LEGAL TOOLKIT
FOR PATIENT ADVOCATES
Report on the patients' participation in decision-making and law-making processes
EDU
Boehringer Ingelheim

2024
ANNUAL REPORT

REVIEW OF ACTIVITIES AND ACHIEVEMENTS OF THE HEALTHCARE EDUCATION INSTITUTE

Report prepared by:

Healthcare Education Institute
The Foundation

Healthcare Education Institute. The Foundation
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Photos:

The following photos were used in the report:

- cover, p. 13, 14, 27 – photos taken during The Access to Care Conference 2024 in Vienna / **Boehringer Ingelheim;**
- p. 19, 25 – photo taken during an educational meeting for patients with LAL-D disease, 20.07.2024, Warsaw, Poland / **Photo author: Karolina Waligóra, Compass Public Relations**;
- p. 25 – photos taken during EURORDIS' #ECRD2024 / **EURORDIS - Rare Diseases Europe;**
- p. 26 - photo taken during The EFPIA CEE Patient Engagement Forum, 26-27.09.2024, Warsaw, Poland / **Photo provided by Oana Scarlatescu, EFPIA** .

All other photos are from the collection of the Healthcare Education Institute.

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Letter from the President

The year 2024 at the Healthcare Education Institute was marked by the completion of major projects: the results of a study on the quality of life of Polish patients with Fabry disease were published, and the "Legal Toolkit for Patient Advocates", a report on patients' participation in decision-making and law-making processes, was launched. We also kept up our efforts in patient education.

The quality of life study we conducted is an important step in the discussion on shaping medical care for Fabry disease patients in Poland. We hope it will help improve their situation and better address their needs.

Meanwhile, the "Legal Toolkit for Patient Advocates" - and I truly believe this - is a genuinely innovative perspective on how patient advocates can fight for patients' rights in their countries and make use of existing legal frameworks, sometimes in completely unconventional ways.

This year, we also continued our journey with PatientAdvocacy.Academy. Every edition brings us great joy, knowing that there are patient advocates around the world eager to learn, improve their skills, and share their experiences. This year's edition was truly a success.

We also didn't forget about rare disease patients from Ukraine - we continued providing humanitarian aid by delivering medical equipment.

I encourage you to check out the report, and I want to extend my heartfelt thanks to everyone who has supported our efforts!

ADRIAN GORETZKI
PRESIDENT OF THE FOUNDATION

About the Healthcare Education Institute

The Healthcare Education Institute, founded in Poland in 2017 by Adrian Goretzki, a rare disease advocate and patient, is a non-profit organization dedicated to improving the lives of people with rare diseases. Our mission is to provide meaningful support to patient communities and those working to advance their care.

Rare disease patients often face unique challenges, including delayed diagnoses, limited treatment options, and low public awareness.

We work to amplify the voices of these communities by educating patient leaders, conducting research, publishing reports, and advocating for systemic change. Our efforts aim to ensure rare disease patients are acknowledged and provided with the medical care they deserve.

Through collaboration with stakeholders, we work to reduce diagnostic delays, enhance treatment access, and adapt healthcare systems to meet the needs of rare disease patients. Together, we aim to foster a healthcare system that truly supports those affected by rare conditions.

Board of Directors

Adrian Goretzki, LL.M.

Founder, President of the Foundation

From 2010 to 2018, he served as leader of the Polish Association for Patients with Primary Immunodeficiencies 'Immunoprotect' and was a 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, RARE Champion of Hope in 2022 and the EURORDIS Black Pearl Award in 2023. Professional lawyer, advisor, and 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 (400k views on YouTube), author of many scientific publications and articles popularizing psychological knowledge, and participant in international conferences.

Executive Staff

Kamila Rzepka, LL.M.

Senior Project Manager

A lawyer by profession with experience in working with patient groups. In 2014-2018 she worked as an project manager in the Polish Association for Patients with Primary Immunodeficiencies "Immunoprotect".

At the Institute, she is responsible for coordinating ongoing projects and cooperation with patient organizations, mainly in matters related to reimbursement, access to treatment and the fight for patients' rights.

Medical Advisory Board

Our priority is to align the foundation's activities with the needs of the medical community and patients. To ensure this, we have a Medical Council consisting of trusted experts we have collaborated with for years.

Prof. Sylwia Kołtan, MD, PhD

Polish National Consultant in the field of clinical immunology, works in the Clinic of Pediatrics, Hematology and Oncology, University Hospital No. 1 in Bydgoszcz; Department of Paediatrics, Hematology and Oncology. Professor of Collegium Medicum of the Nicolaus Copernicus University in Toruń. Member of the Rare Disease Council.

Aleksandra Matyja-Bednarczyk, MD, PhD

Specialist in internal medicine, clinical immunology and allergology, chief of Immune Diseases and Hypercoagulation Outpatient Department, University Hospital in Cracow.

Monika Mach-Tomalska, MD

Pediatrician and clinical immunologist, works in the Immunology Department of the University Children's Hospital of Cracow (UCH).

What do we focus on?

Research & reports

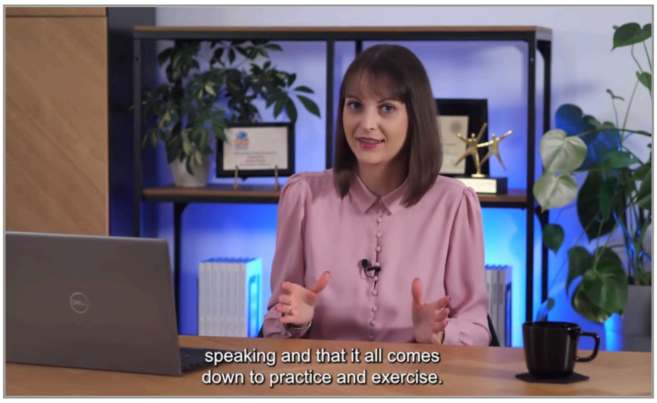
To change reality, it's essential to understand the current situation, the needs of all parties involved, and the system's capabilities. That's why we prioritize research and reports: to gain deeper insights into patients' individual circumstances and develop effective approaches to implement change.

Patient advocacy

We are committed to supporting the best therapies and medical care, prioritizing both the health and quality of life of patients. We advocate for changes that enable patients to lead normal, fulfilling lives, free from the limitations of their conditions.

Patient education & trainings

We guide patient leaders in effectively utilizing available tools and legal mechanisms to advocate for patients' rights. Drawing on our extensive national and international experience, we provide them with practical knowledge, effective strategies, and proven solutions.



Collaboration & change

We believe in teamwork and know that meaningful change requires the involvement of all parties: patient advocates, doctors, stakeholders, and pharma representatives. Through discussions, idea exchanges, and shared experiences, we aim to accelerate progress and inspire change.

“Legal Toolkit for Patient Advocates”. Report on the patients’ participation in decision-making and law-making processes

This report was born out of the real challenges faced by non-profit organizations working to defend patients' rights. Many struggle to navigate complex legal systems in their countries. Our goal was to provide them with practical, proven tools used successfully in different parts of Europe to help make their advocacy more effective. This report brings together these solutions in one place, offering valuable resources for any patient organization leader.

Despite ongoing discussions and recommendations, patients still have limited influence on legal decisions. In most countries, they aren’t formally recognized as experts in shaping laws. While some EU nations are starting to change this through regulatory updates, many patients still lack access to legal mechanisms that allow them to participate effectively.

To address this gap, our Foundation, in collaboration with Boehringer Ingelheim, developed this report as a practical guide for patient advocates. We explored patient involvement in Austria, the Czech Republic, Hungary, Poland, Romania, and Slovakia, working closely with patient organization representatives to ensure real-world insights. A key focus of the report is understanding the legal status of patient organizations - whether they are treated differently from other non-profits and granted specific rights.

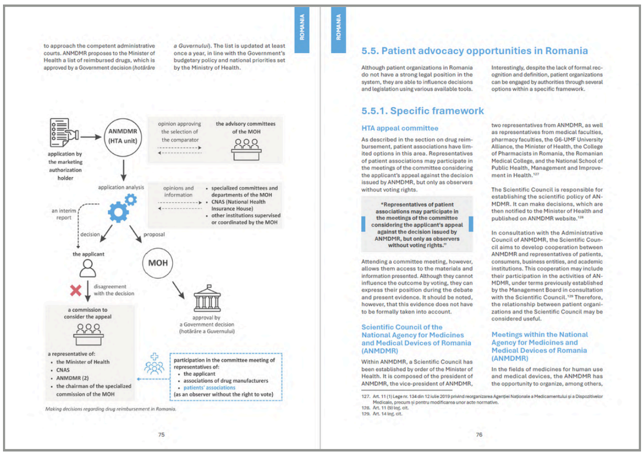
What makes this report unique is its emphasis on practical advocacy tools. We not only highlight existing legal solutions but also showcase alternative strategies that patient organizations can use to make an impact. These include general administrative procedures available to all citizens and collaboration with institutions that hold stronger legal positions. This approach offers new ways to influence healthcare policies, particularly in drug reimbursement. Many patient advocates - especially from smaller organizations - often give up due to limited resources or a lack of clear legal frameworks. Our goal is to show that even within existing systems, meaningful action is possible.

Austria

1. Austria

1.1. The healthcare system in Austria

1.2. Creation of the healthcare law



5.5. Patient advocacy opportunities in Romania

5.5.1. Specific framework

Legal Toolkit for Patient Advocates

The report provides a detailed breakdown of three key areas:
✓ Legal tools specifically designed for patient organizations
✓ General administrative procedures that anyone can use
✓ Alternative advocacy strategies to navigate legal limitations

To deepen the analysis, we also included expert insights from Tomasz Kluszczyński, Strategy Consultant and Founder of ACESO Healthcare Consulting, and Anna Arellanesová, Chair of Rare Diseases Czech Republic. Their perspectives bring valuable context to the challenges and opportunities in patient advocacy.

We hope that the solutions outlined in the report will encourage patient organization leaders to speak up more confidently on healthcare system issues and make greater use of available tools to influence policy decisions We believe that, as a result, patient organizations across Europe will become increasingly involved in shaping healthcare decisions, challenging the outdated perception that they have little influence.

The report is designed to be a valuable resource not only for patient advocates, but also policymakers, and healthcare stakeholders - equipping them with the tools to build a more inclusive and patient-centered healthcare system.

Looking ahead, this project will continue to expand, exploring additional countries and offering even more opportunities to strengthen patient advocacy.

> **THIS REPORT, BY METICULOUSLY EXPLORING ALL FACETS OF PATIENT ENGAGEMENT IN THE CEE, IS OF UTMOST VALUE TO ALL HEALTHCARE STAKEHOLDERS AND OF HIGH UTILITY FOR ALL PATIENT ORGANISATIONS IN THE REGION AND BEYOND.**
> **(...) IT ALSO PROVIDES A USEFUL COMPARISON OF KEY BARRIERS TO OVERCOME AND KEY ENABLERS TO EMBRACE, RESULTING IN A FIRST OF ITS KIND GUIDANCE FOR A STEP-BY-STEP DEVELOPMENT OF A TRULY ANTHROPOCENTRIC AND COLLABORATIVE HEALTHCARE SYSTEM.**

Tomasz Kluszczyński, PHD
Strategy Consultant and Founder
ACESO Healthcare Consulting

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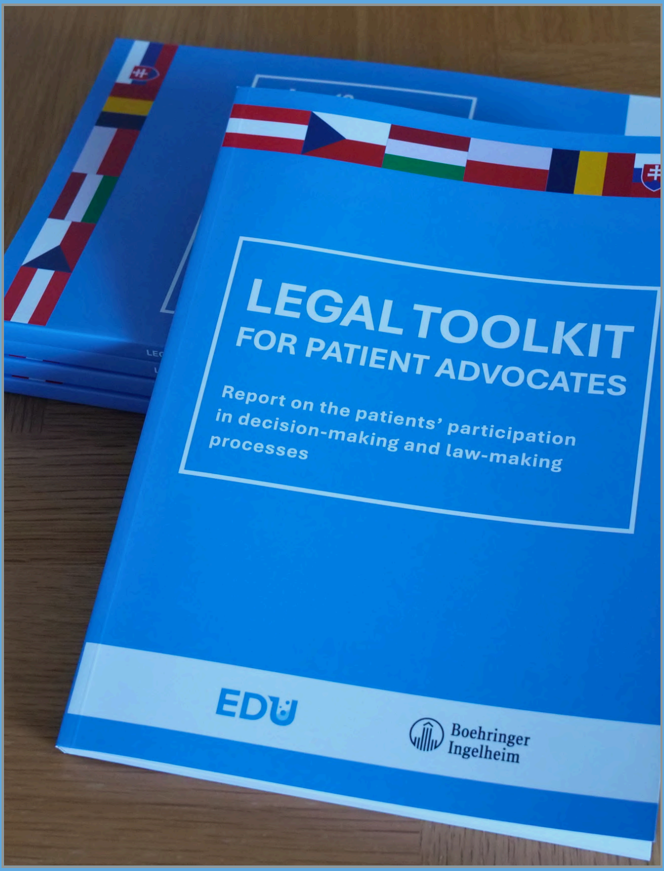
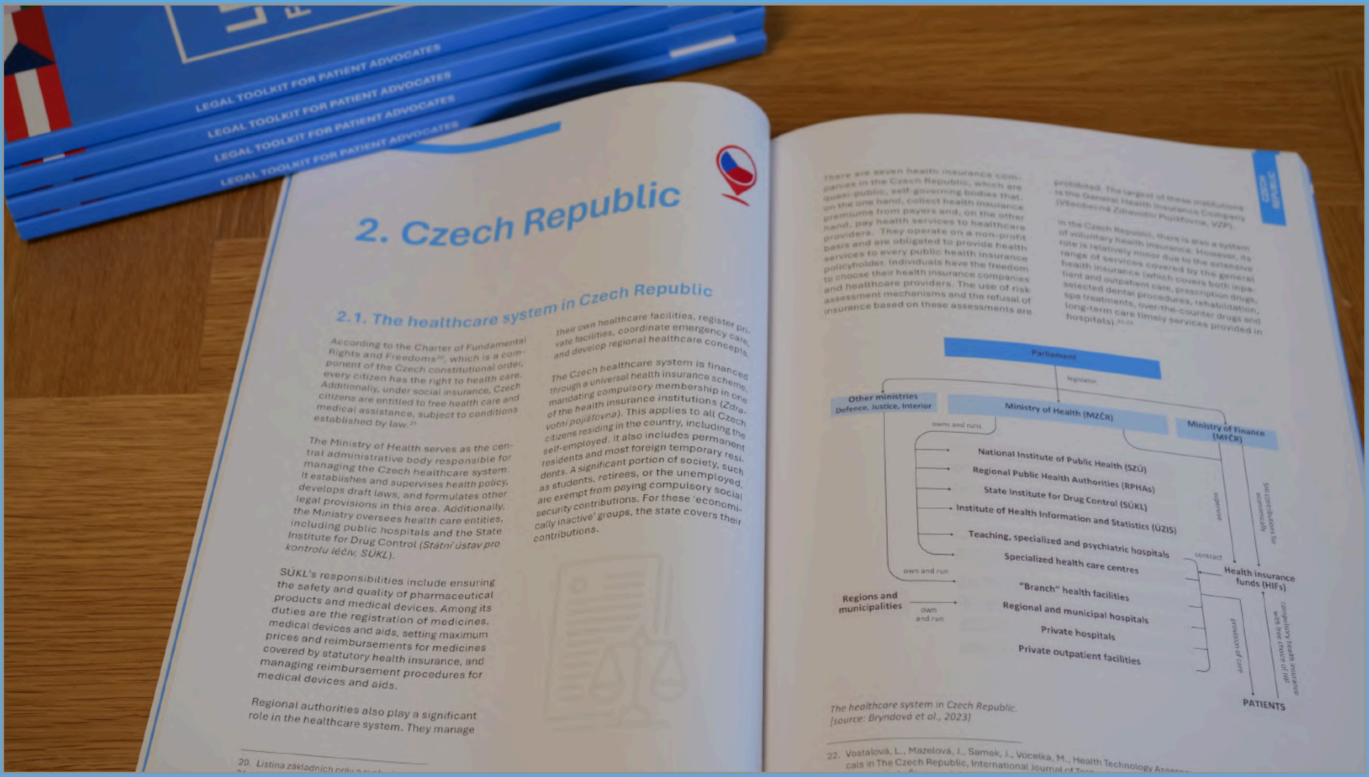
Legal Toolkit for Patient Advocates





2. Czech Republic

2.1. The healthcare system in Czech Republic

According to the Charter of Fundamental Rights and Freedoms[20], which is a component of the Czech constitutional order, every citizen has the right to health care. Additionally, under social insurance, Czech citizens are entitled to free health care and medical assistance, subject to conditions established by law.[21]

The Ministry of Health serves as the central administrative body responsible for managing the Czech healthcare system. It establishes and supervises health policy, develops draft laws, and formulates other legal provisions in this area. Additionally, the Ministry oversees health care entities, including public hospitals and the State Institute for Drug Control (*Státní ústav pro kontrolu léčiv, SÚKL*).

SÚKL's responsibilities include ensuring the safety and quality of pharmaceutical products and medical devices. Among its duties are the registration of medicines, medical devices and aids, setting maximum prices and reimbursements for medicines covered by statutory health insurance, and managing reimbursement procedures for medical devices and aids.

Regional authorities also play a significant role in the healthcare system. They manage their own healthcare facilities, register private facilities, coordinate emergency care, and develop regional healthcare concepts.

The Czech healthcare system is financed through a universal health insurance scheme, mandating compulsory membership in one of the health insurance institutions (*Zdravotní pojišťovna*). This applies to all Czech citizens residing in the country, including the self-employed. It also includes permanent residents and most foreign temporary residents. A significant portion of society, such as students, retirees, or the unemployed, are exempt from paying compulsory social security contributions. For these 'economically inactive' groups, the state covers their contributions.

There are seven health insurance companies in the Czech Republic, which are quasi-public, self-governing bodies that, on the one hand, collect health insurance premiums from payers and, on the other hand, pay health services to healthcare providers. They operate on a non-profit basis and are obligated to provide health services to every public health insurance policyholder. Individuals have the freedom to choose their health insurance companies and healthcare providers. The use of risk assessment mechanisms and the refusal of insurance based on these assessments are prohibited. The largest of these institutions is the General Health Insurance Company (Všeobecná Zdravotní Pojišťovna, VZP).

In the Czech Republic, there is also a system of voluntary health insurance. However, its role is relatively minor due to the extensive range of services covered by the general health insurance (which covers both inpatient and outpatient care, prescription drugs, selected dental procedures, rehabilitation, spa treatments, over-the-counter drugs and long-term care timely services provided in hospitals).[22,23]

The healthcare system in Czech Republic.
[source: Bryndová et al., 2023]

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20. Listina základních práv a svobod

22. Vostalová, L., Mazelová, J., Samek, J., Vocelka, M., Health Technology Assess cals in The Czech Republic, International Journal of Tec

Research on the quality of life and treatment preferences of patients with Fabry’s disease in Poland

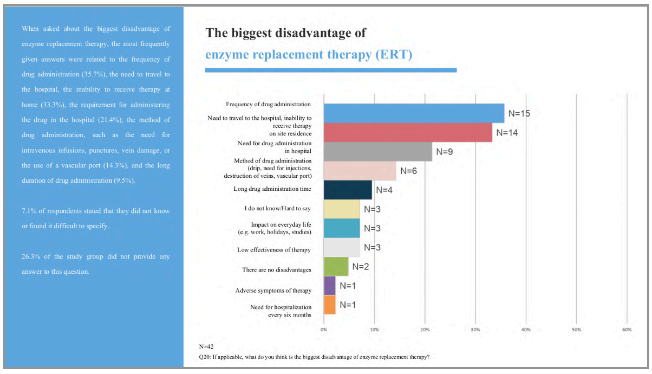
Fabry disease patients in Poland form a small group with specific needs requiring multidisciplinary care. To better understand their needs and the challenges they face daily, we conducted a study focusing on their quality of life as well as their preferences regarding the form and method of treatment for their condition. We believe that this will facilitate discussions with stakeholders about necessary changes and help improve the situation.

For several years, we have had the pleasure of collaborating with the Polish Association of Families with Fabry Disease. Through this cooperation, the idea arose to conduct an in-depth study among patients regarding their daily experiences living with the disease. Last year, we successfully carried out this study with the support of Amicus Therapeutics.

Fabry disease is a rare hereditary disorder caused by an abnormal structure of the GLA gene, which is responsible for encoding the enzyme alpha-galactosidase. This enzyme’s function is to break down certain substances for reuse or elimination. If the gene is incorrectly structured, the amount and activity of the enzyme in affected individuals are insufficient for proper function. As a result, unmetabolized substances accumulate in blood vessels, leading to progressive damage in the body and causing pain.

The first symptoms of Fabry disease usually appear in early childhood, but because they are nonspecific, diagnosis often takes many years. These symptoms include burning pain in the hands and feet, thermoregulation disorders, a red-purple rash, gastrointestinal issues such as abdominal pain, diarrhea, or nausea, corneal deposits, chronic fatigue, and dysfunctions of the kidneys, cardiovascular system, and nervous system. As one can imagine, such symptoms significantly impact the quality of life of patients. Although treatment is available, its effectiveness in completely eliminating symptoms varies. Moreover, one form of therapy can be an additional burden, as it requires frequent hospital visits.

By conducting this study, we aimed to understand how all these factors affect patients - their daily lives as well as their outlook on the future. The study was conducted



When asked about the biggest disadvantage of enzyme replacement therapy, the most frequently given answers were related to the frequency of drug administration (35.7%), the need to travel to the hospital, the inability to receive therapy at home (33.3%), the requirement for administering the drug in the hospital (21.4%), the method of drug administration, such as the need for intravenous infusions, punctures, vein damage, or the use of a vascular port (14.3%), and the long duration of drug administration (9.5%).

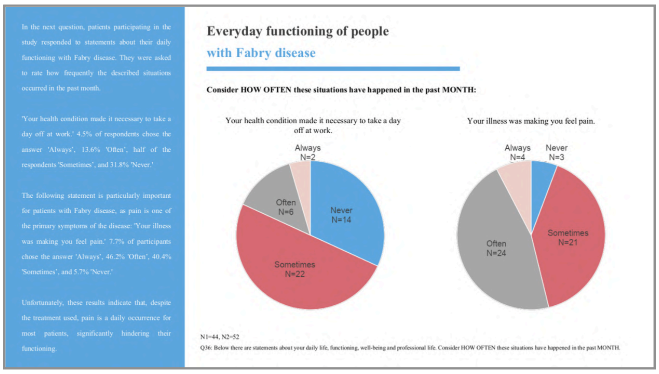
7.1% of respondents stated that they did not know or found it difficult to specify.

26.3% of the study group did not provide any answer to this question.

The biggest disadvantage of
enzyme replacement therapy (ERT)

N=42

Q20: If applicable, what do you think is the biggest disadvantage of enzyme replacement therapy?



In the next question, patients participating in the study responded to statements about their daily functioning with Fabry disease. They were asked to rate how frequently the described situations occurred in the past month.

'Your health condition made it necessary to take a day off at work.' 4.5% of respondents chose the answer 'Always', 13.6% 'Often', half of the respondents 'Sometimes', and 31.8% 'Never.'

The following statement is particularly important for patients with Fabry disease, as pain is one of the primary symptoms of the disease: 'Your illness was making you feel pain.' 7.7% of participants chose the answer 'Always', 46.2% 'Often', 40.4% 'Sometimes', and 5.7% 'Never.'

Unfortunately, these results indicate that, despite the treatment used, pain is a daily occurrence for most patients, significantly hindering their functioning.

Everyday functioning of people
with Fabry disease

Consider HOW OFTEN these situations have happened in the past MONTH:

N1=44, N2=52

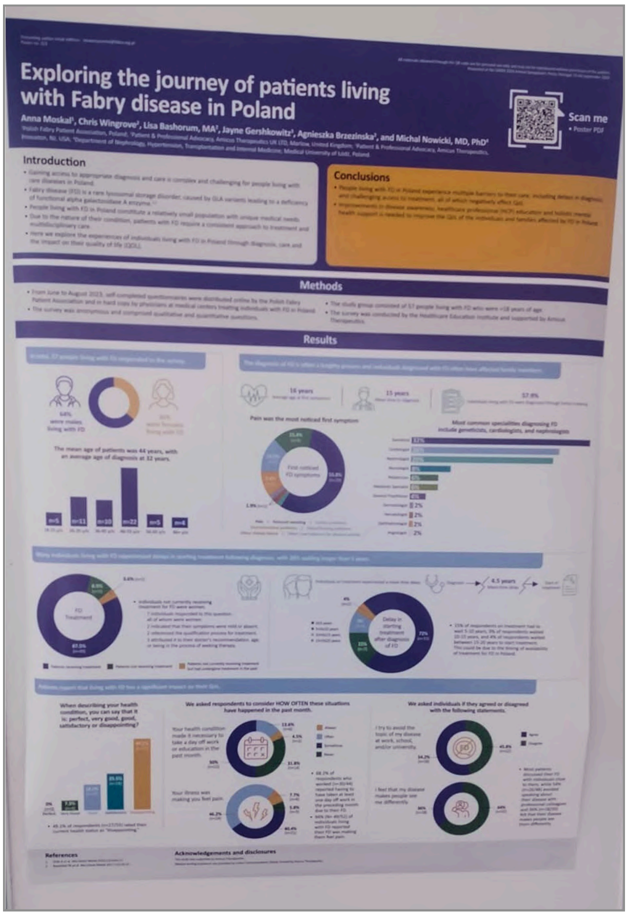
Q36: Below there are statements about your daily life, functioning, well-being and professional life. Consider HOW OFTEN these situations have happened in the past MONTH.

Research on the quality of life and treatment preferences of patients with Fabry's disease in Poland

through anonymous online surveys shared by the patient organization, as well as surveys distributed at facilities treating Fabry disease patients. A total of 57 adult patients with Fabry disease participated in the study.

The study covered the following topics:

- Diagnosis, particularly the initial symptoms of Fabry disease and delays in receiving a correct diagnosis.
- Treatment, including currently used treatment methods, personal preferences, and the pros and cons of different therapies.
- Family experiences, which are extremely important due to the hereditary nature of the disease.
- Personal experiences, focusing on each individual's journey with the disease.
- Quality of life, including health status, future outlook, psychological challenges, and the burdens associated with the disease.



Exploring the journey of patients living with Fabry disease in Poland

Anna Moskal[1], Chris Wingrove[2], Lisa Bashorum, MA[2], Jayne Gershkowitz[3], Agnieszka Brzezinska[3], and Michal Nowicki, MD, PhD[4]

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• Poster PDF

Introduction

Conclusions

Methods

Results

Research shows that Fabry disease significantly impacts patients' lives, affecting their ability to work, social interactions, and overall well-being. Despite receiving a diagnosis and treatment, half of the patients rate their health as unsatisfactory, and one-third have a pessimistic outlook on the future. Pain remains a daily challenge for most, representing a major burden.

The study we conducted in Poland is the first to explore patients' personal preferences regarding treatment methods. While medical factors often limit their choice of therapy, it is crucial to consider their personal needs and motivations whenever possible.

We hope that the study's findings will contribute to discussions on how changes in the medical care of Fabry disease patients in Poland could improve their quality of life and help reduce the pain they face daily.

The coming year will bring an exciting expansion of the project. This research will be extended to other countries, starting with patients in the Czech Republic and Croatia, providing new insights and valuable comparative data.

The research results were presented at the Annual Symposium of the Society for the Study of Inborn Errors of Metabolism 2024 in Porto. In the upcoming year, they will also be showcased at the New Horizons in Fabry Disease Conference in Prague.

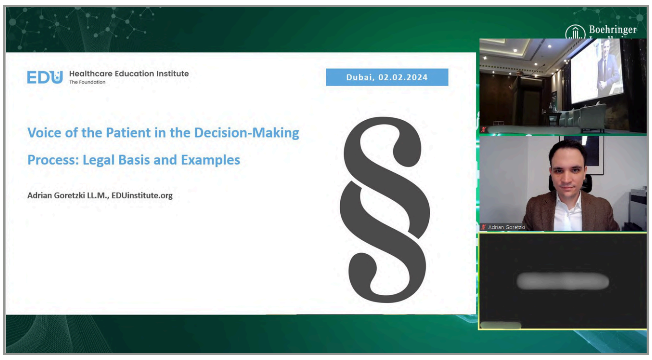
Poster summarizing the research results presented at the Annual Symposium of the Society for the Study of Inborn Errors of Metabolism 2024.

Driving impact: the latest in patient advocacy

Our actions are always aimed at improving the situation of patients by changing standards, legislation, and practices. Over the past year, we have primarily concentrated on drawing attention to the legal aspects of patient advocacy and emphasizing the importance of engaging patient representatives in various initiatives shaping healthcare.

At the beginning of 2024, the president of the foundation, Adrian Goretzki had the honor of speaking at the "Value and Innovation in Healthcare" summit in Dubai (2-3.02.2024), organized by Boehringer Ingelheim. Among the summit participants were government officials and representatives of payers from the Middle East region.

Adrian presented possible legal solutions in this area, including check and balance mechanisms that consider the interests of all stakeholders. He highlighted the importance of integrating patient voices into the legislative process to ensure more transparent and equitable healthcare policies. He pointed to real examples of good practices present in other countries that could be successfully implemented to ensure patient participation in the decision-making process.



EDU Healthcare Education Institute
The Foundation

Dubai, 02.02.2024

Voice of the Patient in the Decision-Making Process: Legal Basis and Examples

Adrian Goretzki LL.M., EDUinstitute.org

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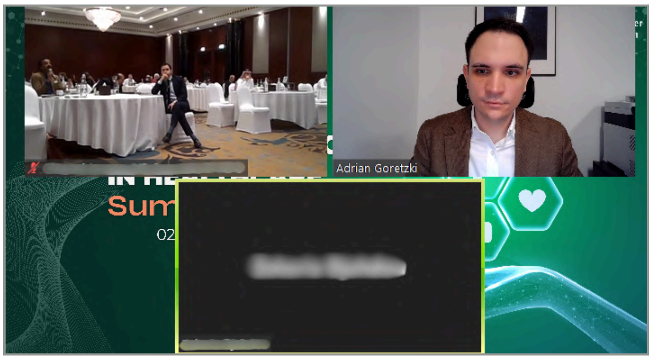


One of the most important events in the world of rare diseases is the annual World Orphan Drug Congress. The European edition last year took place in Barcelona (October 22-25, 2024). We couldn't miss it - in fact, Adrian Goretzki had the pleasure of being among the speakers.

Adrian shared his insights on building a patient advocacy strategy, both within patient organizations and in the industry. He discussed the approaches to take, the tools needed, the groups to involve, and the results that can be expected. His presentation emphasized the importance of cross-sector collaboration to ensure that patient perspectives are effectively integrated into healthcare decision-making processes.

Driving impact: the latest in patient advocacy

For another consecutive year, we also attended the “Access to Care” conference for leaders of patient organizations (November 21, 2024). This fantastic event, organized by Boehringer Ingelheim in Vienna, always brings together top experts. This year, the conference was held in a hybrid format.

The event provided an opportunity to present the results of our months-long work for the first time - the premiere of the report "Legal Toolkit for Patient Advocates," which was created by the Healthcare Education Institute team in cooperation with Boehringer Ingelheim (more about the report itself on pages 7-9 of the annual report).

During his speech, Adrian focused on legal ways to engage patient organizations in decision-making and lawmaking.

He also led workshops for participants based on case studies analysis, which aimed to develop practical decision-making skills in crisis situations concerning healthcare and patient rights. Each case was discussed in detail so that participants could understand the consequences of different approaches and learn how to strategically plan actions to defend patient rights.

We are delighted to share our experience with others and contribute to positive changes in healthcare and patient advocacy. We look forward to more events in the coming year!

> **_Because we believe that rare means exceptional, not unimportant._**

Driving impact: the latest in patient advocacy

Adrian Goretzki during a speech at the "Access to Care" conference in Vienna.

Visit to the Boehringer Ingelheim Campus in Vienna. From the right: Dorica Dan, Dunja Loncaric, Susanne Tatschl, Aistė Štaraitė, Nadia Hallwirth, Hristina Nikolova, Tomasz Kluszczyński, Marcin Czech, Adrian Goretzki, Bertalan Németh, Stoicho Katsarov.

PatientAdvocacy.Academy 2024: Mastering Communication

Our 7th edition of the PatientAdvocacy.Academy - innovative online course for leaders of patient organizations iin the field of rare diseases, has wrapped up with incredible success!

Effective communication is essential for patient advocates, yet many struggle to convey their messages clearly to doctors, NGOs, and the public. Challenges include simplifying complex information, engaging with the media, and building public speaking skills. Recognizing this, the PatientAdvocacy. Academy 2024 focused on sharpening these communication skills, empowering advocates to stand out and effectively connect with their audiences. Participants left equipped to share their mission with impact and confidence.

In just 21 days, participants:

- learned how to turn different audiences into their allies,
- gained knowledge on effectively communicating the most important demands of their patient organization,
- discovered the secrets of media communication,
- learned how to prepare for every conversation and public appearance,
- enhanced their skills in public speaking,
- mastered the art of creating interesting and engaging presentations.

22
participants

17
countries

3
continents

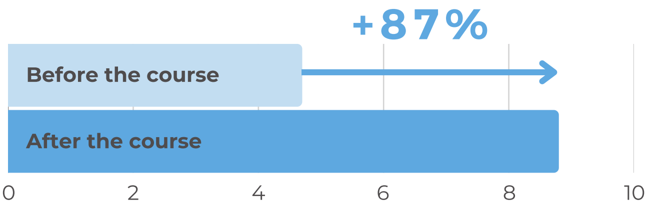
> **“THIS YEAR THE COURSE WAS VERY USEFUL FOR ME, AS IT COVERED TOPICS THAT CONCERN ME.”**
>
> **“I WAS SUPER IMPRESSED BY THIS YEAR'S TOPIC, GREAT INFO FOR MY ORGANIZATION.”**

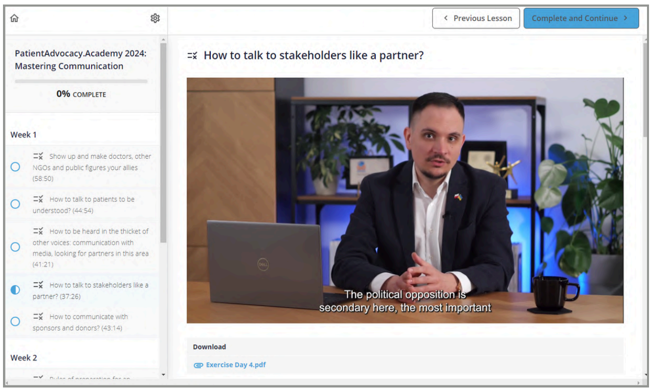
Anonymous opinions from surveys completed by participants after the Academy

How did PAA 2024 actually look like?

1st step: Self-assessing of communication skills

We assess the Academy's effectiveness through surveys conducted before and after each course. This year, results showed a significant improvement in participants' communication skills, with ratings increasing from 4.7 to 8.8, reflecting an 87% boost in self-assessed abilities.





PatientAdvocacy.Academy 2024: Mastering Communication

0% COMPLETE

Week 1

○ Show up and make doctors, other NGOs and public figures your allies (58:50)

○ How to talk to patients to be understood? (44:54)

○ How to be heard in the thicket of other voices: communication with media, looking for partners in this area (41:21)

◐ How to talk to stakeholders like a partner? (37:26)

○ How to communicate with sponsors and donors? (43:14)

Week 2

How to talk to stakeholders like a partner?

The political opposition is secondary here, the most important

Download

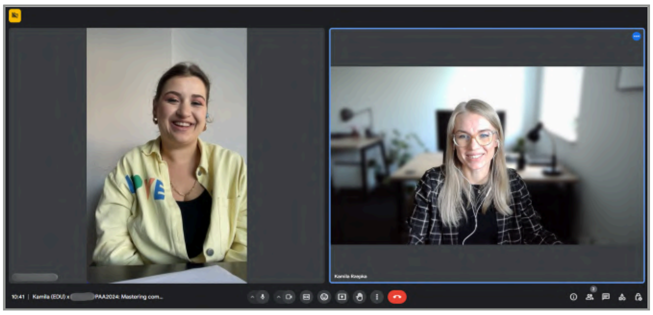
Exercise Day 4.pdf

Engaging video content

The PAA 2024 included 10 video lectures on key communication topics, covering both the theoretical foundations of communication with various individuals and entities, as well as practical skills in public speaking and self-presentatio. To improve accessibility, we provided English subtitles and added Russian and Ukrainian dubbing. Supplementary materials, such as charts and infographics, enriched the content.

1-to-1 preliminary online meetings

We held 1-to-1 preliminary meetings to enhance participant engagement. These sessions helped us connect, identify challenges, and tailor content to relevant issues. The approach encouraged first-timers and allowed returning participants to reflect on their progress, making these meetings a key part of the Academy's success.

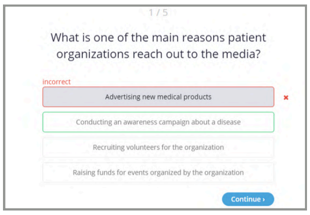


Exercise for Lecture 3:
Presenting the case in the media

Find an article on the internet about the fight for reimbursement or the refusal of treatment. Read it and note which elements are included - check them off on the list below:

- ○ description of the crisis situation
- ○ description of the disease
- ○ statement from a medical expert
- ○ statement from a patient organization representative
- ○ patient story
- ○ worst-case future scenario
- ○ statement from the Ministry of Health or payer

Write down below what you missed the most in the article.

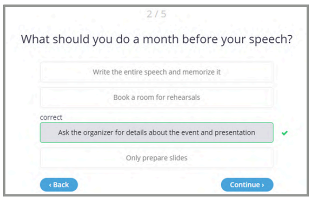


1 / 5

What is one of the main reasons patient organizations reach out to the media?

incorrect

- Advertising new medical products ✗
- Conducting an awareness campaign about a disease
- Recruiting volunteers for the organization
- Raising funds for events organized by the organization



2 / 5

What should you do a month before your speech?

- Write the entire speech and memorize it
- Book a room for rehearsals
- correct: Ask the organizer for details about the event and presentation ✓
- Only prepare slides

Educational materials

Each video lecture featured materials to help participants apply their knowledge, including a printable PDF exercise and a 5-question quiz. The exercises supported actionable solutions, while the quizzes offered an engaging way to review key concepts.

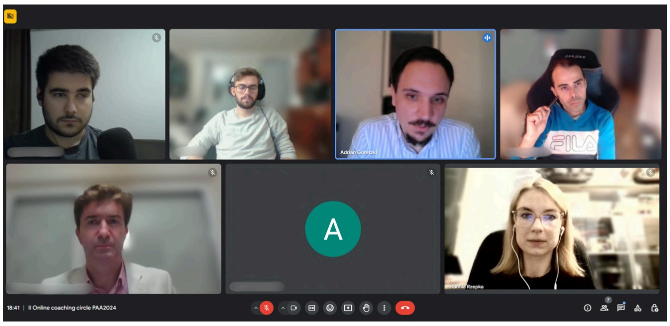
How did PAA 2024 actually look like?

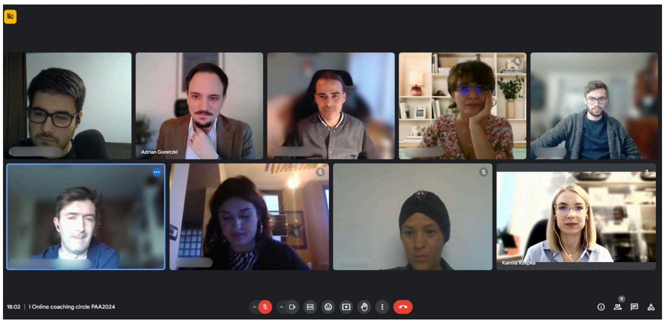
Online video circles

The online coaching circles fostered a strong sense of unity among participants, who learned from and motivated each other while offering valuable feedback. This edition featured two sessions. In the first, participants shared success stories and communication insights. The second session focused on a 1-minute "elevator pitch" challenge, simulating an encounter with the Health Minister. Participants and trainers provided constructive feedback on each pitch's strengths and areas for improvement.

> **“THE ACADEMY UNITES THE ENERGY OF CREATORS AND PARTICIPANTS, WHICH INSPIRES NEW IDEAS.”**

Anonymous opinion from surveys completed by participants after the Academy





Endividual mentoring sessions

This year, participants benefited from dedicated 1-on-1 mentoring sessions with trainers, where they tackled real challenges faced by their patient organizations. These personalized meetings allowed trainers to give each participant their full attention, collaborating on practical, tailored solutions.

Grant competition

Each PAA provides not only training but also funding opportunities for patient organizations to implement new projects.

In this edition, participants were required to complete two tasks. We awarded grants totaling €3,250 to three patient organizations from Ukraine, Serbia, and Romania.

> **“...THE GRANT COMPETITION TASK WAS ESPECIALLY WELL DONE, IT PUSHED US TO REALLY THINK THROUGH WHAT WE'D LEARNED WHILE ALSO LETTING US BRING OUT OUR CREATIVITY. IT WAS A PERFECT BALANCE OF CHALLENGING AND INSPIRING!”**

Anonymous opinion from surveys completed by participants after the Academy

Assessment and opinion of participants of this year's Academy

9,5

Overall course assessment

9,8

Choice of the subject

9,7

Usefulness of the information

9,4

Interesting way of presentation

“This was my second year in the academy. And I have the intention to continue applying for the academy, because the topics dealt in the academy are different every year. The videos are of great quality, and they are easy to follow. It is obvious how much experience Kamila, Bernadeta and Adrian have. (...) they answer all the questions you have and even find the time to make 1 on 1 meetings if someone has the need to discuss some additional topics or questions...”

Kornelije Juranić
Hrvatska Anderson Fabry udruga

“This training will help many understand communication in the patient advocacy field. I am thrilled by how the training exercises were done. Thank you for imparting this wealth of knowledge to a group of patients and patient organizations. This online training was very beneficial to me and relevant in my career field. It will help me enormously.”

Bianca Özcan
Rare Diseases Namibia

If you want to know more about the PAA2024, read our report by clicking on the cover on the right.

PATIENT ADVOCACY ACADEMY 2024

MASTERING COMMUNICATION

THE REPORT

EDU

One-site trainings for patient advocates

The empowerment of rare disease patient advocates is the core activity of the Healthcare Education Institute. We achieve this not only through our PatientAdvocacy.Academy project but also by offering numerous in-person training sessions.

We had the opportunity to support the newly established Polish Association of Patients with LAL-D with our knowledge and experience. We participated in a special educational meeting for patients with this rare condition (July 20, 2024, in Warsaw), aimed at providing practical information about the social support system in Poland.

Adrian Goretzki also led a lecture, guiding participants step by step on how to apply for a disability certificate and social pension.

Agenda spotkania

9.00 - 9.05	Powitanie
9.05 - 10.30	**JAK MÓWIĆ, BY ZOSTAĆ USŁYSZANYM, ZROZUMIANYM I ZAPAMIĘTANYM, Cz. 1** Joanna Górska – dziennikarka telewizyjna programu „Pytanie na śniadanie" (TVP2), trenerka wystąpień publicznych, prezeska Fundacji Silni Sobą
10.30 - 10.45	Przerwa kawowa
10.45 - 11.20	**JAK MÓWIĆ, BY ZOSTAĆ USŁYSZANYM, ZROZUMIANYM I ZAPAMIĘTANYM, Cz.2**
11.20 - 12.00	**FORMY ORGANIZACJI PACJENCKICH – WADY I ZALETY** Adrian Goretzki - prawnik, Healthcare Education Institute
12.00 - 12.45	LUNCH
12.45 - 14.15	**FOUNDRISING KROK PO KROKU, CZYLI WYZWANIA MAŁYCH I DUŻYCH NGO-ów** Adrian Goretzki - prawnik, Healthcare Education Institute
14.15 - 14.25	Przerwa kawowa
14.25 - 15.25	**JAK POMAGAĆ INNYM I NIE WYPALIĆ SIĘ** Adrianna Sobol - psycholog, psychoterapeuta, założycielka platformy "Zdrowie zaczyna się w głowie"
15.25 - 15.35	Podsumowanie i zakończenie

Adrian Goretzki and LAL-D patients during the meeting in Warsaw.

In the fall, at the invitation of the Modern Healthcare Institute and AstraZeneca, we took part in a training session for leaders of rare disease patient organizations in Warsaw (October 12, 2024).

Adrian delivered two lectures - one on the advantages and disadvantages of different legal forms of patient organizations, and the other on fundraising and the challenges NGOs face in this area.

It is always a great pleasure to meet patient advocates in person, working across various therapeutic areas and at different stages of their organizations' development. Such meetings are truly inspiring for both sides.

Humanitarian aid for Ukrainian rare disease patients

The war in Ukraine has now lasted three years, and the needs of rare disease patients remain significant. In 2024, we aimed to support the Ukrainian patient community once again by donating highly specialized medical equipment.

Thanks to the generous support of our donors, in 2024, we were able to help the Ukrainian community of patients with rare diseases, particularly those with primary immunodeficiencies.

The medical equipment needed for subcutaneous drug infusions was purchased and delivered to Ukrainian hospitals in collaboration with the NGO Rare Diseases of Ukraine. We were able to support patients in three facilities: Lviv Regional Clinical Diagnostic Center, KNP "Kyiv City Center of Nephrology and Dialysis", and Chernivtsi Regional Clinical Hospital.

Packages with medical equipment for hospitals in Lviv, Kiyv and Chernivtsi.

Staff of the hospital in Kiyv with donated medical equipment.

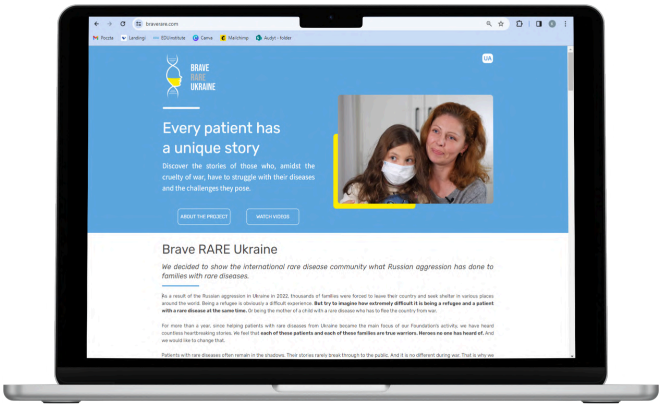
A representative of the NGO "Rare Diseases of Ukraine" with a doctor from Kyiv during the delivery of humanitarian aid.

Brave RARE Ukraine

In 2024, we continued the Brave RARE Ukraine project, which began in August 2023. Our goal was to show the international community what Russian aggression has done to families with rare diseases. In 2023, we published testimonials from patients and their families who fled to Poland to escape the war. In 2024, we focused on the stories of incredibly brave Ukrainian doctors who remained in hospitals in Ukraine.

We published five videos featuring physicians from Kyiv, Lviv, Ivano-Frankivsk, and Ternopil. Doctors working in a war-torn country talk about their everyday lives and how they see the future of the Ukrainian healthcare system. Their dedication to patients, often in the most extreme conditions, is truly admirable.

The project partners include CSL Behring, EURORDIS, Global Genes, Horizon, Ovid Therapeutics, NGO "Rare Diseases Ukraine", NGO "Rare Immune Diseases", and Okhmadyt, Ukraine's largest children's hospital, which treats many patients with rare diseases. Our media partner was Rare Revolution Magazine.



Every patient has a unique story

Discover the stories of those who, amidst the cruelty of war, have to struggle with their diseases and the challenges they pose.

Brave RARE Ukraine

We decided to show the international rare disease community what Russian aggression has done to families with rare diseases.

As a result of the Russian aggression in Ukraine in 2022, thousands of families were forced to leave their country and seek shelter in various places around the world. Being a refugee is obviously a difficult experience. **But try to imagine how extremely difficult it is being a refugee and a patient with a rare disease at the same time.** Or being the mother of a child with a rare disease who has to flee the country from war.

For more than a year, since helping patients with rare diseases from Ukraine became the main focus 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 is no different during war. That is why we

You can read about the project on a dedicated website:

braverare.com

Films featuring Ukrainian doctors

Click on the image to watch the video

Prof. Anastasiia Bondarenko

Physician from Kyiv, taking care of patients with rare diseases

On the first day of the war, her hospital was on the front line, halting patient admissions. Some doctors moved to other hospitals, working and living there for weeks due to the constant influx of patients. Others couldn't reach work, trapped in occupied areas or under fire. Professor Bondarenko stayed with the children in a bomb shelter, assisting patients remotely. Her efforts ensured many received timely treatment abroad.

BRAVE
RARE
UKRAINE

Films featuring Ukrainian doctors


Patients called, even patients with primary immunodeficiency diseases,

Click on the image to watch the video

Dr. Marianna Derkach

Physician from Ivano-Frankivsk, taking care of patients with rare diseases

On the third day of the war, Marianna broke her leg while leaving a bomb shelter. Despite being in a cast and on crutches, she continued treating patients and helping displaced people, feeling a duty to support her medical team. She also talks about the psychological toll on doctors, who must stay focused on their work while worrying about their families' safety outside the hospital.

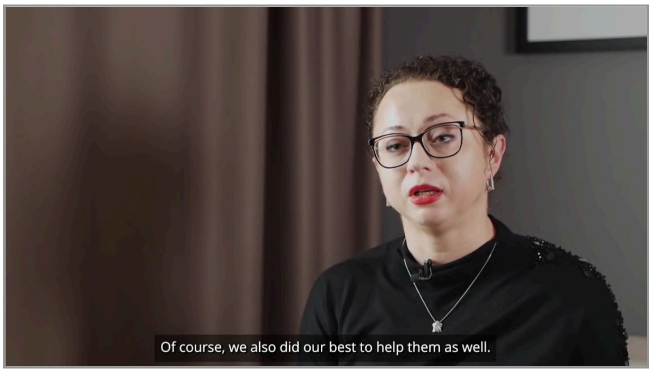
Prof. Oksana Boyarchuk

Physician from Ternopil, taking care of patients with rare diseases

Frequent bomb alarms forced Prof. Boyarchuk and the medical staff to repeatedly move equipment and set up life-saving devices in shelters. The constant alerts were especially challenging for disabled patients struggling to reach safety. With many people displaced, securing medications and equipment for both existing and new patients was uncertain. While some patients fled the country, ensuring continued treatment for those who stayed was essential.


And thirdly, there were patients whom even we sent abroad,

Click on the image to watch the video


Of course, we also did our best to help them as well.

Click on the image to watch the video

Dr. Khrystyna Lishchuk-Yakymovych

Physician from Lviv, taking care of patients with rare diseases

War impacts both the physical and mental health of patients. Doctors had to adapt their support for patients with rare diseases, who worried about access to therapy, the availability of medications, and the safety of staying in Ukraine. Khrystyna notes that while doctors are trained to handle many crises, nothing prepares them for the outbreak of war. Medical staff must remain emotionally resilient to help patients effectively and prevent chaos.

Films featuring Ukrainian doctors

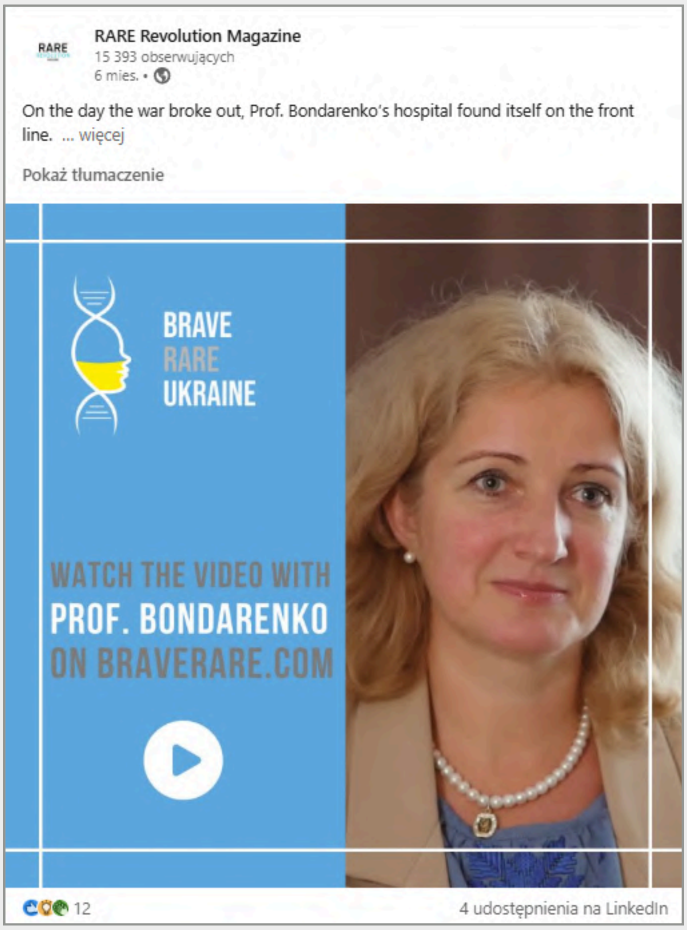
BRAVE
RARE
UKRAINE

Click on the image to watch the video

Prof. Valentyna Chopyak

Physician from Lviv, taking care of patients with rare diseases

Before February 24, 2022, Ukrainian doctors were focused on projects like patient registries, state programs for funding orphan drugs, and implementing life-saving therapies. The war, however, forced a shift to immediate priorities, slowing the progress of healthcare development. Despite this, Professor Chopyak highlights that doctors continue to meet in expert committees to develop care standards for patients.



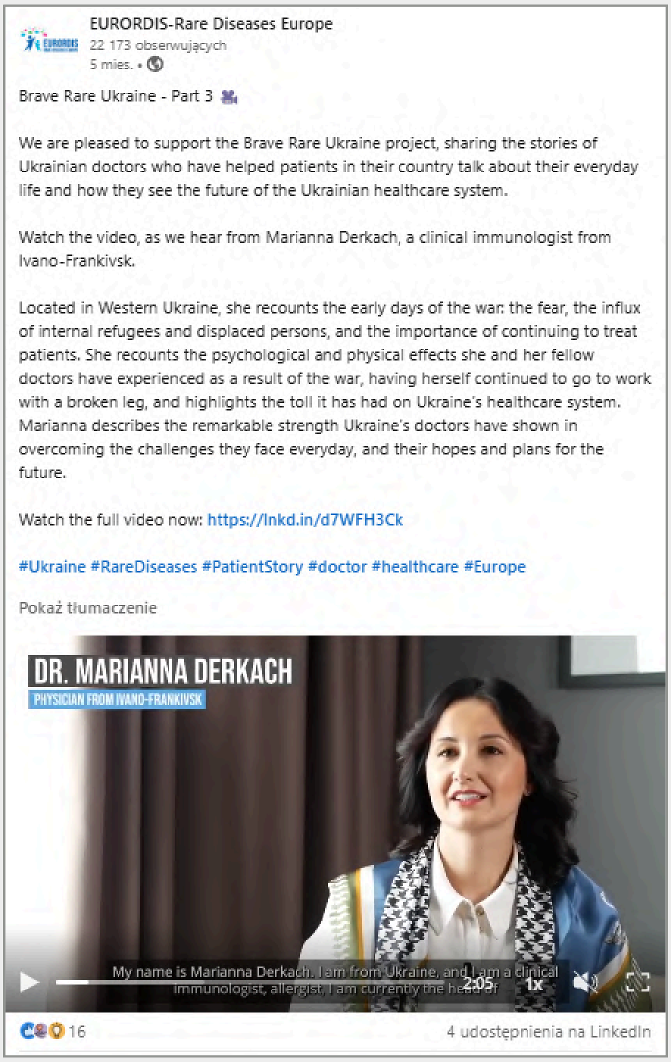
RARE Revolution Magazine
15 393 obserwujących
6 mies. •

On the day the war broke out, Prof. Bondarenko's hospital found itself on the front line. ... więcej

Pokaż tłumaczenie

12

4 udostępnienia na LinkedIn



EURORDIS-Rare Diseases Europe
22 173 obserwujących
5 mies. •

Brave Rare Ukraine - Part 3

We are pleased to support the Brave Rare Ukraine project, sharing the stories of Ukrainian doctors who have helped patients in their country talk about their everyday life and how they see the future of the Ukrainian healthcare system.

Watch the video, as we hear from Marianna Derkach, a clinical immunologist from Ivano-Frankivsk.

Located in Western Ukraine, she recounts the early days of the war: the fear, the influx of internal refugees and displaced persons, and the importance of continuing to treat patients. She recounts the psychological and physical effects she and her fellow doctors have experienced as a result of the war, having herself continued to go to work with a broken leg, and highlights the toll it has had on Ukraine's healthcare system. Marianna describes the remarkable strength Ukraine's doctors have shown in overcoming the challenges they face everyday, and their hopes and plans for the future.

Watch the full video now: https://lnkd.in/d7WFH3Ck

#Ukraine #RareDiseases #PatientStory #doctor #healthcare #Europe

Pokaż tłumaczenie

16

4 udostępnienia na LinkedIn

The project received strong media coverage and was widely shared on social media.

Other

Meetings & events in 2024

Throughout 2024, we engaged in numerous significant meetings, conferences, and training programs. These occasions allowed us to foster meaningful exchanges of knowledge and ideas while broadening our understanding. Every effort was dedicated to advancing our mission of improving the lives of patients living with rare diseases.

The "Value and Innovation in Healthcare" summit

2-3.02.2024, Dubai, United Arab Emirates

Adrian Goretzki had the privilege of being a speaker at the prestigious "Value and Innovation in Healthcare" summit in Dubai, an event hosted by Boehringer Ingelheim.

He highlighted the importance of integrating patient voices into the legislative process to ensure more transparent and equitable healthcare policies.

Details about the event are on page 12 of the report.

EDU Healthcare Education Institute
The Foundation

Dubai, 02.02.2024

Voice of the Patient in the Decision-Making Process: Legal Basis and Examples

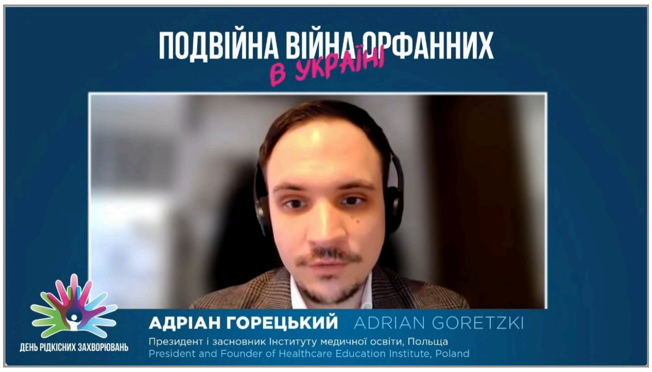
Adrian Goretzki LL.M., EDUinstitute.org

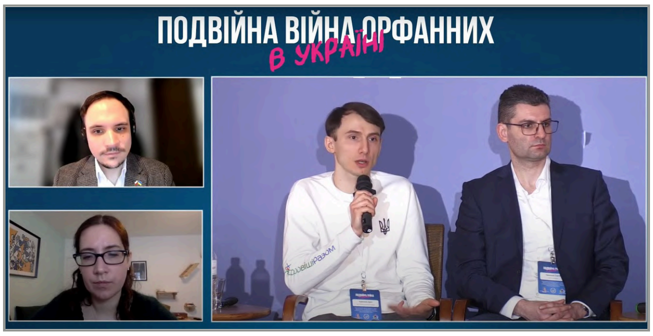
International Rare Disease Day 2024 in Ukraine

27.02.2024, Kiyv, Ukraine

To mark Rare Disease Day, the NGO Rare Diseases of Ukraine organised a discussion panel titled “DOUBLE WAR: Ukraine’s European Vector in Providing Treatment and Support for People Living with a Rare Disease in the Context of a Full-Scale War”.

At their invitation, Adrian Goretzki took part in the discussion, emphasizing the need for long-term support for Ukraine’s healthcare system in his speech.





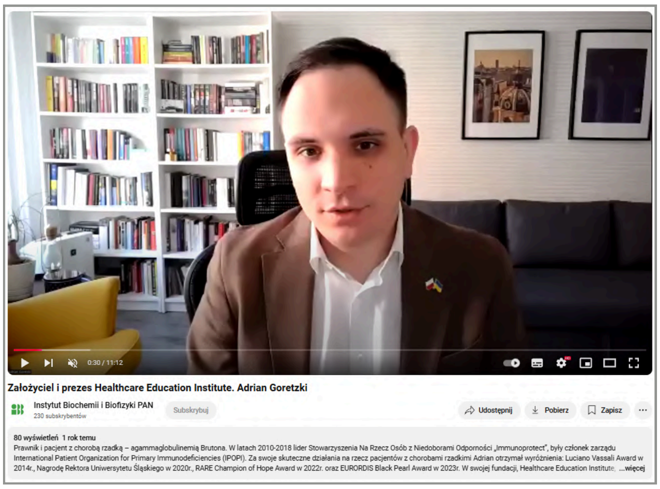
Meetings & events in 2024

The Conference on the occasion of Rare Disease Day

29.02.2024, Warsaw, Poland

Representatives of the foundation participated in a conference organised by the Institute of Biochemistry and Biophysics of the Polish Academy of Sciences to mark Rare Disease Day. The event focused on the challenges of securing funding for rare disease research.

During the panel, Adrian Goretzki highlighted the importance of collaboration, determination and patience in obtaining funds for research and care. He also pointed to insufficient public awareness of rare diseases as one of the obstacles to effective fundraising.



Założyciel i prezes Healthcare Education Institute. Adrian Goretzki

Instytut Biochemii i Biofizyki PAN
230 subskrybentów

80 wyświetleń 1 rok temu
Prawnik i pacjent z chorobą rzadką – agammaglobulinemią Brutona. W latach 2010-2018 lider Stowarzyszenia Na Rzecz Osób z Niedoborami Odporności „Immunoprotect", były członek zarządu International Patient Organization for Primary Immunodeficiencies (IPOPI). Za swoje skuteczne działania na rzecz pacjentów z chorobami rzadkimi Adrian otrzymał wyróżnienia: Luciano Vassali Award w 2014r., Nagrodę Rektora Uniwersytetu Śląskiego w 2020r., RARE Champion of Hope Award w 2022r. oraz EURORDIS Black Pearl Award w 2023r. W swojej fundacji, Healthcare Education Institute, ...więcej

Click on the photo to watch the recording.

The European Conference on Rare Diseases and Orphan Products (ECRD)

15-16.05.2024, Brussels, Belgium

Our foundation was also present at ECRD, the largest patient-led event in Europe dedicated to shaping rare disease policy.

It was a great opportunity for us to meet amazing leaders of rare disease patient organisations from around the world and exchange experiences.

In the photo: Adrian Goretzki with Anna Kole, Global Patient Engagement, UCB.

Spotkanie edukacyjne dla pacjentów z chorobą LAL-D

20.07.2024, Warsaw, Poland

We had the opportunity to support a newly forming organisation: the Polish Association of Patients with LAL-D. Adrian Goretzki conducted training for its leaders on the legal forms of patient organisations.

For more details about the training, see page 19 of the report.

Meetings & events in 2024

XXXIII Economic Forum

3-5.09.2024, Karpacz, Poland

Representatives of the foundation took part in the XXXIII Economic Forum in Karpacz, which every year brings together, among others, experts in the field of medicine and healthcare. We participated in very interesting sessions on public health, rare diseases and HTA.

The EFPIA CEE Patient Engagement Forum

26-27.09.2024, Warsaw, Poland

At the invitation of the organizers, Adrian Goretzki participated in a discussion on patient data and real world evidence in clinical trials and HTA as part of the EFPIA CEE Patient Engagement Forum in Warsaw.

Lecture for Patient Organizations on Interstitial Lung Diseases

7.10.2024, on-line meeting

During an event organised by Boehringer Ingelheim for patient organisation on Interstitial Lung Diseases, Adrian Goretzki delivered a lecture on evidence-based advocacy, discussing the importance of evidence, where to find it, and how to use it in patient advocacy.

Boehringer
Ingelheim

Meetings & events in 2024

Training for Leaders of Rare Disease Patient Organisations

12.10.2024, Warsaw, Poland

During the event for leaders of emerging rare disease patient organisations, Adrian Goretzki shared his experience in communication and fundraising within patient organisations.

For more details about the event, see page 19 of the report.

10.45 - 11.20	JAK MÓWIĆ, BY ZOSTAĆ USŁYSZANYM, ZROZUMIANYM I ZAPAMIĘTANYM, Cz.2
11.20 - 12.00	FORMY ORGANIZACJI PACJENCKICH – WADY I ZALETY Adrian Goretzki - prawnik, Healthcare Education Institute
12.00 - 12.45	LUNCH
12.45 - 14.15	FOUNDRISING KROK PO KROKU, CZYLI WYZWANIA MAŁYCH I DUŻYCH NGO-ów Adrian Goretzki - prawnik, Healthcare Education Institute
14.15 - 14.25	Przerwa kawowa
14.25 - 15.25	JAK POMAGAĆ INNYM I NIE WYPALIĆ SIĘ Adrianna Sobol - psycholog, psychoterapeuta, założycielka platformy "Zdrowie zaczyna się w głowie"

Specific legal framework:
Participation of patient organizations in the HTA process - Czech role model

The involvement of POs was made possible by establishing a legal definition of patient organizations and a list of recognized organizations, which was a significant step towards patient involvement in the management of the Czech healthcare system.

Recently, for orphan drugs, a new pathway gives registered patient organizations and medical societies **a voice** in the administrative procedure to set prices and reimbursement rates.

- **I phase: evaluation process** – they can submit evidence and other comments that are taken into account by regulator in the evaluation report. They can also submit comments on the report itself.
- **II phase: decision-making** – there is an advisory body, which includes two representatives of patient organizations. They have **the right to evaluate** criteria and parameters for making decision based on evaluation report issued by the regulator. **Each member votes on his own behalf.**

The World Orphan Drug Congress Europe 2024

22-25.10.2024, Barcelona, Spain

Our foundation took part in the World Orphan Drug Congress Europe 2024, where Adrian Goretzki delivered a speech on legal strategies for patient advocacy.

Details about the event are on page 12 of the report.

Access to Care Conference

21.11.2024, Vienna, Austria

As every year, we participated in the "Access to Care" conference organized by Boehringer Ingelheim for patient organization representatives. Adrian Goretzki's presentation focused on legal ways to involve patient organizations in decision-making and legislative processes.

More details about the event can be found on page 13 of the report.

2024 on the timeline

2-3.02.2024
Dubai, United Arab Emirates

The "Value and Innovation in Healthcare" summit

27.02.2024
Kiyv, Ukraine

International Rare Disease Day 2024 in Ukraine

29.02.2024
Warsaw, Poland

The Conference on the occasion of Rare Disease Day

15-16.05.2024
Brussels, Belgium

The European Conference on Rare Diseases and Orphan Products

20.07.2024
Warsaw, Poland

Educational meeting for patients with LAL-D disease

3-5.09.2024
Karpacz, Poland

XXXIII Economic Forum

26-27.09.2024
Warsaw, Poland

The EFPIA CEE Patient Engagement Forum

7.10.2024
on-line meeting

Lecture for Patient Organizations on Interstitial Lung Diseases

12.10.2024
Warsaw, Poland

Training for leaders of rare disease patient organizations

22-25.10.2024
Barcelona, Spain

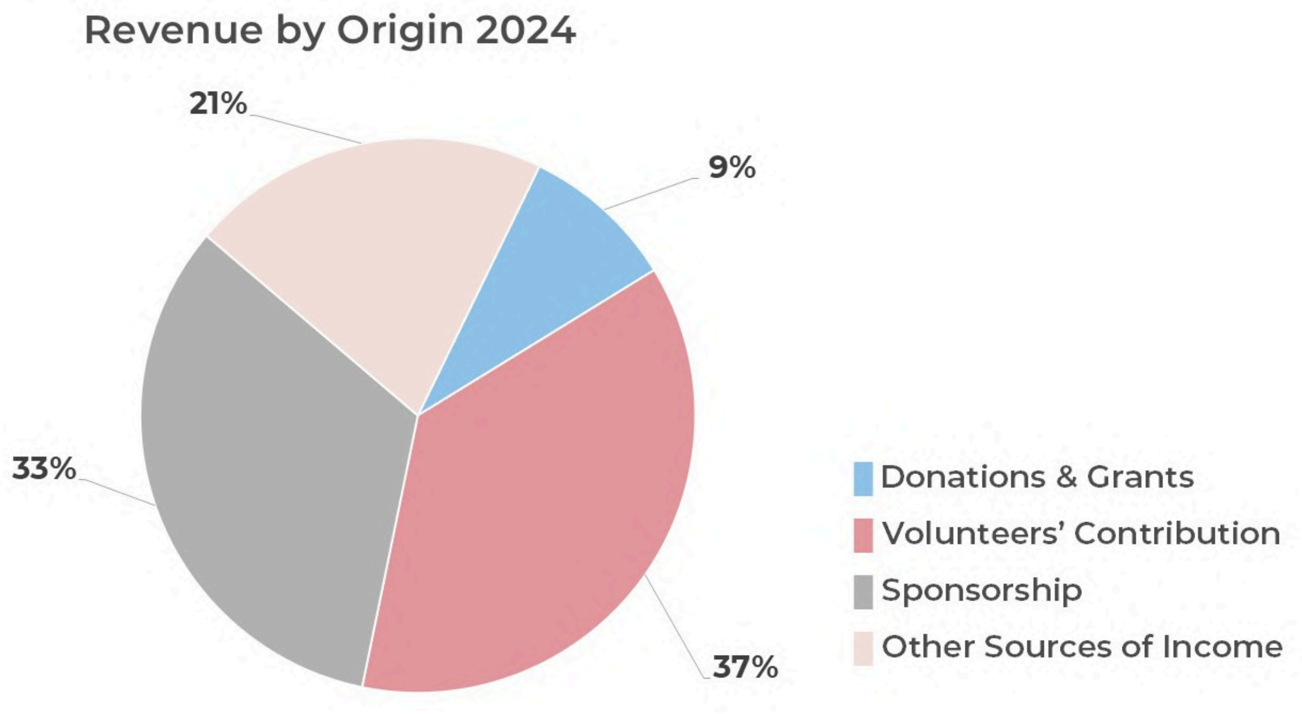
The World Orphan Drug Congress Europe 2024

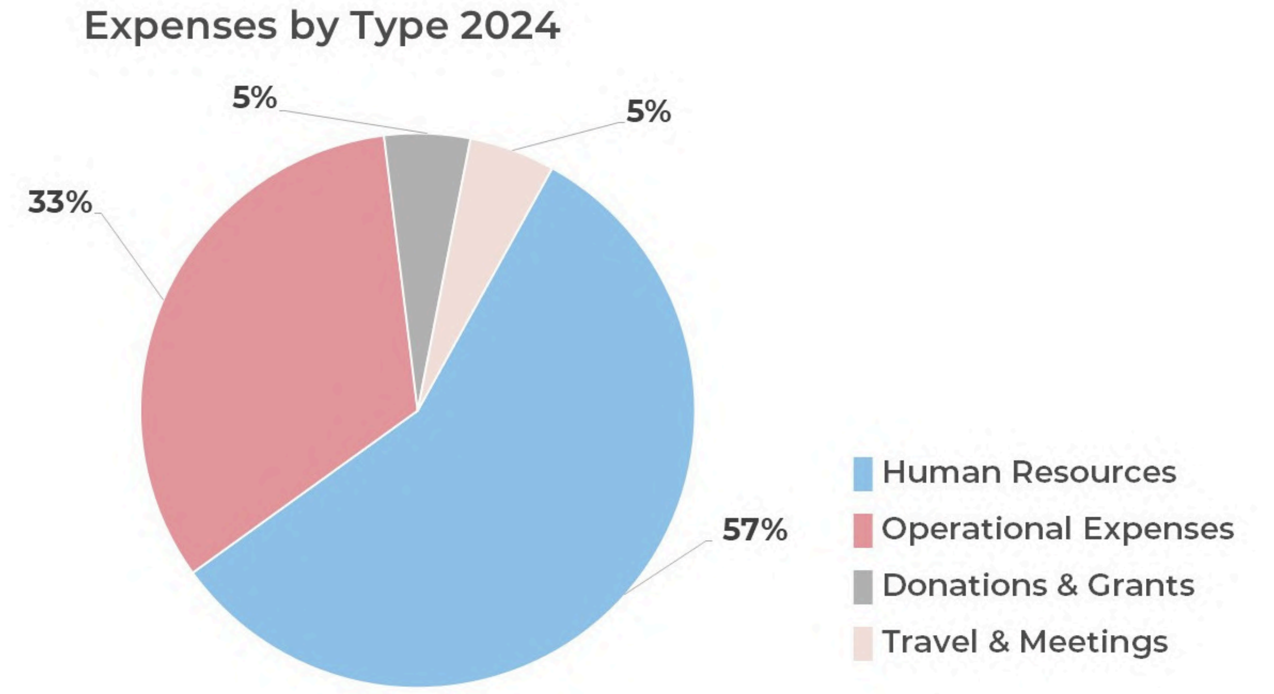
21.11.2024
Vienna, Austria

Access to Care Conference

Transparency

We are a non-profit & VAT-exempted foundation based in Poland. Below you can find information about our funding in 2024.





Partners, donors & sponsors

Collaboration is at the heart of our work. The support of sponsors, partners, and fellow NGOs makes many of our projects possible. We want to extend our heartfelt thanks to all of you for your commitment and collective efforts to support patients with rare diseases worldwide.

Industry:

AMGEN

Amicus
Therapeutics

Boehringer
Ingelheim

CSL Behring
Biotherapies for Life®

Komtur

octapharma

Ovid™
Therapeutics

SO CONNECT

Non-profit organizations:

Global Genes®
Allies in Rare Disease

EURORDIS
RARE DISEASES EUROPE

Stowarzyszenie Rodzin z Chorobą Fabry'ego

ОРФАННІ ЗАХВОРЮВАННЯ УКРАЇНИ
ГРОМАДСЬКА СПІЛКА

RID

Public entities:

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«ОХМАТДИТ»

Media:

RARE REVOLUTION
MAGAZINE

EDU

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