EVERYLIFE
FOUNDATION
FOR RARE DISEASES

2024 ANNUAL REPORT

everylifefoundation.org

THE EVERYLIFE FOUNDATION TEAM

Executive Leadership Team

- **Michael Pearlmutter,** Chief Executive Officer
- **Annie Kennedy,** Chief of Policy, Advocacy, & Patient Engagement
- **Will Nolan,** Chief Marketing & Communications Officer
- **Trova O'Heffernan,** Chief Operating Officer

Policy, Advocacy, & Patient Engagement

- **Lindsey Cundiff,** Associate Director of Policy Programs
- **Syed Ejaz,** State Advocacy Manager
- **Abbey Hauser,** Associate Director of Community Engagement
- **Kendly Jones,** RDLA Program Manager
- **Baillie McGowan,** Director of Policy Research
- **Kathryn Poe,** State Policy Manager
- **Stephanie Riordan,** Senior Director of Patient Programs
- **Laura Romano,** YARR Program Manager
- **Dylan Simon,** Senior Director of Policy
- **Jamie Sullivan,** Vice President of Policy
- **Shannon von Felden**, Vice President of Advocacy

Operations

- **Lina Arslanian,** Associate Director of Finance and Salesforce
- **Hannah Rhee,** Events Manager
- **Mariela Romero,** Executive Assistant
- **Alyssa Terwall,** Vice President of Operations & Events

Development

- **Carrie Borrello,** Annual Giving Manager
- **Ted Brasfield,** Vice President of Alliance Development
- **Stephanie Siddiqi,** Director of Development
- **Collin Sovie,** Alliance Development Manager
- **James Uyeda**, Vice President of Individual Giving

Marketing & Communications

- **Brenda Colmenares,** Director of Communications
- **Katie Wagman,** Policy Communications Manager

TABLE OF CONTENTS

Our Mission

The EveryLife Foundation for Rare Diseases is powered by the rare disease community to improve health outcomes by driving change through evidence-based policy, leading science-driven policy and regulatory research, activating the community to advocate for their rights and needs, and strengthening the rare disease community.

LETTER FROM THE CEO

Dear Friends and Supporters,

As we reflect on 2024, I am filled with immense gratitude for this community. Your passion, perseverance, and partnership power the mission of The EveryLife Foundation for Rare Diseases forward. This past year has once again demonstrated what is possible when a community unites behind the belief every life deserves access to timely diagnosis, appropriate treatment, and equitable care.

2024 was a year of bold action and measurable impact. We advanced evidence-based policies at both the state and federal levels, saw the formation of the FDA's Rare Disease Innovation Hub, led the effort to renew the Rare Disease Pediatric Priority Review Voucher, and further empowered patient advocates through our Rare Disease Legislative Advocates (RDLA) program. We saw an incredible number of rare disease advocates engage with their elected officials, speak out in the media, and participate in Rare Disease Week on Capitol Hill.

Importantly, we continued to invest in and empower the next generation of change makers and focused our efforts on driving change in areas where systemic inequities persist. Our multi-stakeholder coalitions and policy-driven research are laying the groundwork for transformational shifts in how rare diseases are understood, diagnosed, and treated. We remain guided by our belief that policy should be shaped by those most affected.

Every breakthrough we achieve and every barrier we break down is because of your unwavering commitment.

While the road ahead is long, we move forward with urgency, clarity, and relentless hope. Together, we are building a future where every rare disease patient can thrive.

With deep appreciation,

Michael A. Pearlmutter
Chief Executive Officer
The EveryLife Foundation for Rare Diseases

SHAPING RARE DISEASE POLICY

COMMUNITY CONGRESS

Since 2015, the EveryLife 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 our programs and initiatives.

51 New 2024 Members (Patients + Industry)

249 Patient Organizations

99 Industry Organizations

11 Other Partners

359 Total Member Organizations

Thank You to Our 2024 Working Group Co-Chairs

Public Policy

Lauren Stanford
Parent Project Muscular Dystrophy

Amy Nicole Nayar
Novartis

Newborn Screening & Diagnostics

Matthew Ellinwood
National MPS Society

Meagan Perry
Sanofi

Regulatory

Kim Hollander
Oxalosis and Hyperoxaluria Foundation

Adora Ndu
BridgeBio

Access & Value

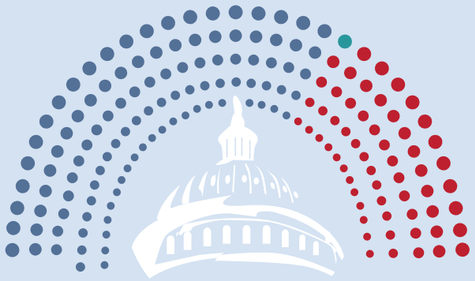
Melissa Kennedy
International Rett Syndrome Association

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 Klobuchar (MN), and **Senator** Roger Wicker (MS)



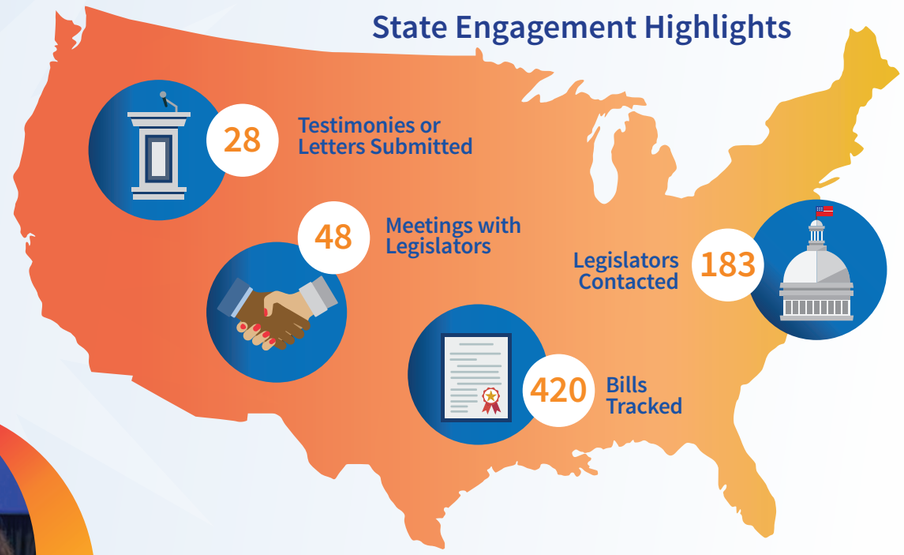
THE YEAR IN POLICY

The EveryLife Foundation worked to elevate patient-centered priorities and ensure that federal and state decision-making reflects the needs of those most affected.

Rare Disease Innovation Hub

Following years of advocacy and engagement from the rare disease community on the need for an Food and Drug Administration Center of Excellence , the FDA announced the formation of the Rare Disease Innovation Hub (RDIH). Following the announcement, the EveryLife Foundation team worked closely with our Community Congress partners in collecting input to inform implementation of the RDIH and its early priorities.

State Engagement Highlights



2024 Legislative and Administrative Engagement

The EveryLife Foundation team supported numerous engagements on the Hill and with members of the Biden administration in 2024, several of which were first-of-their-kind events that demonstrate the product of years of community engagements to build rare disease champions.

The White House held the first ever White House Rare Disease Forum during Rare Disease Week to highlight the Biden Administration's commitment to accelerating the development of treatments and cures for rare diseases and the need to boost support for families. Annie Kennedy represented the EveryLife Foundation by moderating the panel.

The Energy and Commerce Health Subcommittee held the first ever hearing dedicated to rare diseases on Rare Disease Day. The Everylife Foundation supported the committee's preparation and helped prepare witnesses.

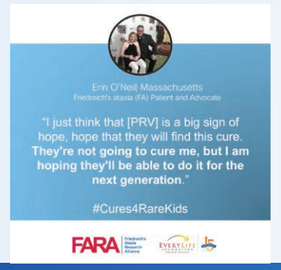
2024 Regulatory Agency Engagement Highlights

Expanded interaction with the newborn screening team at the CDC, leading up to the CDC Newborn Screening Lab Tour organized in conjunction with the EveryLife Foundation's Newborn Screening Bootcamp in Atlanta on September 16-17, 2024.

The EveryLife Foundation team continued meaningful engagement with regulatory agencies to ensure that regulatory events and policies reflected the lived experience and data of rare disease patients.

Rare Pediatric Priority Review Voucher Program Advocacy

The EveryLife Foundation took an increasing leadership role in the efforts to renew the Rare Pediatric PRV Program, aligning with it being in the lead category of our Community Congress Federal Policy Priorities Legislative Grid. The EveryLife Foundation team supported the PRV renewal through direct lobbying, evidence gathering, public education, grassroots advocacy, and a widely supported earned and paid media campaign.



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.

- **8** Webinars
- **4** Share Your Story Webinars
- **4** State Webinars
- **16** Advisory Committee Members

RARE DISEASE WEEK ON CAPITOL HILL

In 2024, the 13th annual Rare Disease Week on Capitol Hill included advocates from 47 states, Washington, D.C., and Puerto Rico.

- **541** Advocates
- **332** Meetings
- **184** Patient Organizations

RARE ACROSS AMERICA

Rare Across America 2024 convened virtual Senate meetings and in-person House meetings at in-district offices.

- **425** Advocates
- **243** Meetings
- **184** Patient Organizations

RARE STATE ADVOCACY DAY NEW YORK & PENNSYLVANIA

In 2024, RDLA successfully organized Rare Disease State Advocacy Days in two locations, New York and Pennsylvania, to address the critical issues impacting rare disease patients.

- **31** NY Meetings
- **30** NY Advocates
- **34** PA Meetings
- **30** PA Advocates

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.

- **87** Advocates
- **81** Meetings

"YARR was the starting point and my permanent home in the rare disease community. It helped me flourish in my own individualized way. I felt content knowing I could be myself and navigate my journey according to what I would like to see in my advocacy. I am fortunate to have found community with strong, empowered, and like-minded people." — **Jonathan Cappiello,** YARR Member & Patient Advocate

RARE HUB WASHINGTON D.C.

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

- **14** 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.

- **5** Topics Covered
- **42** Advocates Awarded Certification

RARE ARTIST

Rare Artist provides a platform for artists to advocate through art.

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

YOUNG ADULT RARE REPRESENTATIVES — YARR

YARRs are highly motivated rare disease community members between the ages of 16 and 30.

- **75** New Members
- **245** Current Members

Speakers Bureau

- **24** Speakers Trained
- **65** Speaking Engagements

FINANCIAL SUPPORT TO THE COMMUNITY

$4,199,790*
Total Community Support 2019-2024

- **$1,078,326** — 2024
- **$753,363** — 2023
- **$788,610** — 2022
- **$770,648** — 2021
- **$589,260** — 2020

- **$615,000** In Educational Scholarships*
- **$25,220** In Event Sponsorships*
- **$151,880** 10 Grants for Tools, Resources & Program Support
- **$6,975** In Donations Made to Organizations*
- **$274,251** In Travel Reimbursements*
- **$5,000** Rare Artist Cash Prizes*

*Data from August 2025

Rare Giving
LIFTING EVERY VOICE

The EveryLife Foundation's Rare Giving program supports organizations that engage patients, caregivers, and others in the community in advocacy and public policy.

$262,800 Total Awards

- **$71,000** Tools & Resources Grants
- **$62,000** Event Sponsorships
- **$129,800** Rare Disease Week Travel Reimbursements

"Receiving the Rare Giving Grant allowed us to offer robust and engaging programming for our Teen Group at our 2024 Family Conference in Aurora, CO! Our teens participated in a 'Telling Your Story' workshop and a 'Transition to Adulthood' workshop, had opportunities to mentor younger patients, and enjoyed a coffee shop outing where they could simply relax, chat, and build relationships with each other. Thank you to the EveryLife Foundation for making this a reality for our teens with HCU!"

— **Liz Carter,** Communications Manager, HCU Network America

#RAREis Scholarship Fund
Powered by the EveryLife Foundation

Thanks to the support of Amgen, 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. In 2023, #RAREis provided its one millionth dollar.

$615,000 In Scholarships Awarded in 2024

- **123** Scholarships Provided
- **116** Diseases Represented
- **37** States Represented

"The #RAREis Scholarship means that I can better focus on my health and school while experiencing less stress about medical school finances. Inspired and informed by my lived experience with Cystic Fibrosis, my rare genetic disease, my goal is to become a physician who can give the gift of medicine to other patients with rare diseases. The EveryLife Foundation for Rare Diseases has reduced the amount of student loans I will need to borrow to achieve this goal. I am incredibly grateful."

— **Jacob Greene,** 2024 #RAREis Scholarship Recipient

RARE FAMILY DAY

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

134 Patients and Families Sponsored

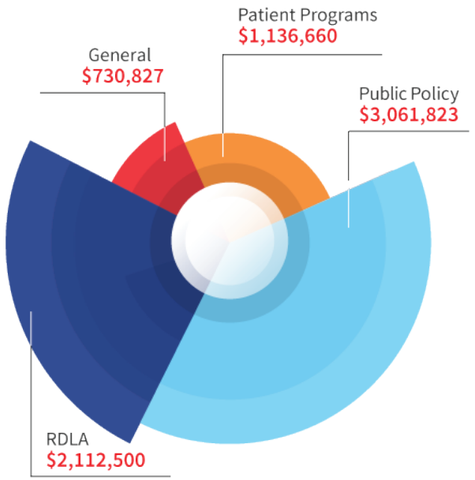
- **6** Rare Artists Attended with their families

"Thank you so much to the EveryLife Foundation for sponsoring our trip to the Rare Family Day! It was such an amazing event and priceless for my daughter to meet other kids like her."

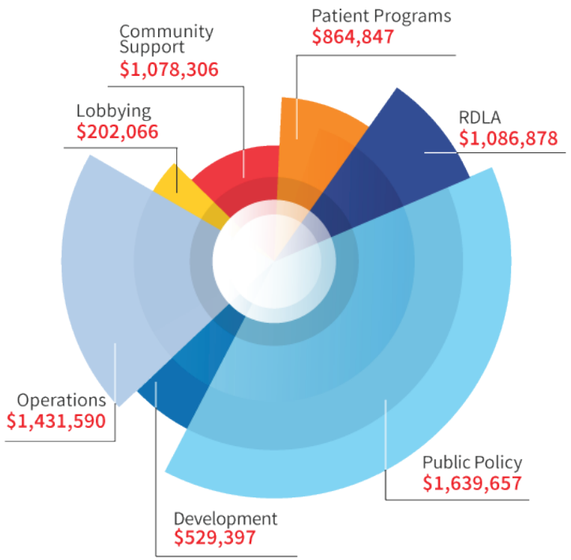
— **Jenna Knowles,** Rare Advocate, Film Writer

FINANCIALS

$7,041,810
2024 Income



$6,832,742
2024 Expenses



THANK YOU TO OUR 2024 SUPPORTERS

$300,000 and Over
- Alexion Pharmaceuticals
- Amgen
- Sanofi
- Ultragenyx Pharmaceutical Inc.

$100,000 - $299,999
- Acadia Pharmaceuticals Inc.
- Argenx US, Inc.
- Biogen Inc.
- CSL Behring
- Genentech, Inc.
- Ipsen
- Kyowa Kirin Co., Ltd.
- Merck & Co., Inc.
- Novartis
- Pfizer Inc
- Sarepta Therapeutics, Inc.
- Takeda Pharmaceutical
- Travere Therapeutics, Inc
- Silicon Valley Community Foundation

$50,000 - $99,999
- Amicus Therapeutics, Inc.
- BioCryst Pharmaceuticals, Inc.
- BioMarin Pharmaceutical Inc.
- Blueprint Medicines
- CHIESI USA, Inc.
- Danaher Foundation
- Exact Sciences Corporation
- GlaxoSmithKline
- Harmony Biosciences
- Johnson & Johnson Healthcare Systems, Inc.
- Mallinckrodt Pharmaceuticals
- Omeros Corporation
- Otsuka America Pharmaceutical, Inc
- Sietstra Family Fund
- Spark Therapeutics, Inc.

$25,000 - $49,999
- Eli Lilly and Company
- Alnylam Pharmaceuticals, Inc.
- Avidity Biosciences
- Biotechnology Innovation Organization (BIO)
- BridgeBio Inc.
- Burroughs Wellcome Fund
- Ionis Pharmaceuticals
- Neurocrine Biosciences, Inc.
- NS Pharma
- Pharming Group
- PhRMA
- PTC Therapeutics, Inc.
- Regenxbio, Inc.
- Servier Pharmaceutics
- Sobi
- Stoke Therapeutics
- Sumitomo Pharma America, Inc.
- Vertex Pharmaceuticals Inc.
- WCG Clinical

$10,000 - $24,999
- AdvaMed
- Agios Pharmaceuticals, Inc.
- Alkermes, Inc.
- Ascendis Pharma
- Astellas Pharma Inc.
- Azafaros B. V.
- Beam Therapeutics
- Boehringer Ingelheim International
- Creyon Bio, Inc.
- Denali Therapeutics
- Edgewise Therapeutics, Inc.
- Faegre Drinker Biddle & Reath LLP
- Insmed Incorporated
- Invitae
- IQVIA Inc.
- Jazz Pharmaceuticals, Inc.
- Mahzi Therapeutics
- Marinus Pharmaceuticals, Inc.
- Mitsubishi Tanabe Pharma America, Inc.
- National Pharmaceutical Council
- Novo Nordisk Inc.
- Parexel International Corporation
- Rare Disease Company Coalition
- Revvity, Inc.
- Rhythm Pharmaceuticals, Inc.
- Savara Inc.
- Stealth BioTherapeutics Inc.
- The Assistance Fund, Inc.
- UCB S.A.
- United Parcel Service of America, Inc.
- Vigil Neuroscience, Inc.

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  Public Health Genetics and Genomics Consultant
- **Stephen C. Groft, Pharm.D.,** Treasurer
  Special Volunteer to the National Center of Advancing Translational Sciences at NIH
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  Regional Vice President, Inclusive Policy & Advocacy, Elevance Health
- **Sarah-Lloyd Stevenson**
  Senior Manager, Public Policy at Amazon
- **Shandra Trantham, Ph.D.**
  Young Adult Rare Disease Advocate

"I honestly felt very alone in my disease journey, often given a glimmer of hope, only for that to turn into a setback. I heavily relied on art, and still will, for an outlet and a way to express my feelings and I am so honored I had a chance to share it with others, and not just anybody, but others who understand the journey through and barriers of rare diseases."

Theresa Alo
2024 Rare Artist Awardee

"I was so excited for my 16th birthday because it was the day I could officially apply for YARR! YARR gives us the opportunity to leverage a life-changing and debilitating diagnosis into something of purpose and teaches us that our stories are our fuel to influence change. I recently spoke about the importance of the PRV at a Congressional Caucus briefing, tying my story to a critical program for rare pediatric patients like me."

Nell Choi
YARR Member and
2023 Rare Artist Awardee

EVERYLIFE FOUNDATION FOR RARE DISEASES

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

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@EveryLifeOrg

Platinum Transparency 2024 Candid.

National Health Council Standards of Excellence Certification Program