

2<sup>ND</sup> MULTI-STAKEHOLDER  
**Symposium** ON IMPROVING  
22-23 **PATIENT ACCESS**  
FEBRUARY 2017 **TO RARE DISEASE**  
HOTEL LE PLAZA **THERAPIES**  
BRUSSELS



**A cooperative process to reach mutually acceptable solutions that respects all stakeholders, improves our common understanding and establishes sustainable mutual trust**

# **PRELIMINARY PROGRAMME**

# DAY 1: Wednesday, 22 February 2017

## 09.00 to 18.30

09.00 – 13.00	<p><b>INTRODUCTION &amp; OPENING PLENARY</b>  <b>EXPRESSION OF STAKEHOLDER INTERESTS</b></p> <p><i>Live video streaming</i></p> <p><i>Co-Chairs: Charles Barker, PrimeMover Associates, USA (confirmed) &amp; Peter O'Donnell, Politico, Belgium (confirmed)</i></p>
09.00 - 09.15	<p><b>Welcome &amp; Introduction</b> Charles Barker, PrimeMover Associates, USA (confirmed)</p>
09.15 – 09.30	<p><b>Setting the scene</b> Yann Le Cam, Chief Executive Officer, EURORDIS (confirmed)</p>
09.30 – 09.40	<p><b>Patient case study</b> Speaker to be named</p>
09.40 – 09.55	<p><b>The role of the European Commission on improving access to rare disease therapies</b>  Xavier Prats Monné, Director General, Directorate-General for Health and Food Safety, European Commission (confirmed)</p>
09.55 – 11.25	<p><b>PANEL discussion:</b> moderated by Co-Chairs</p> <p><b>Industry decision-makers</b></p> <p>What have been the most important changes in the last year?  What are the challenges?  What are the options moving forward?  How do we work together to improve this?</p> <p><b>Panellists (confirmed):</b>  Michael Goettler, Global President, Rare Disease Business, Pfizer  Tuomo Päätsi, President EMEA, Celgene  Additional panellists and questioners to be named  Q&amp;A from on-site and online audiences</p>
11.25 – 11.40	<p><i>Coffee break</i></p>
11.40 – 13.00	<p><b>PANEL discussion:</b> moderated by Co-Chairs</p> <p><b>Policy decision-makers</b></p> <p>What have been the most important changes in the last year?  What are the challenges?  What are the options moving forward?  How do we work together to improve this?</p> <p><b>Panellists (confirmed):</b>  Diane Kleineremans, representative of the Minister of Public Health, Belgium  Additional panellists and questioners to be named  Q&amp;A from on-site and online audiences</p>

13.00 – 14.00	<i>LUNCH (Salon Adolphe Max)</i>
14.00 – 18.30	<b>PLENARY</b>
14.00 – 14.10	<b>Patient case study</b> Speaker to be named
14.10 – 14.25	<b>Collaborating for success</b> Karen Facey, HTAi, UK (confirmed)
14.25 – 14.55	<b>Current state of the art in multi-stakeholder collaborative processes</b> Early dialogue initiatives & expedited regulatory pathways <b>Speakers (confirmed):</b> Hans-Georg Eichler, Senior Medical Officer, EMA Simon Day, Chair, IRDiRC Small-Patients Clinical Trial Task Force, Director, Clinical Trials Consulting & Training Limited, UK Additional speakers to be named
15.10 – 16.15	<b>Multi-stakeholder panel discussion: success factors for collaboration in relation to access</b> <b>Panellists (confirmed):</b> Hans-Georg Eichler, Senior Medical Officer, EMA Ri de Ridder, Director General, RIZIV/INAMI, Belgium Additional panellists to be named Q&A from on-site and online audiences
16.15 – 16.30	<i>Coffee break</i>
16.30 – 18.00	<b>From diverging positions to common interests</b> <b>A collaborative conversation for transformative operational solutions serving the interests of each stakeholder</b> Moderator: Charles Barker, PrimeMover Associates, USA (confirmed)
18.00 – 18.30	<b>Conclusions from Day 1</b> Peter O'Donnell, Politico, Belgium (confirmed)
18.30	End of Day 1

## **DAY 2: Thursday, 23 February 2017**

**08.00 – 18.00**

08.00 – 09.00	<b>PLENARY</b> <i>Co-Chairs: to be named</i>
08.00 – 08.15	<b>Trajectory from Day 1 &amp; Aims of Day 2</b> Session Chairs

08.15 – 08.25	<b>Patient case study</b> Speaker to be named		
08.25 – 08.45	<b>Introduction to breakout sessions</b> Moderators of breakouts		
09.00 – 11.00	<b>BREAKOUT SESSIONS – Emerging options</b>		
	<b>Breakout 1: Quality Data Generation</b>	<b>Breakout 2: Value for money across Europe</b>	<b>Breakout 3: Pricing</b>
	<b>Pan-European disease &amp; product registries to address needs of all stakeholders</b> Moderator & Rapporteur to be named	<b>Common principles for value determination and assessment</b> Moderator: Lieven Annemans, Ghent University, Belgium (tbc) Rapporteur: to be named	<b>Innovative performance based outcome agreements</b> Moderator & Rapporteur to be named
11.00 – 11.15	<i>Coffee break</i>		
11.15 – 12.30	<b>PLENARY</b> <b>Co-Chairs: to be named</b>		
11.15 – 12.30	<b>Feedback and discussion from breakout sessions</b> Co-Chairs & rapporteurs from breakouts		
12.30 – 13.30	<i>LUNCH (Salon Adolphe Max)</i>		
13.30 – 15.30	<b>BREAKOUT SESSIONS - New options</b>		
	<b>Breakout 4: Quality Data Generation</b>	<b>Breakout 5: Value for money across Europe</b>	<b>Breakout 6: Pricing</b>
	<b>How European Reference Networks (ERNs) could become part of the solution / enablers of quality data generation?</b> Moderator & Rapporteur to be named	<b>Proposals for coordination of HTA across Europe: implications for rare diseases</b> Moderator to be named Rapporteur: Julia Chamova, Director, Global Networks (EMEA), ISPOR, Sweden (confirmed)	<b>Potential for European collaboration among payers and companies</b> Moderator: Ri de Ridder, Director General, RIZIV/INAMI, Belgium (confirmed) Rapporteur to be named
15.30 – 15.45	<i>Coffee break</i>		
15.45 – 18.00	<b>PLENARY</b> <b>Co-Chairs: to be named</b>		
15.45 – 16.45	<b>Feedback and discussion from breakout sessions</b> Co-Chairs & rapporteurs from breakouts		
16.45 – 17.45	<b>Panel discussion: Paving the way to a fair, inclusive and on-going multi-stakeholder approach with the potential to generate sustainable, affordable and actionable improvements in patient access to rare disease therapies</b> Panellists to be named		

	Q&A
17.45 – 18.00	<b>Conclusions &amp; closing remarks</b> Yann Le Cam, Chief Executive Officer, EURORDIS
18.00	End of Day 2 – End of symposium