The Parenteral Drug Association presents:

Advanced Therapy Medicinal Products

10 Years of PDA ATMPs:
Organizing the Present to Strengthen the Future

5-6 June 2018
Novotel Amsterdam Schiphol Airport
Amsterdam | The Netherlands
Dear Colleagues,

Welcome to the 10th Anniversary of the 2018 PDA Europe Conference on Advanced Therapy Medicinal Products (ATMPs)!

It is with great pleasure for us to present you with an outstanding conference agenda and training course over the next few days here in Amsterdam.

PDA prides itself in connecting people, science and regulation. The following three days should provide you with ample opportunity to do exactly that.

Over the past ten years, PDA Europe’s ATMPs conference has become a fixture in the calendar of professionals working in the field of cell and gene-therapy-based drug products. It has reliably offered all participants an open forum for exchange of current best-practice approaches all along the path from R&D through Manufacturing to the clinical application of Advanced Therapies and Medicinal Products.

Handling, testing, and quality control of raw materials, novel approaches to flexible facilities, a thorough update and review of EU GMP Regulations for ATMPs as well as a look at Organ-on-Chip technologies and their relevance for the future of cell and gene therapies will be only some of the highlights in this very special two-day program.

Joint lunches, coffee breaks and a fun Networking Event tonight all aim to facilitate connecting you with the PDA family!

Please take advantage of the impressive panel of speakers and come introduce yourself to us, the Chairs of this meeting as well as to our fellow planning committee members, and your fellow attendees.

We are thrilled about the next two days, thank you for coming to Amsterdam!

Sincerely,

Lutz Uharek, MD, PhD, Charité Berlin, Chair
Wilfried Dalemans, PhD, Tigenix, Co-Chair
Manuel Carrondo, ibet
Fabio D’Agostino, Alira Health
Dirk Groenewegen, Cells4Therapy
Margit Jeschke, Novartis
Margarida Menezes-Ferreira, INFARMED
Valerie Pimpaneau, Voisin Consulting
Sol Ruiz, AEMPS
Andrea Traube, Fraunhofer IPA
Tongtong Wang, Eli Lilly
Falk Klar, PDA Europe
Sylvia Becker, PDA Europe, Manager Programs & Events
### Schedule at a Glance

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<thead>
<tr>
<th>Date</th>
<th>Time</th>
<th>Event</th>
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<tr>
<td>5 June</td>
<td>9:00 – 18:30</td>
<td>Advanced Therapy Medicinal Products Conference, Exhibition</td>
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<tr>
<td>5 June</td>
<td>19:00 – 22:00</td>
<td>Networking Event</td>
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<tr>
<td>6 June</td>
<td>9:00 – 16:45</td>
<td>Advanced Therapy Medicinal Products Conference, Exhibition</td>
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<tr>
<td>7 June</td>
<td>9:00 – 17:30</td>
<td>Practical Application of Phase-Appropriate GMP &amp; Quality Principles to Clinical Development of ATMPs Training Course</td>
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For latest information, please visit: [pda.org/EU/atmps2018](http://pda.org/EU/atmps2018)

Join [@PDA_Europe](https://twitter.com/PDA_Europe) on Twitter and post pictures and highlights of this meeting! 

Follow us on LinkedIn [http://linkedin.com/company/pda](http://linkedin.com/company/pda)
Tuesday, 5 June 2018

9:00 Welcome: Opening Remarks & Introductions
Falk Klar, PDA Europe
Conference Chairs:
Lutz Uharek, Charité
Wilfried Dalemans, Tigenix

Opening Plenary

Current Regulatory Status

Moderator: Wilfried Dalemans, Tigenix

9:15 Evolution of the Regulatory Environment: A Decade of ATMPs
Margarida Menezes Ferreira, INFARMED

9:45 ATMPs in Clinical Trials - News and Views
Ilona Reischl, AEGES

10:15 European Pharmacopoeia Hot Topics in the Field of Cell-based and Gene Therapy Medicinal Products
Emmanuelle Charton, EDQM

10:45 Coffee Break, Poster Session & Exhibition

11:15 Update on Genetically Modified Cells in Europe
Marcos Timón, AEMPS

11:45 New EU GMPs Regulations for ATMPs – Industry Considerations for Implementation
Florence Salmon, Novartis

12:15 Panel Discussion:
• Adaptive Approval and GMP for ATMPs
• EU Regulations for Gene Therapy
• Role of Quality Control
• Collaboration of Industry and Health Care Institutions
Moderator: Wilfried Dalemans, Tigenix

13:00 Lunch Break, Poster Session & Exhibition

PARALLEL TRACKS

Session 1:

Track A

Handling of Raw Materials

Moderator: Andrea Traube, Fraunhofer IPA

14:00 Making the Grade: Untangling the Myths of Raw Materials used for the Manufacture of ATMP
Christopher Bravery, Advanced Biologicals

14:30 Container Closure Integrity of Drug Product Containers During Deep Cold Storage
Derek Duncan, LIGHTHOUSE

15:00 Biopreservation: The Connecting Thread in Cold Chain
Alireza Abazari, BioLife Solutions

15:30 Q&A, Discussion

Track B

Approaching the Market

Moderator: Fabio D’Agostino, Alira Health

14:00 A Decade of Marketing Approval of Gene and Cell-based Therapies in the United States, European Union, and Japan
Delphi Coppens, Utrecht University

14:30 Industry Perspective: Challenges and Evolution, Experiences and Prospects
Zaklina Buljovcic, PharmaLex

15:00 Precision Medicine: The Breakthrough is Close - Are We Prepared?
Thomas Solbach, PwC Strategy&

16:00 Coffee Break, Poster Session & Exhibition
## Session 2:

<table>
<thead>
<tr>
<th>Track A</th>
<th>Track B</th>
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<tr>
<td><strong>Understanding the Human</strong></td>
<td><strong>Testing of Quality Parameters</strong></td>
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<tr>
<td><strong>Moderator:</strong> Dirk Groenewegen, <em>Cells4Therapy</em></td>
<td><strong>Moderator:</strong> Manuel Carrondo, <em>ibet</em></td>
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<td><strong>16:30</strong></td>
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<tr>
<td>Future of Pre-Clinical Studies: Multi Organ Chip Platforms</td>
<td>Rapid Alternatives to Conventional Sterility Testing</td>
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<tr>
<td>Beren Atac, <em>TissUse</em></td>
<td>Lauren Drage, <em>GSK</em></td>
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<td><strong>17:00</strong></td>
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<tr>
<td>Organ-on-Chip in Europe: Promise for the Future</td>
<td>Implementation of Matrix Specific Rapid Methods for Sterility Testing on ATMPs</td>
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<td>Janny van den Eijnden, <em>human organ and Disease Model Technologies (hDMT)</em></td>
<td>Thomas Meindl, <em>Labor L+S</em></td>
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<td><strong>17:30</strong></td>
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<td>Human Brain Project: Artificial Intelligence in Healthcare</td>
<td>Raman Trapping Microscopy for Manufacturing and Quality Control of Advanced Therapy Medicinal Products</td>
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<td>Marc-Oliver Gewaltig, <em>EPFL</em></td>
<td>Karin Schütze, <em>CellTool</em></td>
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<td><strong>18:00</strong></td>
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<td>Q&amp;A, Discussion</td>
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<td>End of Day 1</td>
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<td><strong>19:00</strong></td>
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<td>Networking Event: Dinner &amp; Amsterdam Canal Cruise</td>
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The PDA is proud to invite you to a very special Networking Event

**Tuesday, 5 June 2018**

**19:00** – Meeting Point: Hotel Lobby
Joint Bus Transfer

**19:30** – Dinner, Amsterdam Canal Cruise

**22:00** – Bus Transfer back to Conference Hotel
## Wednesday, 6 June 2018

### Parallel Tracks

#### Session 3: Manufacturing Considerations | Quality Control Strategy

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<thead>
<tr>
<th>Time</th>
<th>Track A</th>
<th>Track B</th>
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<tbody>
<tr>
<td>9:00</td>
<td>Developing a Standardized Pre-Fabricated Approach to ATMPs</td>
<td>Quality Aspects of a Cell-based Therapy for Cartilage Repair</td>
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<td>Peter Makowskýj, GCon Engineering</td>
<td>Giulietta Roël, CO.DON</td>
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<td>9:30</td>
<td>Flexible Gene Therapy Facilities – Current Best Practice</td>
<td>Allogeneic Gene Edited CAR T-Cells: Challenges for Quality Control of Clinical Batches</td>
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<td></td>
<td>John Dougherty, DPS Group</td>
<td>Stephan Reynier, Cellectis</td>
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#### 10:00 Coffee Break, Poster Session & Exhibition

#### 10:30 Process Development for T-Cell Expansion in a Stirred-Tank Bioreactor System
- Ruth McDermott, Sartorius Stedim Biotech

#### 11:00 Industrialization of Viral Vector Manufacturing for Gene Therapies
- Clive Glover, Pall Biotech

#### 11:30 Q&A, Discussion

#### 12:00 Lunch Break, Poster Session & Exhibition

### Session 4: Cell & Gene Therapy: Manufacturing Perspectives

#### 13:00 Manufacturing Challenges and Future of Stem-Cell-Based Gene Therapy
- Christine Günther, Apceth

#### 13:30 Manufacturing Strategies for Allogeneic and Patient-Scale Cell Therapies
- Uwe Gottschalk, LONZA

#### 14:00 Vector Safety
- Theresa Wardell, Oxford BioMedica

#### 14:30 Coffee Break, Poster Session & Exhibition

#### 15:00 rAAV Vector Development and Large-Scale Manufacturing Using BEVS Technology
- Jacek Lubelski, uniQure

#### 15:30 Cost of Goods and Supply Chain Economics Analysis for Allogeneic and Autologous Cell Therapies
- Suzanne S. Farid, University College London

#### 16:00 Closing Panel Discussion, Q&A

#### 16:45 Closing Remarks and End of Conference
- Falk Klar, PDA Europe
- Lutz Uharek, Charité
- Wilfried Dalemans, Tigenix
2018 PDA Europe
ATMPs
Committee and Speaker Biographies

and many more....find them all online!

PDA Europe Poster Session

1. **Beyond Adaptive Regulations - What Remains to Be Done to Get Innovative Therapies to Patients?**
   - Giovanni de Grandis, UCL, UK

2. **Assessing Feasible Business Models for Commercialisation of Cell Therapies**
   - Christos Stamatis, UCL, UK

3. **Pharmaceutical Challenges for Cell Therapy Medicinal Products**
   - Ilona Vollrath, Lonza
**Aseptic Technologies S.A.**

Rue Camille Hubert, 7  
5032 Gembloux, Belgium  
Tel: +32 81 409 410  
www.aseptictech.com

ASEPTIC TECHNOLOGIES manufactures innovative aseptic production equipment, designed to provide safer & easier aseptic filling operations. The AT-Closed Vial is provided ready to fill with the stopper secured in place, and sterile. The filling is performed by a special needle piercing the stopper, which is then immediately heat-sealed by laser and capped. The drug product is thus never in contact with the environment. The AT-Closed Vial is compatible with a wide range of biologics and resist perfectly to low temperature storage (-80°C and vpLN2).

**CellGenix GmbH**

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Tel: +49 761888890  
info@cellgenix.com  
www.cellgenix.com

CellGenix is a leading global supplier of high-quality ancillary products, reagents and tools for cell and gene therapy and regenerative medicine applications. As the first company to obtain a GMP manufacturing authorization for cell processing in Europe, we offer more than 20 years of expertise in GMP manufacturing and development of cell therapy products. Our products are used worldwide in clinical trials by academia and industry partners. To ensure a seamless transition from research to commercialization we offer a comprehensive product portfolio together with expert regulatory and technical support. Our products combine a maximum of quality and safety due to the state-of-the-art production, stringent in-house quality control and comprehensive documentation. CellGenix operates a state-of-the-art GMP facility for production of recombinant proteins and cell processing in Freiburg, Germany. A subsidiary is located near Boston in Portsmouth, NH/USA. For more information, visit cellgenix.com
Overview
There has been a surge in clinical development of Advanced Therapy Medicinal Products (ATMPs), also known as Cellular and Gene Therapy (CGT) Products in the USA. Many start up companies, hospitals, universities, and now even large biopharmaceutical companies, are planning or have already entered into manufacturing these genetically engineered virus and human cell products for clinical studies. While the ground rules for good manufacturing practices (GMPs) and quality of recombinant protein and monoclonal antibody manufacturing and control are well established, for ATMPs these are still under development. ATMP manufacturing and control presents unique GMP and Quality challenges, such as the heightened concern about the safety and consistency of the starting material, the safety and quality of the raw materials added during processing, the need to protect against adventitious agent contamination during the entire manufacturing cycle, the limitations of the analytical methods available to characterize these virus and cell products, and the reality that the administered clinical medicinal product is a complex living organism.

Patients in these clinical development programs need to be protected by common sense GMPs and Quality Principles. The available regulatory authority guidance documents (both EMA and FDA) for ATMPs will be thoroughly examined. In addition, the core principles presented in PDA’s 2016 Technical Report 56 ‘Application of Phase-Appropriate Quality System and cGMP to the Development of Therapeutic Protein Bulk Drug Substance (API)’ will be adapted to ATMPs.

Who Should Attend:
This course is designed for those involved or interested in the manufacturing, quality and GMP compliance of ATMPs during clinical stages of development, including Senior Managers, Directors and Managers/Supervisors, QA/QC, Regulatory, Manufacturing and Process Development personnel.

Learning Objectives:
Upon completion of this course, the attendee will be familiar with:
- Explain the importance and underlying GMP and Quality principles for manufacturing, control and compliance of ATMPs during clinical stages of development
- Appreciate the GMP and Quality similarities and differences between protein medicinal products and ATMPs
- Appropriately apply phase-appropriate GMPs and Quality Principles to the clinical development of ATMPs

John Geigert, PhD, RAC, President, BioPharmaceutical Quality Solutions
John Geigert has been a consultant for the past 15 years, specializing in CMC regulatory strategy for the biopharmaceutical industry. He has held senior leadership positions in industry as Vice President of Quality for both IDEC Pharmaceuticals Corporation and Immunex Corporation. He has been a major participant in regulatory approvals for six biopharmaceutical products now commercially available in the U.S. and in Europe, and has over 40 years of experience in the biologic and biopharmaceutical industry. John obtained his B.S. in Chemistry from Washington State University, and his Ph.D. degree in Organic/Analytical Chemistry from Colorado State University. He has served as a member of the PDA the Board of Directors, and is currently the chair of the PDA Biopharmaceutical Advisory Board. He is US Regulatory Affairs Certified (RAC) by the Regulatory Affairs Certification Board (RACB).
<table>
<thead>
<tr>
<th>Time</th>
<th>Session Title</th>
<th>Details</th>
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<tr>
<td>9:00</td>
<td>Welcome &amp; Introduction</td>
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| 9:10  | Understanding the Basics                                    | • Painting the terminology landscape: ATMP, HCT/P, GMP, Quality System, etc.  
      |                                                            | • Introduction to the risk-based, phase-appropriate GMP and Quality approach |
| 10:30 | Coffee Break                                                |                                                                         |
| 11:00 | Major Differences, and the Regulatory Consequences          | • Viruses/cells are not biologic proteins                                
      |                                                            | • GMP and Quality consequences of the differences between gene / cell-based medicines and protein-based medicines |
| 12:30 | Lunch Break                                                 |                                                                         |
| 13:30 | Regulatory Authority (EMA/FDA) Expectations                | • Regulatory authority risk-based considerations to ATMP GMPs and Quality  
      |                                                            | • Regulatory authority guidances for a phase-appropriate approach to ATMP GMPs and Quality |
| 15:00 | Coffee Break                                                |                                                                         |
| 15:30 | Industry Practice in Applying Phase-Appropriate GMPs and Quality to ATMPs | • Adapting PDA Technical Report 56 as a model for phase-appropriate ATMP GMPs and Quality 
      |                                                            | • Lesson learned from industry                                          |
| 17:30 | End of Training Course                                      |                                                                         |
**Venu**

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+31 23 7470338

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**Conference Registration Hours**

Tuesday, 5 June: 8:00 – 17:30  
Wednesday, 6 June: 8:00 – 12:00

**Course Registration Hours**

Thursday, 7 June: 7:00 – 16:30

**Special Requirements**

If you require special accommodations to fully participate, please attach a written description of your needs with your registration form. Specific questions can be directed to registration-europe@pda.org.

**PDA Europe supports the children’s hospice „Sonnenhof”**

The Sonnenhof Hospice, located near PDA’s office in Berlin, offers support and assistance to families with children suffering from incurable and/or debilitating diseases. At Sonnenhof, children, together with their families, can spend the time they have left as they wish and find some relief from their suffering. Instead of purchasing expensive gifts for the conference speakers, PDA has decided to donate this amount to the Sonnenhof Hospice. You can also contribute and help us increase the amount, it is easy:

**buy a package of chewing gums at the registration desk. THANK YOU!**

To know more about the Sonnenhof Hospice, please visit [www.bjoern-schulz-stiftung.de](http://www.bjoern-schulz-stiftung.de)

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**Björn Schulz Stiftung**  
Für eine Zeit voller Leben

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**PDA 2018 Europe Training Course**  
All About Virus Filtration  
18-19 September 2018  
Cologne | Germany
Save the Date
Advanced Therapy Medicinal Products
4-5 June 2019
See you again next year!