**2020 PDA Advanced Therapy Medicinal Products Conference**  
*Cell and Gene Therapy - From Promise to Cure*

June 24-25, 2020 | Washington Marriott Wardman Park | Washington, DC

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**Tuesday, June 23**

4:00 p.m. – 6:00 p.m.

**Wednesday, June 24**

7:00 a.m. – 6:30 p.m.

**Registration Open**

7:00 a.m. – 8:15 a.m.

**Continental Breakfast**

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8:00 a.m. – 9:45 a.m.  
**P1: Promise Fulfilled: How Innovative Therapies are Transforming Lives**  
**US Moderator:** Michael N. Blackton, MBA, Vice President, Quality, Adaptimmune LLC  
**EU Moderator:** Dayue Chen, PhD, Head of T-cell Engineering Process Development, Genentech, a Member of the Roche Group

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8:00 a.m. – 8:15 a.m.

**Welcome and Opening Remarks from Conference Co-Chair**  
Michael N. Blackton, MBA, Vice President, Quality, Adaptimmune LLC

8:15 a.m. – 8:45 a.m.

**Engineered T Cell Therapies: Development, Global Regulatory Approvals, and Future Trends**  
Bruce Levine, MD

8:45 a.m. – 9:15 a.m.

**Industry Representative Invited**

9:15 a.m. – 9:45 a.m.

**Q&A Panel**

9:45 a.m. – 10:30 a.m.

**Refreshment Break in Exhibit Hall**

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10:30 a.m. – 12:00 p.m.  
**P2: Regulating a Harmonized Plan of Attack to Progress Cell and Gene Therapies**  
**US Moderator:** Irving Ford, MSc, Head, CAR T QC Laboratories, Bristol-Myers Squibb  
**EU Moderator:** Richard Denk, Senior Consulting Aseptic Processing & Containment, SKAN AG

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10:30 a.m. – 11:00 a.m.

**To “B” or Not to “B”. Is it acceptable from a risk-based approach to have a Grade A BSC with a Grade B room? This is just one of the million-dollar questions that continues to create divergence among cell and gene therapy manufacturers. As more and more companies are transitioning into the cell and gene manufacturing, it is imperative that global regulatory bodies effectively collaborate to ensure consistent approaches are taken which allows for an acceptable risk-based approach is applied and enforced. This session will highlight global regulatory challenges, current regulatory thinking, and efforts that are being proposed/taken to transition towards a harmonized global regulatory approach for cell and gene therapy manufacturing.**

11:00 a.m. – 11:30 a.m.

**Current Regulatory Challenges: European Perspective**  
**European Medicines Agency Representative Invited**

11:30 a.m. – 12:00 p.m.

**Q&A Panel**
12:00 p.m. – 1:30 p.m.
Lunch

1:30 p.m. – 3:00 p.m.
P3: Regulatory Strategy
Moderator: Marsha L. Steed, Director, Global QC Microbiology and Contamination Control, bluebird bio

1:30 p.m. – 2:00 p.m.
Lesson Learned from an RMAT Designation Request
Stephen Westover, Cook Myosite

2:00 p.m. – 2:30 p.m.
Dennis Williams, PharmD, Senior Vice President, Late Stage Development, Adaptimmune LLC

2:30 p.m. – 3:00 p.m.
Q&A Panel

3:00 p.m. – 3:45 p.m.
Refreshment Break in Exhibit Hall

3:45 p.m. – 5:15 p.m.
P4: The Need for Speed: A Risk-Based Approach to Optimize Time and Patient Safety
Moderator: Lori L. Daane, PhD, Director of Scientific Affairs, bioMérieux

Cellular therapy production is a complex process that occurs over several days and involves multiple aseptic processing steps. Time is critical for both product efficacy and patient survival. The final product cannot be terminally sterilized and there is no time for traditional sterility testing, therefore a risk-based approach to microbial contamination and control is being adopted.

In this session we will first describe a risk-based approach and learn to identify and mitigate risk of contamination for cellular therapy production. A detailed case study will be presented that includes the resources needed for success and the most common risk factors that require extra attention. Lastly, an extensive comparative study will be presented that identifies an ideal and cost-effective testing combination to maximize sensitivity, reduce risk, and minimize labor.

3:45 p.m. – 4:05 p.m.
Risk Assessment Approach to Microbiological Controls of Cellular Therapies
Tony Cundell, PhD, Principal Consultant, Microbiologica Consulting, LLC

4:05 p.m. – 4:25 p.m.
Microbial Control Strategy Case Study for Cell and Gene Therapy: How to Ensure Sterility Assurance for Patient Safety
Marsha L. Steed, Director, Global QC Microbiology and Contamination Control, bluebird bio

4:25 p.m. – 4:45 p.m.
Comparative Performance Evaluation of USP<71>, BacT/ALERT Dual-T, and Bactec FX for Contaminant Detection in Cell Products, Viral Vectors, and Radiolabeled PET-Drugs
Anna F. Lau, PhD, D(ABMM), Chief, Sterility Testing Service, National Institutes of Health

4:45 p.m. – 5:15 p.m.
Q&A Panel

5:15 p.m. – 6:30 p.m.
Networking Reception

Thursday, June 25

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

7:00 a.m. – 8:30 a.m.
Continental Breakfast
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<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Presenter(s)</th>
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</thead>
<tbody>
<tr>
<td>7:15 a.m.</td>
<td>Cell and Gene Therapy Interest Group Breakfast Session</td>
<td>Moderator: Darius D. Pillsbury, Senior Consultant, Valsource LLC</td>
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<tr>
<td>7:15 a.m.</td>
<td>Introduction to the Cell and Gene Therapy Interest Group</td>
<td>Darius D. Pillsbury, Senior Consultant, Valsource LLC</td>
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<td>7:25 a.m.</td>
<td>Updates on Cryopreservation</td>
<td>Brian J. Hawkins, PhD, Chief Technology Officer, Pluristem, Inc.</td>
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<td>7:35 a.m.</td>
<td>Statistical Solutions for the Data Challenges of ATMP</td>
<td>Katherine Giacoletti, Partner, SynoloStats</td>
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<td>8:00 a.m.</td>
<td>Q&amp;A Panel</td>
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<td>10:45 a.m.</td>
<td>P6: Enhancing Flexibility and Compliance for ATMP Manufacturing Facilities</td>
<td>Moderator: Peter J. Makowenskyj, MEng, Director of Sales Engineering, G-CON Manufacturing</td>
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<td>10:45 a.m.</td>
<td>Flip or Flop? Creating Transformative Multi-Product Facilities</td>
<td>Allan Bream, CRB</td>
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<td>11:15 a.m.</td>
<td>Case Study: Realization of Fill/Finish Project for an Oncolytic Immunotherapy ATMP</td>
<td>Laura R. Moody, PhD, Product Manager – Primary Packaging and Pharma Liquid Packaging, North America, Syntegon Pharma Technology</td>
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<td>Time</td>
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<td>12:15 p.m. – 1:45 p.m.</td>
<td>Lunch</td>
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<td>1:45 p.m. – 3:15 p.m.</td>
<td>Concurrent Sessions</td>
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<td><strong>A1: Cell Therapy/Process Development</strong>&lt;br&gt;Moderator: Vijay R. Chiruvolu, PhD, Senior Vice President, Process Development, Kite Pharma, a Gilead Sciences Company</td>
<td><strong>B1: Recent Breakthroughs in Gene Therapy Using AAV Delivered DNA</strong>&lt;br&gt;Moderator: EJ Brandreth III, MBA, Senior Vice President, Quality, Inovio Pharmaceuticals&lt;br&gt;<em>The safe delivery of synthetic genetic material into the nucleus of patients’ cells requires creative approaches, such as Adeno Associated Viruses (AAV). We will look at the latest developments in the AAV manufacturing technology, and the most recent success story of this exciting new class of products.</em></td>
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<td>1:45 p.m. – 2:15 p.m.</td>
<td>The Challenges of Manufacturing Pluripotent Stem Cell-Based Therapies and Engineered Tissues&lt;br&gt;Brian J. Hawkins, PhD, Chief Technology Officer, Pluristyx, Inc.</td>
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<td>2:15 p.m. – 2:45 p.m.</td>
<td>Challenges of Scaling-Up Cell-Based Processes to Meet Phase-Appropriate Requirements&lt;br&gt;Kelly Kemp, PhD, Director, Process Development, ViaCyte</td>
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<td>rAAV Vector Development and Large-Scale Manufacturing Using BEVS Technology&lt;br&gt;Scott Jeffers, UniQure</td>
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<td>2:15 p.m. – 2:45 p.m.</td>
<td>Product Development and Commercialization of AAV Gene Therapy for Hemophilia A&lt;br&gt;Biomarin Representative Invited</td>
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<td><strong>P7: Panel Discussion</strong>&lt;br&gt;Moderator: Darius D. Pillsbury, Senior Consultant, Valsource LLC</td>
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<td><strong>Panel Discussion</strong>&lt;br&gt;Peter W. Marks, MD, PhD, Director, CBER, U.S. FDA&lt;br&gt;Industry Representatives Invited</td>
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<td>5:00 p.m.</td>
<td><strong>Closing Remarks from Conference Co-Chair</strong>&lt;br&gt;Darius D. Pillsbury, Senior Consultant, Valsource LLC</td>
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