In February 2019, more than 800 rare disease ADVOCATES traveled from 49 states, overflowing the meeting rooms at the Ronald Reagan Building and International Trade Center for Rare Disease Week on Capitol Hill. In nearly 300 meetings with Members of Congress and their staff, advocates urged Congress to pass legislation to bring lifesaving treatments and diagnostics to rare disease patients. In August, 600 advocates participated in over 300 meetings with Members of Congress in their district offices.

In 2019, our Community Congress membership grew by fifty percent, giving patient organizations an equal seat at the table - alongside industry partners - to shape policy discussions and propose solutions. This year, we were proud to launch new COLLABORATIONS as well:

- We hosted the first Newborn Screening Bootcamp with Genetic Alliance/Babies First Test;
- We established our first medical foods policy fellow with the National PKU Alliance; and,
- We became a member of the National Health Council.

One of our proudest POLICY ACHIEVEMENTS this year was having the Further Consolidated Appropriations Act signed into law. This Act included appropriations for a study on the public health impact of rare disease to support investments in diagnostics, research funding and development. As we continue to advocate for policies that will pave the way toward treatments and diagnostic opportunities for rare disease patients, we know that this can only be done by working together.

Thank you to the advocates, patient organizations, industry partners, congressional champions and EveryLife team members who have helped patients to have a voice in public policy. By collaborating with stakeholders and activating the patient advocate, we can continue to change policy and save lives.

We hope that you will join us in 2020 as we expand our programs, policy initiatives and team.

Sincerely,

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

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Changing Policy, Saving Lives

The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit, nonpartisan organization dedicated to advancing the development of treatment and diagnostic opportunities for rare disease patients through science-driven public policy.

The EveryLife Foundation is working to:
- Improve the regulatory process
- Close the innovation gap
- Ensure patients receive earliest access to diagnostic and treatment opportunities
- Enhance the patient voice

- Thirty million Americans are living with a rare disease, making it a public health crisis.
- A disease is defined as rare when it affects fewer than 200,000 people in the United States.
- Rare disease patients must wait an average of six years after symptoms present before receiving a proper diagnosis.
- 50% of rare disease patients are children, 30% of children with a rare disease will not live to see their fifth birthdays.
- 93% of the 7,000 known rare diseases have no U.S. Food and Drug Administration-approved therapies.

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93% of the 7,000 known rare diseases have no U.S. Food and Drug Administration-approved therapies.
When Isabel was offered a private bill that would provide a solution just for her to remain in the U.S., Isabel declined saying, "Thank you, but what about everyone else?"

The Further Consolidated Appropriations Act, 2020 (HR 1865) is signed into law. The law includes appropriations for a Government Accountability Office (GAO) study on the cost of undiagnosed and untreated rare diseases. The Foundation’s Community Congress led the efforts in support of this study to be included in the bill.

The Act directs the GAO to study what is known about the total impact rare diseases have on the U.S. economy, including direct medical costs, non-medical costs, loss of income, and the societal consequence of undiagnosed and untreated rare disease. The GAO shall provide a report on its findings no later than two years after the enactment of the Act.

In August 2019, the United States Citizenship and Immigration Services threatened the deferred action status of thousands of immigrants, including rare disease patients seeking medical deferments – like MPS VI patient Isabel Bueso. Isabel became a leading voice in standing up for rare disease patients undergoing treatment through clinical trials who are facing deportation. Isabel’s plight caught the attention of the national media, including People Magazine, The New York Times and ABC News.

The Foundation, in collaboration with the National MPS Society and CAL Rare, rallied 105 patient organizations in support of a legal pathway to reside in the U.S. for migrant individuals who participate in a clinical trial or who are receiving life-saving medical treatment. In December 2019, the Bueso family received official documentation that their deferred action status has been renewed for two years.
The Foundation’s Rare Disease Legislative Advocates (RDLA) program convened a record-breaking number of advocates during Rare Disease Week on Capitol Hill and during August recess for meetings in Member’s district offices.

800 Advocates
Rare Disease Advocates participated in Rare Disease Week on Capitol Hill

227 Patient Organizations
Represented at Rare Disease Week on Capitol Hill

298 Meetings
Confirmed meetings held with Members of Congress or their staff

600 Advocates
Rare disease advocates participated in Rare Across America

303 Meetings
Confirmed with Members of Congress or their staff

49 States
Represented in Rare Across America

"I love the supportive atmosphere and opportunities to network and the ability to be resources for each other. Thank you for this. This gives so many of us a certain happiness we often feel like we’ll never have."

Rare Disease Week 2019 Advocate

“Rare Across America is an amazing opportunity to bring your rare disease story to the attention of your Member of Congress. Building relationships with US Senators, Representatives and their staffers is crucial to gaining legislative support for issues critical to the rare disease community.”

Allison, Litchfield Park, AZ
Each year, RDLA empowers advocates to reach out to their Members of Congress and ask them to join the Rare Disease Congressional Caucus. The caucus is a bipartisan, bicameral caucus to voice constituent concerns, collaborate on ideas, facilitate conversations between the medical and patient community and build support for legislation that will improve the lives of people with rare diseases.

77 Members
YARR membership grew to 77 young adults representing 24 states

10 Speakers
Young Adult speakers at advocacy conferences in 2019

$219,000 Travel Scholarships & Grants
To Capitol Hill or meetings hosted by the FDA and NIH, sponsorships for rare disease advocacy events, and support for capacity building in state and regional patient organizations.

150+ Advocates
Engaged in RDLA Webinars each month

400 Submissions
More than 400 art submissions to the 2019 Rare Artist Contest
In her 2019 RareVoice Award acceptance speech, Nikia Vaughan quoted an African proverb, “If you want to go fast, go alone. If you want to go far, go together.” We couldn’t agree more. By uniting together, we can make a difference for the 30 million Americans living with rare disease.

Community Congress

50% Increase in Community Congress membership

Rare Hub

The Foundation launched the Rare Hub, a community work space created to improve cross-disease collaboration and innovation among rare disease organizations. Located in Washington, D.C., the Rare Hub offers newly renovated shared office space and is the home of the Foundation’s headquarters.

Rare Hub Partners

The National PKU Alliance established the first Medical Foods Policy Fellow with the EveryLife Foundation. With support from the Foundation’s RDLA program, the fellow advocates on behalf of the Alliance and develops advocacy strategies for the passage of the Medical Nutrition Equity Act.

National PKU Alliance

The EveryLife Foundation achieved the National Health Council’s Standards of Excellence, joining 140 patient organizations to provide a united voice for the 160 million people living with chronic diseases and disabilities and their family caregivers.

National Health Council

Newborn Screening Bootcamp

More than 70 rare disease patient advocates and stakeholders attended the first Newborn Screening Bootcamp, co-hosted by the EveryLife Foundation for Rare Diseases and Genetic Alliance/Babies First Test. The bootcamp preceded the American Public Health Laboratories Newborn Screening and Genetic Testing Symposium in Chicago. The full-day event featured expert speakers including: Joseph A. Bocchini Jr., MD, Chair of the Advisory Committee on Heritable Disorders in Newborns and Children; Alex Kemper, MD, MPH, MS, Chief of the Division of Pediatrics at Nationwide Children’s Hospital; and Michael Gelb, PhD, from the University of Washington.

For left: Kylie Barber, National PKU Alliance Medical Foods Policy Fellow, (left) and Kristen Vanags (Georgia PKU Connect Co-Founder (right). At left: Natasha Bonhomme (left), Genetic Alliance Chief Strategy Officer, and Julia Jenkins (right), EveryLife Foundation Executive Director.
The EveryLife Foundation Board of Directors is comprised of a diverse group of individuals who are both personally and professionally dedicated to the development of treatment and diagnostic opportunities for rare disease patients. With decades of experience in the government, nonprofit, finance, science, medicine, industry, and academic sectors, EveryLife’s Board provides valuable guidance on policy. Several of our 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.

Thank you to our 2019 Supporters

INCOME $3,491,507

EXPENSES $3,366,592

Board Contributions $673,250
Italian Street Painting Marin $46,335
Fundraising $227,922
RDLA $1,044,550

Patient Programs $197,950
Policy Programs $1,301,500
Operations $600,354
Alliance Development $319,706
RDLA $759,854
Lobbying $22,500
Policy $947,656

Policy Programs $379,377

Italian Street Painting Marin $117,562
Patient Programs $219,583
Travel Stipends & Sponsorships $219,583

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 2019 SUPPORTERS

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$500,000 - $500,000
- Sanofi Genzyme
- Mallinckrodt

$100,000 - $200,000
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- Takeda Pharmaceutical Co

$50,000 - $99,000
- Alexion Pharmaceuticals
- Alnylam Pharmaceuticals

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- Wave Life Sciences

THANK YOU TO OUR 2019 SUPPORTERS

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  Uppjohn, Pfizer, Inc.

- Emil Kakkis, MD, PhD, Founder
  President/CEO, Ultragenyx
“Because of the Everylife Foundation and the people I have met through them, I have become a more effective advocate for Sarcoidosis and expanded my capacity to advocate for all rare diseases.”

Mary
Rare Disease Advocate