16 December 2016
EMA/853948/2016

Agenda – "Optimising the development of ATMPs to meet patient needs". The fifth annual regulatory conference organised by EBE, in collaboration with the European Medicines Agency (EMA)

16 December 2016, CCT Venues, 40 Bank Street, Canary Wharf, London

Item	Agenda item	Time
1.	**Welcome & Introduction** • Guido Rasi, EMA • Andrea Chiesi, EBE	10:00-10:15
2.	**Session 1: Initiatives to improve ATMPs access to patients** What initiatives have been recently launched to foster the development and registration of ATMPs and improve patient access? EMA Initiative Presenter: Ana Hidalgo-Simon, EMA (15 min) • IMI Initiative Presenter: Salah-Dine Chibout, Novartis (15 min) • FDA Initiative Presenter: Ke Liu, OTAT, CBER, FDA (15 min) • Panel discussion (15 min) Moderator: Paula Salmikangas, Chairperson, CAT Panelists: All presenters	10:15-11:15
3.	**Session 2: Specific requirements for gene therapy medicinal products** What are the challenges in developing and bringing gene therapy medicinal products to the market (biosafety issues, environmental agencies assessment of GMOs, market access)? How do developers respond to these challenges? How can regulators and innovators work together to foster patient access? • Environmental risk assessment for clinical trials with GMO-based ATMPs Presenter: Ann Gorman, Amgen (10 min) • National Competent Authorities experience with environmental risk assessment before setting clinical trials – Procedure and experience in France Presenter: Nils Braun, HCB; Jean-Christophe Pagès, HCB (10	11:15-12:45

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	min) – Procedure and experience in Germany Presenter: Brigitte Anliker, PEI (10 min) • Update on gene therapy medicinal products guideline Presenter: Christiane Niederlaender, member, CAT (10 min) • Industry presentation on market access Presenter: Sven Kili, GSK (15 min) • Panel discussion (30 min) Moderator: Ilona Reischl, member, CAT Panelists: All presenters and Alec Orphanidis, uniQure	
4.	**Lunch**	12:45-14:00
5.	**Session 3: Meeting specific standards for development and commercialisation of ATMPs** What are the manufacturing challenges encountered during early development and to set up clinical trials (definition of starting materials, import of raw materials, process validation…)? How are they handled? How are scale-up challenges covered? How are post-marketing conditions such as registries put in place? • Update on investigational ATMP concept paper guideline Presenter: Ilona Reischl, member, CAT (10 min) • Overview of manufacturing challenges for ATMPs Presenter: Steven Howe, GSK (15 min) • Industry experience with registries for ATMPs Presenter: Diego Ardigo, Chiesi (15 min) • Panel discussion (30 min) Moderator: Esther Choi, BMS Panelists: All presenters and Marit Hystad, member, CAT	14:00-15:10
6.	**Coffee Break**	15:10-15:30
7.	**Session 4: Listening to the stakeholders of innovative medicinal products** Innovative products address areas of high unmet medical need. When in the development process should users be involved in risk/benefit discussions, risk level acceptability? • Viewpoint from a patient organization (10 min): Martine Pergent, IPOPI • Viewpoint from a health care professional (10 min): Prof Christoph Höller, Medical University of Vienna • Viewpoint from a payer (10 min): Dr Anna Bucsics, University of Vienna • Viewpoint from an investor (10 min): Joep Muijrers, LSP VC • Panel discussion (30 min) Moderator: Joep Muijrers, LSP VC Panelists: All presenters	15:30-16:40
8.	**Closing remarks** Paula Salmikangas, Chairperson, CAT Andrea Chiesi, EBE	16:40-17:00