WE HAVE

#30 MILLION REASONS

FOR EUROPEAN

ACTION

ON RARE DISEASES

EURORDIS
RARE DISEASES EUROPE

Rare2030
ACTION

Rare 2030
ACTION

THE FUTURE OF 30 MILLION PEOPLE LIVING WITH A RARE DISEASE IN EUROPE DEPENDS ON POLICY MAKERS TAKING ACTION, NOW.

While great progress has been made thanks to scientific advances and efforts at EU and national levels, too many people living with a rare disease face unmet needs and inequities in accessing a diagnosis, treatments and care, leaving them marginalised in society.

There is currently no overarching action for rare diseases at European level, and policies have not kept pace with new technologies.

Join EURORDIS and the rare disease community in the #30millionreasons campaign for a new EU Action Plan on rare diseases, that will leave no one behind by 2030.

There are 30 million people living with a rare disease in Europe. Every person living with a rare disease is a reason for Europe to have an action plan for rare diseases that leaves no one behind by 2030.

> WE ARE LIVING AN EVERYDAY EMERGENCY
>
> Terkel Anderson,
> President of EURORDIS, living with a rare disease

This factsheet explains why we need this:

3 MORE REASONS FOR ACTION NOW

1

PEOPLE LIVING WITH A RARE DISEASE HAVE A HIGH LEVEL OF *UNMET NEEDS*

Rare diseases are often debilitating, **frequently result in premature death**, and have a huge impact on daily life. 70% of rare diseases affect children, and as 72% of rare diseases are genetic they also affect future generations. People living with a rare disease also report their experience of care as worse than those living with other chronic diseases.[1]

This also makes them a vulnerable and neglected population: the COVID-19 pandemic has shown the impact this can have with 84% of people living with a rare disease in Europe experiencing disruptions to their care.[2] They are disproportionally affected by stigma, discrimination and social marginalisation.

NOW	by 2030
6% of rare diseases have a treatment	**1000** new therapies
5 YEARS to diagnosis	All people living with a rare disease to receive a diagnosis within **6** MONTHS
51% rare disease has a severe impact on daily life	Reduce the level of psychological, social and economic vulnerability by **1/3**

A EUROPEAN *ACTION PLAN* WILL...

SET GOALS TO IMPROVE THE LIVES OF PEOPLE WITH RARE DISEASES FOR EVERY COUNTRY TO ASPIRE TO

---

[1] EURORDIS Rare Barometer Voices, H-Care Survey, January 2021 - [2] EURORDIS Rare Barometer Voices, COVID-19 Survey, November 2020

2

CURRENT RARE DISEASE STRATEGIES ARE OUTDATED

The 2009 Council Recommendation gave the rare disease field a framework to build much of the infrastructure that has brought us progress we see today, including European Reference Networks (ERNs) and National Plans and Strategies across 23 countries in Europe.

However National Plans are mostly obsolete or expired, ERNs have not been duly integrated into healthcare systems, and this legislation has not kept pace with advances in science and technologies - such as newborn screening and advanced therapies - that mean we have new ways to diagnose and treat people living with a rare disease. This needs to happen equally across Europe.

2000	2006	2009	2014	2030
Orphan Medicinal Products Regulation	Paediatric use of medicines Regulation	Council Recommendation on an action on rare diseases	Cross Border Healthcare Directive	ACTION

A EUROPEAN ACTION PLAN WILL...

GIVE A NEW FOCUS TO NATIONAL PLANS TO ENSURE THAT SCIENTIFIC, TECHNOLOGICAL AND THERAPEUTIC ADVANCES REACH EVERY PERSON LIVING WITH A RARE DISEASE IN EUROPE

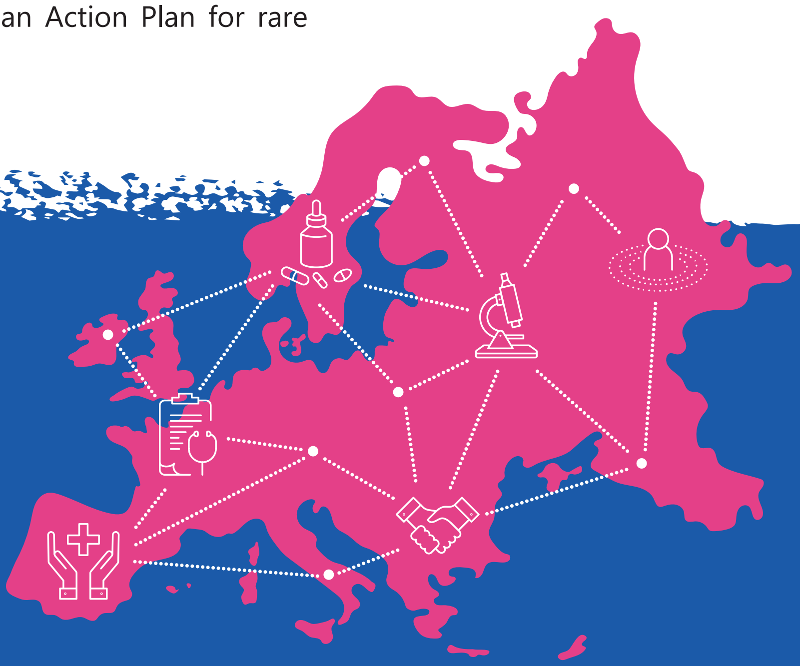
3

RARE DISEASES DO NOT WORK IN SILOS

There is currently no overarching strategy for rare diseases at a European level. The nature of rare diseases - affecting fewer than 1 in 2000 people, and with over 6000 different diseases - means that for the best possible treatment, care, research experts across Europe need to collaborate.

While there are pockets of best practice and excellent work happening in specific areas, rare diseases do not work in geographical or thematic silos. Without a renewed focus at European level through a cross-cutting strategy looking at the pathway as a whole, with data, research and patient partnerships bringing this together, people living with a rare disease are missing out on opportunities to fulfill their potential.

The Rare 2030 Foresight Study set out eight recommendations as a roadmap to achieve this over the next decade: they need to be implemented in a concrete European Action Plan for rare diseases.



A EUROPEAN ACTION PLAN WILL...

BRING TOGETHER ACTIONS, INCLUDING EXISTING LEGISLATION, ACROSS THE RARE DISEASE PATHWAY WHERE THE EU CAN ADD THE MOST VALUE UNDER ONE FRAMEWORK TO FOSTER RESEARCH AND CARE

Rare 2030 ACTION

The ambition and infrastructure set at European level has an impact on the way that every person living with a rare disease in Europe is diagnosed, cared for and treated in society.

An Action Plan will work towards three goals to improve the lives of people living with rare disease:

- **Stop people losing their lives too young from rare diseases**
- **Improve the quality of life of people living with a rare disease**
- **Ensure Europe is a global leader in rare disease innovation**

Ask your local policy makers to support our call for a European Action Plan on rare diseases now to ensure that no one living with a rare disease is left behind!

TAKE ACTION

EURORDIS
RARE DISEASES EUROPE

ABOUT EURORDIS

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.

The Rare 2030 Campaign is led by EURORDIS, in partnership with our European National Alliances and EURORDIS members.