MULTI-STAKEHOLDER

Symposium

ON IMPROVING
PATIENT ACCESS
TO RARE DISEASE
THERAPIES

24–25 FEBRUARY 2016
BRUSSELS

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 (Plenary)
Live video streaming until 14.30
Chair: Peter O’Donnell, Politico, Belgium

10.30 – 14.40 Overarching purpose of the symposium: improving patient access to rare disease therapies
Yann Le Cam, Chief Executive Officer, EURORDIS, France

10.40 – 10.50 Living with haemophilia, a personal story
Cees Smit, Patients Network for Medical Research and Health - EGAN, The Netherlands

10.50 – 11.05 Key access challenges & current initiatives in Member States
Speaker: to be named

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, company representative and payer tbc.
Interviewed by Peter O’Donnell, Politico, Belgium
Q & A (including questions from online audience)

12.30 – 14.00 Lunch (salon Adolphe Max)

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, Glasgow University, Scotland, UK

14.30 – 14.50 Current ways of determining value for rare diseases in Member States
Elena Nicod, London School of Economics, UK

14.50 – 15.10 What is value & how to determine value?
Lieven Annemans, Gent University, Belgium

15.10 – 15.15 Introduction & aims of the breakout sessions
Karen Facey, Glasgow University, Scotland, UK

15.15 – 16.15 Coffee break

15.45 – 17.15 Breakout sessions
To be confirmed

17.15 – 18.30 Conclusions of day 1 (Plenary)
Chair: tbc.

17.15 – 17.35 Feedback from breakout sessions in plenary
4 rapporteurs

17.35 – 18.30 Panel discussion
Alastair Kent, Genetic Alliance UK, company representative, payer and academic, tbc.

18.30 End of Day 1
DAY 2
THURSDAY 25 FEBRUARY 2016
08.30 to 16.15

08.30 – 11.15  From value to appraisal (Plenary)
Chair: Dr. Panos Kanavos, London School of Economics, UK

08.30 – 08.35  Introduction & aims of the day

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?
Representatives from HTA agencies: Dr Anne Lee, SMC, Scotland, UK; Ad Schuurman, ZINL, The Netherlands; Sheela Upadhyaya, NICE, UK

09.35 – 10.35  Simulation exercise of HTA process
Interactive session: Durhane Wong-Rieger, Canadian Organisation 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, UK

11.15 – 11.45  Coffee break

11.45 – 13.15  From appraisal to pricing: simulation exercises in breakout sessions
4 groups: 2 will simulate the CEO’s viewpoint and 2 will simulate the payer’s viewpoint of OMPs value assessment
A moderator, a facilitator and a EURORDIS staff member per group.

13.15 – 14.15  Lunch (salon Adolphe Max)

14.15 – 16.00  Pricing & reimbursement, conclusions & next steps (Plenary)
Chairs: Ri de Ridder, RIZIV/INAMI, Belgium & Yann Le Cam, EURORDIS, France

14.15 – 14.45  Reports from simulation exercises
4 rapporteurs

14.45 – 15.45  Panel discussion including
A Fipra representative: tbc.
Industry representatives: Tsveta Milanova, Celgene, Belgium
Patient representatives: Avril Daly, Retina International, Ireland & Chris Sotirelis, UK Thalassaemia Society, UK
NCA representatives: tbc.
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?

15.45 – 16.00  Conclusions

16.15  End of Symposium