We need action now to address the unmet needs of people living with a rare disease: 79% do not think a cure is possible within the next decade

EURORDIS-Rare Diseases Europe calls for a European Union action plan on rare diseases to meet unmet needs by 2030

1 June 2021, Brussels – EURORDIS-Rare Diseases Europe, 39 National Alliances and 962 members today launch a campaign calling for an immediate coordinated European action plan to improve the lives of people living with a rare disease by 2030.

The call follows the EU backed Rare 2030 Foresight Study recommendations, setting the roadmap for rare disease policies by 2030, and new figures published today on the expectations of nearly 4000 people living with a rare disease over the next decade.

EURORDIS calls on the European Commission and EU Member States to introduce a European action plan on rare diseases. This would bring together existing efforts across different legislation and EU programmes under a coherent framework to spearhead change.

It will align Member States towards the same measurable goals to ultimately improve survival, quality of life and social inclusion. This can also significantly contribute to the United Nations Sustainable Development Goals. If implemented across sectors and countries, the action plan will work towards the measurable goals to:

- Diagnose every person within 6 months instead of the current 5 years average;
- Reduce premature deaths due to rare diseases;
- Reduce the economic, social and psychological burden of rare diseases by 1/3; and
- Bring 1000 new medicinal products, including gene and cell therapies, based on European-led research.

An ambitious EU plan will support an advanced eco-system to ensure that Europe reinforces its status as a world leader in health innovation, precision medicine, genomic health, and digital health innovation.

Without an ambitious and cohesive EU strategy, spanning different policy areas, including but not exclusive to health, we risk both losing the progress the Member States have made since the 2009 Council Recommendation on an action in the field of rare diseases, and missing opportunities to benefit from advances in science and technology.

Yann Le Cam, Chief Executive Officer at EURORDIS, commented:

"We need a European action plan on rare diseases because the world is changing - with new technologies, new knowledge, and new opportunities - and the 30 million people living with a rare disease in Europe cannot be left behind.

Policies on rare diseases should no longer only be about the means and the infrastructures. Instead, they should be centred around measurable outcomes and goals we want to achieve across the European Union while leaving

flexibility to Member States on how to get there. Only with a cohesive approach, adopted now, will we see the change we need by 2030 for people with rare diseases."

Rare 2030 survey results on the future of rare diseases

The urgent need for an overarching strategy on rare diseases has been highlighted in a survey of nearly 4000 people living with a rare disease published today. The pan-European research asked respondents their expectations and priorities with regard to living with their rare disease over the next ten years.

79% of survey respondents said that they did not expect to be cured of their rare disease within the next decade as things stand. As currently only 6% of rare diseases have a treatment, this pace of change is not fast enough.

The Rare 2030 survey on the future of rare diseases set the case for renewed European action on rare diseases, highlighting that:

- **A high level of unmet needs remain for people living with a rare disease**

While 79% of respondents did not expect to have a cure within the next decade, treatments that do not yet exist ranked as the highest priority to improve their care (51%), followed by improving coordination between all healthcare professionals involved in their care (45%) and having consultations with healthcare professionals specialised in their disease (32%)

Respondents also put a focus on how a more holistic approach - including receiving more emotional support (58%), better managing symptoms (49%) and facilitating access to employment (44%) - could improve their lives in the next decade.

- **The pace of change has not kept pace with new technologies and values**

95% of respondents support screening for rare conditions at birth, despite the lack of a harmonised approach across the EU.

Increasing digital connectivity by using remote consultations to discuss their disease with several healthcare professionals was an attractive option for 81% of respondents willing to use these, allowing access to experts in other regions of their country or Europe.

- **Europe needs to establish itself as a global leader in health care and innovation**

Europe should be a world leader in innovation and development to foster inclusive growth, reduce inequalities and ensure that every citizen has access to high-quality care and social protection. Leading by example, it needs to take immediate actions to address the needs of people living with a rare disease, a large segment of its population cumulating vulnerabilities and high-unmet needs.

Cristina, a patient advocate from Italy, shared her reason for European action at action.eurordis.org:

"Too many rare disease patients are still undiagnosed or wrongly diagnosed, there are too many deaths because care and treatment are too expensive, and social care seems complicated. Every person living with a rare disease is a reason for Europe to take action on rare diseases that leaves no one behind by 2030."

EURORDIS would like to thank all the people living with a rare disease who participated in the survey from December 2020 – January 2021.

Read the full report here

Read the factsheet in English, Czech, German, French, Italian, Spanish, Norwegian, Russian, Swedish.

#30millionreasons for European action on rare diseases

EURORDIS has launched a campaign, #30millionreasons, calling upon people from across the rare disease community to share their reasons for European action on rare diseases with Ursula von der Leyen, President of the European Commission.

This EURORDIS-led campaign has the support of 39 National Alliances from across Europe and over 960 member organisations representing the 30 million people living with a rare disease to drive efforts at the national and European level to mobilise policy makers to take action.

Rare diseases do not work in silos: a cohesive European strategy is needed across countries and sectors to ensure progress is made in improving the life of every person living with a rare disease in Europe.

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Contacts

For media enquiries, please contact**:**

Stanislav Ostapenko
Communications Manager
EURORDIS-Rare Diseases Europe
stanislav.ostapenko@eurordis.org
+33 1 56 53 52 61

Anna Kole
Director of Public Health Policy
EURORDIS-Rare Diseases Europe
Anna.kole@eurordis.org

Jessie Dubief
Rare Barometer Research Executive
EURORDIS-Rare Diseases Europe
jessie.dubief@eurordis.org
+33 6 81 35 81 41j

EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of 962 rare disease patient organisations from 73 countries that work together to improve the lives of the 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. Follow @eurordis or see the EURORDIS Facebook page. For more information, visit eurordis.org.

About rare diseases

The European Union considers a disease as rare when it affects less than 1 in 2,000 citizens. Over 6,000 different rare diseases have been identified to date, affecting an estimated 30 million people in Europe and 300 million worldwide. 72% of rare diseases are genetic whilst others are the result of infections (bacterial or viral), allergies and environmental causes, or are degenerative and proliferative. 70% of those rare genetic diseases start in childhood.

Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offerings inadequate, and research limited. Despite their significant overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment, and research benefits.

About #30millionreasons for European action on rare diseases

#30millionreasons is a campaign for a European action plan on rare diseases to ensure that no person living with a rare disease is left behind by 2030. Following the two-year Rare 2030 Foresight Study proposing policy recommendations for a better future for people living with a rare disease, the rare disease community demands

that their future is not left to luck or chance. We are asking our community to share their reason for European action on rare diseases with Ursula von der Leyen, President of the European Commission.

About Rare 2030 Foresight Study

Rare 2030 is a foresight study that gathered the input of over 250 patients, practitioners and key opinion leaders to propose policy recommendations that will lead us to improved policy and a better future for people living with a rare disease in Europe. This was a two-year project is co-funded by the European Union Pilot Projects and Preparatory Actions Programme (2014- 2020).

It followed a four-stage process using foresight:

1. Establishing the existing knowledge base;
2. Identifying trends likely to impact rare diseases over the next decade;
3. Preparing four potential scenarios based on the trends that could play out by 2030, and deciding on the preferred scenario "Investment for social justice", prioritising patient needs-led innovation and collaboration;
4. Develop policy recommendations based on the preferred scenario.

The study was led by EURORDIS-Rare Diseases Europe with nine partners and an expert panel of over 250 people who have contributed to the development of the Rare 2030 eight overarching recommendations.

The Rare Barometer Programme

The Rare Barometer Programme is the EURORDIS survey initiative that brings together almost 15,000 rare disease patients, family members and carers who share their experiences and opinions on the issues that matter to the rare disease community.

The Rare Barometer survey software enables high-quality secure data collection and analysis. The Programme was created to systematically collect patients' opinions on transversal topics and introduce them into the policy and decision-making process, transforming patients' and families' opinions and experiences into figures and facts that can be shared with a broader public and policy makers.