

3<sup>RD</sup> MULTI-STAKEHOLDER

Symposium

13-14 FEBRUARY 2019  
CROWNE PLAZA HOTEL-LE PALACE  
BRUSSELS, BELGIUM

ON IMPROVING  
PATIENTS' ACCESS  
TO RARE DISEASE  
THERAPIES



Let's make a pact to ensure patients' sustainable access  
to rare disease therapies

# PROGRAMME

## **DAY 1: Wednesday, 13 February 2019**

(morning session live video streamed)

**09.00 to 17.00**

09.00 – 11.00	<b>INTRODUCTION &amp; OPENING PLENARY SESSION</b> <i>Why a pact?</i>
09.00 – 09.15	<b>Opening</b>
09.15 – 09.30	<b>Welcome and opening remarks</b> Yann Le Cam, Chief Executive Officer, EURORDIS
09.30 – 09.45	<b>Keynote Speech</b>
09.45 – 09.55	<b>Patient testimony</b> Mencía de Lemus, President, SMA Europe
09.55 – 10.15	<b>Overall framework for investment in life sciences developments for rare disease therapies</b> Nathalie Moll, Director General, EFPIA Co-presenter to be named
10.15 – 10.45	<b>Coffee break</b>
10.45 – 12.05	<b>PANEL discussion: outlining interests by the key constituencies</b> <b>Moderator:</b> Sheela Upadhyaya, Associate Director of the Highly Specialised Technology Programme, NICE <b>Industry perspective:</b> Alexander Natz, Secretary General, EUCOPE <b>Patient perspective:</b> Dimitrios Athanasiou, DMD Patient Advocate, UPPMD Board Member, EMA Patient Expert in DMD Additional panelists to be named
12.05 – 13.00	<b>Presentations on areas of agreement and remaining challenges</b>
13.00 – 14.00	<b>Lunch</b>
14.00 – 15.30	<b>Interactive parallel sessions</b>
	<b>BREAKOUT SESSION 1</b> A new blueprint to spend efficiently and fast-track R&D
	<b>BREAKOUT SESSION 2</b> Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value

	<b>Moderator:</b> Karen Facey, Evidence Based Health Policy Consultant, Senior Research Fellow, University of Edinburgh
15.30 – 16.00	<i>Coffee break</i>
16.00 – 16.30	<b>Feedback session (plenary)</b>
16.30 – 17.00	<b>Wrap-up Day 1</b>

## **DAY 2: Thursday, 14 February 2019**

**09.00 – 14.30**

09.00 – 09.15	<b>Welcome back: take home messages from Day 1 and expectations for Day 2</b>
09.30 – 11.00	<p><b>Interactive parallel sessions</b></p> <p><b>BREAKOUT SESSION 3</b>  A transparent European cooperation framework between national healthcare systems for the determination of fair prices and of sustainable healthcare budget impacts  <b>Moderator:</b> Anna Bucsecs, Project Advisor, MoCA  <b>Rapporteur:</b> Simone Boselli, Public Affairs Director, EURORDIS-Rare Diseases Europe</p> <p><b>BREAKOUT SESSION 4</b>  A continuum approach to evidence generation linked to healthcare budget spending  <b>Moderator:</b> Prof. Eileen Treacy, Clinical Lead, Irish National Rare Diseases Clinical Programme; Director, Irish National Rare Diseases Office; Clinical Professor- Inborn Errors of Metabolism, Trinity College Dublin; Full Clinical Professor-University College Dublin; (Irish representative EC ERN Board of Member States)  <b>Rapporteur:</b> Chris Sotirelis, EURORDIS-Rare Diseases Europe Volunteer</p>
11.00 – 11.30	<i>Coffee break</i>
11.30 – 12.00	<b>Feedback session (plenary)</b>
12.00 – 13.30	<b><i>CLOSING PLENARY: From design to implementation</i></b>
12.00 – 13.00	Panel discussion

13.00 – 13.30	Wrap-up & concrete next steps: EURORDIS
13.30 – 14.30	<b><i>Farewell lunch</i></b>