

# Reframe Rare

## A Rare Disease Day Policy Event

Tuesday 18<sup>th</sup> February 2020

15.00 – 17.00

Room ASP5E3, European Parliament, Brussels

### About rare diseases and Rare Disease Day

Rare diseases affect **30 million people in the EU**, 5% of the EU population, or the **population of Belgium and the Netherlands combined**. The estimate only increases when considering the carers, families and friends who need to support their loved ones.

Rare diseases are chronic, highly complex, progressive and severely disabling, frequently affecting life expectancy and generating specific care needs. Due to their low prevalence, little is known about most rare diseases. As a result, they are poorly diagnosed and their symptoms under-recognised in healthcare and social systems. Even when diagnosis is possible the vast majority of rare diseases do not

have a cure or even a treatment, and research to advance care and develop treatments is limited.

**Ultimately, the 30 million people living with a rare disease in the EU represent a significant group of citizens that need support to be integrated into society.**

OVER  
**6000**  
distinct rare diseases

Affects approx.  
**5%**   
of the population in  
the course of their lives

Each one affects  
fewer than  
**1 IN**  
**2000** PEOPLE

**NO**  
**CURE**   
for the vast majority  
of diseases and few  
treatments available

  
All together, an  
estimated  
**30**  
**MILLION PEOPLE**  
are living with a rare  
disease in Europe and  
**300**  
**MILLION**   
worldwide

### Rare Disease Day 2020 at the European Parliament

[Rare Disease Day](#) is an international initiative to raise awareness about rare diseases and their impact on people's lives. Over the past 10 years, Rare Disease Day has become an internationally recognised awareness-raising campaign, with events taking place in over 90 countries and regions, uniting millions of people living with a rare disease worldwide.

Members of the European Parliament are key partners in the development of the most effective strategies for rare diseases, which are cross-border and EU-wide. The EU enables the rare disease community to create the critical mass of patients, experts, knowledge, guidelines and resources needed by coordinating and adding value to national efforts. **Rare Diseases are an area with high European added value, where coordinated EU action has borne fruit.**

A strong track record of achievements on rare diseases has been established since the first European legislative text concerning rare diseases – the Regulation of 1999 on Orphan Medicinal Products and the subsequent Commission Communication (2008) and Council Recommendation (2009), not to mention the Directive on Patients' Rights in Cross Border Healthcare (2011) – including national plans for rare diseases, investment in research, development of treatments and the establishment of European Reference Networks (ERNs).

**However, there is still a lot to do and we have witnessed a great deal of changes in the 10 years since the Council Recommendation of 8 June 2009 on an action in the field of rare diseases.**

## Time to Reframe Rare

To mark the occasion of **Rare Disease Day 2020**, EURORDIS – Rare Diseases Europe will hold a **policy event at the European Parliament to “Reframe Rare”** and discuss areas of action at EU level that can contribute to the integration of people living with a rare disease into society and improve their lives.

The idea to reframe the current policy strategy was recommended in a recent report of the European Court of Auditors in June 2019, endorsed by the Council of the European Union, and the European Commission has accepted to revise its strategy where appropriate and relevant by early 2023, taking stock of achievements, lessons learned, and persistent challenges.

Taking this into account, the event will follow a format in which a number of speakers (including patients and MEPs) will engage in a **series of conversations around key areas of action at EU level** to precisely highlight **achievements and new opportunities** that have emerged in the field, such as scientific advances, digital tools and big data, as well as the **associated challenges** such as affordability and sustainability that come with them.

The event will also be the occasion for EURORDIS-Rare Diseases Europe to launch a call for Members of the European Parliament to join a network of **Parliamentary Advocates for Rare Diseases**, an informal group of committed MEPs willing to explore and discuss specific challenges faced by people living with a rare disease and ensure stronger EU-wide action through targeted support in health, research, social affairs and other relevant policies.

**We call on stakeholders – from the European Institutions and national governments, the NGO community, academic and research institutions, as well as the private sector – to join and open up the conversation about a renewed framework for rare diseases within the European Union.**

## Programme of the event

15:00-15:15	<b>Welcome and opening</b> Stelios Kypouropoulos, MEP, EPP & Tanja Fajon, MEP, S&D <i>Invitation to Stella Kyriakides, European Commissioner for Health</i>	
	<b>Conversations/ Fireside chats</b> Moderated by <b>Vivienne Parry, OBE, Writer, broadcaster, facilitator</b>	
15:15-15:40	<b>Research and innovation</b> - <b>Claudia Crocione</b> , Managing Director, HHT Europe - <b>Dr. Daria Julkowska</b> , Coordinator, European Joint Programme on Rare Diseases	<b>Keywords:</b> <i>patient engagement in research; cross-country and multi-stakeholder collaboration; sustainability of research; infrastructure</i>
15:40-16:05	<b>Access to medicines</b> - <b>Kateřina Konečn</b> , MEP, GUE/NGL - <b>Mencia de Lemus Belmonte</b> , President, SMA Europe	<b>Keywords:</b> <i>innovation; fragmentation; health technology assessment; value; fair pricing; affordability; sustainability; predictability; patient engagement; patient relevant outcomes.</i>
16:05-16:30	<b>Holistic care</b> - <b>Stelios Kypouropoulos</b> , MEP, EPP - <b>Rebecca T. Skarberg</b> , OIFE	<b>Keywords:</b> <i>inequalities; discrimination; work-life balance; disability assessment; un-adapted care pathways; integrated care; patient engagement; person-centred care; integrated care.</i>
16:30-16:55	<b>Cross-Border Healthcare and access to specialised care</b> - <b>Tomislav Sokol</b> , MEP, EPP - <b>Prof. Alberto Pereira</b> , Coordinator European Reference Network for Rare Endocrine Conditions (Endo-ERN)	<b>Keywords:</b> <i>patients' rights; evidence-based highly specialised healthcare; European Reference Networks; integration into national health systems; data sharing; sustainability; patient engagement.</i>
16:55-17:00	<b>Closing</b>	