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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?

150 character(s) maximum

European Society for Medical Oncology (ESMO) - Rare Cancers Working Group (RCWG)

Please indicate the type of organisation or company:

● Non-governmental organisation
○ Think tank
○ Media
○ Consumer organisation
○ Industry association
○ Company/SME/Start-up
○ Interest group
○ Trade union
○ Other

How many employees/volunteers does your company/organisation have?

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

Where is your organisation/company 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://transparency-register.europa.eu/index_en

● Yes
○ No

Under this registration number:

20 character(s) maximum

947929324977-41

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

○ Yes
● No

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

If other:

100 character(s) maximum

Difficulties in conducting well-powered clinical studies & few available registries / tissue banks.

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:

50 character(s) maximum

Engagement with the EU institutions.

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

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

For rare cancers, robust access to cross-border clinical trials and specialised rehabilitation.

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

In your opinion, in which fields can the 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

ERNs need to be financially sustainable and able to operate effectively across healthcare systems.

In your opinion, how important is the role of the EU in supporting rare disease research and treatment development?

- ● Very important
- ○ Quite important
- ○ Not important
- ○ Counterproductive
- ○ Prefer not to answer

In your opinion, does the EU adequately support research on medicines and treatment for rare diseases?

- ○ Fully agree
- ● Partly agree
- ○ Neither agree, nor disagree
- ○ Partly disagree
- ○ Fully disagree
- ○ Prefer not to answer

What should be changed?

100 character(s) maximum

In your opinion, does the EU adequately support introduction of new medicines and treatment for rare diseases to the market?

- ○ Fully agree
- ○ Partly agree
- ● Neither agree, nor disagree
- ○ Partly disagree
- ○ Fully disagree
- ○ Prefer not to answer

What should be changed?

100 character(s) maximum

In your opinion, does the industry in the EU have the necessary production potential for rare disease medicines?

- ○ Fully agree
- ○ Partly agree
- ● Neither agree, nor disagree
- ○ Partly disagree
- ○ Fully disagree
- ○ Prefer not to answer

In your opinion, does the EU adequately support production potential of the EU industry for rare diseases medicines?

- ○ Fully agree
- ○ Partly agree
- ● Neither agree, nor disagree
- ○ Partly disagree
- ○ Fully disagree
- ○ Prefer not to answer

What should be changed?

100 character(s) maximum

In your opinion, does the EU have a sufficiently large market for economically sustainable commercialisation of new medicines for rare diseases?

- ○ Fully agree
- ● Partly agree
- ○ Neither agree, nor disagree
- ○ Partly disagree
- ○ Fully disagree
- ○ Prefer not to answer

In your opinion, what could be done to make medicines more affordable and accessible to patients?

200 character(s) maximum

Ensure the uptake of Joint Clinical Assessment (JCA) Reports throughout national HTA processes, whilst reducing unnecessary duplication of work amongst HTA agencies in Europe.

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

EU Joint Action on Rare Cancers (JARC) - Rare Cancer Agenda 2030

Do you have any further remarks?

350 character(s) maximum

Rare cancers face difficult access to timely diagnosis, specialised care and a lack of feasibility in developing new therapies. EU-funded programmes are needed to support research on (very) rare tumours. Need to increase clinical trials and facilitate access to cross-border clinical trials in the EU. SANT is well-placed to help achieve these goals.

Background Documents

Data Protection Notice

Contact

Contact Form