EVERYLIFE FOUNDATION FOR RARE DISEASES

15TH ANNIVERSARY

2023 ANNUAL REPORT

everylifefoundation.org

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- **Lina Arslanian,** Associate Director of Finance and Salesforce
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- **Ted Brasfield,** Vice President of Alliance Development
- **Stephanie Siddiqi,** Associate Director of Development
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- **Baillie McGowan,** Associate Director of Policy and Research
- **Priscilla Rodriguez,** Diversity Inclusion Advocacy Manager
- **Dylan Simon,** Director of Policy
- **Emily Stauffer,** State Policy Manager

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"Thanks to Rare Artist, I've never felt more seen or heard."

Nell Choi
2023 Artist-to-Advocate Awardee

Mission

The EveryLife Foundation for Rare Diseases is a 501(c)(3) non-profit, non-partisan 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.

A NEW ERA

As we celebrate the 15th anniversary of the EveryLife Foundation for Rare Diseases, we reflect on the profound impact we have made together and the expansive journey that still lies ahead. This year not only marks a milestone in our history but also heralds the beginning of an exciting new chapter under our joint leadership. We are both deeply honored to steer the Foundation during this pivotal era.

This past year has been marked by significant achievements, including the release of our landmark report "The Cost of Delayed Diagnosis in Rare Disease : A Health Economic Study," which illuminated the avoidable economic and personal impacts that delayed diagnoses place on patients, their families, and the healthcare system. This study underscores the urgent need for enhanced diagnostic tools and services, which can dramatically improve patient outcomes and reduce healthcare costs.

Looking forward, we aim to build on the success of our Community Congress and Rare Disease Week, while also broadening our scope to include cutting-edge science policies which address innovative therapy platforms such as gene and cell therapies, and advanced regulatory science strategies. Our plans are ambitious, reflecting our commitment to meet the urgent needs of the rare disease community with equally robust solutions.

We are setting goals to double our budget and reach over the next 5-7 years, leveraging novel clinical trial approaches and employing AI and big data to shorten the diagnostic odyssey for rare disease patients—a public health imperative that touches us all.

As we embark on this journey of growth and innovation, your continued support and engagement are essential. With your help, we will expand the EveryLife community, solve critical issues, and demonstrate the significant impact of focused action on rare diseases as a public health priority.

Thank you for standing with us through these 15 transformative years. Together, we will continue to break barriers and forge paths toward a future where every rare disease patient receives timely treatment and the possibility of a cure.

Michael Pearlmutter
Chief Executive Officer,
EveryLife Foundation

Vicki Seyfert-Margolis
Board Chair,
EveryLife Foundation

DRIVING POLICY

In 2023, we continued our mission to inform policy changes that enhance the lives of rare disease patients by developing and leveraging impactful evidence. This year, we highlight the significant findings from our new economic impact study, "The Cost of Delayed Diagnosis in Rare Disease," which sheds light on the profound financial and health burdens caused by delays in diagnosing rare diseases. This study enhances our understanding and further solidifies the urgent need for policy interventions that support early diagnosis and access to treatments.

Publications

The Cost of Delayed Diagnosis in Rare Disease: A Health Economic Study
Emphasizes the critical importance of timely diagnosis for rare disease patients by revealing the substantial differences in outcomes between delayed and timely diagnosis.
September 2023

White Papers

Newborn Screening Modernization White Paper
This outlines future visions for the NBS system in the U.S., proposing comprehensive modernization based on expert roundtable discussions.
September 2023

Valuing Rare Disease Treatments in Healthcare: Real Experience, Real Impact
This collaborative project with IVI aimed to improve care for millions affected by rare diseases, focusing on integrating patient and caregiver perspectives into research, enhancing healthcare decisions and access to therapies by exploring patient-centered outcomes and identifying research gaps.
January 2024

Shaping Engagement on Key Issues

Comments on the FDA's ***"Patient-Focused Drug Development Guidance 4"*** emphasized the need for clearer guidance on involving small patient populations and meaningful patient engagement, particularly for the rare disease community.

Comments about the FDA's ***proposed LDT regulations,*** highlighted potential negative effects of a rule that could limit access to critical diagnostics and curb innovation, particularly in small labs and rare disease areas, and recommended modifications to foster continued development of essential diagnostic tools without imposing excessive regulatory burdens.

Scan to find additional publications and comments

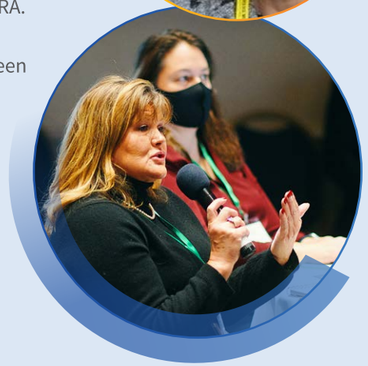
"Driving essential policy change takes determined advocacy and strategic collaboration to ensure that every voice in our community is heard and valued. We're laying the groundwork for a future where rare diseases are not an afterthought, but rather a priority in healthcare innovation and drug development."

Lisa Carlton
EveryLife Board Member
Independent Regulatory Consultant

RARE DISEASE COMMUNITY POLICY IMPACT

United for Progress: Leading Collective Push to Refine Orphan Drug Policies in the Inflation Reduction Act

EveryLife Foundation, in collaboration with NORD and 170 other patient organizations, has actively lobbied Congress to refine the orphan drug exemption in the Inflation Reduction Act (IRA). As we commemorate the 40th anniversary of the Orphan Drug Act, this advocacy underscores the critical need to sustain incentives that have catalyzed progress in rare disease therapy development. This collective effort aims to correct unintended legislative oversights that currently threaten the advancement of treatments vital to the rare disease community.



EveryLife Foundation and its partners took these steps to address issues with the IRA:

- **170** **Collaboration Established:** Formed a coalition with NORD and 170 patient organizations to collectively address the issues.
- **Congressional Advocacy:** Sent a joint letter to key members of Congress, urging the enactment of two specific technical corrections to the orphan drug exemption in the IRA.
- **Clarification of Exemptions:** Advocated for a clear differentiation in the IRA text between orphan designations and approved indications to ensure continued R&D incentives.
- **Preservation of Negotiation Exclusions:** Proposed adjustments to the timeline when orphan drugs become eligible for Medicare negotiation, aiming to extend the period of exclusivity and thus encourage further development of rare disease therapies.
- **Public Engagement:** Called upon community members, stakeholders, and the public to join their advocacy efforts to support these legislative changes.

Advancing Appropriations Priorities

EveryLife Foundation led a successful effort to include three rare disease priorities in the FY24 budget. While the bills were not officially signed into law until 2024, advocacy efforts in 2023 ensured that the language was included in those bills that would eventually pass. The report language that was included will increase rare disease research and support rare disease therapy development.

- ✓ Orphan Products Grants Program: Language was included in the Senate FDA language that highlighted the importance of the FDA Clinical Trials and Natural History Grants program that support rare disease therapy development.
- ✓ Rare Disease Research: Both the House and the Senate supported the National Center for Advancing Translational Sciences (NCATS) rare disease research, recognizing its important role as the center of rare disease research at the NIH.
- ✓ Workshop on Small Patient Population Research: Congress urged the NIH to hold a public workshop, in partnership with the FDA, patient advocates, and other stakeholders, that will address the current efforts and challenges in developing therapies for small patient populations.

Expansion and Focus on State Policy

EveryLife Foundation has significantly expanded its reach and impact in state policy through proactive engagement with legislatures, stakeholders, and the rare disease community.

Advanced Policy Solution Development

- Held **20+ meetings** with state legislators or staff and **40+ meetings** with external partners
- **Passed TX RUSP Alignment Bill,** making it the 11th state to align, resulting in 48% of U.S. newborns being covered by the end of 2023

Empowering the Community to Engage in State Policy

- **Created websites** featuring Copay Accumulator and Access to Care
- **40** **Directly supported 60+ patient advocates** with guidance on RUSP Alignment, Prior Authorization, and Copay Accumulators and more

Supporting National Initiatives

- Tracked **300+ bills**
- Submitted **37 committee testimonies and comment letters**
- **Provided testimony** on Copay Accumulator Bans, Interstate Medical Licensure Compact, and Prior Authorization Reform

SHAPING RARE DISEASE POLICY

COMMUNITY CONGRESS

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.

37 New 2023 Members (Patients + Industry)

236 Patient Organizations

103 Industry Organizations

11 Other Partners

350 Total Member Organizations

Thank You to Our 2023 Working Group Co-Chairs

Public Policy

Lauren Stanford
Parent Project Muscular Dystrophy

Amy Nicole Nayar
Novartis

Newborn Screening & Diagnostics

Meagan Perry
Sanofi

Kim Stephens
Project Alive

Regulatory

Adora Ndu
BridgeBio

Amy Lietman
NTM Info and Research

Access & Value

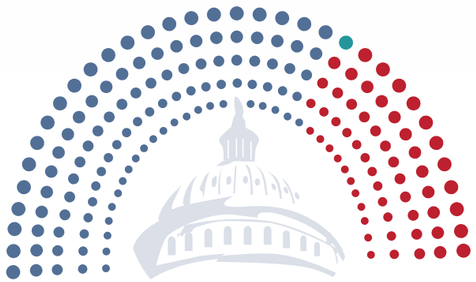
Kelly Maynard
Little Hercules

Emily Roberts
Gentech

RARE DISEASE CONGRESSIONAL CAUCUS

Thank you to the Rare Disease Congressional Caucus Co-Chairs

Representative Gus Bilirakis (FL), **Representative** Doris Matsui (CA), **Senator** Amy Klobachar (MN), and **Senator** Roger Wicker (MS)



172 RDCC Members

House: 142 Members **Senate: 27 Members**

- 110 Democrats (95 House, 15 Senate)
- 1 Independent
- 58 Republicans (47 House, 11 Senate)

The Cost of Delayed Diagnosis in Rare Disease: *A Health Economic Study*

The Impact of Delayed Diagnosis in Rare Disease

Navigating a rare disease diagnosis can require more than six years and 17 medical interventions, on average, after symptoms begin, including emergency room visits and out-of-state specialist appointments.

Delayed Diagnosis Dramatically Increases Cost for Families

This study shows the positive economic impact of newborn screening and early diagnosis, particularly for diseases in which early intervention is key to optimizing long-term health outcomes.

An average **5-year delay in diagnosis** resulted in more likelihood of seeing **3+ specialists**

Delayed diagnosis **shifts healthcare spending away from treatment and supportive therapies** to unnecessary procedures

Cost of the diagnostic odyssey can be eliminated for diseases like SCID, ALD, and Pompe where newborn screening is routine in many states

How Did We Collect These Data?

- Medicare and commercial insurance claims data, including patients with Duchenne, Pompe, ALD, SCID, Fragile X, Wilson, and gMG
- Results from The Rare Disease Financial and Social Impact Survey, completed by 1,409 community members

BENEFITS OF TIMELY DIAGNOSIS

Timely diagnosis improves health outcomes by:

- **Providing earlier access to supportive therapies and treatment**
- **Reducing or eliminating expensive and unnecessary services, tests, and treatments**
- **Preventing deaths and delaying disease complications and physical disabilities**
- **Enabling opportunity to evaluate future family planning based on diagnosis**

ACTION IS KEY TO SECURING TIMELY DIAGNOSIS

The EveryLife Foundation is working to develop policies at the state and federal levels to reduce time to diagnosis. For more information on how you can get involved and support this effort, please visit everylifefoundation.org or contact us at **info@everylifefoundation.org**.

SCAN TO LEARN MORE

EMBOLDENING THE PATIENT COMMUNITY: A YEAR OF ADVOCACY

RDLA Rare Disease Legislative Advocates

Rare Disease Legislative Advocates (RDLA) is a program of the EveryLife Foundation for Rare Diseases designed to support the advocacy efforts of all rare disease patients and organizations.

- **10** Monthly Webinars
- **4** Share Your Story Webinars
- **4** State Webinars
- **13** Advisory Committee Members

RARE DISEASE WEEK ON CAPITOL HILL

In 2023, the 12th annual Rare Disease Week on Capitol Hill included advocates from 47 states, Washington, D.C., and Puerto Rico.

- **600** Advocates
- **300** Meetings
- **300** Patient Orgs

RARE ACROSS AMERICA

Rare Across America 2023 convened virtual Senate meetings and in-person House meetings at in-district offices.

- **447** Advocates
- **278** Meetings

RARE STATE ADVOCACY DAY MADISON, WI COLUMBUS, OH

In 2023, RDLA successfully organized Rare Disease State Advocacy Days in two locations: Madison, Wisconsin on May 17, and Columbus, Ohio on October 17. These events brought together advocates focused on addressing the critical issues impacting rare disease patients.

- **19** Advocates Madison, WI
- **27** Meetings Madison, WI (16 Member Level)
- **26** Advocates Columbus, OH
- **30** Meetings Madison, WI (16 Member Level)

RDLA Rare Disease Legislative Advocates Youth & Teen Advocacy Day

Members of the rare disease community ages 10-18 years old met virtually with members of Congress to share their rare disease experiences. Prior to the meetings, online trainings on legislation and sharing their story were tailored for youth and teens.

- **54** Advocates
- **74** Meetings

RAREHUB WASHINGTON D.C.

A community workspace created to improve cross-disease collaboration and legislative advocacy in the heart of Washington, DC.

- **12** Partner Organizations

Rare Advocacy Learning — EMPOWERING EVERY VOICE

Rare Advocacy Learning launched in 2022 to provide in-depth education and advocacy training, developing a pathway toward year-round advocacy engagement.

- **159** Applicants
- **46** Advocates Awarded Certification

RAREARTIST

Rare Artist provides a platform for artists to advocate through art. In 2023, poetry was added as an accepted medium.

- **20** Finalists
- **228** Pieces Submitted
- **161** Different Diseases

YOUNG ADULT RARE REPRESENTATIVES · YARR

YARR's are highly motivated rare disease community members between the ages of 16 and 30.

- **93** New Members
- **206** Current Members

Speakers Bureau

- **25** Speakers Trained
- **106** Speaking Engagements

Leadership Academy

- **13** Graduates

Summer Series

- **7** YARR Speakers
- **3,250** Total Community Viewers

FINANCIAL SUPPORT TO THE COMMUNITY

$3,121,864
Total Community Support 2019-2023

- **$753,363** 2023
- **$788,610** 2022
- **$770,648** 2021
- **$589,260** 2020
- **$219,583** 2019

$433,175
81 Educational Scholarships

$56,500
15 Event Sponsorships

$50,103
10 Tools, Resources, & Program Support

$6,097
18 Donations Made to Organizations

$202,488
215 Travel Reimbursements to 13 Events

$5,000
Rare Artist Cash Prizes

Rare Giving
LIFTING EVERY VOICE

EveryLife Foundation's Rare Giving program supports individual patients as well as organizations that engage patients, caregivers, and others in the community.

$262,800 Total Awards

$71,000
Tools & Resources Grants

$62,000
Event Sponsorships

$129,800
Rare Disease Week Travel Reimbursements

"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 Clark
Executive Director,
VHL Alliance

#RAREis Scholarship Fund
Powered by the EveryLife Foundation

Thanks to the support of Amgen, EveryLife Foundation established the #RAREis Scholarship Fund to enrich the lives of adults living with rare diseases by providing support for their educational pursuits. In 2023, #RAREis provided its one millionth dollar.

$433,175 In Scholarships Awarded in 2023

87 Scholarships Provided to Patients

595 Diseases Represented

33 States Represented

"I am now able to pursue my dream of making a difference in this field and providing much-needed assistance to these animal owners. I am confident that I will be able to make a positive impact in the lives of people living with rare diseases and their service animals."

Wayne Vance
2023 #RAREis
Scholarship Recipient

"It's joyful to see so many people apply. That shows people that are able to continue education and enter the workforce because of advancement in medication."

Anna Schuster
2023 #RAREis
Scholarship Recipient

RARE FAMILY DAY

EveryLife was pleased to partner with Ultragenyx to bring patients and their families together for Rare Family Day in California.

144 Patients and Families Sponsored

6 Rare Artists Attended with their families

"We cannot thank Ultragenyx and EveryLife Foundation enough for the amazing day! It was magical! Presley probably watches the Rare Family Day video every single day."

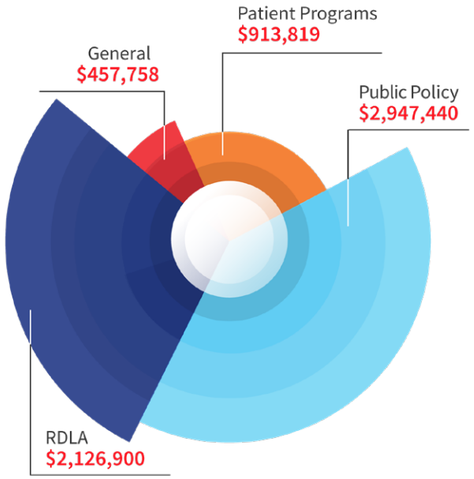
Sky Collins
Mother of Presley Collins (pictured at right)
Diagnosed with Malan Syndrome

"EveryLife Foundation gave me the amazing opportunity to speak about my experience living with Friederichs Ataxia and advocating for rare disease policy at Rare Family Day 2023! I had so much fun and got to finally meet some longtime internet friends as well as amazing people in the rare community, at EveryLife, and at Ultragenyx."

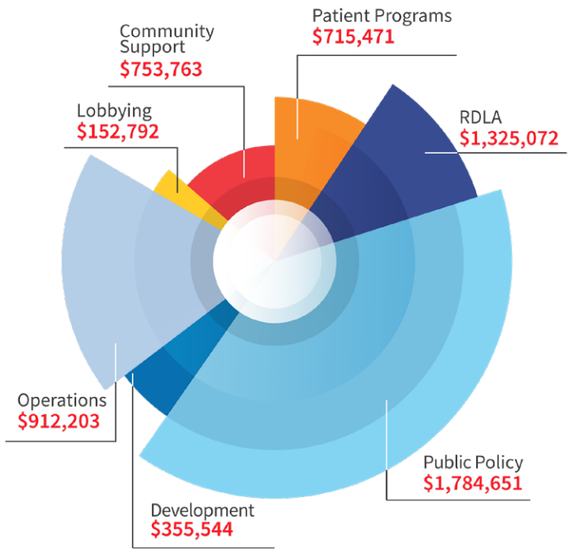
Shandra Trantham, PhD
Rare Disease Advocate
2021 YARR Leadership Academy Graduate

FINANCIALS

$6,445,917
2023 Income



$5,999,496
2023 Expenses



THANK YOU TO OUR 2023 SUPPORTERS

$10,000-$24,999
- AbbVie Inc.
- Adira Foundation
- Agios Pharmaceuticals
- American Society of Gene & Cell Therapy
- Apellis Pharmaceuticals
- Ascendis Pharma
- Astellas Pharma Inc.
- Biotechnology Innovation Organization (BIO)
- Boehringer Ingelheim
- Bristol Myers Squibb
- Burroughs Wellcome Fund
- Edgewise Therapeutics
- Entrada Therapeutics
- Faegre Drinker Biddle & Reath LLP
- Foundation for Sarcoidosis Research
- Global Genes
- Immunovant, Inc.
- Innovation and Value Initiative
- Invitae Corporation
- Kiniksa Pharmaceuticals
- Matthew R. Patterson
- National MPS Society
- National Pharmaceutical Council
- Orchard Therapeutics
- Parent Project Muscular Dystrophy
- Parexel International
- Rare Access Action Project
- Revvity
- Rhythm Pharmaceuticals
- Ritu Baral & Jonathan Feldman
- Servier Pharmaceuticals
- Sick Cells
- Stealth BioTherapeutics
- Stoke Therapeutics
- The Assistance Fund
- William & Carolyn Aliski

$25,000 - $49,999
- Acadia Pharmaceuticals
- Alnylam Pharmaceuticals
- Amryt Pharma
- Avidity Biosciences
- bluebird bio
- Blueprint Medicines
- BridgeBio Inc.
- Eli Lilly and Company
- Enzyvant
- Ionis Pharmaceuticals
- Mitsubishi Tanabe Pharma America, Inc.
- NS Pharma
- Pharming Group
- Sobi
- UCB
- WCG Clinical

$50,000 - $99,999
- Amgen
- Amicus Therapeutics
- BioCryst Pharmaceuticals
- Biogen Inc.
- Chiesi Global Rare Diseases
- CSL Behring
- GlaxoSmithKline
- Harmony Biosciences
- Ipsen
- Jazz Pharmaceuticals
- Johnson & Johnson Innovative Medicine
- Kyowa Kirin, Inc.
- Mallinckrodt Pharmaceuticals
- Neurocrine Biosciences
- PhRMA
- PTC Therapeutics
- REGENXBIO, Inc.
- Sarepta Therapeutics
- Shanahan Family Foundation
- Spark Therapeutics
- Vertex Pharmaceuticals

$100,000 - $299,999
- Alexion Pharmaceuticals
- Argenx US, Inc.
- BioMarin Pharmaceutical Inc.
- Danaher Foundation
- Genentech, Inc.
- Marin Community Foundation
- Merck & Co., Inc.
- Novartis Gene Therapies
- Pfizer Inc
- Silicon Valley Community Foundation
- Takeda Pharmaceutical
- Travere Therapeutics, Inc.

$300,000 and Over
- Horizon Therapeutics USA Inc.
- Sanofi
- Ultragenyx Pharmaceutical Inc.

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  Regional Vice President, Inclusive Policy & Advocacy
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  Community Engagement Coordinator, Team Telomere
- **Michael Pearlmutter**
  Chief Executive Officer, EveryLife Foundation

"My experience at the YARR Leadership Academy significantly enhanced my understanding of rare disease health policy and instilled a newfound confidence in me as a patient. YARR empowered us, young adult advocates, to amplify our voices, share our experiences, and advocate for change."

Royze Cachero
YARR Leadership Academy Graduate
Class of 2023

"Networking with other members of the rare disease community throughout the state of Ohio was exciting. Being able to discuss several different topics vital to the rare disease community helped me feel my voice matters in advocating for positive changes in the state of Ohio."

Jack Wolf
Rare Disease Advocate

"As a healthcare provider, advocate, and rare disease patient, the information provided a dynamic way of understanding the needs and challenges of the rare disease community."

Jamela Gutierrez
Rare Disease Patient & Advocate

"Being part of YARR has been an amazing experience. It's helped me develop skills and gain confidence in public speaking as I continue growing my advocacy."

Marie Abrego
Former YARR Member
from New Mexico

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15TH ANNIVERSARY

501(c)(3) non-profit organization (Tax ID 26-4614274)

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