universal Cells Inc.*

9:30 a.m. – 10:00 a.m.
Questions and Answers/Discussion

9:45 a.m. – 6:30 p.m.
Exhibit Area Open

10:00 a.m. – 10:45 a.m.
Refreshment Break in Exhibit Area

10:45 a.m. – 12:15 p.m.
P2: Applying Analytics to the Development and Manufacture of Cell and Gene Therapy Products
Moderator: Brian J. Hawkins, PhD, Scientific Applications Director, *BioLife Solutions*

Despite recent clinical success, cell and gene therapy products remain complex therapies to both manufacture and distribute. This complexity can influence product consistency, lifecycle efficiency, and developmental costs. This complexity also generates enormous volumes of data that must be effectively collected and managed. If applied correctly, this data can generate deep insight into the manufacturing process and product that are not typically observed at first glance, allowing for a greater understanding of the specific benefits and drawbacks of individual components within a particular workflow. This session focuses on the effective use of data analytics in cell and gene therapy product development and manufacturing to speed the delivery of these promising therapies the patients who most need them.

10:45 a.m. – 11:15 a.m.
Coordinated Efforts to Develop a Robust Standards Infrastructure for Emerging Cell and Gene Therapy Products
Sheng Lin-Gibson, PhD, Chief, Biosystems and Biomaterials Division, *NIST*

11:15 a.m. – 11:45 a.m.
Utilization of Statistical Process Control to Refine CQAs and Define Process Robustness

David Smith, Head of Innovation and Engineering, *Hitachi Chemical Advanced Therapeutics Solutions*

11:45 a.m. – 12:15 p.m.
Questions and Answers/Discussion

12:15 p.m. – 1:45 p.m.
Lunch on Your Own. Exhibit Area Closed. *A listing of local restaurants is available at the PDA registration desk.*

1:45 p.m. – 3:15 p.m.
P3: Automation of Cell Therapy Product Manufacturing
Moderator: Vijay Chiruvolu, PhD, Vice President, Process Sciences & Engineering, *Kite Pharma (A Gilead Company)*

Although the field of cell and gene therapy is booming with renewed enthusiasm, the challenge of manufacturing these products in an efficient and cost-effective way still remains. Most of the current manufacturing processes rely on semi-automated or manual operations performed by scientists and technicians. This session will focus on some of the challenges and opportunities related to implementation of automation in cell therapy manufacturing such as technology selection, cost, stage of implementation, and regulatory implications.

1:45 p.m. – 2:15 p.m.
Automated Approaches for the Process Development and Manufacture of Cell and Gene Therapies
Qasim A. Rafiq, PhD, Associate Professor, *University College London*

2:15 p.m. – 2:45 p.m.
COGs in the Machine: Process Automation as a Key to Commercialization
Ian Gaudet, PhD, Director, Process Development, *Miltenyi Biotec, Inc.*

2:45 p.m. – 3:15 p.m.
Questions and Answers/Discussion

3:15 p.m. – 4:00 p.m.
Refreshment Break in Exhibit Area

4:00 p.m. – 5:30 p.m.
P4: Regulatory Considerations for Development and Commercialization of Cell and Gene Therapies
Moderator: Austin Caudle, Associate Director, Business Development, *IQVIA*

Product approvals in cell and gene therapy has been at the forefront of headlines around the world, particularly over the past year-and-a-half, due in part to updates to the regulatory structure as existing rules were not designed around these types of therapies. Because these therapies are so complex and differ significantly from traditional biological products, they present a variety of challenges to regulatory authorities, manufacturers, developers, healthcare providers, and patients. This session will examine some of the significant regulatory considerations that are impacting cell and gene therapies, including questions relating to designation of expedited programs available to sponsors of regenerative medicine therapies for serious conditions.

4:00 p.m. – 4:30 p.m.
Moving Beyond CAR-T: Non-Engineered Multi-Antigen Specific T Cells Can Drive Significant Therapeutic Benefit for Patients with Lymphoma, Myeloma, and AML
Peter Hoang, President & Chief Executive Officer, *TapImmune, Inc.*

4:30 p.m. – 5:00 p.m.
FDA's Efforts to Advance the Development of Cell and Gene Therapies
Peter Marks, MD, PhD, Director, CBER, *FDA*

5:00 p.m. – 5:30 p.m.
Questions and Answers/Discussion

5:30 p.m. – 6:30 p.m.
Networking Reception in Exhibit Area

Wednesday, October 24

7:00 a.m. – 5:15 p.m.
Registration Open

7:00 a.m. – 8:30 a.m.
Continental Breakfast

7:15 a.m. – 8:15 a.m.
Cell and Gene Therapy Interest Group Breakfast Session

Moderator: Michael Blackton, MBA, Vice President, Quality, CMC, *Adaptimmune, LLC*

In this session, the Cell and Gene Therapy Interest Group will discuss what PDA is doing in support of cell and gene therapy. The recently published Technical Report focuses on the development of control strategies for cell therapy which will be highlighted as attendees dive deep into discussions to advance the understanding of the unique challenges faced by professionals in the cell and gene therapy community in the development and commercialization of their products by promoting scientifically sound and practical technical information.

7:15 a.m. – 7:45 a.m.
Vijay Chiruvolu, PhD, Vice President, Process Sciences & Engineering, *Kite Pharma (A Gilead Company)*

7:45 a.m. – 8:15 a.m.
Questions and Answers/Discussion

8:30 a.m. – 10:00 a.m.
P5: Current Advancements in Product Realization and Lifecycle Management of Cell and Gene Therapy-Based Biopharmaceuticals
Moderator: EJ Brandreth, Vice President, Quality, *Inovio Pharmaceuticals*

The development, clinical supply, and eventual commercialization of cell and gene therapy products involves unique challenges and opens new opportunities in the manufacturing process, as well as in the overall management of the product lifecycle. This session explores recent advancements, hurdles, and creative solutions to gene therapy products which the industry is embracing as these products advance from development through the clinical stages and approach commercialization.

8:30 a.m. – 9:00 a.m.
Potency and DNA Plasmids: A New Paradigm
Patrick P. Pezzoli, Director, Product Characterization, *Inovio Pharmaceuticals*

9:00 a.m. – 9:30 a.m.
Rapid Interventions for EID Using Synthetic DNA Technology
David B. Weiner, PhD, Executive Vice President, Director – Wistar Vaccine Center, W.W. Smith Charitable Trust Professor in Cancer Research, *The Wistar Institute*

9:30 a.m. – 10:00 a.m.
Questions and Answers/Discussion

9:45 a.m. – 4:00 p.m.
Exhibit Area Open

10:00 a.m. – 10:45 a.m.
Refreshment Break in Exhibit Area

10:45 a.m. – 12:15 p.m.
P6: Quality and Manufacturing Challenges in Cell and Gene Therapy
Moderator: Kimberly A. Carnes, Director, Quality Systems, *REGENXBIO Inc.*

Cell and gene therapy manufacturing presents unique quality challenges. Effective risk management, efficient quality systems, and carefully designed facility and engineering controls are critical to successful manufacturing of cell and gene therapy products. This session will focus on the implementation of innovative quality systems and facility design controls to maintain high product quality standards while meeting cycle time demands, particularly as these products move toward commercialization.

10:45 a.m. – 11:15 a.m.
Quality Systems to Enable Rapid Vein-to-Vein Cycle Time
Heather Francis, MSE, Director of Quality Systems and Compliance, *Kite Pharma (A Gilead Company)*

11:15 a.m. – 11:45 a.m.
Integrated Risk Control Strategy for Gene Therapy Supply: Advanced Manufacturing
Thomas C. Page, PhD, VP, Engineering and Asset Development, *FUJIFILM Diosynth Biotechnologies*

11:45 a.m. – 12:15 p.m.
Questions and Answers/Discussion

12:15 p.m. – 1:45 p.m.
Lunch on Your Own. Exhibit Area Closed. *A listing of local restaurants is available at the PDA registration desk.*

1:45 p.m. – 3:15 p.m.
P7: Navigating the Supply Chain and Logistics to Deliver Cell and Gene Therapy Products
Moderator: Audra Riley, Associate Director, Quality Systems, *Merck & Co., Inc.*

Today, the possibility of treating many genetic and infectious disorders is a reality with regulatory market approval of cell and gene therapy products. As we enter a new era of commercial medicine, cell and gene therapy products present unique supply chain and logistics challenges from their traditional counterparts including both the materials and final product perspectives. This session examines procurement challenges in sourcing vectors and challenges associated with integrating the patient into the supply chain.

1:45 p.m. – 2:15 p.m.
Digital Solutions in Personalized Medicine: Best Practices to Drive Safety, Efficiency, and Scale
Heidi M. Hagen, Chief Strategy Officer, *Vineti Inc.*

2:15 p.m. – 2:45 p.m.
Chasing Vector
Richard M. Stout, Director, Global Sourcing & Procurement, *Adaptimmune, LLC*

2:45 p.m. – 3:15 p.m.
Questions and Answers/Discussion

3:15 p.m. – 4:00 p.m.
Refreshment Break in Exhibit Area

4:00 p.m. – 5:00 p.m.
P8: Panel Discussion
Moderator: Michael Blackton, MBA, Vice President, Quality, CMC, *Adaptimmune, LLC*

As the world of cell and gene therapy advances, and as the industry and regulators gain experience and knowledge, strategies will evolve. This session will explore the current state of the industry, regulatory insight, and practical questions regarding cell and gene therapy.

Vijay Chiruvolu, PhD, Vice President, Process Sciences & Engineering, *Kite Pharma (A Gilead Company)*
Anna Kwilas, PhD, Product CMC Reviewer, CBER, *FDA*
Richard M. Stout, Director, Global Sourcing & Procurement, *Adaptimmune, LLC*
David B. Weiner, PhD, Executive Vice President, Director – Wistar Vaccine Center, W.W. Smith Charitable Trust Professor in Cancer Research, *The Wistar Institute*

5:00 p.m.
Closing Remarks from Conference Co-Chair
Vijay Chiruvolu, PhD, Vice President, Process Sciences & Engineering, *Kite Pharma (A Gilead Company)*