WE HAVE

#30 MILLION REASONS

FOR EUROPEAN

ACTION

ON RARE DISEASES
WE NEED EUROPE’S ACTION PLAN FOR RARE DISEASES BECAUSE...

...The EU has the science, the healthcare and the industry to help patients living with rare diseases. Political will and incentives can turn science into treatments!

Boris, France

Image from EURORDIS Photo Contest and is not associated to the reason
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...Why do I have to visit many different doctors to get treatment? I would like to receive holistic treatment from one place.

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… As a physician working in the field of inherited metabolic disorders, I fully support EU-wide initiatives on rare diseases. We need to exchange expertise and study pan-European cohorts of patients with individual rare disorders to improve access to timely diagnosis and effective treatments, and to ultimately ensure better quality of life for patients we are serving.

Viktor, Czech Republic
WEBINAR: #30MILLIONREASONS
CAMPAIGN UPDATE
15 SEPTEMBER 2021
AGENDA & OBJECTIVES

- What are we asking for?
  Review the Action Plan content
  Q&A

- Where are we now?
  What have we done together so far?
  What is coming up next?
  How can you help?
  Q&A
CALLING FOR EUROPE’S ACTION PLAN ON RARE DISEASES NOW
WHAT IS AN ACTION PLAN?

- New instrument in EU policy = Commission Communication
- Part of Better Regulation Agenda to ensure evidence based, simplified and transparent policy making
- Components include:
  - Roadmap
  - Public consultation
WHAT WILL AN ACTION PLAN FOR RARE DISEASES DO?

- Give **renewed focus** to rare diseases as a public health priority: a new Council Recommendation in the field of rare diseases
- Drive **European and national strategies and plans** with adequate funding
- Integrate **European and national levels of action**
- Set **measurable goals** for every country and sector to work towards

Rare 2030  Europe’s Action Plan  Measurable goals to address unmet needs
CURRENTLY TOO MANY OF EUROPE’S CITIZENS LIVING WITH A RARE DISEASE

**DIE PREMATURELY**
- 70% of rare disease start in childhood
- The odyssey to diagnosis lasts an average of 5 years
- Data and experience to understand and best care for rare diseases is often lacking

**EXPERIENCE SIGNIFICANT INEQUALITIES**
- People living with rare diseases are often not integrated in society
- Are impacted significantly in their daily life
- Have a poorer care experience

**LACK ACCESS TO EFFECTIVE TREATMENTS**
- Only 6% of RDs have a dedicated treatment, few are transformative
- In the current trajectory only 600 new treatments for rare diseases may become available by 2030

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CURRENT POLICIES ARE OUTDATED: A NEW POLICY CYCLE TO 2030

2000
Orphan Medicinal Products Regulation

2006
Paediatric use of medicines Regulation

2007
Regulation on Advanced Therapy Medicinal Products

2008
Commission Communication Rare Diseases

2009
Council Recommendation for Rare Diseases

2014
Cross Border Healthcare Directive

12 Trends Rare 2030: New technologies, new values, new expectations

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RARE 2030 RECOMMENDATIONS: A ROADMAP FOR RARE DISEASE POLICY

- Eight detailed policy recommendations across diagnosis, access to healthcare, integrated care, partnerships with patients, research, data and treatments
- Central recommendation for a new European Policy Framework driven by the needs of people living with a rare disease, to guide the implementation of consistent national plans and strategies
Europe’s Action Plan for Rare Diseases would introduce measurable goals, such as those in cancer or obesity, to ensure that the whole of Europe is working towards the same goals, so that inequalities are not exacerbated by the country in which someone lives:

1. Better health and well-being
2. Reduced inequalities
3. Industry innovation and infrastructure
BY 2030…

1. Better health and well-being
   e.g. Reduce diagnostic delays for 90% of people living with a rare disease to six months, from years or decades
   e.g. Extend life expectancy by an average of three years across diseases

2. Reduced inequalities
   e.g. Reduce psychological, social and economic vulnerability of people living with a rare disease and their families by one third

3. Building innovation and infrastructure
   e.g. The approval of 1000 innovative (symptomatic or transformative) treatments for people living with a rare disease
8 Rare 2030 Recommendations

1. European/national plans and strategies
2. Diagnosis
3. Access to care
4. Person-centred care
5. Patient Partnerships
6. Research
7. Data
8. AAA Treatment

Europe’s Action Plan

GOAL 1: Ensuring healthy lives and promoting well-being for all PLWRD

GOAL 2: Reducing inequalities for PLWRD

GOAL 3: Building resilient infrastructure, promoting inclusive and sustainable industry and fostering innovation for PLWRD

4 SDGs

SDG1: Ensure healthy lives and promote well-being for all at all ages

SDG2: Build resilient infrastructure, promote inclusive and sustainable industrialisation and foster innovation

SDG3: Reduce inequalities within/among countries

SDG10: Revitalise the global partnership for sustainable development

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**SDGs**

**ACTION PLAN’S MEASURABLE GOALS:**
1) Better health and well-being 2) Reduce inequalities 3) Industry innovation & infrastructure

**Area of Action 1**
Research
**Supporting actions**

**Area of Action 2**
R&D Policy
**Supporting actions**

**Area of Action 3**
Access
**Supporting actions**

**Area of Action 4**
Diagnosis
**Supporting actions**

**Area of Action 5**
Healthcare
**Supporting actions**

**Area of Action 6**
Holistic care/Human Rights
**Supporting actions**

Cross-cutting areas of action 1) Digital/Data 2) Partnerships

**Special Focus**
*On the 5000 rare diseases affecting less than 1 person in 1 million*

*Working proposal put forward by EURORDIS*
Q&A
WHAT HAVE WE ACHIEVED SO FAR...?

...AND WHAT IS COMING NEXT?
A LAYERED STRATEGY

European Parliament

European Commission

New Policy Framework

National Plans

Better care, treatment, QoL and outcomes for PLWRD

Advocacy and Internal influencing

Grasstops

National Authorities/KOLs/Patient advocates

EU Council (Member States)

Soft/political pressure

Legislative process

Political pressure/agenda setting

Legislative process

Business as Usual: Advocacy and Operations by EURORDIS and its Partners
Timeline
Working Proposal for Europe’s Action Plan for Rare Diseases

2021
- Portugal: 23 Feb 2021 Rare 2030 Policy Conference
- Slovenia: 31 May 2021 Rare 2030 Final Recommendations
- France: 28 Feb 2022 EU Council Presidency High Level Conference on RDs
- Czech Republic: Sept - Nov 2022 Technical Meeting on Europe’s Action Plan

2022
- France: June 2022 - November 2021 Informal discussions with Commission DGs
- France: EU4Health WP 2023
- France: June 2022 ECRD 2022 as official event during the French Presidency
- Sweden: Feb 2023 Presentation of the EC Roadmap
- Spain: April - Sept 2023 Consultations

2023
- Spain: Dec 2023 EC Adoption of Action Plan
EU MEMBER STATES: THE TRIO

1. **RDs on the Trio Presidency Agenda**

   “the Trio [...] will encourage further measures to tackle antibiotic resistance and rare diseases, at the European and at the worldwide level”

2. **France – High Level Conference 28 February 2022**
   - Cabinet of Ministry of Health, Research and European Affairs
   - National Parliament
   - Cabinet of President and Prime Minister

3. **Czech Republic**
   - RD on Agenda of Presidency
   - OD and Pediatric Regulation postponed during this calendar
   - Plans of Technical Meeting on European Action Plan for Rare Diseases
EUROPEAN COMMISSION

- Action Plan Roadmap no earlier than Spring 2023
- A Working Proposal being tested with representatives of the European Commission through:
  - Letter to President Von der Leyen – May 26
  - Informal Andrzej Rys – June 3rd – full proposal for test
  - Emails to Commissioners Dalli, Schinas, June 7-11 + relaunch September
  - Meeting with Stella Kyriakides cabinet – June 17
  - Meeting with John Ryan and Andrzej Rys (DG Sante Unit B and C) – relaunch proposal
Main actions include:

- Revival of Parliamentary Group of Advocates – focus on Action Plan
- Oral question for Resolution
- Late 2021/early 2022 Campaign event leading up to the French Presidency
WHAT IS AN ORAL QUESTION?

- Puts pressure on European Commission for an Action Plan
- Process:
  1. ENVI committee submits question
  2. Conf. of Presidents decide whether and when to table it
  3. Plenary session with debate – most likely w/c 14 Feb
- Role of EURORDIS members: maximising MEP engagement
CALLING ON THE EUROPEAN INSTITUTIONS TO TAKE ACTION

SHARE YOUR REASON FOR ACTION ON RARE DISEASES!

EVERY REASON BRINGS US CLOSER TO A EUROPEAN ACTION PLAN ON RARE DISEASES.

Share yours with Ursula von der Leyen, President of the European Commission.

Can you help us get 3,000 reasons to hand over to Ursula van der Leyen?

1,310 people have given their reason. Will you help us get 1,690 more?

First name *

Last name *

E-mail address *

eurordis.org/30millionreasons
GRASSROOTS: COLLECTING REASONS

- 1439 reasons so far: aim for 2000 by the end of Sep
- Preparing book for hand in – case studies
- Preparing video for event – case studies

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<td>Ireland</td>
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<td>Sweden</td>
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</table>
"My country has shared 753 reasons for European action, now.

"My son is one of only 1000 living with his condition in Europe. We should not have to travel to access the best care and treatment... Working together across Europe is essential for us to share knowledge and expertise to improve his life and those who will follow him."

"I, as my country’s health minister, fully support the call for Europe’s Action Plan."

"We need better coordination to reduce waiting times and clarify care pathways for patients and families." - Simona, Rare Disease Advocate

"It took 7 years to get a diagnosis and 23 years to get the treatment I needed, provided by my country’s health minister, but that is not good enough. Working together across Europe is essential for us to share knowledge and expertise to improve his life and those who will follow him."

"To ensure that all people living with a rare disease get the opportunity to live their lives in the best possible way." - Stephanie, Sweden

"We cannot accept to see children not receiving the existing treatments for economic reasons." - Simona, Rare Disease Advocate
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  - Hand-in of #30 million reasons @RDW
- 31 May 2021: Rare 2030 Final Recommendations
- France
  - June 2021 – November 2021: Informal discussions with Commission DGs
  - Launch of #30 million reasons campaign including formal contacts to EC President, Commissioners and DGs
- 2022
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- June 2022: ECORD 2022 as official event during the French Presidency
ECRD 2022: WHEN AND WHERE?

THE 11th EUROPEAN CONFERENCE ON RARE DISEASES & ORPHAN PRODUCTS

ONLINE
on 27 June - 1 July

Save the date!

eurordis.org/30millionreasons
WHAT CAN YOU DO & WHEN?

Until Nov
- Share your reasons
- Encourage your networks to do the same!

Nov 21 – Feb 2022
- Engage your MEP for the Oral Question with debate

Feb & May 2022
- Save the date: High Level conference
- Save the date: ECRD 2022
WHAT CAN YOU DO?

- Share a reason for why your organisation needs Europe to take action at action.eurordis.org: this will be shared directly with the European Institutions
- Encourage the people you represent to share their reasons!
- Include the call for an Action Plan in meetings with policy makers
- Write to your MEP to ask them to join the Parliamentary Network for Rare Disease Advocates in the European Parliament
- Talk about #30millionreasons for Europe’s Action Plan for rare diseases on social media
CAMPAIGN TOOLKIT

- Platform in 23 languages
- Factsheet in 23 languages
- Rare 2030 recommendations
- Social media board
- Campaign press release
- Letter to MEP template
Rare Barometer survey on the journey of rare disease patients to diagnosis – one survey with two phases

- **Survey goal:** Identify personal and external factors influencing the process of obtaining timely and accurate diagnosis from a patient perspective

- **20-24 September:** Online discussion panel with patient representatives on Opinionway platform. Registration link: [https://q.crowdtech.com/8yqnbk9pIEiDOkGUh8w1UQ](https://q.crowdtech.com/8yqnbk9pIEiDOkGUh8w1UQ)
  - A moderator will ask questions every day
  - Connect to the platform, answer questions, share testimonies/videos/images
  - Participate in the open discussion groups with other people living with a rare disease
  - **Around 15 – 20 minutes each day** for five days – you are free to chose what time of the day!

- **Mid November – end of December:** online questionnaire
THANK YOU!

… May a day come when we do not have to have reasons for everyone to have the right to a rapid diagnosis, access to treatment and the best possible care. Health is not a reason, it is a right!

Maria, Spain