



Rare disease patients' opinion on the future of rare diseases (RARE 2030 Foresight)

November 2020



RARE 2030 SURVEY QUESTIONNAIRE

Goal

Gather rare disease patients' and carers' opinion on policies that may impact their lives.

Objectives

- Take into account patients' and carers' opinion into recommendations for the policies for rare diseases within the next 10 years
- Feed EURORDIS advocacy work with case studies/testimonials
- Prepare EURORDIS long-term advocacy messages

Analysis

Rare Barometer Voices framework will enable to filter and cross the results according to the following criteria:

- Comparison between countries
- Comparison with the general public
- Data by disease/group of diseases
- Patient/carers
- Age
- Gender
- Disease grouping (ePAG grouping)

Translation and reach

Rare disease patients can answer from any country of the world.

The survey is translated in the following languages:

Bulgarian	Finnish	Latvian	Russian
Croatian	French	Lithuanian	Slovak
Czech	German	Norwegian	Slovenian
Danish	Greek	Polish	Spanish
Dutch	Hungarian	Portuguese	Swedish
English	Italian	Romanian	

TEXT IN ORANGE WON'T BE DISPLAYED IN THE QUESTIONNAIRE BUT FEATURED IN THE SOFTWARE SYSTEM

TEXT IN BLUE COMES FROM OTHER SURVEYS AND COULD BE USED FOR COMPARISON (IN THAT CASE IT SHOULD NOT BE CHANGED)

TEXT IN PURPLE IS RARE BAROMETER FRAMEWORK AND SHOULD NOT BE CHANGED FOR COMPARISON PURPOSES

As a reminder, we agreed to:

- Whenever possible, propose statements for respondents to give their opinion on a scale similar to “strongly disagree / partially disagree / partially agree / strongly agree” or others (preferably with an even number of modalities).
- Take into account differences between times of crisis and others.
- Tackle the place of patient organisations in the future of rare diseases.

In this proposition, **39 questions in total** (= max number if all sociodemographic questions, patient representative and EU citizen):

- 4 preliminary questions for further filters (status, country & patient journey)
- 5 questions on remote health care
- 3 questions on cross-border health care / + 1 question only for EU citizens (=90-95% respondents)
- 3 questions on research / + 6 questions only for patient representatives (=5-10% respondents)
- 2 questions on prenatal & new born screening
- 1 open question
- 8-14 sociodemographic questions - only to respondents who are not registered in RBV – *these questions can't be removed*

Patients (EU): 27-33 questions

Patient representatives (EU): 33-39 questions

Non-EU citizens: minus 1 question

RBV participants: minus 8-14 questions

Presentation page of the survey:

What should the future of rare diseases look like?

Give us your opinion on the possible ways to shape the future of rare diseases regarding **your access to health care, medical and social research as well as early diagnosis for children.**

This survey will take around **15 minutes** to complete and will help EURORDIS-Rare Diseases Europe, a non-profit and non-governmental alliance of 900+ patient organisations, to improve the lives of people with rare diseases. We will share the overall results of the survey with you and communicate them (without communicating your individual responses) to decision-makers. Your contact details and any written records of your responses during the study will be kept in secure storage which only the research team can access.

This survey is part of the **Rare 2030 Foresight Study**. For more information please visit rare2030.eu
If you have any questions while taking part in this survey, you can contact rare.barometer@eurordis.org

Preliminary questions

Q1. How would you like to answer this questionnaire? In your capacity as a... (several answers possible)

- patient
 - patient representative
 - Parent of a child living with a rare disease
 - Grandparent of a person living with a rare disease
 - Spouse of a person living with a rare disease
 - Uncle/aunt of a person living with a rare disease
 - Sibling of a child or adult living with a rare disease
 - Other, please specify
- } carer category

Q2. In which country do you live? [select one country in the list of world countries]

Q3. Are you or the person you care for: <i>Patient journey</i>	Yes	No	Not applicable
Diagnosed	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Currently receiving care/treatment to cure or to significantly stabilise the progression of the rare disease	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Currently receiving care/treatment to manage the symptoms of the rare disease	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Receiving psychological or emotional support to help deal with the rare disease	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
In contact with a group of patients or a patient organisation	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

ONLY TO THOSE WHO ANSWERED "NO" TO "RECEIVING TREATMENT OR CARE TO CURE OR STABILISE THE DISEASE", OR TO "MANAGE SYMPTOMS" AT Q3

Q4. You or the person you care for are not receiving treatment for the rare disease because: *Patient journey 2*

- The treatment was stopped
- No treatment exists for the disease
- Existing treatments are not accessible in your country
- Existing treatments are too expensive
- Other, specify

REMOTE HEALTH CARE

Remote healthcare services, such as virtual consultations and electronic prescriptions, have been developing in daily life and are playing a crucial role during the COVID-19 pandemics.

The next few questions will be about how you are willing to use these remote healthcare services within the next 10 years.

Q5. Within the next 10 years, would you be willing to attend remote consultations (via phone, video, email, text, app, etc.):	YES all the time	YES from time to time	YES but only if there is no other option (like in times of crisis)	NO	I don't know
With a general practitioner	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
With a specialist	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
With a doctor specialised in your rare disease who works in another country.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
For consultations where you can discuss your disease with several healthcare professionals (for instance with specialists and your general practitioner)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
If you already know or have already met face to face with the professional you would be having a consultation with	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
If you do not already know or have not already met face to face with the professional you would be having a consultation with	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Q6. Do you think that remote consultations are appropriate for...	Not at all appropriate	Not very appropriate	Fairly appropriate	Very appropriate	No opinion
...giving a diagnosis	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...physiotherapy sessions	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...blood tests or other types of clinical tests or analysis that can be done at home	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...psychological or emotional support like sessions with a psychologist or support group	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Q7. What would be the 3 main obstacles for you to use remote consultations within the next 10 years?

- Technical obstacles such as difficulty accessing the internet or using your phone
- Not having met the care team prior to the remote consultation
- The care needed is not suitable for remote consultations
- Concerns about the safety of the care
- Difficulties in being reimbursed for remote consultations
- Language difficulties, when remote consultations are with a healthcare professional who does not work in your country
- Other, please specify...

Q8. Within the next 10 years, would you be willing to use the following services:	YES all the time	YES but only if there is no other option (like in times of crisis)	NO	I don't know
Prescriptions sent by email (electronic message)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Prescriptions sent by mail (posted letter)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Medicines sent by mail (package sent by your hospital, your pharmacy, a pharmaceutical company...)

Q9. Within the next 10 years, what would be the 2 main reasons for you to use...	Saving money	Saving time	Safety	Accessing higher quality care	Having no other option
...remote consultations	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...remote prescriptions (sent by email or by mail)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

CROSS-BORDER HEALTH CARE

Accessing expertise, treatment or health care in another country can sometimes be critical for rare disease patients. The next few questions will be about your willingness to travel to another country to access health care for your rare disease.

Text in blue is coming from the 2014 Special Eurobarometer 425 on *Patients' Rights in Cross-Border Healthcare in the European Union* – percentages between brackets correspond to answers of the general EU population (Eurobarometer results)

Q10. Different wording for EU and for non-EU

WORDING FOR EU: Within the next 10 years and OUTSIDE TIMES OF CRISES, would you be willing to travel to another country in the European Union to receive medical treatment for your rare disease?

WORDING FOR non-EU: Within the next 10 years and OUTSIDE TIMES OF CRISES, would you be willing to travel to another country to receive medical treatment for your rare disease?

- Yes (33%)
- No (46%)
- It depends on the medical treatment or on the country (16%)
- I don't know (5%)

IF "YES" OR "IT DEPENDS ON TREATMENT OR ON THE COUNTRY" TO Q10

Q11. Different wording for EU and non-EU

WORDING FOR EU: For which of the following reasons would you be willing to travel to another country in the European Union to receive medical treatment for your rare disease? Several answers possible

WORDING FOR non-EU: For which of the following reasons would you be willing to travel to another country to receive medical treatment for your rare disease? Several answers possible

- To receive treatment that is not available in your country (71%)
- To receive better quality treatment (53%)
- To receive treatment from a renowned specialist (38%)
- To receive treatment more quickly (34%)
- To receive cheaper treatment (23%)
- To receive treatment from a provider that is closer to home (6%)
- I don't know (2%)
- Other, please specify (2%)

IF "NO" OR "DEPENDS ON TREATMENT OR ON THE COUNTRY" TO Q10

Q12. Different wording for EU and for non-EU

WORDING FOR EU: For which of the following reasons would you not be willing to go to another country in the European Union to receive medical treatment for your rare disease? Several answers possible

WORDING FOR non-EU: For which of the following reasons would you not be willing to go to another country to receive medical treatment for your rare disease? Several answers possible

- You are satisfied with the medical treatments you receive in your country (55%)

- It is more convenient to be treated near your home (49%)
- You would have issues understanding the language (27%)
- You are not aware of your rights in case things go wrong (23%)
- You do not have enough information about the availability and quality of medical treatments abroad (21%)
- You cannot afford to receive medical treatment abroad (20%)
- You have no information on patient safety and quality of care abroad (20%)
- You are not sure that you would be reimbursed (16%)
- I prefer not to travel
- My doctor advised me not to
- I don't know (4%)
- Other, please specify (4%)

ONLY TO RESPONDENTS LIVING IN EU27

Q13. Here are some statements related to health care received in another country of the European Union. For each of the following, could you please say whether you think it is true or false?	True	False	I don't know
You have the right to receive planned medical treatment in another country in the European Union and to be reimbursed for that treatment by national health authority or healthcare insurer. <i>True</i>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
You cannot get a prescription from your doctor to use in another country in the European Union. <i>False</i>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
You have the right to receive a copy of your medical record from your doctor when you seek to receive healthcare in another country in the European Union. <i>True</i>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
Your doctor may be able to get help in treating you locally by contacting a network of doctors specialised in specific rare disease(s) called a European Reference Network. <i>True</i>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

MEDICAL AND SOCIAL RESEARCH

Medical and social research can greatly improve the quality of life of people living with a rare disease. The next few questions will be about what areas should research focus on to help you live with your rare disease in the future.

Q14. Do you think it is possible and realistic that within 10 years, you or the person you care for...	This is already the case today	YES and this is not yet the case	NO	I don't know
...could be cured from the rare disease: total remission, no symptoms.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...could have their rare disease stabilised: the rare disease would still be present but symptoms would not be progressing.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...could manage the symptoms of the rare disease even if they are still progressing.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...could be supported to manage the psychological or emotional aspects of the rare disease.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...would not be limited by the rare disease to handle routine needs, such as everyday household chores, doing necessary business, shopping or getting around for other purposes. <i>Based on CDC's HRQOL questionnaire¹</i>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

¹ https://www.cdc.gov/hrqol/hrqol14_measure.htm

...could take part in education on equal footing with others (adapted transport, accessibility, adapted schooling measures if needed).	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...could access adapted and accessible employment as well as flexible work arrangements (adjustment of working hours, working remotely).	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...would not be discriminated against due to their rare disease or due to their disabilities, in the various aspects of their daily life.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Q15. Different wording for patients and for patient representatives; randomised order for answers

WORDING FOR PATIENTS: Within the next 10 years, the top 3 priorities to improve care for your rare disease would be access to:

3 answers maximum

WORDING FOR PATIENT REPRESENTATIVES: Within the next 10 years, the 3 top priorities to improve care for PATIENTS LIVING WITH THE RARE DISEASE(S) YOU REPRESENT would be access to:

3 answers maximum

- Better diagnosis of the disease
- Better prevention of the disease
- Better social recognition of the disease
- Consultations with healthcare professionals specialised in the disease
- Better coordination between all healthcare professionals involved in the care of the disease
- Health care that would help ease the symptoms of the disease
- Better coordination between healthcare professionals and social care professionals (social care, education, etc.)
- Treatments and/or therapies that do not yet exist
- Existing treatments that are not yet available in your country
- Existing treatments that are still too expensive for you
- Psychological support
- Other, please specify

ONLY TO THOSE WHO ANSWERED "SYMPTOMATIC TREATMENTS" AT Q15

Q16. Different wording for patients and for patient representatives

WORDING FOR PATIENTS: Within the next 10 years, in order to help ease the symptoms of your rare disease, you would need access to:

WORDING FOR PATIENT REPRESENTATIVES: Within the next 10 years, in order to help ease the symptoms of the disease(s), PATIENTS LIVING WITH THE RARE DISEASE(S) YOU REPRESENT would need access to:

- Medicines
- Physiotherapy
- Diet and physical exercises adapted to the disease(s)
- Meditation and/or other related techniques (e.g. breathing techniques, mindfulness meditation).
- Other, please specify

ONLY TO PATIENT REPRESENTATIVES

Wording of modalities of Q12 and Q13 are based on EJP's "Short guide on patient partnerships in RD research projects".

Q17. As a patient representative, do you think that patient organisations should contribute in research for rare diseases by...	Strongly disagree	Fairly disagree	Fairly agree	Strongly agree	No opinion
...helping researchers recruit participants for clinical trials or other research projects [participation]	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...disseminating information and knowledge about the research project and its results [engagement]	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

...reviewing research proposals to ensure feasibility and relevance of the study from a patient's perspective [engagement]	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...being actively involved in research projects as an official partner or co-investigator [involvement]	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...leading their own research projects	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...raising funds for research on their disease	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Q18. In order to be fully involved in research for the rare disease(s) you represent, what type of support would your organisation need most? (several answers possible)

- Financial support
- Human resources
- Knowledge or abilities
- Nothing
- I don't know
- Other, please specify...

Knowing that financial resources for research on rare diseases are limited, research funding agencies and organisations might set priorities based on four criteria: *available knowledge on the disease, available treatments, severity of the disease and number of people living with the disease.*

The next few questions will be about priorities for RESEARCH ACROSS ALL RARE DISEASES regarding each of those criteria.

Q19. AVAILABLE KNOWLEDGE ON THE DISEASE Within the next 10 years, do you think that available resources for RESEARCH ACROSS ALL RARE DISEASES should focus on...	Strongly disagree	Fairly disagree	Fairly agree	Strongly agree	No opinion
...rare diseases for which there is no or scarce knowledge of the scientific mechanisms of the disease.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...rare diseases for which knowledge of the scientific mechanisms of the disease is more advanced.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...none of the above: research priorities should not be set according to the criteria of the knowledge on the disease.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Q20. AVAILABLE TREATMENTS Within the next 10 years, do you think that available resources for RESEARCH ACROSS ALL RARE DISEASES should focus on...	Strongly disagree	Fairly disagree	Fairly agree	Strongly agree	No opinion
...rare diseases for which no treatment is available, be it to cure the disease or to manage its symptoms.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...rare diseases for which there are already treatments to manage the symptoms of the disease but no curative treatment.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...rare diseases for which there are already treatments to stop the progression of the disease but no curative treatment.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...none of the above: research priorities should not be set according to the criteria of available treatments.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Q21. SEVERITY OF THE DISEASE Within the next 10 years, do you think that available resources for RESEARCH ACROSS ALL RARE DISEASES should focus on...	Strongly disagree	Fairly disagree	Fairly agree	Strongly agree	No opinion
	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

...rare diseases for which a treatment (symptomatic or curative) is available but is very expensive, either for the patient (and family) or for society.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...rare diseases that have very high associated costs for the patient (and family) or for society (e.g. respite care, adapted living)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...none of the above: research priorities should not be set according to the criteria of the severity of the disease.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Q22. NUMBER OF PEOPLE LIVING WITH THE DISEASE Within the next 10 years, do you think that available resources for RESEARCH ACROSS ALL RARE DISEASES should focus on...	Strongly disagree	Fairly disagree	Fairly agree	Strongly agree	No opinion
...rare diseases that affect very few people and for which raising funds and public awareness is harder.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...rare diseases that are more common and where research could benefit more people.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...none of the above: research priorities should not be set according to the criteria of the number of people living with the disease.	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

EARLY DIAGNOSIS FOR CHILDREN

New technologies are being developed at a rapid pace and could be used to diagnose some rare diseases in children at a very early stage, improving their treatment options and quality of life.

The next few questions will be about your opinion on the use of those technologies in the future. Your answers will help EURORDIS-Rare Diseases Europe voice the opinion of the rare disease community on this delicate matter.

Q23. In your opinion, in order to diagnose rare diseases at an early stage, should tests for rare diseases be performed...	YES with the informed consent of the parents	YES as part of a compulsory health programme	NO	I don't have an opinion
...around the time of child's conception (e.g. during in vitro fertilisation, genetic counselling)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...during pregnancy (e.g. medical imaging, tests on amniotic fluid...)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>
...at child's birth (e.g. blood tests, genetic screening...)	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>	<input type="checkbox"/>

Many countries have developed compulsory health programmes to test newborns and diagnose rare diseases at an early stage. However, there are still discussions about what types of diseases should be tested as part of those programmes.

Q24. In your opinion, for which conditions should newborns be tested at birth as part of a compulsory health programme? (several answers possible)

- Conditions that COULD BE prevented and/or treated
- Conditions that CANNOT YET BE prevented and/or treated
- Conditions for which appropriate disease management (diet, education...) can improve health and quality of life
- Conditions that may affect other family members (inherited conditions)
- Conditions that may be developed later in life
- None of the above
- I don't have an opinion
- Other, please specify

HAVE YOUR SAY

Q25. Please describe here, as precisely as possible, what changes you want to see for your rare disease within the next 10 years: *Open question*

SOCIODEMOGRAPHIC INFORMATION

This information will allow us to analyse the data in more depth

IF RESPONDENTS ARE NOT REGISTERED IN THE RARE BAROMETER VOICES DATABASE

Q26. How old are you?

- Under 15
- 15-17
- 18-24
- 25-34
- 35-49
- 50-64
- 65 or older

If you are younger than 18 years old, your parent's consent is needed for your registration. Please ask your parents to respond to the following questions.

Q27. Do you want to receive the results of this survey by email?

- Yes
- No

Q28. Would you like to be contacted by email to participate in our future Rare Barometer surveys on your experience of living with a rare disease?

We carry out a maximum of 3 surveys per year, you will be able to unsubscribe at any time from this contact list

- Yes
- No

IF YES TO Q19 or Q20

Q29. What is your email address?

Q30. Please confirm your email address:

TO ALL RESPONDENTS WHO ARE NOT REGISTERED YET

Q31. Are you:

- Female
- Male

Q32. Please select in the following list the center or hospital in which you or the person you look after is treated (*Several answers possible*). If you cannot find the relevant hospital, please skip this question. *[select a hospital in the list of hospitals attached to an ERN, by country]*

IF RESPONDENTS ARE DIAGNOSED

Q33. How many rare diseases are you living with?

- 1
- 2
- 3
- 4

- 5 or more

EURORDIS represents all of the over 6000 rare diseases as does Rare Barometer Voices. In order to create our questionnaire and registration, we have used the most current and exhaustive list of rare diseases (orpha.net).

If you have difficulties finding your rare disease, it might be a question of spelling or punctuation. Please check on the Orphanet website by typing in the name of your disease and let yourself be guided by the drop-down menu. The search engine will then take you to the name of the disease used by Orphanet; please use the spelling of your disease **as it appears on Orphanet** when selecting your disease.

If you still have problems, please enter the name of your rare disease in "Cannot find the disease" and we will make the connection.

Q34. Which rare disease are you living with?

Please start typing the disease below, select it, and then click outside of the list to proceed to the next page. If you cannot find the rare disease in this list below please type it yourself in the "Cannot find the disease" box.

IF 2 RARE DISEASES OR MORE (Q30)

Q35. Please indicate a second rare or complex disease you are living with:

Please start typing the disease below, select it, and then click outside of the list to proceed to the next page. If you cannot find the rare disease in this list below please type it yourself in the "Cannot find the disease" box.

Q36. Please indicate a third rare or complex disease you are living with:

Please start typing the disease below, select it, and then click outside of the list to proceed to the next page. If you cannot find the rare disease in this list below please type it yourself in the "Cannot find the disease" box.

Q37. Please indicate a fourth rare or complex disease you are living with:

Please start typing the disease below, select it, and then click outside of the list to proceed to the next page. If you cannot find the rare disease in this list below please type it yourself in the "Cannot find the disease" box.

Q38. If you are living with other rare or complex diseases, please list them below:

TO ALL RESPONDENTS WHO DID NOT YET REGISTER TO RBV

Q39. How did you hear about this survey?

- On Facebook
- On twitter
- On the EURORDIS website
- Through a webinar
- Through the EURORDIS eNews
- Through the EURORDIS Member News
- At a conference
- Through a patient organisation
- Through a friend or family member
- Google
- Other, specify

MESSAGE AFTER SAVING THE QUESTIONNAIRE

Thank you for participating in this survey!

To make the voice of rare disease patients stronger, it would be great to have you as an **ambassador** so to help us to spread the word about Rare Barometer Voices!

Share the dedicated webpage with people living with a rare disease and patient organisations around you: eurordis.org/voices

In accordance with the Data Protection laws, you can access, modify, or suppress your information at any time. If you want to exercise this right and obtain information about your data, please contact rare.barometer@eurordis.org

Regarding access to cross-border health care for European Union citizens, please note that:

- To know more about your right to receive planned medical treatment in another European Union country and to be reimbursed for that treatment, please go to [your national contact point](#)².
- You can get a prescription from your doctor to use in another country in the European Union.
- You have the right to receive a copy of your medical record from your doctor when you seek to receive healthcare in another country in the European Union.
- Your doctor may be able to get help in treating you locally by contacting a network of doctors specialised in specific rare disease(s) called a European Reference Network. For more information please visit ec.europa.eu/health/ern_en

² https://europa.eu/youreurope/citizens/health/planned-healthcare/get-more-info/index_en.htm