

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

WE NEED EUROPE'S ACTION PLAN FOR RARE DISEASES BECAUSE...

...Why do I have to visit many different doctors to get treatment? I would like to receive holistic treatment from one place.

Satu, Finland



Image from EURORDIS Photo Contest and is not associated to the reason

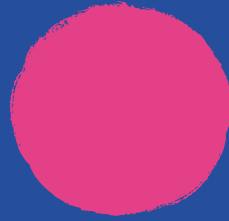
WE NEED EUROPE'S ACTION PLAN FOR RARE DISEASES BECAUSE...

... 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



Image from EURORDIS Photo Contest and is not associated to the reason



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



1

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



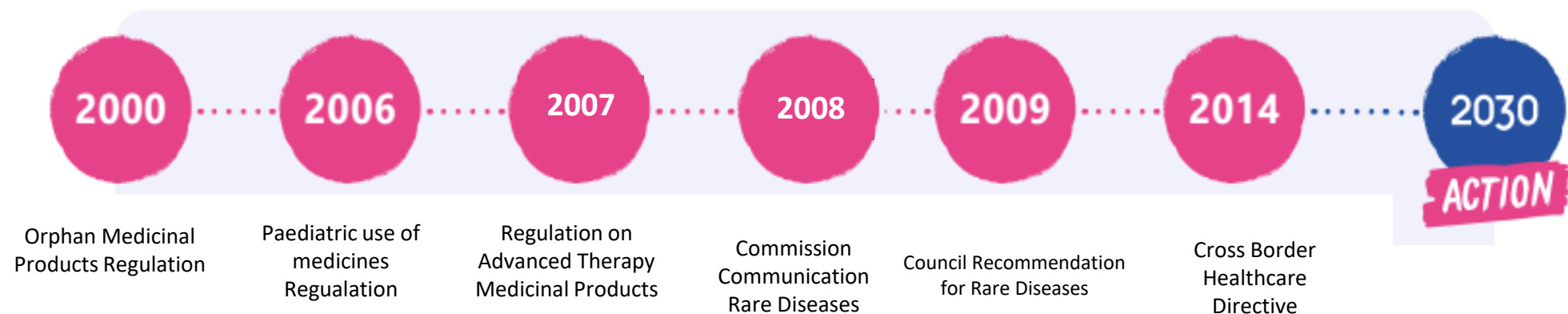
LACK ACCESS TO EFFECTIVE TREATMENTS

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



CURRENT POLICIES ARE OUTDATED: A NEW POLICY CYCLE TO 2030



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

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



SETTING MEASURABLE GOALS

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



SDG3: Ensure healthy lives and promote well-being for all at all ages



SDG 9: Build resilient infrastructure, promote inclusive and sustainable industrialisation and foster innovation



SDG10: Reduce inequalities within/among countries



SDG17: Revitalise the global partnership for sustainable development



SDGs

ACTION PLAN'S MEASURABLE GOALS:

1) Better health and well-being 2) Reduce inequalities 3) Industry innovation & infrastructure

Area of Action 1
Research

Area of Action 2
R&D Policy

Area of Action 3
Access

Area of Action 4
Diagnosis

Area of Action 5
Healthcare

Area of Action 6
Holistic
care/Human
Rights

Supporting
actions

Supporting
actions

Supporting
actions

Supporting
actions

Supporting
actions

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



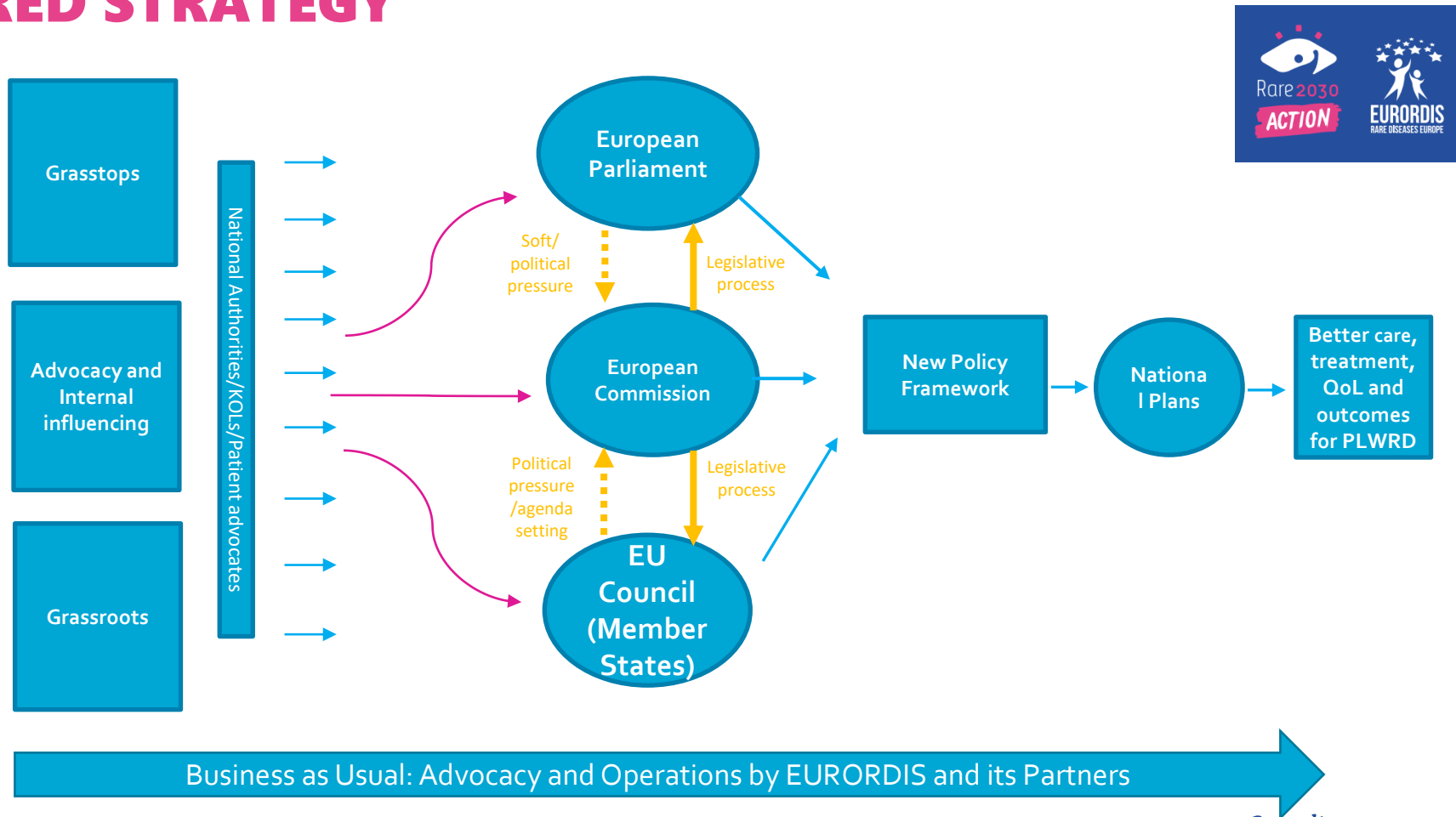
2

WHAT HAVE WE ACHIEVED SO FAR...?

...AND WHAT IS COMING NEXT?

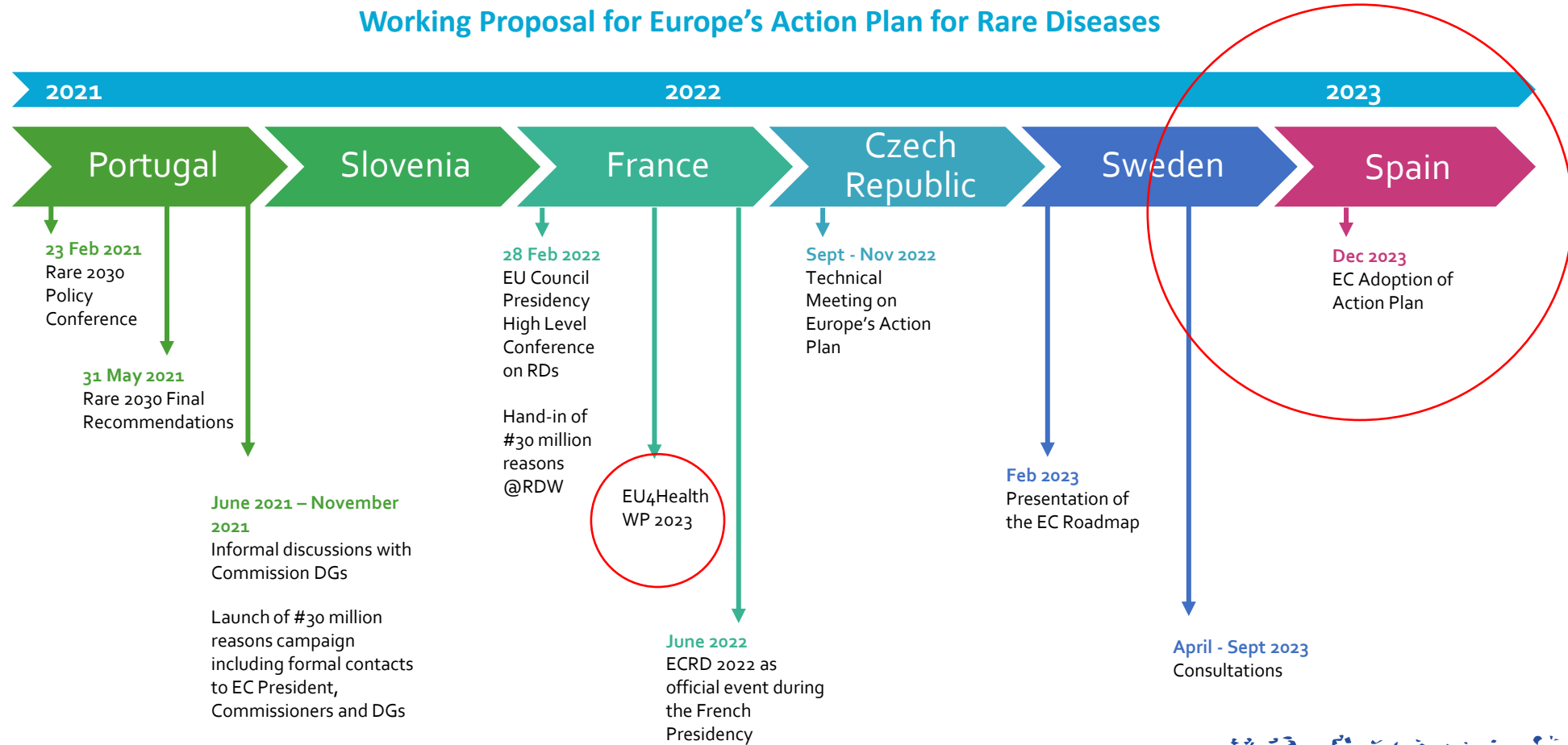


A LAYERED STRATEGY



Timeline

Working Proposal for Europe's Action Plan for Rare Diseases





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

EUROPEAN PARLIAMENT

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



Dear [MEP NAME]

I am writing to you (as your constituent/on behalf of my patient organisation, (insert name)), to ask you to become a member of the Parliamentary Network of Advocates on rare diseases.

There are 30 million people living with a rare disease in Europe. Yet no country can tackle the challenges of rare diseases alone. Their future depends on policy makers taking European action now.

The network of Parliamentary Advocates for Rare Diseases will help tackle challenges faced by the rare disease community by fostering cross-border EU collaboration.

Through the network of Parliamentary Advocates for Rare Diseases, EURORDIS-Rare Diseases Europe aims to bring together members of the European and national parliaments to ensure strong international and local action, shape political input for current and future legislation and integrate rare diseases into all relevant policies at all levels. There are several current and upcoming opportunities where we can continue to raise our points.

In particular, the Parliamentary Advocates will play a crucial in the #30millionreasons campaign, calling for an EU Action Plan on rare diseases to improve the lives of the 30 million people living with a rare disease in



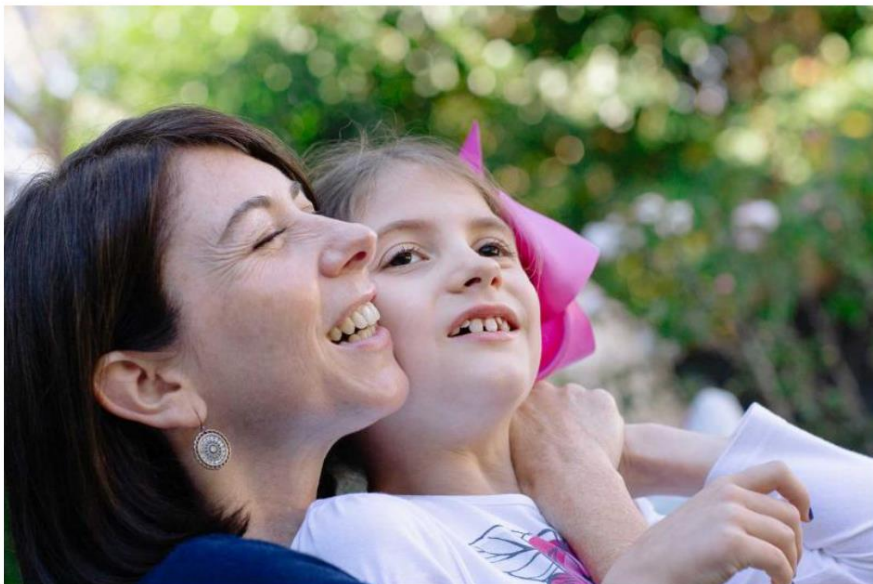
WHAT IS AN ORAL QUESTION?

- 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 *



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

Country	Total number of reasons
Germany	282
Italy	164
Spain	100
Belgium	85
Portugal	84
France	82
Czech Republic	52
Netherlands	49
Finland	43
UK	43
Austria	29
Ireland	27
Sweden	27

MY COUNTRY HAS SHARED 753 REASONS

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

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

“It took 7 years to get a diagnosis and 23 years to get the treatment I needed, provided by one person. I need joint efforts now!”
- Simona, Rare Disease Advocate

“We cannot accept to see children not receiving the existing treatments for economic reasons.”
- Simona, Rare Disease Advocate



NOW
EXAMPLE

“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 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

WE NEED EUROPE'S ACTION PLAN FOR RARE DISEASES BECAUSE...

...Why do I have to visit many different doctors to get treatment? I would like to receive holistic treatment from one place.

Satu, Finland



Image from EURORDIS Photo Contest and is not associated to the reason

WE NEED EUROPE'S ACTION PLAN FOR RARE DISEASES BECAUSE...

... 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.

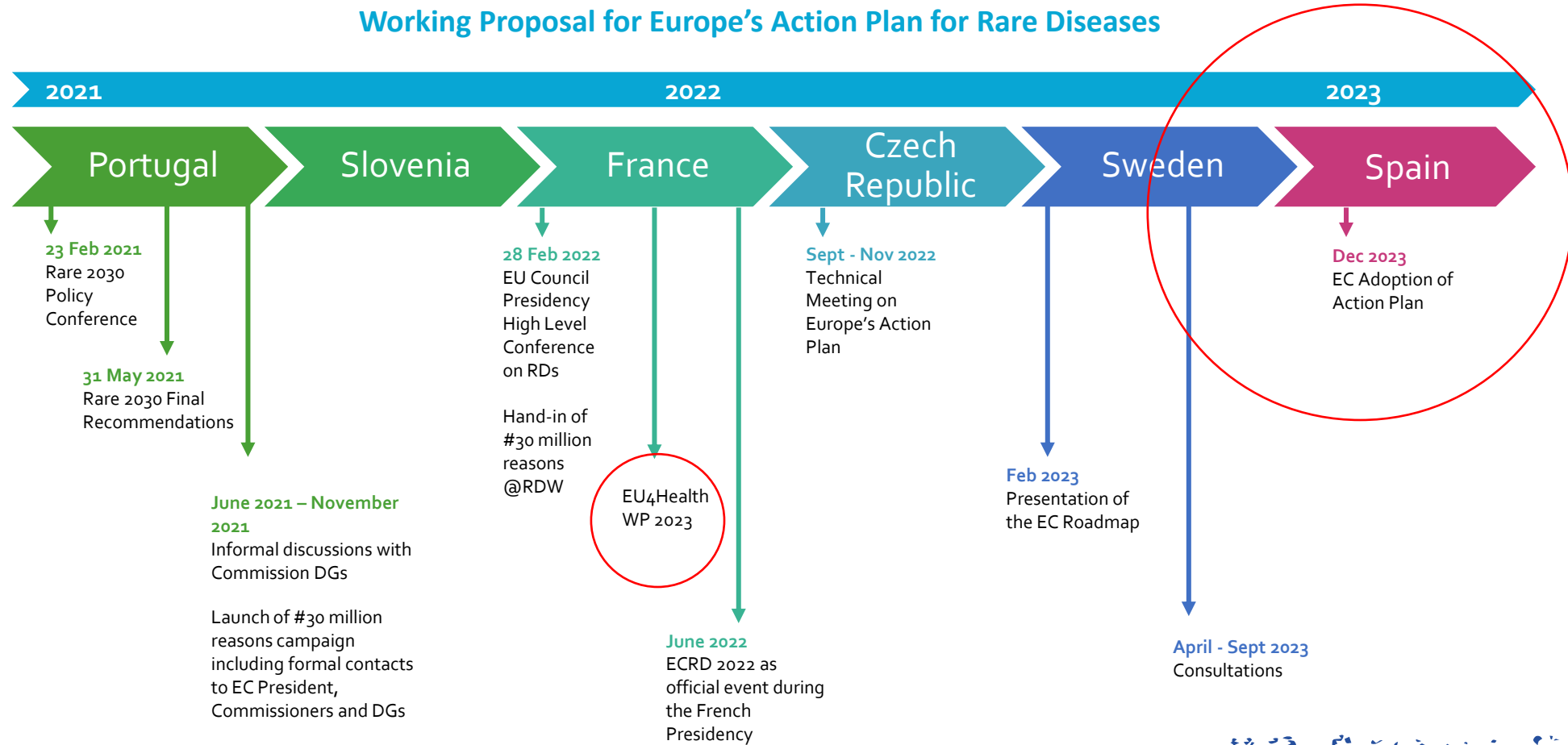
Viktor, Czech Republic



Image from EURORDIS Photo Contest and is not associated to the reason

Timeline

Working Proposal for Europe's Action Plan for Rare Diseases



ECRD 2022: WHEN AND WHERE?

THE 11th EUROPEAN CONFERENCE ON
RARE DISEASES & ORPHAN PRODUCTS



ONLINE

on 27 June - 1 July

2022



Save the date!



WHAT CAN YOU DO & WHEN?



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>
 - 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

