

#Pledge4RD  
#EUElections2019



AS A RETURNING MEP OR CANDIDATE

# PLEDGE YOUR SUPPORT FOR RARE DISEASES

A RARE OPPORTUNITY TO SHOW THE ADDED VALUE OF EUROPE!

To ensure that the **25 - 30 million** people living with a rare disease in Europe and their families are not left behind and achieve their highest potential of health and well-being **you** can pledge to support a **new political framework** that:

1



ENABLES ALL PEOPLE  
LIVING WITH A RARE  
DISEASE TO RECEIVE AN  
ACCURATE DIAGNOSIS AND  
APPROPRIATE CARE WITHIN  
ONE YEAR OF COMING TO  
MEDICAL ATTENTION

2



SUPPORTS HOLISTIC CARE  
AND SOCIAL SYSTEMS  
THAT ARE INCLUSIVE OF  
PEOPLE LIVING WITH A RARE  
DISEASE, THROUGHOUT  
THEIR LIVES

3



SEIZES OPPORTUNITIES IN  
SCIENCE AND INNOVATION  
THAT EMBODY HOPE FOR  
PEOPLE LIVING WITH RARE  
DISEASES AND THEIR CARERS  
AND CAN CHANGE THEIR  
LIVES

**PEOPLE WITH RARE DISEASES AND THEIR REPRESENTATIVES MUST BE ENGAGED  
IN ALL FORA WHERE DECISIONS THAT AFFECT THEIR LIVES ARE MADE.**

We call on you to commit to developing a new political framework, one with stronger collaboration at European level and increased support for Member States.


FIND OUT HOW TO  
**#Pledge4RD**

## WHAT IS A RARE DISEASE?

Rare diseases affect **25-30 million people in the EU**, nearly 5% of the EU population, or the **population of Belgium and the Netherlands combined**. This estimate only increases when considering the carers, families and friends who need to support their loved ones.

**Rare is common:** Many people are affected by a rare disease or know someone who is.

OVER  
**6000**  
distinct rare diseases

Affects approx.  
**5%**   
of the population in  
the course of their lives

Each one affects  
fewer than  
**1 IN  
2000** PEOPLE

**NO  
CURE**   
for the vast majority  
of diseases and few  
treatments available



All together,  
an estimated  
**25/30  
MILLION PEOPLE**  
are living with a rare  
disease in Europe and  
**300 MILLION**  
worldwide

## WHAT IS AT STAKE?

Due to their low prevalence, little is known about most rare diseases. As a result, they are poorly diagnosed and their symptoms under-recognised in our healthcare and social systems.

Even when diagnosis is possible, the vast majority of rare diseases do not have a cure or even a treatment, and research to advance care and develop treatments is limited.

**Ultimately, the 25-30 million people living with rare diseases in the EU represent a significant group of citizens that need your support to be integrated into society.**

## HOW EUROPE IS ADDRESSING THE CHALLENGE OF RARE DISEASES

The most effective strategies for rare diseases are cross-border and EU-wide. **Europe enables the rare disease community to create the critical mass of patients, experts, knowledge, guidelines and resources needed by coordinating and adding value to national efforts.**

Rare diseases are an area with high European added value, where coordinated EU action has borne fruit. Thanks to the collaborative engagement of European and national policy makers, people with rare diseases, their families and patient associations, the landscape has changed drastically during the last years. As a society, we have gone from near ignorance to the recognition of rare diseases as a public health priority in Europe.

## THE ADDED VALUE

OF EUROPE FOR RARE DISEASES:

**25 NATIONAL PLANS**  
on rare diseases in  
2018 compared to  
4 in 2008

Many key European  
rare disease  
**RECOMMENDATIONS  
AND LEGISLATIONS**

**24 EUROPEAN  
REFERENCE NETWORKS**  
for rare and complex  
diseases

**OVER 1 BILLION**  
in RD research  
funded

**154 ORPHAN MEDICINES**  
now on the market, 62 to be used  
in children, since 2000



**BUT THERE STILL IS UNFINISHED BUSINESS...**

## YOUR #PLEDGE4RD

You have the **rare opportunity** to improve the lives of millions of people living with a rare disease, their families and carers through collective European action.

To ensure that the 25-30 million people living with a rare disease in Europe, their families and carers, are not left behind and achieve their highest potential of health and well-being you can pledge to support a new political framework that:



1

**ENABLES ALL PEOPLE LIVING WITH A RARE DISEASE TO RECEIVE AN ACCURATE DIAGNOSIS AND APPROPRIATE CARE WITHIN ONE YEAR OF COMING TO MEDICAL ATTENTION<sup>1</sup>**

- Ensure access to timely and accurate **diagnosis** by increasing awareness and knowledge on rare diseases at the primary care level, expanding access to diagnostic tests and genetic counselling.
- Facilitate **universal access** to high quality rare disease **healthcare** and treatments.
- Promote classification and codification of rare diseases that can support better **recognition of rare diseases** and their integration into national healthcare systems.
- Decrease inequalities across EU countries and regions by promoting **cross-border sharing of expertise and data**, involving patient representatives at all levels and supporting cooperation among their organisations.
- Support initiatives promoting **equal access to therapies** for rare diseases through cross-border collaboration, including joint work to assess the value of advanced therapies.

2

**SUPPORT HOLISTIC CARE AND SOCIAL SYSTEMS THAT ARE INCLUSIVE OF PEOPLE LIVING WITH A RARE DISEASE, THROUGHOUT THEIR LIVES**

- Secure renewed EU-level endorsement of comprehensive **national strategies for rare diseases** that are adequately funded and support the operations of centres of expertise for rare diseases.
- Establish **healthcare pathways** for patients that smoothly link primary care and laboratory resources to specialised centres and European Reference Networks (ERNs).
- Secure EU funding and support for **ERNs** and their **integration into national health systems** to enable them to fulfill the promise of delivering care for people with rare or complex diseases.
- **Bridge the gap between social and health care**, ensuring that people living with a rare disease and their family carers have access to integrated and multidisciplinary health and social care<sup>2</sup>.

3

**SEIZES OPPORTUNITIES IN SCIENCE AND INNOVATION THAT EMBODY HOPE FOR PEOPLE LIVING WITH RARE DISEASES AND THEIR CARERS AND CAN CHANGE THEIR LIVES**

- Continue support for **rare disease research** with increased EU and national funding to secure Europe's role in international research collaboration.
- **Pool resources** to optimise the use of funding, infrastructures and technological platforms that advance rare disease research, optimising public funding both at the EU and national levels.
- Guarantee European cooperation on **patient registries and data collection** in the context of ERNs, to maximise their potential, promote new standards of care and clinical guidelines.
- Prioritise research to address **unmet needs** in therapy development.
- Facilitate research and boost innovation of therapies and **cutting-edge technologies** that **empower** people affected by rare diseases and improve their **quality of life**.

To achieve all the above

**People with rare diseases and their representatives must participate in all fora where decisions that affect their lives are made and be engaged in a meaningful way.**

**The organisations that represent them must be recognised and financially supported, so to maintain the role of patients as major drivers of change.**

<sup>1</sup> In line with the vision of IRDiRC (International Consortium for Rare Disease Research) for 2017-2027.

<sup>2</sup> In compliance with the European Pillar of Social Rights, the UN Sustainable Development Goals and the Convention on the Rights of People with Disabilities

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## HOW ?

1



**DOWNLOAD THE #PLEDGE4RD SIGN** from <http://www.eurordis.org/pledge4RD>

2



**HOLD THE SIGN UP AND SHARE YOUR PHOTO**

Post it on social media using #Pledge4RD #EUelections2019 and tagging @eurordis

We will widely share your #Pledge4RD with the larger public via our social media and communication channels including national rare disease associations under our umbrella.



**SEE TESTIMONIES FROM PEOPLE  
LIVING WITH A RARE DISEASE:  
[HTTP://RARE DISEASE DAY.ORG/VIDEOS](http://rare Diseaseday.org/videos)**



### GET IN TOUCH

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