MULTI-STAKEHOLDER
Symposium
ON IMPROVING
PATIENT ACCESS
TO RARE DISEASE
THERAPIES

Value Determination, Appraisal, Pricing & Reimbursement

IN PARTNERSHIP WITH

A EURORDIS RARE DISEASE DAY® EVENT
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
Philippe de Backer, Member of European Parliament, Belgium

11.05 – 11.20  |  How an EU approach can help improve patient access
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 – 15.00  |  Reports from moderators of simulation exercises

14.30 – 15.00  |  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
PROGRAMME COMMITTEE

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