



Open Letter

#ActRare2024

We, the **Rare Disease community**, committed to enhancing health outcomes and quality of life for the 30 million people living with a rare disease in Europe and their families, convened at the 12th European Conference on Rare Diseases and Orphan Products (ECRD 2024) to shape, discuss and propose clear, goal-driven policy asks with far-reaching impacts on both EU-wide and national policies for rare diseases.

We call on the next European leaders to:

- **Maintain health at the forefront of future policies and programmes.**
- **Develop a comprehensive European Action Plan for Rare Diseases that bridges diverse policy areas and streamlines existing efforts with clear, measurable objectives.**
- **Immediately address the most pressing needs of the rare disease population by integrating the actions ‘within reach’ that we have collectively identified within the work programmes of the next years.**

With the European elections approaching and a new mandate for the European Parliament and the European Commission on the horizon, we advocate for maintaining **health at the forefront of future EU leadership**, underscored by a substantial health budget allocation under the Multiannual Financial Framework 2028–2035. Health must not be sacrificed, and we should all work together to protect and ensure a strong and resilient European Health Union, which goes beyond crisis preparedness. Building a future-proof rare disease policy for Europe means **constructing the engine of an inclusive European Health Union** that propels the entire ecosystem.

It is crucial, now more than ever, to harness this momentum for change and take bold, decisive action. With the foundations already set, the time has come to consolidate a European Health Union for rare diseases.

The rare disease community, gathered at ECRD 2024, draws on the [Rare 2030 Foresight Study and its eight key recommendations](#) – the outcome of two years of collective work by over 250 experts from across the community.

Our foremost appeal to the next European Commission is to develop a comprehensive **European Action Plan for Rare Diseases**, ensuring coordinated action across EU Institutions and Member States to address the fragmented care and support system. Given the broad and compelling consensus among EU institutions and Member States, under the [Czech Presidency](#), on the need for such a plan, prioritising its inclusion in the European Commission’s 2024–2029 Work Programme represents a crucial and inevitable step forward.

This Plan would maximise the impact of past investments by leveraging our collective capacities and accumulated experience, to address the ongoing unmet needs of the 30 million people living with rare diseases and ensure they receive the timely support and care they urgently require.

Detailed policy framework recommendations

During the two days at the ECRD, we have identified **tangible actions ‘within reach’** that can be incorporated into a unified framework of the European Action Plan for Rare Diseases, addressing the needs of people living with rare diseases in a holistic manner.

These policy asks, reflecting the needs and aspirations of various stakeholders – including patient advocacy groups, healthcare professionals, policymakers, researchers, and industry partners – should be translated into actions without further ado, as they could drive immediate, impactful changes to the lives of people with rare diseases. They should therefore be integrated into the Work Programmes of EU4Health, Horizon Europe or other EU programmes spanning from 2025 to 2027.

We call on the European Union to:

European and national policy frameworks for rare disease:

- Establish a **European Action Plan for Rare Diseases**, an overarching framework with clear, measurable objectives. Within this framework, support collaborative actions across EU Member States and other European countries, notably to:
 - identify **common goals** and develop an **integrated system of indicators** that enables to inform, shape and constantly improve decision-making, policies and systems at the EU and national level;
 - create an **EU-wide multistakeholder body** to steer and oversee the above collaborative actions, thereby streamlining efforts and ensuring consistency in care and support for people with rare diseases throughout Europe.

Earlier, faster and more accurate diagnosis:

- Promote **EU-level initiatives** that enhance **cross-country cooperation on newborn screening** to bring newborn screening national programmes across Europe closer together and make them equitably accessible, e.g. by aligning on best available standards, methodologies and scientific advancements, or issuing recommendations to expand the scope of rare diseases in those programmes, among others to increase chances to access further treatments.

Innovative and needs-led research:

- Create a **European development platform and explore novel models** to speed up clinical research and development on **very rare and underserved diseases**. This should be coupled with **ambitious investment funding strategies**, both from the **private and public sectors**.

Timely access to affordable and innovative treatments:

- Unravel the **paradox of patient access to rare disease therapies** in Europe, notably by:
 - exploring the feasibility of **novel payment mechanisms**, from subscription models to a common EU access pathway and cross country negotiations;
 - creating a **common approach to funding treatments for very small populations** based on a solidarity mechanism to provide access across borders.

Enhanced collective action to ensure timely access to highly specialised healthcare:

- Create a **European system to centralise the commissioning and delivery in a few centres of highly specialised healthcare services for very small patient populations** and certain highly specialised complex procedures.

Integrated, person-centred and lifelong holistic care:

- Support EU Member States to implement the United Nations General Assembly Resolution's (A/RES/76/132) call to develop **effective programmes and national strategies to promote mental health and psychosocial support for people living with a rare condition.**
 - o Integrate psychological support as an essential component of holistic, patient-focused care, by enhancing existing medical care to be 'psychologically informed' for people living with a rare condition.
 - o Recognise and support patient organisations to provide community and peer support, and access to trusted information, as the foundation of psychosocial care, enabling earlier detection and access to preventative support.

We recall that these actions would build on an existing rare diseases ecosystem, supported by the EU Institutions and its Member States, encompassing the European Reference Networks for specialised healthcare, that should become a sustainable care infrastructure, the Joint Action JARDIN on their integration into national healthcare systems, Orphanet for data and information, the European Partnership for Rare Diseases ERDERA for rare disease research, and EURORDIS-Rare Diseases Europe representing the patients' voice.

Building on these solid bases, the strategic initiatives we are calling for aim to consolidate a robust framework that addresses the complex challenges faced by the rare disease community, moving us closer to a more inclusive and effective European Health Union.