#RARE DISEASE DAY

RARE DISEASE DAY®

IMPACT OF RARE DISEASES: MORE THAN YOU CAN IMAGINE

5 MARCH, 2025 13:30 – 15:30

JÓZSEF ANTALL (JAN) 4Q1

Hosted by

MEP Stine Bosse, Renew Europe

MEP Adam Jarubas, EPP

Objectives of the meeting

To mark Rare Disease Day 2025 and following the launch of a new European Parliament Intergroup on Cancer and Rare Diseases, MEPs Stine Bosse (Renew, Denmark), Adam Jarubas (EPP, Poland) and EURORDIS-Rare Diseases Europe are organising a special event entitled ***Impact of Rare Diseases: More than you can imagine***. This event aims to recapitulate the issue of rare diseases to EU policymakers at the start of this new political mandate. In particular, it seeks to **foster a deeper understanding of the challenges faced** by people living with rare diseases and their families, while presenting **concrete EU-level solutions** to address these issues effectively.

Taking place at the European Parliament in Brussels, this event is part of a broad range of activities **organised for the annual Rare Disease Day**, a globally coordinated movement advocating for equal opportunities, healthcare access, and timely diagnosis and treatment for people living with rare diseases, regardless of where they live. This year's Rare Disease Day theme, "More than you can imagine," highlights the **profound and far-reaching impact of rare diseases** on individuals, on their families but also on society as a whole if no concrete measures are taken to address them.

Therefore, throughout the event, **thanks to the participation of key EU policymakers, experts and patient representatives**, we will:

- Present the **impact of living with a rare disease** on individuals and their families, while examining the broader effects of rare disease on healthcare systems, social structures, and society at large.
- Illustrate first-hand experiences with the **latest data and evidence**, offering a comprehensive understanding of the current realities faced by the rare disease community.
- Outline **actionable policy solutions** at the European level, focusing on legislative and non-legislative measures that should be prioritised and adopted during this EU mandate.

Event Programme

Moderator: Enrique Terol, Health Counsellor, Permanent Representation of Spain to the EU

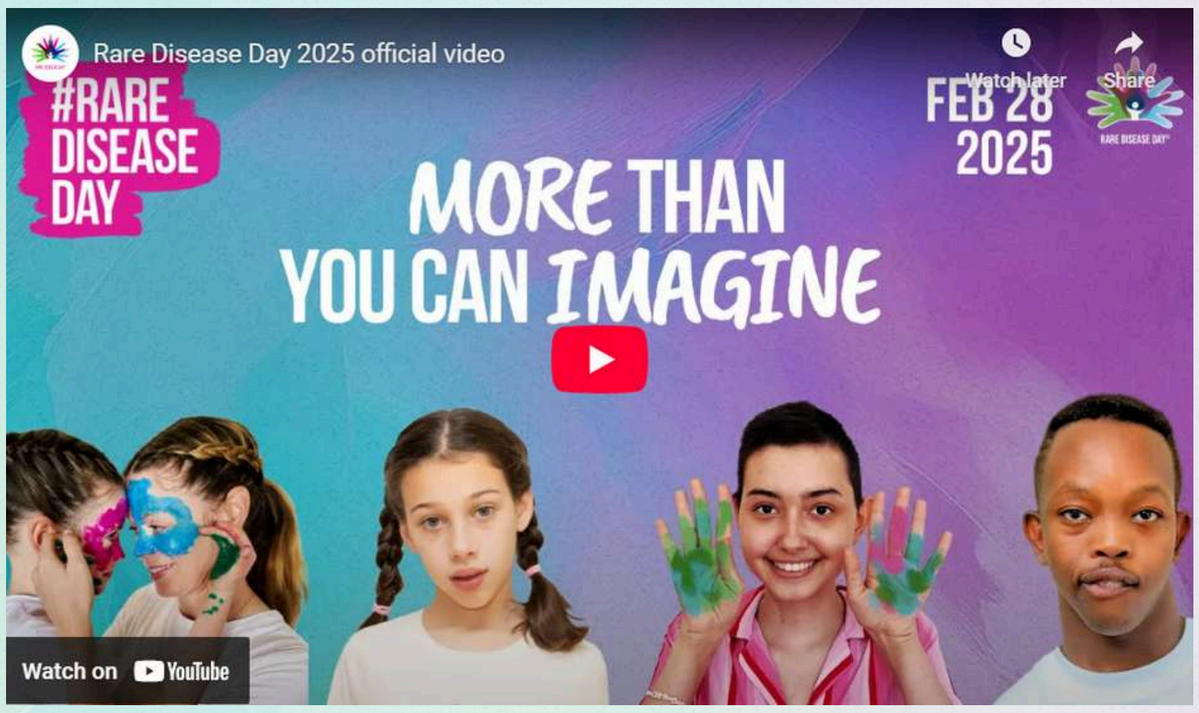
Setting the scene - *13:30-14:00*

Introduction
MEPs Adam Jarubas (EPP) & Stine Bosse (Renew)
Introduction and welcome.

Video of RDD
EURORDIS 2025 Rare Disease Day video.

Setting the scene
Adéla Odrihocká, Rare Diseases Czech Republic
Reflection key results from EURORDIS Rare Barometer surveys as seen through the lens of personal experience.

Impact of Rare Diseases: facts and figures
Tim Wilsdon, Vice President, Charles River Associates
Overview of key figures related to study on impact of RDs.



Watch the Rare Disease Day video here:

Tripartite sessions - *14:00-15:10*

THE DIAGNOSIS OF RARE DISEASES TAKES

5 YEARS ON AVERAGE

Easier, faster and more accurate diagnosis of rare diseases

- *Olaf Riess, Coordinator, Solve-RD, and Centre for Rare Diseases, Tübingen*
- *Thomas Minten, KU Leuven*
- *MEP Vlad Voiculescu (Renew)*

The **diagnosis of rare diseases takes an average of five years**, with young patients and women facing even longer delays. This prolonged "diagnostic odyssey" highlights the **need for tailored strategies to improve diagnosis rates, reduce waiting times, and address inequities**. Limited national capacities for genetic testing and unequal access to evolving technologies contribute to disparities across Europe.

To ensure equal diagnostic opportunities, **EU-level initiatives should enhance cross-country collaboration**, particularly in newborn screening. Strengthening Centres of Expertise (CEs) and European Reference Networks (ERNs) is essential to **streamline diagnoses and improve outcomes** for rare disease patients.

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FEWER THAN 5%

OF KNOWN RARE DISEASES HAVE AN APPROVED PHARMACOTHERAPY

Development and Access to treatments for people living with rare diseases: EU cooperation and support needed

- *Sebastian Honoré, Co-Founder, Cure Lowe Foundation*
- *Stefano Benvenuti, IRDiRC member and Head of Public Affairs and Market Access, Fondazione Telethon*
- *MEP Stine Bosse (Renew)*

It is estimated that **fewer than 5%** of the known rare diseases have at least one approved pharmacotherapy. This issue is compounded by **unequal access to treatments across the EU** due to high costs, healthcare disparities, and reimbursement challenges.

Addressing these inequalities requires **promoting rare disease research, improving funding and coordination**, **and ensuring treatments are accessible and affordable**. Upcoming EU pharmaceutical legislation should balance encouraging innovation with equitable access, involve patients in decision-making, and strengthen cooperation on pricing and procurement to create **a more unified and fair approach** to rare disease care across the region.

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8 IN 10 PEOPLE

WITH RARE DISEASES LIVE WITH DISABILITIES

Holistic care and support to address the impact of living with RDs

- *Adéla Odrihocká, Rare Diseases Czech Republic*
- *Karsten Vanden Wyngaert, Department of Nephrology, Ghent University Hospital and ERKNet Member*
- *MEP Tilly Metz (Greens)*

8 in 10 people with rare diseases live with disabilities, but their disabilities are often not adequately recognised, limiting their access to support. People with rare diseases are also **three times more likely to experience unhappiness and depression** and face **nearly four times higher unemployment rate** than the EU's general population.

These converging limiters to full social participation mean that it is even more key for the rare disease community to have access to **person-centred, lifelong, and multidisciplinary care and support**.

Policymakers should support implementing guidelines to **improve care and support** for the rare disease community, ensuring their inclusion in EU mental health policies, and tackling unemployment gaps through the upcoming review of the European Pillar of Social Rights Action Plan.

Concluding remarks - *15:10-15:30*

EESC Representative
Ágnes Cser, European Economic and Social Committee rapporteur
Institutional remarks linking to latest EESC Opinion on Rare Diseases.

More than you can imagine
Virginie Bros-Facer, CEO, EURORDIS-Rare Diseases Europe
Summary of cross-cutting issues and need for EU Action Plan, as well as continued financial support in the upcoming MFF.

Concluding remarks
MEP Stine Bosse (Renew)
Wrap-up and goodbye.

What next?

For more information on this event and on EURORDIS' advocacy:

Contact Rachel, Public Affairs Manager :*rachel.butcher@eurordis.org*

For more data from the rare disease community:

See EURORDIS Rare Barometer surveys: *eurordis.org/rare-barometer*

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