EVERYLIFE
FOUNDATION
FOR RARE DISEASES

2025 ANNUAL REPORT

everylifefoundation.org

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- **Annie Kennedy,** Chief Mission Officer
- **Will Nolan,** Chief Marketing & Communications Officer
- **Trova O'Heffernan,** Chief Operating Officer

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- **Lindsey Cundiff,** Associate Director of Policy Programs
- **Syed Ejaz,** State Advocacy Manager
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- **Kendly Jones,** RDLA Program Manager
- **Mary Michael Kelley,** Vice President, Patient Community Engagement
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EVERYLIFE FOUNDATION FOR RARE DISEASES

Our Mission

The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization 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.

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LETTER FROM THE CEO

Dear Friends and Supporters,

As I reflect on 2025, I am filled with gratitude and optimism for what we have accomplished together on behalf of the rare disease community. Every achievement highlighted in this report is a testament to the power of patients, caregivers, advocates, researchers, industry partners, policymakers, and supporters working toward a shared vision: a world where every person affected by a rare disease will thrive.

This year, we strengthened our role as a leading voice for rare disease policy and advocacy. We expanded our Community Congress to 387 member organizations, advanced critical policy research initiatives, mobilized more than 800 advocates during Rare Disease Week on Capitol Hill, and continued driving progress on priorities including newborn screening modernization, the Rare Pediatric Priority Review Voucher Program, and access to innovative therapies.

Our advocacy community continued to grow and inspire. Through Rare Across America, state advocacy days, youth engagement programs, and the Young Adult Rare Representatives network, thousands of advocates connected, made their voices heard, and helped educate policymakers about the urgent needs of the rare disease community.

We also deepened our direct support for patients and families. From awarding 108 scholarships and expanding mentorship opportunities for scholarship recipients to providing grants, travel assistance, and educational resources, we remained committed to reducing barriers and creating opportunities for connection, empowerment, and success.

None of this would be possible without your partnership. Together, we are building momentum, advancing solutions, and creating meaningful change for millions of individuals and families affected by rare diseases.

Thank you for standing with us and helping make every life count.

With deep appreciation,

Michael 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 support and insight on urgent policy issues and our programs and initiatives.

50 New 2025 Members (Patients + Industry)

264 Patient Organizations

101 Industry Organizations

22 Ecosystem Partners

387 Total Member Organizations

Thank You to Our 2025 Working Group Co-Chairs

Access & Value

Parisa Sanandaji
Stoke Therapeutics

Brigid Brennan,
Friedreich's Ataxia Research Alliance

Newborn Screening & Diagnostics

Matthew Ellinwood
National MPS Society

Andrew Herbert
Takeda

Public Policy Regulatory

Lauren Stanford
Parent Project Muscular Dystrophy

Gina Cioffi Loud
Chiesi Group

Regulatory

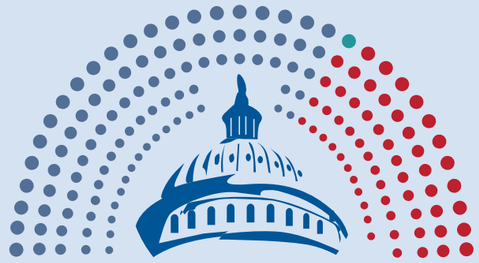
Kim Hollander
Oxalosis & Hyperoxaluria Foundation

Jewell Martin
BioMarin Pharmaceutical

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)



154 RDCC Members

House: 125 Members **Senate: 29 Members**

- 95 Democrats, 81 House, 14 Senate
- 1 Independent
- 58 Republicans, 44 House, 14 Senate

THE YEAR IN POLICY

Policy Research Drives Our Mission

Throughout 2025, the EveryLife Foundation advanced a robust policy research agenda to better understand the needs of the rare disease community and inform evidence-based advocacy.

Key Initiatives Included:

- **Tufts University/NEWDIGS Collaboration:** Developed 10-year projections on the pediatric rare disease therapy pipeline, treatment access, and market trends.
- **International Economic Burden Study:** Partnered with stakeholders across Europe to assess the economic impact of rare diseases on patients, caregivers, and healthcare systems.
- **Medicaid Utilization Analysis:** Conducted a comprehensive assessment of Medicaid's role in supporting the rare disease community.
- **Newborn Screening Roadmap:** Continued efforts to identify opportunities to strengthen and modernize the newborn screening system.
- **Rare Access Initiative:** Advanced patient engagement in payer decision-making through educational resources, committee opportunities, and community-driven recommendations.

Legislative & Administrative Engagement

The EveryLife Foundation remained a leading voice for the rare disease community throughout a year of significant policy activity.

Highlights Included:

- Coordinated a community petition with more than 10,000 signatures supporting federal leadership, biomedical research funding, and public health infrastructure.
- Engaged Congress and federal agencies on the Rare Pediatric Priority Review Voucher (PRV) Program, Newborn Screening Saves Lives Reauthorization Act (NBSSLA), Accelerating Kids' Access to Care Act (AKACA), and Medicaid policy.
- Developed coalition language and conducted congressional outreach to support newborn screening modernization efforts.
- Submitted letters, comments, and recommendations to congressional committees and federal agencies on issues impacting rare disease patients and families.

Regulatory Agency Engagement

The EveryLife Foundation continued meaningful engagement with federal agencies and regulatory partners to ensure patient perspectives informed policy development.

Key Areas of Focus Included:

- Supporting implementation and strategic development of the Rare Disease Innovation Hub (RDIH).
- Engaging FDA on patient engagement, regulatory modernization, and rare disease drug development policies.
- Monitoring progress of the NCATS Whole Genome Sequencing Pilot, CMS Cell and Gene Therapy Access Model, and ARPA-H rare disease initiatives.
- Supporting efforts to strengthen the newborn screening ecosystem through education, collaboration, and stakeholder engagement.

Appropriators' Support for Rare Disease Priorities

The EveryLife Foundation worked with congressional offices and community partners to support sustained federal investment in rare disease programs and infrastructure.

Advocacy Efforts Helped Reinforce Support for:

- The Rare Disease Innovation Hub and its strategic agenda.
- Rare disease research at NCATS.
- Federal newborn screening programs.
- Biomedical and public health research funding critical to rare disease patients and families.

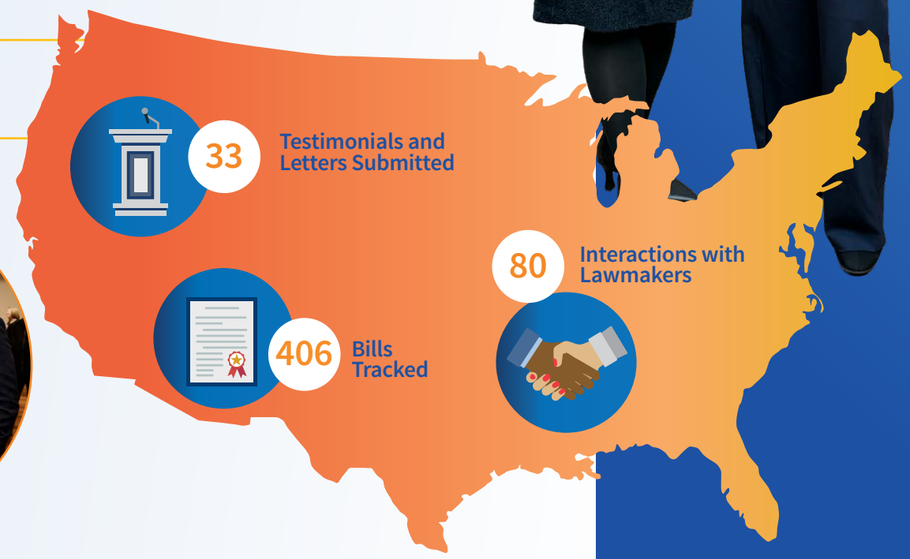
Rare Pediatric Priority Review Voucher Advocacy

The EveryLife Foundation continued its leadership role in advancing reauthorization of the Rare Pediatric Priority Review Voucher Program.

Throughout the Year, the Foundation:

- Coordinated community sign-on efforts and stakeholder engagement.
- Supported congressional outreach and committee engagement.
- Mobilized advocates through educational resources, grassroots actions, and policy updates.
- Maintained momentum around one of the rare disease community's highest federal legislative priorities.

State Engagement Highlights



EMBOLDENING THE PATIENT COMMUNITY

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.

- 9 Webinars
- 4 Share Your Story Webinars
- 4 State Webinars
- 16 Advisory Committee Members

RARE DISEASE WEEK ON CAPITOL HILL

In 2025, the 14th annual Rare Disease Week on Capitol Hill included advocates from 49 states, Washington, D.C., and Puerto Rico.

- 800+ Advocates
- 363 Meetings
- 184 Patient Organizations

RARE ACROSS AMERICA

Rare Across America 2025 convened virtual Senate meetings and in-person House meetings at in-district offices.

- 499 Advocates
- 225 Meetings
- 163 Patient Organizations

RARE STATE ADVOCACY DAY VIRGINIA, MICHIGAN, & NEW JERSEY

In 2025, RDLA successfully organized Rare Disease State Advocacy Days in three locations, Virginia, Michigan, and New Jersey, to address the critical issues impacting rare disease patients.

Michigan	32 Advocates	36 Meetings
Virginia	26 Advocates	45 Meetings
New Jersey	28 Advocates	34 Meetings

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.

- 52 Advocates
- 82 Meetings

A YEAR OF ADVOCACY

RARE HUB WASHINGTON D.C.

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

- 14 Partner Organizations

Rare Advocacy Learning EMPOWERING EVERY VOICE

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

- 5 Topics Covered
- 43 Advocates Awarded Certification

RAL+

- 5 Interactive Discussion Sessions on Health Equity Topics
- 12 Advocates Awarded Certification

EVERYLIFE FOUNDATION FOR RARE DISEASES

Pride in Rare held its first in-person meet-up at Rare Disease Week in 2025. An additional five meetings were held over the course of the year, providing an opportunity for the community to connect and network among others interested in the intersection of advocacy and identity.

RARE ARTIST

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

- 20 Finalists
- 235 Pieces Submitted
- 142 Diseases Represented
- 1 New Medium: Music

YOUNG ADULT RARE REPRESENTATIVES YARR

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

- 76 New Members
- 313 Current Members

Speakers Bureau

- 25 Speakers Trained
- 72 Speaking Engagements

Leadership Academy

- 10 Graduates
- 8 States Represented
- 10 Diseases Represented

FINANCIAL SUPPORT TO THE COMMUNITY

$4,940,674*
Total Community Support 2020-2025

- **$960,467** 2025
- **$1,078,326** 2024
- **$753,363** 2023
- **$788,610** 2022
- **$770,648** 2021

$540,000
In Educational Scholarships*

$25,220
In Event Sponsorships*

$151,880
10 Grants for Tools, Resources & Program Support

$6,975
In Donations Made to Organizations*

$231,392
In Travel Reimbursements*

$5,000
Rare Artist Awards*

*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.

$346,455 Total Awards

- **$76,311** Tools & Resources Grants
- **$73,725** Event Sponsorships
- **$196,419** Rare Disease Week and State Advocacy Day 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!"

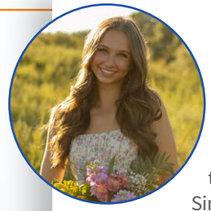
— **Liz Carter,** Communications Manager, HCU Network America

#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.

$540,000 In Scholarships Awarded in 2025

- **108** Community Scholarships provided
  - **104** #RAREis
  - **4** Segalman Family Scholarships for ALS
- **87** Disease Communities Represented
- **35** States Represented
- **39** #RAREis Scholarship Recipients Participated in a Scholarship Mentorship Pilot Program



"I was genuinely excited about how the #RAREis Scholarship would help meet my needs and ease financial stress, but being matched with such an amazing mentor was more meaningful than I ever could have expected. Since I'm going to school out of state and far from home, having someone who truly understands what it's like to live with a rare disease has been incredibly comforting. It's made this experience feel so personal and impactful in a completely different way."

— **Kylie Lindsey,** 2025 #RAREis Scholarship

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.

140 Patients and Families Sponsored

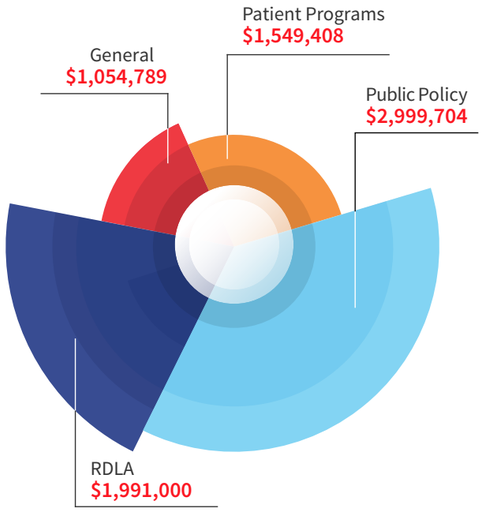
- **7** Rare Artists Attended with their families

"Rare Family Day was not only a great opportunity to be with the rare disease community, but it made me feel seen. For an entire weekend, the weight of everything I hold inside, the exhaustion, the pain, and the unanswered questions were not in the forefront. I did not feel invisible but seen."

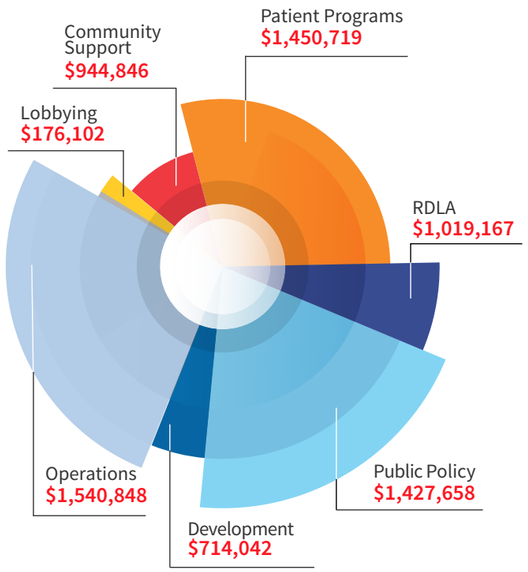
— **Theresa Alo,** Rare Artist Awardee

FINANCIALS

$7,594,901
2025 Income



$7,273,382
2025 Expenses



THANK YOU TO OUR OUR ALLIANCE DEVELOPMENT PARTNERS

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

$100,000 - $299,999
- Acadia Pharmaceuticals Inc.
- Alexion Pharmaceuticals
- Argenx US, Inc.
- CSL Behring
- Genentech, Inc
- GSK plc
- Merck & Co., Inc.
- Novartis
- Pfizer Inc.
- Silicon Valley Community Foundation
- Takeda Pharmaceutical
- Travere Therapeutics, Inc

$50,000 - $99,999
- Alnylam Pharmaceuticals, Inc
- Amicus Therapeutics, Inc.
- Avidity Biosciences, Inc.
- BioCryst Pharmaceuticals, Inc.
- Biogen, Inc.
- BioMarin Pharmaceutical Inc.
- Blueprint Medicines
- Chiesi USA, Inc.
- Ionis Pharmaceuticals, Inc.
- Ipsen
- Johnson & Johnson Services, Inc.
- Kyowa Kirin Co., Ltd.
- Neurocrine Biosciences, Inc.
- Otsuka America Pharmaceutical, Inc
- PhRMA
- Sarepta Therapeutics, Inc.
- Sobi

$25,000 - $49,999
- Alkermes plc
- Astellas Pharma Inc.
- Biotechnology Innovation Organization (BIO)
- Boehringer-Ingelheim International
- BridgeBio Pharma, Inc.
- Burroughs Wellcome Fund
- Denali Therapeutics
- Eli Lilly and Company
- Harmony Biosciences
- Incyte Corporation
- IQVIA Inc.
- Mallinckrodt Pharmaceuticals
- Mitsubishi Tanabe Pharma America
- Moderna
- NS Pharma
- Pharming Group
- PTC Therapeutics, Inc.
- Recordati Rare Diseases
- Regenxbio, Inc.
- Rhythm Pharmaceuticals, Inc.
- Servier Pharmaceutics
- Soleno Therapeutics
- Spark Therapeutics, Inc.
- Stoke Therapeutics
- Sumitomo Pharma America, Inc.
- UCB SA

$10,000 - $24,999
- AdvaMed
- Agios Pharmaceuticals, Inc.
- Ascendis Pharma
- Azafaros B. V.
- Beam Therapeutics
- Catalyst Pharmaceuticals
- Cytokinetics
- Danaher Corporation
- Edgewise Therapeutics
- Elevance Health
- Entrada Therapeutics
- Faegre Drinker Biddle & Reath LLP
- GeneDx
- Gilead Sciences
- Illumina, Inc.
- Insmed
- Jazz Pharmaceuticals, Inc.
- LabCorp
- Mahzi Therapeutics
- National Pharmaceutical Council
- Novo Nordisk Inc
- Ono Pharmaceutical Co., Ltd.
- Orchard Therapeutics
- Parexel
- Revvity, Inc.
- Rocket Pharmaceuticals
- Savara Inc.
- Scholar Rock
- Stealth BioTherapeutics Inc.
- The Assistance Fund
- Vertex Pharmaceuticals Incorporated

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- **Michael Pearlmutter**
  Chief Executive Officer, The EveryLife Foundation
- **Sarah-Lloyd Stevenson**
  Senior Manager, Public Policy, Amazon
- **Shandra Trantham, PhD**
  Young Adult Rare Disease Advocate

"Rare Disease Week gave me the clarity to recognize my purpose and inspired me to pursue advocacy through storytelling. Thank you for helping me see a clear path forward.

Ellie Acuna
Mom of Rare Artist Awardee, Solana

"Rare Artist opened my eyes to a world I didn't know existed. At Rare Disease Week on Capitol Hill, I met another cane user who taught me how to use a blind cane and showed me I wasn't alone. Rare Artist also helped me earn an artist residency where I am creating tactile art for the low vision and blind community. Now I am on my way to doing the things I have dreamt of."

Lena Regina Smith
Rare Artist Awardee

"At times, having a rare disease can feel extremely lonely, but the YARR Leadership Academy really helped me remind myself that there are other people out there overcoming some of the same hardships I am."

Sarah Grace Cattell
2025 YARR Leadership Academy Graduate

EVERYLIFE FOUNDATION FOR RARE DISEASES

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

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Platinum Transparency 2024 Candid.

Putting Patients First
National Health Council Standards of Excellence Certification Program