EURORDIS
RARE DISEASES EUROPE

Parliamentary advocates for rare diseases

A network of European and national members of parliament advocating to improve the lives of people living with a rare disease

#ParliamentAdvocate4Rare

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About rare diseases and the role of the European Union

Rare diseases, often of genetic origin, affect small to ultra-small populations of patients (fewer than 1 in 2,000 individuals). Most rare diseases are chronic, progressive, degenerative, disabling and frequently life threatening. Each of the over 6,000 identified rare diseases affects a very small population, but **altogether they affect 30 million of people in the European Union (EU), around 5% of the EU population**, or the population of Belgium and the Netherlands combined. And, this is without taking into account carers, families and friends. The very notion of rarity, has long justified an approach that goes beyond national borders. Over the last 20 years, efforts to create breakthrough legislation in support of rare diseases and orphan medicines have been driven, for the most part, by the EU institutions.

- There are over **300 million people around the world living with a rare disease**[1], each supported by family, friends and a team of carers.
- There are **over 6,000 identified rare diseases**.
- Rare diseases currently affect **5%** of the worldwide population.
- Each rare disease may only affect a handful of people, scattered around the world, but collectively the number of people living with a rare disease is equivalent to the population of the **world's third largest country**.
- **72% of rare diseases are genetic** whilst others are either the result of infections (bacterial or viral), allergies and environmental causes or are rare cancers.
- 70% of genetic rare diseases **start in childhood**.
- A disease is defined as rare in Europe when it affects **fewer than 1 in 2,000 people**.

The EU Regulation on orphan medicinal products, the Council Recommendation on action in the field of rare diseases and the European Reference Networks demonstrate the added-value a European approach can bring to improving the lives of the people living with a rare disease in Europe.

However, despite advances over the years, many major challenges remain today for European citizens living with a rare disease. These challenges raise important questions as to what more the EU could do to generate further progress or to eradicate inequalities in research, access to medicines, health and social care between and within Member States.

Why a network of elected advocates?

The rare disease patient community is large, well connected and highly skilled. Its spirit is collaborative across very different diseases, countries and stakeholders in Europe. Patients are now being taken seriously and listened to by policy makers, researchers and companies.

Still there is a lot left to do. A significant challenge for patients, professionals, and health and social systems in Europe is the absence of streamlined, integrated pathways to allow people living with rare diseases to navigate health and social care systems. This is particularly problematic in

[1] Research recently published in the European Journal of Human Genetics, article authored by EURORDIS-Rare Diseases Europe, Orphanet & Orphanet Ireland - Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. The analysis is of rare genetic diseases and is therefore conservative as it does not include rare cancers, nor rare diseases caused by rare bacterial or viral infectious diseases or poisonings https://www.nature.com/articles/s41431-019-0508-0

view of the complexity of many of these 6, 000 conditions, and the lack of awareness and understanding in all sectors of society regarding their full impact.

The network of **Parliamentary Advocates for Rare Diseases will help tackle these challenges by fostering cross-border EU collaboration.** The network is made up of MEPs and national MPs who have long supported the cause of rare diseases or who have an interest in areas relevant to rare diseases (public health, social affairs, research and innovation). EURORDIS manages the day-to-day secretariat of the network.

The network's missions are:

- **To explore and discuss specific challenges faced by people living with a rare disease and to ensure stronger EU-wide action through targeted support;**
- **To shape political input for future legislation and programmes, ensuring that rare diseases are made an integral part of EU, national and regional programmes in health, research, social affairs and other relevant policies**.

Through the network of **Parliamentary Advocates for Rare Diseases**, EURORDIS-Rare Diseases Europe aims to bring together members of the European and national parliaments to ensure strong international and local action, shape political input for current and future legislation and integrate rare diseases into all relevant policies at all levels.

The network was launched on 17 October 2017, during the Parliamentary term 2014-2019. For the Parliamentary term 2019-2024, we wish to familiarise more MEPs with our cause and to progressively enlarge the membership national parliaments, creating stronger synergies between European and national policy-makers.

Why join the Parliamentary Advocates for Rare Diseases?

Network members will benefit from opportunities to:

- Participate in **calls to action** in particular areas of policy where efforts by the **EU could make a significant difference for their constituents living with a rare disease**;
- Learn from other parliamentarians and rare disease policy experts through an **exchange of information on rare disease policies and best practices across borders** in the areas of research, access to treatment, healthcare, social care, among others;
- Connect with **patient representatives in their constituency** and the **national alliance in their country**;

- Participate in events that facilitate **multi-stakeholder dialogue** with other policymakers, as well as representatives of healthcare organisations, the private sector and other members of civil society;
- Become proponents of political action in their respective Parliament, through for example the organisation of **hearings, exchanges of views or punctual events** around specific themes, submitting **written / oral questions.**

What areas will the network work on and how can you help?

Last year we called on MEPs to support our initiative and to **#Pledge4RD**. This year, we want to go a step further and see how we can go **from pledge to action.**

#Pledge4RD pillars	How you can help
Enables all people living with a rare disease to receive an accurate diagnosis and appropriate care within one year of coming to medical attention	• As the European Commission (EC) has accepted to revise its strategy where appropriate and relevant by 2023[2], you can help to **reframe** the current **European strategy tacking rare diseases to adequately address the needs of the rare disease community;** • **Health Technology Assessment** - Advancing the work that has been initiated during the previous legislature to support EU-wide coordination on HTA; • **Cross-border healthcare** - Scrutinise activity on the implementation of the Directive on Patients' Rights in Cross-Border Healthcare in line with **report of European Court of Auditors;** • Support a **European table of negotiations on prices of medicines** for all volunteering payers from EU Member States, specifically focusing on very complex and difficult-to-treat diseases; • Propose the creation of a **EU Fund** to support the collection and generation of evidence for those therapies treating rare or very rare diseases; • Explore EU-wide actions to promote **new-born screening** for rare diseases;
Support holistic care and social systems that are inclusive of people living with a rare disease, throughout their lives	• Ensure that in the **MMF 2021 – 2027 and successive annual budget** and related programme, adequate and streamlined funding is appropriated for **activities of the ERNs** and promote the implementation of the recommendations on **ERN integration in national healthcare systems;** • Ensure that measures to achieve **holistic care for people living with rare diseases and their families,** such as access to social services, disability assessment for rare diseases, as emerged in EURORDIS and members' position paper on Holistic Care, are

[2] as recommended in the special report 7/2019 from the European Court of Auditors.

	streamlined in the upcoming European Disability Strategy and that adequate financial support to the necessary innovation of social systems is reflected in the **MFF 2021 – 2027 and successive annual budget.**
Seizes opportunities in science and innovation that embody hope for people living with rare diseases and their carers and can change their lives	• Promote the importance of fostering research in the area of **rare cancers** in the context of the Commission's **Beating Cancer Plan**. • Support the rare disease patient community in getting engaged in the upcoming discussions around the **Pharmaceutical Strategy**, including the legislation **on medicines for special populations** (Regulation on Orphan Medicinal Products and on Paediatrics) ensuring that people suffering from rare conditions have same quality of treatment available; • Support **rare diseases research** an area where cross-country and multidisciplinary cooperation is key and contributes to medical research in general, notably by ensuring adequate support to a successful Partnership like the **European Joint Programme on Rare Diseases**.

We also need your support to:

- Organise a **European Parliament (EP) event** in the context of the **RareImpact** project that addresses the difficulties of patients accessing **advanced therapies**;
- Promote and organise the final meeting of the **Rare 2030 pilot project**, promoted by the European Parliament and co-funded by the European Commission. Rare2030 is a forecast study supported aiming to create possible future policy scenarios and recommendations for rare diseases for the next decade. As a next step, EURORDIS will call for a policy-document that sets the way to the most favourable identified scenario.
- Encourage patient organisations to participate in European policy-making by supporting **2021 Rare Disease Week**, either by meeting your constituents during this week and/or organise a bigger meeting in the EP.

About EURORDIS – Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 860 rare disease patient organisations from more than 70 countries that work together to improve the lives of **30 million people living with a rare disease in Europe**.

By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services.

Are you interested in joining the Parliamentary Advocates for Rare Diseases? Do you wish to know more? Please contact the EURORDIS team (Brussels office): +32 2 274 06 10 or

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