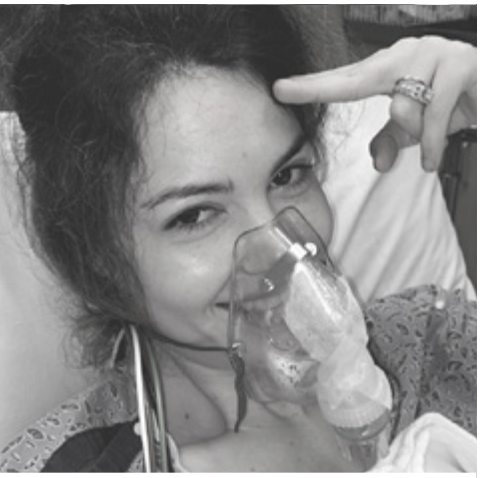
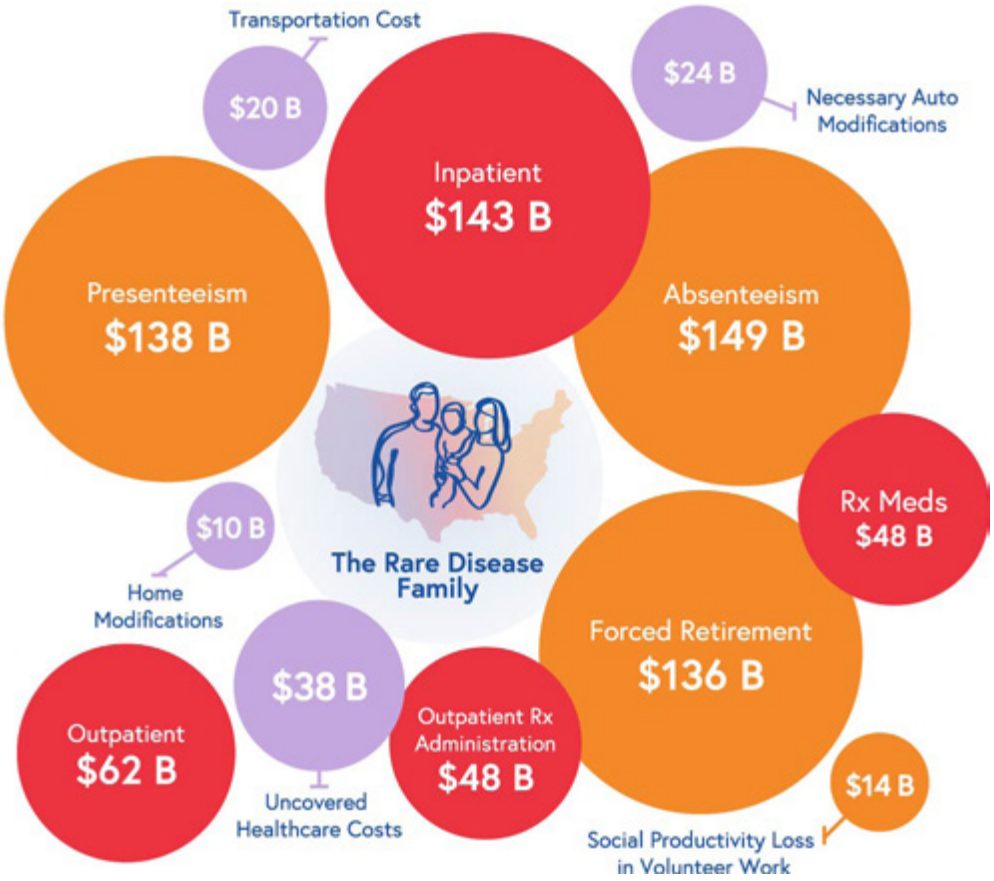


2021

ANNUAL REPORT

America's **Trillion Dollar** Healthcare Crisis



Read about the National Economic Burden of Rare Disease inside →

EVERYLIFE FOUNDATION FOR RARE DISEASES

2021 ANNUAL REPORT

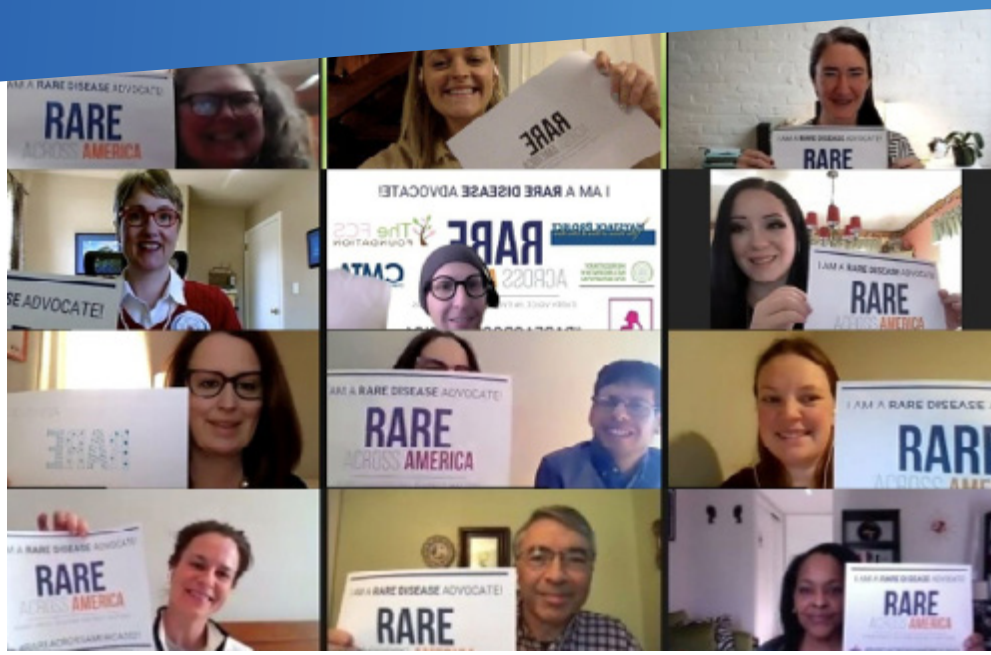


Table of Contents

EVERYLIFE FOUNDATION FOR RARE DISEASES

everylifefoundation.org

@EveryLifeOrg

WHEN THE 'P VALUE' STANDS FOR PATIENT:
DRIVING POLICY CENTERED ON EVIDENCE COLLECTED BY - AND FOR - THE RARE DISEASE COMMUNITY

Our 2021 annual report highlights major rare disease community milestones that have been years in the making and would not have happened without your input and support. The programs, events, and publications you will read about in this report were identified by the patient community for us to lead and we are grateful for the opportunity. Here are a few of our proudest moments that would not have been possible without you, our dedicated patient advocates.

- Thanks to your activism and advocacy on behalf of babies at risk for genetic conditions, lawmakers in Arizona, Georgia, North Carolina, and Ohio passed legislation requiring newborn screening for disorders on the Recommended Uniform Screening Panel (RUSP), bringing the total number of states that have passed RUSP alignment as of the end of 2021 to six.
- With input from 1,399 patient advocates who took the time to complete a complicated survey, we published a groundbreaking report, The National Economic Burden of Rare Disease in the United States. The study defines the trillion-dollar rare disease public health crisis and presents a clear economic argument for substantial investment in rare disease research and drug approval processes.
- At the request of our community, we published two new guides to give patient advocates the knowledge they need to navigate drug development systems, regulatory systems, and insurance reimbursement. Both The Guide to Patient Involvement in Rare Disease Therapy Development and the ICD Code Roadmap Resource Guide are available on our website.
- Thanks to the generosity of our partners in industry and major donors, we were able to provide $770,648 in sponsorships, grants, and scholarships directly to patient organizations and individual patient advocates.

We are grateful to each of you who came to us with ideas for tools, resources, and mission-forward policy. We so appreciate your trust and confidence in the EveryLife Foundation for Rare Diseases! We pledge to continue being accessible, responsive partners to our patient advocates, legislative champions, deeply committed advisors, and partners from across industry, government, and the nonprofit sector. We are ready to expand our programs in 2022 and look forward to continuing our transformational work together.

Julia Jenkins
Executive Director,
The EveryLife Foundation
for Rare Diseases

Mark Dant
Chairman of the Board of Directors,
The EveryLife Foundation
for Rare Diseases

EVERYLIFE FOUNDATION FOR RARE DISEASES TEAM

Operations

Julia Jenkins
Executive Director
jjenkins@everylifefoundation.org

Megan Pinegar
Chief Operating Officer
mpinegar@everylifefoundation.org

Stephanie Riordan
Associate Director of Patient Programs
sriordan@everylifefoundation.org

Alyssa Terwall
Director of Patient Programs and Events
aterwall@everylifefoundation.org

Lina Arslanian
Finance and Operations Manager
larslanian@everylifefoundation.org

Development

Ted Brasfield
Senior Director of Development
tbrasfield@everylifefoundation.org

Stephanie Siddiqi
Alliance Development Manager
ssiddiqi@everylifefoundation.org

Elissa Taylor
Associate Director of Alliance Development
etaylor@everylifefoundation.org

Policy, Advocacy, and Patient Engagement

Lindsey Cundiff
Associate Director of Patient Engagement
lcundiff@everylifefoundation.org

Claire Ellis
Newborn Screening & Diagnostics Policy Fellow
cellis@everylifefoundation.org

Courtney Felle
Patient Engagement Fellow
cfelle@everylifefoundation.org

Annie Kennedy
Chief of Policy, Advocacy, and Patient Engagement
akennedy@everylifefoundation.org

Katelyn Laws
RDLA Program Manager
klaws@everylifefoundation.org

Baillie McGowan
State Policy Manager
bmcgowan@everylifefoundation.org

Jack Meloro
Policy Programs Manager
jmeloro@everylifefoundation.org

Rachelle Raudes
State Advocacy Fellow
rraudes@everylifefoundation.org

Priscilla Rodriguez
Diversity Inclusion Advocacy Fellow
prodriguez@everylifefoundation.org

Dylan Simon
Director of Policy
dsimon@everylifefoundation.org

Jamie Sullivan
Senior Director of Policy
jsullivan@everylifefoundation.org

Shannon von Felden
Senior Director of Advocacy
svonfelden@everylifefoundation.org

Communications & Marketing

Brenda Colmenares
Associate Director of Communications
bcolmenares@everylifefoundation.org

Britta Dornan
Senior Director of Communications and Marketing
bdornan@everylifefoundation.org

Morgan Roth
Chief Communications Strategy Officer
mroth@everylifefoundation.org

DIRECT COMMUNITY SUPPORT $770,648

▲ $200,000 more than in 2020

Rare Giving
LIFTING EVERY VOICE

The EveryLife Foundation's Rare Giving program supports individual rare disease patients as well as organizations that engage patients, caregivers and others in the community. In 2021, the program expanded to offer support for policy engagement, tools and resources.

$450,190 in Grants and Awards

Provided to 80 + rare disease organizations

> "With this additional funding, we were able to strengthen the utilization of our community connections, which expanded access to additional resources and support for the VHL community. We provided training, education, and services to increase our advocacy initiatives and elevate the VHL community's voice and impact."
>
> **- Chandra C. Rare Giving Recipient**

#RAREis Scholarship Fund
Powered by the EveryLife Foundation

Thanks to the support of Horizon Therapeutics, the EveryLife Foundation established the #RAREis Scholarship Fund to enrich the lives of adults living with rare diseases by providing support for their educational pursuits.

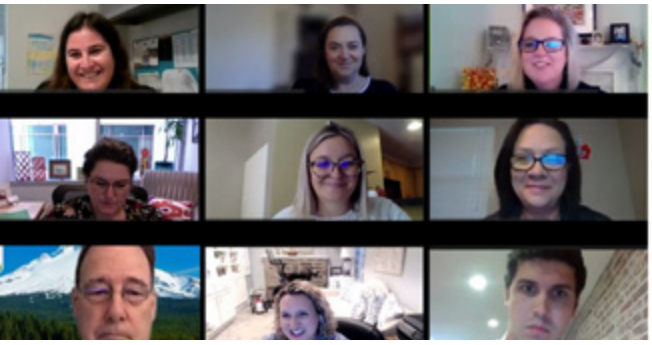
$295,000 in Scholarships

Provided to 59 rare disease patients, 28 states represented

> "The #Rareis Scholarship is helping to alleviate the financial stress of medical school and medical bills. I am able to dedicate more of my focus to growing the rare disease advocacy group I helped to start at my medical school this past year. I am so excited to help more rare disease patients share their stories and become a more empathetic and patient-focused physician in the process."
>
> **- Kimberly T. #RAREis Scholarship Recipient**

EMPOWERING THE PATIENT COMMUNITY

Rare Disease Legislative Advocates (RDLA) is a program of the EveryLife Foundation for Rare Diseases designed to support the advocacy of all rare disease patients and organizations. RDLA believes that every voice matters and that patients are the key to changing public policy.



900 + Advocates

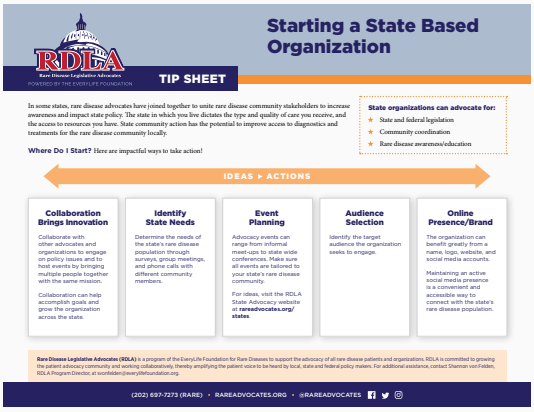
- ✓ Engaged during virtual RDLA Monthly Webinars.

186 Rare Disease Congressional Caucus Members

- ✓ 27 Senators and 159 Representatives.
- ✓ Hosted four briefings in 2021.

Diversity and Inclusion Fellowship

- ✓ Held first Diversity Roundtables event as part of Rare Disease Week 2021.
- ✓ Launched Rare Diversity Hub.
- ✓ Developed resources and tip sheets.



State Advocacy Initiative

- ✓ Worked with and supported rare disease state organizations and leaders.
- ✓ Developed resources and tip sheets.
- ✓ Hosted regional and state meet and greets.
- ✓ Launched the State Advocacy Hub.

RARE DISEASE WEEK ON CAPITOL HILL

2021 marked the 10th anniversary of Rare Disease Week on Capitol Hill. This year the event was transformed into a succesful virtual week resulting in:

704 Advocates

367 Meetings

250 Patient Organizations

RARE ACROSS AMERICA

During virtual Rare Across America, advocates from around the country spoke to their legislative officials and their staff on important rare disease issues. The advocates' meetings and hard work resulted in:

670 Advocates

373 Meetings

48 States + DC and PR

YARR

The YARR Leadership Academy, launched in 2021, is series of online classes offered to a select group of young adults in the rare disease community. Academy students learn about the roles and opportunities for patient representation in policy making, drug development, and the regulatory process and the steps it takes to enter those roles.

13 Graduates

"The YARR academy was an amazing experience for me as I got to learn about many different ways that I, as a patient, can have a seat at the table and use my perspective and experiences to make a difference in the rare disease community."

Shandra T.
Graduating Class of 2021

COLLABORATIVE POLICY

Since 2015, The Foundation's Community Congress has brought together patient organizations, industry leaders, and other rare disease stakeholders to provide advice and insight on urgent policy issues and Foundation programs and initiatives.

COMMUNITY CONGRESS
POWERED BY THE EVERYLIFE FOUNDATION

COMMUNITY CONGRESS MEMBERSHIP

Current Working Groups Include:
4 Permanent Working Groups + 7 Access Small Groups

"We know that the amazing work Community Congress is doing is a very important part of the process. I am so grateful to be here with fellow leaders, parents, caregivers, and providers."

Tuesdi Dyer
Executive Director,
CFC International

"I started participating with EveryLife's working groups when working on rare disease drug development issues. I immediately gravitated to the passion and commitment of patients and advocates I was able to meet."

Estevan Santana
Senior Manager (Policy),
State and Local Government Affairs, Genentech

NEW PORTAL FOR MEMBERS

This site is home for all Comunity Congress news and information. Aside from housing resources for members, the portal provides a platform for meaningful discussions.

SCAN FOR MORE INFO

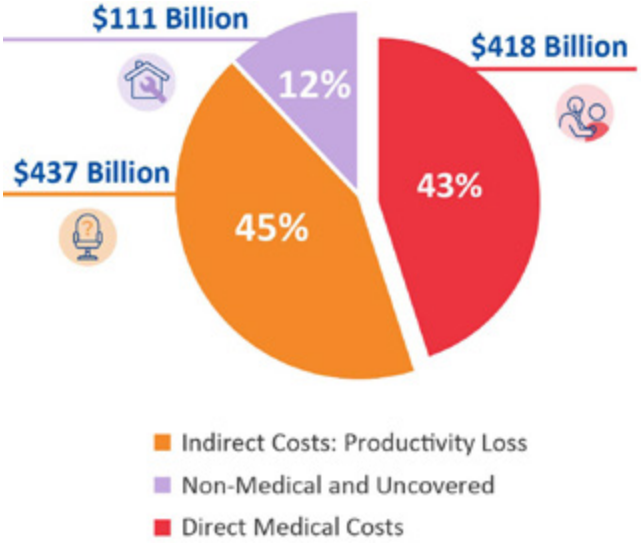
SHEDDING NEW LIGHT - FINANCIAL IMPACT OF RARE DISEASE

Public Policy Working Group

The National Economic Burden of Rare Disease Study

The National Economic Burden of Rare Disease Study is the first of its kind, providing the most comprehensive assessment of the total economic impact of rare diseases in a single year. The results help to ensure that the experience of the rare disease community is reflected accurately in policy discussions. This powerful tool can also increase public awareness of the public health crisis of rare diseases.

The Economic Burden of Rare Disease Reached Nearly $1Trillion in the U.S. in 2019.



"If a family cannot afford to repair an electric wheelchair, buy a hearing aid, fix teeth, or travel to specialists, then the person with a rare disease receives inadequate care. These costs accumulate and limit educational and career opportunities, making it harder to contribute to society."

Steve Smith
Father of a son with a rare disease

"The future of access in many ways depends on a more comprehensive, quantifiable understanding of how innovations in care improve both the health and economic well-being of the patients we serve. The data generated by this study helps define the economic impact of the diagnostic and treatment journey, validate the actual lived patient experience, and provide a rich and abundant dataset that can contribute to future conversations about the importance of prioritizing transformative therapies for people living with rare disease."

Bill Sibold
Executive Vice President,
Head, Specialty Care at Sanofi

Supportive Studies

We teamed up with federal partners, patients and academic researchers who also released novel data uncovering the true impact of rare diseases to amplify public awareness and drive action.

- Can you hear us now? The impact of health-care utilization by rare disease patients in the United States.
- Rare Diseases: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial.
- The IDeaS initiative: pilot study to assess the impact of rare diseases on patients and healthcare systems.

SCAN FOR MORE INFO

LIFESAVING LEGISLATION

Newborn Screening and Diagnostics Working Group

NEWBORN SCREENING

The Foundation has led efforts to pass legislation on the local level to help ensure that babies born in every state have the same opportunity for diagnosis and treatment.

FOUR STATES ADOPTED RUSP ALIGNMENT LEGISLATION IN 2021

Prior to 2021, only two states (California and Florida) had adopted RUSP alignment legislation. In 2021, four additional states – Arizona, Georgia North Carolina, and Ohio – were added to the roster. Lifesaving momentum is carrying on in 2022 in states including Iowa, Maryland, and Mississippi as advocates urge their lawmakers to keep their states on pace with science.

✓ ARIZONA

✓ GEORGIA

✓ NORTH CAROLINA

✓ OHIO

"I am so grateful to our state leaders for doing the right thing and passing this life-saving newborn screening legislation. Georgia parents deserve access to diagnosis and treatment for their babies at the earliest moment possible, so they don't waste years waiting for a diagnosis when every day counts. The science exists. We know how to save these babies' lives. We need our laws to keep up with the science."

Elizabeth Snarey. Parent of a child living with MPS II.

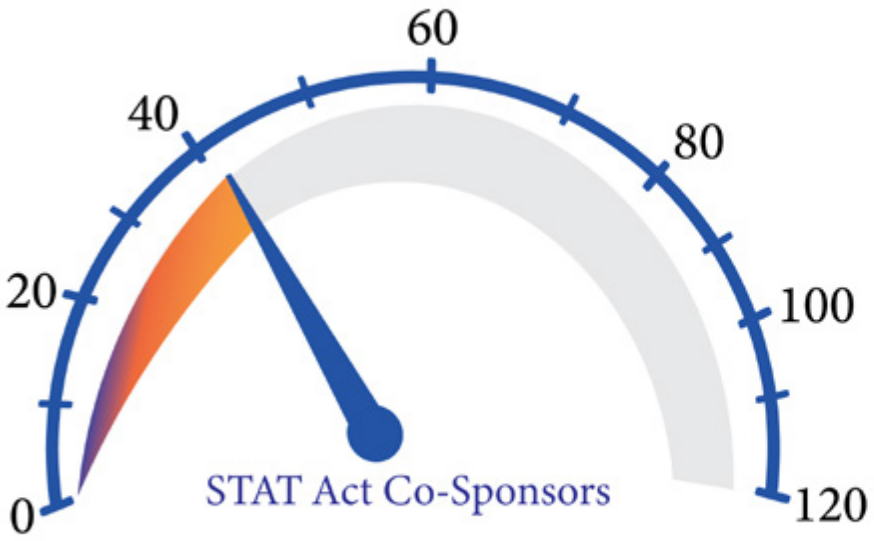
NEWBORN SCREENING MODERNIZATION STUDY

First study to delineate solutions to U.S. newborn screening system challenges and identify the need for modernization to keep pace with innovation.

SCAN FOR MORE INFO

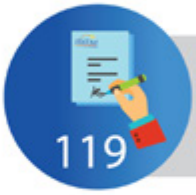
Public Policy and Regulatory Working Groups

The Speeding Therapy Access Today Act of 2021 H.R. 1730/S. 670 (STAT Act), introduced in March, is a bipartisan, bicameral, community-led bill created to improve the development of, and access to, therapies for the rare disease community across the spectrum of rare diseases.



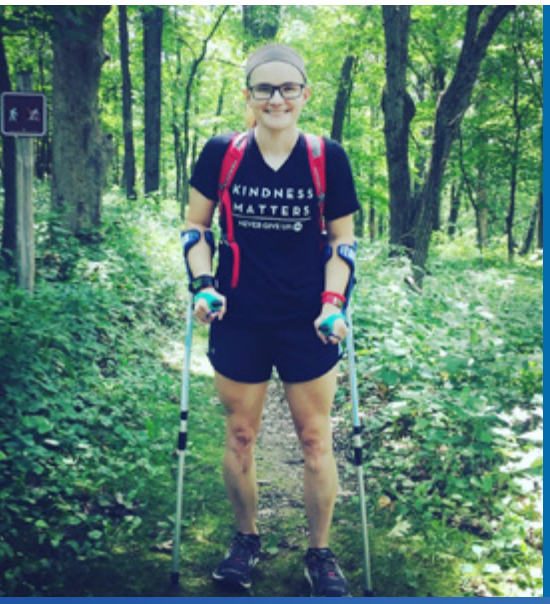
(as of May 2022)

Advocate meetings with Members of Congress held during Rare Across America and Rare Disease Week (as of May 2022).



Rare disease patient organizations joined the community sign-on letter sent to congressional committees (as of May 2022).

Advocate emails and phone calls to Members of Congress (as of May 2022).



"Patients like me need an FDA Rare Disease Center of Excellence to help our community achieve better access to therapy development and coordination of policies and stakeholders involved in rare.
I asked my members of congress to support the STAT Act, because it can change the landscape of rare disease for the better."

Abbey Hauser
Rare Disease Patient and EveryLife Board Member

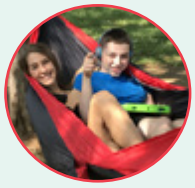
SCAN FOR MORE INFO

DECODING ICD CODES

Access Working Group

ICD CODE ROADMAP RESOURCE GUIDE

The **ICD Code Roadmap** is a first-of-its-kind resource designed to help patient advocacy leaders and their partners understand, evaluate their role, and navigate the process of refining the diagnostic coding system in the U.S.



"Upon learning that new codes for Dravet syndrome were going to become effective, we immediately went to work to spur awareness and adoption of the new codes. They won't benefit anyone if healthcare professionals didn't know they exist."

– Mary Anne Meskis, mother of a son with Dravet Syndrome

"The code specific to Castleman disease wouldn't exist if we hadn't lobbied for it. Someone had to turn hope into action to make this a reality. If you don't do it, oftentimes no one else will."

– David Fajgenbaum, M.D.

SCAN FOR MORE INFO

EMBOLDENED COMMUNITY ENGAGEMENT IN RARE DISEASE THERAPY DEVELOPMENT & REGULATORY PROCESSES

Regulatory and Access Working Groups

PFDD Guide

The **"Guide to Patient Involvement in Rare Disease Therapy Development"** is a resource for all stakeholders to utilize as we work to optimize our rare disease product development efforts.

This landmark tool was developed by nearly 100 community leaders during the PFDD Rare Disease Compendium Roundtables, a series of four virtual workshops that considered the application of FDA Guidances across four lifecycle stages of medical product development.

23 Leadership and Steering Committee Members

88 Subject Matter Experts

20 FDA Guidelines

12 Hours of Workshops

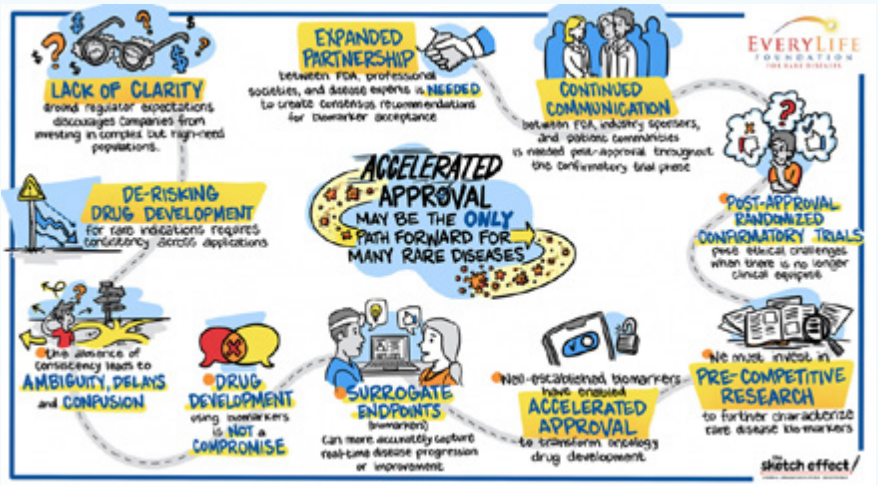
8 Cross-Cutting Topics and Sets of Action Steps

4 Workshop Summaries

112 Resources Linked in this Guide

EFFORTS TO STRENGTHEN AND PROTECT THE ACCELERATED APPROVAL PATHWAY

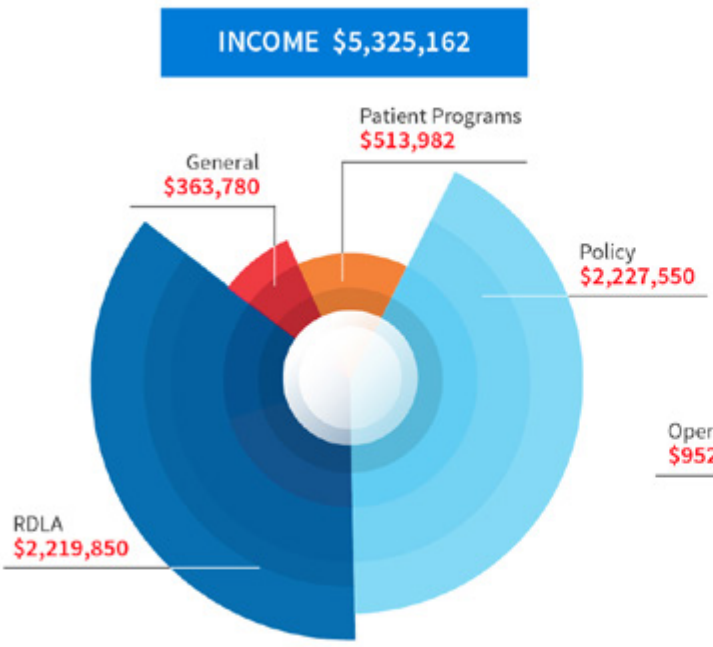
13th Annual EveryLife Foundation Scientific Workshop: Current and Future Barriers to the Utilization of Accelerated Approval Pathway for Novel Rare Disease Therapies.

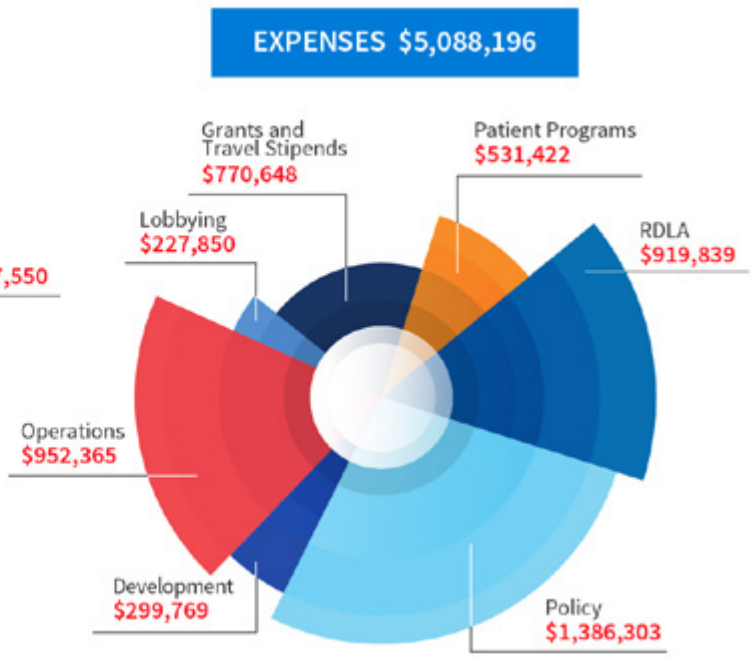


SCAN FOR MORE INFO

2021 INCOME AND EXPENSES

The Foundation receives funding from philanthropic organizations, government, individual donors, and corporations to support its activities. Sponsors are offered recognition for their support, but do not influence Foundation policy priorities or event program content.
To view our complete funding policy, visit: everylifefoundation.org/fundingpolicy





THANK YOU TO OUR 2021 SUPPORTERS

$200,000 and Over
- Horizon Therapeutics
- Sanofi Genzyme
- Travere Therapeutics

$100,000 - $199,000
- Alexion Pharmaceuticals
- argex
- Genentech
- Merck & Co., Inc.
- Novartis Gene Therapies
- Pfizer
- Sarepta Therapeutics
- Takeda Pharmaceutical

$55,000 - $99,000
- Acceleron Pharma
- Amicus Therapeutics
- Biogen
- BioMarin Pharmaceutical
- Biotechnology Innovation Org.
- Chan Zuckerberg Initiative
- Chiesi Global Rare Diseases
- Gilead Sciences
- GlaxoSmithKline
- Greenwich Biosciences
- Ipsen
- Janssen Pharmaceuticals
- Mallinckrodt Pharmaceuticals
- National PKU Alliance
- Neurocrine Biosciences
- PhRMA
- PTC Therapeutics
- Vertex Pharmaceuticals

$25,000- 50,000
- Alnylam Pharmaceuticals
- Astellas Gene Therapies
- Avidity Biosciences
- AVROBIO
- bluebird bio
- Blueprint Medicines
- BridgeBio
- Bristol-Myers Squibb Company
- Global Blood Therapeutics
- Harmony Biosciences
- Ionis Pharmaceuticals
- Mitsubishi Tanabe Pharma America
- Orchard Therapeutics
- Otsuka Pharmaceutical Co.
- Ovid Therapeutics
- Recordati Rare Diseases
- REGENXBIO, Inc.
- Sobi
- Taysha Gene Therapies
- UCB
- Ultragenyx Pharmaceutical
- William & Carolyn Aliski
- WCG Clinical

RARE HUB PARTNERS

- Adira Foundation
- Global Genes
- Little Hercules Foundation
- National Fragile X Foundation
- National MPS Society
- National PKU Alliance
- Parent Project Muscular Dystrophy
- Rare Access Action Project
- RARE-X
- Run for Rare
- Sick Cells
- SYNGAP1 Foundation
- Undiagnosed Diseases Network Foundation

$10, 000- $24,000

- American Society of Gene & Cell Therapy
- Amryt Pharma
- Applied Therapeutics
- AstraZeneca
- Boehringer-Ingelheim International
- CenterView Partners LLC
- Chiasma Inc.
- Chive Charities
- Cowen Inc.
- Daiichi Sankyo Company
- Enzyvant Therapeutics
- EVERSANA
- Homology Medicines
- Invitae Corporation
- Jazz Pharmaceuticals
- Kiniksa Pharmaceuticals
- Matthew R. Patterson
- NS Pharma
- Passage Bio
- PerkinElmer
- Pharming
- Spark Therapeutics
- The Assistance Fund
- United Therapeutics
- Zogenix, Inc.

GUIDESTAR PLATINUM TRANSPARENCY 2021

PUTTING PATIENTS FIRST
National Health Council
Standards of Excellence Certification Program

CHARITY NAVIGATOR 100 OUT OF 100 GIVE WITH CONFIDENCE

BOARD OF DIRECTORS

The Foundation Board of Directors is comprised of individuals with decades of experience in the government, nonprofit, finance, science, medicine, industry, and academic sectors. Several board members are the parents of children with a rare disease, enabling them to offer firsthand knowledge of the challenges facing the rare disease community.

Mark Dant, Chair
Executive Director, Ryan Foundation

Frank Sasinowski, MS, MPH, JD, Vice Chair
Director, Hyman, Phelps & McNamara P.C.

Julia Jenkins, President
Executive Director, EveryLife Foundation for Rare Diseases

Jennifer Bernstein, Secretary
Executive Vice President,
Horizon Government Affairs

Vicki Seyfert-Margolis, PhD, Treasurer
Founder and CEO, MyOwnMed

Emil Kakkis, MD, PhD, Founder
President/CEO, Ultragenyx

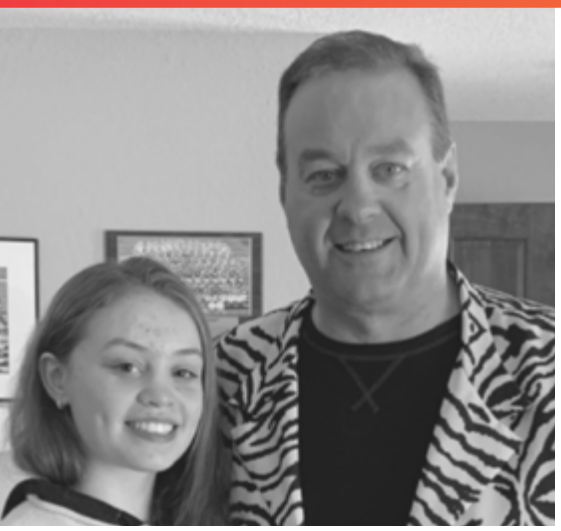
Ritu Baral, Member
Managing Director
Senior Biotechnology Analyst,
Cowen and Company

Richard S. Finkel, Member
Director of Experimental Neurotherapeutics in Translational Neuroscience Program,
St. Jude Children's Research Hospital

Stephen C. Groft, Member
Special Volunteer, National Center for Advancing Translational Sciences at NIH

Abbey Hauser, Member
Young Adult Rare Disease Advocate

Amrit Ray, MD, MBA, Member
Chief Patient Officer,
Biohaven Pharmaceuticals



The EveryLife Foundation works to:

- Empower the patient voice
- Ensure patient access to therapies and cures
- Close the innovation gap
- Eliminate the diagnostic odyssey
- Improve the regulatory process

EVERYLIFE FOUNDATION FOR RARE DISEASES

A disease is defined as rare when it affects fewer than 200,000 people in the United States.

More than thirty million Americans are living with one or more rare diseases.

The economic impact of 379 rare diseases reached nearly $1 trillion in the U.S. in 2019.

Rare disease patients wait an average of 6.3 years after symptoms present before receiving a confirmed diagnosis.

93% - 95% of the more than 7,000 known rare diseases have no U.S. Food and Drug Administration-approved therapies.

501(c)(3) nonprofit organization (Tax ID 26-4614274)

1012 14th St, NW, Suite 500, Washington, D.C. 20005
(202) 697-RARE (7273) info@everylifefoundation.org
everylifefoundation.org

@EveryLifeOrg

The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures.