The Parenteral Drug Association presents the...

2017 PDA Cell and Gene Therapy Conference

December 5-6, 2017 | San Diego, CA
Manchester Grand Hyatt San Diego
Exhibition: December 5-6
#2017CGT

The Journey of Cell and Gene Therapy – Bringing Science to Reality

pda.org/2017CellGene

This preliminary agenda is current as of August 29, 2017

RECORDINGS ARE PROHIBITED AT ALL PDA EVENTS
There has been a tremendous surge in the number of gene and cell therapy clinical trials taking place worldwide due to the great promise of these therapies shown in early clinical trials. CAR cell therapy trials represented 116 registered cell therapy trials in 2016 alone. In order to get these promising therapies developed and approved is a big challenge. The majority of these processes have been initially conceived in academic institutions. As a result, they are manual, use research-grade complex biological raw materials and lack adequate infrastructure, which makes them difficult to scale for commercialization. Furthermore, the analytical methods used for process characterization and batch release are complex, cumbersome, and subjective. As these products move from early stage to late stage and companies push for their approval, the industry needs to work closely together and with regulatory agencies so that appropriate regulations are developed.

Although the knowledge and experience from protein biologics development is useful and can be applied to this field, companies need to work closely with equipment manufacturers, raw material suppliers, and other industry partners to increase the success of manufacturing and the supply of these life-saving products to patients. Through collaboration, there is an opportunity to compare ideas and approaches with other companies, which will provide lasting benefit for these promising therapies.

The 2017 PDA Cell and Gene Therapy Conference provides an excellent opportunity for companies to share facility design, process development and commercialization knowledge, and experience. The Conference will feature presentations from leading pioneer academic researchers and industry experts on topics carefully chosen to meet the needs of this fast growing industry.

Explore current and future applications for these emerging therapeutic entities, focusing on the emerging fields of immunotherapy and gene- and cell-based therapies. Hear directly from the experts about the science and technology needed to bring these innovative products to market and ultimately to the patient. Current and future trends in development and manufacturing will be covered, including next-generation processing and facilities, application of big data for process design and optimization, and accelerating the industry response to healthcare needs.

Join us to learn more about this exciting and rapidly growing field and what you need to do to keep pace with the latest developments!

We look forward to seeing you in San Diego!
GENERAL INFORMATION, REGISTRATION

FOUR WAYS TO REGISTER

1. Click  
pda.org/2017CellGene
2. Fax  
+1 (301) 986-1093
3. Mail  
PDA Global Headquarters  
Bethesda Towers  
4350 East West Highway, Suite 600  
Bethesda, MD 20814 USA
4. Phone  
+1 (301) 656-5900 ext. 115

VENUE

Manchester Grand Hyatt San Diego  
1 Market Place  
San Diego, CA 92101  
Phone: +1 (619) 232-1234  
Website: manchester.grand.hyatt.com  
Rate: Single/Double: $249 plus applicable state and local taxes  
Cut-Off Date: Monday, November 6 (Availability may be limited. Requests will be processed on a first-come, first-served basis. Attendees staying within the PDA block will receive the Conference rate.)

CONTINUING EDUCATION CREDITS

PDA is accredited by the Accreditation Council for Pharmacy Education (ACPE) as a provider of continuing pharmacy education. Participants may sign up to receive Continuing Pharmacy Education (CPE) credits. To do so, participants must sign in at the beginning of the program, submit the provided evaluation forms, and mail the CPE credit request to the address stated on the form. Attendees must be present at the full event to receive CPE credit.

ALERT: ACPE and the National Association of Boards of Pharmacy developed the Continuing Pharmacy Education (CPE) Monitor that allows pharmacists to electronically track their CPE credits. The CPE Monitor will reject any CPE credit requests submitted past 60 days from date of ACPE accredited activity. Always submit CPE activity claims as soon as possible and by the deadline specified on the CPE credit request form.

2017 PDA Cell and Gene Therapy Conference  
ACPE # 0116-0000-17-020-L04-P  |  1.2 CEUs  
Type of Activity: Knowledge

LEARNING OBJECTIVES

At the completion of this event, attendees will be able to:

• Determine manufacturing and quality requirements for immunotherapies, gene and cell therapy products
• Describe continuous manufacturing applications and flexible facility designs of the future
• Demonstrate how big data can be applied to design and optimize manufacturing processes
• Define strategies to accelerate new products to the market
• Interpret the latest trends in microbiological and adventitious agent control strategies
• Identify advanced analytical approaches that can be applied for quality control and real time release
• Explain delivery system design and manufacturing logistics for patient-centered therapies and precision medicine
• Summarize best practices for identifying and introducing new technologies

WHO SHOULD ATTEND

Job Function:
Scientist  |  Executive and Mid-Level Management  |  Project Management  |  Technical Services  |  Supply Chain  |  Manufacturing Application  |  Risk Management

Departments:
Manufacturing  |  Product Development  |  Quality  |  Research & Development  |  Engineering  |  Laboratory Science  |  Information Technology  |  Validation  |  Training  |  Clinical

CONFERENCE REGISTRATION HOURS

Tuesday, December 5: 7:00 a.m. – 5:00 p.m.  
Wednesday, December 6: 7:15 a.m. – 4:00 p.m.

DRESS/ATTIRE

Business casual attire is recommended for all events. The temperature in the meeting rooms tends to be cool, so a jacket or sweater is advised for your comfort.

SPECIAL REQUIREMENTS

For information regarding special needs accommodations, please inquire at the Registration Desk. PDA is committed to make all events accessible to all individuals.

CONTACT INFORMATION

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TUESDAY, DECEMBER 5

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

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

8:00 a.m. – 8:15 a.m.
Welcome and Opening Remarks from Conference Co-Chairs
Michael Blackton, MBA, Vice President, Quality, CMC, Adaptimmune LLC
Vijay Chiruvolu, PhD, Vice President, Process Sciences & Engineering, Kite Pharma

8:15 a.m. – 9:45 a.m.
P1: Gene Therapy: Bridging Healthcare Innovation From Academia to Industry
Moderator: Michael Blackton, MBA, Vice President, Quality, CMC, Adaptimmune LLC

Session Description: Gene therapy is just beginning to deliver on the promise of understanding and treating a variety of medical conditions with amazing results. As clinical trials continue and more knowledge is gained, options for potential treatments increase. This session will address the current progress toward comprehensive treatment options and opportunities in the gene therapy to help patients.

8:15 a.m. – 8:45 a.m.
Gene Therapy
James Wilson, PhD, Director of Gene Therapy, University of Pennsylvania

8:45 a.m. – 9:15 a.m.
Developing Gene Therapies for Rare Diseases, a History of Synergy Between Academia and Industry
Federico Mingozzi, PhD, Spark Therapeutics

9:15 a.m. – 9:45 a.m.
Questions and Answers/Discussion

9:30 a.m. – 6:00 p.m.
Exhibit Area Open

9:45 a.m. – 10:30 a.m.
Refreshment Break in Exhibit Area

10:30 a.m. – 12:00 p.m.
P2: Navigating the Regulatory Environment, Understanding the Challenges, and Sharing Solutions
Moderator: Jean Stanton, MS, Director, J&J Regulatory Compliance, Johnson & Johnson

Session Description: As with any drug, cell and gene therapies must meet the approval standard of being safe and effective. However, there are unique features and challenges for sponsors of these products. Regulatory filing strategies, considered routine for conventional biologics, demand a closer look when developing these biologically complex products. This session will present regulator and sponsor perspectives on the unique regulatory challenges sponsors face and how they can be managed.

10:30 a.m. – 11:00 a.m.
Process and Technology Development of Advanced Therapies, an Industry Perspective
Michael Paglia, MS, Senior Director CMC Operations, Oncorus

11:00 a.m. – 11:30 a.m.
Understanding the Regulatory Challenges and Expectations, a Product Reviewer’s Perspective
Zenobia F. Taraporewala, PhD, CMC (Product) Reviewer, Gene Therapies Branch, CBER, FDA

11:30 a.m. – 12:00 p.m.
Questions and Answers/Discussion
TUESDAY, DECEMBER 5 (CONTINUED)

12:00 p.m. – 1:15 p.m.
Lunch in Exhibit Area

1:15 p.m. – 2:45 p.m.
P3: Development of a Process Control Strategy
Moderator: Diane I. Blumenthal, Head, Technical Operations, Spark Therapeutics

Session Description: The maturing of cell and gene therapy products provides an opportunity to serve patients with options for treatment where none have previously existed. Like all emerging technologies in the biopharmaceutical arena, this presents the need to challenge well-established paradigms and to develop standards that fit the unique nature of this technology. Clinical acceleration can lead to key elements of process understanding lagging behind at a time when a control strategy must be defined. This session will provide an inside look at ways to redefine the approach for the development of a process control strategy that will ensure consistency of process performance and deliver product that meets quality requirements. Examples of challenges and how to overcome them will be presented.

1:15 p.m. – 1:45 p.m.
Developing Control Strategy for Gene and Cell Therapies – Journey of Unique Challenges
Julia O’Neill, MS, CMC Statistician, Tunnell Consulting, Inc.

1:45 p.m. – 2:15 p.m.
Application of Risk Assessments to the Establishment of a Control Strategy for Cell Therapy Products
Darius Pillsbury, Head of Quality Lifecycle Management, Adaptimmune LLC

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

2:45 p.m. – 3:30 p.m.
Refreshment Break in Exhibit Area

3:30 p.m. – 5:00 p.m.
P4: Leveraging Big Data to Speed Cell and Gene Therapy Product Development
Moderator: Brian J. Hawkins, PhD, Senior Application Scientist, BioLife Solutions

Session Description: Rapid advances in “omics” technologies allow scientists and clinicians to accurately assess biological variables at an unprecedented scale. The large data sets generated by these technologies can speed the development of cell and gene therapy products from the benchtop to the clinic. This session will focus on the application of “big data” as it applies to both manufacturing and patient selection.

3:30 p.m. – 4:00 p.m.
Genomics and Gene Editing Tools in Commercializing Pluripotent Stem Cell Therapies
Robert Deans, PhD, Chief Technology Officer, BlueRock Therapeutics

4:00 p.m. – 4:30 p.m.
Novel Feature Selection Strategies for Enhanced Predictive Modeling and Deep Learning in the Biosciences
Tom Chittenden, PhD, VP Statistical Sciences/Founding Director, Advanced AI Research Laboratory, WuXi NextCODE

4:30 p.m. – 5:00 p.m.
Questions and Answers/Discussion

5:00 p.m. – 6:00 p.m.
Networking Reception in Exhibit Area
## WEDNESDAY, DECEMBER 6

7:15 a.m. – 4:00 p.m.
Registration Open

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

### 8:15 a.m. – 9:45 a.m.
**P5: Process Validation and Process Comparability for Cell and Gene Therapy Products**
**Moderator: Vijay Chiruvolu, PhD, Vice President, Process Sciences & Engineering, Kite Pharma**

**Session Description:** Although there is no specific regulatory guidance for comparability and process validation of cell and gene therapy products, concepts from existing guidance for biopharmaceuticals can be applied. Because of the complexity of cell and gene therapy products and difficulties in completely characterizing them, the concept of “process is the product” becomes important. This session will focus on some of the challenges and solutions related to conducting comparability studies and process validation of cell and gene therapy products.

8:15 a.m. – 8:45 a.m.
Process Validation
Regulatory Representative Invited

8:45 a.m. – 9:15 a.m.
**Comparability Studies for Autologous Cell Therapy**
Yoko Momonoi, Senior Fellow, Novartis Pharma AG

9:15 a.m. – 9:45 a.m.
Questions and Answers/Discussion

### 9:30 a.m. – 3:45 p.m.
**Exhibit Area Open**

### 9:45 a.m. – 10:30 a.m.
**Refreshment Break in Exhibit Area**

### 10:30 a.m. – 12:00 p.m.
**P6: Facility, Process Design, and Containment**
** Moderator: Maik W. Jornitz, MS, CEO, G-Con Manufacturing, Inc.**

**Session Description:** Cell and gene therapies have unique process and facility design needs, most importantly robust containment infrastructures as the aseptic processing requirements are rigorous. In addition, since these processes grow over time, it is necessary to scale processes and processing spaces without interrupting the existing space, which has to be contemplated within the design of infrastructure. Questions prevail regarding whether process centers need to be centralized or decentralized, requiring the facilities to be flexible, and potentially relocatable. The presentations within this session will use case examples to address the points outlined above.

10:30 a.m. – 11:00 a.m.
**Design Elements of Autologous Cellular Immunotherapy Manufacturing Plant**
Ernest A. Bognar, Vice President Operations, Gradalis Inc.

11:00 a.m. – 11:30 a.m.
**Trends in the Development, Optimization, and Commercialization of Immunotherapy Processing Facilities**
George Wiker, Executive Director, AES Clean Technology, Inc.

11:30 a.m. – 12:00 p.m.
Questions and Answers/Discussion
WEDNESDAY, DECEMBER 6 (CONTINUED)

12:00 p.m. – 1:30 p.m.
Lunch in Exhibit Area

1:30 p.m. – 3:00 p.m.
P7: Quality Systems and Compliance
Moderator: Donald Startt, Associate Director, Quality, REGENXBIO Inc.

Session Description: Compliance and quality systems play a critical role in the development and manufacture of medicines. The area of cell and gene therapy is relatively new when compared to small molecules or the more established biologics, such as monoclonal antibodies. The regulatory pathways, manufacturing processes, and analytics are not as well defined and as such quality systems must be adaptable to a rapidly changing/unknown landscape. Additionally, many of the companies and organizations developing these products are often small or academic-based. They may not have fully developed quality systems and may leverage numerous external services, such as manufacturing and testing. The purpose of this session is to hear different perspectives on quality systems and what it means to be compliant in this new and dynamic area.

1:30 p.m. – 2:00 p.m.
Implementing Quality Systems with an Externalized Model for Manufacturing and Testing
Kimberly A. Carnes, Associate Director, Quality Systems, REGENXBIO Inc.

2:00 p.m. – 2:30 p.m.
Phase Appropriate Quality System Requirement for Cellular Therapy Manufacturing
Michael O. Skidmore, Consultant, Pharmaceutical Quality Consulting, Inc.

2:30 p.m. – 3:00 p.m.
Questions and Answers/Discussion

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

3:45 p.m. – 5:15 p.m.
P8: Next-Generation Approaches in Gene and Cell Therapies
Moderator: Austin Caudle, MSc

Session Description: The knowledge gained within the field over the past several decades provides much hope for the future of gene and cell therapies. Unfortunately, the journey of an idea from concept to the real world of healthcare is often long and arduous as less than 10 percent of discoveries in the field of medicine make it to the marketplace. The exciting possibility of treating many genetic and infectious disorders is now a reality with the success of clinical trials, novel engineering, and the recent discoveries of tools such as miRNAs and CRISPR/Cas9. Technical hurdles, such as the challenges around how DNA is incorporated into a gene, have been reduced through a new generation of vectors, and stem cell companies are increasingly excited about therapies that are safe and efficacious in treating diseases that are currently untreatable. This session examines the future of gene and cell therapy and explores approaches to overcoming obstacles for progressing discoveries into new devices, drugs, or therapies.

3:45 p.m. – 4:15 p.m.
CRISPRs and Gene Editing: Laboratory Use and Clinical Future
Bob Kesterson, PhD, Professor of Genetics, University of Alabama at Birmingham

4:15 p.m. – 4:45 p.m.
Accelerating the Translation of Scientific Discoveries into Health Benefits for Patients
Jennifer Bond, PhD, Project Leader, CTSI Project Management, Duke Clinical & Translational Science Institute

4:45 p.m. – 5:15 p.m.
Questions and Answers/Discussion

5:15 p.m.
Closing Remarks from Conference Co-Chairs
Michael Blackton, MBA, Vice President, Quality, CMC, Adaptimmune LLC
Vijay Chiruvolu, PhD, Vice President, Process Sciences & Engineering, Kite Pharma
Sponsorship and Exhibit Opportunities are Available!

High-impact, cost-effective sponsorship and exhibition packages are available for the 2017 PDA Cell and Gene Therapy Conference. Exhibit at or sponsor this Conference to gain onsite exposure and showcase your products and services to a target audience of key decision makers. Comprehensive sponsorship packages will provide your company with the opportunity to strengthen brand image, increase visibility, and connect with industry leaders in groundbreaking cell and gene therapies. Sponsorships are available for lanyards, tote bags, notepads, pens, refreshment breaks, lunch, the Networking Reception, and more.

For more information about exhibit and sponsorship opportunities, please contact:

David Hall  
Tel: +1 (240) 688-4405  
Email: hall@pda.org

Alison Caballero  
Tel: +1 (301) 656-5900 ext. 135  
Email: caballero@pda.org
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Manchester Grand Hyatt
Exhibition: December 5-6

1 Contact Information

Prefix          First Name            Last Name
Job Title       Company
Business Address
City             State/Province       ZIP+4/Postal Code
Country         Email
Business Phone   Fax

☐ Substituting for [Check only if you are substituting for a previously enrolled colleague. The fee difference in the prevailing rate is due at the time of substitution. Please note that if you are a non-member substituting for a member, you will be required to pay the difference in the non-member fee.]

Special Dietary Requirements (Please be specific):

Please note: In order to receive the prevailing rate, your registration(s) with payment must be received by PDA by 5:00 p.m. ET on or before the date noted.

☐ Check here to become a member and receive the member price for this event. (Add $279 to your total.)

2 CONFERENCE Registration | December 5-6 Please check appropriate fee (US$).

<table>
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<th>PDA Member</th>
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* For this member type or discounted rate, online registration is not available and must be faxed in.

3 Payment Options All cards are charged in US$.

☐ By Credit Card – Clearly indicate account number, expiration date and billing address.

Total amount $ __________________________

Account Number __________________________

Exp. Date __________________________

Name (exactly as it appears on card) __________________________

Billing Address (must match credit card statement) __________________________

City __________________________

State __________________________

Zip __________________________

Signature __________________________

PDA Federal Tax I.D. #52-1906152

4 CONFERENCE Registration: Register 4 people from the same organization as a group (at the same time) for the CONFERENCE and receive the 5th registration free. Other discounts cannot be applied. All forms MUST be faxed in together.

5 CONFERENCE Registration: Register 4 people from the same organization as a group (at the same time) for the CONFERENCE and receive the 5th registration free. Other discounts cannot be applied. All forms MUST be faxed in together.

CONFIRMATION: A letter of confirmation will be sent to you once payment is received. You must have this written confirmation to be considered enrolled in a PDA event. Please allow one week for receipt of confirmation letter. If you have submitted a purchase order or requested an invoice, please be advised that a credit card guarantee is needed. Please be advised that if your payment or written cancellation notice is not received by October 6, 2017, your credit card will be charged the prevailing rate. SUBSTITUTIONS: If you are unable to attend, substitutions can be made at any time, including on-site at the prevailing rate. If you are a non-member substituting for a member, you will be required to pay the difference in the non-member fee. If you are pre-registering as a substitute attendee, indicate this on your registration form. REFUNDS: Refund requests must be in writing and faxed to +1 (301) 986-1093. (Emails and phone messages are not accepted). REFUNDS FOR CONFERENCE/EVENT: If your written request is received on or before October 6, 2017, you will receive a full refund minus a $200 processing fee. After that time, no refunds or credit requests will be approved. Onsite registrants are not guaranteed to receive Conference materials until all advanced registered attendees receive them. PDA reserves the right to modify the material or speakers/instructors without notice or to cancel an event. If an event must be canceled, registrants will be notified by PDA in writing as soon as possible and will receive a full refund. PDA will not be responsible for airfare penalties or other costs incurred due to cancellation. For more details, contact PDA at info@pda.org or +1 (301) 656-5900. PLEASE NOTE THAT PHOTO ID WILL BE REQUIRED IN ORDER TO PICK UP BADGE MATERIALS ON-SITE. THIS IMPORTANT SECURITY PROEDURE WILL PREVENT ANYONE OTHER THAN THE REGISTRANT FROM PICKING UP THEIR BADGES AND MATERIALS. RECORDING/POTO RELEASE: By registering for these events, I authorize PDA to record and photograph me and to use the recordings/photographs in all formats and media for any purpose, including for education, marketing and trade purposes. I hereby release PDA from all claims arising out of the use of the recordings/photographs, including without limitation all claims for compensation, libel, invasion of privacy or violation of copyright ownership. Tape recordings are prohibited at all PDA conferences.