

3RD MULTI-STAKEHOLDER

Symposium

ON IMPROVING

PATIENTS' ACCESS

TO RARE DISEASE

THERAPIES

13-14 FEBRUARY 2019

CROWNE PLAZA HOTEL-LE PALACE

BRUSSELS, BELGIUM



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
09.30 – 09.45	Keynote Speech
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 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 Breakout 3: Anna Bucsics, Project Advisor, MoCA Breakout 4: Eileen Treacy, ERN Board of Member States; Mater Misericordiae University Hospital, Clinical Professor, Paediatrics, Trinity College Dublin

13.00 – 14.00	Lunch
14.00 – 15.30	<p data-bbox="427 304 813 342">Interactive parallel sessions</p> <p data-bbox="427 409 1436 479">BREAKOUT SESSION 1: A new blueprint to spend efficiently and fast-track R&D</p> <p data-bbox="427 544 1465 925">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 data-bbox="427 987 1460 1057">Moderator: Virginie Bros-Facer, Scientific Director, EURORDIS-Rare Diseases Europe</p> <p data-bbox="427 1075 1465 1184">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 data-bbox="427 1207 1404 1276">BREAKOUT SESSION 2: Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value</p> <p data-bbox="427 1341 1465 1881">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 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 data-bbox="427 1944 1417 2013">Moderator: Karen Facey, Evidence Based Health Policy Consultant, Senior Research Fellow, University of Edinburgh</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 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:</p> <ul style="list-style-type: none"> • 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? <p>Moderator: Anna Bucsecs, Project Advisor, MoCA Rapporteur: Simone Boselli, Public Affairs Director, EURORDIS-Rare Diseases Europe</p>

	<p>BREAKOUT SESSION 4: A continuum approach to evidence generation linked to healthcare budget spending</p> <p>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:</p> <ul style="list-style-type: none"> • 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? <p>Moderator: 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)</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>