

9 MONTHS OF RESCUE

REPORT ON HUMANITARIAN AID FOR UKRAINIAN RARE DISEASE PATIENTS



Since the day the war broke out, we have been supporting patients with rare diseases who are fleeing the horrors of war. We help them both in Ukraine and after reaching Poland.

The war that began in Ukraine is an unprecedented event in recent history. A war in which there are no rules and in which civilian objects, homes, kindergartens, and hospitals are bombed and shelled. A war in which the victims are innocent Ukrainians civilians: mothers, children, and the sick.

A war that has already forced **almost 5 million people to flee the country**. Among them are also patients with rare diseases. And even more patients with rare diseases remain in Ukraine, uncertain of whether they will be able to escape and whether anyone will support them in their evacuations and secure their treatments.

Since the beginning of the war, we have been doing our best to prevent Ukrainian patients with rare diseases from feeling abandoned and left to deal with their difficulties alone. We support them in many ways: we help them to flee the war, we provide treatment in Poland and other European countries, we offer legal assistance and provide information, and finally, we provide on-site support for Ukrainian patients and the medical community.

Now it turns from an ad hoc project, forced by the situation, into a well-thought-out support system with a dedicated team responsible for specific actions.

What are rare diseases?

A disease is rare when it affects less than 1 in 2,000 citizens in given country. But so far, over 6,000 different rare diseases have been identified, which in total affect 3.5 – 6% of the population worldwide.

That is why rare are not that rare; **in Ukraine, there are approximately 1.5 - 2.5 million people with rare diseases.** Some of them suffer from more common and well-known rare diseases such as haemophilia or primary immunodeficiencies, while others from diseases that affect only few persons in all of Ukraine.

80% of rare diseases are of genetic origin and are often **chronic and life-threatening.**



Around
1.5–2 mln
RD patients
live in Ukraine



RD affect
<1:2000
people

There is over

6000

different RD



80% of RD
are genetics

How do we support rare disease patients from Ukraine?

Our work is currently based on four main areas of activity:

1. Helping patients fleeing the war in Ukraine

We provide patients with support at every stage of their journey to Poland or transit to other European countries, including faster transport across the border, organizing accommodation, and access to treatment.

2. On-site support for Ukrainian patients and the medical community

We organize the transportation of medicines and medical equipment to hospitals treating patients with rare diseases in Ukraine and we also finance the purchase of medicines on the spot.

3. Information and legal support for patients

We prepare dedicated websites with information for RD patients, legal opinions, printed materials, translations of medical records, and provide support in legal matters related to staying in the EU.

4. Raising awareness about the situation of RD patients from Ukraine

We speak at industry conferences and stakeholder meetings, give interviews, and take part in podcasts to draw attention to the needs of RD patients in Ukraine and encourage others to act.



Helping patients fleeing the war in Ukraine

- We provide patients with support at **every stage** of their journey to Poland or other European countries
- We organize humanitarian corridors to ensure faster border crossings for patients in serious condition
- We take care of **short-term accommodation** for the patient's family
- We provide access to treatment in Poland (we cover all procedures and hospitalizations and **arrange the first visit to a specialist in a given field**)
- We offer patients and their family members **free psychological care** in Ukrainian
- In the case of transit to other European countries, we provide contact to a patient organization in each country **as well as to a medical clinic treating a given disease**



*This is Yehor, an 11 yo
XLA patient receiving
immunoglobulins
at a Polish hospital*

So far, we have managed to help over 80 families of patients with rare diseases from Ukraine.

Helping patients fleeing the war in Ukraine

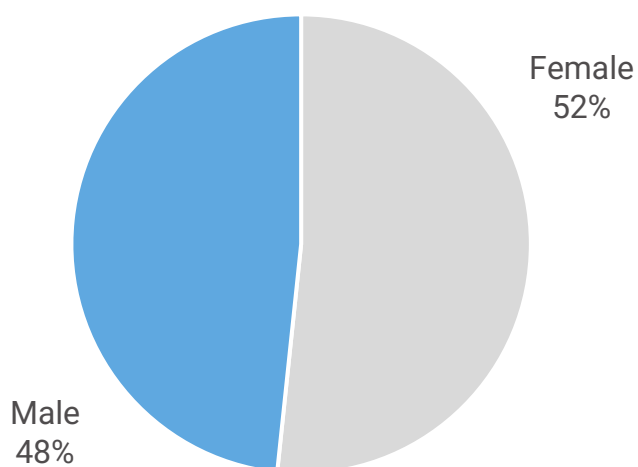
So far, we have helped **over 80 families of patients with rare diseases**, including primary immunodeficiencies, epidermolysis bullosa, or rare cases such as KIF1A.

In the cases of patients in serious condition, we make sure **to create a fast track for them** so that they do not have to wait in long lines at the border. In the first days of the war, the long lines required even several days of waiting, so it was crucial to speed up the process for RD patients.

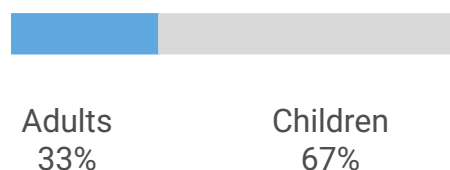
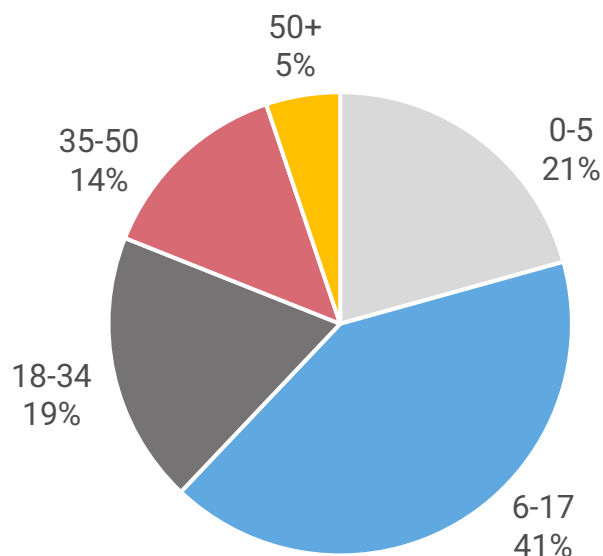
Most of the patients we have helped are children, but more than 30% of them are adults, most often with **very serious forms of their diseases** and requiring advanced treatment outside Ukraine, including transplantations.

Rare disease patients we have helped

Gender

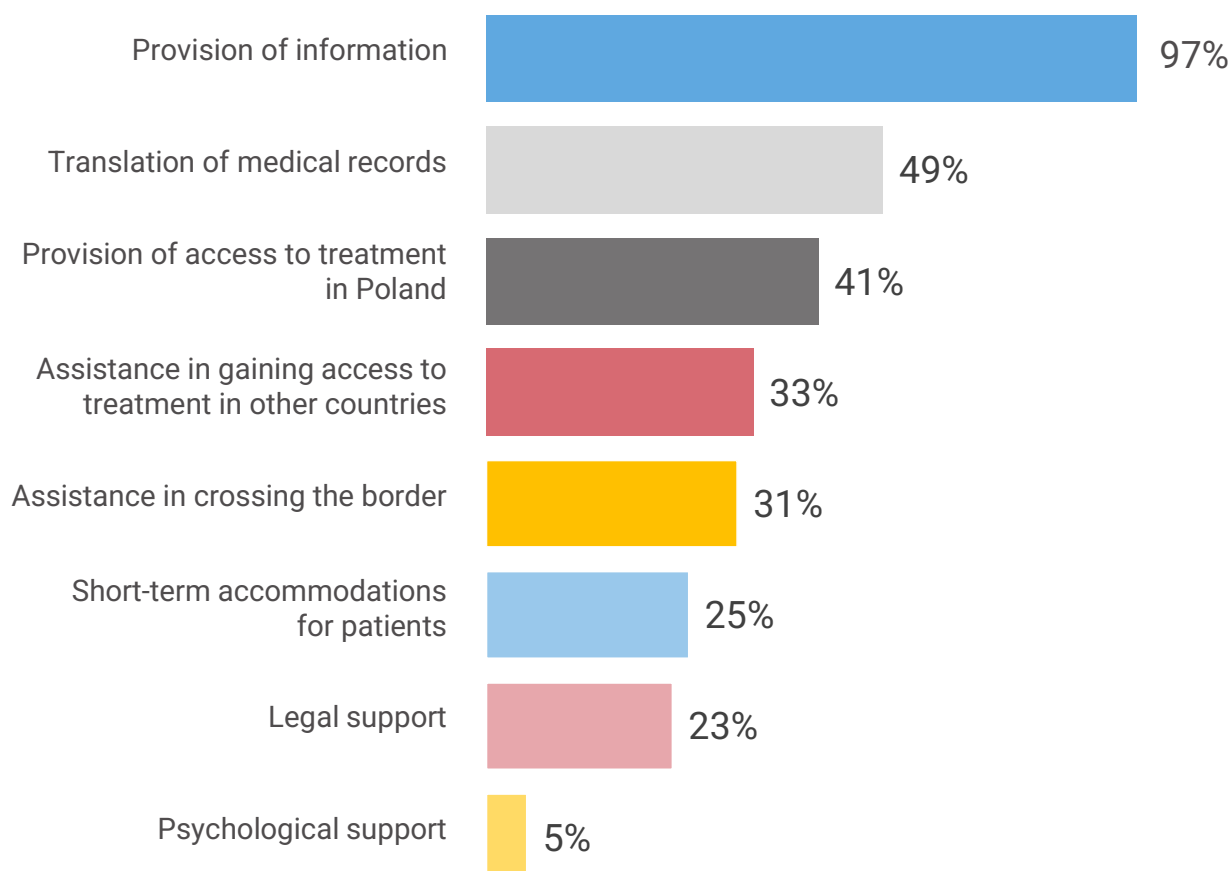


Age of the patients



Helping patients fleeing the war in Ukraine

The most important form of our help is, of course, **providing patients with access to appropriate treatment** – both in Poland and in other European countries. This concerned **three quarters of the patients we helped** (41% are treated in Poland, and 33% obtained access to treatment abroad). To make this possible, in many cases it first required assistance in **crossing the border quickly** (31%), **arranging short-term accommodation** (25%) and **translating medical records** (49%). However, this certainly does not exhaust the scope of our assistance – **we provided information support to almost all patients at various stages of their stay in Poland**. In many cases, it was also highly specialized assistance in the form of **legal advice** or preparation of letters or documents for the relevant offices in Poland (23%).



Helping patients fleeing the war in Ukraine

Patients whom we've helped cross the border and receive treatment in Poland have often suffered from very rare diseases. Some of them, like LAD-I, only occur in 1 in 500,000 cases. KIF1A-related disorder is even rarer: there are only 150 diagnosed patients worldwide. All of them **require a very specialized approach**. That is why our activities are always focused on **providing adequate medical care** in a specialized medical center **as soon as possible**.

List of rare diseases in Ukrainian patients whom we have helped
Autoimmune lymphoproliferative syndrome (ALPS)
Combined immunodeficiency due to DOCK8 deficiency
Common variable immunodeficiency (CVID)
DiGeorge syndrome
Epidermolysis Bullosa (EB)
Glut1 deficiency syndrome (Glut1DS)
Hepatopulmonary syndrome (HPS)
Hyper IgD Syndrome (HIDS)
Hyper IgE Syndrome (HIES)
Hypo-IgM Syndrome
Kabuki syndrome
KIF1A-Related Disorder
Leukocyte adhesion deficiency type-1 (LAD-I)
Myasthenia gravis (MG)
Nijmegen breakage syndrome (NBS)
Noonan syndrome (NS)
Phenylketonuria (PKU)
Severe combined immunodeficiency (SCID)
Spinal muscular atrophy (SMA)
SYNGAP1
Wilson's disease
X-linked agammaglobulinemia (XLA)

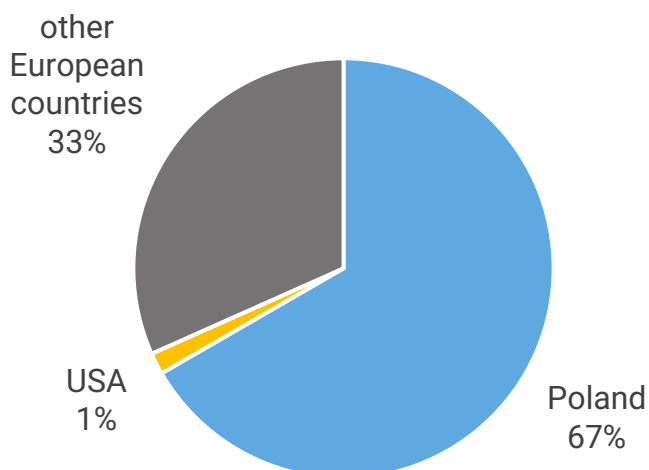
Helping patients fleeing the war in Ukraine

When Poland was the target country, we first provided patients with the necessary support in finding accommodation and completing the necessary formalities (**obtaining refugee status** and a PESEL number). Then, we provided them with **appropriate medical care**: we made appointments with specialist physicians or provided hospitalization in medical centers if their health conditions required it.



Map of hospitals in Poland where RD patients we helped are being treated

Patients who stay in Poland vs. patients for whom we have provided care in other countries



In the cases of patients who decided to travel farther to other countries, we provided contact with patient organizations operating in a given country, obtained information on which medical center they should go to, or contacted doctors directly.

In some cases, patients first stayed in Poland where they received appropriate treatment, and only later decided to travel farther to another country.

Every patient has a unique story (1)

Meet Emilia (Емілія)!

Emilia and her family come from a small village near Lviv, close to the Polish border. She's got an older brother, Markian. She was born in 2021 with a very rare genetic disease called severe combined immunodeficiency (SCID, "the Bubble Boy disease"). This is a very serious type of primary immunodeficiency that most often requires a bone marrow transplant (BMT) in the early months of life.

After being diagnosed with SCID, Emilia was also waiting for BMT, but in the first days of war, the hospital in Kyiv where the procedure was to be performed was bombed. Moreover, the warfare made it impossible to get to Kyiv.

Everyone was frantically looking for another solution. On the third day of the war, we received a call from Emilia's parents. They got our number from doctors from Lviv. It was the very beginning of the war - there were no solutions, procedures, humanitarian corridors, etc. But we knew we had to act.



Thanks to our efforts, after a couple of days, Emilia came to Poland together with her whole family. She was taken care of by doctors in the pediatric department of a university hospital in Bydgoszcz. From the very beginning, she was given exceptional care there. After some necessary tests, they began preparing the transplant procedure for her.

Emilia underwent a successful transplant a couple months ago. Since then, her immune system has started working properly. She feels fine and is recovering surrounded by family in Bydgoszcz.

On-site support for Ukrainian patients and the medical community



- We are in constant contact with hospitals treating patients with rare diseases in Ukraine and we determine their needs for medicines and medical equipment.
- In response to needs, we organize the transportation of medicines and medical equipment to specific hospitals.
- Many of these shipments contain specialized products or equipment needed to treat specific rare diseases, e.g. primary immunodeficiencies, spina bifida, or cystic fibrosis.
- If the situation requires it, we finance the purchase of medicines on the spot.

So far, we have organized 20 shipments of medicines, medical equipment, and other necessities

The total value of medicines and medical equipment that we shipped to Ukraine or bought on site so far is over \$45,000.

On-site support for Ukrainian patients and the medical community

In order to adjust our activities to the current needs of patients and the medical community in Ukraine, we are in touch with doctors from many medical centers treating patients with rare diseases, e.g. in Ivano-Frankivsk, Kyiv, Lviv, Poltava, Ternopil, Vinnitsa, and Zaliztsi.

When it comes to OTC drugs, medical equipment, and personal protective equipment, we organize the purchase and transportation on our own.

We pass on the requests for specialized medicines to entities that are authorized to transport them abroad, such as the Polish government, the Polish Medical Mission, or the Red Cross, **or we finance the purchases of specialized medicines on the spot.**



Map of hospitals in Ukraine for which we have purchased medicines or medical equipment

Immunoglobulins for Ukrainian patients with primary immunodeficiencies

Our foundation provided immunoglobulins to the Children's Clinical Hospital in Poltava to secure the needs of Ukrainian patients with primary immunodeficiencies.

Due to legal regulations, not all medicines can be transported across the border and delivered to hospitals in Ukraine, even as part of humanitarian aid. But it doesn't stop us.

Thanks to cooperation with the Ukrainian producer of plasma-derived medicines, we were able to purchase immunoglobulins needed for the treatment of patients with primary immunodeficiencies. The medicines were delivered directly to the hospital by the manufacturer. Buying medicines on the spot is also an opportunity to support the Ukrainian economy, which is struggling due to the effects of the ongoing war.



"In such difficult days for our country, there are people who will come to our aid. Infinitely grateful to Adrian Goretzki, Bernadeta Prandzioch for the help provided in the form of expensive medicines for children with immune system disorders."

"Poltava Regional Children's Clinical Hospital of the Poltava Regional Council", 05.07.2022

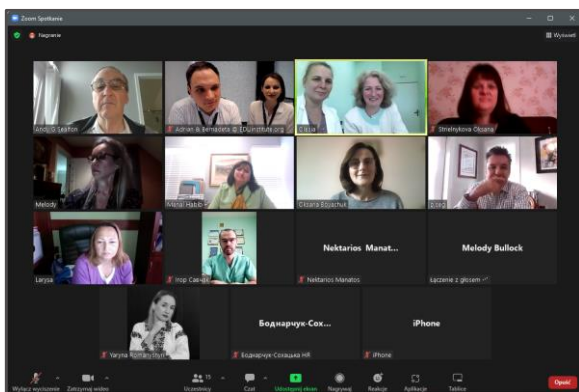
"Pumps for Ukraine" project

Our assistance to patients staying in Ukraine includes not only emergency action, such as the purchase of medicines, but also more long-term projects, such as our **"Pumps for Ukraine"** project.

Currently, there is a lack of infusion pumps in Ukraine, but thanks to the support of our donors, patients with primary immunodeficiencies from Ukraine receive infusion pumps and medical equipment that allow them to administer immunoglobulins at home. The infusion pumps are also used in hospital departments.

So far, infusion pumps and medical equipment have been delivered to hospitals in Lviv, Ternopil, Kiyv, Vinnitsa, and Ivano-Frankivsk. Physicians from these hospitals participated in a special online training sessions on the operation of these pumps so that they could also train their patients.

Further deliveries of pumps are planned in the beginning of 2023.



Training for physicians



Patients in Ukraine are already using infusion pumps

Information and legal support for patients

- We run websites with information for RD patients:

www.ridkisnikhvoroby.pl

www.imunodefitsyt.pl

- We prepare legal opinions regarding access to therapy for Ukrainian patients with chronic diseases and staying in Poland.
- We create educational materials such as information brochures and roadmaps for patients from Ukraine.
- We organize translations of medical documents from Ukrainian to Polish or English.
- We support patients in legal matters related to their stay in Poland (obtaining a PESEL number, refugee status, etc.) or obtaining social benefits.
- We contact the appropriate offices in Poland to receive necessary information and answer questions from patient.



A guide for patients from Ukraine about the healthcare system in Poland

We have 6 translators on 2 continents who translate patients' medical records to ensure they receive appropriate medical care.

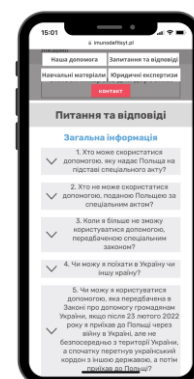
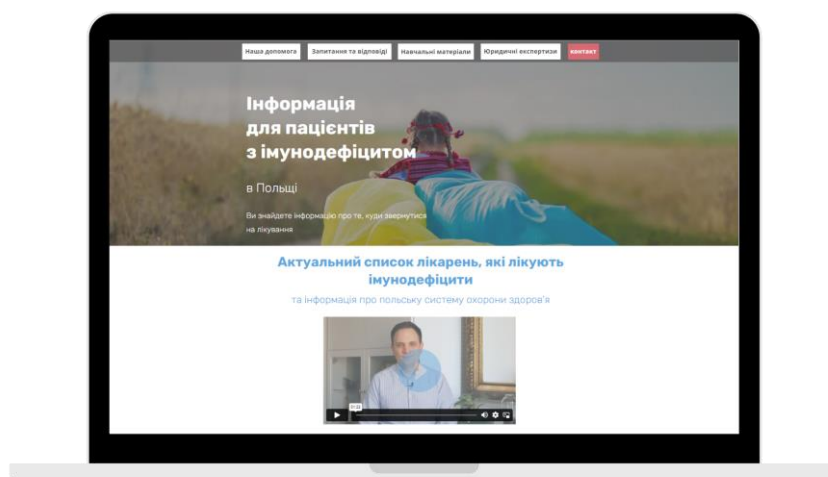
Information and legal support for patients

1. Websites with information for RD patients

In the first weeks of the war, we prepared two websites: one for patients with rare diseases and the other for patients with primary immunodeficiencies. On the websites you can find:

- Contact details for hospitals treating primary immunodeficiencies
- Contact details for Polish verified rare disease patient organizations
- **A frequently updated comprehensive FAQ section with answers to almost 40 questions** regarding access to therapy for Ukrainian patients with chronic diseases in Poland, social assistance, and work and education in Poland
- **Legal opinions in Polish and Ukrainian** indicating that Ukrainian refugees have access to free-of-charge medical care in almost all cases, including therapies for rare diseases.

You can visit our websites by clicking on the pictures!



Information and legal support for patients

2. Brochures

We prepared three printed brochures for patients which are available free of charge at hospital wards where rare disease patients from Ukraine are treated.

- **The first one** presents the healthcare system in Poland from the perspective of RD patients
- **The second** is a Ukrainian-Polish medical dictionary for patients with primary immunodeficiencies. It contains over a hundred of the most important medical terms related to PID and often used in a doctor's office or medical records
- **The latest** brochure is about the social benefits available in Poland for RD patients or caregivers of RD patients who, as a result of the war caused by Russia, were forced to flee from Ukraine to Poland.



click on the covers
to download brochures

Every patient has a unique story (2)

Meet Masha (Маша)!

Masha is a 2-year-old girl from a small village in the Zaporizhzhya region. She's got an older brother, Vanya. She has a **genetic syndrome, Nijmegen syndrome, which is manifested in immunodeficiency, a very high risk of tumors and microcephaly.**

From the moment of birth, she was very small, which drew the attention of doctors, but they couldn't establish a diagnosis for quite some time. The final diagnosis was made after the war broke out.

Until February 24, the life of her whole family was normal. Her mom worked at a school, her dad worked as a welder, and her brother was in kindergarten. She was under the care of her grandparents.

Her mom told us: "When the war started, everything changed. **I woke up in the morning because I heard something flying over the house,** but since Melitopol is not far from us, I thought it was just a plane flying to the airfield. My mother lives in Melitopol. I saw a message from her at five



in the morning "call me" and then I immediately realized that it was something wrong. I called her and she was crying: «Anya, the war has started».

Masha's parents went into the backyard and at the same moment they spotted four rockets overhead, so close that they could see all the details. **They packed and decided to leave their village. Three days later, Russian soldiers entered the village.**

In the new place, they went to the bomb shelter during every air raid; whenever it was, day, night, at any hour. But after some time, Masha began to run a fever. The family thought that it may be because of

Every patient has a unique story (2)

the conditions in the bomb shelter. So, they stopped going to the shelter, tried to stay in safer areas in the apartment during the air raids and she recovered. And after 2-3 months they decided to go to the hospital again in order to somehow track her developments.

An immunologist at the Zaporizhzhia Regional Children's Hospital advised them to go to Lviv, to the Western Ukrainian Specialized Children's Medical Center, where physicians helped them to learn about the disease. But it still wasn't enough, and the conditions were uncertain, as they still lived in Zaporizhzhia region, close to the frontline. **So, they decided to leave Ukraine and go to Poland.**

My mother lives in Melitopol. I saw a message from her at five in the morning "call me" and then I immediately realized that it was something wrong.

With our help, Masha was admitted to a hospital in Wroclaw, had a full examination in the immunology department and consultations with a cardiologist.



For now, Masha feels good and she's under the care of the immunologists. But – as per their advice – she cannot go to the kindergarten yet because of her immunodeficiency. She lacks this connection with peers, but it's for her own good. She has started to get sick a little more often now than before. A common cold can last for 2-3 weeks, so it is a little more difficult for her to get over these common infections than other children.

The whole family is waiting for the Ukrainian victory and the liberation of their territory, as they want to return home to Masha's grandparents and relatives who stayed behind. But, first and foremost, they want to make sure that Masha has the best possible care in Poland.

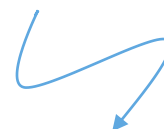
"The situation of patients with RD from Ukraine who fled the war" – a report

In order to understand their situation even better and adjust our humanitarian activities to their needs, in July 2022, we conducted survey research in a group of about 30 Ukrainian patients and patient caregivers whom we helped and who are currently residing in various European countries. The report which we have prepared on the basis of the collected responses is available in Ukrainian and English.

The respondents were asked about such subjects as:

- the period of stay outside Ukraine
- received medical assistance related to their illness
- financial support received by refugees
- greatest difficulties faced abroad
- current housing and work situation
- plans for the future

*Click to read
the entire report*

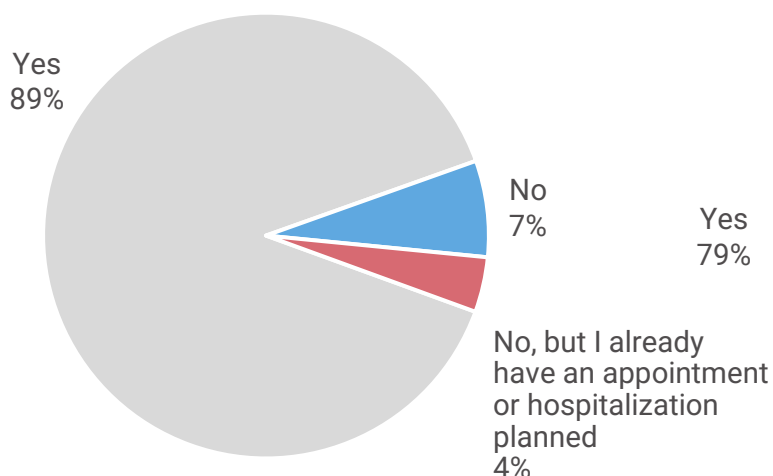


"The situation of patients with RD from Ukraine who fled the war" – a report

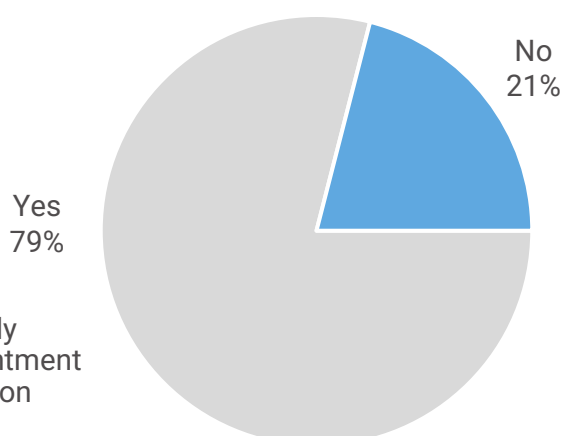
The most important conclusions from the research:

- The vast majority of respondents have received medical assistance in the country where they're currently staying (89%) which means that **the countries receiving people fleeing the war in Ukraine are effectively responding to their needs in the field of medical care**. Even people who, due to their illnesses, **need highly specialized and costly medical procedures**, such as surgeries or even transplantations, are guaranteed them in the countries they came to.
- As many as 61% of respondents indicated that **one of the greatest difficulties related to their disease that they had to face after leaving Ukraine was the language barrier in contact with physicians**. Therefore, it seems right to create educational materials in Ukrainian and to undertake other initiatives aimed at facilitating communication between physicians and patients.
- Almost 80% of respondents indicated that they had received financial assistance in the country where they're currently staying, but in the majority, it was only a one-time financial assistance for refugees. **It seems necessary to think about more long-term forms of support for refugees with rare diseases in the countries that host them.**

Receiving medical assistance while remaining outside Ukraine



Receiving official financial assistance in the country where a patient is staying



"Brave RARE Ukraine" is coming soon

For nine months, since helping patients with rare diseases from Ukraine became the main part of our foundation's activity, we have heard countless heartbreaking stories. We feel that each of these patients and each of these families are true warriors. Heroes no one has heard of. And we would like to change that.

Patients with rare diseases often remain in the shadows. Their stories rarely break through to the public. And it's no different during war. That's why we came up with "Brave RARE Ukraine", a project in which we will tell the stories of those who, amidst the cruelty of war, still must struggle with their diseases and the challenges they pose.

We want to give a voice to patients, their families, doctors from Ukraine and representatives of patient organizations. We want to create a website where we will share their stories in the form of interviews, videos and podcasts.

*Our foundation's team and
Brave RARE patients during
the recordings*

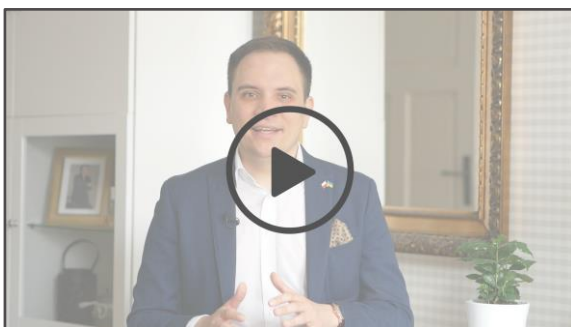


Raising awareness about the situation of RD patients from Ukraine

We appear in front of an international audience to raise awareness about the situation of RD patients from Ukraine

- We participated in the meeting of the network of Parliamentary Advocates for Rare Disease of European Parliament (23.03.2022)
- At the invitation of GlobalGenes, we participated in the *RareCast* podcast hosted by Daniel Levine (May 2022)
- We took part in an online conference for Ukrainian physicians called "Рідкісні захворювання у дітей" (Children with Rare Diseases) (10.06.2022)
- We have prepared a video presentation for participants of the BIO International Convention (San Diego, June 2022)
- We spoke at the 11th European Conference on Rare Diseases (28.06.2022)
- We spoke about Ukraine during the Plasma Protein Forum (Washington, 11-12.10.2022)

On December 9, during **the WORLD FOR UKRAINE SUMMIT**, together with participants from all over the world, we discussed better humanitarian, social and economic solutions to help Ukraine.



Click to listen to
the podcast

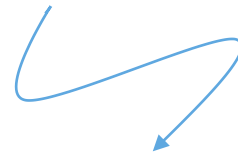
Cooperation with other entities

We also make sure that we are up to date with all initiatives related to helping patients with rare diseases and participate in meetings with other organizations and decision-makers.

- We are in touch with patient organizations supporting RD patients at the national and international level.
- We participate in meetings with RD umbrella organizations.
- We meet with representatives of international and domestic humanitarian organizations.
- We also participated in the meeting of the network of Parliamentary Advocates for Rare Disease of European Parliament (23.03.2022). As a result of the meeting, an appeal was made to the President of the European Commission on the necessity of supporting patients with rare diseases from Ukraine and possible methods of action.
- We closely cooperate with EURORDIS as a part of the "Razem z Ukrainą" project.



Meeting of representatives of organizations cooperating under the project "Razem z Ukrainą" in Warsaw



Representatives of EURORDIS, Singapore Red Cross, Debra International, Rare Diseases Ukraine and EDUInstitute.org in Warsaw



Every patient has a unique story (3)

Say hello to Nastya (Настя)!

She's only seven. She's from Donbas, from Lysychansk in the Luhansk region. She's got an autoimmune disease; in fact, a couple of different diseases: a severe form of overlap syndrome, autoimmune hepatitis, ulcerative colitis and fibrillating cholangitis.

When she was nearly two, she ran a very high temperature and was admitted to the regional hospital. But they couldn't provide a proper diagnosis there. Antibiotics didn't help, so the doctors decided to send her to Kyiv, to the Okhmatdyt hospital, where she was diagnosed and started treatment.

Before the war, the family's entire life revolved around Nastya's disease. She had a very strict diet, she had to have meals regularly, she took her medications at certain times and every three months she had to go to the hospital in Kyiv.

On February 24, the family remembers very clearly that they were woken by explosions, turned on the TV and the news was just horrible - there were bombings all over the country. They began to bring the most



important things down to the basement: documents, medical records, and warm clothes.

And so, their basement life began. Between the airstrikes, Nastya's mom would go back up to the house, try to cook something, but when the explosions started, she had to quickly turn off the gas and go back downstairs. Every trip to the store was a challenge. The stores opened less and less often, and people had to stand in line for hours.

Nastya should've had her normal diet, but it was impossible to keep. She was getting worse - she had lost a lot of weight and had become very pale. Moreover, the medicine was running out. That's when the family decided to leave. But leaving Lysychansk was more difficult than they had anticipated.

Every patient has a unique story (3)

They were shot at constantly. **The trip was terrible. They were crying, shouting, praying and fighting to stay alive.** Finally, they managed to reach Kramatorsk and went to Lviv by train. And volunteers put them on the bus to Poland.

All of their possessions, consisting of some valuables, a doll, a backpack full of medicines and another backpack with food, were confiscated. That's all they had taken with them. That's how they had left.

Nastya's family was brought to the refugee camp in Przybysławice. They were warmly welcomed there. But Nastya was sick. She had stopped eating, had lost even more weight, and her test results were very bad. Nastya's mom started writing posts on the Internet, calling all the hotlines she could find, asking around for help and that's how she got in touch with our foundation. With a little help from us, Nastya got to the right specialists.

She's now under professional care and taking immunosuppressive therapy. She

"It's very difficult when you had everything, when you lived in your country and then everything changes - you lose everything, you become nobody."

can't go to school but is homeschooled instead.

They plan to learn the language first, so as to learn more about the country that welcomed them. **Everything that they had in Lysychansk has been destroyed, so they have nothing to return to.** That's why they decided to stay in Poland. "It's very difficult when you had everything, when you lived in your country and then everything changes - you lose everything, you become nobody", Nastya's mom said. But they're determined to start their life once again here, in Poland.



Media about our actions



"Patient advocate and lawyer Adrian Goretzki, founder and president of the Healthcare Education Institute in Poland, has also helped families of rare-disease patients cross the border and arrange needed medical care.

His organization assists patients to get them quickly across the border, organizes medical care in Poland, translates medical documents from Ukrainian to Polish or English, and buys medicines and medical equipment for hospitals in Ukraine."

Erin Durkin, "National Journal", Washington DC, 4.04.2022

TVP, „Zbliżenia”, 15.03.2022

– Emilia and her mother in the hospital in
Bydgoszcz



"Extraordinary times call for extraordinary efforts. With an impactful early life of advocacy behind him, when the unimaginable happened in Ukraine, Adrian Goretzki was not only in the right place at the right time but had the necessary skills to play his part in stemming a humanitarian crisis for the PI community in the region.

This makes Adrian our RARE Inspiration."

Nicola Miller, "RARE Revolution Magazine",
Autumn 2022

Click to read
the full article

Media about our actions

Click to read
the full article

"The Source. Winter 2022", Magazine of the PPTA



SIX MONTHS OF SUPPORT: HUMANITARIAN AID FOR UKRAINIAN RARE DISEASE PATIENTS

BY ALEXA WETZEL, PPTA DIRECTOR, LEAD FOR EUROPEAN PLASMA
ADRIAN GORETZKI, FOUNDER & PRESIDENT, EDUINSTITUTE.ORG FOUNDATION
BERNADETA PRANDZIOCH-GORETZKI, VICE PRESIDENT, EDUINSTITUTE.ORG FOUNDATION

Adrian Goretzki, a very active patient advocate and primary immunodeficiency (PID) patient himself, together with his wife Bernadeta Prandzioch-Goretzki, a psychologist experienced in working with rare disease patients, started an incredibly challenging undertaking right after the war in Ukraine broke out. They decided to provide support to Ukrainian rare disease patients. PPTA met with Adrian and his wife to talk about their humanitarian activities.

Can you tell us how you help Ukrainian rare disease patients?

The war in Ukraine is an unprecedented event in recent history. It is a war in which there are no rules and in which civilian objects, homes, kindergartens, and hospitals are bombed and shelled. A war in which the victims are innocent Ukrainians civilians, mothers, children, and the sick. A war that has already forced almost 8 million people to flee the country. Among them are also patients with rare diseases. Not to forget, the patients with rare diseases who stayed in Ukraine, uncertain

of whether they will be able to escape and whether anyone will support them in their evacuations and secure their treatment. Since the beginning of the war, we have been doing our best to prevent Ukrainian patients with rare diseases from feeling abandoned and left to deal with their difficulties alone. We support them in many ways. On one hand, we help them flee the war, and on the other hand, we provide them with treatment in Poland and other European countries. But we also offer legal assistance, provide information, and, finally, we arrange on the support for Ukrainian patients and the medical community. What started as an ad hoc project, forced by the situation, has turned into a well thought out support system with a dedicated team responsible for specific actions.

When we speak about rare diseases, how many people are affected in Ukraine?

As a whole, 80% of rare diseases are of genetic origin and are often chronic and life-threatening. In Ukraine, there are between 1.5 and 2.5 million people with rare diseases. Some of them suffer from more common and well-known rare diseases,

such as hemophilia or primary immunodeficiencies, while others have conditions that affect only a few persons in Ukraine.

Today, how are you organized and what actions are taken to support patients?

Our work is currently coordinated based on four main areas of activity:

1. First of all, we help patients fleeing the war. We provide patients with support at every stage of their journey to Poland or transit to other European countries, including faster transport across the border, organizing accommodation, and providing access to treatment.
2. We also provide support for Ukrainian patients and the medical community in Ukraine by organizing the transport of medicines and medical equipment to hospitals that treat patients with rare diseases, finance the purchase of medicines, and provide information and legal guidance to patients.
3. We have created dedicated websites with information for rare disease patients, legal opinions, printable materials, evaluations of medical records, and support for legal matters related to staying in the EU.
4. And most important, we raise awareness about the situation of Ukrainian rare disease patients. We speak at industry conferences and stakeholder meetings, give interviews, and take part in podcasts to draw attention to the needs of rare disease patients in Ukraine and to encourage others to act.

How many families are currently being cared for?

In the past six months, we have been able to help more than 70 families of patients with rare diseases, including primary immunodeficiencies, epidermolysis bullosa, and rare cases such as KIF1A-associated neurological disorders (KANED).

For patients with serious conditions, we make sure to create a fast track so they do not have to wait in long lines at the border. Especially in the first weeks of the war, the long lines required several days of waiting, so it was crucial to speed up the process for them.

Even though most of the patients we helped are children, we have also assisted more than 30 adults, most often with very serious forms of their diseases, that require advanced treatment outside Ukraine, including transplant operations.

The patients we have helped to cross the border and receive treatment in Poland were often affected by very rare diseases that required a specialized approach. That is why our activities are always focused on providing adequate medical care in a specialized medical center as soon as possible.

How did you proceed?

When Poland was the final destination, we first provided patients with support in finding accommodation, and completing the necessary formalities to obtain refugee status and a PESEL number (Polish acronym for "Unified Electronic System for Registration of the Population"). Then we provided them with appropriate medical care, making appointments with specialist physicians or providing hospitalization in medical centers if their health conditions required it.

8 THE SOURCE | WINTER 2022

WINTER 2022 | THE SOURCE 9



EVERY PATIENT STORY IS ONE-OF-A-KIND

"THE PATIENTS WE HAVE HELPED TO CROSS THE BORDER AND RECEIVE TREATMENT IN POLAND WERE OFTEN AFFECTED BY VERY RARE DISEASES THAT REQUIRED A SPECIALIZED APPROACH. THAT IS WHY OUR ACTIVITIES ARE ALWAYS FOCUSED ON PROVIDING ADEQUATE MEDICAL CARE IN A SPECIALIZED MEDICAL CENTER AS SOON AS POSSIBLE."

When patients decided to travel to other countries, we worked with patient organizations operating in that given country, obtained information on which medical center they should go to, or contacted doctors directly. In some cases, patients first stayed in Poland, where they received appropriate treatment, and then decided to continue their journey to another country.

How is the situation in Ukraine now?

We are in constant contact with hospitals in Ukraine that treat patients with rare diseases to inform us about their urgency for medicines and medical equipment.

According to patient needs, we organize the transport of medicines and medical equipment to specific hospitals. Many of these shipments contain specialized products and equipment needed to treat very specific rare diseases, such as primary immunodeficiencies, spinal cord, or cystic fibrosis.

If the situation requires it, we coordinate and finance the purchase of medicines on the ground. So far, we have organized 20 shipments of medicines, medical equipment, and other necessities.

The help and assistance we provide to patients in Ukraine does not only include emergency care but also more long-term projects. In the coming months, with the generous support of our partners, patients with primary immunodeficiencies will receive infusion pumps that will allow them to administer their immunoglobulins at home. These pumps will also be provided to hospital departments. The biggest advantage is that these mechanical pumps, which do not require electrical power, can be used for both intravenous and subcutaneous infusions.

Hospitals in Lviv, Ternopil, Kyiv, Vinnytsia, and Dnipro have already received the pumps. Physicians from these hospitals participated in a special online training on how to operate them so that they can explain it to their patients accordingly. We all medicines can be transported across the border to be delivered to Ukrainian hospitals, even as part of humanitarian aid. We have been confronted by some legal challenges, but this does not stop us. Thanks to the cooperation of a Ukrainian producer of plasma-derived medicines, we were eventually able to purchase immunoglobulins locally, which the manufacturer then delivered directly to the child's hospital in Poltava by the manufacturer. Buying medicines on the ground is also an opportunity to support the Ukrainian economy, which is, of course, struggling due to the effects of the ongoing war.

How do you communicate and provide information to patients?

We have two websites that provide information to rare disease patients: [www.eduinstitute.org](https://eduinstitute.org) (for rare diseases in general) and www.immunodeficiency.org (for patients with PID). For patients with chronic diseases who are in Poland, we have prepared legal opinions regarding access to therapy, created printed materials for patients and physicians, and organized translations of medical documents.

We also provide support for the bureaucratic steps related to their stay in Poland. We help connect the patient and physician in Poland to receive necessary information and answer questions from patients.

Do you have support from other patient organizations?

We exchange information about all ongoing initiatives related to rare disease patients and participate in meetings with other organizations, representatives from humanitarian organizations, and decision-makers on the national and international level. We were able to closely collaborate with EURORDIS as a part of the *Rareness Uniting* (Together with Ukraine) project.

In March, we participated in the European Parliament meeting of the network of Parliamentary Advocates for Rare Disease. As a result, an appeal was made to the president of the European Commission on the necessity of supporting patients with rare diseases from Ukraine and what possible actions can be taken.

If our readers would like to support you, what can they do?

Unfortunately, this war is not coming to an end and the needs are growing. We expect the next wave of rare disease refugees as the winter is approaching.

We encourage the readers to support our efforts helping Ukrainian patients with rare diseases. Please contact us for more details at adrian@eduinstitute.org or +486010363022. There's also a page for individual donations at donations.eduinstitute.org.

More information about the foundation's general activity can be found on our website: eduinstitute.org. We have an international team of dedicated staff and volunteers working together to ensure patients' safety and access to treatment.



Emilia and her parents come from near Lviv, Ukraine. She was born with a rare genetic disease called severe combined immunodeficiency (SCID "the Bubble Boy disease"). This is a serious type of primary immunodeficiency that most often requires a bone marrow transplant in the early months of life. But in the first days of the war while Emilia was also waiting for her transplant, the hospital in Kyiv, where the procedure was to be performed, was bombed. Thanks to our efforts, she came to Poland with her parents and was taken care of by doctors in a pediatric hospital in Bydgoszcz. Emilia has undergone a successful transplant earlier this year. She feels better and is recovering surrounded by family.

10 THE SOURCE | WINTER 2022

WINTER 2022 | THE SOURCE 11

Physicians about our actions



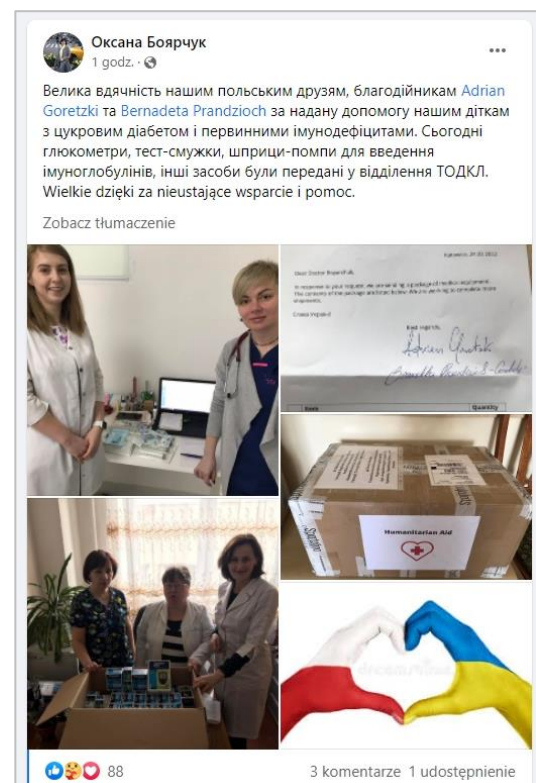
"The war has changed everyone's life! Yet, we are all fighting for the better life! **Some people defend us on the frontline, while others provide us with the necessary aid regarding health issues.** What is really important for us is the support we get from people who deeply care about the Ukrainians. There are so many kind people all around!

We, on behalf of the staff and patients, especially little ones, would like to express our sincere emotions and say "Thanks" to all the great people who have sent such a significant support to us. Your understanding and kindness deserve the best praise."

Dr Veronika Kulchinska, MD, PhD,
hospital in Zaliztsi, 6.04.2022

"Many thanks to our Polish friends, philanthropists **Adrian Goretzki and Bernadeta Prandzioch**, for their help to our children with **diabetes and primary immunodeficiencies.** Today, glucometers, test strips, syringes for the administration of immunoglobulins, other medical equipment were transferred to the department of TODCL. Many thanks for the constant support and help."

Prof. Oksana Boyarchuk, MD, PhD,
hospital in Ternopil, 11.04.2022



NGOs and hospitals about our actions



"Children with spina bifida in the Ternopil region received care products and medicines. We express our sincere gratitude to Oksana Boyarchuk for her constant support and attention to children and to benefactors Bernadeta Prandzioch and Adrian Goretzki for helping the children of Ukraine in these difficult times."

NGO "Union of parents of children with Spina bifida and Hydrocephalus"
"Сяйво Духу", 15.08.2022

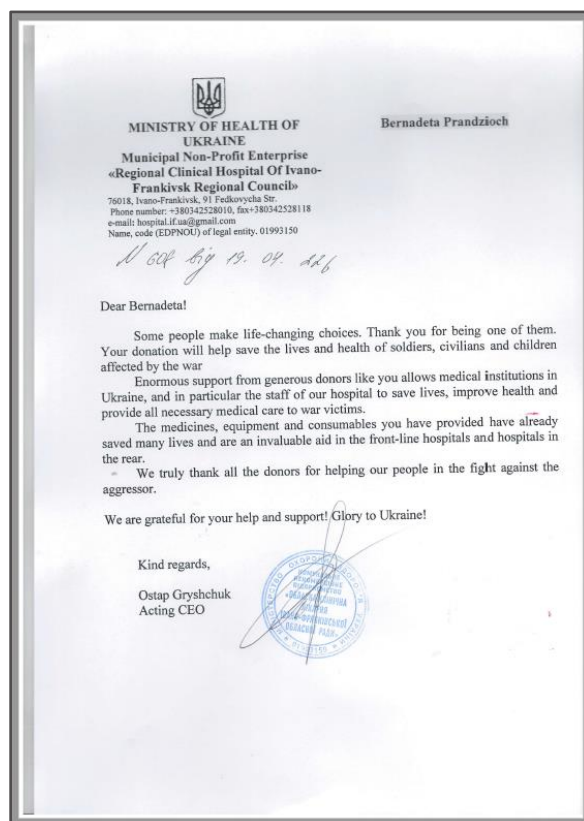
"The administration and the entire team of the Ternopil Regional Children's Clinical Hospital, TOR, express their sincere **thanks to our benefactors Healthcare Education Institute from Katowice, Poland** (...) for the charitable assistance provided to the children who are being treated at our medical institution.

We sincerely thank you for your support and caring attitude during a difficult time for Ukraine. May your kindness and generosity return to you with the warmth of children's hearts."

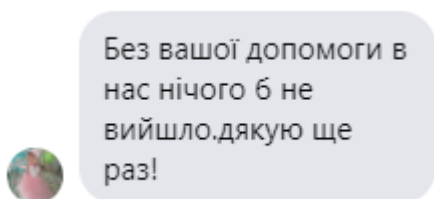
Ternopil Regional Children's Clinical Hospital,
10.08.2022



Hearing from hospitals and patients about our actions



Letter from Ostap Gryshchuk, CEO of the hospital in Ivano-Frankivsk with thanks for the provided medical equipment (19.04.2022)



Without your help, we would not have succeeded. Thank you again!



Доброго дня , дорогий Адріане! Все ніяк не напишу Вам ...хотіла подякувати за допомогу, за підтримку, за мого синочка...ми тоді прокапались і навіть здали кілька аналізів...Господи, які у вас люди...! Як рідні! Хотілось і руки цілувати вашим лікарям та медсестрам ... Спасибі Вам за таке відношення до українських дітей!

11:06

Good afternoon, dear Adrian! It is impossible to write everything... I wanted to thank you for your help, for your support for my son... (...) My God, what kind of people you are...! Like family. I also wanted to kiss the hands of your doctors and nurses... Thank you for this approach towards Ukrainian children.

RARE Champion of Hope 2022

Global Genes, an American non-profit organization dedicated to creating a globally connected community committed to meeting the challenges of rare diseases, has been awarding the RARE Champions of Hope since 2012, through which it honors and recognizes true champions of rare diseases.

This year, at the 2022 Rare Disease Patient Advocacy Summit in San Diego, CA, **Adrian Goretzki received the title of RARE Champion of Hope** for “a lifetime of dedication to helping get treatment to those in need and **especially for a work in helping patients cross the Polish/Ukrainian border in 2022, ensuring access to therapy and other medical care.**”



The invaluable help of our Ukrainian-speaking project manager

Thanks to the financial support of one of our partners, we were able to hire a new employee, **Anastasiia Doroshenko**, a physician specialized in pediatrics. She has extensive experience working with children in various environments and with various conditions, including rare diseases.

As the project manager at the Foundation, **she is responsible for activities related to the comprehensive support of rare diseases patients from Ukraine fleeing war**: from support during their journeys and arranging accommodation to ensuring access to medical care and helping with formal matters.

She also coordinates humanitarian aid for Ukrainian hospitals treating rare disease patients.



Anastasiia Doroshenko, MD

Project Manager, Ukraine response

"Working with people has always been very close and important to me. It's especially valuable to help patients with rare diseases from Ukraine, because they need special attention in peacetime, even more so in such a difficult time for our country."

I'm very proud to be part of a team that does everything possible to help patients from Ukraine overcome difficulties and not feel alone with their problems."

Cooperation and Support

Industry Donors



Non-profit organizations



You can also become a partner and support our activities securing the lives of patients from Ukraine – contact us!

About the Foundation

The Healthcare Education Institute was established in 2017 on the initiative of Adrian Goretzki, someone with XLA and a patient advocate with over 10 years of experience in working for patient communities. In our daily activities, we strengthen the voice of patient communities, educate patient group leaders, create reports, and conduct research and studies, all in order to ensure that patients with rare diseases are noticed and receive the best possible medical care.

Since the war broke out, we do everything to support RD patients from Ukraine. We now have an international team of dedicated volunteers from the USA to Ukraine working together to ensure patients' safety and access to treatment.

You can read more about our everyday work on: eduinstitute.org.

Board of Directors



Adrian Goretzki, LL.M.
Founder, President of the Foundation

From 2010 to 2018, served as leader of the Polish Association for Patients with Primary Immunodeficiencies 'Immunoprotect' and was a former member of the executive board of the international umbrella association, the International Patient

Organization for Primary Immunodeficiencies (IPOPI). **For his successful patient advocacy, Goretzki was given the Luciano Vassali Award in 2014, the University of Silesia Rector's Award in 2020 and RARE Champion of Hope in 2022.** In 2021, he was shortlisted for the EURORDIS Patient Advocacy Award in 2021. Professional lawyer, advisor, and keynote speaker. Fascinated with opportunities given by new technologies in the field of education.



Bernadeta Prandzioch-Goretzki, M.Psy, B.Ed
Vice-President of the Foundation

Psychologist experienced in working with patients with rare diseases, active in this area since 2014. At the Institute, she is responsible for the scientific part of our job, focusing on coordinating studies and reports. Researching the area of quality

of life in patients with rare diseases. Author of the handbook for patients with primary immunodeficiencies, *The Fullness of Life*. **Successful TEDx speaker (nearly 400k views on YouTube)**, author of many scientific publications and articles popularizing psychological knowledge, and participant in international conferences.

Support us and join those for whom rare means exceptional, not unimportant.

You can support our actions and donate via PayPal.

Just use the QR code on the right or click [HERE](#).



You can also donate directly to our bank accounts by entering *"Donation to help rare disease patients from Ukraine"* in the title of the transfer.

Details:

Healthcare Education Institute. The Foundation,
Gliwicka 74/4, 40-854 Katowice, Poland

SWIFT/BIC: PPABPLPK (BNP Paribas)

- PL (donations in PLN): PL45 1600 1055 1830 5274 2000 0001
- INT (donations in EUR): PL34 1600 1055 1830 5274 2000 0005
- INT (donations in USD): PL07 1600 1055 1830 5274 2000 0006

You can also become our Industry Donor or Business Partner.

If you or your company would like to make a corporate donation and it requires a contract, please let us know. We are a **non-profit & VAT-exempted foundation** based in Poland (a legal equivalent of US 501(c)(3) organization status).

If you have any questions or would like to support our activities in different way, please contact us:

Healthcare Education Institute. The Foundation

✉ office@eduinstitute.org

☎ +48 609 363 932

