



Strength in community:

Shared insights and opportunities to drive progress across the rare disease community

These are the faces of rare disease community members who have shared their stories across #RAREis channels.

AMGEN

#RAREis...

Contents

- INTRODUCTION 1**
 - Uncovering Issues: #RAREis survey and summit.....2
- THE CHALLENGES: BARRIERS TO PROGRESS IN RARE DISEASE 3**
 - 1. Access to adequate care3
 - 2. Organizational funding limitations.....4
 - 3. Impact of mental health.....4
 - 4. Diversity, equity and inclusion barriers5
 - 5. Transition of care.....6
- THE OPPORTUNITIES: PROGRESS THROUGH PARTNERSHIP 7**
 - Community connections.....7
 - Collaborations with other advocates across disease types.....7
 - Cross-sector partnerships.....8
- CALL TO THE COMMUNITY: RETHINKING LOCAL ACTIVATION FOR GLOBAL IMPACT 9**
 - Journey toward progress in rare disease.....9
 - Accelerating these efforts: a challenge to the community.....9
- APPENDIX 11**
 - #RAREis Global Advocate Grant reach.....11
 - #RAREis survey respondents.....11
 - Survey findings12
 - Rare disease advocacy group partnerships.....12
 - About #RAREis13
 - About #RAREis Global Advocate Grant.....13
 - About Amgen.....13

Introduction

Recent progress in scientific discovery, diagnostic proficiency and therapeutic momentum in rare diseases have driven a realization: it may be time to rethink our understanding of “rare.”

It's true that individually, rare diseases each affect a tiny fraction of a percent of the world's population. Yet collectively, the thousands of identified rare diseases affect millions of people around the world. If all of the people with rare diseases worldwide inhabited their own country, it would be the third largest by population, after China and India.¹

This large, diverse population faces numerous challenges throughout the patient journey that are starkly distinct from experiences in prevalent diseases that can be readily recognized, diagnosed and treated. Instead, the path from first discovering symptoms to a proper diagnosis is often many years or even decades with several doctors due to ill-defined disease manifestations, poor understanding of genetic markers and misdiagnoses. Once diagnosed, most known rare diseases have few or no treatment options, leaving individuals and their care providers navigating through an array of approaches to help manage their disease. Care management over time remains complex and burdensome for these individuals, their families and providers, and the healthcare system.

Global policy leaders and advocates for these diseases have faced unique obstacles in fostering sustainable progress due to highly varied experiences and lack of care options. Unlike many prevalent diseases, presumably no single focused infusion of funding or community imperatives will provide solutions that can benefit every rare population.

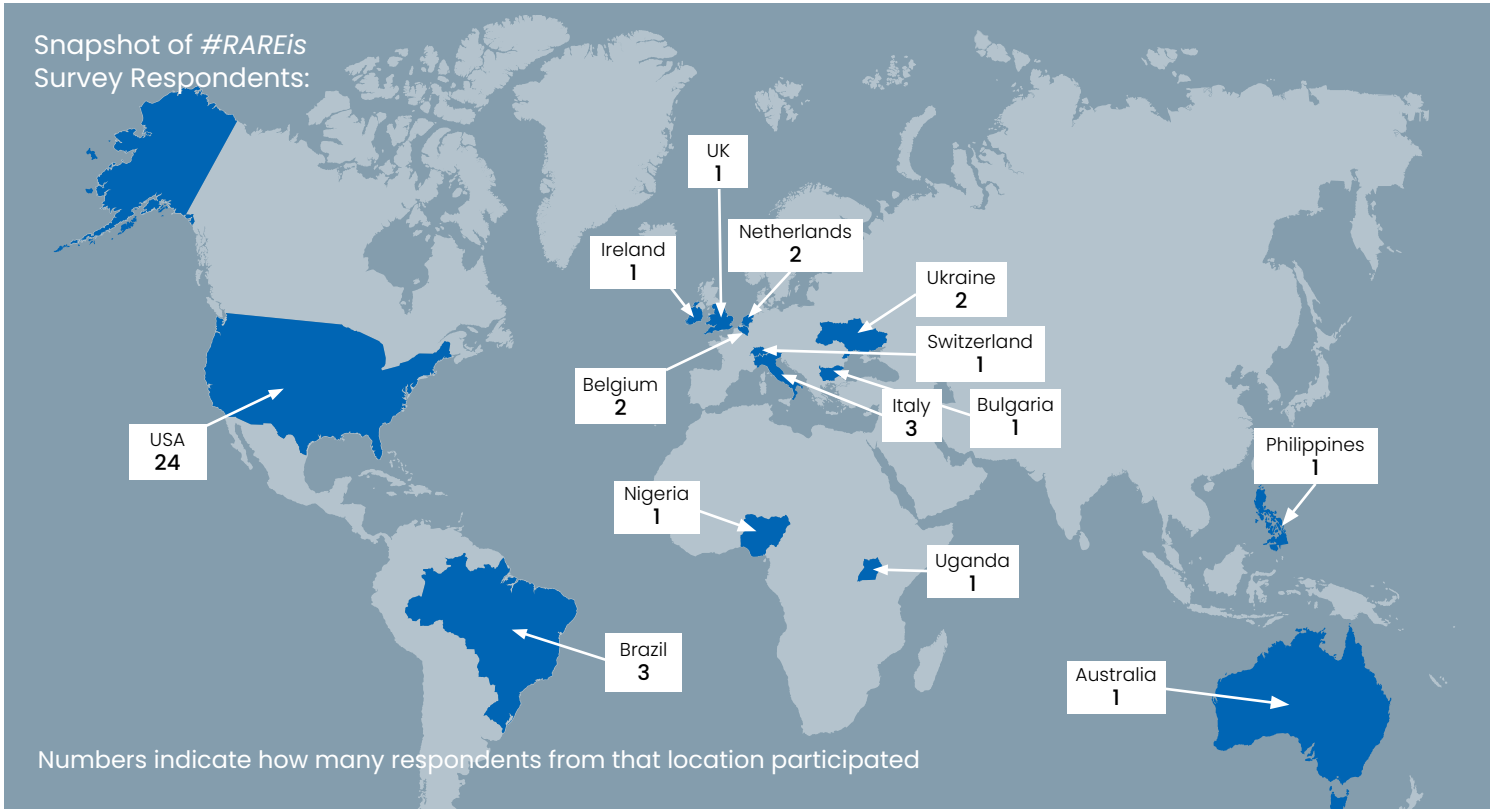
But on closer evaluation, the picture is considerably different. By listening to community voices who are making steady, progressive change for people with rare diseases, we find that in fact there are meaningful commonalities in the process and pathway to progress.

Despite unique circumstances, there are shared mindsets and mechanisms that can benefit many communities, and numerous organizations, like Global Genes, National Organization for Rare Disorders, EURORDIS – Rare Diseases Europe, Rare Disease Diversity Coalition, The EveryLife Foundation for Rare Diseases, and more representing the broad rare disease community are activating and delivering on this view. Cultivating shared philosophies and insights can create efficiencies in impactful actions that can reduce the investment and time required to generate real change for communities who are urgently awaiting answers.

By reaching into the rare community and shining spotlights on the key issues and drivers at the hyper-local level, we are generating momentum that can be globally applicable. The #RAREis program led by Amgen (previously Horizon Therapeutics) was implemented for this purpose. Established to identify and share stories and resources to address common issues facing the rare disease community, the program has engaged advocates from around the world, providing grant support at the micro level in order to generate macro momentum. For more details, see Appendix A: #RAREis Global Advocate Grant Reach.

¹ #Resolution4Rare: Global campaign for the first UN Resolution on Persons Living with a Rare Disease (EURORDIS.org)





UNCOVERING ISSUES: #RAREis Survey and Summit

In Fall 2023, the #RAREis team conducted a survey and virtual summit to help articulate and clearly define some of the challenges that can feel distinct to a particular disease, but in truth impact much broader communities. Representing the voices of 44 advocates from a diverse community of diseases and 14 different geographies,² the survey addressed known trends that have been raised by rare disease advocates, with an intent to more deeply understand the constraints they face as well as the opportunities they see to accelerate progress with additional resources and support.

The survey was followed by a virtual summit, during which advocates were invited to discuss more intimately the issues that were raised in the survey, sharing experiences, perspectives and ideas for collaboration. In total, 16 organizations joined the summit representing seven countries.³

Participants in both forums described critical issues that hinder progress, while highlighting the areas where collaboration and insight sharing has begun to move the needle toward common impact for the rare disease community.

#RAREis Survey: Uncovering Key Issues Across Known Trends



Global Rare Community



Community Challenges



Mental Health



Diversity, Equity and Inclusion



Transitions of Care



Organization Scope: Challenges and Opportunities



Collaboration

²Australia, Belgium, Brazil, Bulgaria, England, Ireland, Italy, Nigeria, Philippines, Switzerland, The Netherlands, Uganda, Ukraine and the United States

³Brazil, Ireland, Italy, Spain, Uganda, Ukraine and the United States

What can the community learn from these inputs? It is increasingly clear that local infusions of effort and support are having a ripple effect to global impact. **The future of disease advocacy is simply stronger together.**

This white paper reviews the opportunities to rethink how we drive progress in this large global community.

Quotes from summit participants are attributed, while quotes from the survey are anonymous.

Melanie McDonald said when her daughter was diagnosed with Prader-Willi Syndrome (PWS), the closest specialist they could find was in Florida, a long way from their home in New Hampshire. Melanie and her family faced challenges navigating travel and accessing resources needed to coordinate time and financial support to travel to Florida for appointments. Even with a specialist, Melanie still needed a local team to help manage the disease. Yet, she found that most clinicians weren't familiar with PWS, so she had to educate them.

"It was only because of connecting with an advocacy organization and getting the lay of the land and early diagnosis package that we could navigate her diagnosis. And even then, it was extremely hard. There was nobody within 500 miles of our home that had expertise in how to care for our daughter. So we became the specialists. We educated all the doctors."

—Melanie McDonald,
Prader-Willi Syndrome Association

THE CHALLENGES: Barriers to progress in rare disease

While some of the most significant issues and constraints to progress in the rare disease community remain specific to the rarity of the condition itself, a range of critical, interrelated issues face many rare disease states. Survey respondents highlight five persistent issues that constrain their efforts to fully support their communities:

1. Access to adequate care

Top 3 Drivers of Suboptimal Access



- Location of care
- Lack of provider education
- Financial burdens of care

Healthcare access issues plague proper care for people with a range of diseases around the world. In recent years, these challenges have become more prominently raised by global public health authorities, health ministries and regulators and voices across the ecosystem with demands for novel solutions that can improve global health and reduce mortality. Some of the most prominent drivers of suboptimal access include:

Location of Care

Access to specialized care at the local level is a common challenge around the world. In many low- and middle-income countries and rural regions, fundamental care infrastructure remains inconsistent, and care for specialized diagnostics, treatment and monitoring is even more fragmented.

Even in upper-middle and high-income countries, access can be regionalized, demanding significant travel time and investment for individuals and families to get the right answers and treatment for their condition.



Lack of Provider Education

Unlike prominent conditions for which disease etiology is well documented and prominently known by medical experts, the rare disease community is uniquely plagued by a lack of provider education about these conditions. Many physicians are taught “when you hear hoofbeats, think of horses, not zebras,” a moniker coined by Dr. Theodore Woodward in the 1940s. In the rare disease community, not all hoofbeats are horses, doctors should be aware of the 400 million people worldwide who are zebras. Only a small proportion of health care professionals have known expertise or, in many cases, have ever seen individuals with these rare conditions.

Financial Burdens of Care

The ability to find an expert, gain an accurate diagnosis and receive proper care are each associated with financial considerations that can be prohibitive for individuals and their families. Certainly in the small proportion of rare diseases where treatments are available, the cost of the therapies themselves can be daunting. And in many cases, insurance coverage is suboptimal or unavailable for these costs. Financial burdens also account for the need to travel or even relocate to enable care in a specialized environment, costs associated with disease management

“As a non-profit umbrella patient organization in Europe, the legal options to form partnerships with a financial background are very limited. We work beyond the borders, so national sponsors are not available. Most of our work is done by patients and caregivers themselves without any financial benefit. The budget limits the projects we can do for patients.”

– Survey respondent

“We are limited in our bandwidth and programming expansion due to staffing capacity as well as sustainability considering we have a growing patient community.”

– Survey respondent

beyond therapeutics, such as physical therapy or assistive equipment, and the indirect costs associated with time away from work and school.

2. Organizational funding limitations

Critical Areas Impacted by Limited Funds



- Attention, partnerships and research funding
- Patient education, support and care resources
- Staff capacity and expertise

Adequate funding is a nearly universal constraint in patient advocacy, yet a particularly poignant challenge in the rare disease community where finite resources must be spread across many unique conditions. Advocates in the rare community must consistently voice the urgency and need for change, and the value of funding to enable accelerated impact. With limited funds, advocates report constraints across several critical areas that hinder their ability to deliver on their missions, such as:

- **Championing attention**, partnerships and research funding for their specific populations
- **Comprehensive patient education**, support and care resources
- **Ability to attract and retain staff members** and volunteers with relevant skills and expertise to deliver high impact actions that can lift the broader community.

3. Impact of mental health

The Impact of Mental Health in Rare Disease:



95% of patients and

90% of caregivers experience mental health challenges

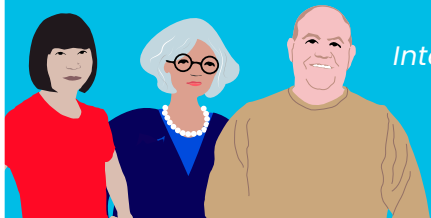
Advocates representing rare disease communities report an overwhelming, nearly universal burden of mental health on both individuals and caregivers of

"We are always putting the patient first and the care of that patient requires a degree of vigilance that is difficult to sustain. Seventy-five percent of marriages where there is a child with a special need end in divorce. Being a single parent multiplies the stress/impact of caregiver burden significantly."

– Survey respondent

"All he [a family member living with Sickle Cell Disease in Uganda] worries about is when his next crisis will be... He feels like he's living a life alone while other people are living a healthy life."

– Issac Okello,
Raising Hope
International Friends



those with rare conditions. Much of this is reported to be driven by the isolating experience of living with a rare disease. Not only are these individuals unlikely to meet others who face the same condition, their families, friends and even physicians are unlikely to understand their experience. And caregivers internalize that same sense of isolation and desperation throughout the experience, which can strain family relationships and dynamics.

People with rare conditions often face extended diagnostic odysseys, visiting many different physicians and specialists and often facing multiple misdiagnoses over months or years before finally receiving an accurate diagnosis. Typically, it takes 5 years or more to receive a correct rare disease diagnosis. For those who do receive the right answers, in many cases, the care options remain suboptimal, leaving them impacted by the prospect of a life-altering experience with no treatment or cure. Advocates supporting these individuals struggle to provide the right kind of education, counseling and support services to help manage mental health considerations for these populations.

4. Diversity, equity and inclusion barriers

Core Challenges in Patient Advocacy



- Socioeconomic
- Financial
- Language
- Diversity of staff
- Diversity in research

More than half of survey respondents highlighted challenges associated with diversity, equity and inclusion as a barrier to progress for their rare disease communities. These barriers center primarily on inclusive patient care and advocating for progress that can benefit their entire population facing that condition.

Inclusive Patient Care

With increasing global access to information through the internet, advocates are connecting with more people every day who face these rare conditions. This also means reaching a more diverse audience with different language utilization, cultural assumptions and health literacy. Advocates report challenges in providing resources, education and support that can meet the needs of their broadened communities. In particular, language translation and simplification of complex medical terminology are cited as primary barriers that advocates are not always equipped to address.

Equity in Clinical Research

Despite more medical attention and clinical research in the rare disease community, communities that have been historically marginalized remain vastly underrepresented in research efforts. As just one example, while people of color make up nearly 40% of the U.S. population, this population represents just 2-16% of participants in clinical trials.⁴ With an understanding of the complex genetic drivers of many rare diseases, there is growing agreement of the importance of testing novel therapeutics among a fully representative group of individuals affected by the target disease

⁴ Addressing Demographic Disparities in Clinical Trials (hbr.org)

(gender identity, race, ethnicity, etc.) to ensure the broad effectiveness of these therapies for diagnosed populations. And increasingly, governmental regulators and health authorities are implementing requirements to support diversity in clinical trials. Recent guidance from the FDA aims to enhance the diversity of clinical trial populations through criteria, enrollment practices and trial designs.⁵

Yet advocates face challenges in not only pushing for research programs to be more inclusive in their design and recruitment, but also in educating and encouraging underrepresented populations, like people living with rare disease, to actively engage in the research process. Fears and mistrust based on catastrophic historical research events as well as race, cultural barriers and socioeconomic factors all impede these populations from participating. Unethical, discriminatory investigator behavior and a lack of informed consent in healthcare and research like the U.S. Public Health Service Syphilis Study at Tuskegee, forced sterilization of Indigenous women by Indian Health Services, and collection and use of Henrietta Lacks' (HeLa) cells by Johns Hopkins Hospital contribute a lack of trust among communities of color in clinical research and have led to significant changes in clinical transparency, bioethics, research oversight and human rights protections.

"Diverse patient populations [in clinical trials] have been thought of as a "nice-to-have" and not a baseline. It's a must-have; we must start from that position."

– **Grace Whiting,**
The Erdheim-Chester Global Alliance

"Getting the FDA to fully understand that data standards [in rare disease communities with small populations] can limit access from a diversity standpoint."

– **Survey respondent**



5. Transition of care

Transition of Care Challenges: From Pediatric to Adult



- Process guidance to transition care teams
- Emotional support for decision-making and independent disease management

The majority of survey respondents called attention to the unique challenges faced by young individuals with rare diseases during the transition from pediatric to adult care settings. In many cases, people with conditions identified through newborn or pediatric screenings now have established care pathways and teams. This process is disrupted as they transition providers, insurance companies and begin to manage their care independently as young adults. Further, for severe conditions previously associated with a very limited lifespan, many patients are living into their adult years now and there are often few adult care providers who specialize or have expertise in these conditions.

Advocates cite challenges in several areas of support desperately needed to assist patients through this transition and ensure continuity of appropriate care where possible:

Process Guidance to Transition Care Teams

No single process exists to help patients identify and begin the process of switching specialists, support teams, social workers and others involved in care. As such, advocates struggle to provide roadmaps and guidance that can apply to many individuals vs. personalized strategies. Further, they have limited options to support referrals to new specialists who can maintain continuity of care.

Emotional Support for Decision-Making and Independent Disease Management

The transition to independently managing a chronic disease can be emotionally exhausting for young adults. Advocates report the need for

⁵ Enhancing the Diversity of Clinical Trial Populations — Eligibility Criteria, Enrollment Practices, and Trial Designs Guidance for Industry (FDA.gov)

comprehensive resources and support tools to help individuals during this critical phase of their lives.

Each of these key issues demands its own attention, focus and funding to support patient communities. Yet insights derived from survey participants have identified complementary strategies and opportunities that can deliver meaningful impact in many populations.

"EVERY medical center that specializes in our condition is a pediatric center. Not one adult center exists worldwide."

– Survey respondent

THE OPPORTUNITIES: Progress through partnership

The diverse and challenging barriers identified by leading advocates in rare disease illustrate a daunting task to address resource constraints and deliver on unmet needs. Yet through thoughtful funding mechanisms and collaborations, advocates are finding ways to multiply their impact in their rare communities. The [#RAREis Global Advocate Grant program](#) has enabled important programs that demonstrate this potential, and could be replicated across many other areas.

Advocate participants defined several meaningful mechanisms of opportunity:

Community connections

Even with limited funds, advocates in the rare disease community are providing impactful and meaningful change by fostering strong community connections. Increasingly accessible online forums allow for advocates to facilitate information and experience sharing by connecting members of the rare community virtually. These platforms enable peer and caregiver engagement settings that not only enable exchange of culturally relevant and localized resources and education, but also address mental health, diversity and inclusion as well as

"Our program helps connect patients, caregivers and even family members to help them realize they are not alone. Connecting with others who are on similar journeys is a key element not only in education and support but also in mental health."

– Survey respondent

"Technology has been a blessing in that it has provided a means for rare families to connect with one another despite the distance. We are extremely grateful for organizations like #RAREis, Global Genes, and NORD for helping provide a place where rare disease individuals and organizations can connect to support one another."

– Survey respondent

transition of care challenges by directly accessing the experiences of others going through the same issues. On a micro and macro level, advocates who are successfully fostering connections within and across their communities are enabling real progress through personal partnership and support.

Collaborations with other advocates across disease types

Nearly all (91%) respondents in the survey reported partnerships with peers as a mechanism to share insights, resources and support opportunities for their constituents. In some cases, joint events create cost efficiencies but also spur new partnerships and identified shared issues that are harder to uncover in virtual or non-personal settings. Further, partnering with organizations focused in other disease areas or even pan-disease organizations provides a non-competitive environment to support patients, expand referral networks and identify categories of resources and support that can benefit patients and families regardless of their specific rare disease circumstances.



"We have found greater value in building relationships and partnerships with other nonprofits, even if they don't have the same disease group as us...if we can help elevate another disease group in the rare disease space, we've still won."

– Survey respondent

Cross-sector partnerships

Advocates also highlight the importance of pursuing collaborations with organizations across the rare disease ecosystem, including research institutions, hospitals, pharmaceutical companies and governments. These types of multi-dimensional partnerships help with patient support at various points of interaction, for example, through third-party funding for screening and genetic testing to rapidly accelerate the path to a proper diagnosis. In addition, joint efforts can serve to advance treatment ambitions, expand the reach of research and amplify comprehensive awareness and educational activities. Many efforts supported by funding partners serve the vital role of capacity and infrastructure building, addressing many of the constraints advocates often cite as rate-limiting factors to providing sustained support within their communities.

"We've partnered with a company that is offering free, rapid genetic testing to help patients get their diagnosis and get them on the path to treatment. The fact that the test is rapid is huge because it's critical in the early days for children to get the treatment. The other genetic tests were just taking too long."

**– Liz Molloy,
GACI Global**

"[Our organization] has achieved success through strategic partnerships and collaborations. By working closely with fellow advocacy agents, pharmaceutical industry partners, insurance industry partners, and healthcare facilities, [we have] been able to leverage collective expertise and resources to benefit the sickle cell disease community. These partnerships have facilitated improved access to care, increased awareness, and enhanced support systems for individuals and families affected by sickle cell disease."

– Survey respondent

Connections with public health leaders and organizations support the critical need for visibility of these rare diseases and enable dialogue that can advance policy changes on critical topics like newborn screening, and in some cases is influencing resource allocation for research funding and care access.

"Our engagement with government entities has yielded tangible outcomes, such as policy changes that enhance the lives of families dealing with rare conditions. By working together, we've made significant strides in advancing our mission, increasing support, and creating a more informed and compassionate society that stands united with the rare disease community."

– Survey respondent

Importantly, raising topics that are of relevance to the rare disease community, such as the need for more uniform access to diagnosis and treatment and the importance of diversity and inclusion principles, supports broader healthcare conversations and reinforces the need for policy shifts that can support a wide array of healthcare issues. For example, highlighting the need for diversity in clinical trials for heterogeneous conditions has become a priority for the U.S. Food and Drug Administration and other health authorities in recent years, with guidelines and



expectations now set in many trial platforms across disease areas.⁶

It should be noted that advocate respondents cautioned that these collaborations often take time to build momentum and many have faced challenges in relation to political motivations and competition for attention and focus. Yet, they serve critical purposes for progress that would be otherwise difficult to achieve independently.

Through the **#RAREis Global Advocate Grant**, **Adrian Goretzki**, president and founder of the **Healthcare Education Institute**, has been able to support **Ukrainian refugees living with rare diseases during the war**. In 2022, grant funds helped the organization develop educational brochures for 10 hospitals in Poland where Ukrainian patients with rare diseases are treated. In addition, they launched two patient portals and produced a series of video support guides for Ukrainians with rare diseases. In 2023, they used grant funds to create the **Brave RARE Ukraine campaign**, a series of videos featuring patients and their families, doctors and patient advocates from Ukraine.



CALL TO THE COMMUNITY:

Rethinking local activation for global impact

Journey toward progress in rare disease

The complex dynamic of advocating for the millions of people living with rare diseases around the world will not be solved with a single imperative. The community continues to grapple with strategies to improve diagnosis, treatment and quality of living for every individual with these rare diseases. The issues highlighted by the participants of the **#RAREis** survey – access to care, funding limitations, mental health, DE&I and transition of care – all represent significant issues that require global coordination and dedicated efforts to design and implement meaningful solutions.

However, examples from around the world highlight innovative and creative approaches advocates are taking today to address these, one disease and one topic at a time. Through programs like the **#RAREis Global Advocate Grant**, advocacy leaders are making a real difference for rare disease families.

Accelerating these efforts: a challenge to the community

Sharing insights and opportunities like those raised by the advocates in this survey can foster accelerated momentum through continued like-minded efforts and information sharing. Many foundational issues that are being addressed through these types of collaborations can be similarly applied to other disease areas and can provide real efficiencies in

⁶ Clinical Trial Diversity (FDA.gov)

the design, structure and implementation of meaningful, life-changing support programs.

These opportunities highlight the importance of community leaders – advocates, public health voices and industry alike – approaching these joint efforts as true partnerships. The challenges faced by the rare disease community can only be effectively addressed through committed, multi-year and ongoing efforts that leverage the strengths and contributions of all partners involved while addressing mutual goals to advance the state of care.

With scientific advances readily emerging to address unmet needs, coordinated partnerships can provide advocates with the tools, resources and capacity to effect real change in these communities through, for example:

- More readily accessible genetic testing and diagnosis pathways to shorten the diagnostic odyssey
- Facilitation of diverse participation in research programs that could generate novel therapies
- Integration of specialist networks and access to screening in lower income countries
- Inclusive support to relieve some of the mental and emotional burdens of living with a rare disease.

Perhaps more importantly, the insights above raise a fundamental principle that can redefine the approach to community support in rare diseases: namely that it benefits everyone to view these diseases as *one coordinated group* instead of individual diagnoses requiring distinct

mechanisms for support. If we can approach the community collectively, mining for and highlighting commonalities that can benefit many, we can drive faster and more effective progress worldwide.

Momentum in this philosophy is being driven by renowned global organizations whose missions are oriented toward improving care across rare diseases, such as Global Genes, National Organization for Rare Disorders, EURORDIS – Rare Diseases Europe, Rare Disease Diversity Coalition, The EveryLife Foundation for Rare Diseases, and many others.

More information on the impact of these organizations can be found at RAREisCommunity.com/resources.

The #RAREis community website provides valuable resources that can guide support on transitions of care and genetic testing



The outcomes driven by these organizations can offer learnings and inspire new thinking for organizations across the rare disease ecosystem.

In total, long-term cross-sector partnerships, sharing of insights and learnings, and proactive dialogue are key to moving faster for those who urgently await new solutions. As demonstrated through the #RAREis Global Advocate Grant program, supporting organizations that are living these principles is the catalyst for meaningful change that could redefine what it means to live with a rare disease for millions of people around the world.



APPENDIX

#RAREis Global Advocate Grant reach

The #RAREis Global Advocate Grant provides support to organizations that offer crucial programs and service for people living with rare diseases.



72
Advocacy
Organization



21
Countries



~70
Different Rare
Diseases

These statistics pertain to recipients from the years 2022 and 2023.

#RAREis survey respondents

A special thank you to the #RAREis survey respondents for contributing to this white paper.

11q Research & Resource Group

AGO Alliance (formerly AG02)

ALD Alliance

Aliança Distrofia Brasil

Alliance for Pulmonary Hypertension

Associação Brasileira da Síndrome de Prader-Willi

Associazione Nazionale Sindrome di Noonan e RASopatie ODV

Avery's Hope

Beck-Fahrner Syndrome Foundation

Bulgarian Society of Patients with Pulmonary Hypertension

Chordoma Foundation

Dutch Liver Patients Association

EBF3 HADDS Foundation

ECD Global Alliance

European Huntington Association

Foundation for Neuromuscular Support Nigeria

Foundation for Sarcoidosis Research

GACI Global

Glanzmann's Research Foundation

Instituto Atlas Biosocial

International Gaucher Alliance

Jansen de Vries Syndrome Foundation

MEPAN Foundation

NF Patients United – Global Network of Neurofibromatosis Patient Organizations

NGO (Rare Diseases of Ukraine)

Pediatric Epilepsy Surgery Alliance

PFIC Network

Philippine Society for Orphan Disorders

PNH Ukraine

Prader-Willi Syndrome Association USA

Project FAVA

PTEN Hamartoma Tumor Syndrome Foundation

Raising Hope International Friends

SCN2A Italia – Famiglie in Rete APS

SCN2A Australia

SHINE Syndrome Foundation

Soft Bones: The US Hypophosphatasia Foundation

The E.WE Foundation

The Life Raft Group

The Rory Belle Foundation

The Stiff Person Syndrome Research Foundation

Uniti per la P.I.P.O. ETS


Usher Syndrome Ireland

Virginia Sickle Cell Network

Survey findings


To further comprehend and gather insights on global advocacy issues in the rare disease community, the 72 #RAREis Global Advocate Grant recipients were surveyed to more deeply understand the biggest challenges they face and areas for opportunity to accelerate progress. Respondents to the anonymized 25-question survey represented **44 rare disease advocacy organizations** across **14 countries**.

Access to Adequate Care: Top 3 Drivers of Suboptimal Access




- Location of care
- Lack of provider education
- Financial burdens of care

Core Challenges in Patient Advocacy




- Socioeconomic
- Financial
- Language
- Diversity of staff
- Diversity in research

Critical Areas Impacted by Limited Funds




- Attention, partnerships and research funding
- Patient education, support and care resources
- Staff capacity and expertise

Transition of Care Challenges: From Pediatric to Adult



- Process guidance to transition care teams
- Emotional support for decision-making and independent disease management

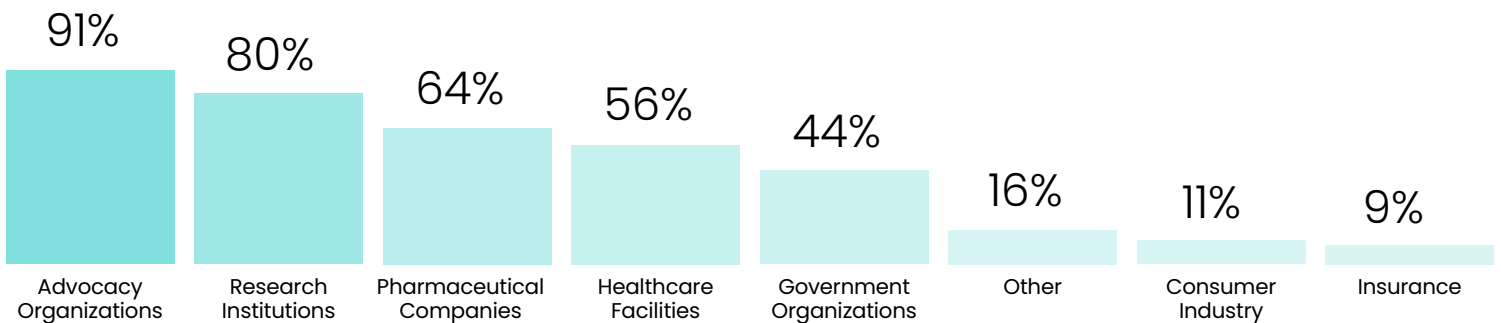
The Impact of Mental Health in Rare Disease:



95% of patients and 90% of caregivers experience mental health challenges

Rare disease advocacy group partnerships

The sectors outlined below have been recognized by recipients of the Global Advocate Grant as those with which partnerships have been established.



Organizational Partnerships



About #RAREis

Amgen (formerly Horizon Therapeutics) launched the #RAREis program in 2017 to elevate the voices, faces and experiences of people living with rare diseases, as well as highlight programs and resources for the rare disease community. Since then, it has grown into a multi-faceted program focused on building equity for the rare disease community.

About #RAREis Global Advocate Grant

Recognizing the significant needs around the world, the #RAREis Global Advocate Grant program was established in 2022 to provide critical financial assistance and foster growth for underserved advocacy organizations around the world. The grant program is designed to support the rare disease community by providing financial assistance to U.S. and global patient advocacy groups working to advance, educate and address the needs of the community. In two years, 72 advocacy organizations from 21 countries supporting nearly 70 different rare diseases have been awarded the #RAREis Global Advocate Grant.

In 2024, up to 75 organizations will be awarded.

For more information and to apply, visit RAREiscommunity.com/Grant.

About Amgen

Amgen harnesses the best of biology and technology to make people's lives easier, fuller and longer. We draw upon our deep knowledge of science to push beyond what's known today. With roots in the biotech revolution, we are one of the world's leading independent biotech companies.

We are on the cutting edge of medical discovery, reliably inventing powerful therapies. We are focused on fighting the world's toughest diseases and helping millions of people globally.

For more information, visit Amgen.com.

