EVERYLIFE
FOUNDATION
FOR RARE DISEASES

2022 ANNUAL REPORT

everylifefoundation.org

EVERYLIFE TEAM

Executive Team

- **Julia Jenkins,** Executive Director
- **Megan Pinegar,** Chief of Operations
- **Annie Kennedy,** Chief of Policy, Advocacy and Patient Engagement

Operations

- **Alyssa Terwall,** Director of Patient Programs and Special Events
- **Stephanie Riordan,** Associate Director of Patient Programs
- **Lina Arslanian,** Finance and Operations Manager
- **Chrissy Lanham,** Event Coordinator

Patient Engagement

- **Lindsey Cundiff,** Associate Director of Patient Engagement
- **Courtney Felle,** YARR Program Manager

Policy

- **Jamie Sullivan,** Senior Director of Policy
- **Dylan Simon,** Director of Policy
- **Baillie McGowan,** Associate Director of Policy and Research
- **Emily Stauffer,** State Policy Manager
- **Jack Meloro,** Policy Programs Manager

Advocacy

- **Shannon von Felden,** Senior Director of Advocacy
- **Katelyn Laws,** RDLA Program Manager
- **Amanda Houdeschell,** State Advocacy Manager
- **Priscilla Rodriguez,** Diversity Inclusion Advocacy Manager

Development

- **Ted Brasfield,** Senior Director of Development
- **Stephanie Siddiqi,** Alliance Development Manager
- **Collin Sovie,** Alliance Development Manager

Communications

- **Britta Dornan,** Senior Director of Communications and Marketing
- **Brenda Colmenares,** Associate Director of Communications

TABLE OF CONTENTS

"Rylie's confidence has grown so much, and a lot of it has come from the opportunities and experiences [Rare Artist] helped provide—we're very grateful."

Stephanie Erbacher
Mother of 2022 Rare Artist Awardee, Rylie Erbacher

"Attending these [Rare State Advocacy Day] smaller legislative meetings gave me a renewed confidence in my ability to share my story with legislators. I left the event feeling incredibly proud to be helping make a difference for the rare disease community right here in Massachusetts."

Laura Romano
YARR Leadership Academy Graduate
YARR Speakers Bureau

A TRANSFORMATIVE YEAR

In 2022, the world began to emerge from the COVID-19 pandemic and so did the rare disease community. In February, we hosted Rare Disease Week on Capitol Hill online amidst an Omicron surge. By December, we were toasting RareVoice Awardees in person at Arena Stage in Washington, D.C. While the pandemic has been a difficult time for the world, it shed a light on new ways for the rare disease community to engage with one another and with policymakers.

Thanks to the embrace of technology, far more patients, caregivers, and others touched by rare disease are making their voices heard on public policy, and they are doing it on their own terms — when and from where they prefer. The Foundation is proud to have transformed its advocacy events and activities, making them more accessible and inclusive. We are committed to maintaining virtual advocacy opportunities and continuing to offer innovative ways for advocates to participate.

We were pleased to resume providing financial support to enable in-person advocacy. In 2022, we provided a record $788,610 in travel reimbursements, scholarships, and event sponsorships, bringing the Foundation's total direct community support since 2019 to $2.3 million.

Additionally, the Foundation has continued to develop evidence which is key to successful policy change. The universal adoption of the findings from our National Economic Burden of Rare Diseases Study proved the power of compelling data and the impact it can make when combined with personal stories. In 2022, the Foundation led the generation of more papers, publications, and guides than ever before. This growing body of evidence will help ensure the experience of the rare disease community is reflected accurately in policy discussions.

As 2022 ended, we marked a bittersweet milestone — the retirement of our board chair Mark Dant due to term limits. While Mark is no longer on our board, he remains a hero to our community and his work will have a lasting impact for years to come. We welcomed parent and patient advocate Frank Sasinowski as our new board chair as well as three new board members.

Thank you for your support and engagement during this year of transformation and growth. Without you — the patients, caregivers, patient organizations, industry partners, and legislative champions — none of our work would be possible. We hope that you will join us in 2023 as we continue to advocate for diagnostics, treatments, and cures.

Sincerely,

Julia Jenkins
Executive Director,
EveryLife Foundation

Frank Sasinowski
Chairman of the Board of Directors,
EveryLife Foundation

EVIDENCE TO INFORM POLICY

Advancing science-driven legislation and policy is our mission. In 2021,i the Everylife Foundation sponsored the community led Economic Burden of Rare Diseases Study which was groundbreaking in its comprehensive assessment of rare disease as a public health crisis. Following this study, the Foundation has led the development of numerous publications, papers and guides to ensure the experience of the rare disease community is reflected accurately in policy discussions.

Publications

A Blueprint To Advance Patient-Centered Core Impact Sets, Health Affairs, June 2022

Patient-Centered Core Impact Sets: What They are and Why We Need Them, The Patient, June 2022

Common Challenges and Identified Solutions for State Newborn Screening Programs during COVID-19 Pandemic, International Journal of Neonatal Screening, Jan. 2022. Informed by the 2020 Rare Disease Scientific Workshop

The National Economic Burden of Rare Disease in the United States in 2019, Orphanet 2022.

The Economic Burden Of Rare Diseases: Quantifying The Sizeable Collective Burden And Offering Solutions, Health Affairs, February 2022

Scan to find additional publications and comments

Guides

(PFFD) The Guide to Patient Involvement in Rare Disease Therapy Development, informed by the 2021 PFFD Compendium workshop Series

Healthcare Decision Making For Young Adults With Rare Diseases – a resource guide launched by the Young Adult Rare Representatives to provide resources for supportive, responsive, and inclusive healthcare for young adult patients.

White Papers

A Community Vision for a Rare Disease Center of Excellence at the FDA

Rare Disease Week on Capitol Hill 2021, Diversity Roundtable Discussions

Comments

FDA's Diversity Plans to Improve Enrollment of Participants from Underrepresented Racial and Ethnic Populations in Clinical Trials: Guidance for Industry.

"Data-driven publications are an important tool to influence policy making. The Foundation's focus on developing the evidence needed to drive policy for rare disease patients has been incredibly impactful on Capitol Hill."

Jennifer Bernstein
Horizon Government Affairs
EveryLife Board Member

RARE DISEASE COMMUNITY POLICY IMPACT

In 2022, the EveryLife Foundation and rare disease advocates made progress in advancing policies beneficial to the community on both the state and federal level.

Prescription Drug User Fee Act, or PDUFA VII, Reauthorized

Thanks to the robust engagement of patient advocates over several years, PDUFA VII was reauthorized in September. The law reauthorizes the collection of user fees from drug manufacturers. It also represents a continuation of the FDA's commitment to advancing the fields of rare disease therapy development and patient engagement. The FDA's commitment includes:

- ✓ The establishment of Rare Disease Endpoint Advancement (RARE) pilot;
- ✓ The establishment of the Split Real Time Application Review (STAR) pilot;
- ✓ Advancing patient-focused drug development within CBER; and
- ✓ Refining and advancing policies to support use of Real-World Evidence (RWE) and innovative trial designs.

Federal Spending Package Benefits Rare Disease Community

Congress included many healthcare policy riders in its end of year spending bill. The inclusion of these items is a direct result of advocates developing relationships with their representatives and making these priorities known. Several of these policies originated in the **Speeding Therapy Access Today Act** for which thousands of patients and caregivers advocated. Here are the highlights that reflect the strong engagement of the rare disease community:

- ✓ Increased funding for FDA, ARPA-H, and NCATS for research in the development of new therapies and diagnostics for rare diseases;
- ✓ Critical funding to support a National Academy of Medicine study of the current newborn screening landscape, which reflects recommendations for enhancements to the entire newborn screening system. (This language had been a central part of the Newborn Screening Saves Lives Reauthorization Act);
- ✓ Reauthorization of the Orphan Products Grants Program, which includes new language adding regulatory science challenges in future grant funding opportunities;
- ✓ Accelerated Approval reform to strengthen and enhance this vital regulatory pathway used to get innovative therapies to patients at the earliest possible moment;
- ✓ Policies aimed at improving clinical trial diversity;
- ✓ Guidelines for pre-approval payor and drug manufacturer communication engagement;
- ✓ Appropriations report language to advance the coverage of genetic testing for children served by Medicaid, aligning with goals of the Precision Medicine Answers for Kids Today Act;
- ✓ Language requiring several new studies and reports from the FDA to better understand how external expertise is incorporated in the review of rare disease treatments (a core component of the HEART Act); and
- ✓ A two-year extension for telehealth flexibilities that have enabled increased access to telehealth for Medicaid and Medicare beneficiaries since the start of the COVID-19 public health emergency.

Many rare disease communities, including RDLA partners, had significant wins included in the spending bill. Among them, the Lymphedema Advocacy Group successfully saw the passage of the Lymphedema Treatment Act, a product of years of advocacy.

In addition, the bill secures appropriations to continue the operations of the Undiagnosed Diseases Network, a lifeline for so many in the rare community who are left without answers on their diagnostic odyssey.

Four States Adopted RUSP Alignment Legislation

Since 2016, the EveryLife Foundation has led and supported efforts to pass legislation on the state level to help ensure that babies born in every state have the same opportunity for diagnosis and treatment.

✓ Passed the RUSP alignment in Iowa, Pennsylvania, Mississippi, and Maryland, bringing the total number of the RUSP aligned states to ten.

Rare Disease Community Wins Victory Over Oregon's Harmful Accelerated Approval Proposal

In early 2022, the state of Oregon submitted a request to the Centers for Medicare and Medicaid Services (CMS) to waive Medicaid coverage of treatments approved via the accelerated approval pathway. In response, the EveryLife Foundation, along with its Community Congress partners and hundreds of rare disease advocates and patient organizations, submitted public comments objecting to this provision in Oregon's waiver request. The outreach worked. On September 28th, CMS approved the **Oregon Health Plan's 1115 Demonstration Waiver** WITHOUT the provision that would have permitted the state to exclude coverage for prescription drugs approved using the accelerated approval pathway, thereby protecting access to safe and effective therapies that are approved using the accelerated approval pathway.

COLLABORATIVE POLICYMAKING

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.

45 New Total Organizations (Patients + Industry)

116 Industry Organizations

228 Patient Organizations

13 Other Partners

349 Total Member Organizations

Thank You to Our 2022 Working Group Co-Chairs

Public Policy

Cristina Casanova Might
UDNF

Chris Porter
Travere Therapeutics

Newborn Screening & Diagnostics

Kim Stephens
Project Alive

Stacey Frisk
Sarepta Therapeutics

Regulatory

Amy Leitman
NTM Info & Research

Paul Howard
Amicus Therapeutics

Access & Value

Khrystal Davis
Texas Rare Alliance

Francis Rienzo
Ipsen

Kelly Maynard
Little Hercules Foundation

NEW STUDY COMING IN 2023!

Assessing the Cost of Delayed Diagnosis in Rare Disease: A Health Economic Study

7 Diseases

17 Technical Advisors

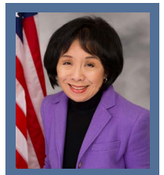
10 Expert Contributors

12 Funding Partners

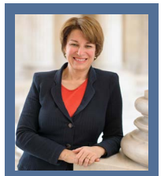
Rare Disease Congressional Caucus

Thank you to the Rare Disease Congressional Caucus Co-Chairs

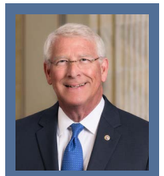
Representative
Gus Bilirakis (FL)



Representative
Doris Matsui (CA)

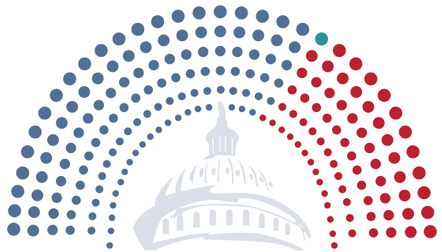


Senator
Amy Klobuchar (MN)



Senator
Roger Wicker (MS)

We were pleased to welcome Congresswoman Matsui as the Democratic House Co-Chair. We are incredibly grateful to former Representative G.K. Butterfield for his service to the Rare Disease Community.



197 RDCC Members

House: 169 Members **Senate: 28 Members**

- 128 Democrats, 113 House
- 1 Independent
- 70 Republicans, 58 House

Newborn Screening

Modernization Roundtables

The roundtables brought together over 100 newborn screening stakeholders, representing a wide variety of expertise including leading patient advocacy organizations, state public health officials, pharmaceutical companies, academia, and clinical providers.

Unique Participants — **108**

- I — Roundtable I: Emerging Newborn Screening Topics: June 6
- II — Roundtable II: Policy Discussion June 9
- III — Roundtable III: Policy Prioritization July 21

Innovate & Modernize — **4 Priorities**

During the roundtables, four priorities arose as critical opportunities to innovate and modernize the existing newborn screening system

1. Improve and expand federal support and oversight
2. Establish a regional lab network
3. Increase access to population-level data
4. Integrate next-generation, evidence-based genomic testing

The final paper, released in 2023, will include proposed, actionable policy changes to achieve newborn screening modernization as envisioned by the stated priorities, including stakeholders to engage, agencies to involve, and proposed implementation pathways for each actional policy change.

Thank you to the Newborn Screening Modernization Roundtable Planning Committee

EMPOWERING 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 of all rare disease patients and organizations.

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

RARE DISEASE WEEK ON CAPITOL HILL

In 2022, the 11th annual Rare Disease Week on Capitol Hill was held virtually and attracted advocates from 49 states, Washington, D.C., Puerto Rico and the Virgin Islands.

- **950** Advocates
- **370** Meetings
- **297** Patient Orgs

RARE ACROSS AMERICA

Rare Across America featured the Foundation's first hybrid congressional engagement as constituent meetings with Representatives were held in-person and meetings with Senators were held virtually. The 2022 event took place August 8-19.

- **416** Advocates
- **242** Meetings

RARE STATE ADVOCACY DAY MASSACHUSETTS

RDLA piloted their first Rare Disease State Advocacy Day in Boston, Massachusetts on June 9. Advocates learned about many issues facing rare disease patients in Massachusetts, including co-pay accumulators, licensing for genetic counselors, and the Interstate Medical Licensure Compact and discussed these important issues.

- **15** Advocates
- **18** Meetings

RAREHUB WASHINGTON D.C.

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

- **13** 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.

- **52** Advocates Awarded Certification
- **221** Applicants

RAREARTIST TO-ADVOCATE

Rare Artist featured 20 Artist -to-Advocates trained in one-on-one advocacy coaching with EveryLife staff to learn how to use the power of art in their advocacy efforts.

- **20** Finalists
- **199** Artists Participated
- **99** Different Diseases

YARR
YOUNG ADULT RARE REPRESENTATIVES

YARR's are highly motivated rare disease community members between 16 and 30 years old.

- **63** New Members
- **171** Current Members

Speakers Bureau
- **25** Speakers
- **70** Speaking Engagements

Leadership Academy
- **11** Graduates

FINANCIAL SUPPORT TO THE COMMUNITY EXCEEDS $780,000

14% **Of the Foundation's 2022 budget went directly to the community through travel stipends, grants, educational scholarships and other financial support.**

$2,329,101
Total Community Support 2019-2022

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

- 81 Educational Scholarships
- 18 Policy and Advocacy Tools Sponsorships
- 16 Event Sponsorships
- 58 Donations Made to Organizations on Behalf of Patient Participation in Rare Disease Week
- 59 Travel Reimbursements to 11 Events

Rare Giving
LIFTING EVERY VOICE

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

$346,938 Total Awards

- **$89,000** Advocacy Tools Grants
- **$101,503** Event Sponsorships
- **$107,000** Rare Disease Week Participating Organizations
- **$49,435** Travel Reimbursements

"The scholarship was invaluable to my organization, without it, I would have been unable to go. I am deeply grateful for the support and guidance the Everylife Foundation has provided to us and so many other rare disease organizations."

Leslie Baldwin
Director, Strategic Advancement
Texas Rare Alliance

"This event will advocate for more sickle cell disease services for Maryland bringing together SCD patients, families, and other advocates to train them on how to educate the Maryland legislature about sickle cell disease and the need for services."

Derek Robertson, MBA, JD, CHC
President, The Maryland Sickle Cell Disease Association

#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.

$405,000 In Scholarships Awarded in 2022

- **81** Scholarships Provided to Patients
- **67** Diseases Represented
- **35** States Represented

"The skills and credentials I've earned with the help of the #RAREIs Scholarship will be directly put to use. The opportunity to take a deep dive with studies at the intersection of non-profit management and population health have enabled me to make a difference in the Myasthenia Gravis community!"

Laura Chandler
Myasthenia Gravis Foundation of America

"Not only has this scholarship helped fund my college education, but it has opened an opportunity for me to learn how to build the courage to advocate for myself and others, which is a priceless award that I will be forever grateful for."

Jordan Howard
#RAREis Scholarship Recipient
YARR Leadership Academy Graduate

RARE FAMILY DAY

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

- **159** Patients and Families Sponsored
- **4** Rare Artists Attended with their families

"What an absolutely wonderful and moving event. How rare is it to get to meet others like us from the rare disease realm and then get to meet those that are working tirelessly to help us out in the medical community. I walked away, feeling so seen, supported and understood."

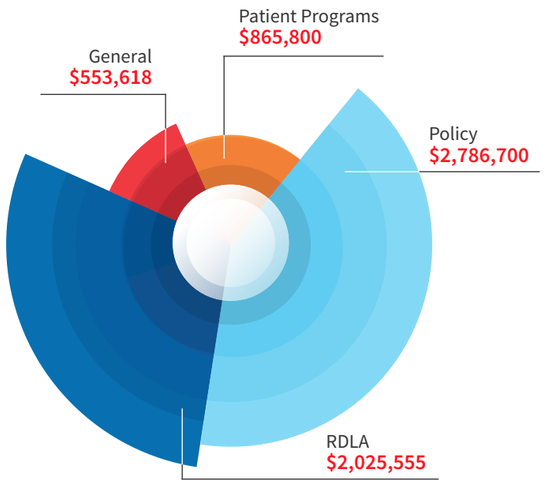
Adare
2021 Rare Artist Awardee, *pictured above*
Kīpaipai Art Foundation

"On behalf of our family, thank you so much for including us for Rare Family day; It was such an honor. Oonagh said she felt so happy to see her Sarge so big and it was so fun to see her other friends art, too. Oonagh loved meeting her rare friends in person. We can't believe how incredibly special everyone made us feel, from the first moment arriving in California! Thank you, thank you!"

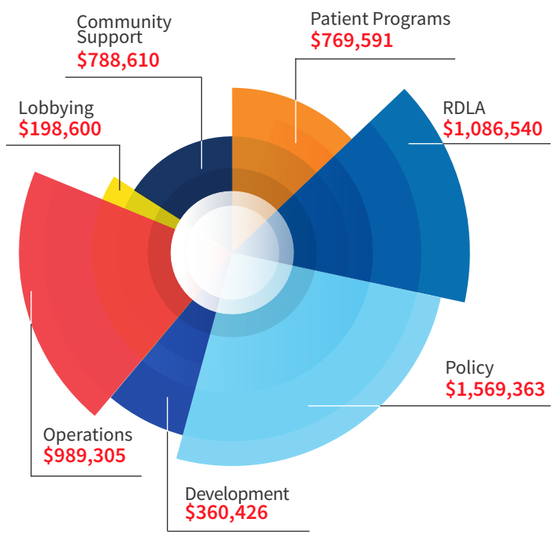
Kelly Newman
Mom to Oonagh Newman,
2021 Rare Artist Awardee
Oonagh pictured above with her sister, Odelle

FINANCIALS

$6,231,673
2022 Income



$5,762,435
2022 Expenses



THANK YOU TO OUR 2022 SUPPORTERS

$10,000-$24,999
- Acadia Pharmaceuticals
- Adira Foundation
- Agios Pharmaceuticals
- Albireo Pharma
- Ascendis Pharma
- Blueprint Medicines
- Boehringer Ingelheim
- Burroughs Wellcome Fund
- Cytokinetics
- Entrada Therapeutics
- EVERSANA
- Global Blood Therapeutics, Inc.
- Insmed, Inc.
- Invitae Corporation
- Kiniksa Pharmaceuticals
- Matthew R. Patterson
- National MPS Society
- Orchard Therapeutics
- Parent Project Muscular Dystrophy
- PerkinElmer
- Rare Access Action Project
- RARE-X
- Servier Pharmaceuticals
- Sick Cells
- Stealth BioTherapeutics
- The Assistance Fund
- Zogenix, Inc.

$25,000 - $49,999
- Alnylam Pharmaceuticals
- Amryt Pharma
- Astellas Gene Therapies
- Avidity Biosciences
- Biotechnology Innovation Organization
- BridgeBio
- Bristol-Meyers Squibb Co.
- Eli Lilly and Company
- Enzyvant
- Ionis Pharmaceuticals
- Mitsubishi Tanabe Pharma America
- NS Pharma
- Pharming
- PhRMA
- Ritu Baral & Jonathan Feldman
- Sobi
- UCB
- Vertex Pharmaceuticals
- William & Carolyn Aliski

$50,000 - $99,999
- Amicus Therapeutics
- BioCryst Pharmaceuticals
- Biogen
- BioMarin Pharmaceutical
- Chiesi Global Rare Diseases
- Gilead Sciences
- GlaxoSmithKline
- Ipsen
- Jazz Pharmaceuticals
- Mallinckrodt Pharmaceuticals
- Merck & Co., Inc.
- Neurocrine Biosciences
- Novartis Gene Therapies
- Otsuka Pharmaceutical Co.
- PTC Therapeutics
- REGENXBIO, Inc.
- Sarepta Therapeutics
- Shanahan Family Foundation
- Spark Therapeutics
- Takeda Pharmaceutical

$100,000 - $299,999
- Argenx US, Inc.
- Chan Zuckerberg Initiative
- Danaher Foundation
- EveryLife Endowment Fund
- Genentech
- Harmony Biosciences
- Janssen Pharmaceuticals
- Pfizer Inc
- Ultragenyx Pharmaceutical

$300,000 and Over
- Alexion Pharmaceuticals
- Horizon Therapeutics
- Sanofi
- Travere Therapeutics

BOARD OF DIRECTORS

- **Frank Sasinowski, MS, MPH, JD, Chair**
  Director, Hyman, Phelps & McNamara, P.C.
- **Vicki Seyfert-Margolis, PhD, Vice-Chair**
  Founder and CEO, MyOwnMed
- **Stephen C. Groft, Pharm.D., Treasurer**
  Special Volunteer, NCATS NIH
- **Jennifer Bernstein, Secretary**
  Executive Vice President, Horizon Government Affairs
- **Julia Jenkins, President**
  Executive Director, EveryLife Foundation
- **Ritu Baral**
  Managing Director & Senior Biotechnology Analyst, Cowen & Company
- **Lisa Carlton**
  Parent Advocate, Vice President Global Regulatory Affairs, REGENXBIO
- **Cristina Casanova Might, BS, MBA**
  Parent and Patient Advocate, CEO Welcomed Co.
- **Merrill Freidman**
  Regional Vice President Inclusive Policy & Advocacy, Elevance Health
- **Abbey Hauser**
  Young Adult Patient Advocate
- **Emil D. Kakkis, MD, PhD**
  Founder & President Emeritus
  President/CEO, Ultragenyx
- **Amrit Ray, MD, MBA**
  Rare Parent, Biopharmaceuticals R&D Executive and Board Director

Thank You, Mark Dant

In December, after nine years of service, including serving as Chair of the Board since 2017, Mark Dant, Executive Director of the Ryan Foundation retired from the Board of Directors.

Mark pictured at left with his son, Ryan Dant, at the 2022 RareVoice Awards.

"An outstanding day of rare disease advocacy in Boston at the State House! Wonderful networking opportunity with other Rare Disease advocates & tremendous chance to meet 1:1 with State legislators to discuss these critical matters. A brilliant idea, no doubt we are stronger together!"

Jennifer Vitelli
Rare Disease Advocate & Parent
Massachusetts PANDAS Council

"I really appreciated how many opportunities we had throughout the [YARR Leadership Academy] course to make connections with other advocates and other leaders in the rare disease community."

Carter Hemion
YARR Leadership Academy Graduate
YARR Speakers Bureau

"Rare Advocacy Learning walked me through the process of how a bill gets drafted, proposed to funding and voting. The capstone project made me really think about where I wanted to focus my energies for the Rare Disease community and helped me create goals so I can be a stronger advocate moving forward."

Mary Mecham
Rare Disease Advocate & Parent
Author

EVERYLIFE FOUNDATION FOR RARE DISEASES

501(c)(3) non-profit 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

Platinum Transparency 2022 Candid.

National Health Council Standards of Excellence Certification Program®

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.