Call to action

Addressing the unmet medical needs for people living with a rare disease in Europe and beyond

Affecting up to 36 million people in the European Union alone, **rare diseases are many, invisible and most often not a priority for our healthcare systems**.

Partnering 4 PNH

RAREDISEASEDAY.ORG

By signing this call to action, we pledge our commitment to continue the search for much needed solutions to address the many unmet medical needs of the rare disease patient community. We consider the below points as guiding actions for policy makers when revising and implementing the upcoming Orphan Medical Products (OMP) and General Pharmaceutical legislation:

Allocate funds from EU and national budgets to strengthen the capacity of the Centres of Expertise and enhance the competencies of the European Reference Networks (ERN) in order to offer long-term protection to people living with a rare disease.

Reduce extended diagnosis journeys, address misdiagnosis and "undiagnosis" by adopting more innovative diagnosis tools, increasing availability of genetic testing and solidifying pathways from first symptoms to care at Member State and EU level.

Build a more resilient ecosystem for research and innovation for all rare diseases, independent of existing knowledge of a disease, availability of existing treatments, disease severity or prevalence and in which all healthcare stakeholders work closely to create robust regulations that build upon the success of existing policy frameworks and incentives, fostering continuous rare disease research and innovation.

Use disease-specific instruments in health technology assessments (HTA) that capture the frequency and severity of specific symptoms from a physical, psychological, functional, and social perspective, providing a holistic view of the impact of the disease on patients' quality of life.

Strengthen training of healthcare professionals on rare diseases and provide more opportunities for specialisation in order to reduce time to diagnosis, improve patient care and outcomes.

Empower patients and caregivers in decision-making to place patients at the centre of healthcare systems and improve outcomes through health literacy, capacity-building, shared decision-making and support to self-management.

Reduce inequalities and expedite access to treatment through joint health technology assessments, create more opportunities for early collaboration in the evidence generation process between regulators, developers, HTA bodies and patient representatives at EU level and improve national reimbursement processes for orphan drugs.

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