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#RareEU2019

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

#RareEU2019

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

A EURORDIS **RARE DISEASE DAY®** EVENT

IN PARTNERSHIP WITH

HTAi HEALTH TECHNOLOGY ASSESSMENT INTERNATIONAL
PGEU GPUE
EPF European Patients Forum
hope
eahp european association of hospital pharmacists
ISPOR Improving healthcare decisions
efpia European Federation of Pharmaceutical Industries and Associations
EuropaBio The European Association for Bioindustries
EUCOPE European Confederation of Pharmaceutical Entrepreneurs AISBL

RATIONALE & OBJECTIVES

EURORDIS-Rare Diseases Europe is organising this 3rd Multi-Stakeholder Symposium to build on the outcomes of the previous multi-stakeholder meetings held in 2016 and 2017.

We will use the collaborative multi-stakeholder approach set out in the position paper 'Breaking the Access Deadlock to Leave No-one Behind' (January 2018) to develop and garner broad endorsement for a 'roadmap' document. The roadmap will offer solutions and set out commitments by all players in the rare disease community to accelerate the development of effective therapies and guarantee timely and universal access to them.

Timing for this initiative is of particular importance: European Parliamentary elections will take place in May 2019 and the related changes in the European institutions, in particular at the European Commission, will subsequently take place. The ongoing discussion around the establishment of the Multiannual Financial Framework for the period 2021 – 2027 provides additional opportunities.

Therefore, it is of essence for the rare disease community to have a clear and shared 'roadmap' that both highlights the critical need for a European vision and offers practical solutions that can be implemented by the current and future European parliamentarians by 2025.

DAY 1
WEDNESDAY 13 FEBRUARY 2019
09.00 to 17.00

Morning session until 13:00 live video streamed and open to press

09.00 – 11.00 OPENING PLENARY SESSION
Why a pact?

09.00 – 09.15 Introduction
Moderator: Sandra Nestler-Parr, Co-Chair, Rare Disease Special Interest Group, ISPOR; Trustee, Alpha-1 UK Support Group; Head of Market Access (TTR), Akcea Therapeutics

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 Brădățan**, Secretary of State, Ministry of Health of Romania

09.45 – 9.55 There is a new treatment approved, when can I get it? The example of SMA: **Mencía de Lemus**, President, SMA Europe

09.55 – 10.15 Overall framework for investment in life sciences developments for rare disease therapies
Anthony Humphreys, Head Of Sector, Regulatory Affairs Committee, Support and Community Procedures, European Medicines Agency
Nathalie Moll, Director General, EFPIA

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
Regulatory perspective: Anthony Humphreys, Head Of Sector, Regulatory Affairs Committee, Support and Community Procedures, European Medicines Agency
Policymakers'/payers' 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)
Healthcare industry perspective: Alexander Natz, Secretary General, EUCOPE and **Nathalie Moll**, Director General, EFPIA
Patient perspective: Dimitrios Athanasiou, Board Member, WORLD DUCHENNE ORGANIZATION / UPPMD
Investors' perspective: Stijn Vanacker, Global Healthcare Portfolio Manager, ROBECO

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
Breakout 3: Anna Bucsics, Project Advisor, MoCA
Breakout 4: 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; Irish representative EC ERN Board of Member States

13.00 – 14.00 ***Lunch***

14.00 – 15.30 Interactive parallel sessions

Room: Clarity & Vision, 8th floor

BREAKOUT SESSION 1: A new blueprint to spend efficiently and fast-track R&D

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 rare disease care, research and medical innovation.

Moderator: Virginie Bros-Facer, Scientific Director, EURORDIS-Rare Diseases Europe
Rapporteur: Victoria Hedley, Rare Disease Policy Manager, Newcastle University; John Walton Muscular Dystrophy Research Centre, MRC Centre for Neuromuscular Diseases, Institute of Genetic Medicine
Speaker: Hanneke van der Lee, Clinical Epidemiologist, Academic Medical Center (AMC)
Speaker: Mark Turner, Professor/Consultant in Neonatology, University of Liverpool, Co-Coordinator, Conect4Children
Speaker: Ralf-Dieter Hilgers, Institute Director, University Hospital Aachen
Speaker: Andreas Jung, Senior Physician Pulmonology, Children's Hospital Zurich

Room: Ballroom, ground floor

BREAKOUT SESSION 2: Improving Multi-Stakeholder Early Dialogues to Optimize Determination of Value

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 to consider the potential approaches for improving these dialogues.

Moderator: Karen Facey, Evidence Based Health Policy Consultant, HTAi
Rapporteur: Birthe Holm, President, Rare Diseases Denmark
Speaker: Kristina Larsson, Head of Orphan Medicines, EMA
Speaker: Margaret Galbraith, Project Manager, Haute Autorité de Santé (HAS), EUnetHTA
Speaker: Adam Parnaby, Senior Director, Market Access Policy Worldwide Markets, Celgene Sarl
Speaker: François Houÿez, Information & Access to Therapies Director & Health Policy Advisor, EURORDIS-Rare Diseases Europe

15.30 – 16.00 ***Coffee break***

16.00 – 16.30 Feedback session (plenary)
Rapporteurs from Breakout Sessions 1 & 2

16.30 – 17.00 Wrap-up Day 1
Sandra Nestler-Parr, Co-Chair, Rare Disease Special Interest Group, ISPOR; Trustee, Alpha-1 UK Support Group; Head of Market Access (TTR), Akcea Therapeutics

DAY 2
THURSDAY 14 FEBRUARY 2019
09.00 to 14.30

09.00 – 09.30 Welcome back: take home messages from Day 1 and expectations for Day 2
Sandra Nestler-Parr, Co-Chair, Rare Disease Special Interest Group, ISPOR; Trustee, Alpha-1 UK Support Group; Head of Market Access (TTR), Akcea Therapeutics

09.30 – 11.00 Interactive parallel sessions

Room: Ballroom, ground floor

BREAKOUT SESSION 3: A transparent European cooperation framework between national healthcare systems for the determination of fair prices and of sustainable healthcare budget impacts

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:

- Can a collaborative framework for negotiation 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 BeNELuxA be scaled up and made sustainable?
- Is it possible to move to a European table of negotiation?

Moderator: Anna Bucsics, Project Advisor, MoCA
Rapporteur: Simone Boselli, Public Affairs Director, EURORDIS-Rare Diseases Europe
Speaker: Angela McFarlane, Market Development Director, IQVIA
Speaker: Lieven Annemans, Senior Full Professor of Health Economics, Interuniversity Center for Health Economics Research (ICHER), Ghent University
Speaker: Alexander Natz, Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)
Speaker: Valérie Paris, Senior Health Policy Analyst – Project Leader, Organisation for Economic Co-operation and Development (OECD)
Speaker: Yann Le Cam, Chief Executive Officer, EURORDIS-Rare Diseases Europe

Room: Clarity & Vision, 8th floor

BREAKOUT SESSION 4: A continuum approach to evidence generation in post approval phase 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 Centred 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?

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; Irish representative EC ERN Board of Member States
Rapporteur: Chris Sotirelis, Patient representative, EURORDIS-Rare Diseases Europe
Speaker: Josie Godfrey, Director, JG Zebra Consulting
Speaker: Thomas Morel, Director, Global Patient-Centred Outcomes Research & Policy, UCB
Speaker: Diego Ardigò, R&D Rare Diseases Unit Head, Chiesi Group

11.00 – 11.30 ***Coffee break***

11.30 – 12.00 Feedback session (plenary)
Rapporteurs from Breakout Sessions 3 & 4

12.00 – 13.30 CLOSING PLENARY
Moderator: Sandra Nestler-Parr, Co-Chair, Rare Disease Special Interest Group, ISPOR; Trustee, Alpha-1 UK Support Group; Head of Market Access (TTR), Akcea Therapeutics

12.00 – 12.15 Keynote Speech
Andrzej Rys, Director, Health Systems, Medical Products and Innovation, Directorate General Health and Food Safety (DG SANTE), European Commission

12.15 – 13.00 Panel discussion, from design to implementation
Breakout session moderators:
Breakout Session 1: Virginie Bros-Facer, Scientific Director, EURORDIS-Rare Diseases Europe
Breakout Session 2: Karen Facey, Evidence Based Health Policy Consultant, HTAi
Breakout Session 3: Anna Bucsics, Project Advisor, MoCA
Breakout Session 4: 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; Irish representative EC ERN Board of Member States

13.00 – 13.30 Wrap-up & concrete next steps: **Yann Le Cam**, Chief Executive Officer, EURORDIS-Rare Diseases Europe

13.30 – 14.30 ***Farewell lunch***