



2022

Global Genes RARE Impact Grants Report

[GLOBALGENES.ORG](https://www.globalgenes.org)

Global Genes Grants

Over the past decade, Global Genes' RARE Impact Grant Program has provided funding opportunities for rare patient organizations that are a part of the Global Advocacy Alliance to make specific, tangible differences in the communities they serve. These funds support and steward initiatives to develop educational tools and resources to improve health equity in underserved and underrepresented populations, convene rare disease stakeholders, empower people to improve their financial health and financial literacy, and provide urgent relief for rare disease patients and families impacted or displaced by the war in Ukraine.



28
Organizations
Supported



29
Grants
Awarded



\$415,483.50
Awarded in
Grants

In 2022, Global Genes supported and stewarded 29 grants, totaling more than \$415,000, to 28 organizations addressing:

Health Equity

Addressing Diversity, Equity, Inclusion, Accessibility
in the Global Rare Disease Community

\$113,786.00

RARE Meet-Ups

In-Person Meetings & Community Building Opportunities

\$70,912.50

Financial Advocacy

Understanding & Navigating Financial Challenges

\$60,785.00

RARE Relief UKR

Supporting Organizations Providing On-The-Ground
Support for Ukrainian Families

\$170,000.00



Receiving the generous grant from Global Genes was enormously impactful for the 3rd GAND Gathering & Scientific Conference. The grant allowed us to keep registration costs for families low, so that more families could attend the conference. It also helped make the conference more robust with the presence of Dr. Pierson, an under-funded scientist who traveled from California. The funding also allowed meals for the families attending, as well as high-quality "Life with GAND" books. In short, the extra funding allowed us to make a conference that was substantive, meaningful, and comfortable for all.

- Helping Hands for GAND

Thank you to the organizations who made these grants possible





Health Equity in RARE Patient Impact Grant

The Health Equity in RARE Patient Impact Grant Program, part of the larger Health Equity Initiative, provided an opportunity for patient advocacy leaders to improve outreach strategies and address challenges that affect underserved and underrepresented patient communities. This grant was awarded to Global Advocacy Alliance members who were interested in expanding their outreach, education, and awareness efforts supporting cultural awareness and humility. The grant gave foundation leaders and patient advocates the ability to stand up data collection tools, resources, and processes to better engage or understand the needs of their underserved communities, ultimately leading to more robust data sets for their rare disease, established standards of diagnosis and care, and a more comprehensive understanding of their disease and patient journey. Projects addressed inequities including but not limited to race, ethnicity, age, gender identity, religion, socioeconomic status (income, level of education, and occupation), geographic location, and/or linguistic barriers. Outcomes included the development of research, tools, resources, and programs that provide guidance and assistance to rare disease patients and their families.

In 2022, Global Genes received 72 applications for this grant opportunity. Eight of the 72 applicants received funding. The grantees were awarded up to \$15,000 or 50% of their annual revenue. Awardees included:





\$113,786
Awarded



72
Applications



8
Awardees

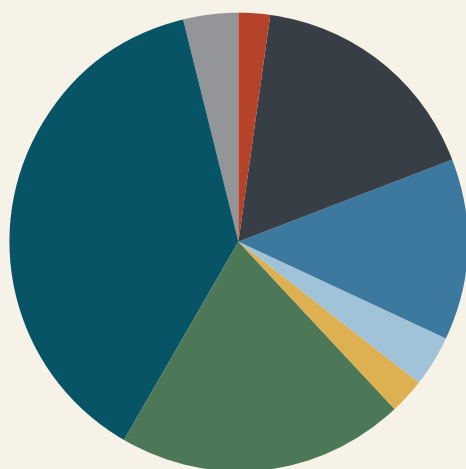


+12,000
Reach

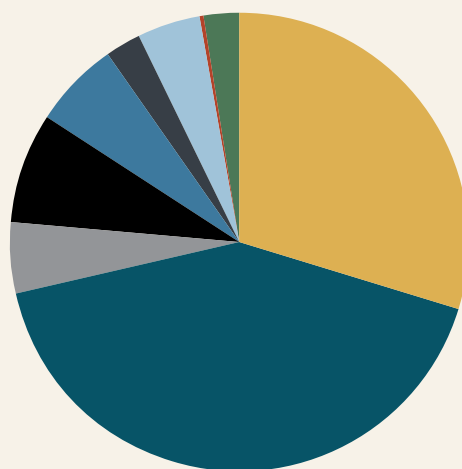


The project has opened our eyes to the needs and that this is just the start of our commitment to not only community members with the identified linguistic and geographic needs, but also the many other factors underserved community members face. - **Myhre Syndrome Foundation**

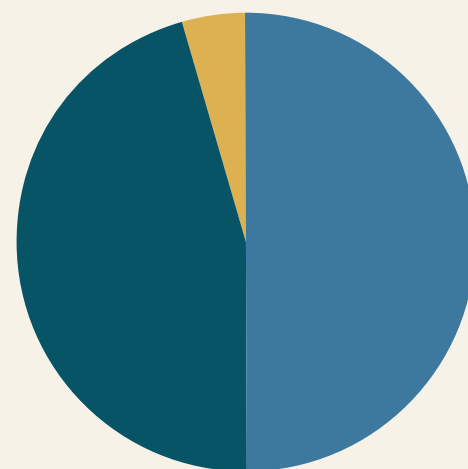
Awardees - Ethnicity breakdown



Awardees - Language breakdown



Awardees - Gender breakdown



American Indian or Alaska Native - 2.5%

Asian - 16.7%

Black or African American - 12.8%

Middle Eastern or North African - 3.7%

Native Hawaiian or Pacific Islander - 2.4%

White - 20.3%

Hispanic / Latino/a/x / Spanish Origin - 37.9%

Other - 3.7%

English - 29.8%

Spanish - 41.7%

Hindi - 5.1%

Mandarin - 7.8%

French - 6.0%

Italian - 2.7%

German - 4.4%

Urdu - 0.1%

Arabic - 2.5%

Female/woman/girl - 50.1%

Male/man/boy - 45.7%

Other - 4.2%

Thank you to the organizations who made these grants possible



International Prader-Willi Syndrome Organisation



International Prader-Willi Syndrome Organisation subtitled educational films and translated consensus documents into Mandarin, Turkish and Arabic for patients, caregivers, and healthcare professionals who may currently or in the future support people with Prader-Willi Syndrome (PWS) and their families. The organization offers translated guides in more than 20 languages on their website.

3 blog posts about this project can be found here:



The translated and interpreted resources are on topics that are essential for caregivers and health providers supporting people with PWS to understand. These include resources on diet and nutrition, exercise, and promoting positive behavior, all of which are of daily relevance to caregivers. By producing and widely circulating documents oriented towards medical professionals by means of PWS networks, families will be able to offer current and reliable information to the medical professionals supporting their family members with PWS. This is in addition to the medical professionals who will be sent the information directly by IPWSO. This is very important in the context of rare diseases in countries where most medical professionals will never have encountered someone with PWS.

Impact

By introducing healthcare professionals and caregivers to these resources, people will have access to free services provided by IPWSO. This could include diagnostic testing service, advice line service, grants for conferences and workshops, and online mentoring programmes. These services are all designed with the goal of improving the quality of life of people with PWS and their families.

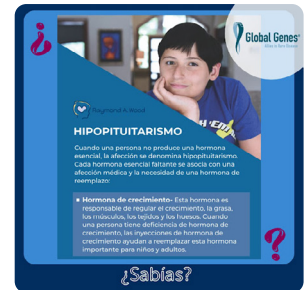
Following a recent request from the Egyptian PWS Network, they are hoping to offer a series of webinars that will be interpreted into Arabic in 2023. These will be designed specifically to meet the needs of English and Arabic-speaking families of people with PWS in Africa.

Raymond A. Wood Foundation



Raymond A. Wood
foundation

Raymond A. Wood Foundation created a website, pamphlet, and video series through its Spanish Language Education and Outreach Program, which provided access to educational resources for the Spanish language population that is affected by craniopharyngiomas. The Spanish resource website offers information, resources, and ways to learn about how they can support patients and caregivers. The Spanish resource pamphlet was sent to 30 doctors and medical facilities for distribution. Resource videos were translated into Spanish, and posted on the website and social media posts.



Spanish language patients and caregivers will learn how the foundation can support them through its programs. The success of this outcome was shown through two new applications by Spanish speaking families for the blood sodium meter program. This program supports pediatric patients facing the most challenging aspects of diabetes insipidus. Additionally, they have seen an increase in caregiver participation in support groups by 50% since the beginning of the program.



RAWF will increase networking with endocrine clinics (doctors, nurses, social workers, clinic representatives) to expand awareness of outreach programs and strive to meet the needs of all hypothalamic-pituitary brain tumor patients and caregivers. While they did increase networking with social workers and some doctors, networking to doctors did not increase as significantly as they had hoped. RAWF hopes to continue to expand knowledge of work through the community, patients, medical conferences, and current connections within the medical community.

Impact

- Spanish language patients and caregivers will use first language resources to make informed decisions that will lead to better medical care.
- Through feedback from Spanish language program participants they will be able to understand additional needs and develop programming for continued support. They have begun to get initial feedback regarding additional barriers to care faced by Spanish speakers such as access to care, delay of diagnosis, citizenship hardships, and financial resources. The concerns brought to the organization's attention has increased awareness of these additional barriers and allowed RAWF to begin trying to determine how they can best support Spanish speakers (and others) facing additional barriers and challenges.
- The program will be a catalyst that will assist RAWF in expanding first language resources, programs, and services for other underserved populations such as Arabic and Mandarin speakers. RAWF views the Global Genes grant as a catalyst for future outreach to caregivers and survivors in the community who need resources in first or preferred languages such as Arabic and Mandarin.
- The newly implemented three year strategic plan aims to increase outreach and resources for underrepresented populations within the community. The Global Genes grant helped them start to achieve that goal, and has been a catalyst in outreach efforts. This program was invaluable in connecting with the current Spanish speaking population, and increasing outreach to new Spanish speaking caregivers and survivors.

Myhre Syndrome Foundation



Myhre Syndrome Foundation's 4You project translated their website into Spanish, German, French, and Italian, and also provided translation of key informational documents to bring greater inclusion and understanding, to patients, caregivers and associated care teams and providers.

Of the 81 Myhre Syndrome Patient and Family Handbooks requested from July through October 2022, 65% of requests were from countries outside of the U.S., and 25% of requests were for Spanish, French, German, and Italian. There were 426 translation requests for the MSF website, and MSF experienced an increase in visits to the MSF website for these languages: Spanish 174%, German 705%, French 670%, Italian 270% (these figures are based on the data 3 months before the website was activated with the translation option and 3 months after).



While Myhre Syndrome Foundation was established in the U.S., they have always functioned as a global organization, historical feedback was that they were centered on U.S. audiences, and this feature now helps demonstrate their commitment to the entire global audience. As evidence, earlier this year the funding of a research study in Belgium recognized the organization's international research relationships.

Myhre Syndrome Foundation had five families reach out and share more of their story. Since the handbook launched and the website has been translated, they had nine more families sign up to the Myhre Syndrome Patient Registry, taking the total number to 90. This was promoted and explained in the handbook and so has had a positive impact on communicating the power of patient data.

Impact

- Myhre Syndrome Foundation had enquiries about supporting Dutch and Portuguese speakers. The organization will look to further support these requests with specific fundraising and/or other grant opportunities.
- The impact on Myhre Syndrome Foundation as an organization is that it has internally promoted many more conversations on DEI practices and increased the awareness of the impact geographic and linguistic barriers have on the community.
- The project has opened the eyes of the organization to the needs of community members with identified needs of language and geography, but also many other obstacles underserved community members face.



I love it, thank you so much for bringing this together in one place. I can now refer to this handbook time and again and take it to appointments too.

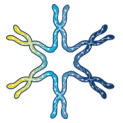


I think this is a very helpful handbook, especially for those newly diagnosed.



The Handbook is changing lives! Thank you so much for creating this first of its kind tool for our Myhre Community.

Team Telomere, Inc.



Team Telomere
An International Community for
Telomere Biology Disorders

Team Telomere developed and translated critical material into Spanish and French, creating more

accessible resources through effective distribution, and forming systems for sustainable community outreach. Materials covered the diagnostic journey, disease management, and evidence-based treatment. The organization also created and offered effective and accessible routes of community connection through strengthening the network between patients, care teams, and the patient advocacy organization. Live translation was available at Family Chat and connections with networks were made, shining light on the importance of these concepts and issues. In addition, the organization was able to distribute translated care packages, educational material, and community resources to partner institutions and families.



In the short term, the organization saw a significant increase in the number of individuals with Telomere Biology Disorders served. They were able to connect with individuals in the community that the organization previously had no connection with. They connected with parts of the Spanish speaking community that the organization was not connected to in the October Family Chat. Additionally, they hosted a French Family Day, and patients were so appreciative that there were now resources translated into French. Moreover, while Team Telomere has a strong network within the English-speaking population as well as in the regions that they have sent global ambassadors, this network is more segmented within the Spanish speaking community. They were able to bridge this gap by expanding the team to include a Hispanic/Latino Ambassador, Mayra. The organization took what they learned to the Scientific Conference, and connected with the medical and scientific community to share findings as well as hear their experiences and suggestions for action moving forward. They tracked the areas of greatest resource utilization and have focused conversations with the target audience. This led the organization to change the forms that the organization will use to include a translation resources question included in all future calls and webinars.

Impact

- Since the community is now able to access critical information in a manner that they understand, there is more trust in the care received and the health care system. This impact has spilled over to care teams and the care that they provide. Care teams have reached out to them for translated educational and community resources as they are able to use these resources to better explain TBDs, disease progression, management, and care while offering care and comfort through this rare disease journey.
- Current research on Telomere Biology Disorders is limited in non-White, non-English speaking populations. Since conducted research and clinical trial enrollment is not representative of the population impacted by TBDs, outcomes cannot be translated. However, with increased understanding and more resources, the organization anticipates that there will be more reach and research done on non-White populations, which will make research efforts more equitable and translatable to all populations.

PFIC Network, Inc.



PFIC Network organized and hosted a series of virtual one-on-one meetings and six workshops for patients in India and Pakistan, utilizing interpretation services to break linguistic barriers and increase patient representation in their global patient advocacy program. Through this project, the organization created a needs assessment survey incorporating a validated instrument. The survey results and workshop discussions produced country-specific advocacy action plans (one for India, one for Pakistan). PFIC Network shipped Hindi and Urdu PFIC Educational Brochures to the Indian and Pakistani hospitals. They also gained a deeper understanding of wealth disparities within both countries that further impact quality of medical care and quality of life for PFIC and other rare disease patients.

Results from the survey and workshop discussions sparked new conversations with healthcare providers on near-term initiatives that could be made much more feasible with the help of their expertise and professional networks. The workshops also provided a platform for patients and healthcare providers to interact directly and exchange perspectives on obstacles facing PFIC treatment in their native languages. As a result, patients, healthcare providers, and PFIC Network established relationships that will be essential to the collaborative multi-stakeholder effort necessary to addressing the needs of patients in those countries.

During the workshops, healthcare providers encouraged patients to create consistent support group meetings and register formal advocacy organizations based on the success they have seen these activities produce in other rare diseases in their countries (e.g. Lysosomal Storage Disorders). These same providers also offered to help in organizing such groups and making them accessible to other PFIC patients at their centers.



Impact

- The PFIC stakeholder community is global, and this project revealed that the REACH framework can be successfully implemented to help address the unique challenges of underserved patients in developing countries. PFIC expects the program's success in India and Pakistan to stimulate requests globally. PFIC Network will actively execute this framework for members of the International Alliance, to make PFIC Network's mission and advocacy objectives more equitable and accessible for all.
- Project REACH also increased diversity in PFIC Network's patient and healthcare provider engagement. Historically, the most engaged patients live in the U.S., Canada, and Italy – but this project revealed that they can stimulate more inclusive and broader-reaching engagement by taking active steps to make programs more accessible through direct outreach and targeted education. This project also empowered patient leaders by breaking down linguistic barriers so they could speak to their struggles in their own language and truly feel heard and understood. This coordinated multi-stakeholder attention and strategy is a resource that PFIC patients in India and Pakistan have never had before.
- The healthcare providers involved in both countries provided invaluable feedback and resources, including additional interpreters through personal contacts for Tamil and Telugu. They connected the organization with many more patients than the organization could have originally reached without their help, inspired trust between patients and the PFIC Network, and provided insight into the survey results that contributed to realistic strategy creation for the advocacy action plans.

SCN2A Asia Pacific



SCN2A Asia Pacific translated their “What is SCN2A” and “Clinical Trials - All You Need to Know” brochures into Chinese, Japanese, Korean, and Hindi, and developed a clinical trials microsite translated into the same languages. In addition, the

organization developed a contact list for 39 clinicians across the region, and shared resources with these new contacts to share with SCN2A families.

Data gathering through this increased outreach will expedite the ability to build a case for greater resource allocation and decipher the specific needs of families in different countries. With clinical trials for SCN2A beginning later this year and having a better understanding of SCN2A patients in the region, they can better advocate for equity of access to clinical trials in the region.

At the beginning of this project, SCN2A Asia Pacific connected with regional families about the resources and asked for their input. The co-design engagement of the project was not at the level they had hoped for when they set out, but this project has taught them valuable lessons and now has more depth to the access and equality policy, which is under review.



Impact

- This grant has given the organization the resources to branch out and it expands reach that would have taken them much longer than the time frame in this project.
- They have shared the clinical trials website with other regional SCN2A groups who are looking to partner with them to ensure the page is as accessible as possible. To this end, the organization extended the number of languages on the website translation to Spanish, German, Italian, and French. This impact will build stronger global collaborations which are imperative in rare diseases.
- The organization has expanded professional networks and built a presence and level of respect as a leading patient organization in the region. This will help them work more closely with clinicians and researchers.
- They have had messages of gratitude from family members that they have not been left behind - that they are taking them along and ensuring all families feel supported, and that they have the right tools to be able to advocate (and educate) on this rare condition that their family member has been impacted by.
- The organization has been able to utilize the networks they previously had to amplify the work and also produce a more professional resource. This has led to signing an agreement with a medical writing and communications company to assist them with further work in this area.
- Additionally, SCN2A Asia Pacific has started conversations with the industry to build stronger partnerships with those who are working on research and/or clinical trials for SCN2A. They will ensure the information is kept up to date and inline with the policy for the website and organization.

Cystic Fibrosis Research Institute



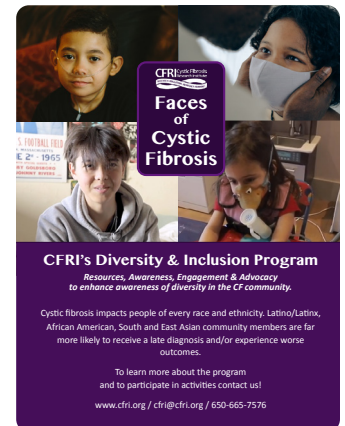
The Faces of CF Diversity and Inclusion outreach project advanced CFRI's goals to expand awareness of cystic fibrosis and its symptoms among communities of color, while raising awareness of diversity among the CF community and medical care providers. CFRI created informational flyers that noted the

incidence of cystic fibrosis among people of color, and outlined the symptoms of the disease. The flyers encouraged those who were experiencing these symptoms to push their doctor to test for cystic fibrosis. They created a podcast in Spanish featuring two Latinx mothers of children with cystic fibrosis who described the diagnostic journey and the tools and resources that helped them. They subtitled numerous podcasts and conference presentations in Spanish and Hindi to expand resources.

CFRI expanded and enhanced existing printed resources – including bilingual informational flyers, and the extremely popular CF in the Classroom/Fibrosis Quística en la Clase, which for years has remained the most requested printed resource. They expanded the number of podcasts available in Spanish, or subtitled in Spanish and Hindi. By offering expanded resources in multiple formats, CFRI is addressing the different learning styles of the community.

They are addressing mental health issues that are common in the CF community – particularly symptoms of depression and anxiety. Through the monthly online support group, they provide mental health support to Latinx members of the community who are more comfortable communicating in Spanish. For people of color with CF, it has been expressed that they feel that they are rare within rare, and the offering of the peer-to-peer support group facilitated by a CF social worker has been welcomed by participants.

CFRI is bringing the issue of diversity to the forefront among CF care providers through surveys, the provision of materials to CF centers for distribution to their patients and families, and in the near future, the offering of a diversity and inclusion workshop to providers nationwide. Based on the positive response to a survey of social workers, and their identification of resources needed by their patients and families of color, they now have new goals and objectives moving forward. While not an outcome, they will also survey Spanish-speaking patients to identify needed resources.



Impact

- CFRI's Faces of CF Diversity and Inclusion Program is further advancing the organization's goals to expand awareness of diversity among the CF community and medical care providers, while raising awareness of the disease and its symptoms among communities of color. With greater awareness, people of color will be more likely to receive testing for CF, and have access to early interventions that can improve health and quality of life.
- The project has inspired staff, Board, and volunteers to generate new ideas on ways to best meet the needs of all members of the community, as well as ideas for educating the broader community about cystic fibrosis and the community's diversity. Greater thought is put into flyers, social media posts, and website updates, to ensure that all members of the community who access these resources will feel welcomed and included.
- CFRI will educate funders about the vital need for inclusivity, and anticipate their shared goal to expand access. CFRI's leadership has been and remains fully committed to expanding revenues to cover the costs associated with ongoing outreach and expansion of resources.

COMBINEDBrain, Inc.



COMBINEDBrain translated the ClinGen Surveys into French, Italian, and German, which reached an additional 220 million people. ClinGen medical surveys are internationally acceptable by the medical and research community, and collected on other large platforms, such as RARE-X. In

the short term, with these translated materials, engagement from the international and immigrant communities in patient-entered natural history studies will be significantly improved.

COMBINEDBrain shared the translations back to the ClinGen Project for dissemination around the world. Breaking down language barriers is necessary in order to include the experience of diverse populations in health research. Increasing diversity led to improved research, diagnosis, standards of care, and a more comprehensive and inclusive understanding of disease and the patient journey.

COMBINEDBrain plans to share these translations with all other researchers and foundations directly, and through the Matrix and ClinGen platforms. Matrix made the translations available to additional Patient Advocacy Groups (PAGs) outside COMBINEDBrain that also use the Matrix platform. ClinGen shared translations with other associated registries and will make the surveys available on the ClinGen website. COMBINEDBrain shared translations with other rare disorder umbrella groups (such as NORD, Global Genes, Eurodis, and RARE-X) and communicated the availability of the surveys to their member organizations.



Impact

- The inclusivity and equity of participation in natural history studies made possible with these translated ClinGen surveys prove to families around the world that their experiences matter. More healthcare providers in non-English speaking countries have been encouraged to participate in developing clinical guidelines and conducting clinical trials, which helps to create trust and encourage more patient and caregiver participation in clinical research, ensuring that research priorities are reflective of the needs of the wider rare disease community. Setting the right research priorities is vital to accelerating progress toward treatments and cures that have clinically meaningful outcomes for the patients.
- COMBINEDBrain was able to delve deeper into the inequities that the PAGs face when trying to collect data and properly represent their population. The collaboration allowed the group to better understand how to identify and solve an inequity within their realms.
- PAGS will have more voices heard and therefore more data available for research.



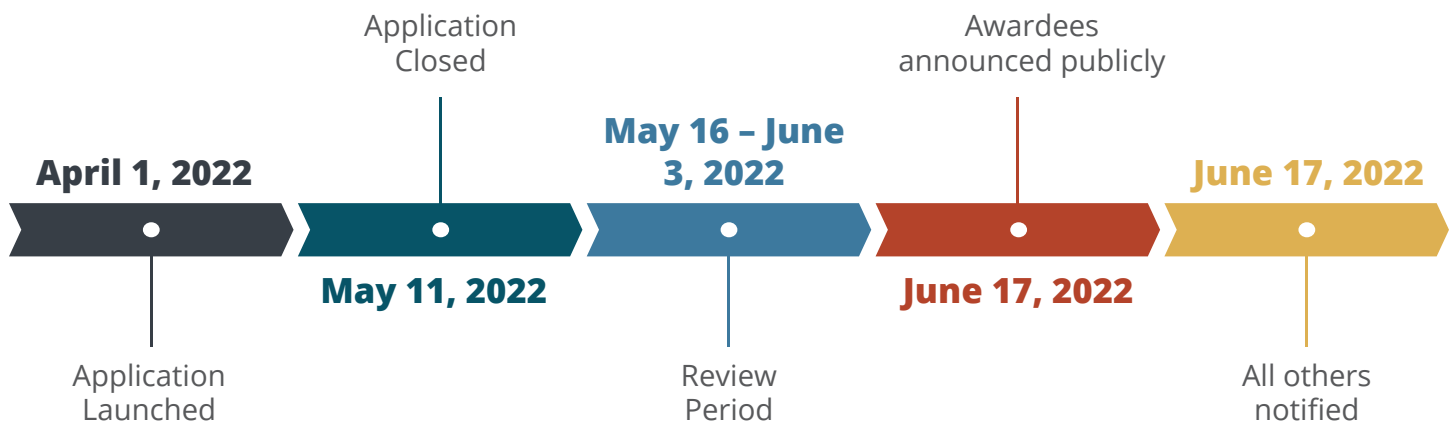
Financial Advocacy in RARE Patient Impact Grant

A part of the Global Genes Health Equity Initiative, the 2022 Financial Advocacy Patient Impact Grant program empowers patient advocacy organizations to educate their communities on tackling issues that impact financial well-being and financial literacy for patients.

Through this grant program, proposed projects addressed cultural, socioeconomic (income, age, level of education, religion, and occupation) and/or linguistic barriers and factors that contribute to the community's limited access to and understanding of financial literacy and financial well-being. This was done through research or development of tools, resources, and programs that provide guidance and assistance to underserved and underrepresented rare disease patients and families as they encounter financially stressful situations that may impede access to diagnosis, therapy, and disease management.

In 2022, Global Genes received 40 applications for this grant opportunity. Nine of the 40 applicants received funding. Grantees were awarded up to \$7,500 or 50% of their annual revenue. Awardees included:





\$60,785
Awarded



40
Applications



9
Awardees

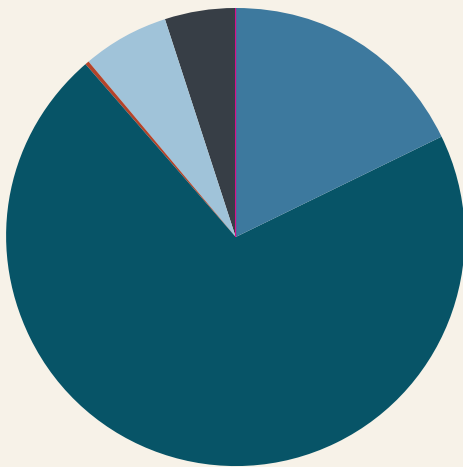


+150,000
Reach

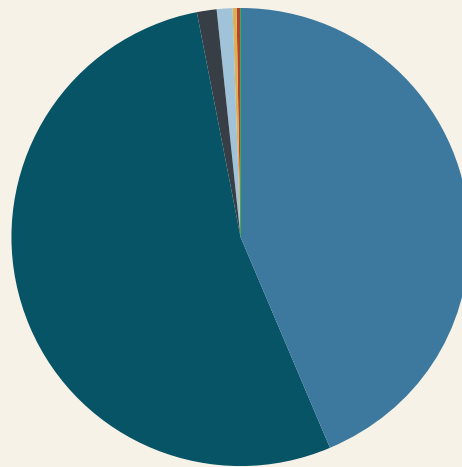


This gives us actual data that has confirmed the reports from lived experience of many of our patient families. The data will be useful as we disseminate to health professionals and scientists to understand the burden beyond physical care and psychosocial impact. - [The Global Foundation for Peroxisomal Disorders](#)

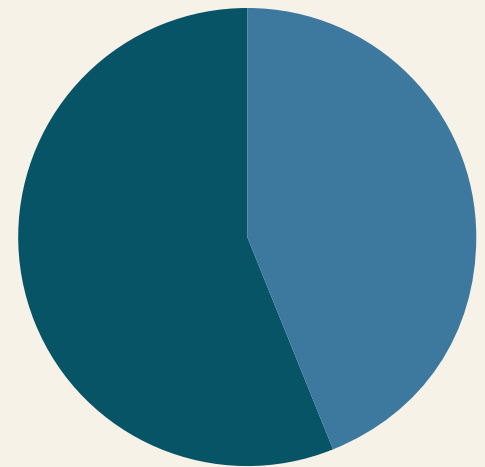
Awardees - Ethnicity breakdown



Awardees - Language breakdown



Awardees - Gender breakdown



Thank you to the organizations who made these grants possible



Prader-Willi Syndrome Association – USA



Prader-Willi Syndrome Association – USA (PWS) created a free downloadable kit of materials called “[Rare\(ly\) Missed Opportunities: Your PWS Financial Guide](#)”, to provide resources addressing both the direct and indirect financial issues that PWS caregivers may face. This important resource was distributed to over 35,000 constituents via email and social media, and includes:

- An interactive state-by-state map highlighting waiver information
- Downloadable PDF documents that provide an overview of FMLA, ABLE accounts, SSI, SSDI, and Early Intervention
- Videos providing a framework and information about Financial Planning and Special Needs Trusts
- A recording of a webinar providing step by step instructions on navigating the resources

Outcomes

- The launch of this project and resulting materials are helping make critical information more readily accessible to PWS caregivers. Families are better able to ascertain what help is available to them by state and access vital information parents and caregivers need to navigate through the challenging financial impacts of this diagnosis.
- New customer relationship management tools measure increases in family support calls related to Financial Support/Benefits, and the organization will be watching this closely. They plan to perform comparisons of the data quarter to quarter and year to year as these resources are shared more widely.
- Two professionals who presented in videos have offered to work with families one on one to talk through their financial needs and concerns.
- PWS was able to develop critical relationships with CMS (Center for Medicaid Services) and the Departments of Health and Human Services (or equivalent) within each state, as they did state-by-state research, allowing them to better assist families as they navigate each state’s process.

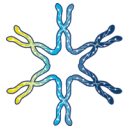


Impact

As a result of these ever-growing resources in the toolkit, families feel more informed and empowered to get the financial support that they need, and (for families starting out) will be able to avoid many of the problems that come with a lack of planning and support. The toolkit has provided these resources and benefits:

- Caregiving options, including waivers that provide increased in-home caregiver support (respite, etc.), and opening the door to new residential solutions providing relief for long-term parents/caregivers.
- Families of children with PWS (ages 0-3) are better informed about the benefits and cost-savings that come with state or federally funded Early Intervention and in-home healthcare nurse services.
- More families have the foresight and knowledge to plan for their loved one’s financial future (ABLE account, etc.).
- More parents/caregivers are able to access FMLA as well as other employer-provided benefits to protect employment and (as a result) ensure family financial stability.

Team Telomere Inc.

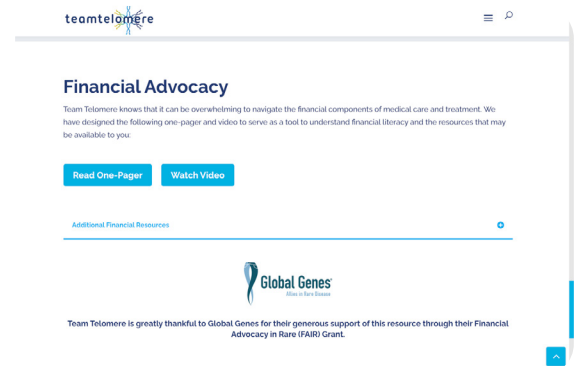


Team Telomere
An International Community for
Telomere Biology Disorders

The Financial Advocacy Patient Impact Grant allowed Team Telomere Inc. to complete a three-phase project that included financial literacy and well-being resources, a one page resource sheet, and a webinar on healthcare financial literacy, spanning all types of mediums (web resources, visual resources, and audio resources). Team Telomere have estimated that these resources have already been utilized by approximately 100 community members, and project that this resource will assist 350 people in the community.

Outcomes

- Individuals in the community have an increased sense of agency and empowerment to make informed decisions that support their financial wellbeing. With the burden of navigating financial advocacy and literacy taken off of individuals, they have more empowered confidence and are able to make better informed financial decisions.
- Team Telomere has expanded capacity as a patient advocacy organization, and are now better equipped to serve the needs of the community by directing families to a variety of resources that they know can greatly help them.
- These resources are also being utilized by clinicians, social workers, and other members of the care team, as they help families navigate complex management and treatment.



Video Webinar

What is financial literacy?

Team Telomere's Financial Advocacy & Literacy Resource Roadmap

Impact

- The developed resources are geared towards the disease community and their unique needs. In the long-term they can see that this resource will be part of the care management plan of individuals to build their own unique financial advocacy and literacy roadmaps.
- Team Telomere is certain that this project will go great lengths, and they hope that at least 90% of the community will interact with one or more project deliverables. They also foresee that at least 80% of those individuals will have a greater understanding of financial literacy and financial literacy. The organization anticipates at least 70% of individuals will utilize one or more tools/lessons that they have outlined for the betterment of their financial advocacy.

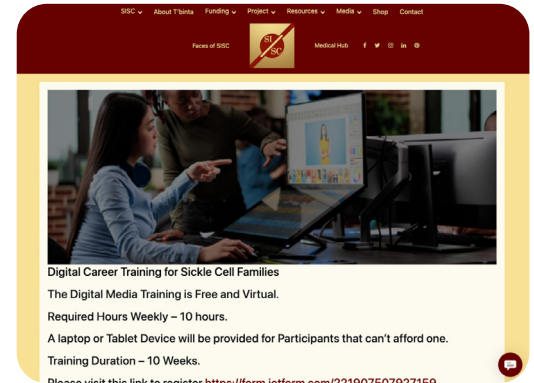
Alaafia Women's Corporation



Alaafia Women's Corporation provided those living with Sickle Cell disease with training and resources for digital careers that will lead to financial independence. Through this program, twenty-five participants were able to explore career options that have lowered levels of mental stress.

Outcomes

- Alaafia provided three weeks of basic computing training via North Star for all the participants, as basic computing is a requirement of a digital training program, which many participants lacked.
- Participants agreed that this training should be a standard educational learning method for people with Sickle Cell.



Impact

- Alaafia Women's Corporation is in the process of registering the digital vocational center for accreditation.
- Once accredited, they plan to expand this program in the next two years.
- The organization encouraged participants to keep pushing and provided them with the support they needed with resources.



At Alaafia, we believe that financial independence is crucial for Sickle Cell families to thrive, and we are grateful to Global Genes for making this possible through the grant. With this support, we empowered families to take control of their financial futures and focus on what really matters - their health and well-being. - Alaafia Women's Corporation



As a Sickle Cell warrior, I never imagined having a career that could be pursued from the comfort of my own bed. Thanks to the Global Genes grant, I was able to obtain the skills necessary to become a Graphic Designer and pursue a flexible and virtual career pathway. This grant has not only provided financial support but has also given me the opportunity to create a life that is fulfilling and empowering. - Sickle Cell patient

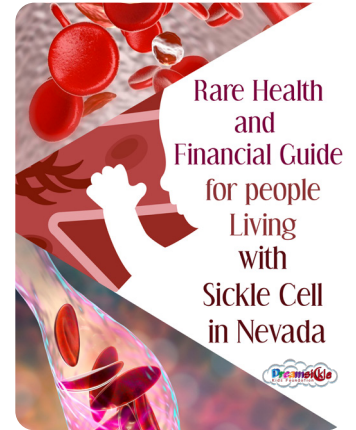
Dreamsickle Kids Foundation



Dreamsickle Kids Foundation created a digital book for the 1,000 patients with Sickle Cell Disease (SCD) in Nevada and their caregivers. The digital book provided resources with emphasis on the coverage benefits that could be of direct benefit to patients with SCD, which ultimately addressed health equity and financial literacy needs in this community.

The ebook provides information for improved knowledge on health insurance coverage, empowers patients to make better use of their health insurance, includes information on options to maximize disability benefits, and provides financial information to empower them to make better financial decisions and promote health literacy.

In addition to being available online through the Dreamsickle Kids Foundation website, postcards and flyers have been made available at medical facilities, and taken to community engagements for patients to be able to access anywhere at any time. Workshops were created to further discuss needs within the community, and answer questions and provide clarity on the information in the ebook.



Impact

- The 1,000 patients with Sickle Cell Disease in Nevada will be equipped with information to better advocate for themselves with providers and medical insurers.
- Medical providers and insurers and the major health insurance companies will have access to a simplified overview of the SCD bill, which they are required to follow, to provide the best healthcare management and coverage within policy guidelines created through legislation.
- By creating this ebook, it allowed them to take further action in legislative advocacy to ensure that the entire Nevada SCD population is aware of the changes and has access to the information.
- This project has given the organization the confidence to further expand the resources provided by Dreamsickle Kids Foundation, including links to SCD specialty providers in Nevada, a list of qualified SCD community health workers in the state, and a list of SSI lawyers in Nevada who may be able to assist in receiving SSI.

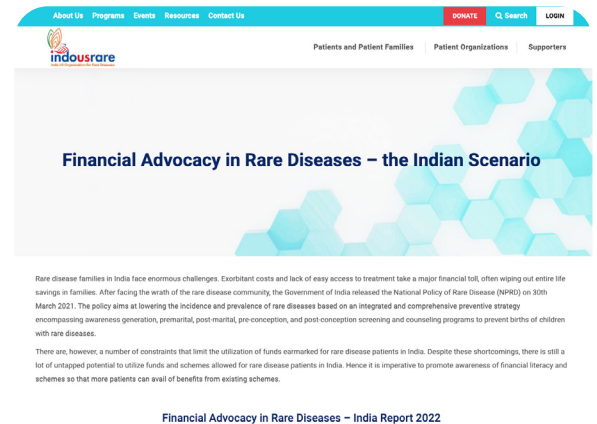


I am grateful for the financial health guide created by Dreamsickle Kids. I was unaware of all of the wonderful health benefits enacted due to the passing of AB254 (SCD bill in Nevada), one being coverage for supplements like the Vitamin D and Folic Acid I take daily and pay an out of pocket cost for that can easily add up to \$50 or more a month even with Medicaid. Because of learning of the coverage for the supplements in the eBook, I was able to contact my health insurance carrier and make sure my supplements were covered in full going forward, and I was able to get a reimbursement for my last 3 payments for supplements! " – **Naja Bagner, Nevada Sickle Cell Patient**



IndoUSrare determined the availability and accessibility of various schemes for financial assistance for rare diseases in India, created educational resources based on the findings, and created awareness on available financial schemes among the rare disease community in India.

IndoUSrare has compiled a [list of financial resources](#) available to rare disease patients in India that will be updated as more information becomes available. In addition, a comprehensive report was generated using information gathered from a combination of literature analysis, surveys (information requests), and stakeholder interviews, and is available to download on the IndoUSrare website. Lastly, IndoUSrare conducted a webinar to consult with experts in the field to understand their perspectives on financial awareness and to discuss ways to access existing financial schemes in the country. The recording of the webinar is available on the IndoUSrare [YouTube channel](#) and [website](#).



Video Webinar



Impact

- The study summarizes the current understanding of awareness and accessibility of financial aid available in India. They learned that the greatest financial burden for families is in accessing treatment.
- Increased awareness of available government and non-government avenues of financial support for rare disease patients, and a better understanding of the eligibility for these schemes, through the report and webinar.
- A better understanding of the barriers to the release of funds from the Government for rare disease treatment.

Foundation for Sarcoidosis Research

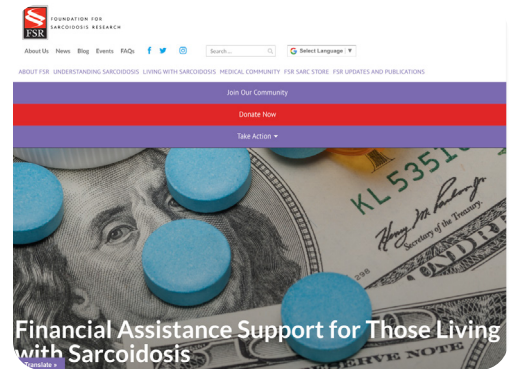


FOUNDATION FOR
SARCOIDOSIS RESEARCH

Foundation for Sarcoidosis Research (FSR) surveyed its community to gain a deeper insight of the financial challenges experienced, and in turn, educated and addressed those challenges through a new webpage and a financial planning educational webinar featuring financial expert Martin Shenkman. FSR also created an infographic

with financial and estate planning information for those living with sarcoidosis and their loved ones. The new financial assistance page includes a prescription assistance card, a link to the webinar, basic financial and estate planning steps, the financial educational infographic, and links to patient assistance programs.

FSR identified financial knowledge gaps and concerns around financial planning from those in the community and through the development of the financial website and infographic, FSR was able to provide more resources and information to help close the financial knowledge gaps. This helped increase the overall financial planning knowledge among community members. Through this program, FSR was able to provide resources to help increase knowledge around prescription assistance programs. The financial resources webpage includes a prescription assistance card from a proud partner, NeedyMeds, and a list of Patient Assistance Programs that provide financial assistance for drugs included in approved treatment guidelines of treatment options for sarcoidosis. This page also lists patient resources such as information about a co-pay assistance program, rare disease education support, social security and disability benefits, and veterans' affairs disability information. In addition, this project included promotion and marketing of these resources including an e-blast and social media posts.



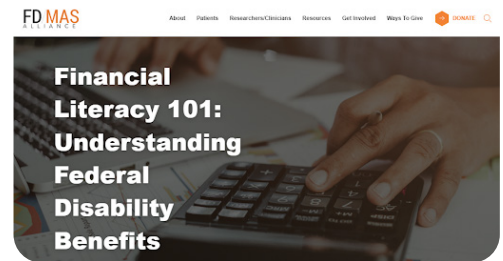
Impact

- This project has been impactful for those in the community as it has been developed as a lasting resource and has reached thousands impacted by sarcoidosis. FSR has delivered valuable financial resources through a comprehensive, educational webpage that has reached more than 1,300+ as well as shared educational messaging to more than 62,000+ who will be able to utilize the education and information in their sarcoidosis journey. FSR utilized impactful messaging to further help spread the word about this information and invite others to share effective messaging with the community. See below for examples.
- Through delivering information about the tools and resources this initiative supported, they anticipate more members of the community will be able to plan for their financial future, have access to resources to help them through struggles in their journey that include accessing treatment and quality healthcare, along with other patient assistance programs.
- Moreover, FSR learned through this initiative that the sarcoidosis community experiences significant financial barriers due to living with sarcoidosis. As such, FSR will continue to provide resources, tools, and education to the community to increase and improve financial literacy knowledge.

FD/MAS Alliance



FD/MAS Alliance (previously known as Fibrous Dysplasia Foundation) created a new website, conducted webinars, hosted a roundtable forum, and created a video that synthesized the forum and webinars. Two public workshops on Disability Benefits featured an expert wealth advisor with a specialization in supporting families with disability and special needs. These workshops were designed based on the input of the Patient Advisory Council, ensuring that the output was based on community needs rather than conjecture. For both of these workshops, a patient advisor acted as a host, answering questions submitted privately before and during the webinar. From those workshops, the organization created a resource page with the full recording of the workshop as well as sample worksheets, frequently asked questions, and helpful links. This resource page will benefit in the coming months as FD/MAS focuses on search engine optimization efforts on this page.



In addition to the formal post-survey and the experience of people who attended the webinar live, FD/MAS also wanted to elevate the conversation about financial resources within the community. After the webinars were completed, they saw this happen in real-time within the Slack channel of the Patient Advisory Council. These outcomes demonstrate the start of a more long-term aim: empowering those in the FD/MAS community to organize and engage around topics of financial advocacy.

Impact

- Ultimately, FD/MAS Alliance expects to see a regular, ongoing conversation about financial issues for those facing rare, lifelong debilitating illnesses. They hope that the discussion of financial literacy and well-being becomes as open and as prominent as the regular, ongoing community conversations about FD/MAS treatments and research.
- This program is just the beginning of creating a healthier pathway for FD/MAS patients that enables them to live more comfortably physically and financially, seeking care when it is most needed, rather than when they think they can afford it. Over the next year, FD/MAS Alliance will monitor the web resource page and both informal and formal conversations amongst the Patient Advisory Council and through contacts to the PAC@fibrousdysplasia.org email in order to see how this program affects those numbers.



Michael [expert speaker and wealth advisor] broke down and explained the benefits program in a way that I finally understand, which provided a starting point for me. In the past, I'd been completely overwhelmed and had no idea where to begin.

- Webinar Participant



[The resources] clarify a lot of information that when doing the research on your own can be overwhelming and disheartening. The formatting of this webinar helped my understanding of the information in a way I can digest it and understand it.

- Webinar Participant

The Global Foundation for Peroxisomal Disorders



The Global Foundation for Peroxisomal Disorders (GFPD) formally identified and assessed the unique financial challenges faced by those living with peroxisomal disorders through targeted survey questions that were piloted at the GFPD Family and Scientific Conference in mid-June.

The research generated from this project is providing detailed initial information identifying the specific financial literacy and hardship concerns and challenges of families affected by a peroxisomal disorder. It addresses what the financial needs are, what needs are not being met, and how the GFPD can strengthen accessible, relevant network support for patients and families, with the ultimate goal of improving health outcomes for patients. Educational and patient-support offerings now include responsive financial information and guidance for families. In addition to the webinar created as part of this project, the new data and insight is helping GFPD plan for future programming focused on supporting families in navigating rare disease financial burdens.

GFPD will be able to quickly integrate insights gained from the research into existing programming in the form of webinars and meet-ups with ongoing opportunities for families to provide additional feedback as their needs evolve. This project inspired additional questions and ongoing consideration by families and GFPD staff and board around gaps in awareness and understanding of practical financial issues and potential hardships.



Impact

- The GFPD serves more than 600 families around the world – in all 50 U.S. states and 40 countries globally – who have received a diagnosis of a peroxisomal disorder in the Zellweger spectrum (PBD-ZSD) or the related single enzyme deficiencies, and these numbers continue to grow. Because peroxisomal disorders can affect anyone, there is also extreme socio-economic diversity within the GFPD community. For example, 25% of survey respondents reported less than \$75,000 in annual household income, which is less than the median family income for the United States.
- Long-term impact of this project will be that the majority of GFPD families responding to future surveys will view the GFPD as a trusted, supportive resource that can help them identify and navigate financial resources in their own communities.
- By initiating discussion around the potentially delicate topic of financial hardship, the GFPD can begin to tailor educational and patient support resource tools responsively. This may include increased and focused support for families navigating medical expenses, health insurance claims, and connecting families with financial resources in their own communities.
- The data collected through this project has also helped to inform the planning for a longer-term GFPD 3-year strategic initiative to create a best-practice based educational model, TeleECHO, designed to bridge patient/family/physician/researcher communications, strengthen network supports, and improve patient health outcomes.

All India Institute of Medical Sciences (AIIMS), New Delhi, India



All India Institute of Medical Sciences (AIIMS) developed a webinar with objectives to expand outreach to rural populations, enrich existing knowledge about rare diseases, encourage early diagnosis and holistic evaluation, provide educational support and family counseling, expand advocacy groups, and inform about management therapies. AIIMS also sought to create awareness around the national rare disease policy, place an emphasis on de-stigmatization and inclusion in society for those living with a rare disease, and international scientific collaborations. The focus was on diagnosis and management of neuromuscular disorders, especially DMD and SMA. As of October 2022, over 3,000 participants benefited from this outreach.

The role of parent advocacy groups, NGOs and the Government in promoting employment and helping the patients to gain access to these opportunities for better societal acceptance and better quality of life were discussed. The amenities available from NGOs in terms of rehabilitation activities available at district and peripheral level for a non-ambulant child with SMA and DMD in India were also discussed.

During the webinar sessions, the policy makers have highlighted the various financial schemes for the benefit of the maximum population. A few patients have already received the drugs with the help of the Humanitarian access program, crowd funding from Impact guru, and financial assistance from Coal India. Many other patients are in the pipeline under various humanitarian access programs. They have more than 1000 SMA and 2000 DMD patients under follow-up. The aim would be to additionally deliver the already existing services to 30-40% more children suffering from such rare neuromuscular disorders like DMD and SMA.

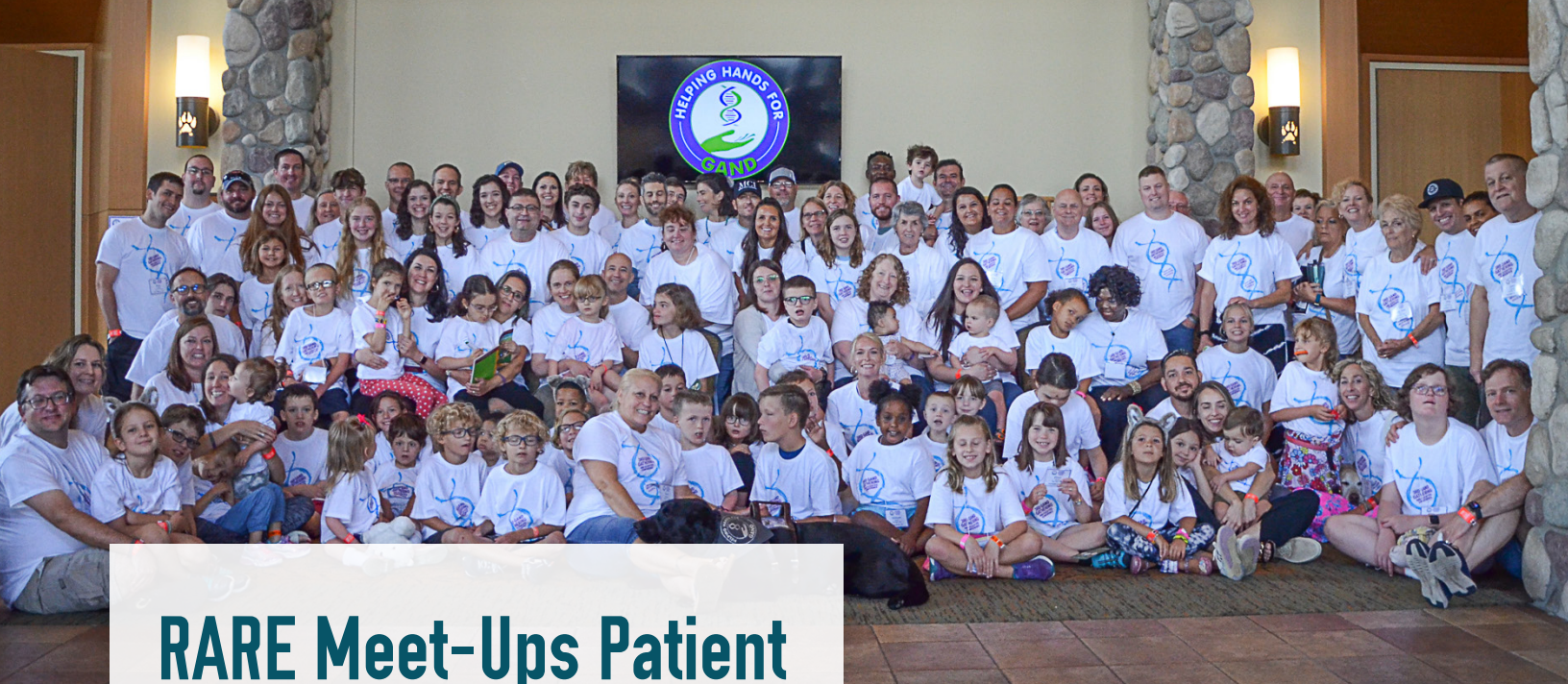
The necessity of parental counseling in rare diseases like SMA and DMD, issues faced by parents of children with special needs and how parental support groups can help them in coping with such issues were discussed.



Outreach Webinars: Financial Advocacy For Rare Disease

Impact

- All the webinar sessions were very informative and additionally were focused on what variations are required in self, community, and policies at a national and global platform for delivering the maximal benefits to the SMA and DMD patients in near future. Current challenges to implementing new-born screening programmes, recognising that there are differences from country to country and sometimes even within a country is very essential.
- In all these sessions, they recognised that, although these diseases might be individually rare, the challenges for patients, families, and care-givers are common to a significant number of children throughout the country.

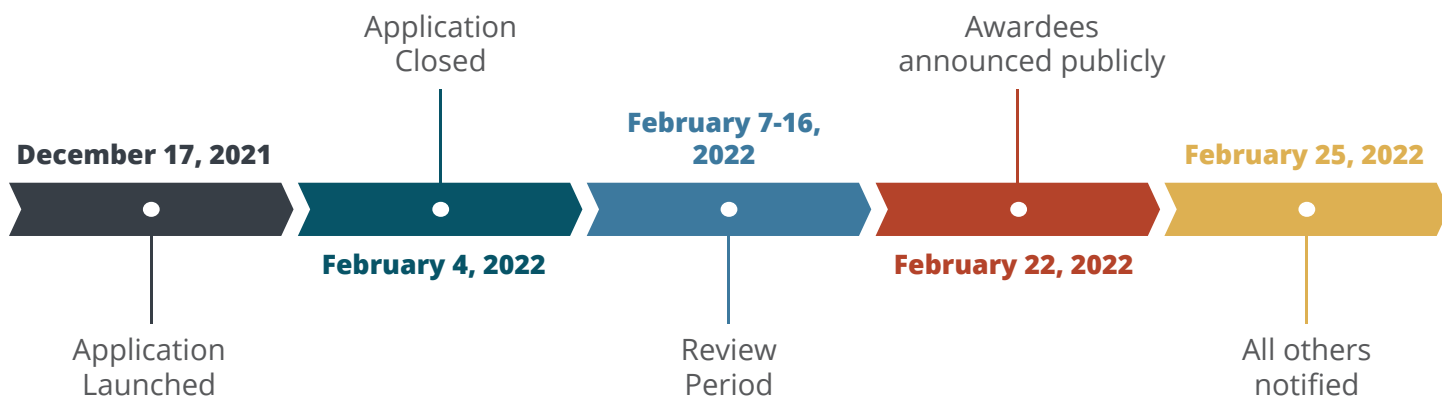


RARE Meet-Ups Patient Impact Grant

After two years of isolation and canceled in-person events, Global Genes was excited to partner with advocacy organizations to support their efforts to further their mission and work in the rare disease community. Through the Rare Meet-Up Patient Impact Grants, Global Genes provided up to \$15,000 in grant funding and public relations, social media and event planning support to five U.S.-based organizations and one international organization. The meet-ups were held between April and November and brought together key stakeholders to foster collaboration, social support, provide education, and raise awareness. This was the first year the RARE Meet-Up Patient Impact Grant was offered.

Awardees included:





\$70,912
Awarded



78
Applications

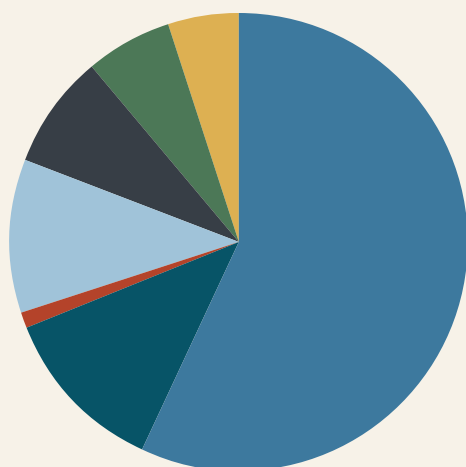


6
Awardees

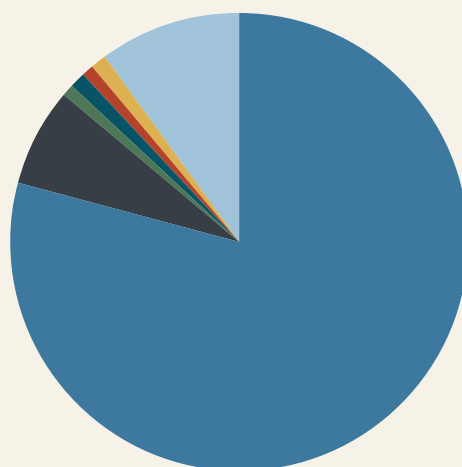


+15,000
Reach

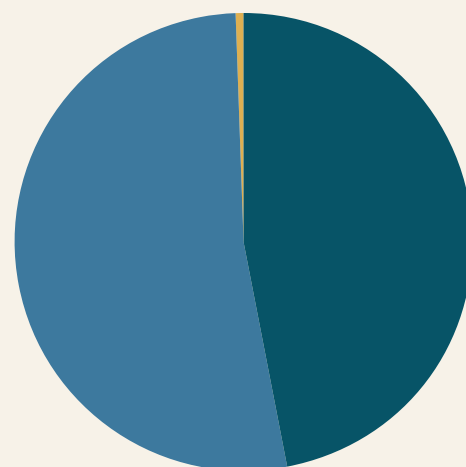
Awardees - Ethnicity breakdown



Awardees - Language breakdown



Awardees - Gender breakdown



[Read more about the programs here](#)

Thank you to the organizations who made these grants possible





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Linking arms and broadening our circle of connections with families living with someone with a rare disorder like Phelan McDermid Syndrome will have a long term impact on our lives. Being able to share experiences and see others in different stages brings hope and community. Knowing your family isn't alone on this journey is transforming. I saw families able to let down their guard and allow others to help with their children, giving them time to be able to eat dinner or talk to another adult. It brought hope to families just beginning the journey of rare disorders to see others living it ahead of them and alongside them. We are so grateful for being able to attend this event made possible by Global Genes. - **Phelan McDermid Syndrome Foundation Participant**



Helping Hands for GAND 3rd GAND Gathering & Scientific Conference



Helping Hands for GAND held their 3rd GAND Gathering & Scientific Conference in July at the Great Wolf Lodge in Mason, Ohio. This meet-up allowed individuals with GAND and their families to come together to support and learn from each other and professionals about development and medical issues associated with GAND, as well as ongoing scientific research from GAND researchers, speech language pathologists, and others.

The families appreciated the opportunity to socialize with, network with, and get to know other GAND families. Friendships among GAND siblings blossomed. Children with GAND spent time with others who communicate the way they do, i.e. with severe speech limitations, and with their AAC devices. GAND families with young children were able to see older children who have GAND and get a sense of how their child might grow up. People who are new to GAND learned more about this exceedingly rare condition from Dr. Pierson, the leading researcher on GAND who has conducted the largest case study on GAND. There was an energizing spirit of warmth and connection and a true sense of community building that GAND families really need.



Impact

- New GAND families met Drs. Pierson and Young who explained their past work and their plans for continued research. This connection encourages families to contribute to research—both through their own participation and by raising money among their communities. This in turn will lead to more awareness of GAND, streamline the diagnostic odyssey, and potentially, drug treatment to mitigate the effects of GAND. Families new to GAND were able to enroll in Dr. Pierson's case study, thus directly contributing to research.
- GAND families learned about apraxia and the specific types of speech therapy that are proven to help people with apraxia the most, as well as other health complications that they can better identify. They were shown different ways to approach school for a child with GAND, and how to advocate for their child's needs.
- As a young organization made for a tiny community, this conference is one of its core functions. Fostering connections between GAND families is the most important thing they can do at this point, and face-to-face contact is instrumental, especially with the interruption of in-person events due to COVID-19. GAND is so incredibly rare that most people do not have the opportunity to meet others with GAND outside of conferences.



It has been 4 years since our last meet up and the power of meeting other families with children with GAND in person is just priceless. We learned about new therapies we could try and created strong connections with GAND families to increase our support system. We were also able to share our experience with other newly diagnosed families that helped to provide encouragement to them as they navigate this rare syndrome. - Parent Participant

Raising Hope International's 2022 Sickle Cell Convention in Uganda



Raising Hope International hosted the 2022 Sickle Cell Convention, the first of its kind in Uganda. The Sickle Cell Convention brought together stakeholders, including the Commissioner, Non-Communicable Diseases, the National Sickle Cell Program Coordinator, sponsors, and service providers from across the globe to examine and discuss the role and impact of policy frameworks in the management of Sickle Cell disease in Uganda and globally. This event allowed Raising Hope International to:

- Increase confidence in tackling sickle cell disease by caregivers in Uganda because of the knowledge and best practices that were shared by the different presenters.
- Strengthen collaboration with other civil society organizations, medical technocrats and institutions, and ministry of health.
- Create an interactive digital platform that has enabled them to further the discussion on sickle cell disease with other local and international stakeholders.



This convention increased awareness on sickle cell disease, particularly on underrecognized disease complications and unmet needs and challenges amongst all related stakeholders.

- Participant



Impact

- This convention provided a platform where various volunteers of our organization were recognized for their contribution to the fight against sickle cell disease. This has greatly motivated them and also attracted more individuals and organizations that are willing to be part of this struggle.
- This conference raised the organizational profile and exposed brand recognition to a wider audience both locally and internationally, including relationships with potential donors committed to improving the lives of people affected by sickle cell disease.
- Raising Hope International has improved networking skills and gained valuable experience in organizing events of such magnitude, and improved internal capacity for organizational management.



It taught me how to handle sickle cell disease patients and how to take care of them and what medication they should be on.

- Healthcare Professional



This meetup increased and encouraged me more about advocacy and passionately creating awareness about sickle cell disease.

- Participant

Phelan-McDermid Syndrome Foundation's Family Camp



Phelan-McDermid Syndrome Foundation (PMSF) hosted a Family Camp weekend retreat for families of children with Phelan-McDermid Syndrome (PMS) at Morgan's Wonderland in San Antonio, TX, an

inclusive camp setting. The meet-up focused on parent and sibling support and mitigating mental health challenges. New families were matched with veteran families to help acclimate them to the community and find other families who share their experience. Prior to camp, participants received informational flyers and participated in a one-hour session for siblings, moms, dads, and new families.



Impact

- One family learned how much their daughter loved therapeutic horseback riding. She clearly communicated her interest and vocalized with glee when the horses walked by. That family intends to look for a riding program near their home.
- Even though the community has strived to stay connected virtually through social media, there is no replacement for being in person. For a weekend, families were able to sit by a pool while their children splashed and played in a safe setting – something that is not taken for granted in this community.
- PMSF was thrilled that Dr. Phelan joined, wearing her cowboy hat and boots! She was available to talk 1:1 with families to answer their questions, listen to their concerns, and meet the children and adults with PMS. It is not every day that families with a rare disease can spend a weekend with the person who defined their child's syndrome.
- One mom moved from doubting that her 3-year-old son could do the camp activities only to learn that he loved the zip line. She said “he smiled, he smiled” when they came down from their ride.
- The siblings participated in the camp activities and created their own games with their new friends. It is important for brothers and sisters to know they are important and that there are other siblings like them.

[Read more about one participant's experience here.](#)



This meet was a great experience for myself and my family especially being that it was our 1st PMSF Event. We got to meet many families that are going through the same or very similar situations as ourselves. Our child got to play with other PMS children which was wonderful to see her interacting with them. We can't wait until the next event. - Parent Participant



This meet-up brought us together with other parents affected by Phelan McDermid Syndrome. I got to see what others like myself were going through and the varying degrees of the syndrome in their loved ones. I left the meet-up with an overall sense of calm and reassurance, that we are not alone and now have friends and families to talk to and be a part of where there is no judgment, having to apologize, or explain the actions of our loved one. - Parent Participant

Bleeding Disorders Alliance



The Bleeding Disorders Alliance of Illinois hosted a meet-up for people with rare bleeding disorders from Illinois, Indiana, Wisconsin, and Missouri to help build a community of support, teach empowerment strategies to overcome barriers in healthcare, and provide resources needed to live life with a rare bleeding disorder. This event brought together 65 people with ultra-rare bleeding disorders to share their stories, attend sessions, begin building a community, and plan for the future, creating a Bill of Rights and planning a follow-up conference.



Impact

- Each person who participated had a heartbreaking story that contained a long diagnostic journey, lack of support/understanding from healthcare professionals, and physical complications, which in most cases are not necessary in 2022. They were each armed with the tools to empower themselves moving forward for better health and life outcomes. One example was provided by one of the hematologists who spoke: that recently after diagnosing one person with an URBD he was able to work diagnosing an additional 31 family members with the same condition. People are dying in the U.S. today because they are not being accurately diagnosed. The people who attended this event are committed to doing whatever is necessary to lower time to diagnosis, get them to BDA federally funded hemophilia treatment centers and support them to not be isolated as each of them were before attending the event.
- It also became clear that even after diagnosis, participants are not being treated with the best available medication, the proper protocols, and even at times by the right doctors. One of the attendees – a 51-year-old man – is still being seen by a pediatrician because he is being told that is the only person in his area who knows anything about his disorder.
- A key finding, which led the organization to apply for the Meet-Up grant, was the X-linked bias that many in the community experience. Hemophilia, the most notable bleeding disorder, is an X-linked inherited condition. Medical schools today still focus mainly on this condition and unless a practitioner specializes, they are unlikely to ever learn about everything that can go wrong in the bleeding and clotting cascade. Changing this is a key initiative for the organization's strategic direction for the future.
- The organization will work with people with inherited bleeding disorders and their families, arming them with the self-direction to understand that both men and women have bleeding disorders. They are changing the educational approach to include empowerment as a key point in all presentations. This community must be able to skillfully and successfully work with their medical teams to help them understand how their symptomatology can – and in many cases – is a bleeding disorder.



It was wonderful to meet other people and families that have had experiences similar to me. Wonderful first time connection within the community. - Meet-Up Participant



It was amazing to hear and see how families get excited/happy to get involved. How new ideas started to come up for future meetings. - Meet-Up Participant

Youngtimers, Inc.



The Youngtimers meet-up focused on bringing together the organization's leaders - co-founders, patient advisory board members, and other stakeholders - for a critical in-person capacity building weekend to strategize and plan for the future of their newly established foundation serving the early onset familial Alzheimer's disease (EOFAD) community. Working together with a strategic planning and organizational development specialist, the leadership came away with a detailed and well-informed fundraising strategy to support a 3-year vision, plan to expand community outreach opportunities for EOFAD stakeholders, and a programming plan that addresses the changing landscape for caregivers, in-person interactions, and healthcare services.



These discussions also pulled in different skill sets, aiding in the creation of a well-informed organizational vision and goals. The group identified critical areas of improvement for the disease journey and have mapped out a plan to address these areas with peer support, online education, partnerships with expert researchers and professionals, and connecting with the disease community throughout the year.

Impact

- The planning that occurred during this meeting will lead to improved patient outcomes (time to diagnosis, understanding genetic testing, mental health), clinical trial participation, and accelerated research and treatment development. It provided Youngtimers with an informed framework (fundraising, operational growth strategies, community engagement strategies) to launch the organization to the next level, providing more education, more support, and more services to the EOFAD community.
- The organization established plans to improve outreach and education to all EOFAD stakeholders. By educating individuals, healthcare providers and social workers about EOFAD, Youngtimers can facilitate a proper diagnosis of EOFAD and help families better understand autosomal dominant inheritance patterns.



I found the discussions over our two days to be unbelievably valuable. To have the chance to get the key leaders and participants from our board/organization together in person, and to have a professional, skilled facilitator to lead us, allowed us to make tremendous progress in better understanding who we are, what impact we want to have, and how we can best operationalize our efforts, including building the organizational structure and fundraising programs we will need to have in place to scale our efforts. I couldn't be more excited about the impact we will be able to have on this rare, but very important patient community and more energized by the discussions and planning efforts from this past weekend's meetings.

Congenital Hyperinsulinism International



Congenital Hyperinsulinism International (CHI) hosted its first ever regional meet-up in Southern California at the

Cayton Children's Museum in Los Angeles. In addition to having an opportunity to meet other families affected by Congenital Hyperinsulinism (HI), the meeting focused on the HI Global Registry (the only patient-reported HI natural history study) and the importance of patient reported studies in the development of new treatments. Children, accompanied by a caregiver, also had a planned fun activity and party at the museum while parents learned. Participants were able to learn about:

- Why CHI is leading a patient-powered research effort to collect data on the experience of living with congenital hyperinsulinism, and how this research is already serving the CHI community by supporting research and drug development.
- Specific drug development programs, hearing directly from representatives of the studies with an opportunity to have their specific questions answered.
- Ways to support CHI, including potential donors and someone able to draft resolution language and lead the campaign to garner funding for CHI Centers of Excellence.



Impact

- CHI was able to enroll more people in the natural history study, which will continue to drive research for new treatments and cures. This is extremely important in the congenital hyperinsulinism community because there is currently only one approved treatment for the condition, and it is effective in less than half of all patients. The learning from the registry also has the power to decrease diagnosis time.
- Improved well-being of families affected by the condition: the alleviation of stress, which occurs when individual members of a community can meet and share their experiences, is a very important outcome. Lasting relationships, which are formed through the meet-up, can have a long-term impact on the quality of life for those attending.



RARE Relief Impact Grant

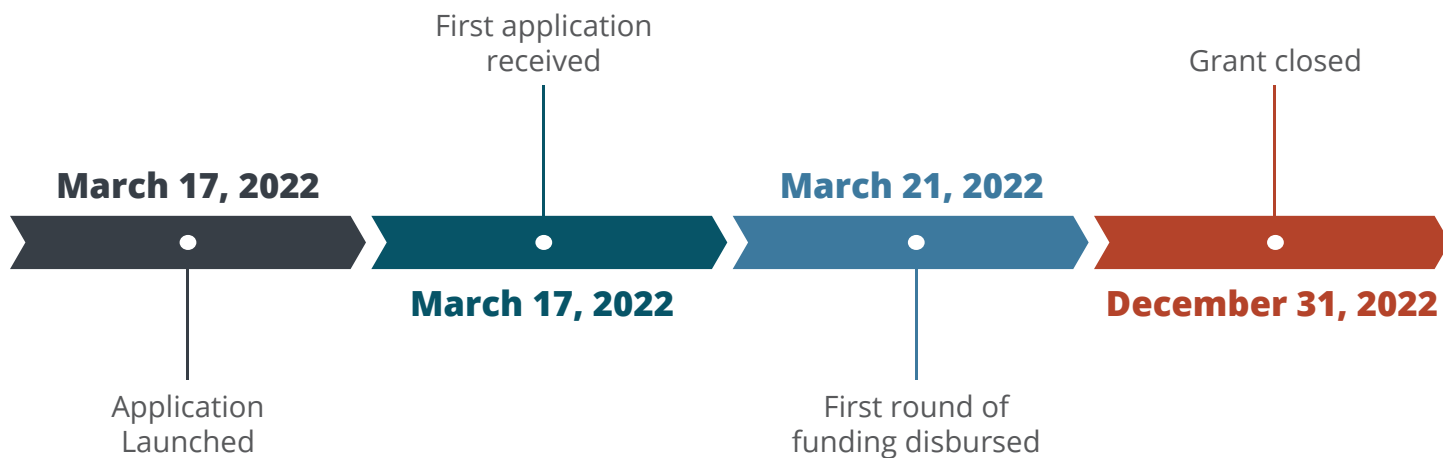
The RARE Relief Impact Grant was developed in collaboration with partners from around the world to provide support for rare disease families from Ukraine who have been affected or displaced by the ongoing war. Global Genes gave six grants to organizations with boots on the ground in and around Ukraine to connect rare disease families and patients to resources and networks of support in neighboring countries.

“

Thanks to the support of grant funding, we were able to focus our efforts on providing the necessary care to patients, on finding drugs and arranging their delivery, to provide psychological assistance, to interact with volunteer, state and local services to more effectively solve the problems that patients had after the start of the war in Ukraine. - **Healthcare Education Institute**

“

It was important for them to go through this path: from despair to confidence that we will be able to survive and win in these difficult conditions. It is important to feel the support of friends from all over the world, it really helps a lot not to give up and keep fighting! We are very grateful to you!!! Thanks!!! - **PHA Ukraine**



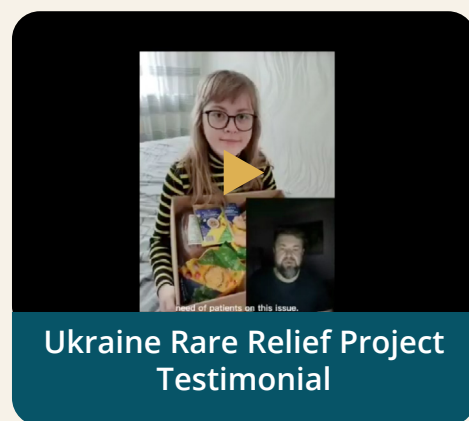
Check out these videos



\$170,000
Awarded



6
Awardees



Thank you to the organizations who made these grants possible



Healthcare Education Institute

Since the day the war broke out, Healthcare Education Institute has been supporting patients with rare diseases who are fleeing the horrors of war. Through their grant funded project, the organization has been able to provide support to rare disease patients at every stage of their journey to Poland including quick transport across the border and connections to accommodations and treatment. The organization was also able to organize the transport of medicines and medical equipment to hospitals treating rare diseases in Ukraine, establish a website that included translation and legal support materials and raise awareness about the situation of rare disease patients in Ukraine.

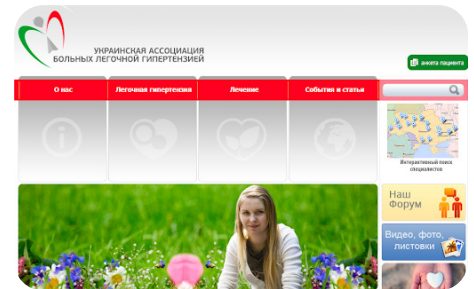
WEBSITE



PHA Ukraine

After the start of the war in Ukraine (February 24, 2022), many patients with pulmonary hypertension were forced to move to safer regions of Ukraine or went to other countries. About 20% of patients moved to other countries (Poland, Slovakia, Italy, Germany, Spain, France, Ireland, Latvia, etc.). More than 40% moved to other regions of Ukraine. But today, most of them have already returned to their usual places of residence (if they did not end up in the occupied regions). For many of them, there was a threat of interruption of treatment, as the supply chain for drugs under the state program was affected, and many drugs became difficult to buy in pharmacies. Through this grant from Global Genes, PHA Ukraine was able to provide regular treatments for 294 patients, deliver basic and supportive medicine to two medical centers and distribute iloprost nebulizer to 18 patients. The organization was also able to provide financial assistance to 14 patients, which was then used to purchase train tickets for relocation or temporary housing.

WEBSITE



NGO All-Ukrainian Association for the Care of Patients with Cystic Fibrosis

Due to the Russian invasion of Ukraine, patients diagnosed with cystic fibrosis were unable to receive appropriate treatment and adhere to treatment/therapy regimens. UACPCF created a logistics and coordination center to provide humanitarian assistance to patients who remained in Ukraine and patients who were forced to leave the country. The Center provides advice on internal movement in Ukraine and abroad, advises on medical services in Ukraine and abroad, coordinates the logistics of humanitarian aid, purchases medicines, medical equipment, food, and hygiene kits. Through the grant funding received from Global Genes, UACPCF coordinated the logistics of humanitarian aid for patients diagnosed with cystic fibrosis who remained in Ukraine, purchased medicines and medical equipment, and provided food and hygiene kits to patients.

WEBSITE



Eurordis

In response to the unfolding humanitarian crisis impacting the people of Ukraine, and in particular in response to the significant challenges facing Ukrainians living with a rare disease, EURORDIS established Razem z Ukraina (Together with Ukraine). Razem z Ukraina has provided:

- Family assistance to support Ukrainian families to access health care, navigate the social benefit systems, get access to housing and schools, etc.
- Appropriate (adapted) housing for families affected by a rare disease, either through EURORDIS' partnership with Airbnb and/or via directly commissioned housing through coalition members
- Equipment (e.g. wheelchairs, medical supplies) procured and shipped where they are needed, as well as support travel for Ukrainian families and the staff and volunteers who help them
- Psychological support for Ukrainian individuals, children or families, referred by coalition members and facilitated directly by EURORDIS.



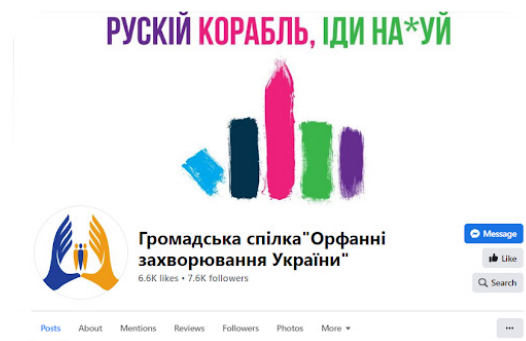
Through these priority areas and actions, Razem z Ukraina aspires to address the varied critical needs of a minimum of 500 Ukrainian families living with a rare disease who are currently in, coming to, and/or going through Poland.

[WEBSITE](#)

RARE Disease Ukraine

On the 24th of February, 2022, the Russian Federation started war against sovereign Ukraine. As a result, most of the patients with rare diseases and their families had to leave their homes. From the first days of war, the NGO, Rare Diseases of Ukraine, has been doing everything possible to solve any issues of patients who found themselves in a difficult situation. Special attention was paid to the provision of uninterrupted treatment, evacuation to European countries, providing humanitarian aid to hospitals and to those patients who remained in their homes. In order to improve the service of patients with rare diseases, work was carried out to equip two laboratories of medical genetics. The organization also allocated funds for purchase of oxygen concentrators, which are vital for patients of certain nasologies, and to the purchase of medicines for patients with rare diseases, among them, for example, Enbrel injection solution.

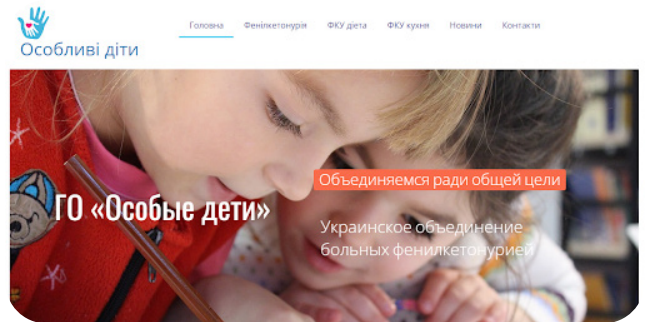
[WEBSITE](#)



PKU

Before the start of a full-scale war in Ukraine, there were more than 1700 people with phenylketonuria. With the beginning of the war, about 30-35% left the territory of Ukraine. Most of the patients with PKU were in the eastern and central part of Ukraine, the part that is now most affected by shelling. As a result of this grant, PKU was able to:

- Rented premises for the placement of the organization, production, and storage space for humanitarian assistance.
- Purchased equipment for production: Pasta press-1pc, dehydrator-1pc, drying cabinet-2pcs, stirrer for dry mixes-1pc, oven - 1pc, mixer for dough-1pc.
- Launch production and at the moment, about 200 people with specialized food packages with low protein content have been provided with grant funds. Food packages include:
 - » Baking mixes, pasta, gnocchi mixes, pancake mixes, biscuits, etc. (essential products for 1 month).
- Material assistance was provided to more than 40 families to move from the "hot" areas to the calmer territories of Ukraine.
- Material assistance for living was provided to about 25 families who found themselves in a difficult financial situation due to the war.
- Closed the need for specialized therapeutic amino acid mixtures for more than 50 people.
- Purchased the raw materials for production that will cover about 100 more specialized food sets and it is possible to provide 15 people with specialized therapeutic amino acid mixtures.



WEBSITE

Thank you to the organizations who made these RARE Impact Grants possible





Global Genes®
Allies in Rare Disease

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