ADVANCED PROGRAMME

# 39th Workshop of the EURORDIS Round Table of Companies (ERTC)

25th February 2025, 09.30-16:30 CET
DoubleTree by Hilton, Brussels

# Addressing systemic inequalities through patient-driven innovation & research

Recent discussions have emphasised the urgent need to sustain innovation and enhance research investment to help Europe regain its global competitive advantage. While this approach could revitalise Europe's public health system, the rare disease community has consistently called for a shift towards needs-driven innovation, guided by collective responsibility and equity-focused investment decisions.

As the new college of European Commissioners begins its five-year term, accompanied by the development of a new financial framework, this workshop will explore how a patient-centred approach to research and innovation can address systemic inequalities and reduce gaps in health equity.

**This workshop aims to:**

- Demonstrate the added value of the rare disease patient community in shaping research, development and access pathways;
- Review our community's advocacy asks by addressing barriers to needs-led innovation;
- Explore opportunities to embed these priorities into future European research policies and funding programmes.

**Programme at a glance:**

Opening remarks will argue for a greater policy shift towards needs-driven research and innovation, where collective responsibility guides investment decisions, reduces gaps in health equity and helps revitalise Europe's public health system. Participants will be invited to reflect and review progress and shortcomings towards delivering on this scenario, as initially set out by the rare disease community in the Rare 2030 recommendations (2021).

Smaller group discussions will then focus on how to add value and create solutions across the value chain to influence future policies and funding mechanisms on the following topics:

**Morning parallel sessions:**

- Accelerating clinical trials to ensure research innovations translate into tangible health benefits
- Capitalising on public-private investments to foster patient-driven rare disease research

**Afternoon parallel sessions:**

- Bridging the gap: strengthening European healthcare systems for equitable patient access;
- Advancing equity and diversity in diagnosis.

This workshop will help us re-affirm that innovation driven by a collective voice to address unmet needs will not only benefit the rare disease community but will also bring in its wake crucial healthcare advances for society as a whole.

| 9.00 - 9.30 | Registration |
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| 9.30 - 9.40 | **Welcome & Introduction by the Co-Chairs**<br>• **Pauline Evers,** Patient Advocate, Dutch Federation of Cancer Patient Organisations<br>• **Gabriella Almberg,** Global Head of Health System Policy & Public Affairs, UCB Pharma |
| 9.40 – 9.50 | **Setting the scene**<br>• **Virginie Bros-Facer,** Chief Executive Officer, EURORDIS - Rare Diseases Europe |
| 9.50 - 10.00<br>10.00 - 11.00 | **Opening plenary: 5 years until 2030**<br>**EURORDIS keynote**<br>• **Valentina Bottarelli,** Public Affairs Director - Head of European Advocacy, EURORDIS - Rare Diseases Europe<br>**A multi-stakeholder perspective**<br>• **Carmen Laplaza Santos,** Head of Unit Health Innovations and Ecosystems, European Commission<br>• **Avril Daly,** President, EURORDIS - Rare Diseases Europe & Chief Executive Officer of Retina International<br>• **Nathalie Moll,** Director General, the European Federation of Pharmaceutical Industries and Associations (EFPIA)<br>• **Alexander Natz,** Secretary General, European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)<br>**Q&A** |
| 11.00–11.30 | Morning Comfort Break |
| | **Morning Break-out Sessions** |

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| 11.30– 12.30 | **Parallel session 1: Accelerating clinical trials to ensure research innovations translate into tangible health benefits**<br>The ACT-EU (Accelerating clinical trials) aspiration is to bring better, faster and optimised clinical trials by improving the clinical trials environment in the European Union through harmonisation, innovation and collaboration with stakeholders.<br><br>In this session, we will explore which strengths and solutions we can leverage to make clinical trials more conducive for health benefits of people living with a rare disease. Amongst topics to be discussed are patient engagement in clinical trials design and set-up, recruitment and retention of participants in clinical trials, innovative approaches for small population clinical trials, and trials optimization.<br><br>• **Kristina An Haack**, Senior Global Project Head Lysosomal Storage Disorders & Neuromuscular Diseases, Sanofi<br>• **Denis Lacombe,** Director General, European Organisation for Research and Treatment of Cancer (EORTC)<br>• **François Houyez,** Information & Access to Therapies Director & Health Policy Advisor, EURORDIS<br>• **Sabine Furst-Recktenwald**, Principal Medical Director, Precision Safety, Roche<br><br>**Parallel session 2: Capitalising on public-private investments to boost patient-led rare disease research**<br>While the concept of public-private partnerships in rare diseases research is widely accepted by the rare disease ecosystem, translating it into practice and having an efficient and systematic approach to it still remains to be achieved.<br><br>In this session, speakers will discuss the current bottlenecks that hinder optimal public-private collaborations as well as innovative models of patient-driven innovation and how those could be replicated and upscaled. The acceleration hub of the newly funded research alliance ERDERA to accelerate research funded by public bodies to enable further public-private collaborations will also be introduced.<br><br>• **Magda Chlebus,** Executive Director Science Policy & Regulatory Affairs, The European Federation of Pharmaceutical Industries and Associations (EFPIA)<br>• **Alexandre Méjat,** Deputy Director – International Scientific Networks, AFM-Telethon & EURORDIS - Rare Diseases Europe Board Member<br>• **Graham Slater**, EURORDIS - Rare Diseases Europe Board Member<br>• **Matt Bolz Johnson,** Mental Health & Wellbeing Lead and Healthcare Advisor, EURORDIS - Rare Diseases Europe |
| 12.30-13.30 | **Lunch** |

| | Afternoon Break-out Sessions |
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| 13:30-14:45 | **Parallel session 1: 'Bridging the gap': strengthening European healthcare systems for equitable patient access**<br>Can supply diversity, innovation incentives, and novel payment and pricing models advance sustainable access to therapies and more resilient healthcare systems? While stakeholders agree on their importance, implementation challenges remain. This session will provide a collaborative space to explore solutions and unlock their potential for sustainable rare disease ecosystem. To set the stage, findings from several research efforts—including a multi-stakeholder study on the costs and benefits of innovative payment models—will be presented before a panel discussion.<br><br>Panel discussions will explore ways to improve access, with a particular focus on advanced therapies, while fostering innovation and ensuring the sustainability and resilience of European healthcare systems.<br><br>• **Yannis Natsis,** Director, European Social Insurance Platform<br>• **Mikel Berdud,** Senior Principal Economist, Office of Health Economics (OHE)<br>• **Mariette Driessens,** Policy Officer, VSOP - Patientenkoepel voor zeldzame aandoeningen<br>• **Diana Sinkevich,** Vice President, Market Access EU & International & Global HEOR, Rare Diseases, Chiesi<br>• **Anja Schiel,** Senior Adviser; Lead Methodologist in Regulatory and Pharmacoeconomic Statistics, Norwegian Medical Products Agency (NOMA)<br>• **Lourdes Álvarez Callejo,** Public Servant of Common Portfolio of the NHS and Pharmacy, Spanish Ministry of Health<br><br>**Parallel session 2: Advancing equity in newborn screening**<br><br>This session will explore whether achieving equity in newborn screening (NBS) requires improvements to traditional screening methods or broader systemic changes. Can optimizing existing NBS programs bridge gaps in access, or are deeper policy and regulatory shifts needed? What role do public trust, ethical considerations, and healthcare systems play in making NBS truly accessible to all?<br><br>The session will also focus on genetic NBS and discuss whether the advances in technology can ensure better access. Speakers will discuss new genetic NBS pilots, public dialogues, and changes in policy and regulations that are needed to enable large-scale implementation of genetic NBS. The session will also look into practical steps for organizing a viable and broadly acceptable genomic NBS program, and how innovations can translate into meaningful, equitable outcomes for all newborns.<br><br>• **Maria Martinez-Fresno,** Director Medical Affairs, Illumina<br>• **Tamara Dangouloff,** Project Manager Baby Detect - CRMN Liege, Belgium |

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| | • **Zhana Chokheli** Patient Advocate, Georgian Alliance for Rare Diseases |
| 14.45 – 15.00 | **Coffee Break** |
| | **Closing plenary** |
| 15:00 – 16:00 | **Wrap up remarks & key takeaways** |
| 16:00 – 16:30 | **Farewell from the Co-Chairs** |