The EveryLife Foundation for Rare Diseases is a 501(c)(3) nonprofit dedicated to accelerating biotech innovation for rare disease treatments through science-driven public policy. We can do more with the science we already have and bring life-saving treatments to the rare disease patients who need them.

There are only approximately 500 treatments that have been approved by the Food and Drug Administration (FDA) for the 7000 rare diseases which affect more than 30 million Americans. The science exists for many of these diseases to be treated; however, they may never be developed because of roadblocks in the development process, such as a lack of investment and a challenging regulatory environment. The Foundation works with patient organizations, industry, academia, FDA, and National Institutes of Health (NIH) to improve the clinical development process through our Community Congress, annual Rare Disease Scientific Workshop, and legislation at the state and federal levels.

The Foundation seeks practical policy solutions that will:

• Close the innovation gap for the 95% of rare diseases that have no FDA-approved treatment
• Ensure patients receive earliest access to diagnostic and treatment opportunities
• Improve the regulatory process and advance regulatory science for rare disease therapies
• Enhance the patient voice in policymaking, drug development and regulatory decision-making

While the Foundation remains focused primarily on regulatory issues, we also support the rare disease community by spearheading unique programs that fulfill unmet needs and by collaborating with many other organizations to empower the rare disease community.

We look forward to building on the legislative successes of 2016 to be even more effective in 2017, and continuing to grow our network of rare disease advocates across the country.
The Community Congress is a membership-based program that convenes patient organizations, industry leaders and other rare disease stakeholders. The Community Congress meets virtually throughout the year and at an annual meeting in November in Washington, DC. Working groups on Public Policy, Regulatory Science and Newborn Screening inform the Foundation’s scientific and policy goals. The working groups worked on educational materials, such as webinars and a toolkit.

The annual Rare Disease Scientific Workshop brings together experts from industry, academia, federal agencies and other stakeholders to develop strategies, policies and guidances to improve the predictability and efficiency of the drug development process. Each workshop tackles an urgent regulatory issue and produces actionable results to help guide improvements in policy. The 2016 Scientific Workshop focused on Evaluating Models to Expand Access for Rare Disease Patients, and featured speakers from the FDA, industry and patient organizations. A paper covering the findings of this workshop will be submitted for publication in 2017.

The OPEN ACT (Orphan Product Extensions Now, Accelerating Cures and Treatments) would bring hundreds of safe, effective and affordable medicines to rare disease patients within a few years by providing a small incentive to biopharmaceutical companies to repurpose major market drugs for the treatment of rare diseases. The Foundation secured the support of more than 180 patient advocacy organizations for the legislation which was included in the 21st Century Cures Act which passed the House of Representatives in July 2015. Unfortunately, it was not included in the final version of the 21st Century Cures Act which was signed into law.
The Foundation played a pivotal role in the passage of the 21st Century Cures Act, providing feedback to Energy and Commerce Committee members on initial concepts, working closely with Members of Congress and their staff throughout the process, and energizing our advocacy base at critical points along the bill’s journey towards law.

The most significant piece of rare disease-related legislation since the Orphan Drug Act in 1983, the 21st Century Cures Act provided new funding, regulatory improvements and a host of other improvements for the rare disease community. Introduced in the House in May of 2015 by Representatives Fred Upton (R-MI) and Diana DeGette (D-CO), the bill gained momentum in 2016 and was signed into law in December by President Obama following broad bipartisan support in both the House and the Senate.

To ensure the bill’s passage, the Foundation spurred the rare disease community with frequent engagement that grew support and ensured Members of Congress heard from rare disease advocates across the country. Prominent examples of this include a photo campaign in support of #CuresNow and a united day of action with Global Genes and the National Organization for Rare Disorders (NORD). The Foundation also published an op-ed in Forbes encouraging Congress to pass the legislation, ran an ad in Politico and created a video of rare disease advocates to help build momentum for the Act. Two of our most active advocates, Max and Lisa Schill, were invited to the bill signing.

Moving forward, the Foundation will engage with the FDA and Congress to ensure that the Act is implemented in a way to fully realize its potential to dramatically enhance and improve medical research and innovation to benefit patients with rare diseases.
Legislative Milestone: California Newborn Screening

In 2016, the Foundation successfully passed SB 1095 in California, a critical first step for expanding and enhancing newborn screening practices throughout the country. Authored by Senator Richard Pan, a pediatrician, SB 1095 ensures that California screens for a disease within two years of its addition to the federal government’s Recommended Uniform Screening Panel (RUSP), a list of disorders screened at birth and recommended by the Secretary of the Department of Health and Human Services (HHS).

SB 1095 was met with broad bipartisan support at every step in its legislative odyssey. SB 1095 received unanimous support through all committee hearings as well as in full California State Assembly and California State Senate votes. In September, California Governor Jerry Brown signed Senate Bill 1095, ensuring that babies born in California are provided the most comprehensive screening process in the United States.

More than 120 patient organizations supported the bill, including NORD, Global Genes and the Muscular Dystrophy Association. Because of its comprehensive newborn screening platform, California currently saves $9.32 on every dollar spent on newborn screening through avoided surgeries and medical costs. As SB 1095 takes effect, California will begin to screen for MPS I and Pompe by August of 2018 and other diseases within two years of their addition to the RUSP.

“Once a screening has undergone rigorous scientific analysis at the federal level, often taking years to complete, there is no reason to delay, and to do so is catastrophic for families... SB 1095 ensures California implements recommended newborn screening when an early diagnosis and treatment can prevent disability and save lives.”

- California State Senator Dr. Richard Pan, author of SB 1095

Early Diagnosis

Unanimous Support

Lifesaving Policy
RDLA is a collaborative program designed to support the government advocacy of individual rare disease patients and caregivers as well as patient organizations. By growing the patient advocacy community and working collectively, we can amplify our many voices to ensure that rare disease patients are heard in state and federal government.

RDLA provides free resources for successful grassroots advocacy, including:
- Action alerts
- Monthly legislative webinars and newsletters
- Event listings on public policy calendar
- Legislative Scorecards
- Online advocacy tools to contact Congress

This week of events brings rare disease community members from across the country to Washington, DC for education on legislation and training for effective advocacy. The week includes a rare disease documentary screening, Legislative Conference, Lobby Day, Rare Disease Congressional Caucus briefing and Rare Artist reception. In 2016, more than 400 advocates participated in at least one event during the week. More than 130 patient organizations took part in Lobby Day.

Rare disease patients and caregivers can register to have meetings scheduled for them with their Representatives and Senators in their local offices during the Congressional summer recess. This provides advocates the opportunity to build and strengthen relationships with Members of Congress.
In its role as a clearinghouse of information for the rare community, RDLA hosts monthly webinars to discuss policy initiatives, legislation and regulations that could impact rare disease patients and their families. These monthly webinars provide an opportunity for advocates to hear from patient organizations and Congressional staff. On average, 125 people participate in the webinar each month.

The bipartisan Rare Disease Congressional Caucus is a forum for Members of Congress to facilitate conversations between the medical and patient community and build support for legislation that will improve the lives of people with rare diseases. In 2016, it was chaired by Senators Orrin Hatch (R-UT) and Amy Klobuchar (D-MN) as well as Representatives Leonard Lance (R-NJ) and Joseph Crowley (D-NY). RDLA assists with membership and manages quarterly Caucus briefings which address timely issues such as fostering patient engagement, the importance of FDA and NIH, and legislation affecting the rare disease community.

The RareVoice Awards honor rare disease advocates for their efforts at the state and federal levels as well as Congressional and government agency staff who have taken action to benefit the rare community. Senator Orrin Hatch (R-UT) and Representative Joseph Crowley (D-NY) were honored with special recognition at the 2016 awards.

To prepare advocates for In-District Lobby Day meetings during the summer Congressional recess, RDLA hosted regional Legislative Conferences in Boston, Chicago and Seattle. At the conferences, advocates learned about the legislative process, legislation currently under consideration and effective advocacy techniques to build relationships with Members of Congress and their staff.
In 2016, we provided more than $100,000 in financial support to the rare disease community through the Rare Giving program. The Foundation granted more than $50,000 in travel stipends to enable advocates to participate in Rare Disease Week on Capitol Hill. Additional stipends allowed advocates to attend in two public workshops hosted by FDA. The Foundation also provided sponsorships for patient and physician education events that facilitated collaboration across diseases or that engage patients, caregivers and other rare disease community members in public policy.

The North American Metabolic Academy (NAMA) is a professional training program for fellows in biochemical genetic diseases and treatments hosted by the Society for Inherited Metabolic Disorders (SIMD). NAMA’s goal is to encourage interested trainees to pursue careers as biochemical geneticists with a knowledge of inborn errors of metabolism and exposure to the diagnosis and management of metabolic disease. In 2016, the Foundation raised $70,000 to support this essential program.

“Thanks to the Rare Giving Program, I was able to attend a workshop on the Expanded Access Navigator at the FDA. As a patient/parent advocate, the face time with the FDA officials, pharmaceutical representatives, and other patient community stakeholders was effective, powerful and transformative.”

- Cristina Might, Co-Founder, NGLY1.org
The Rare Artist contest highlights the talents of patients, siblings, caregivers and others in the rare disease community and encourages them to express their experience with rare disease through art. In 2016, Rare Artist continued to grow, receiving more than 350 entries. Rare Artist artwork was showcased at receptions during Rare Disease Week on Capitol Hill and the Biotechnology Innovation Organization (BIO) International Convention as well as in galleries at the RareVoice Awards and Italian Street Painting Marin event.

Italian Street Painting Marin, the Foundation’s local community outreach program, showcases the fine art of street painting and supports quality arts-based programs and experiences for under-served students. In 2016, Italian Street Painting Marin provided more than $10,000 in grants awards to local arts-based programs. The 2016 Ciao Bella Roma event featured stunning artwork from more than 100 Madonnari (street painters). Reproductions of works from the late artist Mauricio Saravia, who lost his life to the rare disease McCune-Albright, were also displayed.

“When I am in a lot of pain and I don’t want to take a pain pill, or I already have and it’s not working, sitting there and drawing and focusing on what my hand is doing on the paper takes my mind away from the pain.”

- Megan Howard, Rare Artist Award Recipient
Income and Expenses

2016 Expenses: $2,193,734
2016 Income: $2,279,913

The Foundation’s Board covers fundraising, lobbying and operational expenses. This ensures every donation and sponsorship directly supports our programming. At the end of 2016, the Foundation’s cash reserves were approximately $530,000.

A Rare Affair is held annually in San Francisco the evening before the J.P. Morgan Healthcare Conference. This fundraising event features high-level networking along with wine tasting from some of the most exclusive vineyards in California and gourmet food in the famous Sir Francis Drake Hotel. Guests include CEOs of investment firms, biopharmaceutical companies and associated industries. Funds raised support Foundation programs.

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