

Urgent Support and Aid Needed for Ukrainian Patients with Rare Diseases and their Families

Michael Wilbur, Anna Kole

EURORDIS-Rare Diseases Europe, Plateforme Maladies Rares, Paris, France

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AT 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, relief efforts must include specific consideration for the needs of this vulnerable population.

The urgent need regarding people with a rare disease in Ukraine is for help to get vulnerable families safely out of the country and help to get essential medicines and supplies in.

There are an estimated 2 million people in Ukraine living with a rare disease. There are >6,000 rare diseases. They are heterogenous conditions, but what unites them is that they are not well understood (experts are also rare), difficult to diagnose, and most often do not have a cure. Those affected by a rare disease typically require frequent and complex care. Many also have disabilities that make movement very difficult. In the context of a war, these families have extreme difficulty leaving their homes without support to access care or retrieve food and supplies needed for survival, even when these are available.

Because it requires highly specialised clinical knowledge to care for a rare disease where some treatment is available, many families who were able to relocate to the West of the country can no longer access therapies that were only available in Kyiv or other areas providing specialised care. To seek safety in another country, many require additional support for transport within Ukraine to the border; once on the other side of the border, they may need adapted accommodation and very quick access to specialised care in a new country (where access to such treatments and care may not be widely understood by first responders and local healthcare professionals).

Preliminary exchanges with patient organisations in Ukraine have revealed evidence of shortages of medications and supplies already for a number of rare diseases. It is understood that in some cases, treatments procured and paid for by Ukraine before the war have no safe channel to



get in. In many cases, including cystic fibrosis, epidermolysis bullosa, and Angelman syndrome for example, there are organised patient groups on both sides of the border who are willing and able to help with collection and transfer of badly needed materials, but there is as of yet no official corridor and no major aid organisation willing to get involved with this effort in a systematic way. EURORDIS-Rare Diseases Europe¹ is liaising with agencies on their behalf as it is extremely difficult for this rare and dispersed vulnerable population to get on the radar of agencies and authorities as individual communities.

This means that if and when the ‘aid corridor’ agreed by Russia and Ukraine is put into effect, there will be little or no opportunity for patients with rare diseases to benefit.

Because many Ukrainians have family or friends already living in Poland, and because of language skills and a large, shared border, many Ukrainians are moving into Poland. 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, patients with rare diseases need more support beyond other refugees, for example to find their specialty centres and access to specialised treatments: a role that patient organisations are mobilising to help with within Poland and other countries. It is also anticipated that many further refugees will go to other countries within the European Union (EU), and so expert centres need to anticipate and ideally facilitate the arrival of Ukrainian refugees.

There have been very impressive examples of grassroots efforts of patient groups supporting vulnerable patients to cross the border safely and access appropriate accommodation and care on the border. Organisations like Fundacja SMA,² Debra International³ and EB Polska,⁴ and Edu⁵ and NoRo⁶ in Romania have mobilised effective networks to support dozens of families to safely cross into Poland, Romania, and elsewhere and to access care on the other side.

"In times of crisis, relief efforts must include specific consideration for the needs of this vulnerable population"

However, there are many vulnerable patients with rare diseases in Ukraine who would like to leave but are unable to because:

- > They need specialised transport to accommodate their medical devices or disability;
- > They cannot get 'fast tracked' across the border and their care needs mean they cannot wait in long lines overnight (there are reports of some border crossings having waits of 2-3 days); and
- > Men aged 18-60 years are not allowed to leave the country. In cases where a man is the carer of his spouse, or where a child's care requires two parents, there is no option for the parents to be separated. In Ukraine, many women do not have driving licenses and so cannot travel by car alone. There are also cases where a man is the sole carer of a child.

The immediate priorities therefore include:

1. For large aid agencies to facilitate moving vulnerable people with rare diseases safely and quickly out of Ukraine and to facilitate groups to legally get essential supplies into the country; and
2. For Ukrainian authorities to recognise the specialised needs of this population, including the immediate need for exemption for men who have a son, daughter, or partner with a rare disease to leave the country. The exemption for children should be regardless of the age of the child, as many fathers still need to care for their disabled children even when they are >18 years of age. This exemption will mean the family can safely leave the country if that is their need.

EURORDIS are currently reaching out to Ukrainian authorities, along with our member patient organisations there. We are also reaching out to the World Health Organization (WHO),⁷ Red Cross,⁸ Doctors without Borders (Médecins Sans Frontières),⁹ and United Nations Office for the Coordination of Humanitarian Affairs (UN OCHA)¹⁰ amongst others, to help us with this.

We are continuing to help patient organisations to connect to each other to exchange knowledge and information.

EURORDIS recognises and appreciates that there is an exemption in place for fathers of disabled children <18 years of age, and there have been some examples that show that this exemption is being respected by Ukrainian border guards when evidence is provided. But we need to be able to assure families who are afraid to risk making the long journey to the border that they will be allowed to cross, and expand this exemption so that it reflects the reality of caring for someone with a rare and complex condition. ■

Further resources

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