EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

21 April 2010
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Human Medicines Development and Evaluation

10 Years of the Orphan Regulation in Europe Conference Draft Agenda

3-4 May 2010, conference room 2A

Co-Chair:
Committee for Orphan Medicinal Products (COMP) – European Medicines Agency (EMA)

3 May: registration: 9:00 - 10:00, start: 10:00

Welcome and opening remarks Mr T. Lönngren (EMA)	10 min
Gene therapy Dr A. Aiuti (San Raffaele Telethon Institute for Gene Therapy (HSR-TIGET))	20 min
A regulation addressing the needs for drug development in rare diseases Mr M. Terberger (DG Sanco)	20 min
The implementation of the EU Orphan Regulation, what has changed for drug development Prof. K. Westermark (COMP Chair)	20 min
Coffee break (30 min)	
The experience up to date Dr F. Butlen / Dr S. Tsigkos (EMA)	20 min
Collaboration and exchange of information between COMP and other Agency's scientific Committees Dr P. Salmon (CHMP) / Prof. H. Devlieger (PDCO) / Dr C. Schneider (CAT Chair)	30 min
Questions and discussion	15 min
Lunch (12:45 to 13:45)	
The European Orphan Regulation: from hope to reality Mme F. Grossetête (Member of the European Parliament)	15 min
Is the Regulation addressing patients' needs? Mr Y. Le Cam (Eurordis)	20 min
Pharmaceutical industry perspective Dr C. Edfjäll (Celgene International)	20 min

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Academia perspective Dr A. Vellodi (Great Ormond Street Hospital)	20 min
Questions and discussion	15 min
Coffee break (30 min)	
Rare diseases and the need for international collaboration Dr T. Coté (FDA OOPD)	15 min
EU initiatives for research on rare diseases Dr Ch. Kessler (DG Research)	20 min
Public health impact of the Regulation • Clinical trials and networks • National plans Ms S. Aymé (Orphanet)	20 min
Questions and discussion	15 min

End: approximately 17:00

4 May: registration and coffee: 8:30 - 9:30, start: 9:30

Introduction to day 2	5 min
Rare metabolic diseases Prof. F. Platt (University of Oxford)	25 min
Parallel workshops: **Workshop 1: Development of products for rare diseases: incentives, reality and future direction** (room 2A) Chair: Dr P. Evers (Dutch Federation for Cancerpatientorganisations (NFK)) **Workshop 2: Research for rare diseases: translation into new drugs for rare diseases. The role of academic researchers** (room 2D) Chair: Mr D. Caizergues (Genethon) **Workshop 3: Patients' view on the Regulation's implementation. What is still needed?** (room 2G) Chair: Prof. V. Straub (Institute of Human Genetics, Newcastle University)	2 h
Lunch (12:00 to 13:00)	
Presentation of workshops conclusions Chair: Prof. K. Westermark (COMP Chair) and Dr J. Llinares Garcia (EMA)	3 x 10 min
Panel discussion: the next 10 years • Patients' view: Dr F. Bignami (Eurordis) • Academia's view: Dr P. Laforet (Hôpital Pitié-Salpêtrière) • Industry's view: Dr E. Tambuyzer (Genzyme) • Regulators' view: Dr J. Llinares Garcia (EMA)	1 h
Final conclusions Mr T. Lönngren (EMA)	10 min

End: 15:00