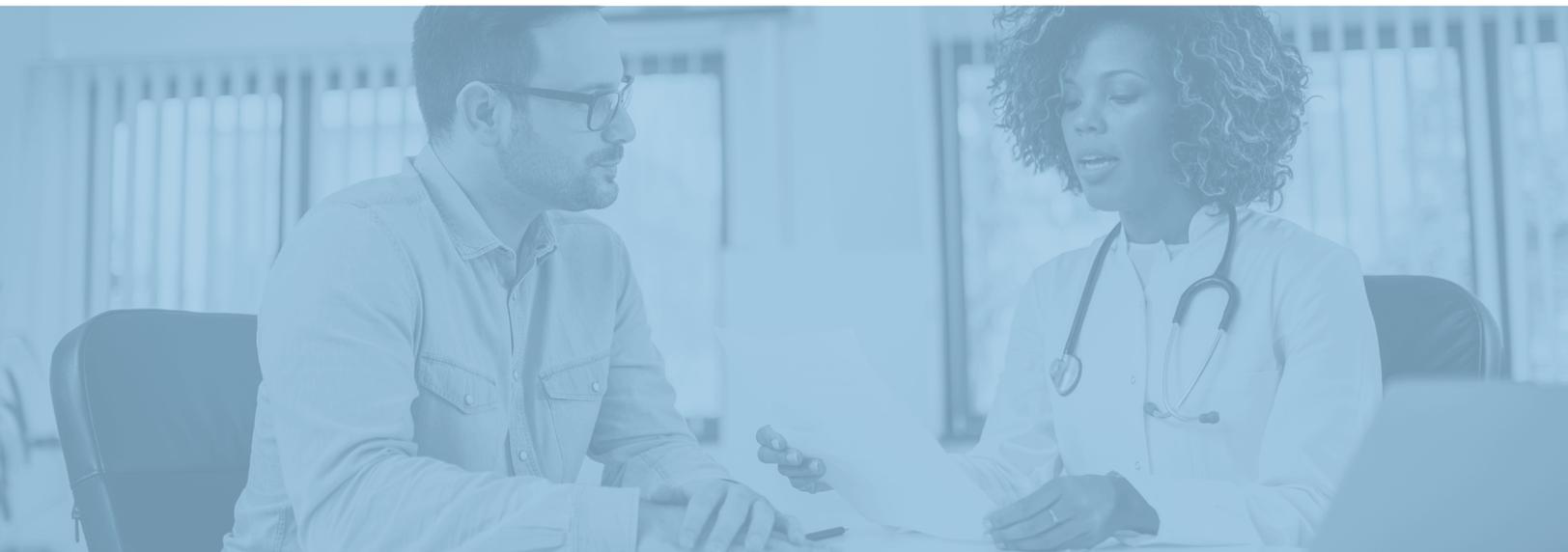




SEPTEMBER 2021

# Financial Literacy and Navigation In Rare Disease



# About **Global Genes**®

---

Global Genes is a 501(c)(3) non-profit organization dedicated to eliminating the burdens and challenges of rare diseases for patients and families globally. In pursuit of our mission we connect, empower, and inspire the rare disease community to stand up, stand out, and become more effective on their own behalf—helping to spur innovation, meet essential needs, build capacity and knowledge, and drive progress within and across rare diseases. We serve the more than 400 million people around the globe and nearly 1 in 10 Americans affected by rare diseases. If you or someone you love has a rare disease or are searching for a diagnosis, contact Global Genes at 949-248-RARE or visit our resource hub at [www.globalgenes.org](http://www.globalgenes.org).

DONATE TODAY: **Text RARE to 41444**  
**Visit <https://globalgenes.org/donate-now>**

FOLLOW US:  
**@globalgenes**

# Acknowledgments

---

**Lauren Alford**

Senior Marketing Communications  
Director

[Global Genes](#)

**Nikki Berry**

Senior Brand Manager

[Global Genes](#)

**Hira Chowdhary, MPH, MS**

Senior Program Manager,  
Engagement Programs

[Global Genes](#)

**Marie Daghlian**

Copy Editor

[Global Genes](#)

**Craig Martin**

Chief Executive Officer

[Global Genes](#)

**Pam Rattananont, MPH**

Vice President, Development and  
Marketing

[Global Genes](#)

**Christian Rubio**

Vice President, Strategic  
Advancement

[Global Genes](#)

**Pam Todd**

Educational Resource Strategist

[Global Genes](#)

# Contributors

---

**Cristol O’Loughlin**

Founder and CEO, Angel Aid Cares  
[angelaidcares.org](http://angelaidcares.org)

**Doug Strott**

Founder, The Scheuermann’s  
Disease Fund  
[sdfund1.org](http://sdfund1.org)

**Kathleen McGlone Campbell**

Rare Mom, Teacher, Science Lab  
Coordinator, and STEM Educator

**Christina Hartman**

Vice President, External Affairs  
The Assistance Fund  
[tafcares.org/](http://tafcares.org/)

**Nola Just, MPH CCRP**

Clinical Research Program  
Manager, Division of  
Gastroenterology, Hepatology and  
Nutrition, Children’s Hospital of  
Philadelphia, and SCD Advocate  
and Ambassador for Sick Cells  
[chop.edu](http://chop.edu)  
[sickcells.org](http://sickcells.org)

**Thistle Editorial LLC**

[thistleeditorial.com](http://thistleeditorial.com)

***Supported by generous  
charitable contributions from***  
Pfizer, Inc  
Horizon Therapeutics

# Table of Contents

<b>Introduction</b>	<b>2</b>
Financial Literacy and Navigation in Rare Disease	2
<b>Steps to Improve Healthcare Access and Affordability</b>	<b>4</b>
Identify Potential Roadblocks to Healthcare Access and Affordability	4
Anticipate Potential Costs of Care	5
Navigators	8
Advocate for Yourself and Your Family	8
Establish Your Healthcare Team	13
Finding Care Providers	13
Steps Toward Effective Communication	14
Share Care-Related Decision-Making with Your Healthcare Team	14
Care Coordination	15
<b>Navigating Access to Different Types of Care</b>	<b>18</b>
Specialized Care	18
Access to Medicines	18
Clinical Trial Participation	20
Support Your Mental Health	21
Telehealth	24
<b>Health Insurance Literacy Matters</b>	<b>25</b>
Money Matters: Planning For Financial Success	27
Build a Financial Knowledge Bank	28
<b>Financial and Health Insurance Literacy Resources</b>	<b>31</b>
<b>Glossary</b>	<b>34</b>
<b>References</b>	<b>39</b>

# Introduction

---

## Financial Literacy and Navigation in Rare Disease

Approximately one in 10 people living in the United States are affected by rare diseases.<sup>1</sup> According to the 2020 *National Economic Burden of Rare Disease* study conducted by the Everylife Foundation for Rare Disease, families living with rare diseases absorb more than \$966 billion in costs for their diagnosis, care navigation, and treatment. The economic burden of rare disease is vast and comprises a widescale public health challenge.

If you are living with a rare disease or caring for someone with a rare disease, you already know how navigating healthcare and finding the finances to cover care can quickly strain household and individual finances and contribute to financial hardship. The search for an accurate diagnosis is often expensive and time-consuming, and medical costs can be high. Household finances can also be negatively impacted if you or your caregivers lose wages from time out of the workforce or are unable to find employment with flexible leave or health insurance. At the same time, health care transactions, plans, and terms are often complex, with several roadblocks on the way to care that absorb time, energy, and can cause distress.

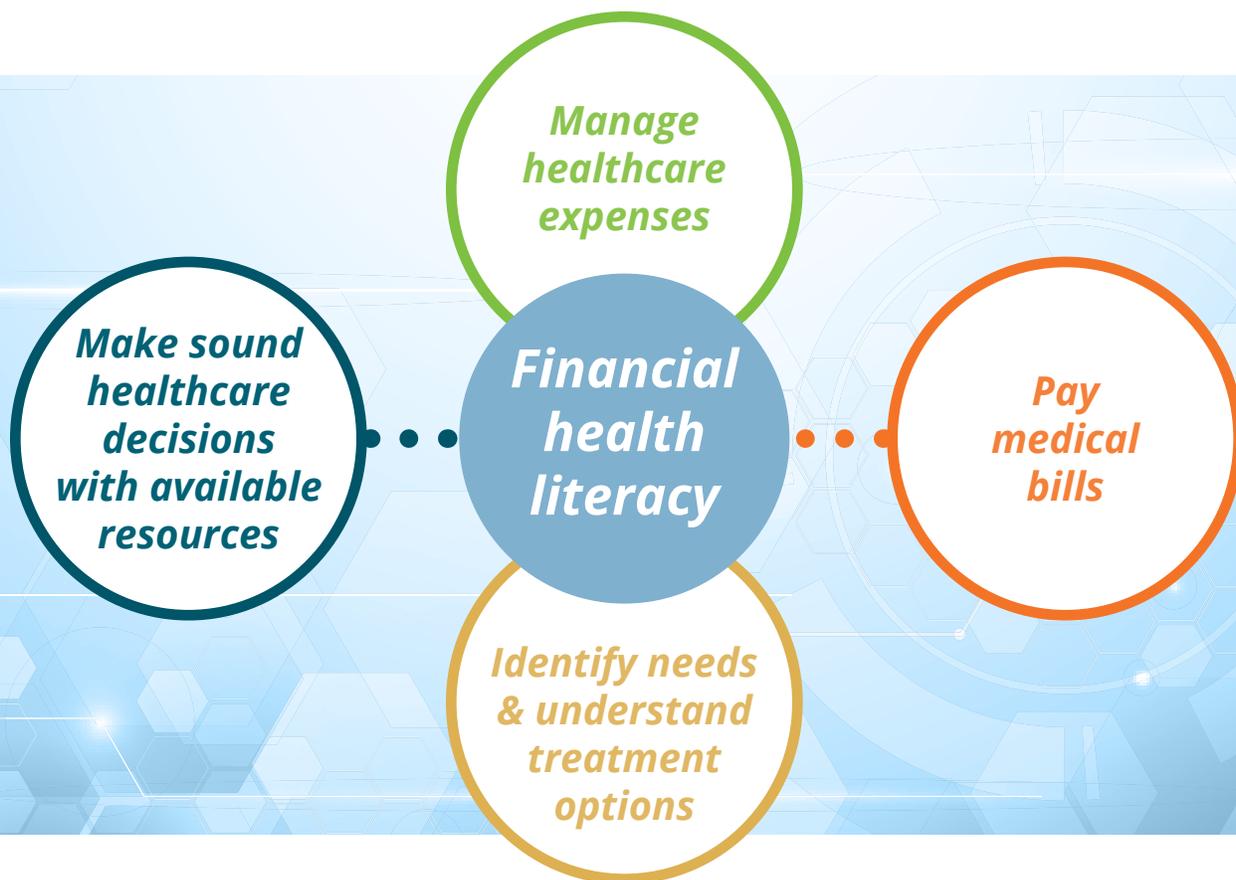
Few of us have been exposed to financial and health insurance literacy education in school, college, or through work. But the path to financial literacy and effective healthcare navigation has a roadmap.

The goal of this toolkit is to provide information and resources to help you navigate access to healthcare, build financial confidence, and identify ways to pay for healthcare.

## Financial and Health Insurance Literacy

**Financial literacy** in the context of health involves the ability to assess, understand, and use financial information in ways that support good health and financial outcomes.<sup>2</sup> Financial literacy in the context of health and healthcare involves being equipped to:

- Identify your needs and understand your treatment options
- Make sound healthcare decisions with available resources
- Manage healthcare expenses
- Pay medical bills



**Health insurance literacy** involves building knowledge and skills to do the following:

- Find and evaluate information about health plans
- Select the best plan given financial and health circumstances
- Use the plan once enrolled<sup>3</sup>

Building health insurance literacy skills can increase your awareness of financial or social benefits to which you or your family member might be entitled following diagnosis.

This toolkit focuses on the following topics:

1. Potential roadblocks to healthcare access and affordability
2. Anticipating costs of care
3. Working with your healthcare team
4. Advocating for your healthcare needs
5. Navigating access to healthcare
6. Financial literacy
7. Steps to build a financial knowledge bank

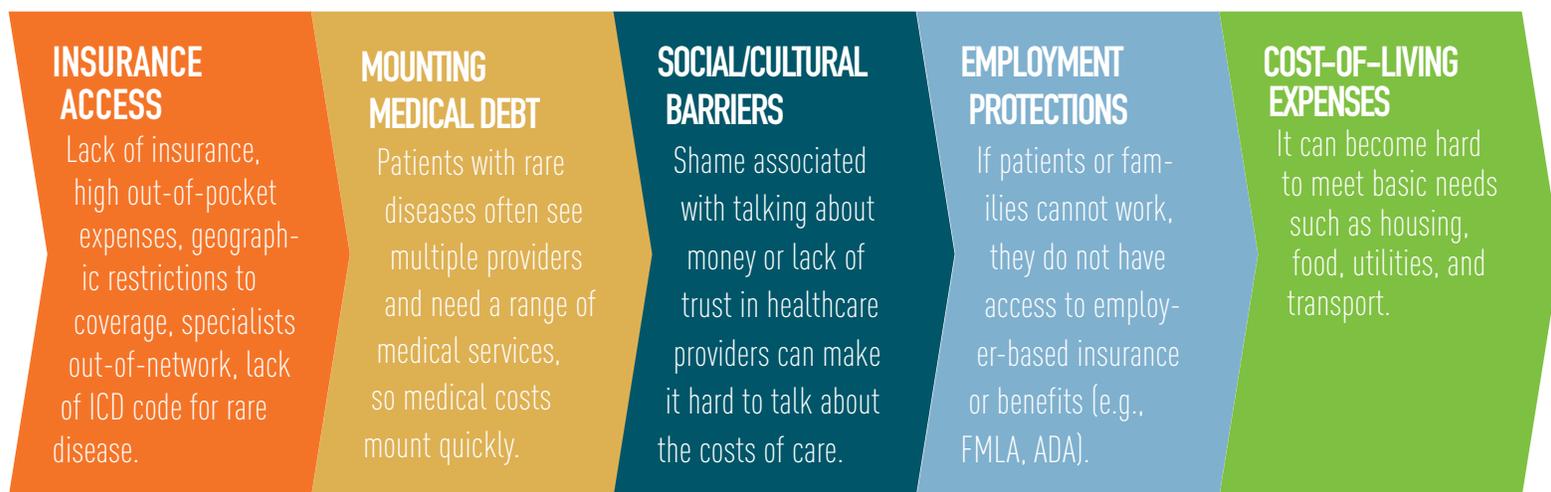
Financial and health literacy terms used in this toolkit are highlighted on first reference. You'll find a full list of terms used [here](#).

# Steps to Improve Healthcare Access and Affordability

## Identify Potential Roadblocks to Healthcare Access and Affordability

You might already have encountered or are aware of roadblocks that make it challenging to access and pay for healthcare (Figure 1).

**Figure 1. Potential Roadblocks to Healthcare Access and Affordability**



*Adapted from a presentation given by Alan Richardson, Executive Vice President of Strategic Patient Solutions, Patient Advocate Foundation, at the Global Genes 2020 RARE Patient Advocacy Summit.*

Due to delays and missteps, it can take five to seven years before some patients are accurately diagnosed with a rare disease. During this time, patients might see several doctors, have multiple tests, and perhaps be hospitalized or need specialized care. These tests and care services incur expenses that might not be reimbursed because there is not yet an **International Classification of Diseases** (ICD) code for your disease.

When there is no specific code, providers use codes that describe symptoms but not the underlying disease. This means that patients and providers often have to make the case for each care step, including not only therapies, but also equipment or procedures, and often have to pay more out-of-pocket for these services. In addition, health insurance companies have implemented strategies such

as managed care plans, cost sharing, tiered-services, and benefit reductions. These strategies might reduce healthcare costs at the health system level but increase costs to individuals.

Awareness of these potential roadblocks can prepare you to move over or around them should they appear. See **Pro Tip 1** for self-advocacy action if you are denied coverage for care.



## PRO TIP 1: SELF-ADVOCACY IN ACTION

Ask your insurer for the explanation of benefits and the denial of coverage so that you are clear about the grounds of denial. As the expert in living with your disease, you can help the insurance company understand why you need the treatment.

- Educate your insurance company about your disease and your needs. Share your rare disease story and why your circumstances might require an “exception to the rule” approval.
- Practice what you are going to say with a friend or family member so that you can feel confident being on the phone with insurance representatives.
- Call the nurse hotline for your insurance company—nurses are usually in tune with what’s going on in other departments. If a nurse is unavailable, ask to speak to a supervisor.
- Write down who you speak to, the date, time, and anything that the insurer needs from you.
- Ask your doctor to initiate the appeals process.
- Don’t take no for an answer. Be polite and persistent and get the answer you need.
- If you come to a dead end, find a health lawyer in your state who can assist with insurance appeals and represent you in court.
- The RARE Toolkit on Navigating Health Insurance has additional information on this topic.

## Anticipate Potential Costs of Care

### Types of Medical and Non-Medical Care

The kind of care that people living with rare disease need vary by disease, age, and associated disabilities. The care that someone needs can also change over time. Common types of care include hospitalizations, emergency room visits, travel to disease specialists, tests, and surgeries as well as medications. These types of care are defined as medical or non-medical, but both incur costs (Table 1).

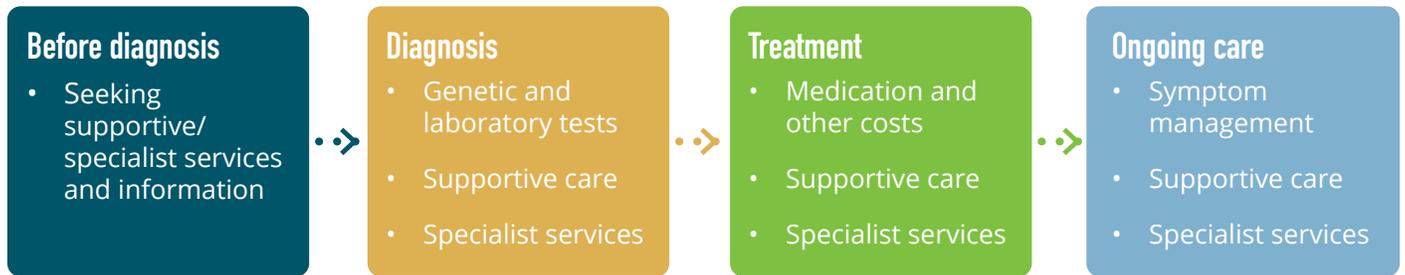
**Table 1. Examples of Care Costs**

Medical Costs of Care	Non-Medical Costs of Care
Treatment (medications, surgeries)	Personal care assistance
Emergency room visits	Adaptability resources for the home
Hospitalizations	Mobility assistance equipment
Symptom management	Travel to see disease specialists
Long-term supportive care	Adapted transport
Specialized medical nutrition or formulas	Psychological support
Tests	Medical devices
General nursing and nursing aide support	Education
Specialist or rehabilitation services (e.g., physical, occupational, or speech therapy, respiratory support)	Posture

### Increasing Costs of Care

Many of these types of care are not directly covered by health insurance or are covered only after meeting a high **deductible**. In some cases, such as **out-of-network** specialist care, costs incurred do not count toward **out-of-pocket** limits. High deductibles, high copays, and more narrowly defined **in-network** care have become more common features of health insurance plans in the last ten years. At the same time, many rare disease patients and their families are under- or uninsured or have health insurance with high deductibles and coinsurance payments. In the last decade, the price of health insurance premiums has increased by 55 percent and the average deductible for marketplace plans is more than \$3,000.<sup>4-5</sup> As a result, out-of-pocket costs have increased for many patients and their families and may occur at different points along the rare disease journey (Figure 2).

**Figure 2. Points of Financial Strain across the Rare Disease Journey**



The following tactics can help you avoid being hit by a surprise bill for costs of care or high out-of-pocket expenses:

- Know what your **health insurance plan** covers before medical bills mount up.
- If you do pay for care out-of-pocket before your health insurance covers costs, you can request retrospective reimbursement for a portion of the amount you have already paid.
- Talk with your health care provider before they order diagnostic tests or make treatment decisions (**Pro Tip 2**).
- Plan ahead for costs associated with hospital or clinic visits, including:
  - » Hospital and clinic parking alone can cost \$2,000 or more per year.<sup>6</sup>
  - » Food for caregivers during treatment days/hospital stays and clinic visits can be expensive.
  - » Gas for travel back and forth to appointments especially if traveling out of state to see specialists.
  - » Lodging if traveling out of state to see specialists.
- If you are employed, Human Resources should be able to tell you if your employer offers a **health savings account**. This account can help you set money aside for unanticipated and out-of-pocket costs.
- Where possible, negotiate medical bills.



## RARE TIP



Most medical bills are negotiable. You can pre-negotiate a 10 to 50 per cent discount for medical services if you pay cash, if you pay up front, or on check-in. Even after you receive the bill you can negotiate payment plans and sometimes if you can settle the bill 2-3 months into a payment plan you can ask the insurance company for a further discount. You can save thousands of dollars in medical expenses by asking for a discount.

– CRISTOL O'LOUGHLIN, CEO, ANGEL AID CARES



## PRO TIP 2: EXPECT TRANSPARENCY ABOUT COSTS OF CARE

Many of us feel shame when talking about our finances, which can make it difficult to talk with providers about the costs of care. But new approaches and laws in healthcare mean that providers are supposed to involve patients in cost conversations through a process of **shared decision-making**. Many health plans, consumer groups, and state governments also support price transparency through cost comparison tools.

If your providers do not ask you about your preferences about care or raise the issue of cost, ask for a cost estimate or if resources or tools are available to support shared decision-making about treatment and the costs of care.

Questions you can use include:

- What printed, audiovisual, or digital information is available about the costs of the available treatment or supportive care options?
- Are there any price comparison tools available to help you to make informed decisions about treatment costs? Price comparison tools include:
  - » *Healthcare Bluebook*
  - » *New Choice Health*
  - » *Fair Health*

- What resources are available for patients facing or dealing with financial burdens associated with the costs of care?
- Who do I go to for questions about the cost of care?
- Can your provider refer you to a patient **financial assistance program** if you are unable to pay the costs of care?

Resources such as *FAIR Health* offer information on average care costs associated by CPT and zip code.

### Navigators

Your hospital or provider might be able to provide access to **financial navigators** who are trained to provide guidance and direction toward resources to reduce the costs of care and medications. Ask your provider if they can put you in contact with a financial navigator.



Nola Juste, from the Division of Gastroenterology, Hepatology and Nutrition at the Children's Hospital of Philadelphia, has sickle cell disease. She recommends using a financial navigator where possible. She

had a second opinion visit and was called by the financial navigator in the state hospital who reviewed her insurance and billing.

## Advocate for Yourself and Your Family

Living with a rare disease probably means that you or your caregivers spend a lot of time searching for relevant disease-related information, contacting and liaising with providers and specialists, sourcing and acquiring adaptive equipment, and seeking support services. While each rare disease has its own characteristics and impact on quality of life, people living with rare diseases often share similar experiences and challenges, such as having limited information about available treatment and care, lengthy times to diagnosis, and a sense of isolation. These experiences can offer a powerful platform for advocating for your or your family member's health needs.

# Challenges with Access to Durable Medical Equipment



Christina Hartman, of The Assistance Fund, whose daughter has a rare disorder, notes that access to durable medical equipment, such as wheelchairs or safety beds, can be challenging. Here is her story –

*We needed a special needs safety bed for my daughter. We weren't really sleeping at night, since she could get up at night and wander the house, which we'd had to baby proof because she has very little executive function. We'd either have to sleep with her, or, when she outgrew her crib, we ended getting a nondurable inflatable special needs safety travel bed that she slept in for a couple of years. Finally, I was able to get a special needs safety bed after 16 months of back and forth with the insurance company. The insurance company tried to say it was "furniture" and denied it. Now we have the safety bed, a wheelchair stroller, and a Rifton Chair and when the supplier came to the house to deliver the wheelchair stroller and Rifton Chair [we already had the safety bed], they had us sign a waiver saying we'd be responsible for approximately \$5,000 per item if our insurer denied coverage. I've had to battle every little thing with insurance.*

## PRO TIPS

- Check whether your patient advocacy organization runs an equipment share program
- Local charities, small grants, or personal fundraisers are options to fill a funding gap for equipment.

The following steps can also help you to build advocacy know-how and benefit from the pooled expertise of other people living with rare disease:

- **Become an informed patient/caregiver.** Having access to the right tools and information is vital to advocating for yourself or your rare disease family member. Many rare disease patient advocacy organizations offer the following resources:
  - » Information helplines about clinical trials and financial assistance
  - » Support services for caregivers
  - » Education to build knowledge capacity in the scientific landscape around rare disease
  - » Training in communication skills that are essential for engaging with healthcare providers and shared decision-making about care
  - » Guidance on resources to support access to education that addresses your child's needs
- **Cultivate your expertise.** Although it might feel daunting to be the expert in your rare disease, no one knows about living with a rare disease better than patients and their caregivers. Your experiences offer a starting point for physicians and other healthcare providers to plan your care and provide support.
  - » Learn how to identify and describe your healthcare needs.



- » Share your story and describe your symptoms so that you and your provider have a foundation for communication. This foundation will help your provider identify and refer you to appropriate specialists and other services, if needed.
  - » Ask questions that will help you understand medication instructions, billing, and other topics.
  - » Be as proactive as possible.
- **Find a supportive rare disease group.** Being a member of a supportive group can help you connect with people who share your rare disease or symptoms and build self-advocacy skills. It can be helpful to acknowledge that everyone needs love and support from family, friends, and peers who understand what it is like to live with a rare disease.
    - » [RareConnect.org](https://rareconnect.org) is one place to start looking for rare disease support groups communities, or use this [Global Genes guide](#) to build a support group for your community.
    - » [Global Genes Rare Foundation Alliance](#) is a coalition of over 750 rare disease organizations that can help you find your community
    - » [The RareGuru](#) app can also help you find other people that are experiencing similar challenges to you. The app is free and available in the United States, Canada, Australia, New Zealand, and Ireland.
  - **Get involved.** Patient advocacy organizations are central to gathering, producing, and sharing information about rare disease. Consider joining an advocacy organization such as Global Genes to help raise public awareness of rare disease and ensure that patient voices are heard by health care systems, pharmaceutical manufacturers, politicians, and your community.

# Advocate for an Individualized Education Program for Your Child

**Individuals with Disabilities Education Act.** Education is a non-medical cost that can strain household resources and even, in some circumstances, lead to debt. Under the Individuals with Disabilities Education Act (IDEA), public schools are required to provide special education services for all children aged three to 21, at no cost to families.

**IDEA** also entitles children with disabilities two years old or younger to receive early intervention services, such as physical, occupational, speech, and nutritional therapy. *The Early Childhood Technical Assistance Center* provides additional detail about early intervention and Individualized Family Service Plans (IFSP).

**Individualized Education Program.** Individualized education programs (IEP) are plans of action that map out the special instruction, physical or medical accommodations for learning, behavioral support, and academic intervention your child needs. Parents play a critical role in identifying and communicating about their child's disability-related needs to other members of the IEP team. The IEP team determines the type and level of services to be offered to meet those needs and is typically comprised of the following:

- One or both parents
- At least one general education teacher
- At least one special education teacher
- A school district representative

- An expert to interpret the special education diagnosis and plan (e.g., a school psychologist).

**Establishing an IEP.** Each U.S. state has its own process for establishing an IEP, but usually, when a parent feels their child needs evaluation for an IEP, the Committee for Special Education at their state Department of Education is the first stop. Once evaluations are completed, the IEP team reviews the reports and makes service or intervention recommendations.

## RARE TIP

Kathleen McGlone, a rare advocate and teacher, emphasizes how important it is for parents to advocate for the best possible plan for their child and identify the types of evaluations they think their child needs.



She recommends the following strategies to support an effective IEP process.

- **Bring Your Support Team.** Take someone with you to IEP meetings such as a parent who is familiar with the process or a special education lawyer, who can record meetings and advocate for you. If you wish to record an IEP meeting, you must give the Committee on Special Education prior notice.
- **Be Aware of the Timeline.** The timeline for establishing an IEP varies from state to state but expect to

begin the evaluation process within three weeks of filing an IEP request. It can take at least three months for evaluations to be completed, depending on the number of providers involved in evaluation (e.g., occupational, speech, physical therapy, neuropsychological, psychosocial).

- **Request an Independent Educational Evaluation.** The Department of Education can drive the evaluation process. However, IDEA entitles parents to request an independent educational evaluation and to select their preferred providers to conduct the evaluations. You can also request second opinions and ask for as many evaluations as required. Under IDEA, the state is required to pay for evaluations and second opinions.
- **Evaluate Provider Expertise.** Call providers to ensure that they have expertise in your child's rare disease. Ask how many evaluations they have done concerning this rare disease and how much they charge. On receipt of this information, the Department of Education should send payment to the provider.
- **Request a Second Opinion.** Parents can request a second opinion for an IEP, as well as several evaluations. If parents think their children need a range of therapies, such as occupational, speech, physical, and music therapy, paraprofessionals, or counseling, they can advocate for evaluations and for these therapies to be included in an IEP.
- **Read the Evaluation Report.** Reports hold critical information to support the design of an IEP that

is tailored to your child. Should a Committee on Special Education offer a particular therapy or service less frequently than you know your child needs, direct the Committee to the report details.

**Appealing IEP Decisions.** Independent education evaluation is not always approved. As a parent, you can appeal a negative decision. You can also appeal the levels of services offered for your child following evaluation and request an impartial hearing. Kathleen McGlone notes that it is important for parents to be well-informed for hearings and appeals and present a variety of data to the judge, including photographs, drawings, and additional evaluation information (which the state is required to pay for).

It can be challenging to mount an appeal, but they can be successful.

## RARE TIP

"People are scared that they will not win an Impartial Hearing. They think they do not know enough to navigate the legal system but you do! You as a parent will win for your child. You win especially when you know your child's needs. You are the best advocate for your child and an Impartial Hearing officer will listen to you about the needs of your child. You must have all of your data and reports ready to present your case. You know what your child has, if it's a rare genetic disorder, and if much is not known about their disorder you are their voice. Impartial Hearing officers will listen to you because you are the most educated on your child's condition. Then your child's case is pretty simple to win. "

– KATHLEEN MCGLONE, RARE MOM



## Establish Your Healthcare Team

Your primary care provider should be able to manage your day-to-day care and help you to maintain your overall health, treat any symptoms, approve medical equipment, and refer you to relevant specialists. But dealing with rare disease symptoms, treatment, lifestyle changes, and physical/psychological challenges needs team support. Members of your team might include family members, physicians, and other healthcare providers like nurse practitioners, physician assistants, pharmacists, case managers, clinical social workers, and financial advocates. Access to this collective support can help you coordinate care across different services and medical specialties and build confidence and skills in disease self-management.

## Finding Care Providers

Finding a physician with expertise in your rare disease is one of the most important medical decisions you are likely to make. The doctors and healthcare providers available to you are largely driven by what health insurance plans allow. Doug Strott, founder of the Scheuermann's Disease Fund, also recommends finding referrals for physicians and surgeons from other contacts in your patient advocacy group or network and reading testimonials from other patients.

Consider the following steps when searching for a physician or surgeon with specialist expertise in your rare disease.

- **Be prepared.** Create a list of questions to ask the physician about their expertise with your

rare disease. See and adapt these examples from the Scheuermann's Disease Fund.

- **Enlist a Friend.** Take someone with you when you see a physician, especially for the first time. They can take notes and even audio record, providing you request permission upfront.
- **Bring Test Results and Medications.** It is important for potential physicians to have as much information as possible about your past medical history and current treatment, so that they can create a tailored treatment or surgical plan.

The following additional resources can also help you find providers that specialize in your rare disease.

---

Obtain a list of providers that your health plan allows (in-network) from your insurance company's website.

---

Peer review websites provide patient ratings for physicians (e.g., <http://www.vitals.com>).

---

Patient advocacy organizations that focus on your rare disease can provide information on providers and specialists.

---

The [\*National Institutes of Health Genetic and Rare Disease Information Center\*](#) provides guidance on finding specialists.

## Steps Toward Effective Communication

Communicate with your healthcare team so that they can anticipate your healthcare needs and coordinate your care.

**Keep track of information about your healthcare needs.** Many providers use electronic health or medical records to communicate with each other and support care coordination.

- Create and maintain a care notebook to gather and organize information about your or your family member's care. Care notebooks can be print, online, or app-based, such as *My Medical App*. Learn more from Global Genes about creating and maintaining a *care notebook*.
- Have a written summary of your care plan.
- The *Global Commission on Rare Disease* has launched a campaign to ensure that patients and caregivers have access to secure technology that allows rapid and efficient sharing of detailed medical records with multiple providers.

Use the patient portal to ask for your medical records to be sent to relevant providers and specialists to ensure everyone is on the same page about your needs.

### **Ask for self-management support.**

Providers will not always be familiar with your or your family member's rare disease. You might need to drive the communication process between providers, especially if you are seeing

more than one provider, or providers at different clinics. Monitor and document your condition so that you can:

- Alert your care team to changes in symptoms, daily functioning, or disabilities
- Ask for support in managing complex medication regimens
- Maintain adequate levels of nutrition and physical activity
- Find psychological and mental health support

**Ask providers to explain unfamiliar terms.** Healthcare providers often use medical or health insurance terms that are not always easy to understand. If you hear terms that sound unfamiliar, ask your provider to explain what they mean.

## Share Care-Related Decision-Making with your Healthcare Team

Shared decision-making is a collaborative process in which providers and patients jointly make decisions about care after discussing available options, potential benefits and risks, and considering the patient's values and preferences. There are different ways to approach shared decision-making. The Three Talk Model describes a common model that your provider might use (Figure 3).<sup>7</sup>

**Figure 3. The Three Talk Model for Shared Decision-Making**



In shared decision-making, patients are supported to make informed decisions about care and invited to reflect on their values and preferences, sometimes with the help of decision aids. Decision aids are available in many formats (e.g., print, video) and usually include information about the risks and benefits of treatment. Although there are currently few validated decision aids for rare diseases, the following websites share information that could be helpful in shared decision-making:

- [www.eurodis.org](http://www.eurodis.org)
- [www.orpha.net](http://www.orpha.net)
- [www.patientslikeme.com](http://www.patientslikeme.com)
- [www.rareconnect.org](http://www.rareconnect.org)

### Care Coordination

Care coordination involves working with your providers to avoid duplicating effort, tests, and interventions and to achieve shared outcomes. In rare

disease, it is common to need access to multiple specialists and therapists across different locations and days. Some healthcare providers or insurance companies employ care coordinators—such as registered nurses, health educators, or patient navigators—to help manage care and keep track of complex care arrangements.

Examples of care coordination duties include:

- Coordinate insurance coverage
- Plan healthcare visits (e.g., schedule visits, reconcile medications, order refills)
- Follow-up with providers involved in you or your child’s care
- Answer your questions



## PRO TIP 3: STRATEGIES TO OPTIMIZE CARE COORDINATION

Not all healthcare systems have a designated person tasked with care coordination. Christina Hartman of The Assistance Fund recommends asking your main point of contact, such as your pediatrician or primary care provider, for help to coordinate care across specialist departments and settings. Some rare disease organizations have developed mobile health apps to support care coordination.

The Marfan Foundation *healthcare management tool* allows people living with Marfan syndrome to collect and store health information from disparate providers and create a portable health record.

The *Good2Go MyHealth Passport*, developed by Shriners Hospitals for Children Canada, is an online tool that stores personal health data. A summary can be printed as a personalized data card and shared with providers or in emergency situations.

Other strategies to support care coordination include:

**Create a health passport.** Or find a way to keep all your healthcare files in one place. You might have to share healthcare documents or information with several providers if they are not connected via electronic health records.

**Lean on your patient or rare disease organization.** These organizations often provide coordination tools and resources for particular rare diseases.

**Talk with your pediatrician or primary care provider.** Written care plans, patient registries, and having a key point of contact are all methods that can support care continuity. Ask your provider what options are available.

Nola Juste encourages patients with rare disease to be proactive and take the lead in driving care coordination. She says, “Patients can take their medical needs elsewhere. Medical care is not cheap. If your provider gives you a hard time because you request that they check in with your other providers, you can say, ‘I’ll find somebody else.’ And I have done that.”



– NOLA JUSTE, CLINICAL RESEARCH PROGRAM MANAGER FOR THE DIVISION OF GASTROENTEROLOGY, HEPATOLOGY AND NUTRITION AT THE CHILDREN'S HOSPITAL OF PHILADELPHIA

### Pro Tip 3 Continued...

Nola, a Clinical Research Program Manager for the Division of Gastroenterology, Hepatology and Nutrition at the Children's Hospital of Philadelphia, recommends the following strategies to optimize care coordination.

- Build rapport with your physician so you feel comfortable asking them to take the lead in scheduling when you have same-day visits with more than one provider in the same health system.
- If you receive care from providers in different health systems or clinics, ask your primary provider to check in with other providers and share healthcare records.
- Ask your provider how you can partner together to work toward the same goals, prevent complications, and improve or maintain quality of life. This shows providers that you are advocating for yourself and value partnership with them.
- Create a system for organizing all your care coordination communication documents, questions, responses, and tasks. Digital platforms like [Evernote](#) or [Google](#) make it easy to store and retrieve information but even a file folder or box will work.



# Navigating Access to Different Types of Care

## Specialized Care

In addition to primary care, patients living with rare disease typically need access to specialized clinical care. European Reference Networks (ERN) were established in 2017 to promote diagnosis, treatment, and provision of care across a wide range of rare disorders. ERNs focus on pooling and providing access to knowledge and expertise about rare diseases across geographic boundaries. Learn more and access the ERN directory [here](#).

In the United States, some rare disease foundations have established databases of physicians with expertise in treating certain rare diseases (e.g., like this [database for Scheuermann's Disease](#)), or networks of accredited specialty centers or Centers of Excellence, including:

- [Cystic Fibrosis Foundation](#)
- [Von Hippel-Lindau Alliance](#)
- [Charcot-Marie-Tooth Association](#)
- [Children's Tumor Foundation](#)
- [Sarcoidosis Centers of Excellence](#)
- [Children's National Rare Disease Institute](#)
- [Sickle Cell Disease Association of America](#)

Centers of Excellence are structured around the goal of providing coordinated, specialized care for patients with rare diseases. Centers of Excellence are also usually actively engaged in research. Patient advocacy groups, specialist physicians, and other stakeholders are usually involved in establishing Centers of Excellence. Learn more about how to establish a rare disease Center of Excellence from [Global Genes](#) and from the [UK's Centres of Excellence for Rare Diseases](#).

## Access to Medicines

Most rare diseases do not yet have an approved treatment. Therapies that are approved specifically for rare conditions can be extraordinarily expensive. Prescription drug costs and associated insurance payments can contribute to high out-of-pocket spending, even for patients with private insurance coverage (in the United States) or in countries with state insurance. Patients and their families in the United States are being asked to share an increasing proportion of drug costs (e.g., through higher deductibles) even though wages have not increased for many years and the overall cost of living in many parts of the United States remains high.

## Resources to Get Access to Medicines

The following resources potentially offer access to medicines that might otherwise be difficult to afford:

- **Expanded Access/Compassionate Use Programs.** These programs are options to consider as pathways to access investigational drugs. Expanded Access/Compassionate Use drugs are medicines that are still in clinical development and not yet approved by the U.S. Food and Drug Administration (FDA) or the European Medicines Agency (EMA). Talk to your provider if you are thinking about exploring access to medicines via an Expanded Access/Compassionate Use Program and learn more about these programs at [fda.gov](http://fda.gov) and, for Europe, [ema.europa.eu](http://ema.europa.eu).
- **Prescription Assistance.** **Prescription assistance** services like the *Partnership for Prescription Assistance* and Medicare's *Pharmaceutical Assistance Program* are helpful if you do not have health insurance or are without prescription drug coverage for access to medicines.
- *Patient Services, Inc.* (PSI) provides financial support and guidance for qualified patients with specific, rare chronic diseases. The following services provide additional information, including about medication coupons and rebates, and copay assistance:
  - » *GoodRx*
  - » *HealthWell Foundation*
  - » *NeedyMeds*

- » *RxAssist*
- » *RxHope*
- » *Rx Outreach*
- » *The Assistance Fund*
- » *National Organization for Rare Disorders (NORD)*

## Drug Manufacturer Patient Assistance Programs (PAPs)

Manufacturers can also supply a drug to a group of patients with an identified need via specific criteria (treatment protocol) that defines the population that can have access to the drug. This access is usually free of charge to patients, although manufacturers might charge a fee to cover manufacturing and/or shipping costs. Drug manufacturer patient assistance programs can also help pay for medications if your insurance plan denies coverage for a particular drug. Many of these services are provided via third party arrangements with patient advocacy or other groups. PAPs can assist with the following services:

- Assign a case manager who will contact the insurer on your behalf, determine whether the drug is covered, identify what your benefits are, and appeal a denial if necessary.
- Work with your doctor to provide information to the insurance company that explains your diagnosis, tests results, treatment decisions, and why a specific drug is needed.
- Provide copay cards or coupons to reduce the cost of the drug.

The *Patient Advocate Foundation* provides guidance about drug manufacturer patient assistance programs and connects patients who qualify for free medications or medications at minimal cost.

### Clinical Trial Participation

Clinical research in rare diseases is expanding, not least because patients, caregivers, and patient advocacy organizations have begun to demand treatments and management strategies that reflect the needs and goals of rare disease communities.<sup>8</sup> The *Center for Information and Study on Clinical Research Participation* provides answers to frequently asked questions about what clinical trial participation entails. Learn more about the possible benefits and risks of clinical trial participation from from this Global Genes resource, *Cell and Gene Therapy for Rare Disease Clinical Trials FAQ*.

**Interventional clinical trials** test the risks and benefits of drugs that are not approved by the FDA or the European Medicines Agency. Participation in an interventional clinical trial might offer access to drugs that are under investigation. Clinical trials also study the natural history of diseases to build up a picture of how a condition develops or how patients are affected over time. **Natural history studies** provide information that can be used to design clinical trials that investigate therapies. Learn more from Global Genes about clinical trial participation [here](#).

- You can search for open clinical trials focused on a treatment for your rare disease at *Clinicaltrials.gov* or the *WHO International Clinical Trials Registry Platform*.
- The following organizations provide education and resources to support patients with rare diseases considering clinical trial participation:
  - » *Center for Information and Study on Clinical Research Participation*
  - » *National Institutes of Health Genetic and Rare Disease Information Center*
  - » *National Institutes of Health Rare Diseases Clinical Research Network*
- **Patient registries** are also tools for research. Registries are typically online databases that collect, store, retrieve, analyze, and share information on children and adults with a diagnosed rare disorder. The Marfan Foundation and Backpack Health created a patient registry to improve access to real-world, de-identified, pooled data about Marfan's syndrome that also supports patients with digital health tools they can use to manage health records and receive customized information to support care. Find out whether there is a patient registry for your or your family member's rare disease. Learn more from Global Genes about patient registries [here](#).



## CAREGIVERS AND MENTAL HEALTH

Caregivers—especially mothers, who are the dominant caregivers for most patients with rare diseases—are often working part- or full-time caregiving and can be exposed to a variety of psychological stressors at different points in the caregiving journey, including the loss of friendships or juggling caregiving with other family relationships. Financial stress or distress is one of several stressors in the lives of rare disease caregivers that can contribute to chronic psychological stress.<sup>14</sup> See **Pro Tip 4** for tips on caregiver self-care. A range of topics and helpful tips are presented in The National Alliance for Caregiving resource *The Circle of Care Guidebook for Caregivers of Children With Rare and/or Serious Illnesses*.

### PRO TIP

“With a stressful event, your stress levels peak and then recover. With caregiving stress, you peak and stay within a narrow range of stress for longer. You can handle the first, fifth or tenth denial by the insurance company, but when you get to the fifteenth or twentieth denial then everything becomes a crisis for our caregiving community. That’s what you call chronic stress because you are anticipating and ruminating on this experience of denial.”

– CRISTOL BARRETT O’LOUGHLIN  
CEO, ANGEL AID CARES

### Support Your Mental Health

Research suggests that sadness, depression, and anxiety are common experiences among people in the rare disease community.<sup>9-12</sup> The largest study of adults with rare disorders and mental health in the United States reported in 2020 that people with rare disease are more likely to suffer depression and anxiety than most of the general population.<sup>9</sup>

The stressful and unpredictable complexity of rare disease, inadequate access to a social and emotional support network, the day-to-day realities of care—multiple clinic visits, mobility issues, dietary restrictions, difficulties with sleep, fatigue, pain, and other factors—places enormous strain on emotional and mental health. Certain rare diseases are also associated with neuropsychiatric symptoms.<sup>13</sup>

Mental health is as important as physical health. Although there is very little research on the links between mental health and rare disease, research and policy are beginning to grow. A 2019 *EURODIS* position paper on holistic, person-centered care in rare disease recommends an annual mental health assessment to ensure monitoring and interventions to avoid depression and other mental health issues. The *National Alliance on Mental Illness* provides mental health education and access to resources, such as support groups and information about evidence-based psychological therapies that have been shown to reduce depression and anxiety among people with chronic conditions, including *Cognitive Behavioral Therapy* and *Acceptance and Commitment Therapy*.



Steps to support mental health include:

- Cultivate leisure and social activities that bring pleasure and increase contact with friends and relatives.
- Seek respite care from peers, your community, or rare disease organizations.
  - » **Get connected.** *Global Genes* is an online community that connects individuals affected by rare diseases from all over the world.
  - » The *National Alliance for Caregiving* provides additional resources for caregivers to support respite and other needs.
- Ask your provider for information about available *psychological support*, including mental health evaluation.
- Although techniques to support mental health are highly personal, a recent *Rare Disease UK* study identified the following coping techniques as having positive and protective impact on mental health:
  - » Relaxation or mindfulness practices
  - » Crafts
  - » Writing/journaling
  - » Physical activity

## Mental Health Emergencies

If you or someone you care for experiences a mental health crisis, the following organizations can help:

- *Disaster Distress Helpline*: The Substance Abuse and Mental Health Services Administration provides 24/7 crisis counseling 365 days of the year.
- *Crisis Textline*: Crisis counselors available via text in the United States, Canada, the United Kingdom, and Ireland.
- *National Suicide Prevention Lifeline*
- *Angel Aid Cares*: This U.S.-based organization offers up to 6 hours of free mental health support with licensed therapists via its Give an Hour program to help mothers address a crisis—such as financial distress—for which she has no other sources of mental health support.



Often, caregivers put their own physical, social, emotional, and financial health on the back burner to support their rare disease loved one. It is important for rare parents to find and get support for their own health and well-being to carry on caregiving.

Support comes in many forms, and includes:<sup>15</sup>

- Information support, e.g., education about the disease and referrals for medical care
- Tangible support, e.g., childcare,

respite care, or help with daily living tasks

- Emotional support, e.g., feeling validated and understood by others
- Companionship support, e.g., engaging in activities with others, resulting in a sense of belonging



## PRO TIP 4: CAREGIVER BALANCE AND SELF-CARE



Cristol Barrett O'Loughlin recommends the following series of steps to help you identify the support you need to continue caregiving—including the financial side of caregiving—in a sustainable way.

- Be forgiving of yourself. Acknowledge that caregiving is hard for everyone.
- Give yourself permission to seek support from others.
- Build your well-being team so that you can practice self-care.
- Team members could include a friend, neighbor, or someone from church to provide respite care and allow you to sit down for a couple of hours to pay bills, get into financial balance, or create space for self-care practices (e.g., physical activity, journaling, yoga).
- Financial experts, financial navigators, or health insurance financial advocates could also be members of your well-being team.
- Health insurance companies provide access to financial advocates in other mature disease states (e.g., breast cancer) to help navigate health insurance. Ask for the same service.
- Describe the impact of caring on your physical and mental health.
- Help the insurer understand that they need to support you so that you can continue to care for your child.

## Telehealth

Telehealth is a broad term that includes phones, computers, and other digital devices and platforms to facilitate healthcare appointments and remotely monitor patients without healthcare providers and patients being in the same physical space. Some insurance companies cover telephone access to in-network providers or contract with

telehealth providers with very low copays (e.g., *LemonAid*). Many states mandate that Medicaid and private insurers provide coverage for telehealth to the same extent as coverage for in-person /local care. However, states vary in how they define, reimburse, or regulate telehealth. Telehealth might also be an option if you need to see a specialist who is geographically distant

## Opportunities for Using Telehealth Expanded in 2020

In 2020, many rare disease patients used telehealth services to access care. In response to the emergence of COVID-19 in 2020 the Centers for Medicare and Medicaid relaxed some of the previous regulatory requirements for using telehealth, and many commercial insurers expanded access to telehealth services.

According to a report by the *National Organization for Rare Disorders* (NORD) telehealth visits accounted for 69 percent of total health visits across the United States in 2020. A pilot program launched in 2019 by *Children's National Hospital* in Washington, DC reports that telehealth can help to strengthen communication across the healthcare team, streamline patient visits, and increase access to specialist expertise in rare disease. NORD and 34 other rare disease organizations are currently exploring ways to ensure that telehealth and remote monitoring technologies can continue to offer access to care in rare disease, including specialized care and opportunities to participate in clinical trials and gain access to investigational drugs.

Telehealth can be used for the following types of consultations:

**Telegenetic Counseling.** Diagnosis and remote delivery of genetic counseling.

**Telepsychiatry.** On-demand access to mental health specialty care (e.g., via *Ginger*).

**Urgent/Emergency Room Care.** Many health systems offer telehealth consults for urgent or emergency health care needs.

# Health Insurance Literacy Matters

Health insurance is complex and many people in the United States struggle to fully understand basic health insurance terms. Health insurance companies recognize this complexity. Many have launched programs to provide plain language summaries of health insurance benefits. America's Health Insurance Plans offers a resource to answer frequently asked questions about health insurance at [MyHealthPlan.guide](#).

**Health insurance.** Health insurance is designed to help pay for health care, including providers, medications, preventive screenings, and other medical services. As the policyholder, you pay a premium and the insurance company pays for some or all the costs of your care. There are many different sources of health insurance, including through government (public) or commercial plans (private).

- Search for information about how to buy health insurance and coverage in your state at: <https://www.healthinsurance.org/>
- NORD's state insurance map provides state-specific resources related to health insurance.

## **Employer-based health insurance.**

Employer-based insurance plans are available for full-time employees, and, in some cases, part-time employees. Companies that provide plans for employer-based insurance include Blue Cross Blue Shield, Cigna, Aetna, and United HealthCare. The cost of plans varies with the premium automatically deducted from employee paychecks. Because employer-based plans may change year-to-year you might also have to change providers.

**Medicaid.** Medicaid is a joint federal and state health insurance program that helps people with low incomes, people with disabilities, and people who are receiving Supplemental Security Income (SSI) pay for health care. In most states, if you are already receiving SSI you qualify for Medicaid and are automatically enrolled when SSI is awarded. Learn more about Medicaid eligibility and enrollment at the [Social Security Administration](#).

**Medicare.** Medicare is a federal health insurance program for people who are 65 years or older, certain people under 65 years with disabilities who receive

Social Security benefits after a two-year waiting period or have certain diseases (i.e., amyotrophic lateral sclerosis and end-stage renal disease). In some states (e.g., Massachusetts), both Medicare and Medicaid are available as dual coverage.

**Healthcare Insurance Marketplace or Exchange.** Health insurance plans are available for people ineligible for Medicaid or without employer-based insurance via online exchanges that are run either by individual states or the federal government (*health insurance marketplace*). Open Enrollment usually

occurs toward the end of each calendar year.

**Variation in plan coverage.** Health insurance plans vary in terms of what they cover, the deductible that you pay before the plan will start paying for care, and the level of copay. The coverage in health insurance plans can also vary from state to state. Health insurance brokers are knowledgeable about available plans and can help you compare plans.



## PRO TIP 5: COVERING GAPS IN CARE: LONG-TERM DISABILITY INSURANCE

Long-term disability (LTD) insurance is a vital resource for people living with rare diseases. If you anticipate having an inherited disorder but have not yet been diagnosed, consider acquiring **life insurance** or employer-based or private short- or **long-term disability insurance** prior to diagnostic testing.

LTD provides a proportion of wage replacement, approximately 50 to 70 percent of salary, if you are unable to work for an extended period. LTD also provides coverage after *short-term disability insurance* ends.

The extent of benefit varies by policy. Learn more about LTD from the *Patient Advocate Foundation*.



Doug Strott of the Scheuermann's Disease Fund emphasizes that it is important to plan for the possibility of not being able to work, especially if you are living with a rare disease that is progressive or degenerative.

– DOUG STROTT , FOUNDER, THE SCHEUERMANN'S DISEASE FUND

- Talk with your employer's Human Resources to determine the cost of and access to LTD insurance.
- Consider taking a secondary private LTD policy to complement employer-based LTD. Insurance brokers are a good source of information about private LTD policies.
- If your insurance company terminates your LTD, consult with lawyers that specialize in ERISA (Employment Retirement Income Security Act) law.



### PRO TIP 6: KEEPING TRACK OF INSURANCE COVERAGE

**Check that your health insurance covers your/your family member's rare disease** as well as the following:

- Specialist care, physical therapy, long-term therapy
- Regular medications before meeting the deductible
- Referral from a primary care provider to see a specialist
- Preauthorization before treatment
- Step therapy before using certain medications
- Medical equipment
- Out-of-network providers
- Co-pays
- Preventive care, reproductive health, and mental health

Find a full checklist here to help prioritize the benefits you need in your health insurance plan: [RARE Tookits™: Navigating Health Insurance](#).

**Keep your insurance information where it is easy for you to find** and, if necessary, share with a designated family member or friend if you become incapacitated. [The Global Genes RARE Tookits™: Navigating Health Insurance](#) can help you keep your insurance profile and medical information together in one place.

**Create a healthcare proxy or power of attorney.** Identify someone you trust to be your healthcare proxy in case there are times when you are unable to be the decision-maker about your health. Designating two people to act in your place minimizes potential conflict of interest. If you want to allow providers to share your medical information with a family member or someone you trust, ask for a medical release form.

**Create a living will or advance directive.** This written document allows patients to give explicit instructions about medical treatment should they become terminally ill or permanently unconscious.

If you find it difficult to talk about money or feel financially stressed, you are not alone. Money is a source of stress for most people in the United States. Almost 40 percent of adults find it difficult to meet their monthly expenses or do not have enough savings to cover unexpected expenses.<sup>16</sup> Many people say they would need to borrow or sell something to pay for unexpected expenses or would be unable to cover the expense at all.<sup>16</sup>

### Build a Financial Knowledge Bank

**Financial toxicity** is a term used in cancer care to highlight the burden many patients face in paying for care. In rare disease, medical bills and costs of treatment can rapidly mount up and lead to financial toxicity, which can significantly impact health. For instance, studies in rare disease and in cancer show that between 53 percent to 80 percent of adults use savings to pay for medical expenses.<sup>10</sup> But not everyone has savings.

### Steps to Build a Financial Knowledge Bank

Building a financial knowledge bank is a first step toward taking control over your finances and improving your ability to meet daily living needs and pay for healthcare now and in the future. Taking control of your finances can also help to reduce the chronic stress and psychological strain that comes from healthcare-related financial hardship.

- **Build knowledge about money matters.** According to the U.S. Treasury's Financial Literacy and

Education Commission, there are six parts to financial literacy: earn, spend, save, invest, borrow, and protect. Learn more about financial terms from the *Consumer Financial Protection Bureau*.

## Money Matters<sup>17</sup>

In 2018, **137.1 million** adults reported financial distress due to medical bills in the prior year.

**20% of adults** had major, unexpected medical bills to pay in 2019 (median expense \$1,000-\$1,999).

**18% are left with unpaid debt** from unexpected medical bills.

**25% of adults** in 2019 skipped necessary medical care because they were unable to afford the cost of care.

- **Review your financial habits:** The *Federal Deposit Insurance Corporation* offers tools and resources to help adults become confident about managing finances and building savings to create a buffer against unanticipated care expenses. Some states offer Individual Development Account (IDA) programs. IDAs are savings accounts that match the deposits of low- and moderate-income

savers if they participate in financial education and use the savings for targeted purposes such as post-secondary education or homeownership. Learn more about IDAs [here](#).

- **Create a healthcare needs budget.** Review your healthcare needs statements for the past few months or years and analyze your average monthly costs associated with those plans for the following items:
  - » Annual visits with your primary care provider and specialists
  - » Prescription drugs that you take regularly
  - » Medical supplies (e.g., injections, equipment, supplies, procedures)
  - » Use the [Health Insurance Cost Profile](#) tool to track your health insurance expenses

- **Take a financial literacy course or speak with a financial counselor.**
  - » Several organizations, like [Fair Credit](#), provide free financial management classes on topics such as budgeting, credit awareness, and debt management.
  - » [Money Smart](#) and [MyMoney](#) are government-sponsored financial education programs designed to support and provide resources to build financial literacy. The Office of the Comptroller of the Currency has also created a [Financial Literacy Resource Directory](#).
  - » Some organizations provide web- or phone-based information and assistance. For instance, [www.usaa.com](http://www.usaa.com) includes a toll-free number for access to financial advisors who will respond to questions about overlapping financial and health issues.



# Global Genes Financial Advocacy RARE Patient Impact Program

Learn about how organizations dedicated to financial wellbeing and advocacy are helping patients, caregivers and families.

## **The Littlest Tumor Foundation**

The Littlest Tumor Foundation conducted a focus group and research supported by the Global Genes RARE Patient Impact Program. The Littlest Tumor Foundation plans to expand its financial literacy resources for young adults with neurofibromatosis and improve access to resources that help improve overall wellbeing.

## **Mission:Cure**

The team at Mission:Cure conducted a survey to better understand the financial challenges and barriers that patients face when seeking care for chronic and recurrent acute pancreatitis. The survey findings help understand the financial toll of navigating the medical system in search of a diagnosis and/or a treatment plan while suffering with pain, hopelessness, and poor outcomes. Mission:Cure is dedicated to designing for hope and resilience to provide holistic and compassionate care.

## **Illinois Spina Bifida Association**

The Illinois Spina Bifida Association provided financial literacy and advocacy resources from the National Disability Institute and ABLE National Resource Center as part of 1:1 training for patients. The organization also provides resources on financial resilience, financial planning, and also offers financial assistance and scholarships. A survey conducted in 2020 highlights insurance government assistance gaps for Spina Bifida Families.

## **National Gaucher Foundation**

The National Gaucher Foundation (NGF) promotes financial preparation on its blog, highlighting key money milestones for a healthy financial future in 7 Financial Tips for People with a Chronic Disease. NGF provides valuable resources to improve financial literacy of people with Gaucher disease. The organization has also created the National Gaucher CARE Foundation (NGCF) to help relieve the financial burden on people who have Gaucher disease by offering assistance with insurance premiums or out-of-pocket expenses.

## **United Mitochondrial Disease Foundation**

The United Mitochondrial Disease Foundation (UMDF) created a comprehensive booklet addressing the financial challenges confronted by people affected by Leber Hereditary Optic Neuropathy (LHON), a condition causing blindness. A Guide to Navigating LHON with Money in Mind is a regional guide to help the California LHON community become familiar with resources that can limit negative financial impacts. The UMDF is working on expanding this resource for additional regions.

## 2021 Awardees

- The E.WE Foundation
- The Akari Foundation
- ALD Connect
- The DDX3X Foundation
- RareKC Foundation
- United Mitochondrial Disease Foundation
- Angelman Syndrome Foundation
- Dup15q Alliance

Learn more about Global Genes Rare Patient Impact Grant here.

# Financial and Health Insurance Literacy Resources

The National Institutes of Health provides tips for finding financial aid in rare disease. In addition to the organizations listed in Table 2, search the National Financial Resource Directory for organizations that might have a financial program to address your needs.

## Health Insurance and Financial Literacy Resources

Government Websites
Agency for Healthcare Research and Quality
Centers for Disease Control and Prevention
Department of Health and Human Services
HealthCare.gov
Healthy People 2020
Information and Insurance Oversight
National Institutes of Health
Senate Health, Education, Labor and Pensions Committee

Commercial and Nonprofit Organizations
AHIP Coverage
America’s Health Insurance Plans
American Enterprise Institute
American Institutes for Research
Center for Patient and Consumer Engagement
Consumers Union
Enroll America
Institute of Medicine
Kaiser Family Foundation
National Association of Insurance Commissioners
National Health Information Center
Robert Wood Johnson Foundation
The George Washington University, Milken Institute School of Public Health
The Urban Institute

## General Organizations that Provide Financial Assistance Resources

ORGANIZATION	PROGRAM FOCUS
<b>Air Charity Network (Angel Flight)</b>	Provides access for people in need who are seeking free air transportation to specialized health care facilities or distant destinations due to family, community, or national crisis.
<b>Angel Flight East</b>	AFE serves the Northeastern region of the United States and links with other volunteer pilot organizations to provide air transportation via a volunteer pilot network.
<b>Children’s Flight of Hope</b>	Provides air transportation (commercial and private) for children and a caregiver to travel for clinical trials, specialist visits.
<b>Chive Charities</b>	Provides financial support for therapy equipment and sessions not covered by insurance, mobility items, developmental and learning devices, and other medical needs as recommended by a medical professional.
<b>Families USA</b>	Works to promote high-quality, affordable health care for all Americans.
<b>Global Genes</b>	Global Genes is a global non-profit advocacy organization for individuals and families fighting rare and genetic diseases.
<b>Little Zebra Fund</b>	Physician-led non-profit organization that provides financial support to patients for clinically indicated genetic testing.
<b>Living in the Light</b>	Patient advocacy initiative using photography and narrative to educate the biotech and medical communities about living with rare diseases. In 2020 it launched a campaign and funds to support those staying home to protect the lives of children, family members, and friends living with rare disease during the COVID-19 pandemic. <i>Stay Home for Rare™</i>
<b>Miracle Flights</b>	Provides commercial plane tickets to all U.S.-based medical treatment facilities at no cost to families.

<b>NORD Patient Assistance Program (RareCare)</b>	<p>Assistance programs to help patients obtain lifesaving or life-sustaining medication they could not otherwise afford. The 57 programs provide medication, financial assistance with insurance premiums and co-pays, diagnostic testing assistance, and travel assistance for clinical trials or consultation with disease specialists.</p>
<b>Partners for Prescription Assistance (PPA)</b>	<p>Helps to connect qualifying patients with an appropriate assistance program.</p>
<b>PSI Patient Assistance Program (Co-Pay)</b>	<p>Patient Services, Inc. (PSI) provides financial support and guidance for qualified patients with specific, rare chronic diseases. The PSI team is passionate about accessibility and affordability to treatment. PSI also offers a variety of legal services free of charge through the PSI-A.C.C.E.S.S. Program for specific rare disease communities. Through PSI assistance programs patients and their families rediscover hope and health.</p>
<b>Rare and Undiagnosed Network (RUN)</b>	<p>Empowers rare and undiagnosed patients and their families with genomic information and community through advocacy, networking, and support.</p>
<b>The Assistance Fund (TAF)</b>	<p>Independent charitable patient assistance foundation that helps patients and families facing high medical out-of-pocket costs by providing financial assistance for their copayments, coinsurance, deductibles, and other health-related expenses.</p>
<b>The Patient Advocate Foundation</b>	<p>A national 501 (c)(3) non-profit charity that provides direct services to patients with chronic, life threatening and debilitating diseases to help access care and treatment recommended by their doctor. PAF provides case management, co-pay relief, financial aid funds, scholarships, and educational resources.</p> <p><i><a href="#">Insurance Denials &amp; Appeals</a></i>  <i><a href="#">National Financial Resource Directory</a></i></p>

# Glossary

---

**Advance Directive:** A written document that allows patients to give explicit instructions about medical treatment should they become terminally ill or permanently unconscious.

**Claim:** A request for payment that you or your healthcare provider submits to your health insurer after you receive medical services and/or other items like medical equipment.

**COBRA:** Consolidated Omnibus Budget Reconciliation Act (COBRA) is a law that allows you to take your group health insurance policy when leaving employment. Under COBRA, you are responsible for the full cost of premiums, as well as administrative fees.

**Coinsurance:** An amount you pay for services even after the deductible is paid. Coinsurance may be a percentage amount. For instance, you pay 20 percent of the cost of services or drugs and your employer pays 80 percent.

**Compassionate Use Programs:** Pathways to drugs that are still in clinical development and not yet

approved by the U.S. Food and Drug Administration or the European Medicines Agency. Also called Expanded Access Programs.

**Coordination of benefits:** A way to determine which health plan is responsible for paying a claim when your healthcare is covered by more than one policy.

**Copay:** A fixed amount you pay for a specific service (e.g., visit to the doctor's office), procedure, or drug.

**Cost-sharing:** The amount you are responsible for paying out-of-pocket under your plan for covered services, excluding the cost of premiums and non-covered services.

**Deductible:** The amount you pay out-of-pocket toward health costs before your insurance begins to cover costs. A policy may have different deductibles for certain aspects of the plan, such as prescription drugs.

**Denial:** The decision by a health insurer to refuse payment for a specific service, treatment, drug, and certain medical equipment/supplies.

**Disease Self-Management:** Disease self-management is a strategy to support people living with chronic disease through education and training in problem-solving skills.

**Exclusions:** Conditions or treatments that a health insurance plan will not cover.

**Expanded Access Programs:** Pathways to drugs that are still in clinical development and not yet approved by the U.S. Food and Drug Administration or the European Medicines Agency. Also called Compassionate Use Programs.

**Financial Assistance Program:** Refers to a range of government and non-profit programs that provide financial assistance to help pay for health care, including prescription assistance.

**Financial Distress:** A situation in which a person has insufficient funds to pay for basic needs, including health care.

**Financial Toxicity:** A term used in cancer care to highlight the burden many patients face in paying for care. In rare disease, medical bills and costs of treatment can rapidly mount up and lead to financial toxicity, which can significantly impact health.

**Financial Literacy:** Involves the ability to assess, understand, and use financial information in ways that support good health and financial outcomes.

**Fixed Expenses:** Costs that stay roughly the same each month and are paid on a regular basis, such as weekly or monthly. Rent or mortgage, car payments, and insurance premiums are examples of fixed expenses.

**Financial Navigators:** Trained to provide guidance and direction toward resources to reduce the costs of care and medications.

**Health Insurance Literacy:** Involves building knowledge and skills to find and evaluate information about health plans, select the best plan given financial and health circumstances, and use the plan once enrolled.

**Health Insurance Plan:** A policy document that describes the coverage you buy with a premium to help pay for health care, including providers, medications, preventive screenings, and other medical services.

**Health savings account (HSA):** An account you can use to save money toward health costs and expenses that your insurance does not cover. If you do not use all the money in a calendar year it rolls over to the next year.

## **International Classification of**

**Diseases Codes:** Health care services in the United States are reimbursed on the basis of International Classification of Diseases, 9th Revision (ICD-9) or ICD-10 codes. Of the approximately 5,500 – 9,000 rare diseases, only 500 have a specific ICD diagnosis code. When there is no specific code, providers use codes that describe symptoms but not the underlying disease. This means that patients and providers often have to make the case for each care step, including not only therapies, but also equipment or procedures, and often have to pay more out-of-pocket for these services.

## **Health Maintenance Organization**

**(HMO):** Provides health services through a network of doctors, hospitals, clinics, and laboratories that are either employed by or contracted with the HMO. These plans generally will not cover out-of-network care except in an emergency. HMOs often provide integrated care and focus on prevention and wellness. Patients are required to have a designated primary care physician, who will direct you to specialists as needed. If you use doctors or hospitals outside of the network, you may have to pay the full cost of these services.

**In-network providers:** Doctors and other providers that are included in your health insurance plan and that provide services for discounted rates that lower any costs you might have to pay.

**Interventional Clinical Trial:** Tests the risks and benefits of drugs that are not approved by the U.S. Food and Drug Administration or the European Medicines Agency. Participation in an interventional clinical trial might offer access to drugs that are under investigation.

**Life Insurance:** Provides financial support to someone after you die (a beneficiary). You can add riders to your policy to help you access the benefits of the policy while you are living.

**Living Will:** A written document that allows patients to give explicit instructions about medical treatment should they become terminally ill or permanently unconscious.

**Long-Term Disability Insurance:** Employer-based or private LTD policies are options to consider if you are employed and anticipate having a rare disease but have not yet been diagnosed. LTD provides a proportion of wage replacement. The extent of benefit varies by policy.

**Major medical:** Comprehensive and long-term coverage that usually includes doctor's office visits, hospitalization, medical supplies and services, prescription drugs, and other healthcare expenses.

**Natural History Studies:** Studies to acquire information that can be used to design clinical trials that investigate therapies.

**Out-of-network providers:** Doctors and other providers that are not included in your health insurance plan. You usually pay out-of-pocket to see these providers.

**Out-of-pocket maximum:** the total amount of money your plan expects you to pay in a calendar year for health care.

**Patient portal:** A secure online website that provides 24-hour access to your personal health information, including doctor visits, discharge summaries, and lab results. Some patient portals allow you to message your providers and request prescription refills.

**Patient Registry:** Registries are databases that collect, store, retrieve, analyze, and share real-world, de-identified information on children and adults with a diagnosed rare disorder.

**Pharmacy benefit:** The prescription drugs a health insurance plan covers under its drug benefits policies. This list may be called the medical benefit formulary list or the drug list.

**Preauthorization:** Process in which your health insurer decides whether a treatment plan, procedure, or prescription drug is medically necessary.

**Point of Service:** A plan that provides greater flexibility for patients to use doctors outside of its network. Under a POS plan your primary care physician can refer you to a doctor in or out of the network, however, using doctors in the network cost less than using doctors outside the network.

**Preferred Provider Organization (PPO):** A group of providers that sell their services by contract to health insurance companies. PPOs allow greater flexibility to use providers outside of the plan's network. Using doctors and hospitals in the network cost less than using those outside of it, but unlike a Point-of-Service plan, you do not need a referral from your primary care physician to use the services of a specialist.

**Premium:** The monthly fee you pay for coverage. Policies with lower premiums often require you to pay higher deductibles and copayments for medical services. A policy with a low premium does not mean it is the least expensive.

**Prescription Assistance:** Services that provide financial support and guidance for qualified patients with specific, rare chronic diseases if you do not have health insurance or are without prescription drug coverage access to medicines.

**Price Comparison Tools:** Web-based tools that allow consumers to find a range of prices for procedures or health care interventions that you need. Price comparison tools allow you to search by town or zip code.

**Referral:** In many plans your primary care doctor must refer you to see a specialist.

**Restrictions to Coverage:** Health insurance companies place restrictions on certain services or drugs to limit their use. Restrictions might also be called formulary restrictions. Services or drugs that are covered by restrictions usually require prior authorization before use and step therapy.

**Shared decision-making:** Shared decision-making is a collaborative process in which providers and patients jointly make decisions about care after discussing available options, potential benefits and risks, and take into consideration the patient's values and preferences. In shared decision-making, patients are supported to consider options and make informed decisions about care. There are different ways to approach shared decision-making.

**Step therapy:** Requires you to try one or more medications that are approved for your diagnosis before moving or "stepping up" to a therapy that might not be approved but is effective for your diagnosis.

# References

---

1. National Center for Advancing Translational Sciences. Genetic and Rare Diseases Information center. FAQs About Rare Diseases. 2020. <https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases#:~:text=There%20may%20be%20as%20many,at%20between%2025%2D30%20million>. Accessed February 8, 2021.
2. MacLeod S, Musich S, Hawkins K, Armstrong DG. The growing need for resources to help older adults manage their financial and healthcare choices. *BMC Geriatr* 2017;17(1):84.
3. Parragh ZA ODMB. Health literacy and health insurance literacy: Do consumers know what they are buying? [http://www.allhealthpolicy.org/wp-content/uploads/2017/01/Health-Literacy-Toolkit\\_163.pdf](http://www.allhealthpolicy.org/wp-content/uploads/2017/01/Health-Literacy-Toolkit_163.pdf). 2017. Accessed February 8, 2021.
4. Kirzinger A HL, DiJulio B, Muñana C, Brodie M. Kaiser Health Tracking Poll – October 2017: Experiences of the Non-Group Marketplace Enrollees. <https://www.kff.org/health-reform/poll-finding/kaiser-health-tracking-poll-october-2017-experiences-of-the-non-group-marketplace-enrollees/>. Henry J Kaiser Family Foundation. 2017. Accessed February 8, 2021.
5. Rae M LL, Claxton G, Cox C, Long M, Damico A. Patient Cost-Sharing in Marketplace Plans, 2016. <https://www.kff.org/health-costs/issue-brief/patient-cost-sharing-in-marketplace-plans-2016/>. : Henry J Kaiser Family Foundation. 2016. Accessed February 8, 2021.
6. Angelis A, Kanavos P, López-Bastida J, et al. Social/economic costs and health-related quality of life in patients with epidermolysis bullosa in Europe. *Eur J Health Econ* 2016; 17 Suppl 1(Suppl 1): 31-42.
7. Elwyn G, Durand MA, Song J, et al. A three-talk model for shared decision making: multistage consultation process. *BMJ* 2017;359:j4891.
8. Morel T, Cano SJ. Measuring what matters to rare disease patients - reflections on the work by the IRDiRC taskforce on patient-centered outcome measures. *Orphanet J Rare is*. 2017; 12(1):171.
9. Bryson BA, Bogart KR. Social support, stress, and life satisfaction among adults with rare diseases. *Health Psychol* 2020; 39(10): 912-20.

10. Shire. Rare Disease Impact Report: Insights from patients and the medical community 2013.
11. EURODIS. Achieving Holistic Person-Centred Care to Leave No One Behind. [http://download2.eurordis.org/positionpapers/Position%20Paper%20Holistic%20Care%20for%20Rare%20Diseases\\_Final.pdf](http://download2.eurordis.org/positionpapers/Position%20Paper%20Holistic%20Care%20for%20Rare%20Diseases_Final.pdf), 2019. Accessed February 8, 2021.
12. UK Rare Disease. Living with a rare condition: the effect on mental health – Rare Disease UK. 2018. <https://www.raredisease.org.uk/wp-content/uploads/sites/7/2018/07/living-with-a-rare-condition-the-effect-on-mental-health-pdf.pdf>. Accessed February 8, 2021.
13. Bogart KR, Dermody SS. Relationship of rare disorder latent clusters to anxiety and depression symptoms. *Health Psychology* 2020;39(4):307-15.
14. Prather AA, Epel ES, Portela Parra E, et al. Associations between chronic caregiving stress and T cell markers implicated in immunosenescence. *Brain Behav Immun* 2018;73:546-9.
15. Bryson BA, Bogart KR. Social support, stress, and life satisfaction among adults with rare diseases. *Health Psychol* 2020;39(10):912-20.
16. Board of Governors of the Federal Reserve System. Report on the Economic Well-Being of U.S. Households in 2019, Featuring Supplemental Data from April 2020. Washington, D.C. Federal Reserve Board. 2020. Accessed February 8, 2021.
17. Yabroff KR, Zhao J, Han X, Zheng Z. Prevalence and Correlates of Medical Financial Hardship in the USA. *J Gen Intern Med* 2019;34(8):1494-502.

*Disclaimer: This content is for informational purposes only, you should not construe any such information or other material as legal, tax, investment, financial, or other advice. Nothing contained in our materials constitutes a solicitation, recommendation, endorsement, or offer by Global Genes.*

**Hope.** It's in our genes.



28 Argonaut, Suite 150  
Aliso Viejo, CA 92656

+1-949-248-RARE (7273)

**Follow us:**

[www.globalgenes.org](http://www.globalgenes.org)

[@globalgenes](https://twitter.com/globalgenes)



©Global Genes 2021. All rights reserved.