21 November 2019

Draft Agenda

"Handling the ATMPs challenges to timely meet patient needs"

The eight annual regulatory conference organised by EBE

4 December 2019, NOVOTEL AMSTERDAM CITY, Europaboulevard 10, 1083 AD, Amsterdam, The Netherlands

Time	Agenda item
9.30 – 10.00	**Registration**
10.00-10.15	**Welcome & Introduction** - Enrica Alteri, EMA - Esther Choi, EBE/EFPIA ATMP WG Chair
10.15-11.20	**Session 1: Enhancing AAV vectors manufacturing** Adeno-associated virus (AAV) vectors are among the common vectors used for the development of gene therapy medicinal products. Their utilisation raises issues in terms of vector design and manufacturing process scale-up. This session will highlight some of the current challenges related to the development of gene therapy medicinal products using adeno-associated virus and how they may be addressed. • Considerations for rAAV manufacturing scale up Presenter: Jacek Lubelski, UniQure (15 min) • Maintaining quality attributes throughout AAV product development Presenter: Séverine Marconi, Horama (15 min) • European regulators perspectives Presenter: Marcel Hoefnagel, MEB (15 min) • Panel discussion (20 min) Moderator: Harm Hermsen, Propharma Panellists: All presenters + Patrick Celis, EMA
11.20-11.40	Coffee break
11.40-12.45	**Session 2: Optimising clinical trial design for ATMPs development** The clinical development of a medicinal product requires the generation of data regarding its safety and efficacy, which then allow the definition of its benefit-risk ratio. While this overarching paradigm applies to any medicinal product, the standard Phase 1/Phase 2/Phase 3 approach as well as classical study design is somewhat disrupted in the context of ATMP development. This session will discuss some of the creative manners ATMPs developers have used to optimise clinical trial design • Considerations for the use of biomarkers and novel end-points Presenter: Keith Wonnacott, Pfizer (15 min) • Considerations for selection of appropriate controls Presenter: Samantha Parker, Lysogene (15 min) • European regulators view Presenter: Spiros Vamvakas, EMA (15 min) • Panel discussion (20 min) Moderator: Keith Wonnacott, Pfizer Panellists: All presenters +Mindy Leffler, Casimir + Ana Hidalgo-Simon, EMA
12.45-14.00	Lunch

"Handling the ATMPs challenges to timely meet patient needs"

The eight annual regulatory conference organised by EBE, 4 December 2019, Amsterdam

Time	Agenda item
14.00-15.10	**Session 3: ATMPs development for paediatric patient treatment** There has been a surge of interest in using gene therapies to treat rare diseases, many of which affect paediatric patients. Moving into paediatric studies can pose unique challenges not encountered in adult studies. There is also a need to balance the urgency to move into paediatric patients against the appropriate demonstration that there will a reasonable chance of benefit. Finally, it is important to consider the impact of delaying access to adults while paediatric development continues. Each of these challenges is impacted by the unique nature of gene therapies. This session will explore and discuss the various issues related to the development of gene therapies for paediatric patient treatment. • Industry perspective for paediatric patient treatments Presenter: Keith Wonnacott, Pfizer (15 min) • Patients perspective Presenter: Mindy Leffler, Casimir, in collaboration with Cara O'Neill, Cure Sanfilippo Foundation (15 min) • European regulators perspective Presenter: Professor Fernando de Andres Trelles, PDCO (15 min) • Panel discussion (25 min) Moderator: Samantha Parker, Lysogene Panellists: All presenters + Giovanni Lesa, EMA
15.10-15.30	Coffee break
15.30-16.30	**Session 4: Towards an appropriate implementation of Hospital Exemption** In the EU, all ATMPs are required to obtain a marketing authorisation via the centralised procedure, with the exception of those falling under Article 3(7) of Directive 2001/83/EC, the so-called "Hospital Exemption (HE)". Manufacturing of ATMPs falling under HE is authorised at national level and it is left to Member States to define HE requirements within their legal frameworks. The Utrecht University recently completed a study on the implementation of HE in several Member States. This session will discuss the study findings and possible solutions to address the concerns due to the divergence in HE implementation across EU and to improve the transparency on the use of HE, in order to provide information about innovative treatment options for patients without therapeutic alternatives. • Utrecht University study Presenter: Delphi. Coppens, Utrecht University (25 min) • Panel discussion (35 min) Moderator: Jacquelyn Awigena Cook, Celgene Panellists: All presenters + Ken Genenz (Takeda)+ Annie Hubert (ARM) + Pauline Meij (Leiden University Medical Center)
16.30–17.00	**Closing remarks** - Enrica Alteri, EMA - Jacquelyn Awigena Cook, EBE/EFPIA ATMP WG Vice-Chair