New global survey to understand rare disease patients' experience of treatments

The new global Rare Barometer Voices survey on rare disease patients' experience of treatments is now live. The survey is available in 23 languages and is designed to provide an up-to-date insight into the unmet treatment needs of people living with a rare disease.

In 1999, the European Regulation on orphan medicinal products stated 'patients suffering from rare conditions should be entitled to the same quality of treatment as other patients'. Yet, in a short survey we conducted in 2017, 24% of respondents said that they had not been able to get the medical treatment they needed in the last 12 months as it was not available in their country, a figure which was 17% higher than for the general population. This new survey will provide more detailed facts and figures which can be used to advocate for positive change with regards to access to, research on and administration of treatments for the rare disease community.

Please support this survey by sharing it with your networks – the more people who respond and share their experiences, the more powerful our voice will be.

Survey link: http://bit.ly/eurordis-survey

What is the purpose of this survey?

The purpose of this survey is to help us better understand rare disease patients' experience of treatments and to generate facts and figures to help us to communicate these experiences to policy makers and institutions. Where possible, the results will be available for your country, disease and disease grouping and will be shared with you via an infographic which you can use in communications with your networks. Through asking questions such as whether treatments are available, whether these treatments are accessible and if these treatments have resulted in positive or negative experiences, we will be able to better measure and understand the unmet treatment needs of people living with rare diseases.

These insights will help us to build and strengthen our positions on a number of different themes, including:

- prioritisation of unmet treatment needs
- proposing practical and implementable solutions to accelerate the development of, and timely access to all, rare disease treatments and therapies
- reinforcing good clinical practices for paediatric and adult rare disease patients
- the evaluation of EU orphan drug regulation and the wider EU regulatory framework

- identification of off-label use of treatments.

Who can respond to the survey?

This global survey is open to anyone from any country in the world who is living with a rare disease, as well their family members and carers. The survey is available in 23 different languages. All responses are anonymous and will be kept in secure storage only accessible to our research team.

How can you take the survey?

The online survey can be accessed here and should take no more than 10 minutes to complete.

How long is the survey open for?

The survey closes on Tuesday 30 April.

What will the results tell us?

The survey responses will be used to develop insights into the experience of people living with a rare disease in relation to treatments. The Rare Barometer Voices framework enables the results to be analysed in a number of different ways. Firstly, we will be able to generate an overview of rare disease patients' experience of treatments at a global level. We will also be able to breakdown the results further to see any disparities in patients' experiences of treatment across geography, disease group, gender and age. In particular, we will be able to compare the paediatric experience to that of the adult population to show the specific challenges faced by each group.

How will the results be shared?

We will share the results with patient organisations, policy makers and the general public and use the findings in our advocacy work to drive real change for people living with a rare disease. The key insights will also be shared with all respondents via email and social media and the full report and associated infographic will be made available on the Rare Barometer Voices website.

Where response rates allow, upon request we will be able to create infographics to show country, ePAG (European Patient Advocacy Group) grouping and disease specific results. Requests for these tailored infographics can be sent to rare.barometer@eurordis.org.

How can you support this survey?

In addition to completing the survey yourself, please also share it with your networks via email and social media. The more people who respond and share their experiences, the more impactful our results will be to help drive real change for people living with a rare disease.

Survey link to share: http://bit.ly/eurordis-survey

Suggested social media message: New @eurordis survey now live! Is there a treatment for your rare disease? Are you able to access it when and where you need it? Take the new #RareBarometer survey on rare disease patients' experience of treatments and share your views http://bit.ly/eurordis-survey

Download the Rare Barometer Voices survey promotion toolkit here.

Rare Barometer Voices

Rare Barometer Voices is the EURORDIS survey initiative that brings together over 8,000 rare disease patients, family members and carers who share their experiences and opinions on the issues that matter to the rare disease community.

The Rare Barometer Voices survey software enables high-quality, secure data collection and analysis. Rare Barometer Voices is a EURORDIS-Rare Diseases Europe initiative and part of the wider Rare Barometer Programme, created to systematically collect patients' opinions on transversal topics and introduce them into the policy and decision-making process. The objective of the programme is to transform patients' and families' opinions and experiences into figures and facts that can be shared with a wider public and policymakers.

EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 800 rare disease patient organisations from 70 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe.

By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services. Follow @eurordis or see the EURORDIS Facebook page. For more information, visit www.eurordis.org.

Rare diseases

The European Union considers a disease as rare when it affects less than 1 in 2,000 citizens. Over 6,000 different rare diseases have been identified to date affecting an estimated 30 million people in Europe and 300 million worldwide. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offerings inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.

Contact

For more information or questions on Rare Barometer Voices or the rare disease patients' experience of treatments survey, please contact the Rare Barometer team at rare.barometer@eurordis.org