

MULTI-STAKEHOLDER

Symposium

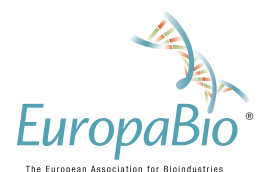
24-25
FEBRUARY 2016
HOTEL LE PLAZA
BRUSSELS

ON IMPROVING
PATIENT ACCESS
TO RARE DISEASE
THERAPIES



Value Determination, Appraisal, Pricing & Reimbursement

IN PARTNERSHIP WITH



A EURORDIS **RARE DISEASE DAY®** EVENT



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 Gattermann, CSL Behring, Germany Group 2: (Versailles Salon Ground floor) – Moderated by Laura Gutierrez, Celgene, Belgium Group 3: (Memling Salon Lower Level) – Moderated by Anna Bucsecs, 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 Gattermann, CSL Behring; Laura Gutierrez, Celgene; Anna Bucsecs, 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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