Press release

EURORDIS-Rare Diseases Europe responds to Czech Presidency Call to Action on rare diseases calling for a European Action Plan for Rare Diseases

***25 October 2022, Prague* – A Call to Action on rare diseases, alongside a specific call to the European Commission for a European Action Plan on rare diseases, was today presented by Jakub Dvořáček, Deputy Health Minister of the Czech Republic.**

The Expert Conference is taking place in Prague, as part of the Czech EU Presidency, between 25- 26 October, gathering ministry representatives, coordinators from the European Reference Networks, industry, researchers and patient advocates to establish the next steps for strengthened collaboration on rare diseases.

Speaking at the Expert Conference on Rare Diseases "Towards a New European Policy Framework: Building the future together for rare diseases", Jakub Dvořáček set out the Call to Action prepared by the Czech Presidency. France has already endorsed the document.

> *"The French Presidency gave us a chance to build on their success. We see Europe is changing and rare diseases are exactly the field where we can progress and change lives. I believe that together, we can move forward on all the areas presented in the Call.*
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> *It's only the Czech Presidency Call to Action for a few more weeks. We invite the support from all Member States."*
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> – Jakub Dvořáček, Deputy Health Minister of the Czech Republic.

Reacting to the Call to Action, Yann Le Cam, Chief Executive Officer at EURORDIS-Rare Diseases Europe said:

> *"This Call to Action reinforces the urgency and will of Member States to strengthen collaboration on rare diseases at the European level. It is a major step forward, with Czechia establishing a clear stand on how a European Action Plan on Rare Diseases will lead our health union."*
>
> – Yann Le Cam, EURORDIS Chief Executive Officer.

The Call to Action draws on discussions at the Expert Conference to include several recommendations:

- **To adopt a European Action Plan on Rare Diseases to support and complement ongoing and future efforts at both the EU and Member State level** to reduce the unmet needs of the 20 million people living with a rare disease in the EU.

- **To support the early diagnosis of people living with a rare disease,** specifically by supporting the initiatives to ensure availability and equally accessible NBS programmes in the EU that can benefit from a coordinated EU-wide approach, as well as to support an approach to an expanded number of disease areas and countries across Europe to better diagnose currently "unsolvable" cases.

- **To evolve the incentives framework** to maintain predictability for sponsors while enhancing Europe's competitiveness through the upcoming revision of the Orphan Medicinal Products and Paediatric Regulation.

- The Call to Action also puts forward a strong **message to improve access to treatments,** including further strengthening European cooperation in pricing and negotiations, while respecting current division of competences.

- On **holistic care and integrating European Reference Networks into national health systems,** the call recognises the need for sustainable and proportionate investment from national and EU budgets, and a fully-fledged data strategy for rare diseases in alignment with the European Health Data Space.

EURORDIS has been calling for a European Action Plan on rare diseases – a goal-based strategy – since it emerged as the main recommendation of a large-scale multi-stakeholder foresight study Rare 2030 in 2021. This has gathered the support of MEPs, experts and over 2000 families with rare diseases.

We welcome the Call to Action and the commitment of the Trio Presidency - France earlier this year and now the Czech Republic - to support a European Action Plan on Rare Diseases. Through this high-level engagement, we have observed a clear demonstration of Member State support for more strengthened collaboration on rare diseases.

The rare disease community is now calling on all Member States to endorse the Czech call to action in view of the EPSCO Council in December and on the European Commission to introduce a new policy framework on rare diseases.

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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