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European Parliament Consultation on Rare Diseases

Fields marked with * are mandatory.

Thank you for launching the European Parliament survey on **Rare diseases**. The survey is open until 31 March 2025.

A disease is called a rare disease if it affects no more than five people in 10 000. While each rare disease affects a very limited number of people, the number of different rare diseases is estimated to be between 6 000 and 8 000. Thus, the total number of people living with a rare disease in the EU is estimated to be between 27 and 36 million. The total number of people living with a rare disease in the EU is therefore estimated to be between 27 and 36 million. The small number of patients per rare disease poses many problems, in particular with regard to their diagnosis, care and treatment. These include low market incentives to develop treatments for rare diseases, limited expertise in research, diagnosis and treatment of rare diseases, and the high cost of existing medicines.
The purpose of this public consultation is to provide a basis for the forthcoming work of the Public Health Committee to gain a better understanding and detailed knowledge of the challenges faced by people affected by rare diseases and the views of people working with or involved in rare diseases.

This survey is voluntary and anonymous. We do not ask for names or similar data. If you provide such information in the free text fields, it will be removed before your contribution is included in the detailed results of the survey. If you feel that a question would allow an individual to be identified, or if you simply do not feel comfortable answering it, please skip it without answering.

Click here to read our **Data Protection Notice** about how we will use the information you provide when completing this survey.

We greatly appreciate your participation in the survey.

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* Are you are replying as:
- ○ an individual
- ● a patient/support group
- ○ a healthcare professional, researcher, healthcare provider, research institute or university
- ○ an international organisation, government or other public body
- ○ a stakeholder or interest group

What is the name of your organisation?

100 character(s) maximum

EURORDIS-Rare Diseases Europe (covering all rare diseases: genetic, autoimmune & rare cancers)

How many employees/volunteers does your organisation have?

- ○ 1-9
- ● 10-49
- ○ 50-249
- ○ 250-499
- ○ 500 or more

Where is your organisation based?

- ○ Austria
- ○ Belgium
- ○ Bulgaria
- ○ Croatia
- ○ Cyprus
- ○ Czechia
- ○ Denmark
- ○ Estonia
- ○ Finland
- ● France
- ○ Germany
- ○ Greece
- ○ Hungary
- ○ Ireland
- ○ Italy
- ○ Latvia
- ○ Lithuania
- ○ Luxembourg
- ○ Malta
- ○ Netherlands
- ○ Poland
- ○ Portugal
- ○ Romania
- ○ Slovakia
- ○ Slovenia
- ○ Spain
- ○ Sweden
- ○ Outside the EU

Is your organisation registered in the EU Transparency Register? [https://transparencyregister. europa.eu/](https://transparencyregister.europa.eu/)

- ● Yes
- ○ No

Under the following registration number:

20 character(s) maximum

Has your organisation received funding from the EU in the last five years?

- ● Yes
- ○ No

Is your organisation mainly focusing on:

- ○ patients of all needs
- ● patients with rare diseases
- ○ other specific group of patients

Is your organisation active in:

○ One of the EU Member State
● In multiple (or all) EU Member States
○ Not active in any EU Member State

What are the major issues of rare disease patients according to your organisation?

☑ Difficulty in accessing care
☑ Hinders for accessing cross-border healthcare
☑ Difficulty in diagnosis
☑ Limited access to treatments
☑ High cost of care
☑ High cost of treatments
☑ High costs of accessing care (travel costs, etc)
☑ Access to support in the daily life
☑ Access to psychological support
☑ Other

To note: you can select more than one response for these questions, including the option " Other", where you can add further text up to 100 characters.

If other:

100 character(s) maximum

See evidence on ‘tiny.cc/RBresults’ & Rare 2030 report (‘rare2030.eu’: p.44,57,64,70,82,95,107,119)

Do you take actions to raise awareness about rare diseases and if so through which means?

☐ We do not take such actions
☑ Online promotion campaigns
☑ Off-line promotion campaigns
☑ TV or radio awareness
☑ Conference speeches
☑ Studies and publications
☑ Press articles
☑ Organising events
☑ Trainings
☑ Advertisements
☑ Presentations in schools
☑ Gadgets distribution
☑ Other

If other:

100 character(s) maximum

Rare Disease Day global campaign. Partnering, networking, advocacy: tiny.cc/wpg24

What are the major issues that your organisation faces?

☐ Reaching patients
☑ Raising awareness on the importance of rare diseases
☑ Raising funds
☐ Mobilisation of volunteers

☐ Finding staff with adequate knowledge about rare diseases
☑ Other

If other:

100 character(s) maximum

Funding uncertainty (e.g. EU operating grant), sustaining NGO's mission & securing independence

Do you collaborate with other stakeholders?

☐ We do not collaborate with other stakeholders
☑ Public entities in your country
☑ Public entities outside your country
☑ International bodies (including the EU)
☑ Patient and support groups
☑ NGOs/Charity organisations
☑ Healthcare institutions (hospitals, clinics...)
☑ Health professionals organisations
☑ Industry
☑ Media
☑ Other

If other:

100 character(s) maximum

‘tiny.cc/wpg24’ p.42-45: academia, clinicians incl. ERNs, geneticists, social workers, others

What types of resources or support services do you find most helpful for rare disease patients?

☑ Rare disease registries and databases
☑ Specialised centres and networks
☑ Genetic testing
☑ Telemedicine and digital health tools
☑ Multidisciplinary care teams
☑ Mental health and social support services
☑ Patient support groups
☑ Single contact points for all medical and administrative needs
☑ Information on ongoing and new clinical trials for patients and doctors
☑ Access to clinical trials
☑ Other

If other:

100 character(s) maximum

Resource Centres: info, services, connect infrastructures & stakeholders, support holistic needs

In your opinion, what types of resources or support services for rare disease patients need improvement?

☑ Rare disease registries and databases
☑ Specialised centres and networks

- ☑ Genetic testing
- ☑ Telemedicine and digital health tools
- ☑ Multidisciplinary care teams
- ☑ Mental health and social support services
- ☑ Patient support groups
- ☑ Single contact points for all medical and administrative needs
- ☑ Information on ongoing and new clinical trials for patients and doctors
- ☑ Access to clinical trials
- ☑ Other

If other:

100 character(s) maximum

Financial means, training GPs, genetic counselling; physical & psychological support

In your opinion, in which fields can EU improve its actions?

- ☑ Level of EU funding for research
- ☑ Time necessary for approval of new treatments/therapies
- ☑ Access to cross border healthcare
- ☑ Collaboration and coordination between healthcare professionals
- ☑ Collaboration and coordination between healthcare institutes/centres
- ☑ Patient engagement
- ☑ Specialised centres for rare disease
- ☑ Support networks
- ☑ Harmonisation of Member States' legislation
- ☑ Coordination of multinational research
- ☑ Other
- ☐ No need for improvement

If other:

100 character(s) maximum

NGO funding; coordination of EU RD Action Plan (multistakeholder EU body to steer RD policies)

In your opinion, in which fields can EU intervene more efficiently than the Member States alone?

- ☑ Support for research networks
- ☑ Support for development of database for research
- ☑ Support for development of database (information repository) for patients
- ☑ Coordination of national legislation
- ☑ Regulating the market
- ☑ Increased international cooperation
- ☑ Funding for research/medicines/treatments
- ☑ Awareness raising
- ☑ Other

If other:

100 character(s) maximum

Crossborder research,trials & care; mechanism/fund to improve access to treatment, joint procurement

Are you aware of EU actions in the area of rare diseases?

☐ I am not aware of any EU action in the area of rare diseases
☑ European Experts group on rare diseases
☑ Screen4care project
☑ European Reference Networks
☑ EUROCAT network
☑ Funding for R&D
☑ EU RD platform
☑ European Rare Diseases Research Alliance (ERDERA)
☑ Other

If other:

100 character(s) maximum

rare2030.eu; JARDIN, JARC Rare Cancer Agenda, EMA/COMP & ACT-EU; RD Horizon Europe & IHI projects

Is there anything else you would like to share regarding your views on care for rare diseases?

350 character(s) maximum

EU action plan on rare diseases: a strategic framework with common goals, KPIs bringing together EU policies on diagnostics, treatment access, crossborder care, innovation, social & mental health support, research; enhances health systems capacities for RD & leverages national strategies; to achieve the targets set in rare2030.eu recommendations

Background Documents

Data Protection Notice

Contact

Contact Form