

3RD MULTI-STAKEHOLDER

Symposium ON IMPROVING
PATIENTS' ACCESS
TO RARE DISEASE
THERAPIES

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



EURORDIS
RARE DISEASES EUROPE

**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	INTRODUCTION & OPENING PLENARY SESSION <i>Why a pact?</i>
09.00 – 09.15	Opening
09.15 – 09.30	Welcome and opening remarks Yann Le Cam, Chief Executive Officer, EURORDIS-Rare Diseases Europe
09.30 – 09.45	Keynote Speech Tiberius-Marius Brădăţan, Secretary of State, Ministry of Health of Romania, representing Sorina Pinteau, Minister of Health of Romania
09.45 – 09.55	Patient testimony Mencía de Lemus, President, SMA Europe
09.55 – 10.15	Overall framework for investment in life sciences developments for rare disease therapies Nathalie Moll, Director General, EFPIA Co-presenter to be named
10.15 – 10.45	Coffee break
10.45 – 12.05	PANEL discussion: outlining interests by the key constituencies Moderator: Sheela Upadhyaya, Associate Director of the Highly Specialised Technology Programme, NICE Policymakers perspective: Diane Kleinermans, Advisor to the Ministry of Health and Social Affairs; Ministry of Public Health and Social Security; National Institute for Health and Disability Insurance (INAMI-RIZIV) Industry perspective: Alexander Natz, Secretary General, EUCOPE Patient perspective: Dimitrios Athanasiou, DMD Patient Advocate, UPPMD Board Member, EMA Patient Expert in DMD Investors perspective: Stijn Vanacker, Portfolio Manager, Robeco Global Stars & Analyst - Global Healthcare Additional panelists to be named
12.05 – 13.00	Presentations on areas of agreement and remaining challenges Breakout 1: Victoria Hedley, Rare Disease Policy Manager, Newcastle University; John Walton Muscular Dystrophy Research Centre, MRC Centre for Neuromuscular Diseases, Institute of Genetic Medicine Breakout 2: Karen Facey, Evidence Based Health Policy Consultant, HTAi UK

	<p>Breakout 3: Anna Bucsecs, Project Advisor, MoCA</p> <p>Breakout 4: Eileen Treacy, ERN Board of Member States; Mater Misericordiae University Hospital, Clinical Professor, Paediatrics, Trinity College Dublin</p>
13.00 – 14.00	Lunch
14.00 – 15.30	<p>Interactive parallel sessions</p> <p>BREAKOUT SESSION 1: A new blueprint to spend efficiently and fast-track R&D</p> <p>Rare diseases R&D in Europe can be improved to overcome fragmentation, leading to more effective use of data and resources, faster scientific progress to decrease unnecessary hardship and improve the lives of people living with a rare disease. In this specific context, it is timely to maximize the potential of already funded tools, networks, projects and programmes by supporting them further, scaling them up, linking them together, and most importantly, adapting them to the needs of end-users through implementation tests in real settings. This session will discuss how such a concerted effort is necessary to develop a sustainable ecosystem allowing a virtuous circle between RD care, research and medical innovation.</p> <p>Discussion: <i>How to speed up clinical trials: greater acceptance of new models</i> How to develop faster and more efficiently (methods for clinical trials)</p> <p>Paediatric research and Asterix: Hanneke van der Lee, Clinical Epidemiologist, Academic Medical Center (AMC)</p> <p>Making the link between paediatrics and ERNs: Mark Turner, Co-coordinator, University of Liverpool, Conect4Children</p> <p>Moderator: Virginie Bros-Facer, Scientific Director, EURORDIS-Rare Diseases Europe</p> <p>Rapporteur: Victoria Hedley, Rare Disease Policy Manager, Newcastle University; John Walton Muscular Dystrophy Research Centre, MRC Centre for Neuromuscular Diseases, Institute of Genetic Medicine</p> <p>BREAKOUT SESSION 2: Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value</p> <p>Early Dialogues and Scientific Advice initiatives have traditionally provided authoritative advice to medicine’s developers on specific questions about their confirmatory clinical trial(s). Over the years, the advice from regulators and scientific experts has evolved to be more of a two-way dialogue that can include patients and a range of agencies such as HTA/payers and to consider issues beyond the confirmatory trial. Such dialogue is essential in the development of treatments for rare diseases where traditional clinical development programmes may not be possible and where wider evidence</p>

	<p>sources may be needed to determine the value of a treatment. Patients' views are needed to focus on outcomes that matter and ensure study feasibility, but understanding how patients and their representatives can be involved in these technical discussions over the life cycle of treatment is not straightforward. This session will take views from all stakeholders on a fictitious case study to consider the potential approaches for improving these dialogues.</p> <p>Developer perspective: Adam Parnaby, Senior Director, Market Access Policy Worldwide Markets, Celgene</p> <p>Moderator: Karen Facey, Evidence Based Health Policy Consultant, Senior Research Fellow, University of Edinburgh</p> <p>Rapporteur: Birthe Holm, President, Rare Diseases Denmark</p>
15.30 – 16.00	Coffee break
16.00 – 16.30	Feedback session (plenary)
16.30 – 17.00	Wrap-up Day 1

DAY 2: Thursday, 14 February 2019

09.00 – 14.30

09.00 – 09.15	Welcome back: take home messages from Day 1 and expectations for Day 2
09.30 – 11.00	<p>Interactive parallel sessions</p> <p>BREAKOUT SESSION 3: A transparent European cooperation framework between national healthcare systems for the determination of fair prices and of sustainable healthcare budget impacts</p> <p>Full and quick access to therapies remains difficult across Europe for people living with a rare disease. Difficulties in negotiating the monetisation of the value</p>

of these therapies is one of the key issues to address, one where increased collaboration at European level could bear fruits. This session aims to provide an overview of what the current situation is, what is currently working and applying potential solutions workable throughout the continent for the benefit of the patients. The discussion will aim to respond to question such as:

- Can a collaborative framework for negotiation could be put in place?
- How do we negotiate a fair price at EU level, based for example on experience such as MoCA?
- Can collaborative and voluntary experiences such as BeNELuxAI be scaled up and made sustainable?
- Is it possible to move to a European table of negotiation?

Academic perspective: Lieven Annemans, Senior Full Professor of Health Economics, Interuniversity Center for Health Economics Research (ICHER), Ghent University

Yann Le Cam, Chief Executive Officer, EURORDIS-Rare Diseases Europe

Moderator: Anna Bucsecs, Project Advisor, MoCA

Rapporteur: Simone Boselli, Public Affairs Director, EURORDIS-Rare Diseases Europe

BREAKOUT SESSION 4: A continuum approach to evidence generation linked to healthcare budget spending

This session aims to address some of the challenges and barriers to approval for access and reimbursement of orphan medicinal products in the post-approval phase, addressing the following questions:

- How can we measure the required meaningful health outcomes for patients to enable informed reimbursement decisions?
- What can be the role of Patient Centres Outcomes Measures (PCOMs) (or PROMs)?
- How to involve multiple stakeholders in data sharing and ways to facilitate this collaboration?
- What are the realistic expectations of risk-sharing reimbursement models?

Measuring what matters to patients (PROMs and other indicators from IRDiRC task force outcomes): Thomas Morel, Director, Global Patient-Centred Outcomes Research & Policy, UCB

NIH model to be used with ERNs (repurposing/incentives + data sharing for companies/IP): Diego Ardigo, R&D Rare Diseases Unit Head, Chiesi Group

Real-world examples: DMD-Hercules project (collaboration with industry + defining own outcomes): Emily Crossley, Co-founder and joint-CEO, Duchenne UK

	<p>Moderator: 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)</p> <p>Rapporteur: Chris Sotirelis, EURORDIS-Rare Diseases Europe Volunteer</p>
11.00 – 11.30	<i>Coffee break</i>
11.30 – 12.00	Feedback session (plenary)
12.00 – 13.30	<i>CLOSING PLENARY: From design to implementation</i>
12.00 – 13.00	Panel discussion
13.00 – 13.30	Wrap-up & concrete next steps: EURORDIS
13.30 – 14.30	<i>Farewell lunch</i>