Press release

EURORDIS-Rare Diseases Europe calls on the European Commission for decisive action on rare diseases

Ministerial Conference hosted by the French EU Presidency in Paris on Rare Disease Day 2022 sees an appetite for coordination on rare diseases from EU Member States, the European Parliament and WHO-Europe, however, reinforces the European Commission's lack of a strategic vision and ambition to keep up with the challenges.

28 February 2022, Paris **- On Rare Disease Day, EURORDIS-Rare Diseases Europe and its 988 member patient organisations welcome the commitments by France, Czech Republic and Sweden – holding the current and next four EU Council Presidencies – towards a European Action Plan on Rare Diseases, but call for greater urgency and concrete actions from the European Commission following their moderate response.**

> *"This Rare Disease Day we had an unprecedented opportunity to take decisive steps towards a new policy framework for rare diseases under French leadership. We are grateful for the drive and support of Member States and the European Parliament today in their impulse for a new ambitious strategy toward Europe's Action Plan on Rare Diseases.*
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> *However, the European Commission is politically resisting this call for a coordinated rare disease framework from EU Member States, the European Parliament, WHO Europe, and stakeholders, as well as patients and civil society. The Commission opposes to act upon the high unmet needs and persistent challenges of the 30 million people living with a rare disease; and this can cost million years of life if we don't act now, if we leave it to the next Commission."*
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> – Avril Daly, Vice President of EURORDIS-Rare Diseases Europe

At a Ministerial Conference "Research and Care Pathways: For a European policy on rare diseases" today in Paris, hosted by the French Ministry of Health under their EU Council Presidency and in accord with Czech Republic and Sweden, ministers and delegates from as many as 18 EU countries, decision makers, clinicians and civil society from around Europe came together to discuss how to collectively prepare for a new European strategy on rare diseases. The first and last one was 13 years ago.

The discussion around rare diseases issues for health and innovation pathways started with a word of welcome from Clément Beaune, French Secretary of State for European Affairs, who praised significant advances in the area of rare diseases in the past years and emphasised the importance of a European plan for rare diseases, similar to Europe's Beating Cancer Plan, to continue serving the best interests of European citizens living with a rare disease.

The French Minister for Health, in his speech, called on Europe to define a roadmap that would see the development of a comprehensive policy framework on rare diseases, saying that *"the Europe of health is no longer a supplement to the soul of the European Union. This is evidenced by the call to unite all stakeholders – patients, carers, families, researchers – in advocating for a European plan for rare diseases. In 2008, we already had the intuition that the specificities of rare diseases made this an area where the European Union could bring strong added value. If these initiatives prove us right, European action for rare diseases would help Member States become more efficient by optimising the allocation of resources, and by sharing and coordinating expertise and data."* A call for legislative change received support, with other EU Member States contributing to the debate, including Czech Republic, Belgium, Spain, Germany, Sweden, Netherlands, Latvia, Hungary, Portugal, and Cyprus.

Jakub Dvořáček, Czech Deputy Minister, stated that people in Europe *"need an overarching European Action Plan or similar policy initiatives in order to federate and synergize various initiatives, make them effective and impactful, and avoid working in disparate 'silos'."* On behalf of the Belgian government, Frank Vandenbroucke, Minister of Health and Social Affairs, also expressed support for coordinated action on rare disease, adding that *"the answer is that the European Union is strong. We can change the current situation together and that is why I fully support the idea of a European plan for rare diseases. We need to reorient the orphan drugs regulation and move away from the concept 'one size fits all'."*

Latvian Minister of Health, Daniels Pavluts, and Dr. Ildikó Horvath, Hungary's State Secretary for Health, put particular focus on the issue of equity, emphasising the importance of a joint European effort *"so that rare does not mean alone."*

Representing the European Commission – which would need to initiate a European response – Health Commissioner Stella Kyriakides in a pre-recorded video called for joint action at the EU level, saying that *"working together"* and *"pulling resources is the only way forward"*. She emphasised the success of the Pharmaceutical Strategy and promised to present a proposal for European Health Data Space in due time. This statement fell short, however, of expectations, not only of the rare disease patient community but also of the national policy makers. Instead, it saw the repetition of ongoing actions – all important, welcomed and critical for change – but all in silos, and therefore insufficient to address the challenges of people living with a rare disease in Europe in a comprehensive manner. EURORDIS-Rare Diseases Europe also regretted that the Health Commissioner did not attend in person, unlike many Health Ministers and MEPs.

> *"We recognise and praise the past, on-going and planned initiatives of the European Union, but this Commission needs to stop the prevailing siloed thinking. The ongoing evaluation of the Cross Border Health Care Directive, the revision of the EU Regulation on Orphan Medicines, the launch of European Health Data Space and more, are not federated by clear public health objectives. They are therefore missing the point. An urgent strategy to coordinate and learn from each area and each country, and to create the eco-systems for progress is paramount to make measurable change."*
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> – Yann Le Cam, Chief Executive Officer of EURORDIS-Rare Diseases Europe

It's been a year since the Rare 2030 Foresight Study published its recommendations. This project was initiated by the European Parliament and funded by the European Commission to bring together experts and patients from around Europe to propose policy solutions for measurable change by 2030, with the main recommendation for a coordinated European policy framework for rare diseases. These recommendations are rooted in extensive consultation and promoted by all stakeholders. Since then, it has received wide support from the European Parliament and individual Member States but has remained on the shelf of the European Commission.

Belgian MEP and active rare disease advocate Frédérique Ries, who initiated the Rare 2030 Foresight Study, argued: *"A comprehensive and concerted approach is needed globally to create synergies, and enrich and share European best practices, and strengthen each step of the care pathway. What Europe needs is a real action plan for rare diseases. A battle plan that will also regain momentum by truly supporting the updating or adoption of ambitious new national plans – many of which you know have expired, are outdated or are still missing. And urgency means no more waiting for 2024 and the next Commission. Patients cannot wait."*

This was further reinforced by French MEP Trillet-Lenoir who said that *"we need Europe to address major public health issues. We need an action plan for rare diseases because we are stronger together. Such a plan would mark the success of the European Health Union…Because making the European Health Union is about fighting inequalities. All the testimonies we have heard show the many disparities in access to diagnosis and treatment that exist today in Europe."*

Call for an updated, coordinated and goals-based European Strategy for Rare Diseases today

The last rare disease strategy and the only one so far was over a decade ago. Since then, technology, science and indeed legislation have progressed. A coordinated strategy is the only way to ensure that actions are considered and coordinated in a comprehensive manner to reach meaningful goals for patients, families and for society at large.

It should also act as a catalyst for a new round of National Rare Disease Plans or Strategies, which have lost momentum and are no longer integrated nor even coordinated at European level. Latest research as part of the Rare 2030 Foresight Study from October 2020 shows that three EU Member States had never introduced a plan, and seven had technically expired policies. The National Plans of nine more countries were due to expire at the end of 2020.

"What we are proposing is a federated approach, where experts from these areas can come together and ensure that on-going and future European and Member State actions are aligned and optimised. We are also seeing the desperate need

for a European strategy as an impetus for Member States to drive National Strategies to support common goals towards the best diagnosis, treatment, and care for 30 million persons living with a rare disease wherever they are born.

The current rare diseases package is fragmented, lacks clear objectives, misses opportunities to engage all relevant stakeholders, and is disconnected from the reality of international competition, science, and technologies. Unfortunately, we see a Commission, which by not taking decisive, immediate action towards an ambitious, comprehensive, integrated, and goal-based approach is essentially kicking this initiative into the long grass for the next Commission. This is to the detriment of the 30 million people living with a rare disease in Europe. Millions of years of life will be lost to political resistance," adds Le Cam.

The call for Europe's Action Plan for Rare Diseases, for which EURORDIS has been advocating since June 2021, has received endorsement from EU Member States and Members of the European Parliament – in a plenary debate in November and in a follow-up letter signed by 43 MEPs, led by MEP Frédérique Ries. Over 2000 European citizens have also shared their personal reasons to act with the European institutions through the #30millionreasons for European Action on Rare Diseases campaign. It's in the European Commission's hands to initiate action.

EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of 984 rare disease patient organisations from 74 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.

Rare 2030 Foresight Study

The Rare 2030 Foresight Study, initiated by the European Parliament and funded by the European Commission, published its recommendations a year ago. Over two years, 250 experts, thousands of patients and a group of Europe's Young Citizens were mobilised through consultations, interviews, surveys and conferences to agree on what the next decade of rare disease policies should look like. This resulted in eight recommendations to meet the unmet needs of people living with a rare disease by 2030, including for a new European policy framework.

Campaign #30millionreasons

EURORDIS-Rare Diseases Europe asked European Federations, National Alliances for Rare Diseases and 988 Patient Organisation Members, as well as the wider rare disease community to share their personal reasons for change through the #30millionreasons for European Action on Rare Diseases.

Across Europe, over 2100 people – those living with a rare disease, parents, siblings, friends, advocates, healthcare professionals and researchers – shared their reasons why Europe must act. These are personal experiences, hopes and fears that are moving, motivating, sometimes devastating, impassioned and humbling.

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