# 33rd Workshop of the EURORDIS Round Table of Companies (ERTC)

# Laying the ground for European Action on Rare Diseases: the role of industry

## Overarching theme

Recent EURORDIS Round Table of Companies (ERTC) discussions placed an emphasis on the need for a new ecosystem in Europe that addresses unmet medical needs and ensures sustainability for healthcare systems. In a context of fast-evolving technological advances and scientific promises that have the potential to dramatically improve lives, persisting inequalities remain for the 30 million Europeans living with a rare disease for whom the absence of or lack of access to an approved treatment has yet to be addressed.

Building on previous advocacy milestones in rare diseases, this upcoming workshop will be an opportunity to reflect further on these persisting inequalities through focusing our discussion on key solutions:

- A common European fund to support the generation of evidence across the whole life cycle of products and to ensure attractiveness of the European R&D ecosystem for rare diseases
- A consolidated and structured cooperation in Europe on pricing and negotiations

Through these propositions, EURORDIS invites industry to think beyond the revision of the EU general pharmaceuticals legislation by supporting a coordinated and concerted European effort to achieve a renewed policy framework on rare diseases.

## Workshop objectives

This workshop will bring together participants spread across pharmaceutical, biotech and clinical research organisations (CRO) together with pharmaceutical and biotech syndicates, patient advocates, regulators, healthcare professionals and academics with the following objectives:

- **Enhance** understanding of the proposed new policy framework for European Action on Rare Diseases and its key elements
- **Engage** participants for preparedness of achieving the proposed new policy framework on rare diseases
- **Consider** the work performed by the EURORDIS-EFPIA structured dialogue on improvements to access to rare diseases therapies
- **Contextualize** two key proposals (EU fund for generation of evidence and structured cooperation) for improved access to medicines in the context of a comprehensive policy framework for rare diseases that goes beyond current soft/hard legislation relevant to availability, accessibility, and affordability of treatments for rare diseases
- **Provide** a platform for expression on points of consensus and concerns within EURORDIS' two key proposals cited above
- **Prepare** participants for the 11th European conference on rare diseases and orphan products (ECRD 2022)

# PROGRAMME

**27-28 April 2022**
**14.00-18.00 CET**

## DAY 1: EU Fund for generation of evidence across the whole life cycle of an orphan medicinal product

**Day 1 Co-Chairs:**

**Dimitrios Athanasiou**, World Duchenne Organization
**Vittoria Carraro**, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)

## OPENING SESSION

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| 14:00 – 14:10 | **Welcome and Introduction**<br>**Bo Kruger,** Facilitator |
| 14:10 – 14:25 | **European Action on Rare Diseases**<br>**Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe |

## SESSION 1: What and why an EU fund for generation of evidence?

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| 14:25 – 14:45 | **What is the problem, how can an EU fund help?**<br>**Simone Boselli,** Public Affairs Director, EURORDIS-Rare Diseases Europe |
| 14:45 – 15:00 | **Case study: Thalassaemia International Federation**<br>**Androulla Eleftheriou**, Executive Director, Thalassaemia International Federation |
| 15:00 - 15:25 | **Small group discussions: Pros and cons of this type of EU approach**<br>**Bo Kruger,** Facilitator |

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| 15:25 – 15:45 | **Highlights and questions from small group discussions**<br>**Co-Chairs:**<br>**Dimitrios Athanasiou,** Board of Directors, World Duchenne Organization<br>**Vittoria Carraro,** Associate Director Government Affairs, EUCOPE |
| **15:45 – 16:00** | **COMFORT BREAK** |

## SESSION 2: How will EU fund for generation of evidence work?

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| 16:00 – 16:50 | **Panel discussion: How to eliminate roadblocks and create the change needed?**<br>**Moderator: Tresja Bolt,** Senior Healthcare Consultant, Harwood Levitt Consulting<br>Identify key problems/themes around assessment, therapeutic options available and funding. Pros and cons debate.<br>1. Regulator: **Ana Hidalgo,** Head of Advanced Therapies, European Medicines Agency (EMA)<br>2. Payer: **Hans Georg Eichler,** Consulting Physician, Association of Austrian Social Security Bodies<br>3. Patient Expert: **Loris Brunetta**, Board Member, Thalassaemia International Federation<br>4. Industry: **Francis Pang,** Vice President of Global Market Access, Orchard Therapeutics<br>5. Payer: **Sheela Upadhyaya,** Associate Director Highly Specialised Technologies, National Institute for Health and Care Excellence (NICE) |
| 16:50 – 17:00 | **Q&A between participants and panellists** |
| 17:00 – 17:10 | **COMFORT BREAK** |
| 17:10 – 17:20 | **Scoping document: vision and scope**<br>**Tina Taube,** Director Market Access & Orphan Drug Policy Lead, European Federation of Pharmaceutical Industries and Associations (EFPIA)<br>**Simone Boselli,** Public Affairs Director, EURORDIS-Rare Diseases Europe |
| 17:20 – 17:40 | **Small group discussions** |
| 17:40 – 17:50 | **Highlights from small group discussions** |

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| 17:50 – 18:00 | **Closing summary & next steps**<br>**Bo Kruger,** Facilitator<br>**Co-Chairs:**<br>**Dimitrios Athanasiou,** Board of Directors, World Duchenne Organization<br>**Vittoria Carraro,** Associate Director Government Affairs, EUCOPE |

# DAY 2: A consolidated and structured cooperation in Europe on pricing and negotiations

**Day 1 Co-Chairs:**

**Dimitrios Athanasiou**, World Duchenne Organisation
**Tina Taube**, European Federation of Pharmaceutical Industries and Associations (EFPIA)

## OPENING SESSION

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| 14:00 – 14:10 | **Welcome and Introduction**<br>**Bo Kruger,** Facilitator |

## SESSION 1: How can we achieve equitable access to rare disease therapies?

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| 14:10 – 14:25 | **How do we move forward and envisage something different?**<br>**Vicky McGrath**, Chief Executive Officer, Rare Diseases Ireland |
| 14:25 – 15:10 | **Panel Discussion: How can we achieve equitable access by improving processes?**<br>Moderators**: Dimitrios Athanasiou,** Board of Directors, World Duchenne Organization & **Tina Taube,** Director Market Access & Orphan Drug Policy Lead, EFPIA<br>1. Patient perspective: **Declan Noone,** President, European Haemophilia Consortium<br>2. Industry experience: **Oswald Bentinck**, Vice President - Head of Market Access EMEA, Novartis Gene Therapies<br>3. Experience/learnings Beneluxa (payer): **Eveline Klein Lankhorst**, Coordinator Managed Entry Agreements, Dutch Ministry of Health |

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| 15:10 – 15:30 | **Q&A between participants and panellists** |
| 15:30 – 15:45 | **COMFORT BREAK** |
| 15:45 – 16:10 | **Breakout groups** |
| 16:10 – 16:25 | **Highlights from small group discussions** |

## SESSION 2: What do we want to build upon?

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| 16:25 – 16:55 | **What do we want to build upon?**<br>**Co-Chair: Dimitrios Athanasiou,** Board of Directors, World Duchenne Organization<br>• Regulator: **Anja Schiel**, Special Adviser, NoMA and Alternate Member, Scientific Advice Working Party (SAWP), European Medicines Agency (EMA)<br>• Patient Expert: Building to a joint action with payers: **Yann Le Cam,** Chief Executive Officer, EURORDIS-Rare Diseases Europe |
| 16:55 – 17:05 | **COMFORT BREAK** |
| 17:05 – 17:20 | **The Next Generation of Rare Disease Drug Policy: Ensuring Both Innovation and Affordability**<br>**Caroline Pearson,** Senior Vice President, National Opinion Research Center (NORC), University of Chicago |
| 17:20 – 17:40 | **Facilitating more efficient negotiation for innovative therapies, a value-based negotiation framework**<br>**Amanda Whittal,** Senior Consultant, Dolon<br>**Julien Patris,** Country Manager Belgium & Luxembourg, Head of Policy International Markets, Alnylam |
| 17:40 – 17:50 | **Self-reflection and key learnings from Day 2**<br>**Bo Kruger,** Facilitator |
| 17:45 – 18:00 | **Closing summary & next steps**<br>**Co-Chairs:**<br>**Tina Taube,** Director Market Access & Orphan Drug Policy Lead, EFPIA<br>**Dimitrios Athanasiou,** Board of Directors, World Duchenne Organization<br>**Bo Kruger**, Facilitator |