Conference Agenda

Tuesday, 3 June 2014

9:00	**Welcome**	Georg Roessling, *PDA Europe*

Session 1: Regulatory Updates — *Moderator:* Juan Bueren, *Co-Chair*; Sol Ruiz, *AEMPS*

Since the Regulation on advanced therapies in the European Union came into force, a number of gene and cell therapy medicinal products have applied for marketing authorization application through the centralized procedure. Four medicinal products have been approved so far and several others are under evaluation. Advanced therapy medicinal products pose specific regulatory challenges as compared to other medicines through their development. These aspects will be reviewed and discussed. Promising ATMPs currently under development will also be presented.

9:15	**Five Years of ATMPs Review – What to Consider in an Application**	Jean-Hugues Trouvin, *University of Paris Descartes*
10:00	Coffee & Exhibition	
10:30	**Hemophilia B as the Perfect Model to show Efficacy of a Gene Therapy Product**	Amit Nathwani, *University College, London*
11:00	**Clinical Breakthrough Example of Cell Therapy**	Karen Walker, *Novartis*
11:30	Q & A, Discussion	
12:00	**Lunch Break & Exhibition**	

Session 2: Control Strategy Development for Cell & Gene Therapy — *Moderators:* Michele Myers, *GSK*; Valerie Pimpaneau, *Voisin Consulting*

A robust manufacturing control strategy is key to ensuring the reproducible quality production of any pharmaceutical. Given the known variability of biological starting materials and our relatively limited ability to characterize cell and gene therapy products, development of a detailed control strategy for their production is all the more essential and yet there is ambiguity about what constitutes a complete control strategy and how best to articulate it for such complex products. Speakers will share their experience and provide examples to illustrate the knowledge gained through the application of a risk-based approach to control strategy development and how it informs and provides strong foundation to process development, process validation and comparability studies. A panel discussion with regulators will provide further opportunity for guidance and exchange.

13:00	**Developing an Effective Manufacturing Control Strategy for Cellular Therapy Products**	Jean Stanton, *JCJ*
13:30	**Developing a Control Strategy for a Gene-Modified T Cell Product**	Margit Jeschke, *Novartis*
14:00	**Development of the Manufacturing Process for an Autologous HSPC Gene Therapy for ADA-SCID**	Nina Kotsopoulou, *GSK*
14:30	**Consistency in Cell Therapy**	Margarida Menezes Ferreira, *Infarmed*
15:00	Panel Discussion with Regulators & Task Force Members	
15:45	**Coffee Break & Exhibition**	

Session 3: Late Stage Product Development & Life-Cycle Management

Moderators: Sol Ruiz, *AEMPS*
Giovanni Migliaccio, *Istituto Superiore di Sanità*

The complexity of cell to cell interaction in vivo renders non-clinical studies less useful in predicting efficacy and/or safety during the early development stages. First in Man and repeated Phase-I/II studies are presented as a tool to develop a functional product as well as to manage the bioequivalence studies required when multiple master cell banks need to be used. Examples of how to manage the development plan with complex advanced medicinal products will be presented.

16:15	**Clinical Studies as Tools for Manufacturing Development**	Giulio Cossu, *University of Manchester University College, London*
16:45	**Allogeneic Cell Banking Strategy**	Christopher Bravery, *Advanced Biologicals*
17:15	Panel Discussion	
18:00	**End of Day 1 & Networking Reception**	

Wednesday, 4 June 2014

Session 4: Pre-Clinical and Clinical ATMPs

Moderator: Wilfried Dalemans, *Tigenix*
Georg Roessling, *RDA Europe*

Preclinical and clinical evaluation of ATMPs' safety and efficacy necessitates a tailored approach somewhat different from the classical evaluations, adapted to the particular composition and mode of action of this type of medicinal products. Relevance and proper design of preclinical studies as well as selected examples of preclinical and clinical case studies will illustrate how product developers tackle these studies and generate the required data for product evaluation.

9:00	**Analysis of Viral Integration Sites and its Relevance for the Safety Follow-Up of Patients**	Alessandro Aiuti, *The San Raffaele Telethon Institute for Gene Therapy*
9:30	**On the Relevance and Use of Animal Models for Pre-clinical and Clinical Development**	Beatriz Silva Lima, *University of Lisbon*
10:00	Coffee Break & Exhibition	
10:30	**Gene Therapy of Neural Diseases**	Nathalie Cartier, *Inserm*
11:00	**Cell Therapy for Liver Genetic and Orphan Diseases**	Sarah Snykers, *Promethera*
11:30	Panel Discussion	
12:00	**Lunch Break & Exhibition**	

Session 5: New Developments in the ATMPs World

Moderator: Manuel Carrondo, *IBET*
Wilfried Dalemans, *Tigenix*

Given its inherent complexity, the ATMPs field progresses through a "learning by doing" slow process. New concepts and ideas have to be thoroughly tested preclinically before further studies in early stage clinical trials can be conducted; the issues highlighted then require and justify looking for new approaches and results in a virtuous spiral of improvements. This grown knowledge based on the biological/clinical approaches as well as on the necessary improvements regarding process development will be presented, and future outcomes discussed.

13:00	**Transposons for Molecular Medicine**	Zoltan Ivics, *PEI*
13:30	**Expansion of hMSCs on Microcarriers in Single-use Bioreactors**	Sylvain Ribaud, *Merck Millipore*
14:00	Coffee Break & Exhibition	
14:30	**Process Development Up- and Downstream for MSC**	Paula Alves, *IBET*
15:00	**Scaffolds to Build Organs for Children: Allogeneic or Xenogeneic?**	Paolo De Coppi, *Great Ormond Street Hospital*
15:30	**The GENEGRAFT European Project: Ex-vivo Gene Therapy for Recessive Dystrophic Epidermolysis Bullosa using COL7A1 SIN Retroviral Vector**	Alain Hovnanian, *Inserm*
16:00	Q & A, Discussion	
17:00	Closing Remarks	