
WELCOME!

HOUSE KEEPING RULES

Q&A

Please add your questions in the Q&A chat!

Use the ‘regular’ chat to introduce yourself or make comments or suggestions!

Your microphone is going to be muted by default

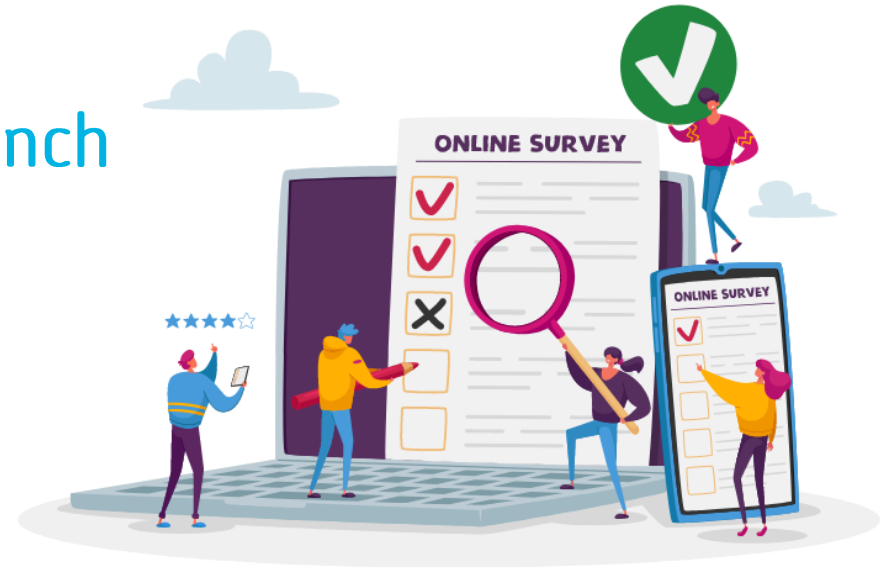
Use the “raise hand” button if you want to raise a question or share a point of view in public. The organisers will unmute you and give you the floor.

SOCIAL PARTICIPATION & INDEPENDENT LIVING FOR PEOPLE LIVING WITH A RARE DISEASE

A Rare Barometer Survey

Webinar for the survey launch

10/07/2024



OVERVIEW

1. SOME CONTEXT: EURORDIS' ADVOCACY AND RARE BAROMETER
2. SHAPING THE QUESTIONNAIRE
3. INSIGHT INTO THE QUESTIONNAIRE
4. HOW TO ENCOURAGE PARTICIPATION IN THE SURVEY
5. SURVEY RESULTS - WHAT EURORDIS WILL PROVIDE
6. PATIENT ORGANISATIONS' USE OF RESULTS

THE SURVEY IS LIVE!

10 JULY → 8 SEPTEMBER 2024

TARGET POPULATION

All people living with a rare disease and their family members

WORLDWIDE

25 languages

ALL rare diseases

tiny.cc/RB_DailyLife

1 SOME CONTEXT

EURORDIS-RARE DISEASES EUROPE

A unique, non-profit alliance of over 1000 rare disease patient organisations from 74 countries

Development & Access to Diagnostic & Therapies

Data & Digital Health

Healthcare Policy & Services

Research Policy & Activities

Social Policy & Services

PEOPLE FIRST

based on evidence and experience of unmet need and preferences

EMPOWER

To empower organisations and advocates to ensure that the rare disease community is well represented in policy discussions and decision-making at the EU level.

PARTNER

To establish and facilitate networks with strategic partners and key stakeholders.

ADVOCATE

To advocate for policies and innovative solutions driven by the needs of people living with rare diseases

OUR VISION

EURORDIS' vision is a world where all people living with a rare disease can have longer and better lives and can achieve their full potential, in a society that values their well-being and leaves no one behind.

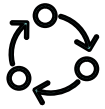
Eddison, xeroderma pigmentosum

Achieving their full potential:

Recognised as equal citizens with their rights fully respected

Diagnosed timely and accurately

Supported with state-of-the-art medical and social care, or cured



Included in society in all aspects of life and **enabled to live independently**

Strategic Objectives: 2021-2030

2- Delivering on 6 priority areas

By 2030, EURORDIS will have made contributions to the goals of
(Based on the Foresight Study Rare 2030):

- Integrated medical and social care with a
holistic life-long approach and inclusion in society

Goal of reducing the social, psychological and economic burden [or negative impact] by one third by 2030

What are we advocating for?

Position paper: holistic care

Pillar 1	Pillar 2	Pillar 3
Quality and adequate social services and policies	Integrated care: bridging health and social care	Equal rights & opportunities: Disability rights, independent living Employment Non-discrimination

https://www.eurordis.org/publications/position-paper-achieving-holistic-person-centred-care/

Disability:

Persons with disabilities include those who have long-term physical, mental, intellectual or sensory impairments which in interaction with various barriers may hinder their full and effective participation in society on an equal basis with others

UNCRPD Art. 1

Independent living:

equal right to live in the community

full inclusion and participation in the community

Opportunity to **choose where and with whom they live**

access to a range of in-home, residential and other community support services

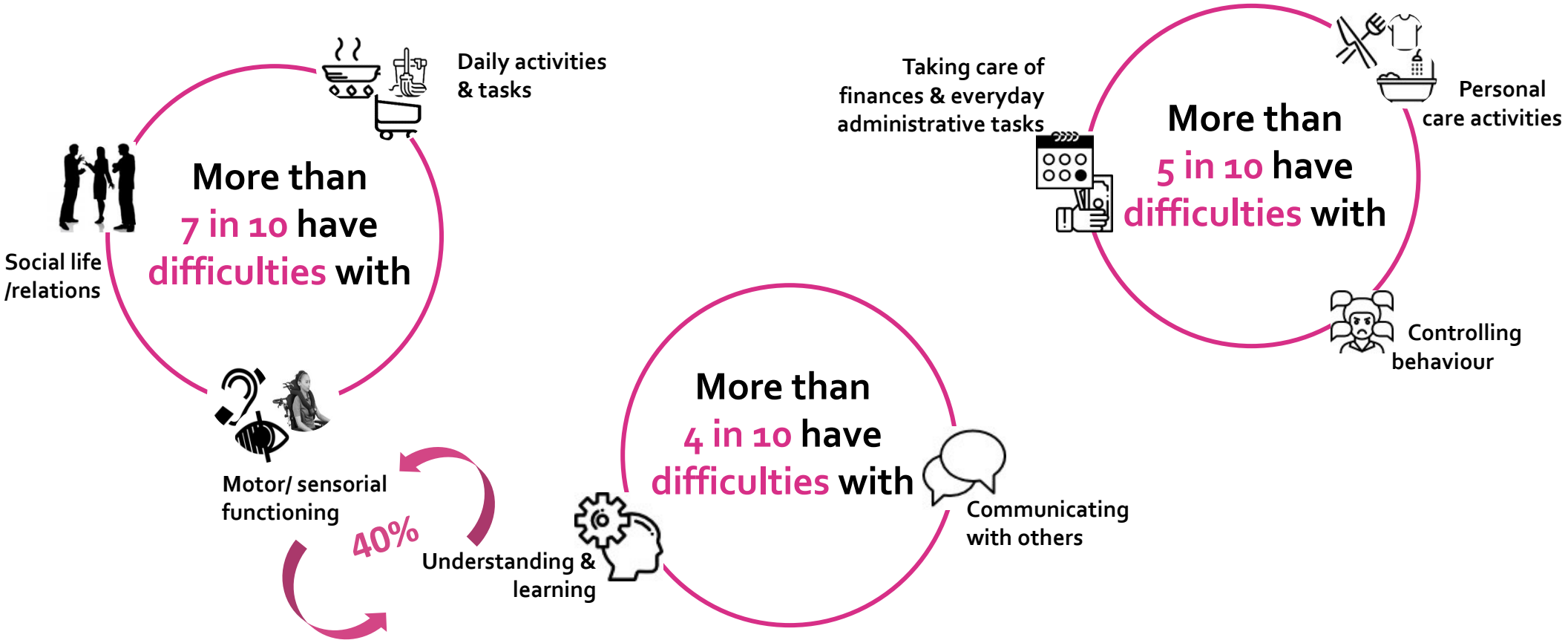
Community services and facilities available and **responsive to their needs.**

Evidence from a previous Rare Barometer Survey suggests that most people living with a rare disease face numerous barriers in their daily live and live with disabilities…

Now we need to know more about the many barriers they face to live to their full potential.

And more about why their disabilities are often not recognised.

What we know about people with rare diseases:



Survey "Juggling care and daily life: The balancing act of the rare disease community", 2017.

Disability Assessment is a barrier for 53%

34% report facing inadequate assessments.
19% do not receive an assessment at all but consider they would need one

Survey "Juggling care and daily life: The balancing act of the rare disease community", 2017.

But we need more evidence on…

The barriers to social participation and independent living, and their preferences.

Types of disabilities people with a rare disease live with

What is going wrong with the recognition of their disabilities.

How is their access to the support they need.

The human rights perspective

The problem is not our diseases and disabilities, but a society and a system that poses barriers and leaves out people with rare diseases.
There is nothing wrong with us.
It's up to society to change.

Goes further than the social model.
There is no excuse to discriminate or deny access to equal opportunities.

Source: https://www.wdv.org.au/wp-content/uploads/2022/04/Understanding-Disability-Mar2022-v3-SB.pdf

THE RARE BAROMETER PROGRAMME

eurordis.org/voices

EURORDIS' survey initiative to support evidence-based advocacy

Robustly collects experiences and opinions of people living with a rare disease and their close family members, on topics that directly affect them.

Transforms those experiences and opinions into facts and figures to feed the advocacy work of the rare disease community.

WHAT

Not-for-profit initiative

WHO

Run independently by EURORDIS-Rare Diseases Europe

WHEN

Created in 2016

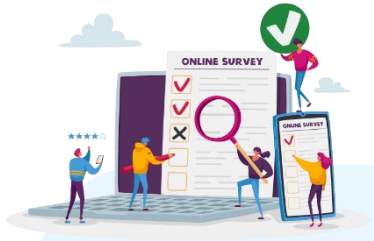
WHY

Evidence-based advocacy

THE RARE BAROMETER PROGRAMME

eurordis.org/voices

EURORDIS' survey initiative to support evidence-based advocacy



Surveys

People living with a rare disease & family members

1-3 studies per year

25 languages

Worldwide

Up to 13,000
respondents to our surveys

Panel

20,000+
people living with a rare disease registered

2,300+ rare diseases

120+ countries

People DO NOT have to register to participate in surveys

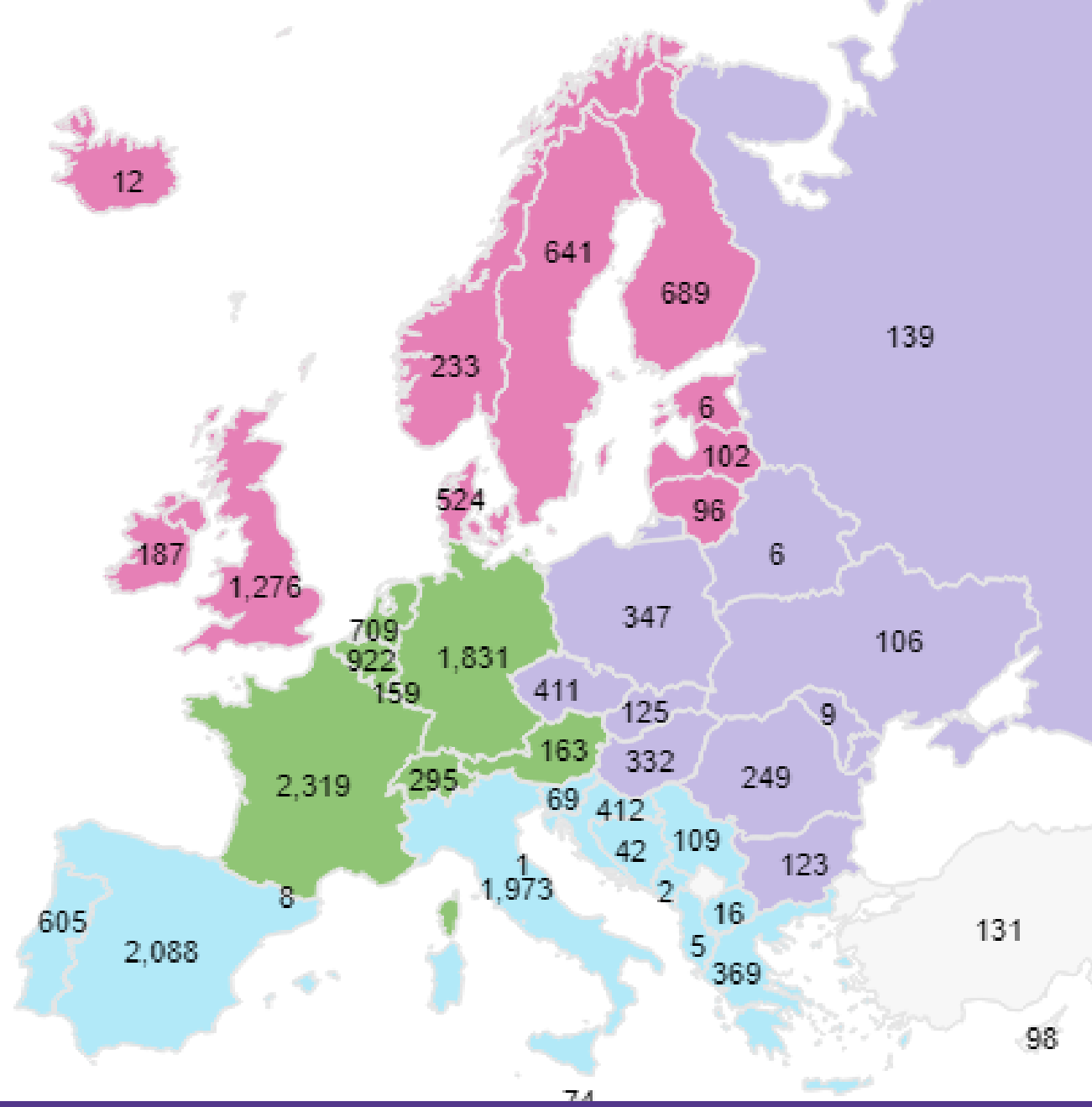
Make your voice heard!

Collective results shared
with participants, patient organisations, decision makers and the wider public

Information is only accessible to the Rare Barometer team, saved on a secured server in Europe

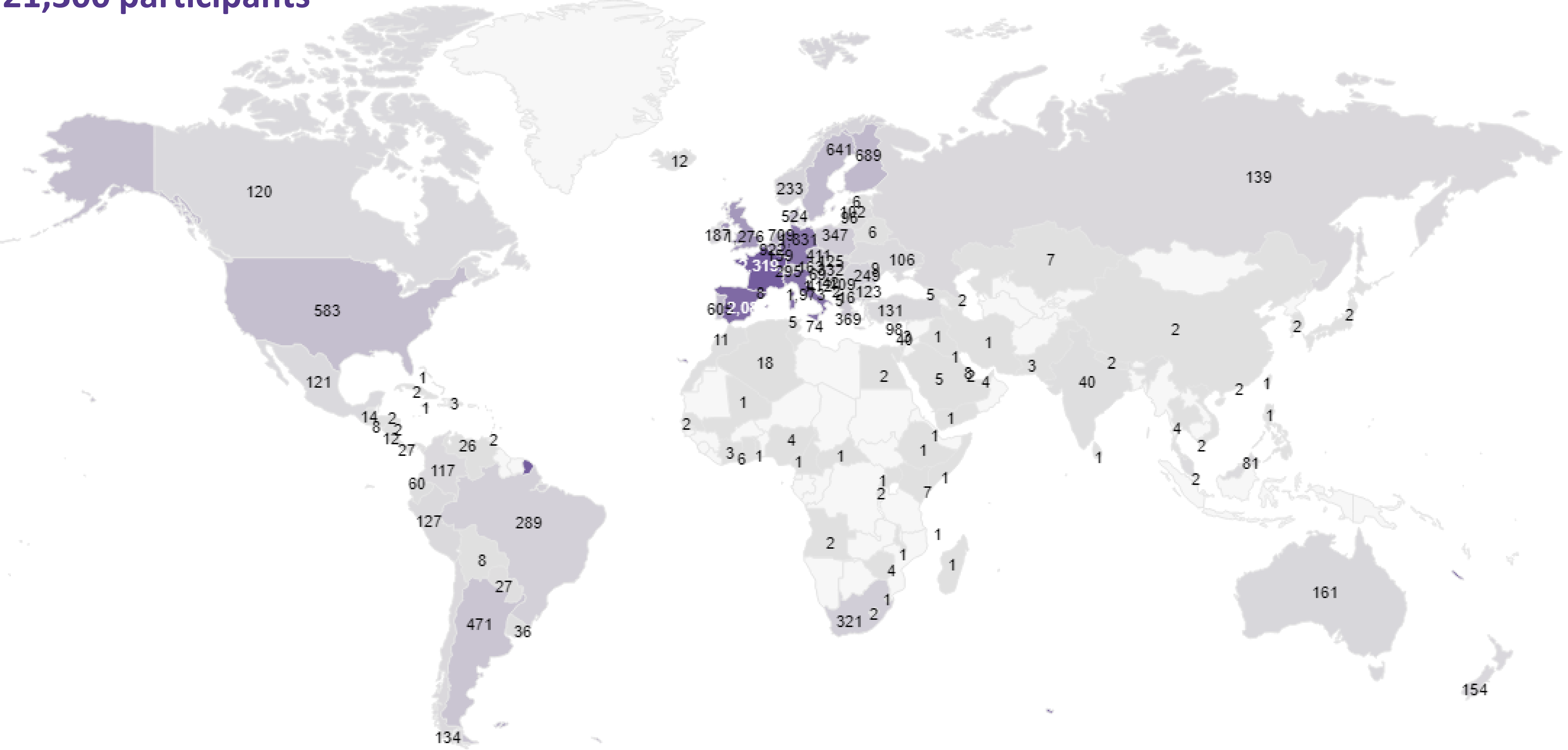
RARE BAROMETER PANEL

Over 21,300 participants



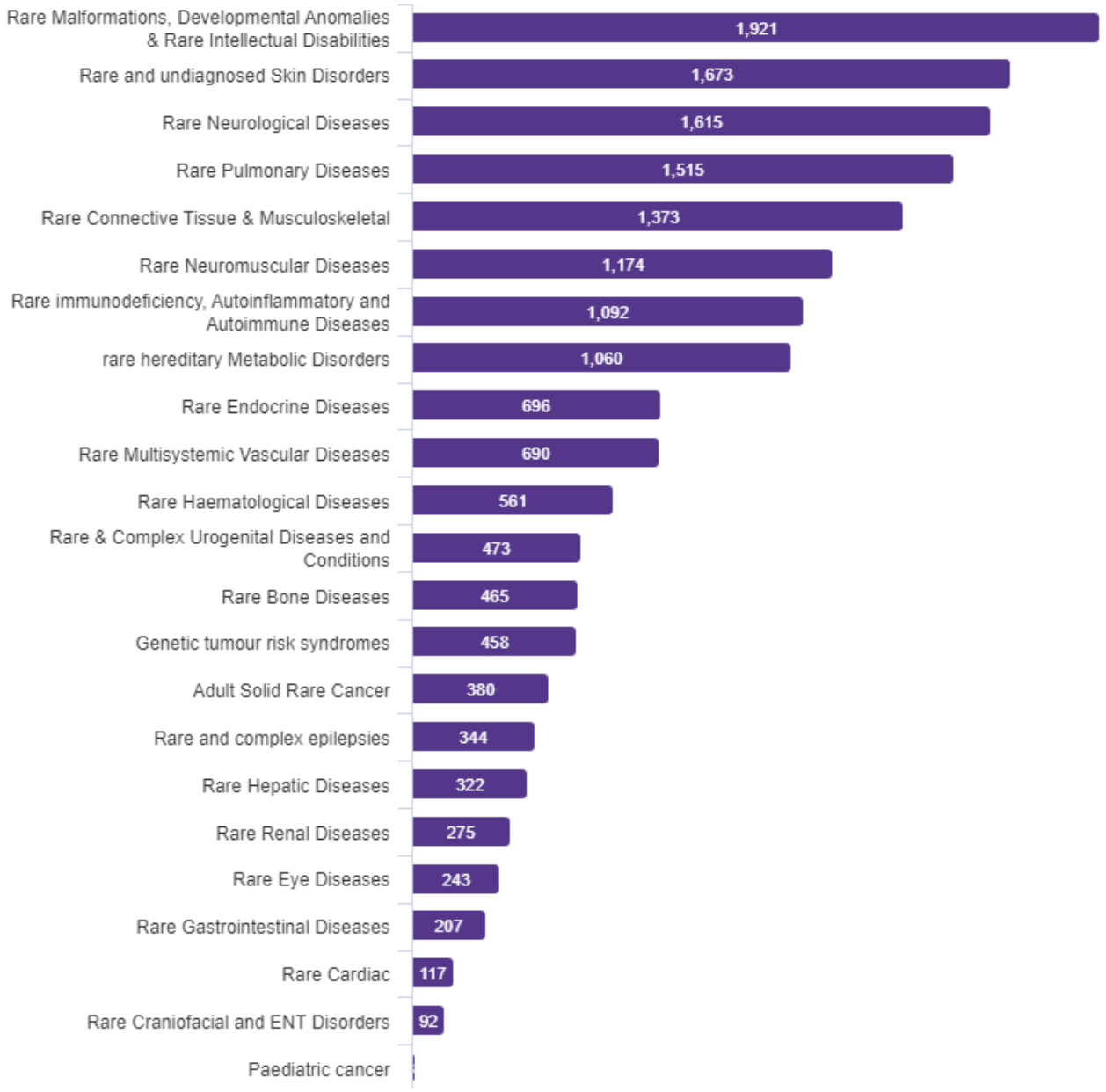
RARE BAROMETER PANEL

Over 21,300 participants



RARE BAROMETER PANEL

Orphanet disease classification (number of participants)



RARE BAROMETER PANEL Individual diseases (number of participants)

Cystic fibrosis	590
Hypermobile Ehlers-Danlos syndrome	505
Hereditary haemorrhagic telangiectasia	464
Neurofibromatosis type 1	276
Sarcoidosis	268
Tuberous sclerosis complex	186
22q11.2 deletion syndrome	183
Systemic sclerosis	174
Myasthenia gravis	166
Duchenne muscular dystrophy	151
Williams syndrome	150
Common variable immunodeficiency	125
Addison disease	114
Perineural cyst	110
Behçet disease	109
Rett syndrome	107
Arnold-Chiari malformation type I	106
Osteogenesis imperfecta	105
Autosomal systemic lupus erythematosus	99
Classical Ehlers-Danlos syndrome	98
Primary sclerosing cholangitis	97
Fragile X syndrome	95
Granulomatosis with polyangiitis	87
Phenylketonuria	85
Marfan syndrome	83
Fabry disease	81

Gaucher disease	80
Idiopathic pulmonary arterial hypertension	77
Chronic inflammatory demyelinating polyneuropathy	73
Proximal spinal muscular atrophy	69
Angelman syndrome	66
Dravet syndrome	65
Wilson disease	63
Interstitial cystitis	62
Prader-Willi syndrome	62
Facial dysmorphism-developmental delay-behavioral abnormalities syndrome due to 10p11.21p12.31 microdeletion	60
Idiopathic intracranial hypertension	60
Autoimmune hepatitis	57
Facioscapulohumeral dystrophy	57
Friedreich ataxia	57
Hereditary spastic paraplegia	57
Hereditary angioedema	54
Diffuse cutaneous systemic sclerosis	52
Familial Mediterranean fever	52
Neurofibromatosis type 2	51
Glycogen storage disease due to acid maltase deficiency	49
Classical-like Ehlers-Danlos syndrome type 1	48
Alpha-1-antitrypsin deficiency	47
Huntington disease	47
Primary ciliary dyskinesia, Kartagener type	47
Scleroderma	46

Pulmonary arterial hypertension	45
Turner syndrome	45
Acute inflammatory demyelinating polyradiculoneuropathy	44
Desmoid tumor	44
Acromegaly	43
Narcolepsy type 1	43
Large congenital melanocytic nevus	41
Non-acquired panhypopituitarism	41
Pseudomyxoma peritonei	41
Pulmonary arterial hypertension associated with congenital heart disease	41
Vascular Ehlers-Danlos syndrome	41
Idiopathic/heritable pulmonary arterial hypertension	40
Noonan syndrome	40
Atypical Rett syndrome	39
Polycythemia vera	38
Acute intermittent porphyria	37
Classic phenylketonuria	37
Cushing disease	37
Eosinophilic granulomatosis with polyangiitis	37
Idiopathic pulmonary fibrosis	37
Mixed connective tissue disease	37
Lupus erythematosus panniculitis	36
MeniÃžre disease	36
Charcot-Marie-Tooth disease type 1A	35
Paroxysmal nocturnal hemoglobinuria	35

TAILORED RESULTS

Collective results shared with EURORDIS members and ERNs if respondents' anonymity is ensured

	European/ international results for one disease or group of diseases	Results for one country on all rare diseases	Specific results (one group of diseases in one country…)	European results for diseases of each ERN	European results
EURORDIS European & international Federations	X				X
EURORDIS National Alliances		X			X
Other EURORDIS members			X		X
European Reference Networks (ERNs)				X	X
Anyone (EURORDIS website)					X

2

SHAPING THE ONLINE QUESTIONNAIRE

OBJECTIVES OF THE SURVEY

- Estimate the level of **participation in social activities** such as education, work or leisure.
- Identify **barriers or facilitators** in doing those social activities.
- Understand **preferences and needs** regarding **living arrangements and personal assistance**.
- Collect **experiences with disability assessment**.
- Identify the main difficulties in accessing **social and disability rights**.

DESIGNING THE QUESTIONNAIRE

Background document

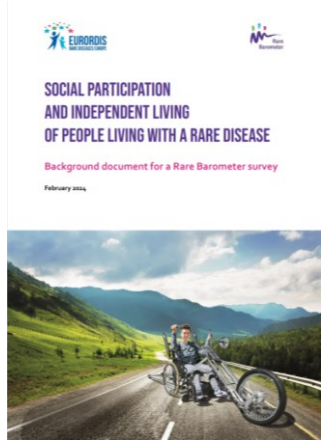
Topic Expert Committee

Writing the questionnaire

Testing the questionnaire

Translating the questionnaire

Reviewing the translations



OBJECTIVES OF THE BACKGROUND DOCUMENT:

- Reviewing the literature on disabilities among people living with rare diseases.
- Identifying determinants of **social participation** at work and among children living with a rare disease and/or disabilities.
- Identifying determinants of some elements of **independent living**, such as housing and housing preferences, access to personal assistance or assistive devices.
- Analyse **disability frameworks**, including **disability measurement** and **disability assessment methods** throughout Europe.
- Identify **existing questions and surveys** that could be used for comparison with other populations.

DESIGNING THE QUESTIONNAIRE

Background document

Topic Expert Committee

Writing the questionnaire

Testing the questionnaire

Translating the questionnaire

Reviewing the translations

Adéla Odrihocká, Patient representative, Rare Diseases Czech Republic

Ariane Weinman, Public Affairs Senior Manager, EURORDIS-Rare Diseases Europe, France

Dorica Dan, Patient representative, Romanian National Alliance, Romanian Prader-Willi Association, Romania

Gavin McDonough, Orphanet Disability Project Manager, France

Haydn Hammersley, European Disability Forum, Social Policy Coordinator,

Jakub Gietka, Patient representative, Aiming for the Future Foundation

Kirsty Hoyle, Patient representative, Metabolic Support UK

Maria Montefusco, National competent authority, patient representative, Sweden

Pauline McCormack, Medical Sociologist, Newcastle University, UK

Petra Bruegmann, Patient representative, European MEN Alliance e.V., Germany

Petra Rantamaki, European Association of Service Providers for Persons with Disabilities, Finland

Sara Rocha, Women's Committee of the European Disability Forum, European Council of Autistic People, and President of the PT association Voz do Autista, Portugal

Stavros Goulidis, Ministry of Labour and Social Affairs, responsible for personal assistance reform, Greece

Valentina Bottarelli, Public Affairs Director & Head of European and International Advocacy, EURORDIS-Rare Diseases Europe, Belgium

DESIGNING THE QUESTIONNAIRE

Background document

Topic Expert Committee

Writing the questionnaire

Testing the questionnaire

Translating the questionnaire

Reviewing the translations

Rare Barometer team

Accessibility: wording adapted to people living with a rare disease and to family members, presentation, colours.

DESIGNING THE QUESTIONNAIRE

Background document

Topic Expert Committee

Writing the questionnaire

Testing the questionnaire

Topic Expert Committee, EURORDIS' Council of national alliances and Council of European Federations

Translating the questionnaire

Reviewing the translations

DESIGNING THE QUESTIONNAIRE

Background document

Topic Expert Committee

Writing the questionnaire

Testing the questionnaire

Translating the questionnaire

Reviewing the translations

Professional translators specialised in health

Survey available in 25 languages:

Bulgarian, Croatian, Czech, Danish, Dutch, English, Finnish, French, German, Greek, Hungarian, Italian, Latvian, Lithuanian, Norwegian, Polish, Portuguese, Romanian, Russian, Slovakian, Slovenian, Spanish, Swedish, **Turkish, Ukrainian**

DESIGNING THE QUESTIONNAIRE

Background document

Topic Expert Committee

Writing the questionnaire

Testing the questionnaire

Translating the questionnaire

Reviewing the translations

THANK YOU to all our reviewers!

Mária Ábele (Hungarian), Jurn Anthonis (Dutch), Petra Brugman (German), Dorica Dan (Romanian), Jessie Dubief (French), Stavros Goulidis (Greek), Malin Grande (Swedish), Gulcin Gumus (Turkish), Lene Jensen (Danish), Stephanie Juran (Swedish), Anja Helm (German), Jelena Malinina (Lithuanian), Martina Bergna (Italian),Judit Baijet (Spanish), Maria Montefusco (Swedish), Adéla Odrihocká (Czech), Oleksandra Oliinyk (Ukrainian), Hüseyin Örün (Turkish), Cindy Penningnieuwland (Dutch), Claudio Pirola (Italian), Veronica Popa (Romanian), Alba Parejo (Spanish), Petra Rantamaki (Finnish), Rebecca Skarberg (Norwegian), Zuzana Smith (Slovakian), Tanja Zdolsek (Slovenian), Hrvatski savez za rijetke bolesti/ Croatian Association for rare disease (Croatian)

3 INSIGHT INTO THE ONLINE SURVEY

THE SURVEY TOPICS

1

DISEASE CHARACTERISTICS & DAILY DIFFICULTIES

2

PARTICIPATION IN SOCIAL ACTIVITIES

3

DISABILITY ASSESSMENT & INDEPENDENT LIVING

DISEASE CHARACTERISTICS & DAILY DIFFICULTIES

- **Status**: person with rare disease/family member; confirmed diagnosis, initial diagnosis, partial diagnosis, unsolved case, other.
- **Diagnosis**: name of the disease(s) (Orphanet list)
- **Diagnosis journey**: time since the diagnosis (initial or confirmed)
- **Types & frequencies of daily difficulties based on the Washington Group Questionnaire:** seeing, hearing, walking or climbing steps, remembering or concentrating, self-care (washing, dressing…), communicating, pain, fatigue
- **Adapted question from the GALI questionnaire**
- **Self-definition of person with disability**: visible/invisible
- **Support**: access, barriers

PARTICIPATION IN SOCIAL ACTIVITIES

- **School / higher education (for students / pupils):**
  - **Type of school**: mainstream school with or without accommodation or adaptations, specialised school, homeschooling, higher education)
  - **Level of participation**: activities with other students, recreational activities, moving around at school, using educational materials and equipment, communicating with other students/pupils and adults at school. Based on the CASP questionnaire.
- **Neighborhood and community participation:** leisure activities with friends, structured events (e.g. team sports, clubs, holiday of religious events, concerts, parades and fairs), moving around the neighborhood and community, communicating with others

PARTICIPATION IN SOCIAL ACTIVITIES

- **Work participation:**
  - **Employed**: type of work contract (full time/part time, fixed-term/permanent); specific support or workplace accommodation / sheltered employment, preferences in this regard and possible improvements;
  - **Unemployed**: preferred type of contract, reasons for unemployment (linked to the symptoms of the rare disease, lack of work accommodation, caregiving responsibilities, fear of losing financial benefits, others).
- **Social support:** Adapted from the EHIS questionnaire (European Health Interview Survey)
- **Voluntary work:** involved or not, why and why not (Adapted from the SILC questionnaire - Statistics on Income and Living Conditions)
- **Discrimination**

DISABILITY ASSESSMENT & INDEPENDENT LIVING

- **Disability assessment:** already went through the process or not, for which purpose(s), type of information required during the assessment, type of professionals involved, results of the assessment, difficulties and dissatisfactions, open question.

- **Living arrangements:** types of benefits accessed, housing arrangements (type, satisfaction, preferences), with whom they spend most of their days with (other family members, people with disability.

- **Personal assistance:**

  - **When access to a personal assistant**: how he/she was chosen, possible difficulties, number of days of PA and number of hours per day, types of tasks covered and needed but not covered

  - **When no access to but need of a personal assistant**: reasons for not having one, which types of tasks they would need assistance with

3

SHARE THE WORD!

SURVEY DISSEMINATION

Survey open until 8 September

Invitations sent to participants in the Rare Barometer panel

Already
500+
respondents!

Global communication

From **July 10th,** use the communication toolkit to encourage participation in your network!
eurordis.org/voices

Follow dissemination

EURORDIS members & ERNs can request a dashboard to follow the survey participation in their community!
rare.barometer@eurordis.org

Reminders

Send reminders to your network!

Check our social media!

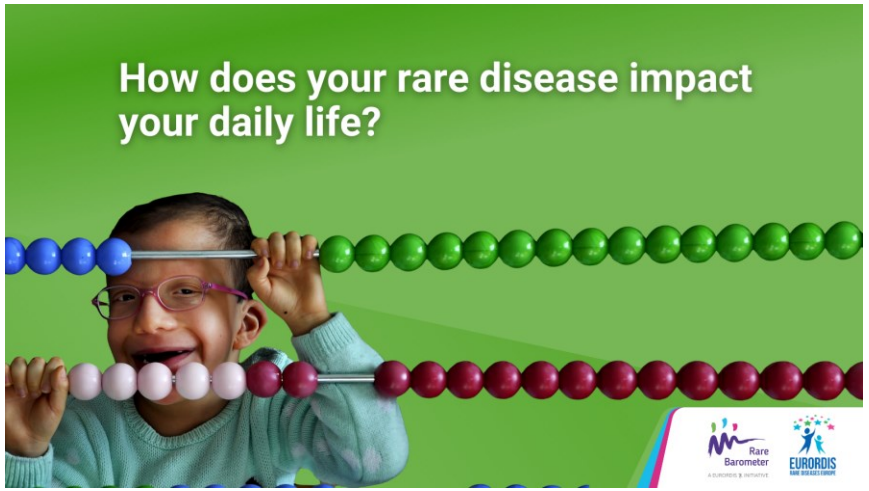
Final reminder

Send a last reminder a week before the survey closes!

HOW TO COMMUNICATE ABOUT THE SURVEY?

Spread the word in your network!

- Send an email to your community – still the most efficient communication action!
- Share the survey on social media
- Host a webinar or Facebook live session
- Link the survey to a local/national/international campaign or awareness day
- Invite the Rare Barometer team to come and speak in your community!

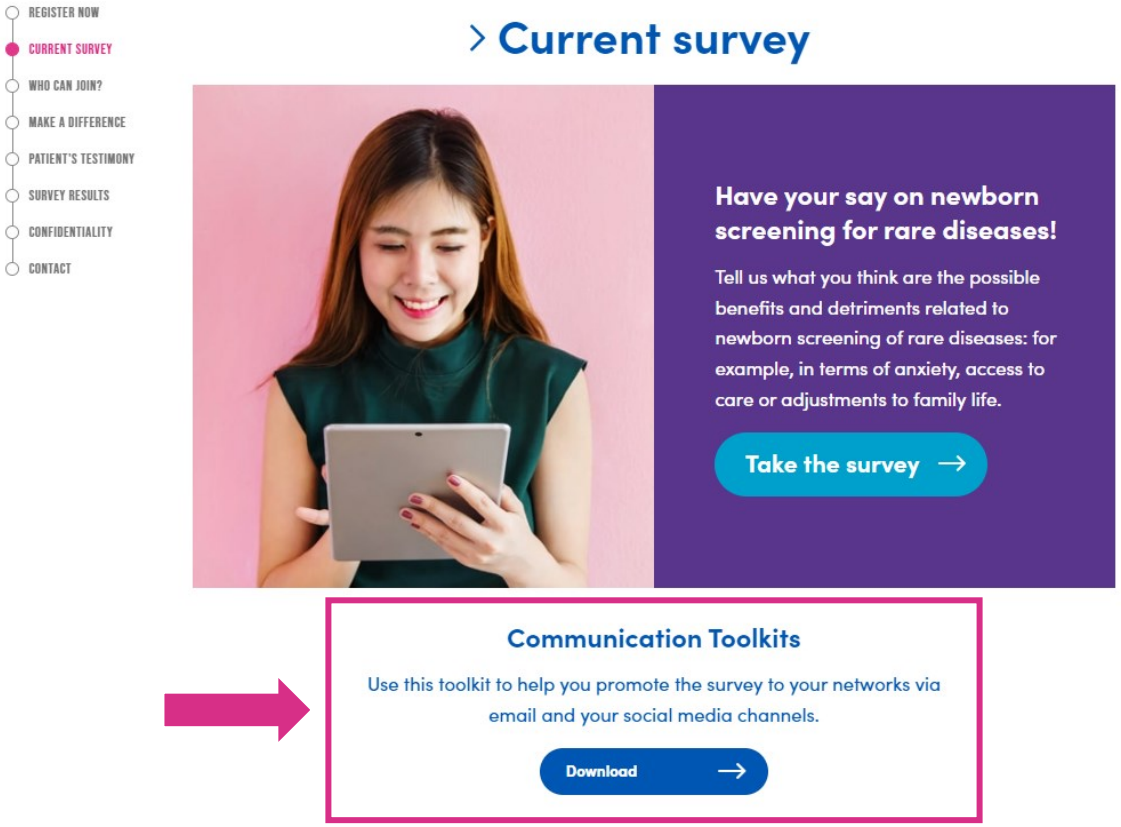


#RareBarometer

tiny.cc/RB_DailyLife

COMMUNICATIONS TOOLKIT

eurordis.org/voices



- Available in 25 languages
- Social media images, suggested social media messages, email template, logos
- Possibility to add your organisations' logo
- Blank versions of social media images available to adapt messages in your native language

COMMUNICATIONS TOOLKIT IN 25 LANGUAGES

Suggested email & social media

Subject: Make your voice heard: take the new Rare Barometer survey on the impact of rare diseases on everyday life!

The new Rare Barometer survey on the impact of rare diseases on everyday life is now live!

By asking questions on your participation in daily activities, such as school, leisure and work, and on what you would need to live life at its fullest, we will be able to advocate for people with a rare disease to access their rights and participate in society on an equal basis with others.

This survey is open to people living with a rare disease and their family members from any country in the world. It is translated in 25 languages.

To make your voice heard and share your experience, we invite you to take 20 minutes to fill out the survey by clicking here, before 8 September: tiny.cc/RB_DailyLife

Overall results will be shared with everyone who responded to the survey and communicated to patient organisations, policy makers and the general public, in order to drive real change for the rare disease community. Rare Barometer complies with the General Data Protection Regulation (GDPR).

You can find more information on the survey here: tiny.cc/RB_DailyLife_Info

If you have any questions while taking part in this survey, you can contact rare.barometer@eurordis.org

Thank you very much in advance for your participation,

Survey now live! Take the new #RareBarometer survey on the impact of rare diseases in everyday life 👉: tiny.cc/RB_DailyLife

📣 Are you between 18 and 30 years old and living with a rare disease? Tell us how your condition impacts your life! 👉tiny.cc/RB_DailyLife
Share your experience to help all people living with a rare disease live life to its fullest! Survey available in 25 languages 🌍💬 #RareBarometer #RareDisease #YoungAdvocates #MakeADifference #EURORDIS

⏰Final call! The #RareBarometer survey on the impact of rare diseases on everyday life closes on 8 September! Share your experience to help all people living with a rare disease live life to its fullest! Survey available here in 25 languages: tiny.cc/RB_DailyLife

Visuals for social media

QR code for printed materials

REACHING OUT TO YOUNGER PARTICIPANTS

Targeted social media posts included in the communication toolkit to encourage participation of **young adults**

You are between 18 and 30 years old and living with a rare disease?
Tell us how your condition impacts your life!
Share your experience to help all people living with a rare disease live life to its fullest!
How is school/university with a rare condition?
Your voice is powerful and can drive positive change!

🌍💬 #RareBarometer #RareDisease #YoungAdvocates #MakeADifference #EURORDIS

COMMUNICATION ACTIONS: GET PREPARED!

Theme - staying clear and relevant:

- **Everyday life / daily life**: avoiding conceptual terms, e.g. 'social participation', 'independent living'
- **Impact** of the rare disease is clear but only refers to the 'medical model'
- Mix with communications messages closer to a '**human rights model**'
- **Not focusing on disability**: avoid using *only* pictures of people living with a rare disease and a visible disability, to encourage everyone to participate

Example of suggested social media messages →

How does your rare disease impact your daily life?
Tell us about it in the new #RareBarometer survey!
What would it take for you to live your everyday life to its fullest?
The #RareBarometer survey on the impact of rare diseases on daily life closes on 8 September!
Tell us how your rare disease impacts your life!

TAILOR YOUR COMMUNICATION TO YOUR NEEDS!

You do **not** have to **mandatorily** use the communications toolkit.

If you want to create communications material tailored to your needs, please make sure that you include:

- Survey link: tiny.cc/RB_DailyLife
- Rare Barometer logo (and the EURORDIS logo)
- #RareBarometer to your social media posts
- 20 minutes to fill and closes on September 8th
- Available in 25 languages, worldwide, to all people living with a rare disease & their close family members
- Survey results shared with participants & decision-makers
- How you intend to use the results of the survey

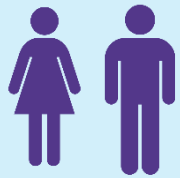
REQUEST TAILORED ONLINE DASHBOARDS

rare.barometer@eurordis.org

Available in all 25 languages

Follow your dissemination work and get a first insight into the results :

- COUNTRY
- DISEASE
- DISEASE GROUP
- DISEASE GROUP PER COUNTRY
- DISEASE GROUP BY REGION



Number of participants
and sociodemographic information

Preliminary results for most questions

Example of online dashboard: https://www.sphinxonline.com/tiny/v/OD0blzqHVD

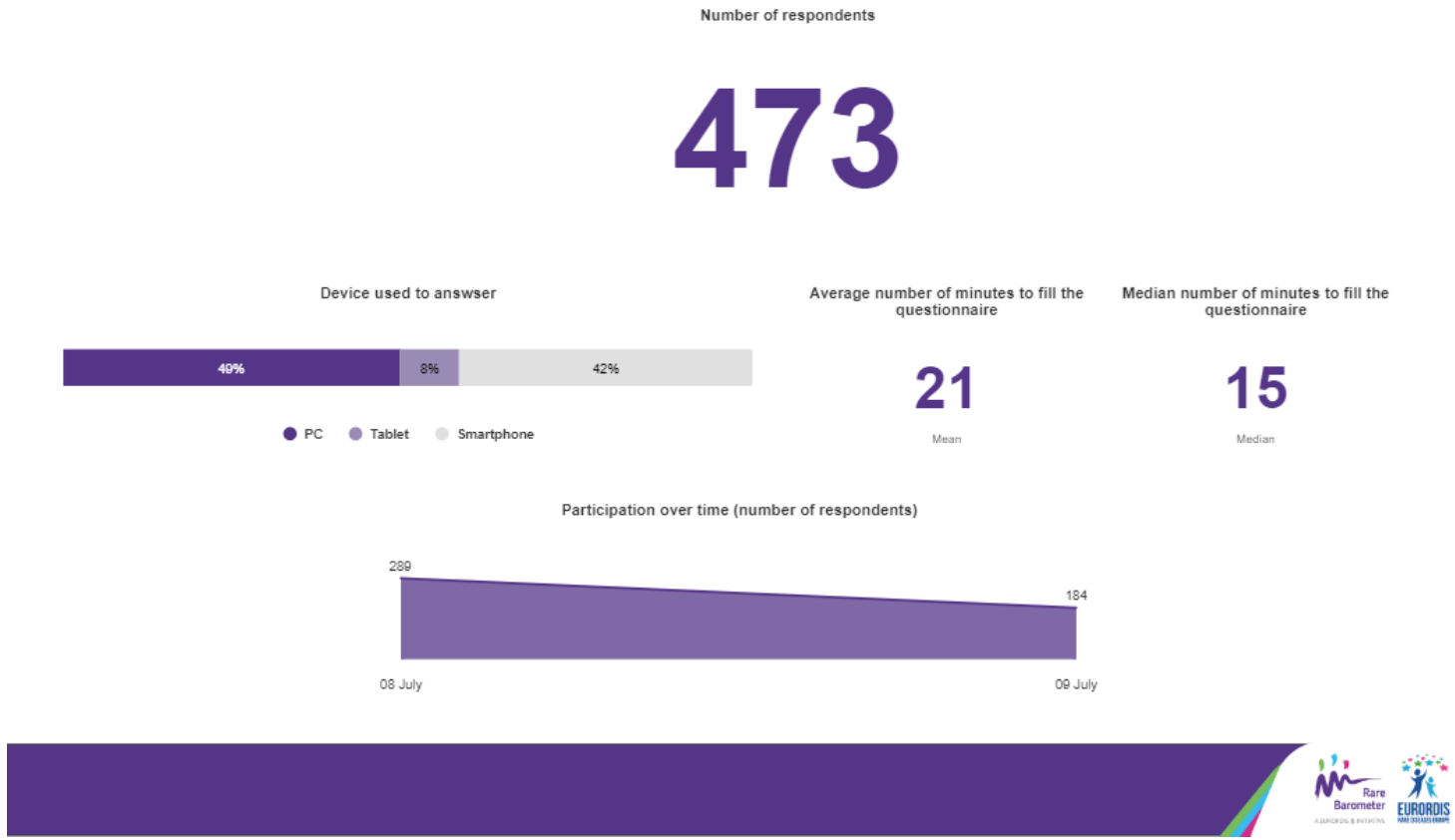
TAILORED DASHBOARD FOR DISSEMINATION

Follow responses in your community!

25 languages

Tailored for EURORDIS members & ERNs

per country, geographical region, disease, group of diseases



Example of online dashboard: https://www.sphinxonline.com/tiny/v/OD0blzqHVD

4 SURVEY RESULTS - WHAT EURORDIS WILL PROVIDE

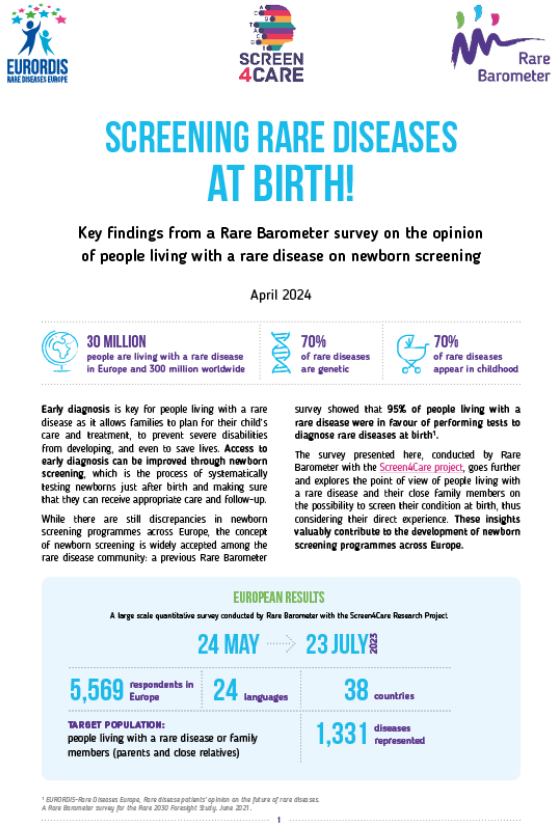
EUROPEAN RESULTS – END 2024

REPORT
available in
English
30-50 pages

Example: eurordis.org/publications/rb-nbs-survey-results/

EUROPEAN RESULTS – END 2024

LONG FACTSHEET
4 pages
Available in English



SCREENING RARE DISEASES AT BIRTH!

Key findings from a Rare Barometer survey on the opinion of people living with a rare disease on newborn screening

April 2024

30 MILLION people are living with a rare disease in Europe and 300 million worldwide

70% of rare diseases are genetic

70% of rare diseases appear in childhood

Early diagnosis is key for people living with a rare disease as it allows families to plan for their child's care and treatment, to prevent severe disabilities from developing, and even to save lives. **Access to early diagnosis can be improved through newborn screening**, which is the process of systematically testing newborns just after birth and making sure that they can receive appropriate care and follow-up.

While there are still discrepancies in newborn screening programmes across Europe, the concept of newborn screening is widely accepted among the rare disease community: a previous Rare Barometer survey showed that **95% of people living with a rare disease were in favour of performing tests to diagnose rare diseases at birth**[1].

The survey presented here, conducted by Rare Barometer with the Screen4Care project, goes further and explores the point of view of people living with a rare disease and their close family members on the possibility to screen their condition at birth, thus considering their direct experience. **These insights valuably contribute to the development of newborn screening programmes across Europe.**

EUROPEAN RESULTS

A large scale quantitative survey conducted by Rare Barometer with the Screen4Care Research Project

24 MAY → 23 JULY 2023

5,569 respondents in Europe | 24 languages | 38 countries

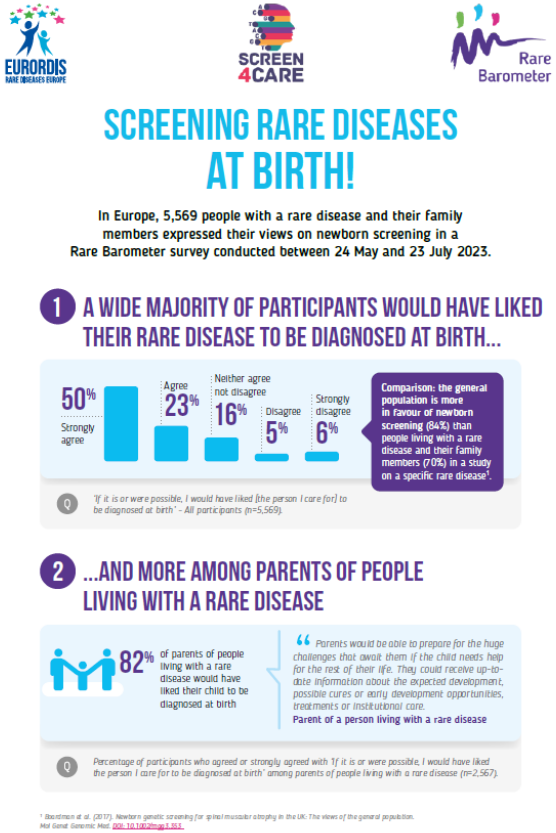
TARGET POPULATION: people living with a rare disease or family members (parents and close relatives)

1,331 diseases represented

[1] EURORDIS-Rare Diseases Europe, Rare disease patients' opinion on the future of rare diseases. A Rare Barometer survey for the Rare 2030 Foresight Study, June 2021.

1

SHORT FACTSHEET
2 pages, available in 15 languages + other languages on demand



SCREENING RARE DISEASES AT BIRTH!

In Europe, 5,569 people with a rare disease and their family members expressed their views on newborn screening in a Rare Barometer survey conducted between 24 May and 23 July 2023.

1 A WIDE MAJORITY OF PARTICIPANTS WOULD HAVE LIKED THEIR RARE DISEASE TO BE DIAGNOSED AT BIRTH...

50% Strongly agree | Agree 23% | Neither agree not disagree 16% | Disagree 5% | Strongly disagree 6%

Comparison: the general population is more in favour of newborn screening (84%) than people living with a rare disease and their family members (70%) in a study on a specific rare disease[1].

Q "If it is or were possible, I would have liked [the person I care for] to be diagnosed at birth" - All participants (n=5,569).

2 ...AND MORE AMONG PARENTS OF PEOPLE LIVING WITH A RARE DISEASE

82% of parents of people living with a rare disease would have liked their child to be diagnosed at birth

"Parents would be able to prepare for the huge challenges that await them if the child needs help for the rest of their life. They could receive up-to-date information about the expected development, possible cures or early development opportunities, treatments or institutional care."
Parent of a person living with a rare disease

Q Percentage of participants who agreed or strongly agreed with "If it is or were possible, I would have liked the person I care for to be diagnosed at birth" among parents of people living with a rare disease (n=2,567).

[1] Beardmore et al. (2017). Newborn genetic screening for spinal muscular atrophy in the UK: The views of the general population. Mol Genet Genomic Med. DOI: 10.1002/mgg3.353

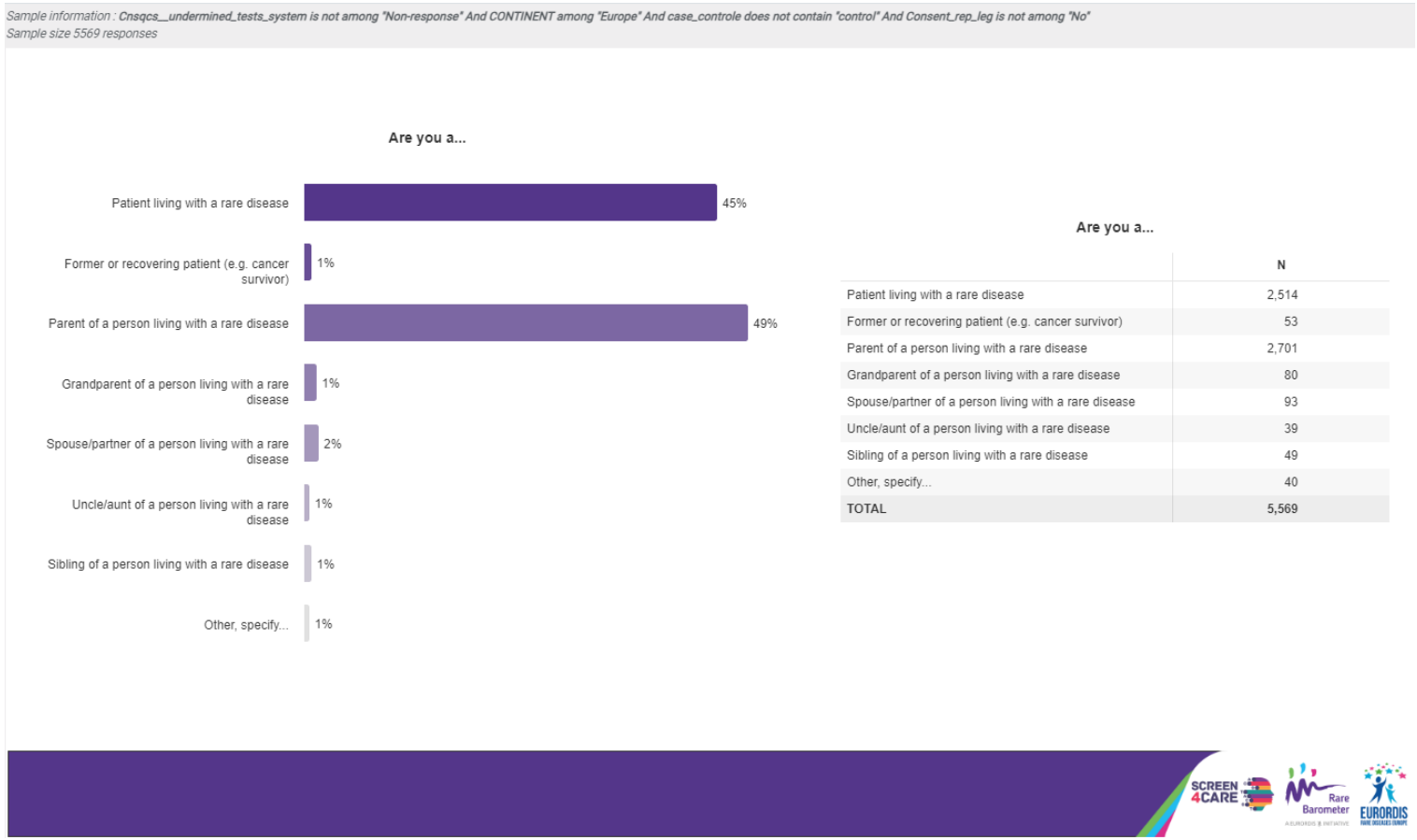
Example: eurordis.org/publications/rb-nbs-survey-results/

EUROPEAN RESULTS – END 2024

DASHBOARD

Each question of the questionnaire
Descriptive statistics
About 30 pages

25 languages



Example: eurordis.org/publications/rb-nbs-survey-results/

TAILORED RESULTS

rare.barometer@eurordis.org

Collective results shared with EURORDIS members and ERNs if respondents' anonymity is ensured

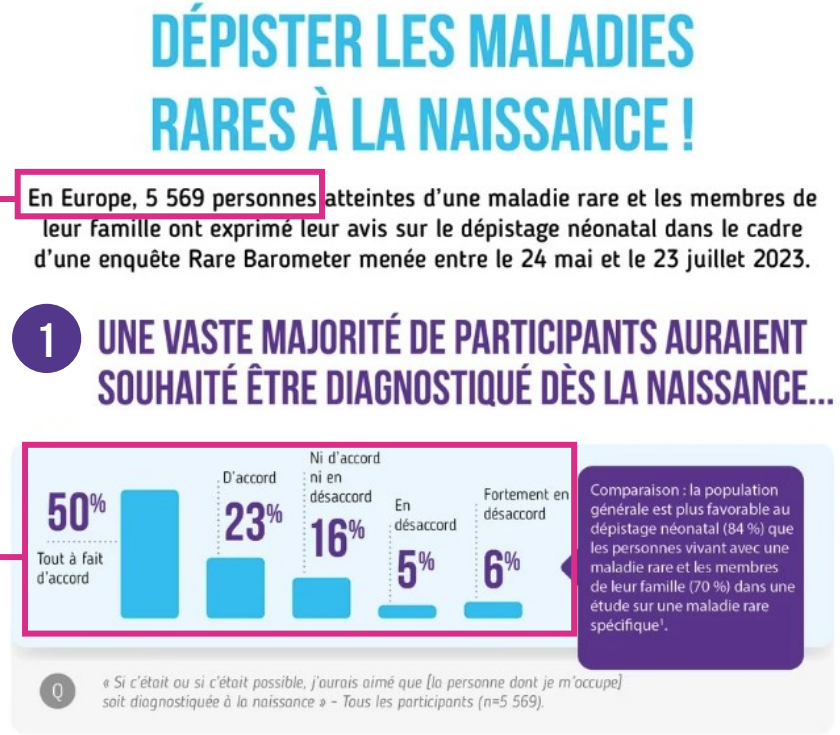
	European/ international results for one disease or group of diseases	Results for one country on all rare diseases	Specific results (one group of diseases in one country…)	European results for diseases of each ERN	European results
EURORDIS European & international Federations	X				X
EURORDIS National Alliances		X			X
Other EURORDIS members			X		X
European Reference Networks (ERNs)				X	X
Anyone (EURORDIS website)					X

TAILORED RESULTS

FACTSHEET
2 pager
7 languages + on demand

Tailored for EURORDIS members & ERNs if more than 30 respondents:
per country, geographical region, disease, group of diseases

Specific key results



DÉPISTER LES MALADIES RARES À LA NAISSANCE !

En Europe, 5 569 personnes atteintes d'une maladie rare et les membres de leur famille ont exprimé leur avis sur le dépistage néonatal dans le cadre d'une enquête Rare Barometer menée entre le 24 mai et le 23 juillet 2023.

1 UNE VASTE MAJORITÉ DE PARTICIPANTS AURAIENT SOUHAITÉ ÊTRE DIAGNOSTIQUÉ DÈS LA NAISSANCE...

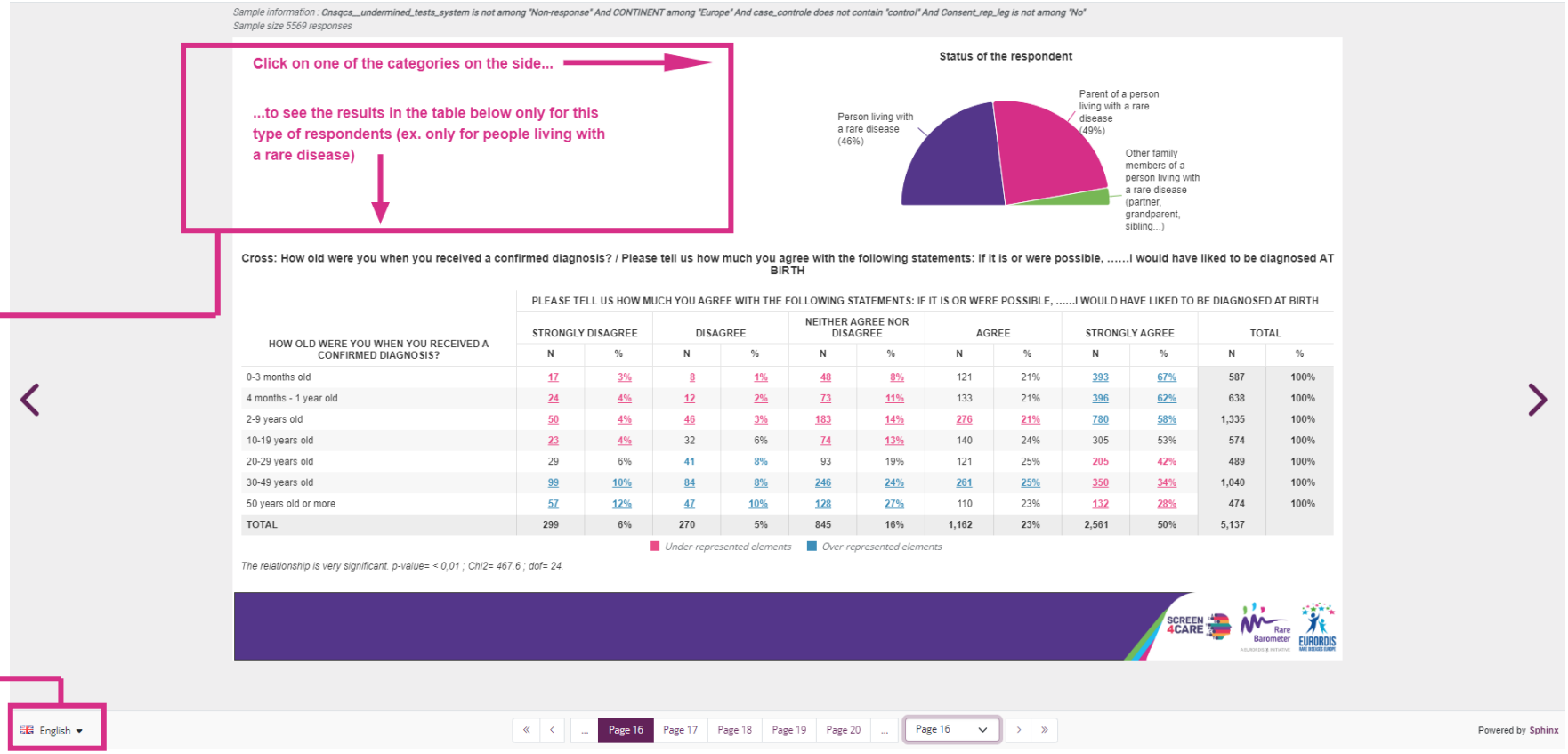
TAILORED RESULTS

ONLINE DASHBOARD

Each question of the questionnaire
Descriptive statistics

Possibility to filter crossings for:
- people living with a rare disease
- parents
- other family members

25 languages



PLEASE NOTE:

- People participating in our surveys **always receive the European results**!
  - Overall results will be shared with everyone who responded to the survey and will also be communicated to patient organisations, decision-makers and the general public.
- You do **not** need to register to the Rare Barometer in order to participate!
  - However, by registering to Rare Barometer, you will be the first to hear about new surveys, and the first to receive the results!
- We encourage you to **complete the survey to the end!**
  - We know it's a long survey, but it's very important to complete it to the end for us to take your answers into account!

5 USE OF RESULTS

European advocacy:
Ongoing priorities

All people living with rare diseases and disability must access:

- ⊕ Adequate disability assessment and recognition
- ⊕ Adequate social protection and independent living support

European advocacy:
Upcoming opportunities

EU level:

Mid-term review of the EU Disability Strategy 2021-2030, to propose flagship initiatives.

EC guidance on independent living and inclusion in the community.

EU framework for Social Services of Excellence for persons with disabilities.

EU Disability Platform discussions - aiming to present results in plenary.

National level:

Guidance for Member States to improve disability assessment processes.

Ongoing reforms of independent living and community-based services.

Raising awareness:

Of the diverse disabilities and barriers experienced by the rare disease community.

Of the social inequalities faced by people with rare diseases.

EXAMPLE OF HOW SURVEY RESULTS HAVE BEEN USED

Communicate about the results on your website
https://www.radiorg.be/fr/le-parcours-diagnostique-des-personnes-atteintes-de-maladies-rares-resultats-de-lenquete-rare-barometer/

Analyse the targeted results yourself
https://covid-19.geneticalliance.org.uk/wp-content/uploads/2020/07/Covid-19-Rare-Reality.pdf

Issue an academic publication
https://journals.sagepub.com/doi/full/10.1177/2397198321999927

Compare with your own past survey findings
https://www.sciencedirect.com/science/article/pii/S2666535221000756

QUESTIONS AND ANSWERS

THANK YOU FOR PARTICIPATING!

Share the link to our survey on your social media

TODAY!

tiny.cc/RB_DailyLife

#RareBarometer

RESOURCES:

- PDF version of the questionnaire: tiny.cc/DailyLife_questionnaire
- Link to the survey: tiny.cc/RB_DailyLife
- Link to the communications toolkit: eurordis.org/voices
- Link to the page presenting the survey: tiny.cc/RB_DailyLife_Info
- Example of academic publication: https://www.nature.com/articles/s41431-024-01604-z
- Example of dashboard: https://download2.eurordis.org/rarebarometer/Dashboards/DiagnosisOdyssey_results_Europe_EN.pdf
- Example of factsheet: https://www.eurordis.org/wp-content/uploads/2024/04/RB-FactSheet-NBS_short4.pdf
- Example of a report: https://www.eurordis.org/wp-content/uploads/2024/05/RB_NBS_report_vff.pdf
-

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rare.barometer@eurordis.org