

## EURORDIS Round Table of Companies (ERTC)

# Digital Transformation as a Catalyst of Change for Rare Diseases

### Overarching theme

EURORDIS has increasingly been advocating for the need to build a more resilient health data ecosystem that acknowledges the specificities of rare disease data and responds to the high unmet needs of our patient community.

A year ago, our workshop discussions already touched on the impact of health data on the regulation of medicines, in light of the fast developments of science, technology and data analytics. **In our upcoming workshop, we will build the discussion further around health data access and sharing.**

In the context of the European Commission's proposal for a regulation on a European Health Data Space (EHDS), EURORDIS is actively reflecting on the opportunity this legislation represents to address the challenges and expectations of the rare disease community – both in terms of developing robust standards to ensure secure, ethical and responsible data sharing and allowing health data to be seamlessly shared across borders.

This legal act could become a game changer for digital transformation of healthcare in Europe. Not only will it empower people to access and share their health data in a common and safe space, it has also the potential to improve diagnosis, advance research and innovation in new medicines, support policy-making and regulatory activities, and reinforce healthcare delivery.

### Background

On 3rd May 2022, the European Commission launched the proposal for a Regulation for the European Health Data Space (EHDS). The European Health Data Space is a health specific ecosystem comprised of rules, common standards and practices, infrastructures and a governance framework that aims at:

- empowering individuals through increased digital access to and control of their electronic personal health data, at national level and EU-wide, and support to their free movement, as well as fostering a single market for electronic health record systems, relevant medical devices and high-risk AI systems (*primary use of data*)
- providing a consistent, trustworthy and efficient set-up for the use of health data for research, innovation, policy-making and regulatory activities (*secondary use of data*)

The creation of such an ecosystem is especially relevant for the rare disease community. There are 6000 various rare diseases affecting over 30 million people across Europe, but only 6% of all known rare diseases have curative treatment. The rare disease community has many unmet needs. The limited number of patients alongside the scarcity of available knowledge and



expertise on these diseases make rare diseases a field that could greatly benefit from a Regulation on EHDS.

Once adopted, the legal act could become a fundamental game changer for digital transformation of healthcare in the EU and offer new opportunities that ultimately improve lives through enhanced and safe access to data to improve diagnosis, treatment, care and research for rare disease patients.

### Workshop objectives

This workshop will explore the opportunities and challenges of the upcoming European Health Data Space Regulation, as well as develop concrete solutions on how to make digital transformation truly advantageous for the rare disease community and all the other players in healthcare with the following objectives:

- **Explore** the role of data and innovation in healthcare and rare diseases;
- **Deepen** the understanding of the European Health Data Space Regulation and its link with the current legal system;
- **Learn** from each other about secondary health data uses and the potential of AI and machine learning to assess real-world medical information and support healthcare decision-making for rare diseases;
- **Reflect** on the safe ways to strengthen and expand the reuse of health data for research and innovation purposes in the health sector within the context of the upcoming legislative changes;
- **Debate** and gain new insights on technical and ethical issues in relation to health data sharing;
- **Gain** practical knowledge on the EU decision-making and get acquainted with the positions of different stakeholders on digital healthcare transformation;
- Have an opportunity to **network**, build connections and share perspectives and experience with other participants.

## FINAL PROGRAMME

19 October 2022

09.00-17.00 CET

### Co-Chairs:

Dorica Dan, President, Romanian National Alliance for Rare Diseases  
Elin Haf Davies, Chief Executive Officer, Aparito

8.30 – 9.00	<b>Registration</b>
9.00 - 9.15	<b>Welcome &amp; Introduction</b> <b>Dorica Dan</b> , President, Romanian National Alliance for Rare Diseases <b>Elin Haf Davies</b> , Chief Executive Officer, Aparito
9.15 – 9.30	<b>Setting the scene</b> <b>Yann Le Cam</b> , Chief Executive Officer, EURORDIS-Rare Diseases Europe
9.30 – 10.00	<b>Introduction to the European Health Data Space Proposal</b> <b>Jerome de Barros</b> , Policy Officer, European Commission
10.00-10.30	<b>Transferability of Real-World Evidence and the Value of Health Technologies</b> <b>Bertalan Nemeth</b> , Senior Health Economist, Syreon Research Institute
10.30-10.40	<b>Fair data sharing without oversharing</b> <b>Nawel Lalout</b> , Project Manager, FAIR Duchenne Data, World Duchenne Organization
10.40-11.10	<b>Digital Health Tools, Challenges &amp; Best Practices – Industry perspectives</b>  <b>David Palacios Valero</b> , Executive Director Patient Advocacy & Engagement, Astellas Gene Therapies <b>Kathrin Langguth</b> , Head Digital Pharma Policy, Bayer AG Pharmaceuticals <b>Anne-Sophie Chalandon</b> , Head of Global Public Affairs, Rare Diseases & ATMP Policy, Specialty Care, Sanofi <b>Irene Kanter-Schlifke</b> , General Manager Patient Solutions, Biogen Digital Health <b>Jennifer Pougnet</b> , Global Data Policy Strategy Lead, Roche
11.10-11.45	<b>Networking Coffee Break</b>
11.45-12.30	<b>Panel discussion: Ethical aspects of secondary health data uses</b> <ol style="list-style-type: none"><li><b>Daniel Theisen</b>, Director, ALAN - Maladies Rares Luxembourg</li><li><b>Teodora Lalova-Spinks</b>, PhD researcher, KU Leuven</li><li><b>Zoi Kolitsi</b>, Senior Researcher, Digital Health Strategies, I-HD The European Institute of Innovation through Health Data</li><li><b>Pauline McCormack</b>, Senior Lecturer, Newcastle University</li></ol>



12.30 – 12.40	<b>Introduction to role play exercise</b> <b>Jelena Malinina</b> , Patient Data Director, EURORDIS-Rare Diseases Europe
<b>12.40-14.00</b>	<b>LUNCH</b>
14.00-15.30	<b>Exploring the European Health Data Space Proposal in practice (Role play exercise)</b>
<b>15.30 – 16.00</b>	<b>Refreshments</b>
16.00-16.30	<b>Role Play Conclusions</b>
16.30-16.40	<b>Key take home messages from a healthcare corporate</b> <b>Sergio Diaz</b> , Patient Advocacy Manager Europe, IQVIA
16.40-17.00	<b>Wrap-up, Key messages &amp; Next steps</b>