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

**EveryLife Foundation for Rare Diseases**
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- 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.
The EveryLife Foundation for Rare Diseases was founded by Dr. Emil Kakkis in 2009. Kakkis, a renowned geneticist, has dedicated his life to the development of novel drug treatments for rare diseases. These treatments have saved the lives of thousands of children, children like Ryan Dant.

In 1991, doctors told Ryan’s parents that he would not live to see adolescence due to a rare disease called mucopolysaccharidosis 1 (MPS 1), which had no known treatment. The Dants were determined to change that. Starting with a bake sale netting $342, they raised over $3 million to further research for MPS. The funds proved critical to Dr. Kakkis in the development of Aldurazyme.

Ryan became one of the first patients to receive Aldurazyme, when the clinical trial began in 1998. The response was dramatic. Ryan regained motion in his joints and the swelling in his liver and spleen subsided. Ryan went on to graduate from the University of Louisville.

Despite positive results, the FDA required a second trial, adding years and millions of dollars in funding before Aldurazyme was approved in 2003. In 2009, Dr. Kakkis started the EveryLife Foundation to drive legislative and policy change and bring needed treatments to rare disease patients. In 2017, Mark Dant became chairman of the EveryLife Foundation Board and continues to advance this critical mission.
EVERYLIFE FOUNDATION POLICY IMPACTS

2019

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

The Foundation’s Rare Giving program expanded its financial support, providing nearly $300,000 in small grants and travel stipends to patients and patient organizations.

The Newborn Screening Save Lives Reauthorization Act (H.R. 2507) passed the House of Representatives. The Foundation educated and activated thousands of advocates in support of the bill.

The Foundation’s RDLA program convened a record-breaking number of advocates during Rare Disease Week on Capitol Hill. The event drew 800 advocates who traveled from 49 states plus the District of Columbia. Four-hundred and fifty of these advocates participated in 298 meetings with Members of Congress.

2017

Florida unanimously passed newborn screening legislation (SB1124) in just three months. The Foundation led the lobby efforts of more than 86 patient organizations that supported the bill.

2016

California unanimously passed the EveryLife Foundation-sponsored newborn screening legislation (SB1095) in just seven months. The Foundation coalesced 120 patient organizations to support the bill.

21st Century Cures Act is signed into law. The Foundation partnered with hundreds of rare disease organizations and individual advocates across the country to host days of action and a social media campaign in support of the legislation.
EVERYLIFE FOUNDATION POLICY IMPACTS

**2015**

Impacted FDA guidance on toxicology requirements, reducing drug approval time by a year. When the FDA began applying stricter standards for toxicology studies (the process of evaluating the safety of potential drugs), clinical trials were driven overseas, leaving U.S. patients unable to participate. To address this crisis, the Foundation held its Rare Disease Scientific Workshop on "Rationalizing Safety Testing to Enable Clinical Studies and Approval in the U.S. for Rare Disease Treatments." In less than a year, the FDA implemented the Foundation’s recommendations allowing for flexibility in toxicology requirements and resulting in the reduction of U.S. drug approval time by nearly a year.

**2012**

Food and Drug Administration Safety and Innovation Act (FDASIA) signed into law. The House and Senate versions of the FDASIA bill each included separate provisions specific to the rare disease community. Rare disease patient groups were divided in their support between the two bills. In response, the Foundation led an innovative strategy, encouraging organizations to sign a letter urging Congress to include all five provisions benefiting rare diseases: ULTRA, Patient Focused Drug Development, Pediatric Priority Review Voucher Program, EXPERRT Act and the Breakthrough Therapy Designation. Within 24 hours, more than 120 organizations had signed the Foundation’s letter. The approach paid off. When FDASIA was signed into law in July 2012, the bill included all five rare disease provisions.

**2011**

Unlocking Lifesaving Treatments for Rare Diseases Act (ULTRA) introduced. The Foundation worked with Congress to draft the bill’s language and secure the support of more than 300 patient organizations.

**2010**

Led the effort to form the bipartisan, bicameral Rare Disease Congressional Caucus. The Caucus now stands at 161 Members, making it one of the largest health-related legislative caucuses.

**2009**

Revolutionized the way rare disease organizations collaborate on common goals. During its CURETHEPROCESS campaign, EveryLife individually contacted grassroots patient organizations, educating them on drug development and regulatory process barriers. Nearly 200 patient organizations signed the CURETHEPROCESS pledge. Not since the Orphan Drug Act (passed in 1983) had the rare disease community been activated and united behind a common cause.
Enhance Newborn Screening and Diagnostic Opportunities

Pass the Newborn Screening Saves Lives Reauthorization Act (H.R. 2507 and S. 2158), which would support America’s most successful public health program. Currently, this lifesaving legislation has expired, putting the health of newborn babies at risk.

Pass state legislation requiring newborn screening for disorders recommended by the federal Recommended Uniform Screening Panel (RUSP). Newborn screening programs vary widely by state, leading to disparate health outcomes. A child’s life should not depend on their zip code.

Pass the Advancing Access to Precision Medicine Act (H.R. 4393), which would help ensure children and young adults living with an undiagnosed condition will have access to DNA sequencing clinical services that are currently out of reach.

Expand Rare Disease Expertise at the FDA

Establish a Rare Disease Center of Excellence (COE)
Given the challenges and, therefore, the unique expertise needed to advance the development and review of products for rare diseases, this COE would serve as a consultative and crosscutting body to build knowledge and capacity and consult with review divisions in reviewing applications for rare-disease therapies. Section 3037 of the 21st Century Cures Act provides FDA statutory authority for the establishment of such Inter-Centers; and innovators have long believed that a Rare Disease COE would provide the necessary resources and support to allow offices across FDA to more consistently and efficiently review novel products for these conditions.
Incentivize the Development of Therapies

Preserve the Orphan Drug Act
In the 10 years prior to enactment of the Orphan Drug Act (ODA), only 10 FDA approved rare disease therapies were developed by private industry. Since enactment of the ODA, more than 750 new orphan therapies have been approved. This innovation would not have been possible without the ODA’s exclusivity protections and tax incentives for research. We must find more ways, not fewer, to incentivize the development of treatments and cures for these small patient populations.

Reauthorize PDUFA
The Prescription Drug User Fee Act (PDUFA) was created by Congress in 1992 and authorizes FDA to collect fees from companies that produce certain human drug and biological products. Since the passage of PDUFA, user fees have played an important role in providing critical infrastructure within FDA and expediting the drug approval process. PDUFA VII provides an opportunity to improve the rare disease drug development process.

Promote Access to FDA-Approved Therapies

Support Alternative Payment Models
Our current healthcare system was not designed to pay the costs of a one-time potentially curative therapies, such as gene therapy. Innovative payment models are emerging to account for these novel therapies, but existing statute remains a barrier. The solution is a flexible framework that would enable biopharmaceutical companies and payers to develop customized approaches that ultimately foster patient access.

Study the Economic Burden of Rare Disease
Proposals to lower healthcare costs have largely focused on the price of existing therapies, rather than on the cost of not diagnosing and treating patients. The out-of-pocket cost to the taxpayer, the economy, caregivers, families, and the patient needs to be examined through a comprehensive study and considered when making policy decisions.

The EveryLife 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/
Rare Disease Legislative Advocates (RDLA) provides free resources, tools, and events for patients, caregivers and organizations.

**Rare Disease Week on Capitol Hill**
Rare Disease Week on Capitol Hill brings rare disease community members from across the country together to be educated on federal legislative issues, meet other advocates, and share their unique stories with legislators.

**Young Adult Representatives of RDLA**
The Young Adult Representatives of RDLA (YARR) is a highly-motivated group of 16-30 year olds from the rare disease community. YARR instills confidence in the next generation of rare disease advocates and provides support in their advocacy journey.

**Rare Giving**
Rare Giving provides travel stipends to rare disease advocates, enabling them to meet with Members of Congress the FDA and NIH, and ensuring that policymakers hear directly from patients. Rare Giving also sponsors patient organization’s events that engage advocates in public policy.
Community Congress

Community Congress is a strategic advisory council made up of more than 200 patient organizations, industry leaders and rare disease stakeholders, providing advice and insight on urgent policy issues impacting the availability of treatments, diagnostic opportunities, and access to approved therapies.

Rare Artist

The Rare Artist Contest exhibits the unique gifts of individuals affected by rare diseases and promotes the expression of their stories through art.

Rare Hub

Rare Hub is a newly expanded office in the heart of Washington, DC. The Rare Hub offers affordable, shared office space designed to foster alliances and innovation among rare disease organizations to amplify the community’s voice.

Rare on the Road

RARE on the Road, a Rare Disease Leadership Tour, in partnership with Global Genes, builds and activates the rare disease community at the local level, by hosting regional one day training sessions for rare disease patients, caregivers, and other advocates.
YEARS OF
CHANGING POLICY,
SAVING LIVES

Interview Opportunities

“EveryLife is the organization that changed not just something, but changed everything. When advocates come to an EveryLife event, they see that they can make a difference. They may not have the same disorder, but they all have the same drive and, together, they really can move mountains.”

Mark Dant
EveryLife Foundation Board Chair and Parent Advocate

A retired law enforcement officer who, along with his wife, spearheaded the funding to develop the first MPS enzyme replacement therapy, a treatment that would save their son’s life. For more than 25 years, Dant has served as a key advocate, helping to advance groundbreaking rare disease policy.

“While we are now witnessing a ‘Golden Age’ of rare disease advocacy and scientific advancement, 93 percent of the 7,000 known rare diseases still have no FDA-approved therapy. Thirty million Americans suffer from a rare disease. That’s a public health crisis. We need patient advocates now more than ever to continue to make their voices heard. They have been, and always will be, the key to innovation.”

Julia Jenkins
EveryLife Foundation Executive Director

An expert in grassroots organizing and legislative advocacy, Jenkins has been with the Foundation since its founding and has helped revolutionize the way rare disease organizations collaborate on common goals.

“We are in new era in which scientific advances in many areas have yielded the possibility of profound new rare disease treatments, sometimes bordering on ‘cures’. These therapies have the potential to change the way we prevent, diagnose and treat rare diseases. Supporting policies that further incentivize the development of these transformative therapies, diagnostics, and access paradigms is critical.”

Annie Kennedy
EveryLife Foundation Chief of Policy and Advocacy

An expert in rare disease public policy who has led efforts to pass and implement groundbreaking legislation that incorporates the patient experience in therapy development, establishes collaborations with federal agencies and builds a regulatory infrastructure for rare disease products that has reshaped the therapeutic, care, trial, newborn screening, valuation, and access environments.

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