Press kit

Addressing the challenges of people living with a rare disease in Ukraine

In the best of times, the 30 million people living with rare diseases in Europe require specific actions and support to achieve equitable access to health and social needs. In times of crisis such as war and displacement but also pandemic, relief efforts must include specific consideration for the needs of this vulnerable population. This is the mission of EURORDIS - Rare Diseases Europe.

There are an estimated 2 million people in Ukraine living with a rare disease. There are over 6000 rare diseases. These are heterogeneous conditions but what unites them is that they are not well understood or researched (experts are also rare), difficult to diagnose and most often do not have a cure. Those affected by them typically require frequent and complex care. Many also have disabilities that make movement very difficult. **In the context of war, these families have extreme difficulty leaving their homes to access care or retrieve food and supplies that they need to survive without support, even when these are available.**

1. What are the unique challenges that people living with a rare disease in Ukraine are facing?

a. Inability to access highly specialised clinical knowledge to continue treatment

Where there is a treatment for rare diseases, they require highly specialised clinical knowledge which is only available in Kyiv or in other areas providing specialised care. **Many families who were able to relocate to the West of the country can no longer access therapies**.

Many Ukrainians are moving to Poland, because of family or friend ties, a similar language and the large shared border. Poland has been welcoming to Ukrainian refugees, and they have the same access to care as Poles and have been offered free train travel within Poland. However **rare disease patients need support beyond other refugees, for example, to find their speciality centres and access to specialised treatments** - a role that patient organisations are mobilising to help with within Poland. We also anticipate that many will go to other countries within the EU, and so expert centres need to anticipate and ideally facilitate the arrival of Ukrainian refugees.

> A mother and child with a rare metabolic condition were making their way out of Ukraine, via Moldova, but with the intention of continuing on to the EU. Requires access to medication, advice on countries where they would be most easily able to access medication and health services, and general

> support with resettlement. A patient organisation was able to secure short term access to medication.

b. Shortages of medicines and supplies in Ukraine

Preliminary exchanges with our member organisations in the area have evidenced shortages of medications and supplies already for a number of rare diseases in Ukraine. It's our understanding that in some cases treatments procured and paid for by Ukraine before the war have no safe channel to get into the country. In many cases, including Cystic Fibrosis, Epidermolysis Bullosa (EB) and Angelman's Syndrome, for example, there are organised patient groups on both sides of the border who are willing and able to help with the collection and transfer of badly needed materials, but there is as of yet no official corridor or no major aid organisation willing to get involved with this effort in a systematic way.

Even though we believe Nova Poshta, a private Ukrainian postal and courier company, is still making deliveries within Ukraine, we're exploring options for patients with rare diseases to receive medicines and medical devices through this system. Most importantly, we need to ensure that patients in territories not controlled by the Ukrainian government can benefit through 'aid corridors'.

EURORDIS is currently trying to conduct a rapid needs assessment which is extremely difficult for this rare and dispersed vulnerable population.

> Urgent challenges have been reported across the community. For example, the Ukrainian Cystic Fibrosis patient organisation, who represent 995 registered Cystic Fibrosis patients, is experiencing shortages of oxygen. People are in urgent need of portable inhalers and portable oxygen concentrators (for those who are oxygen-dependent) and medicines including enzymes, antibiotics, vitamins, Ursofalk, sodium chlorine 3%, 6%,7%.

c. Crossing the border as a person living with a rare disease or carer

Border restrictions for men aged 18-60

Many primary caregivers who are men are unable to leave Ukraine due to border restrictions and exemptions which do not recognise the reality of caring for someone with a rare and complex condition. There is an exemption already in place for fathers of children under 18. However, many parents still need to care for their disabled children even when they are over 18. There are also husbands who are the primary carer for their wives who have a rare disease or disability. This means families cannot safely leave the country because they are dependent on the men in their families to support a child or partner with a rare disease.

There is an immediate need for the exemption of men who have a son, daughter or partner with a rare disease to leave the country, regardless of the age of their dependents.

Delays in border crossing

To seek safety in another country, many require additional support to be transported to the border due to disability or medical devices. For many, this isn't an option because they cannot get

"fast-tracked" across the border and care needs mean they are unable to wait in line, with reports of the queue at borders taking 2-3 days.

Once on the other side of the border they may need adapted accommodation and to get very quick access to specialised care in the host country (where access to such treatments and care may also not be to standard).

> A family with a child with a rare neurological condition travelled to Poland. After waiting 24 hours they were advised that the wait is another 2-3 days. The family has abandoned the queue to seek treatment in Ukraine.
>
> A family travelling with a child with a rare intellectual disability are stuck in a 17km queue. Running out of hygiene supplies required for the care of the child, seeking to be prioritised.

2. Suggested angles to explore

- **How do requirements for men to stay in Ukraine affect the most vulnerable rare disease patients?** Men are unable to take their family to safety due to the lack of exemption for men who are carers over 18.

- **How does the European rare disease infrastructure hold up in a crisis?** European Reference Networks, established to connect rare disease experts across Europe through specialised hospital units, are mobilised to support the flow of patients to the European Union from Ukraine. How will this work in practice?

  We also recognise the long-overdue need to establish a surveillance and monitoring mechanism to track the needs and contacts of this already scattered population for the most efficient public health response through infrastructures such as a registry, helplines and centralised information platform (website) at the European level.

- **A united patient community: rare diseases know no borders.** We have numerous examples of grassroots efforts of EURORDIS member patient organisations supporting vulnerable patients to cross the border safely and access appropriate accommodation and care on the border. Organisations like Fundacja SMA, SMA Polska, and NoRo in Romania have set mobilised effective networks to support dozens of families to safely cross into Poland, Romania and elsewhere and get access to care on the other side.

  We are also reaching out to the WHO, the Red Cross, Doctors without Borders (Medecins Sans Frontieres), UN OCHA amongst others, to facilitate getting vulnerable people with rare diseases safely and quickly out of Ukraine and to help legally get essential supplies in

- **How will the solidarity mechanism - intended for access to emergency medicines - affect people living with a rare disease who need planned treatment?**

  The European Commission has set up a dedicated European system for swift transfers of persons in need of medical care among EU Member States, with the aim to ease the pressure on the health care systems of the Member States bordering Ukraine. The mechanism will support the matching of the needs for transfers of patients in the bordering EU Member States with offers and capacities for treatment and care available

in hospitals in other EU Member States, as well as to securely exchange the information needed.

- **What reimbursement rules will apply?** While the temporary protection Directive 2001/55/EC activated by the European Union Council's Justice and Home Affairs (JHA) body should provide necessary medical or other assistance to persons enjoying temporary protection who have special needs, it is unclear how these people will be able to access reimbursed medicines in Member States.

3. Who can you speak to?

We would be delighted to help you understand how the invasion of Ukraine has impacted on people living with a rare disease by connecting you to various people around Europe.

- National Rare Disease Alliances in Ukraine, Poland and Romania who have an overview of the rare disease infrastructures and number of people affected in their countries.
- EURORDIS member patient organisations representing individual conditions who have unique challenges.
- Individuals who have provided testimonies of their challenges and experiences.
- Coordinators of the European Reference Networks which work across Europe to connect specialised healthcare, knowledge and data.
- EURORDIS Ukraine taskforce members who are experts on the situation across Europe
- Aid agency partners

4. What has EURORDIS done?

1. Set up an **internal 'Ukraine Taskforce' (**ukraine@eurordis.org) to help us coordinate the work. We are evolving our ways of working as our understanding of what is needed changes.
2. Drafted and disseminated **a statement** and our CEO has made a video version for social media.
3. Responding to **individual questions and requests** for help as best we can, either by rerouting to a patient org where one exists or problem-solving with huge networks of heroic volunteers that are emerging.
4. **Targeted fundraising**, which has raised almost 10,000 EUR so far.
5. Set up an online resource centre for patients and patient organisations trying to help in and around Ukraine. This collects information about where refugees can go but it is also aiming to document evidence because we and our members and other NGOs will need this to have evidence to back up their requests.

6. Put out **a call for volunteers**, which is already live and going out on social media today and you can help us disseminate this: helpukraine@eurordis.org. This can be of help to us potentially, but also allows us to help our members to mobilise.

7. Call with Council of National Alliance and European Federation for Rare Diseases to establish a basis to collaborate and prioritise the next steps.

8. Organised a European Round Table of Companies **emergency** call shortly to understand how pharmaceutical and biotech companies can support people living with a rare disease in Ukraine.

9. Wrote a letter to Ukrainian authorities requesting to widen exemption criteria for men leaving the country to include all men caring for someone with a rare disease.

10. Contacted Polish authorities asking to **facilitate transportation of rare disease treatments** to people living in Ukraine.

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About EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 980 rare disease patient organisations from 74 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.

About 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. 72% of rare diseases are genetic whilst others are the result of infections (bacterial or viral), allergies and environmental causes, or are degenerative and proliferative. 70% of those genetic rare diseases start in childhood.

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.