31st ERTC Workshop of the EURORDIS Round Table of Companies

Pharmaceutical Strategy for Europe: sustainable therapeutic development to leave no rare disease behind

Overarching theme: In the context of the ongoing consultation on the new public health programme from the European Commission, this workshop will convene patients, policy makers, regulators, industry and payers to reflect on and discuss ways in which Europe's competitiveness and attractiveness can be enhanced and safeguarded. Exchanges will also focus on key elements of the EU Regulation on orphan medicinal products which can help to shape a sustainable ecosystem addressing rare disease patients' unmet medical needs.

Workshop overview:

Taking stock of 20 years of therapeutic development in rare diseases and looking at the forecast if we keep operating in a static environment, we will look at the changes needed to fulfil patients' unmet medical needs and to unlock the potential of research & development for currently disregarded diseases.

In particular, we will reflect on reframing the system of incentives within - and outside of - the EU regulation on orphan medicinal products, so that Europe provides an attractive environment to de-risk investment in medicines development. We will examine how multi-stakeholder collaboration can help to bridge public and private research and how important it is to ensure that an early dialogue with regulators, HTA bodies and payers is taking place.

Beyond the regulation, we will discuss which conditions are needed to ensure a sustainable therapeutic development and a coordinated and accurate funding agenda/investment strategy, which places an emphasis on pre-competitive knowledge generation and on the efforts required to reconcile development and access to medicines.

The discussions will also reflect on a common European approach that highlights the importance of continuous data generation to support health systems in providing equitable access to rare disease treatment and care.

In this workshop, we will call on decision makers at all levels and across stakeholder groups to commit to and develop the necessary framework and mechanisms to ensure people living with a rare disease in Europe ultimately benefit from the therapies that are developed.

Let us collectively recognise the need to implement new policies that are fit-for-purpose to actively design our preferred future for people living with a rare disease, and make Europe the next world leader in research and access for rare disease medicines.

31st ERTC Workshop of the EURORDIS Round Table of Companies

22-23 MARCH 2021
12.00 - 19.00 CET

DAY 1: Explore incentives linked to unlocking the potential of research and development in rare diseases and in particular, in disregarded areas

DAY 1: OBJECTIVES

Identify the changes needed in the rare disease R&D environment to fulfil unmet medical needs

Discuss how to reframe the system of incentives within - and outside of - the EU Regulation on OMP so that Europe provides an attractive environment to de-risk investment in medicines development

Explore how to unlock the potential of research and development for currently disregarded diseases and ensure sustainable therapeutic development so to reconcile development and access to medicines

OPENING SESSION

12:00 – 12:10	**DAY 1 Facilitator**: **Gerrit Heijkoop,** Live Online Events
12:10 – 12.20	**Welcome introduction, setting the scene & goals for the workshop** **Yann Le Cam**, Chief Executive Officer, EURORDIS-Rare Diseases Europe
12.20 – 12.30	**Patient testimony** **Angelo Loris Brunetta,** Thalassaemia International Federation
12.30 – 12.45	**Small group introductions and reactions**

SESSION 1: Setting the scene

Objective: *Taking stock of 20 years of therapeutic development in Rare Diseases, of the forecast in a static environment and of the changes needed to fulfil patient unmet medical needs*

12.45 - 12.55	**Setting the stage** **Moderator: Anna Kole**, EURORDIS-Rare Diseases Europe
12.55 - 13.15	**Key findings of the Rare 2030 study performed by Imperial College London** including projections and areas where no progress is forecast if the environment remains static and an analysis of inequalities **Marisa Miraldo,** Imperial College London Audience interaction
13.15 - 13.35	**Impact assessment – EU legislation on medicines for rare diseases and paediatric medicines: where we are,** including an analysis of the main trends observed from the contributions received to date **Tidde Goldhoorn,** European Commission Audience interaction
COMFORT BREAK	
13.45 – 13.55	**Patient perspective** **Simone Boselli,** EURORDIS-Rare Diseases Europe
13.55 – 14.25	**Panel discussion:** how to ensure the EU Regulation on Orphan Medicinal Products addresses the Rare 2030 recommendations taking into account the results of the studies and consultations presented above **Panel moderator: Anna Kole,** EURORDIS-Rare Diseases Europe • **Simone Boselli**, EURORDIS-Rare Diseases Europe • **Martine Zimmerman**, Alexion • **Maurizio Scarpa**, MetabERN • **Tidde Goldhoorn**, European Commission • **Julia Sabine Wahl**, Copenhagen Economics
14.25 – 14.35	**Small group discussions**
14.35 - 14.50	**Questions & Answers with the Panel**
14.50 – 14.55	**ENERGISER**

BREAK

SESSION 2: Introducing potential solutions

Objective: *Discuss how to reframe the system of incentives within - and outside of - the EU Regulation on OMP so that Europe provides an attractive environment to de-risk investment in medicines development*

15.00 – 15.05	**Introduction** **Moderator: Elizabeth Vroom**, World Duchenne Organisation (UPPMD) & EURORDIS Board of Directors
15.05 – 15.25	**How to better shape the eco-system: the new ILAP Scheme** **Daniel O'Connor**, The Medicines and Healthcare Products Regulatory Agency (MHRA) Audience Interaction
15.25 – 15.45	**A multi-criteria decision approach for ranking unmet needs in healthcare** **Irina Cleemput**, Belgian Health Care Knowledge Centre (KCE) Audience Interaction
15.45 – 15.55	**Case Study: MOCA – how the transparency value framework has been used** **Emanuele Degortes,** Vifor Fresenius Medical Care Renal Pharma Ltd.
15.55 – 16.15	**Small group discussions and feedback**
BREAK	
16.25 – 16.55	**Panel Discussion:** the importance of early dialogue / criteria to define unmet medical needs for decision-making / transparency value framework – how this resonates with the current reflection around the revision of the EU Regulation on Orphan Medicinal Products • **Violeta Stoyanova-Beninska,** Chair, EMA Committee on Orphan Medicinal Products (COMP) • **Pauline Evers,** Levenmetkanker (Live With Cancer Movement) • **Angelo Loris Brunetta,** Thalassaemia International Federation • **Emanuele Degortes,** Vifor Fresenius Medical Care Renal Pharma Ltd.
16.55 – 17.15	**Questions from participants**
17.15 – 17.20	**Personal reflections**

SESSION 3: How to unlock the potential of currently disregarded diseases and ensure sustainable therapeutic development

Objective: *Explore how to unlock the potential of currently disregarded diseases and ensure sustainable therapeutic development so to reconcile development and access to medicines*

17.20 – 17.25	**Introduction** **Moderator: Virginie Hivert,** EURORDIS-Rare Diseases Europe
	Interview-style conversations with developers – Perspectives around collaborative approaches that help to address unmet medical needs and to forge new pathways along the R&D lifecycle
17.25 – 17.35	**Diego Ardigò**, Head of R&D, Global Rare Diseases, Chiesi Farmaceutici S.p.A
17.35 – 17.45	**Leah Bloom**, Senior Vice President, External Innovation and Strategic Alliances, Novartis Gene Therapies
BREAK	
17.55 – 18.05	**Mattias Rudebeck**, Global Medical Science Director, Genetics & Metabolism, Sobi
18.05 – 18.15	**Arianna Greco,** Senior Vice President, Head of International Legal, Alnylam
18.15 – 18.45	**Panel Discussion**
18.45 – 19.00	**Wrap-up, key messages and next steps**

DAY 2: Explore incentives linked to unlocking access

DAY 2: OBJECTIVES

Recognise the urgency and need to implement new policies that are fit-for-purpose to actively design our preferred future for people living with a rare disease

Understand the benefits of a common European approach to access rare disease treatments beyond the regulation

Acknowledge the importance of continuous data generation to support health systems in providing equitable access to treatment and care

SESSION 1: Problem Statement

Objective: *"The only thing that stays the same is everything changes", yet we still face the same difficulties we've been facing for the past decade. What key challenges can we approach with a fresh spirit and new ideas? Significant inequalities and systemic failure to address the needs of people living with a rare disease still exist across Europe despite tremendous strides in technology. Only by implementing new policies fit-for-purpose for this decade can we actively design our preferred future for people living with a rare disease.*

	DAY 2 Facilitator: Tamsin Rose, Friends of Europe
12.00 – 12.10	**Interactive welcome** **Facilitator: Tamsin Rose,** Friends of Europe
12.10 – 12.20	**Recap of main elements from Day 1 and bridge to Day 2** **Moderator: Simone Boselli**, EURORDIS-Rare Diseases Europe
12.20 – 12.40	**Case study: Cystic Fibrosis** **Hilde De Keyser,** CF Europe Audience interaction
12.40 – 13.00	**Case study: Osteogenesis Imperfecta** **Ingunn Westerheim,** OIFE Audience interaction

13.00 – 13.20	**EFPIA Patients W.A.I.T. indicator 2020/21 survey results** **Max Newton**, IQVIA Audience interaction
13.20 – 13.40	**Rare Barometer: Access to treatment: unequal care for European rare disease patients** **Sandra Courbier**, EURORDIS-Rare Diseases Europe Audience interaction
13.40 – 14.00	**Small group discussions**
BREAK	

SESSION 2: Introducing potential solutions

Objective: *Establishing streamlined regulatory, pricing and reimbursement policies is the only viable way forward. These policies should encourage a continuum of evidence generation along the full life cycle of a product or technology as well as the patient journey from diagnosis to treatment access. A European ecosystem able to attract investment in areas of unmet need, foster innovation, and address the challenges of healthcare system sustainability is required to provide equitable access to care.*

14.15 – 14.20	**ENERGISER** **Tamsin Rose,** Friends of Europe
14.20 – 14.25	**Introduction** **Moderator: Sheela Upadhyaya,** Rare Disease Expert
14.25 – 14.55	**Presentation of policy proposals from Expert Group on Orphan Medicines Incentives** **Julia Sabine Wahl**, Copenhagen Economics Audience Interaction
14.55 – 15.15	**How to embed real world evidence into the process example** **Toon Digneffe**, Takeda Audience Interaction
15.15 – 15.35	**Future HTA process for the benefit of patients** **Matteo Scarabelli**, EURORDIS-Rare Diseases Europe Audience Interaction
15.35 – 15.45	**Industry perspective: Role of differential pricing in improving access** **Ansgar Hebborn,** Roche Audience Interaction

15.45-16.00	**Small group discussions**
BREAK	
16.05 – 16.25	**Panel Discussion: Exploring ways to develop a sustainable European ecosystem to provide equitable access to care** • **Julia Sabine Wahl,** Copenhagen Economics • **Toon Digneffe**, Takeda • **Matteo Scarabelli**, EURORDIS-Rare Diseases Europe • **Ansgar Hebborn,** Roche
16.25-16.50	**Reflections and 'Asks' from breakouts + additional Q&A**
16.50-16.55	**Closing reflections & considerations for next session** **Moderator: Sheela Upadhyaya,** Rare Disease Expert
16.55 – 17.00	**ENERGISER** **Tamsin Rose,** Facilitator
BREAK	

SESSION 3: Call to action

Objective: *We're asking decision makers at all levels and across stakeholder groups to commit to and develop the necessary framework and mechanisms to ensure equitable access to care for people living with a rare disease in Europe.*

17.10 – 17.15	**Introduction** **Moderator: Tamsin Rose,** Friends of Europe
17.15 – 18.30	**Panel discussion:** How to coordinate activities on access and innovative processes? • **Yann Le Cam,** EURORDIS-Rare Diseases Europe • **Pierre Delsaux**, European Commission • **Maurizio Scarpa**, MetabERN • **Edith Frenoy**, EFPIA • **Alexander Natz,** EUCOPE
18.30 – 19.00	**Wrap-up, key messages and next steps**