Rare 2030

Foresight in Rare Disease Policy

CHEAT SHEET:
MEETING YOUR MEP

A resource created for EURORDIS Rare Disease Week

EURORDIS
RARE DISEASES EUROPE

MEETING WITH YOUR MEP:
A NEW GENERATION OF POLICIES FOR RARE DISEASES (22-26 FEBRUARY 2021)

This document has been created to help you prepare and deliver your message to an MEP during Rare Disease Week (22- 26 February 2021).

Please feel free to adapt your message to address the specific needs in your country or disease area.

THIS DOCUMENT INCLUDES:

INTRODUCING YOURSELF

Please start by introducing:

- Who you are;
- What country you come from (and if you wish more details on city/regions etc. if the MEP is from your country);
- The Patient Organisation you represent, and what disease or disease group or National Alliance you represent.

The reason for this meeting:

- You are part of a group of patient advocates that have come together to advocate for better policies for rare diseases;
- You are part of a programme "**Rare Disease Week**", a week-long series of events and preparatory webinars that EURORDIS-Rare Diseases Europe put together to enable you to participate in advocacy activities at the European level, to influence the EU decisions that have a direct impact on the lives of people living with a rare disease. This is in the lead up to Rare Disease Day, a global awareness campaign, on 28 February.
- You are here therefore **on behalf of all patients with rare diseases in Europe.**

Important reminder - Why are we speaking with one voice?

- People living with a rare disease in Europe face a large number of common, recurring challenges that can only be addressed with collective efforts. This is what the rare disease patient community has been doing over the last decade, speaking with one voice.
- We need to be cohesive and share some common messages: the result of these collective actions will eventually affect our individual lives, communities and countries.

For more tips on public speaking, please see the summary note from

Webinar 3 - Soft Skills.

THINGS TO REMEMBER GOING INTO THE MEETING

KEY MESSAGE FOR YOUR MEP:

- We need a new generation of Rare Disease Policies

  Despite progress made, the current policies are no longer fit for purpose.

  The Rare 2030 Foresight Study - initiated by the European Parliament - has set out eight recommendations for a better future for people living with rare diseases.

KEY ASK FOR YOUR MEP:

- Join the network of Parliamentary Advocates for Rare Diseases:

  Receive information and briefings on relevant legislation

  Help us advocate for rare diseases

KEY MESSAGE: WE NEED A NEW GENERATION OF RARE DISEASE POLICIES

SUPPORTING INFORMATION:

» **About Rare Diseases**

» **Why a new generation of RD policies?**

» **Why now?**

» **What is Rare 2030 and how can it help?**

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About Rare Diseases:

Don't forget that your MEP is not an expert in rare diseases. Depending on their experience and interests, don't be afraid to come back to basics to set the scene.

- 30 million people across Europe live with a rare disease.
- A disease is rare when it affects fewer than 1 in 2000 citizens - there are over 6000 different rare diseases.
- Rare diseases are often chronic, progressive, disabling and life threatening.
- Most rare diseases have no cure and no treatment available.
- Rare disease patients face inequities in accessing diagnosis, care and treatments.
- People living with a rare disease frequently face significant social and financial burdens.
- We need to work together across Europe because each disease can affect just a few individuals, medical expertise is scattered and knowledge and data is scarce.
- Europe has done a lot for rare diseases and there has been fruitful collaboration to date, but there is so much more left to do.

KEY MESSAGE: WE NEED A NEW GENERATION OF RARE DISEASE POLICIES

Why do we need a new generation of Rare Disease policies?

In the past decade we have seen great strides forward for people living with a rare disease in Europe. But the reality is that unmet needs still persist and the current policies are no longer fit for purpose.

For people living with a rare disease in Europe:

- It still takes on average four years to receive a diagnosis;
- Only 6% have access to a treatment for their disease;
- 8 in 10 patients and carers have difficulties completing simple daily tasks.

Today's policies do not keep pace with tomorrow's needs as new technologies emerge and health priorities shift.

A new policy framework for rare diseases in Europe allows us to build on the foundations of the previous decade to drive tangible change for all people living with a rare disease by 2030.

Why now?

Because there is still a lot to do:

- To promote a **patient-centred approach** to rare diseases that addresses unmet needs of the rare disease population.
- To integrate the **new technologies** that have emerged over the past 10 years across data collection, research, medicinal products and equipment, diagnostics, and patient care.
- To ensure that Europe continues to enable 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.

KEY MESSAGE: WE NEED A NEW GENERATION OF RARE DISEASE POLICIES

Because there is strong impetus from other EU Institutions and the European Parliament itself!

- The recently published EU Court of Auditors Report provides an additional impetus for an updated framework for rare diseases. The report recommends that by 2023, "the Commission should: (a) assess the results of the rare disease strategy [the Council Recommendation on European Action in the field of Rare Diseases in 2009] (including the role of the European Reference Networks) and decide whether this strategy needs to be updated, adapted or replaced".

- The European Parliament in 2020 adopted a Resolution on post-COVID public health strategy in Europe that called for a EU Action Plan for Rare Diseases.

Last but not least because the Rare 2030 Foresight Study for rare disease policy has prepared the ground for change.

What is Rare 2030 and how can it help?

Over the past two years, experts from across the rare disease field - patients, carers, clinicians, researchers, industry and policy makers - have come together through the Rare 2030 Foresight Study for Rare Diseases to make an important decision: what future do we want for people living with a rare disease in Europe?

Rare 2030 prepared the ground for building the new generation of rare diseases policies!

- Initiated as a Pilot Project by the European Parliament and then co-funded by the European Commission.

- A participatory and inclusive study between January 2019 and March 2021 with an expert panel of over 250 key thought leaders and thousands of people living with a rare disease (via the RareBarometer survey)

- Employed foresight methodologies to create optimal future scenarios for rare disease policies, then mapped out the way to achieve these scenarios through strategy and policy changes.

- As a result, **eight overarching recommendations** took shape in eight key policy areas setting out the steps that need to be taken to ensure that the future of 30 million people living with a rare disease is not left to luck or chance. **These recommendations outline the new generation of policies that are needed for rare diseases in the next decade.**

KEY ASK: CALL ON YOUR MEP TO BECOME A MEMBER OF THE NETWORK OF PARLIAMENTARY ADVOCATES

The Network of Parliamentary Advocates for Rare Diseases is a structured way to support rare diseases in parliament.

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).

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.

EURORDIS manages the day-to-day secretariat of the network.

How this will help support the Rare 2030 recommendations:

The Rare 2030 recommendations will form the basis on which EURORDIS – Rare Disease Europe and its member organisations will be forming our opinion about upcoming legislative debates.

Achieving our goals by 2030 relies on legislative changes across many different areas, not just one. There are several current and upcoming opportunities where we can continue to raise our points:

- Current legislative work around the Data Governance Act
- Upcoming introduction of legislation concerning a European Health Data Space
- The Social Pillar Action Plan
- Upcoming revision of the pharmaceutical strategy
- Upcoming work around the Cross Border Healthcare Directive

As part of the network, we will provide detailed briefings on these areas to drive changes for a better future for people living with a rare disease.

CUSTOMISING YOUR MESSAGES

While we plan to present the same key asks and will have common messages, you can bring your personal touch. In fact, this is very important: it can help the MEP understand the need for the policy change you are asking for and helps to build rapport with them.

Please introduce:

- Your story – why and how you got involved in advocating for rare diseases

Remember - the importance of your story comes from how it helps your MEP to relate to you and your message (see the summary notes from Webinar 4 - Storytelling for Patient Advocacy).

If time allows, please expand on:

- Specific Rare 2030 recommendations that you want to pick to illustrate the areas in which there is still work to do, or that are important to you or your country;
- Your MEP's areas of interest.

EURORDIS will provide you with the relevant information on the MEPs and their areas of interest or political action prior to the meeting. This may help you to focus on a specific area or policy ask that they are interested in.

Please refer to the Rare2030 Recommendation (see Annex) for key messages in that area! EURORDIS can also help you to find the right resources (position papers, etc.)

ANNEX: THE RARE 2030 RECOMMENDATIONS

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The future of people living with a rare disease cannot be left to luck or chance. To play a positive role in future, we call upon Europe's institutions and Member States to deliver on our eight recommendations for a new generation of rare disease policies by 2030.

RARE 2030 RECOMMENDATION 1 - LONG-TERM, INTEGRATED EUROPEAN AND NATIONAL PLANS AND STRATEGIES

> A European policy framework for rare diseases defined by societal responsibility, equity and driven by the needs of people living with a rare disease should guide the implementation of consistent national plans and strategies, secure major investments at both the European level and by governments that are fairly shared across Europe in order to pool scarce resources, share expertise and information, scale-up good practices and provide access to timely and accurate diagnosis and the highest available quality of treatment and care for people living with a rare disease, no matter where they live in Europe. Both EU and national policies are supported by measurable outcomes that are monitored and assessed by a multistakeholder body on a regular basis.

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RARE 2030 RECOMMENDATION 2 - EARLIER, FASTER, MORE ACCURATE DIAGNOSIS

> The time to diagnosis should be shortened - whilst avoiding erroneous and subsequent negative consequences - which should be achieved by better use and accessibility of currently effective and available diagnostic testing technologies, best practices and programmes. New technologies and innovative approaches must be driven by patient-needs and applied rapidly and strategically. Inequalities in access to diagnosis and ensuing care must be eradicated through the harmonisation of standards and programmes across Europe (and beyond). A particular focus is necessary for patients with undiagnosed rare diseases, which demands greater and more strategic global collaboration via data-sharing and diagnostic platforms and infrastructures.

RARE 2030 RECOMMENDATION 3 - ACCESS TO HIGH QUALITY HEALTHCARE

> Political, financial, operational and technical support at European, national and regional levels should be provided to establish a mature highly specialised healthcare ecosystem that, in collaboration with patient organizations and all relevant stakeholders, leaves no person living with a rare disease with uncertainty regarding their diagnosis, care and treatment.

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RARE 2030 RECOMMENDATION 4 - INTEGRATED AND PERSON-CENTRED CARE

> Implement EU-wide and national actions by all stakeholders that guarantee integration of people living with a rare disease in societies and economies, enabling them to live life to their full potential, by implementing innovative solutions and approaches to integrated and person-centred care along the full lifespan of people living with a rare disease.

EU-wide and national actions must be undertaken by all stakeholders to guarantee equal opportunities and access to the labour market, active support for employment, fair working conditions, social protection and inclusion and integrated and person-centred, long-term care for people living with rare diseases and their families.

RARE 2030 RECOMMENDATION 5 - PARTNERSHIP WITH PATIENTS

> An overall culture, reflected in policies and funding, that encourages the meaningful participation, engagement, involvement and leadership of people living with rare diseases in the research, care and development of diagnostic tools, treatments and innovative solutions to improve the health and social status, healthcare delivery, autonomy, quality of life and well-being of people living with a rare disease in Europe. Patient partnership should be encouraged in both the public and private sectors and people living with rare diseases and their representatives may often serve as a partnering link between the two. diseases, which demands greater and more strategic global collaboration via data-sharing and diagnostic platforms and infrastructures.

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RARE 2030 RECOMMENDATION 6 - INNOVATIVE AND NEEDS-LED RESEARCH AND DEVELOPMENT

> Maintain basic, clinical, social and translational research on rare diseases as a priority by increasing the funds for competitive and pre-competitive research, establishing greater incentives in more neglected areas (or in areas of high unmet needs), and supporting infrastructures required to expedite discovery and knowledge acquisition. Research in public health, social sciences, healthcare organisation, health economics and health policy research must also be promoted, to ensure that research outputs are applied for the benefit of people living with a rare disease.

RARE 2030 RECOMMENDATION 7 - OPTIMISING DATA FOR PATIENT AND SOCIETAL BENEFIT

> All European data sources of relevance to addressing the challenges faced by people living with rare diseases should be federated in a continuum encompassing epidemiological, healthcare, research, quality of life and treatment-related data, and should be linked at the global level where possible. Sharing of data for care and research should be optimised across infrastructures and countries, relying upon commonly adopted codification systems (Orphanet nomenclature), harmonised standards and interoperability requirements. Cohesive data ecosystems should be developed at national level, linking seamlessly via Findable, Accessible, Interoperable and Reusable (FAIR) data approaches to an integrated European ecosystem, positioned within the European Health Data Space and centred on robust ERNs, the European Platform on Rare Disease Registration, and other key infrastructures. Legal and ethical guidelines and regulations should incentivise practices that best lead to addressing these challenges while respecting international, national and regional laws and conventions – particularly the preferences and privacy of people living with rare diseases and their families.

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RARE 2030 RECOMMENDATION 8 - AVAILABLE, ACCESSIBLE AND AFFORDABLE TREATMENTS

> Establish streamlined regulatory, pricing and reimbursement policies. These policies should encourage a continuum of evidence generation along the full life cycle of a product or technology as well as the patient journey from diagnosis to treatment access. A European ecosystem able to attract investment in areas of unmet need, foster innovation, and address the challenges of healthcare system sustainability.

FURTHER RESOURCES

Consult the following online documents for further information on rare diseases, EU policy, and the Rare 2030 project & policy proposals

THE RARE 2030 PROJECT

Rare 2030 website

Rare 2030 Knowledge Base

Rare 2030 Foresight Study
(available in 6 languages)

LEGAL DOCUMENTS & EU POLICY

2009 Council recommendation on a European action in the field of rare diseases
(available in 22 languages)

EU Rare Disease Policy

About the EU Parliament

About the MEPS

2030 Agenda for Sustainable Development

NGO Committee for Rare Diseases

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