37th Workshop of the EURORDIS Round Table of Companies (ERTC)

21 February 2024

09.30-17.00 CET

DoubleTree by Hilton Brussels City

Transforming Rare Disease Foresight into Action

How can additional measures support the development of available, accessible and affordable treatments for rare diseases patients?

Overarching goal and scope

As we are all looking to the details of the revision of the EU general pharmaceutical legislation, we know that **no change will happen without the leadership from academics, researchers, patient groups and developers** to make the most of opportunities arising from an ever-evolving healthcare environment.

This workshop will invite participants to **think beyond the legislation**, reflecting on rare disease patients' expectations of the proposed reform and their understanding of its shortcomings, taking stock of key milestones along our advocacy journey for patient engagement, and discussing how additional instruments can further support the development of and access to orphan and paediatric medicines.

Drawing on the Rare 2030 recommendations (February 2021), which set out the roadmap towards a new, future-proof policy framework for rare diseases in Europe, this workshop will bring forward and discuss the **added value of three disruptive instruments**, designed to help streamline the medicines development path and better integrate patients in the decision-making processes that affect their lives:

- Orphan Drug Development Plan:

This plan is both a concept and a tool derived from the Orphan Drug Development Guidebook, which the International Rare Diseases Research Consortium (IRDiRC) originally created to help developers navigate the rare disease ecosystem. It includes a series of guiding principles on how to start and conduct efficient (and sustainable) drug development in rare diseases until initially identified unmet medical needs are addressed. It also ensures that patient input is captured all along the medicines lifecycle.

- PRIority MEdicines (PRIME) scheme:

To enhance early dialogue with developers, the European Medicines Agency (EMA) established PRIME, which is based on a combination of iterative scientific advice and provisions for the accelerated assessment of promising medicines that address unmet medical needs. Whilst this voluntary scheme could allow for better patient involvement, it has proven to be of crucial benefit for rare diseases at the time of orphan drug designation and market authorisation.

- Mechanism of Coordinated Access to Orphan Medicinal Products initiative:

Another example of early dialogue involving developers, patients and payers, the Mechanism of Coordinated Access (MoCA) to Orphan Medicinal Products aims to support a more equitable access to rare disease therapies. 10 years after this voluntary framework was established, it is time to learn from experience and expand this pilot to a more structured and sustained platform, and to explore ways in which it can interplay with the two previous instruments.

Throughout the day, sessions will demonstrate how our community has positioned these instruments in rare disease advocacy, how these have evolved in light of the revision of the legislation and how the underlying concepts within these instruments can be adapted to bring us closer to the goal of **developing 1000 new therapies by 2030** for the benefit of rare disease patients and society at large.

This workshop will conclude by placing these three tools into broader context, reflecting on perspectives from across the rare disease community (including paediatric onset, rare cancers), and against the backdrop of the EU Regulation on Health Technology Assessment and the ongoing reflections around improved collaboration between Member States in negotiations on pricing and reimbursement and single procurement.

PROGRAMME

Co-Chairs:
Tomasz Grybek, Foundation of Borys the Hero, Poland and EURORDIS Board of Directors
Kevin Loth, Managing Director, WDLBio Ltd.

9.00 – 9.30	**REGISTRATION**
9.30 – 9.45	**Welcome & Introduction by the Co-chairs**
9.45 – 10.00	**Setting the scene** **Simone Boselli,** Public Affairs Director, EURORDIS-Rare Diseases Europe
10.00 – 10.20	**An Orphan Drug Development Plan as a Process to Streamline the Path to Rare Disease Patients** **Virginie Hivert,** Therapeutic Development Director, EURORDIS-Rare Diseases Europe **Michela Gabaldo,** Member of the IRDiRC Regulatory Scientific Committee
10.20 – 10.40	**PRIority MEdicines (PRIME): Navigating Lessons and Future Legislative Horizons** **Pauline Evers,** Policy Officer, Dutch Federation of Cancer Patient Organisations, NFK **Kevin Cunningham,** PRIME Scientific Coordinator at European Medicines Agency
10.40 – 11.00	**Multistakeholder Early Dialogues to Facilitate Patient Access.** **María Cavaller Bellaubi,** Patient Engagement & Therapeutic Development Director, EURORDIS-Rare Diseases Europe **Anna Bucsics,** Project Advisor for MoCA (Mechanism of Coordinated Access to Orphan Medicinal Products)
11.00–11.30	MORNING COMFORT BREAK
11.30-12.50	**Reality Check and Future Ambitions. Panel and interactive discussion.** **Moderator: Simone Boselli,** Public Affairs Director, EURORDIS-Rare Diseases Europe **Kristina Larsson,** Head of Orphan Medicines at the European Medicines Agency **Violeta Stoyanova-Beninska,** Chair of the Committee for Orphan Medicinal Products (COMP) **Johan Pontén**, Senior Manager International Affairs, Dental and Pharmaceutical Benefits Agency, TLV

	Laura Liebers, Director Policy & Intelligence, International Regulatory team, Vertex **Mark Waker**, Patient Access Lead, UK & Ireland, Mereo BioPharma
12.50-13.00	**Morning Wrap-up and Personal Reflection by the Co-chairs**
13.00-14.30	**LUNCH**
14.30-14.50	**Do Development, Review and Reimbursement Frameworks Need Adapting to Improve Evidence Generation and Financially Sustainable Access for Rare Disease Products?** **Tina Wang,** Senior Manager- HTA programme and Strategic Partnership, CIRS
14.50-16.15	**Authorised, but Accessed? Panel and interactive discussion.** **Moderator: Kevin Loth**, Managing Director, WDLBio Ltd. **Jakub Dvořáček,** Deputy Minister for the Ministry of Health of the Czech Republic **Yann Le Cam**, Chief Executive Officer, EURORDIS-Rare Diseases Europe **Benedetta Baldini,** Senior Policy Advisor, European Social Insurance Platform, ESIP **Delphine Heenen,** Childhood Cancer International Europe Committee Member **Anne-Sophie Chalandon,** Head of Global Rare Diseases and CGT Policy, Sanofi **Ludovic Helfgott,** Executive Vice President and Head of Rare Disease, Novo Nordisk
16.15 – 16.30	**AFTERNOON COMFORT BREAK**
16.30 – 16.40	**Key Takeaways from a Healthcare Corporate** **Dakota Fisher-Vance,** Associate Director, Global Patient Advocacy, Biocryst Pharmaceuticals
16.45-16.55	**Summary and Key Takeaways from a Patient Advocate** **Daniel de Vincente,** President, ASMD Spain, FEDER and EURORDIS Board of Directors
16:55-17:00	**Closing and Next Steps** **Kevin Loth,** Managing Director, WDLBio Ltd.