MULTI-STAKEHOLDER

Symposium ON IMPROVING PATIENT ACCESS TO RARE DISEASE THERAPIES

24-25
FEBRUARY 2016
HOTEL LE PLAZA
BRUSSELS

EURORDIS
Rare Diseases Europe

Value Determination, Appraisal, Pricing & Reimbursement

IN PARTNERSHIP WITH

HTAi

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A EURORDIS RARE DISEASE DAY® EVENT

RAREDISEASEDAY.ORG

DAY 1
WEDNESDAY 24 FEBRUARY 2016
10.30 to 18.30

10.30 – 14.30	**Introduction & Opening Plenary** Live video streaming until 14.30 Chair: Peter O'Donnell, Politico, Belgium
10.30 – 10.40	**Overarching purpose of the symposium: improving patient access to rare disease therapies** Yann Le Cam, EURORDIS, France
10.40 – 10.50	**Living with haemophilia, a personal story** Cees Smit, EGAN/VSOP, the Netherlands
10.50 – 11.05	**Key access challenges & current initiatives in Member States**
11.05 – 11.20	**How an EU approach can help improve patient access** Philippe de Backer, Member of European Parliament, Belgium
11.20 – 12.20	**Panel** with all keynote speakers and Andrea Chiesi, Chiesi Farmaceutici S.p.A., Italy **Interviewed** by Peter O'Donnell, Politico, Belgium **Q & A** (including questions from online audience)
12.30 – 14.00	***Lunch (Salon Adolphe Max & Esterel Restaurant)***
14.00 – 14.30	**How can the European Commission & Member States help improve patient access to rare disease therapies?** Vytenis Andriukaitis, EU Commissioner for Health & Food Safety, EU
14.30 – 15.45	**Value determination** (Plenary) Chair: Karen Facey, Edinburgh University, Scotland, UK
14.30 – 14.50	**Current ways of determining value in Member States** Elena Nicod, London School of Economics, UK
14.50 – 15.10	**What is value & how to determine value?** Lieven Annemans, Ghent University, Belgium
15.10 – 15.15	**Introduction & aims of the breakout sessions** Karen Facey, Edinburgh University, Scotland, UK
15.15 – 15.45	***Coffee break (Salon Adolphe Max)***
15.45 – 17.15	**Breakout sessions** **1) The social value of Orphan Medicinal Products: European Social Preference Measurement (ESPM) Study Project** Moderator: Michael Schlander, InnoValHC & University of Heidelberg, Germany Rapporteur: Mohit Jain, BioMarin Europe Ltd., UK **2) Principles for Value Assessment and Funding Processes in Rare Diseases.** Moderator: Adam Hutchings, Dolon Ltd., UK Rapporteur: Lieven Annemans, Chair of the European Working Group on HTA for OMPs **3) Immediate Patient Access and Improving Information for HTA Decisions.** Moderator: Trevor Leighton, Shire, Switzerland - Rapporteur: Alastair Kent, Genetic Alliance UK **4) Value Determination of rare disease interventions: Is the EVIDEM framework the right tool?** Moderators: Dima Samaha, LASER Analytica, Canada & Josep Torrent-Farnell, Autonomous University Barcelona, Spain - Rapporteur: Adrian Haigh, PTC Therapeutics, Switzerland
17.15 – 18.30	**Conclusions of Day 1** (Plenary) Chair: Charles L. Barker, CMI Concord Group & Harvard University, USA
17.15 – 17.35	**Feedback from breakout sessions** Mohit Jain, BioMarin; Alastair Kent, Genetic Alliance UK; Adrian Haigh, PTC Therapeutics
17.35 – 18.30	**Panel discussion** Alastair Kent, Genetic Alliance UK, Bhash Parasuraman, Pfizer, USA; Mark Nuijten, A2M & Universities of Maastricht and Groningen, the Netherlands
18.30	***End of Day 1***

DAY 2
THURSDAY 25 FEBRUARY 2016
08.30 to 16.45

08.30 – 11.15	From value to appraisal (Plenary) Chair: Panos Kanavos, London School of Economics, UK
08.30 – 08.35	Introduction & aims of the day Panos Kanavos, London School of Economics, UK
08.35 – 09.35	Panel: How HTA agencies assess advanced therapies & medicines for rare diseases. Can we use the same processes? What are they doing differently to deal with these new challenges? Scott Bryson, SMC, Scotland, UK; Ad Schuurman, ZINL, the Netherlands; Sheela Upadhyaya, NICE, UK
09.35 – 10.35	Simulation exercise of HTA process Durhane Wong-Rieger, Canadian Organization for Rare Disorders, Canada
10.35 – 10.45	Q & A
10.45 – 11.05	Collaborative negotiations Charles L. Barker, CMI Concord Group & Harvard University, USA
11.05 – 11.15	Introduction to the simulation exercises Adam Hutchings, Dolon Ltd., UK
11.15 – 11.45	***Coffee break (Salon Adolphe Max)***
11.45 – 13.15	From appraisal to pricing: simulation exercises in breakout sessions
11.45 – 13.15	Group 1: (Paola Salon 1st Floor) – Moderated by Ruediger Gatermann, CSL Behring, Germany Group 2: (Versailles Salon Ground floor) – Moderated by Laura Gutierrez, Celgene, Belgium Group 3: (Memling Salon Lower Level) – Moderated by Anna Bucsics, University of Vienna, Austria Group 4: (Theatre Room) – Moderated by Paolo Siviero, Principia SGR, Italy
13.15 – 14.30	***Lunch (Salon Adolphe Max & Esterel Restaurant)***
14.30 – 16.45	Pricing & reimbursement, conclusions & next steps (Plenary) Chairs: Ri de Ridder, RIZIV/INAMI, Belgium & Yann Le Cam, EURORDIS, France
14.30 – 15.00	Reports from moderators of simulation exercises Ruediger Gatermann, CSL Behring; Laura Gutierrez, Celgene; Anna Bucsics, University of Vienna; Paolo Siviero, Principia SGR, Italy
15.00 – 16.30	Panel discussion: Pricing & Reimbursement Martin Andrews, GSK, UK; Avril Daly, Retina International, Ireland; Jo de Cock, RIZIV/INAMI, Belgium; Tsveta Milanova, Celgene, USA; Chris Sotirelis, UK Thalassaemia Society, UK **Questions to panel:** Can the price be claimed only on value? What else? Is the value-based price really what they want to see? What more? What would be new, sustainable & trusted models?
16.30 – 16.45	The Perspective and Expectations of the Legislators Françoise Grossetête, Member of European Parliament, France
16.45	***End of Symposium***

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#RareEU2016

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