RARE DISEASE DAY 2020
POLICY BRIEFING
FROM PLEDGE TO ACTION

European Parliament, Brussels
Room 3H1
18th of February, 10h00 – 11h00

EURORDIS.ORG
Our agenda for today

Welcome and opening

What are rare diseases? What is the network, rationale, main purpose

What topics will be working on? Where does EURORDIS have expertise in to offer?

How can you help concretely?

Q&A/Closing
WHAT ARE RARE DISEASES?
There are 6,000+ identified rare diseases


#RareDiseaseDay
29 FEBRUARY 2020
72% OF RARE DISEASES ARE GENETIC

whilst others are the result of infections (bacterial or viral), allergies and environmental causes or are rare cancers.


#RareDiseaseDay
29 FEBRUARY 2020
70% of genetic rare diseases start in childhood.


#RareDiseaseDay
29 February 2020
300 MILLION PEOPLE WORLDWIDE living with a rare disease


#RareDiseaseDay 29 FEBRUARY 2020
8


#RareDiseaseDay
29 February 2020
Collectively the number of people living with a rare disease is equivalent to the population of the world's 3rd largest country.

300 million people worldwide are living with a rare disease.

#RareDiseaseDay
29 February 2020
The EU added value (1): Pooling knowledge to improve diagnosis
The EU added value (2): partnership for research
The EU added value (3): therapies development

Orphan medicines key figures

Since 2000

- 2216 Orphan designations
- 176 Authorised OMPs
- 68 To be used in children
- 5 Removed from the market
- 62 Marketed, but no longer “orphans”

To date

- 209 Orphan designations included in authorised indication
- 109 Products with a marketing authorisation and an orphan status in the European Union

17 December 2019
Rare diseases seriously impact everyday life

7 in 10 patients & carers reduced or stopped professional activity due to their or their family member’s rare disease.

8 in 10 patients & carers have difficulties completing daily tasks (household chores, preparing meals, shopping etc.)

2/3 of carers spend more than 2 hours a day on disease-related tasks.

3 times more people living with a rare disease and carers report being unhappy and depressed than the general population*

* Rare Barometer Voices sample compared to International Social Survey Programme, 2011
An insight into the participation of rare disease patients in research

Key findings of a Rare Barometer Voices survey on the participation of rare disease patients in research:

Top 3 obstacles to rare disease research:
- Lack of public funding
- Lack of private funding
- Small patient population

Top 3 priorities for patients:
- Develop therapies
- Improve diagnosis
- Identify mechanisms & causes of rare diseases

What type of research do patients participate in?

1/3 of rare disease patients have participated in medical research, among them:
- 59% to develop treatments & therapies
- 52% in research on quality of life
- 16% to develop genetic therapies

What matters to patients?

- Participating in research to help science & the community
- Receiving clear information about the research project
- Having a quality relationship with the researchers

Rare Barometer Voices is a EURORDIS-Rare Diseases Europe online survey initiative. It brings together over 8,000 patients, carers and family members to make the voice of the rare disease community stronger.

Thank you to all Rare Barometer Voices participants and partners! To read the full report, register to participate in future surveys or for more information, visit: eurordis.org/voices
Only 5% have already experienced a **curative** treatment

3% a treatment to **prevent** the disease

31% a treatment to **slow down** the disease

62% a symptomatic treatment

69% have already experienced a treatment

31% have never experienced any treatment
During the past 12 months...

<table>
<thead>
<tr>
<th>Reason</th>
<th>Rare disease patients</th>
<th>General public</th>
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<tbody>
<tr>
<td>Were unable to get their medical treatment because they could not pay for it</td>
<td>12%</td>
<td>6%</td>
</tr>
<tr>
<td>Were unable to get their medical treatment because it was not available where they live</td>
<td>22%</td>
<td>7%</td>
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<tr>
<td>Were unable to get their medical treatment because the waiting list was too long</td>
<td>14%</td>
<td>9%</td>
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WHAT IS THE NETWORK?
Why a network of Parliamentary Advocates for Rare Diseases?

- European Union added-value of action on rare diseases
  - Need of action beyond national borders

- Breakthrough legislation driven mostly by the EU institutions & implemented at national level
  - Need for collaboration at EU level & between the EU and national level

- Create a pool of advocates amongst policy makers
  - Need to raise awareness amongst decision-makers
  - Strong signal of Parliamentarians commitment & relevance of rare diseases
Core missions of the Parliamentary Advocates for Rare Diseases

1. Explore and discuss specific challenges faced by people living with a rare disease

2. Shape political input for future legislation and programmes in a new framework for Rare Diseases

3. Work to make rare diseases an integral part of EU, national and regional programmes in health, research, social affairs and other relevant policies
From Pledge to Action

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.
1. Reframe the current European strategy tackling rare diseases to adequately address the needs of the rare disease community

2. Advancing the work that has been initiated during the previous legislature to support EU-wide coordination on HTA

Support holistic care

1. Ensure that in the MMF 2021 – 2027 and successive annual budget and related programme, **adequate and streamlined funding is appropriated for activities of the European Reference Networks (ERNs)**

2. Promote the **implementation of the recommendations on ERN integration in national healthcare systems**;

3. Ensure that measures to achieve holistic care for people living with rare diseases and their families, such as access to social services, disability assessment for rare diseases are streamlined in the upcoming **European Disability Strategy**
Foster research

1. Promote the importance of fostering research in the area of rare cancers in the context of the Commission’s Beating Cancer Plan.

2. Support the rare disease patient community in getting engaged in the upcoming discussions around the Pharmaceutical Strategy, including the legislation on medicines for special populations (Regulation on Orphan Medicinal Products and on Paediatrics).

3. Support rare diseases research by ensuring adequate support to a successful Partnership like the European Joint Programme on Rare Diseases.
Organise a European Parliament (EP) event in the context of the RareImpact project that addresses the difficulties of patients accessing advanced therapies;

Promote and organise the final meeting of the Rare 2030 pilot project, promoted by the European Parliament and co-funded by the European Commission.

Encourage patient organisations to participate in European policy-making by supporting 2021 Rare Disease Week, either by meeting your constituents during this week and/or organise a bigger meeting in the EP.
EURORDIS EXPERTISE

EURORDIS.ORG
Our mission

EURORDIS works across borders and diseases to improve the lives of people living with a rare disease

869 Member patient organisations

71 countries (28 EU countries)

44 National Alliances of rare disease patient organisations

72 European Federations for specific rare diseases

1997 Founded in

2,500 Outreach to over patient groups

440 Over volunteers

40+ Staff members with offices in Paris, Brussels and Barcelona
Advocating, empowering & engaging patients in the areas of:

- Research
- Diagnosis
- Treatments
- Healthcare
- Rare disease policy
- Social care
EURORDIS volunteers at the heart of policy making

**440+ volunteers involved in EURORDIS activities**

- **Drug Information, Transparency and Access Task Force (DITA)**
  focusses on the areas of product information, transparency of the regulatory process and access to medicines

- **ePAG Advocates** who are members of the EURORDIS **ePAG Steering Committee** active across 24 European Reference Networks

- **Rare Disease Day Working Group**
  Co-creating the global campaign

- **THA Task Force**
  to facilitate the participation of patients in health technology assessment activities

- **340+ RareConnect moderators**
  animate the RareConnect communities

- **30+**

- **SPAG (Social Policy Action Group)**
  advocating for holistic & integrated care

- **Therapeutic Action Group (TAG)**, composed of EURORDIS volunteers in the scientific committees and working party at the **European Medicines Agency (EMA)**
HOW CAN YOU HELP CONCRETELY?