



HELPING UKRAINE TO BUILD BACK BETTER FOR RARE DISEASES

A EURORDIS report on the challenges faced by people living
in Ukraine affected by a rare disease

SEPTEMBER 2022



OVERVIEW

Since Russia invaded Ukraine on 24 February 2022, the normal flow of life in the country was interrupted in many ways. International organisations including the **United Nations** have documented the “biggest refugee crisis since the Second World War” with an estimated 8 million people who have fled Ukraine and more than 8 million Ukrainians displaced internally. In the regions hardest hit by the war, the humanitarian situation is very severe.

In the epicentre of the humanitarian crisis, although often forgotten or left behind, there are vulnerable populations, including people living with a rare disease, who require special attention and support to address their complex needs.

In the build-up to the fully fledged war in Ukraine, Ukrainian public awareness and government policy had been making significant progress. In 2015 Ukraine adopted the Law on Rare Diseases, which aspired to guarantee uninterrupted, free of charge, lifelong treatment and support for Ukrainians living with a rare disease (hereinafter - ULWRD), including the provision of essential medicines and relevant nutritional products. Following this milestone achievement, the government has covered treatments for 17 rare diseases through its state procurement programmes.

In October 2021, only 4 months before the invasion, Ukraine adopted an Action Plan to implement its National Strategy for Rare Diseases, with dedicated funding set aside. Many of the action points, though rigorously planned, were put on hold by the war.



In this report, we outline the issues still facing ULWRD, and explain why these issues remain today and will endure for the medium to long term. We will also set out why we believe the issues for ULWRD go beyond disaster relief. The war has imperilled the progress the country has been making and many organisations have an important role to play to help.

In Ukraine, when faced with destruction, people often say: 'We will build back better.' And so, we all have a role to play to help Ukraine to build back better for rare diseases.



Anzhelika,
Phenylketonuria



Danylo,
Phenylketonuria

The information in this report is derived from a series of meetings with patient organisations, NGOs, clinicians, and the Government of Ukraine. It also includes information received through a structured survey – the Rare Barometer programme – which gives us concrete insights into the needs of Ukrainian families.

It is important to recognise that this is a rapidly changing situation. This report was compiled between June and July 2022, and what is true today may be different in a few weeks' time. EURORDIS will endeavour to review the situation and provide updates as needed.

A vulnerable population on the move face challenges managing their conditions

Based on the data from the **Rare Barometer** Survey on the needs of ULWRD, nearly 40% of Ukrainian families living with a rare disease were forced to move, and have been at least temporarily displaced internally or left the country (see Figure 1).



Figure 1

The war and its consequences have been traumatic and created disruption for all Ukrainians. However, as those living with a rare disease typically require frequent and complex care or have disabilities affecting their mobility, in the context of war, these families face even greater difficulty to leave their homes to access care, food or other vital services, even when these are available.

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 immediate access to specialised care in a new country - which may not be widely understood by first responders and local health care professionals.



Medicine for
Pulmonary hypertension



According to the results of the Rare Barometer Survey, 8 in 10 Ukrainian rare disease patients are affected by at least one interruption of the care they usually receive:

7 to 8 in 10

of those who moved to
another city within Ukraine

6 to 7 in 10

of those who left
Ukraine

5 to 6 in 10

for those who are
still living in Ukraine

For those living with the conditions benefiting from state procurement, the availability of rare disease medicines often depends on their registered residence. Given the high mobility of the Ukrainian population since February 2022 and the interruption of the previous links with their usual health care facilities, it became increasingly difficult for ULWRD to receive their medicine and food supplies.

This is even more complex now due to the dynamic nature of the war: families come back to their homes and then they flee again - be it because of their changing perception of the war or the availability of appropriate housing options. This makes it difficult for the authorities to allocate supplies across the country in an equitable way.

This, combined with the general disruption of supply chains, goes some way to explaining why our survey also shows that nearly half of people with rare diseases displaced within Ukraine have had difficulty renewing a prescription and accessing the treatment that they need (see Figure 4).

Patient Organisations at the forefront of helping Ukrainians living with a rare disease

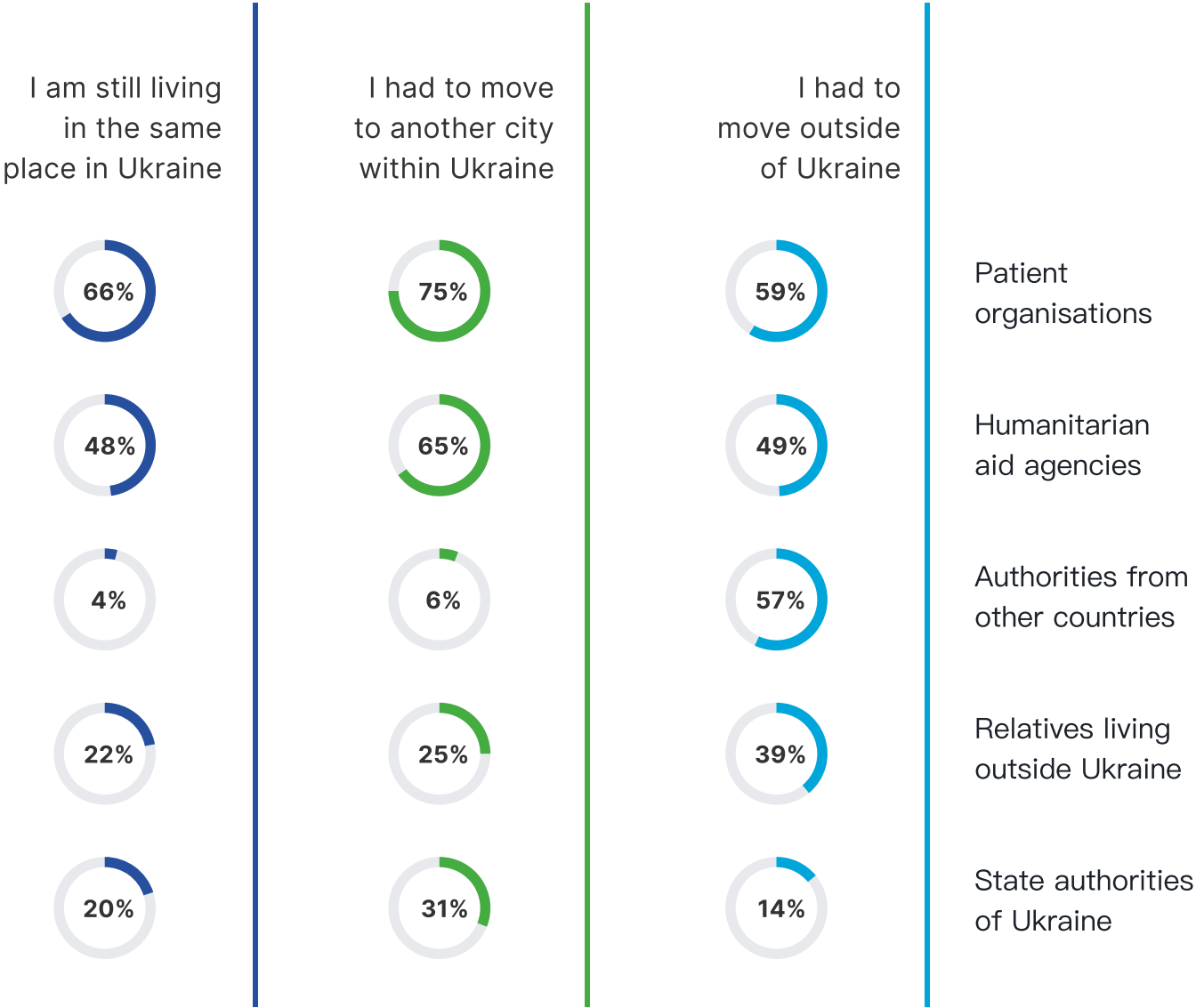
As witnessed by researchers¹, during the first months of the war all humanitarian aid inside Ukraine was implemented by local actors. Rare disease patient organisations in Ukraine have been at the forefront of supporting ULWRD, to a large extent with the support from foreign patient organisations, charities, donors, and pharmaceutical companies.

¹ See the report at:

www.humanitarianoutcomes.org/sites/default/files/publications/ukraine_review_2022.pdf

Our Rare Barometer Survey figures show the majority of ULWRD have been in contact with patient or aid organisations or volunteers since the beginning of the war (see Figure 2). Medicines, food and other basic supplies, as well the information on how to get access to services or benefits were among the main types of aid provided to ULWRD (see Figure 3).

Since the beginning of the war, have you been in contact with any of the following organisations or persons?

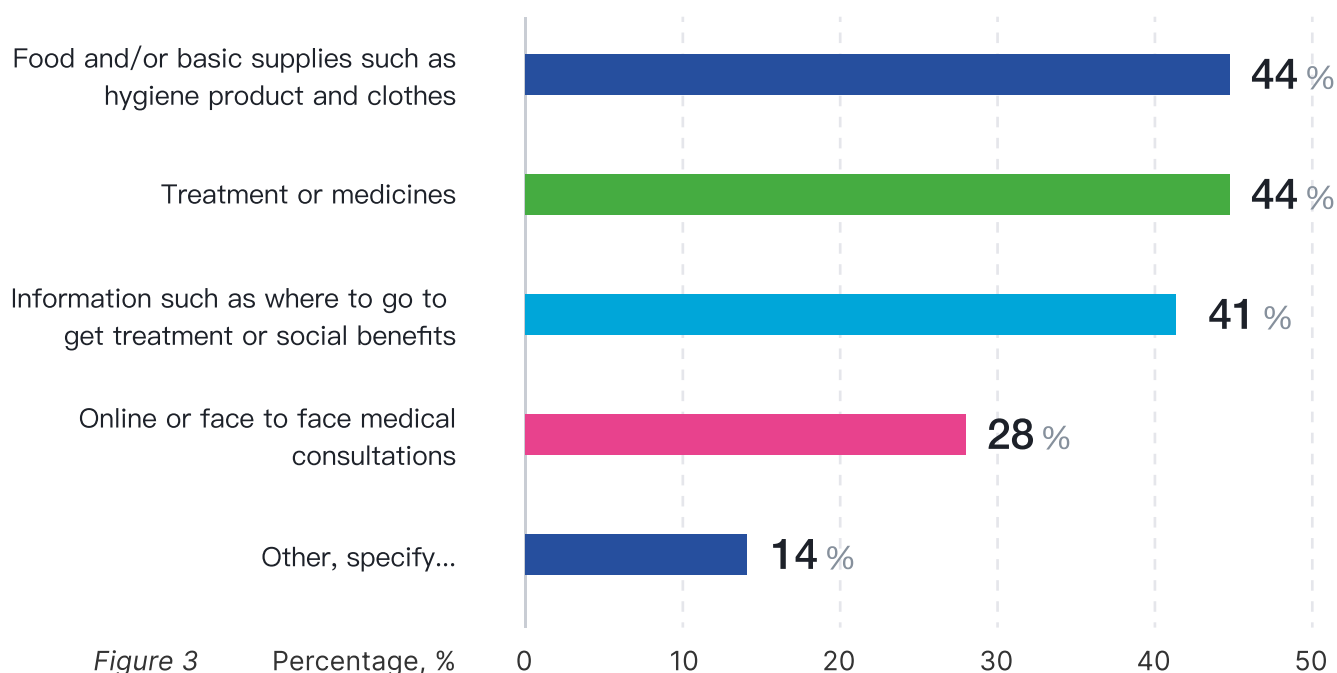


Myroslava,
Pulmonary
hypertension



Figure 2

What type of help did you receive from them? They provided...



In particular, the following assistance was provided to Ukrainian families living with a rare disease, by Ukrainian patient organisations:

- Helping ULWRD to move abroad, e.g. searching and arranging temporary accommodation for patients and their families who had to leave their homes.
- Legal and informational support for ULWRD. Legal help has been particularly important for those who left behind or lost their documents - some of which are crucial either to leave the country or to receive access to certain services.
- Covering the critical needs of ULWRD in medicines, nutrition, and medical equipment - if this could not be covered by the state or/and local budgets.
- Providing medicines and equipment to Ukrainian clinics working with rare disease patients.



Daryna,
Phenylketonuria

While the effort put into supporting families of ULWRD is steadily significant, **it must be noted that with time passing by and the world's attention shifting towards other crises, the amount of humanitarian aid imported in Ukraine is visibly decreasing.** Ukrainian patient groups warn us that even if the needs of ULWRD for this year might be covered due to the combination of the incoming donations and the rest of the previously procured/received treatments, the situation is expected to get much more problematic in the following years.

To help with the urgent humanitarian challenges provoked by the war, the international aid community should maintain a focus on supporting the Ukrainian rare disease community, and pharmaceutical companies should continue to donate medicines even though Ukraine is no longer dominating the headlines.

Maksym,
Pulmonary
hypertension



Regenerating momentum for the national strategy for Rare Diseases

Unsurprisingly, Ukrainian health care providers and government officials have had to focus on the realities of running a health service during the war. This focus on emergency response has certainly slowed progress on the new action plan to implement the national strategy on rare diseases in Ukraine. Because rare diseases were high on the agenda in the pre-war period, this means that in many ways the national strategy for rare diseases is another casualty.

Areas of the action plan heavily impacted include:

- Connecting Orphanet to the Ukrainian digital health care system and initiating the mapping of rare disease patients in Ukraine.
- Creating a network of centres of expertise on rare diseases in Ukraine.
- Expansion of the newborn screening programme.
- Extension of state-guaranteed treatments to more rare diseases. Among them: SMA, Duchenne and Tuberous sclerosis (including the promotion of registration of innovative medicines in Ukraine).

Despite these setbacks, officials, clinicians, and patient groups in Ukraine are working hard to press ahead with the core elements of this plan. Geneticists are being trained. Doctors are working on translations of Orphanet documentation into Ukrainian. The Ministry of Health continues to engage and look into solutions.

European clinical experts, European patient groups, and global pharmaceutical companies all can play an important role in supporting Ukraine to make up for the lost time by helping with training, sharing information and (in the case of pharmaceuticals) registering products in Ukraine and looking for sustainable medium-to-long-term agreements that reflect the new burdens placed on the Ukrainian health system.

Baby of a person
living with Pulmonary
hypertension



The point about registering medicines is particularly important as many people from Ukraine have been getting access to products not usually available in the country - either because they got treatment abroad, the product was donated, or because under the current simplified procedures for the importation of medicines as humanitarian aid, even unregistered drugs can be exceptionally brought in the country. Without the long-term and generous engagement of companies, what will happen to these families when the war is over, people move home, or donated medicines are no longer available?

Donation
of medicine
for Pulmonary
hypertension



THE HIGHEST PRIORITY OUTSTANDING ISSUES

Hanna,
Pulmonary
hypertension



1. Access to medicines and supplies

In Ukraine, PLWRD receive much-needed medicine and other supplies (e.g. specialised nutrition) from a two-level system: the government organises national-wide procurement channelled through the MoH; the regional level operates through municipalities and local budgets. For ULWRD staying in Ukraine, the situation with access to treatments and other specialised products, thus, varies significantly from one region to another and even within regions. ULWRD displaced internally face significant challenges as they become subordinated to new municipalities and health facilities.

As suggested by the Rare Barometer Survey, difficulties in obtaining a renewal or change of an orphan medicinal product concern a significant number of respondents (four in ten on average), especially for those who have moved from where they lived (see Figure 4).

Those ULWRD who moved abroad still find it difficult to fully satisfy their needs. For some, this can be about the difficulties of integrating into a different health system. Sometimes supplementary treatments available in Ukraine are not available in the country where people have relocated. In other cases, protocols of treatment in Ukraine and in the host country vary significantly. For some patients, getting an appointment with a needed specialist takes weeks or months which might mean an interrupted treatment and risks a regression in their condition.

Do you or the person you care for need and have problems accessing... Renewal or change of medications?

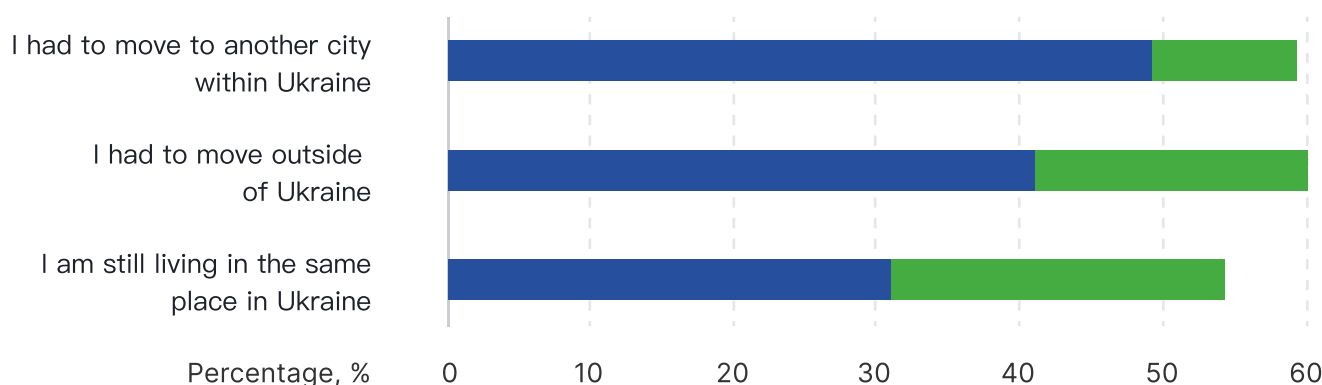


Figure 4

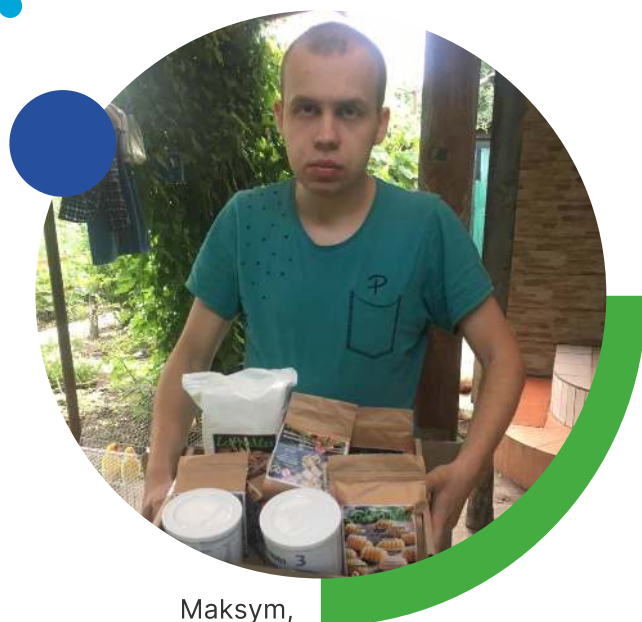
Yes since the war began

Yes but it was already the case before the war

2. Covering basic needs

Covering basic needs, especially housing, is equally important inside Ukraine as in other countries of displacement. Many people's houses were damaged, destroyed or are in occupied cities. Many no longer have jobs, financial means, or a place to live.

Based on the findings of the Rare Barometer Survey, access to the following essential goods and services is seriously hindered (see Figure 5):



Maksym,
Phenylketonuria

The treatment or medicines you need and used to have access to

Money, resources or income to live

A suitable place to live in

Enough food, or being able to cook food

Enough or good enough clothes, bedding and blankets

Safe water for drinking and cooking

Soap, water, sanitary material and a suitable place to wash

Clean toilets

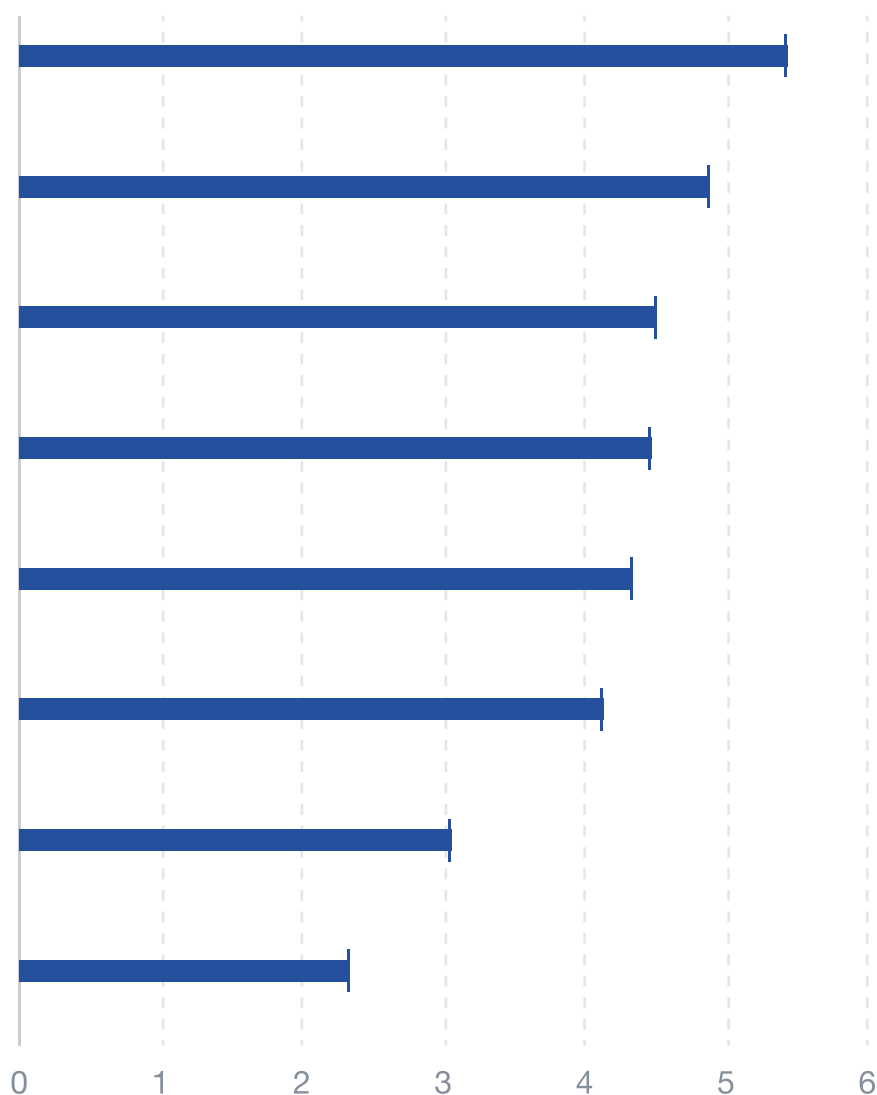


Figure 5

0
No problem

5
Moderate
problems

10
Catastrophic
problems

3. Access to care and medical consultations

In Ukraine, for some diseases that require in-patient care (e.g. for patients with cystic fibrosis who require hospitalisations 2-3 times per year), it is difficult to place the patient with health care facilities. Due to the active hostilities, especially affecting the country's Southern and Eastern regions, the general population and some clinicians left the country or are internally displaced. Some health care facilities are now destroyed or non-functional. Thus, for the rest of the clinics, mainly in Central and Western Ukraine, the number of patients becomes larger and more difficult to manage.

This challenge has the potential to become a longer term one, as European countries look to fast-track Ukrainian health care professionals so they can more easily work abroad. For rare conditions, this means that patients are left without any access to an expert on their disease.

Another challenge for accessing care and medical consultations concern areas that are under occupation or affected by active hostilities. If people are unable to leave, one of the only options for them to access health care services would be through telemedicine.

According to the results of the Rare Barometer Survey on Ukraine, there is a clear willingness to participate in specialised online consultations (see Figure 6). This can be part of the solution, when clinical expertise is not located in the same place as the patient - especially when a local general practitioner can participate.

Would it be helpful for you or the person you care for to participate in online consultations with the following specialists...

- Yes and we have the appropriate device and Internet connexion
- Yes but we don't have the appropriate device and Internet connexion
- No
- I don't know

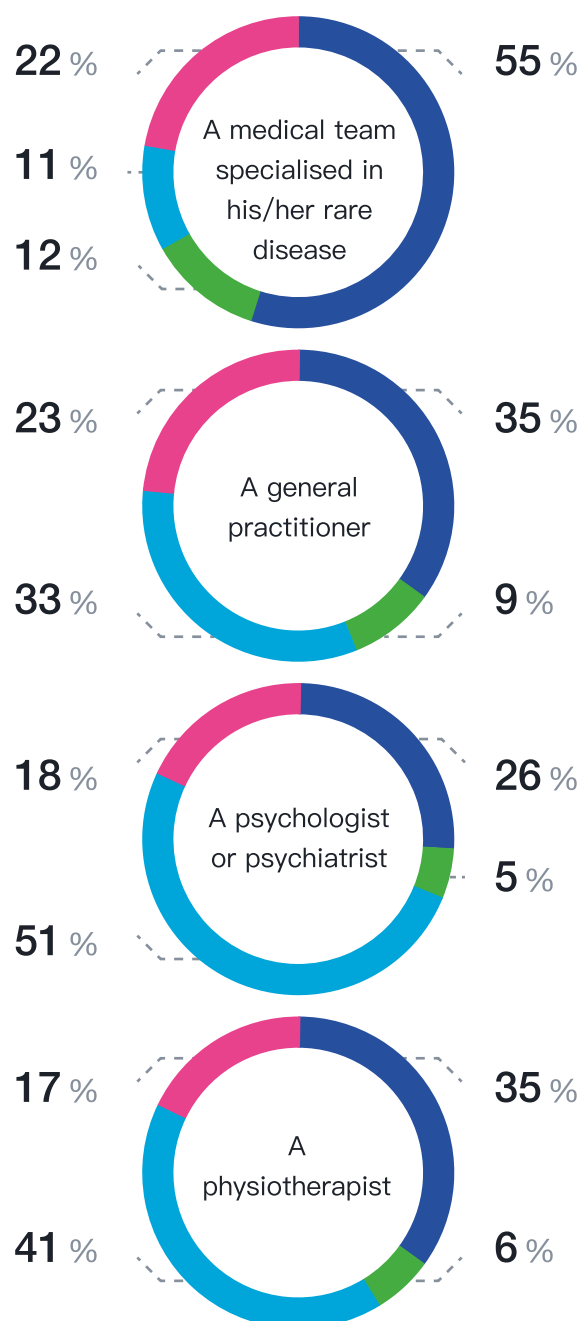


Figure 6

4. Supporting ULWRD looking to leave the country

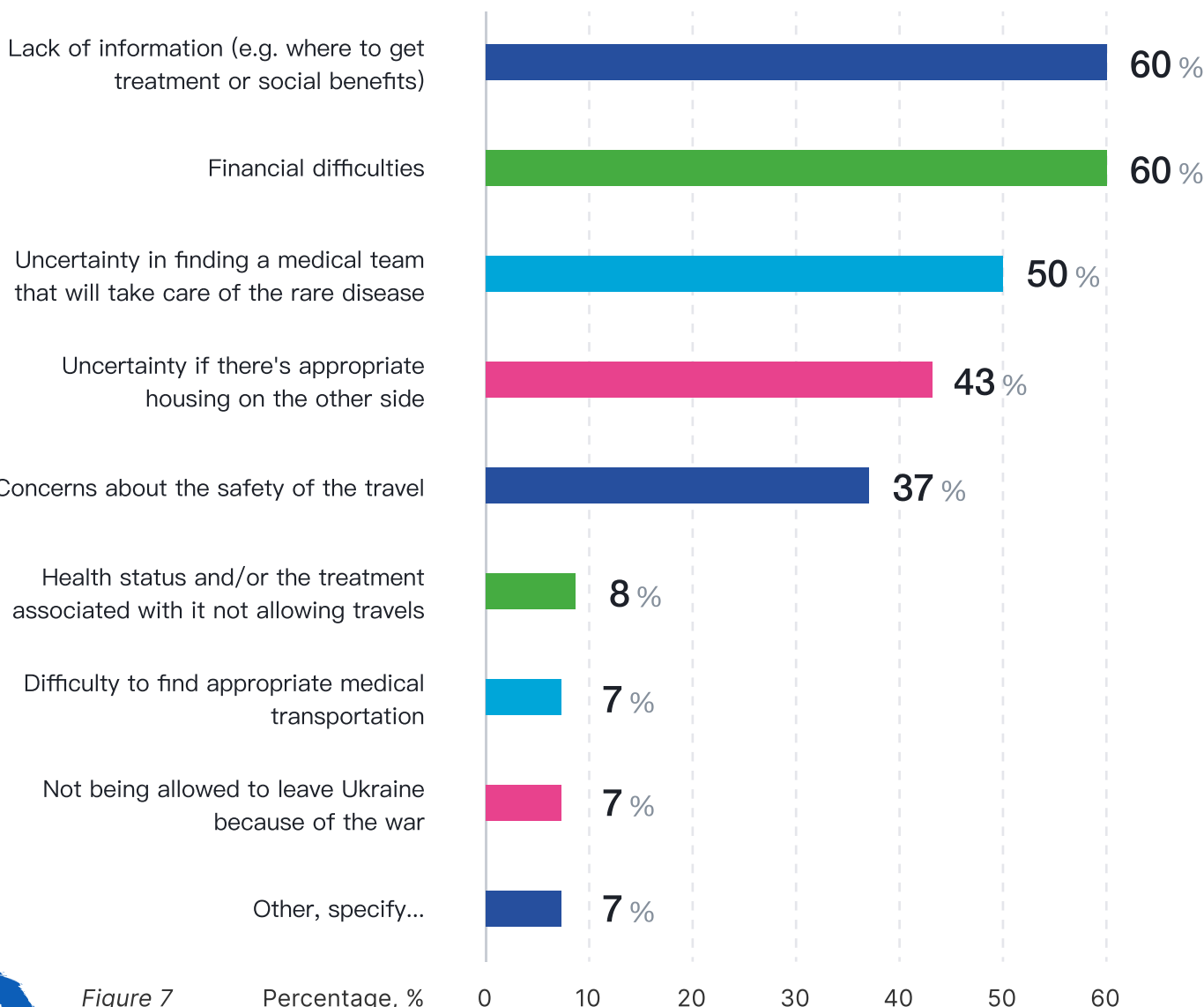
As follows from the results of the Rare Barometer Survey, 2 in 10 of those still living in Ukraine would have sought to leave the country if they could.

The two main obstacles preventing those families from leaving Ukraine is the lack of information such as where to get treatment or social benefits (quoted by 61% of the respondents) and financial difficulties (also 61%) (see Figure 7).

Other significant challenges for those willing to flee the war, as witnessed by Ukrainian patient organisations, include finding a recipient part (a healthcare facility) in the new country, leaving occupied territories or the active hostilities areas, and organising medical transportation (if necessary).

EURORDIS estimates that addressing the needs of this category of ULWRD is potentially among the most necessary fields of intervention given their feasibility and impact.

What are the main obstacles preventing you or your family from leaving Ukraine? (several answers possible)



5. Providing psychological support

Perhaps unsurprisingly, mental health has deteriorated in a context of uncertainty and threat.

Previous research shows people affected by rare diseases are three times more likely to report sadness and depression than the general population. Now, these families have a double burden.

Based on the data from our survey (see Figure 8) 85% say they are very often, often, or sometimes depressed or unhappy since the start of the war, with 59% saying they are often very often unhappy. 77% report not being able to solve their problems. 60% say they feel isolated.

3 in 10 say they would take advantage of online psychological support if available.

Those who are still living in Ukraine (in the same or another city since the war began) express a widespread feeling of uncertainty: 7 in 10 don't know if they are safe or say that they are probably safe where they are. There is a feeling of imminent threat for 26% who do not feel safe and protected where they are. The feeling of uncertainty is still prominent among those who left Ukraine (52%) but the feeling of direct threat only concerns a few (3%).

Since the war began, how often..

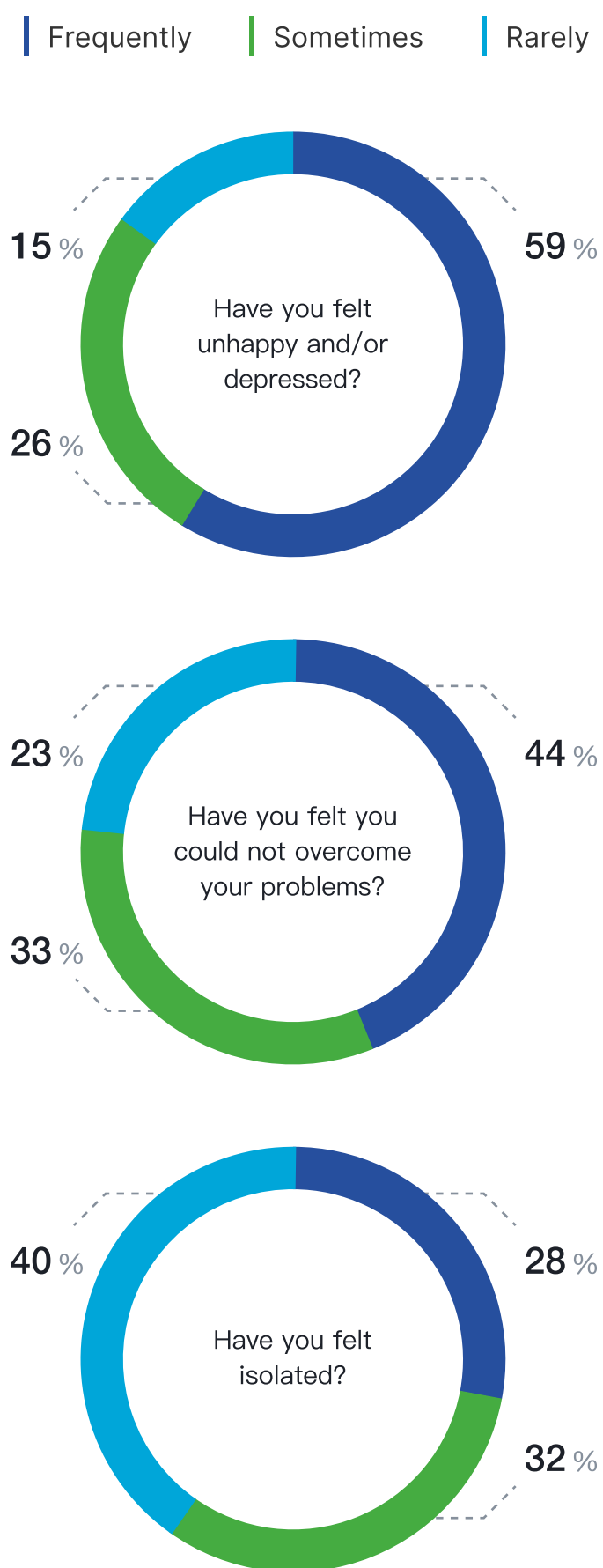


Figure 8

EURORDIS RECOMMENDATIONS FOR THE SHORT AND MEDIUM TERM

We hope that the information helps demonstrate that the challenges facing Ukrainians living with a rare disease are severe, fast-evolving, and likely to last for the medium-to-long term. There are clear issues related to providing immediate short-term care and support to people who need it, but also a real need for actors to engage with Ukraine strategically to help it to make up for lost time on the implementation of its national strategy.

Key recommendations by actor are as follows:

Humanitarian organisations can help by linking with Ukrainian patient organisations to provide **basic needs** such as food, accommodation, hygiene products and financial support. They can contribute by helping to maintain public interest in the ongoing needs in Ukraine. For the next phases of their response in Ukraine, and for all future crises, they can adopt a **proactive approach** to assessing the needs of PLWRD and include them in the design of their response programmes. EURORDIS is working with aid agencies to better identify and understand the needs of this population, and we are open to share information and experience with any aid organisation upon request.

The **European Commission** has done a lot to try to support Ukraine. It can further help by providing financial support to countries providing treatments for Ukrainian rare disease patients that are not available (or no longer available) for them in Ukraine to maintain sustainability of health systems in receiving countries. It can ensure that its funding programmes reach the most vulnerable and empower civil society, given their role in providing support to ULWRD. It can also create a framework that allows an official partnership between ERNs and Ukrainian institutions so that clinical experts in Europe have more options on how they can support their Ukrainian colleagues.



Patient organisations can continue to provide the much needed hands-on help to support the logistical needs of families coming from Ukraine. The latter often need help in navigating local systems, accessing health care and housing, getting their children into appropriate schools and making links to their new if temporary communities. Patient groups can also refer Ukrainian families to EURORDIS' programmes that may help them, including our short-term housing programme with Airbnb and our programme providing free online psychological support to ULWRD.

European Reference Networks, HCPs, and healthcare workers can help by supporting the Rare Disease Virtual Hub - hosted in Barcelona's Children's Hospital - in their work to help Ukrainians in the EU navigate European specialty centres. Clinicians can look for new and innovative ways to support their generalist colleagues in Ukraine to provide specialist medical advice to displaced families. HCPs and clinicians can participate in opportunities to train Ukrainian clinicians, speak in virtual conferences, and even look to build speciality skills in Ukrainian doctors now working in European clinics so they come back with additional skill set. Ukrainian health professionals should ideally become integrated "as official affiliated partners to the ERNs [European Reference Networks] with the concept of seamless care on the European territories"².

² The European Reference Networks for rare and complex diseases respond to the Ukrainian crisis, The Lancet Regional Health - Europe, Volume 19, 2022, 100464, ISSN 2666-7762,

doi.org/10.1016/j.lanepe.2022.100464



Roman, Pulmonary hypertension

Pharmaceutical companies should continue making short-term donations of medicines needed as much today as they were in March. They are also strongly encouraged to register their products in Ukraine to reduce the risk of sudden loss of access when the war is over. Finally, they can look for sensible and sustainable longer-term arrangements with the Ministry of Health of Ukraine and, where necessary, involvement of international organisations to secure access to medications in Ukraine.

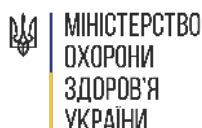
If we all do our part, together we can help Ukraine to build back better for rare diseases.



NON-GOVERNMENTAL ORGANISATIONS, PATIENT GROUPS, AND PUBLIC ENTITIES CONTRIBUTING TO THIS REPORT



EURORDIS-Rare
Diseases Europe



Ministry of Health of
Ukraine



National Specialised
Children's Hospital
"Okhmatdyt" of the Ministry
of Health of Ukraine



All-Ukrainian organisation
"Orphan diseases of
Ukraine"



Charitable foundation
«Sister Dalila»



NGO "Association of
Patients with Pulmonary
Hypertension"



NGO "Ukrainian
Association of Patients
with Phenylketonuria"
Special Children"



NGO "Growth"



Charity Fund
"Wings of Hope"



NGO "All-Ukrainian
Association for Helping
Patients with Tuberculous
Sclerosis"



DEBRA Ukraine



All-Ukrainian Association
of Aid to Cystic Fibrosis
Patients



Charitable Foundation
"Orphanni Synytsi"



Charitable Foundation
"Children with spinal
muscular atrophy"

Public organisation "Harmony of Movement"

NGO "Association of disabled people -
patients with mucopolysaccharidosis"

NGO "Step Forward"

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EURORDIS is proud that the design of this document has been implemented by a Ukrainian professional: Alona Bululukova, Kharkiv.