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 millions of people suffering from rare diseases.

There are approximately 500 approved treatments for 7,000 rare diseases which affect more than 30 million Americans. The science exists for many of these diseases to be treated, however, treatments 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, Food and Drug Administration (FDA), and National Institutes of Health (NIH) to improve the clinical development process through our Community Congress, annual Rare Disease Scientific Workshop, and legislation.

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 patient advocates.
The OPEN ACT (Orphan Product Extensions Now, Accelerating Cures and Treatments; S 1421/HR 971) would bring hundreds of safe, effective, and affordable medicines to rare disease patients within the next several years by incentivizing drug makers to repurpose major market drugs for the treatment of rare diseases. The Foundation secured the support of more than 150 patient advocacy organizations for the legislation, and was successful in having it included in the 21st Century Cures Act which passed the House of Representatives with broad bipartisan support in July 2015.

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 the fall in Washington, DC. Three working groups inform the Foundation’s scientific and policy goals, and provide valuable input on the prioritization of future initiatives. In 2015, the Regulatory Working Group submitted comments to the FDA on the Draft Guidance on Rare Diseases: Common Issues in Drug Development.

The annual Rare Disease Scientific Workshop brings together experts from industry, academia, FDA, NIH, 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. At the 2015 workshop which focused on Incorporating the Patient Perspective in Rare Disease Drug Development, the Foundation released a draft Framework for Establishing Disease Burden, Disease Measurement, and Benefit-Risk Assessments as Part of Rare Disease Drug Development. This framework was accepted for publication by Nature Reviews Drug Discovery.

The Foundation is working to increase the uniformity and breadth of rare diseases screened in newborns at the state level. Through collaboration with patient organizations and physician experts, we are gathering the evidence needed to implement science-based policy solutions to improve legislative effectiveness and eliminate the time-consuming, state-by-state, disease-by-disease burden. The Foundation drafted legislation to introduce in California in 2016 to help eliminate the legislative delay in newborn screening and ultimately ensure that patients are treated as early as possible.
ADVOCACY

Rare Disease Legislative Advocates (RDLA) is a collaborative program which supports the advocacy of all rare disease organizations and empowers patients, caregivers and others in the rare community to be effective advocates. RDLA resources and events are free for advocates and government officials.

RDLA provides:
- Monthly webinars to highlight key legislation at the state and federal levels
- Congressional scorecards
- Online advocacy tools including action alerts
- Consulting on legislative strategy
- Office space in Washington, DC

This week of events brings more than 300 rare disease community members from across the country to Washington, DC to educate them on legislation and train them to be effective advocates. The event schedule includes a documentary screening, legislative conference, lobby day, Rare Disease Congressional Caucus briefing and Rare Artist reception.

Rare disease patients and caregivers can register to have meetings scheduled for them with their Representative and Senators in their district offices during the Congressional summer recess. This provides advocates the opportunity to build and strengthen relationships with Members of Congress.

Established in the House of Representatives in 2009, the Rare Disease Congressional Caucus expanded to include the Senate in 2015 with Senators Orrin Hatch (R-UT) and Amy Klobuchar (D-MN) as co-chairs. RDLA manages quarterly Caucus briefings and assists with membership.

The RareVoice Awards honors 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.
The Foundation donated more than $100,000 in 2015 through the Rare Giving program in support of individual rare disease patients, caregivers and other advocates as well as patient organizations. Grants include travel scholarships for patient advocates to attend workshops and other public events hosted by the FDA and NIH, and to participate in events on Capitol Hill. The Foundation also provides sponsorships for patient and physician education events that facilitate collaboration across diseases.

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 2015, the Foundation raised $60,000 to support this essential program.

The Rare Artist contest highlights the talents of patients, siblings, caregivers and others in the rare disease community and encourages them to express themselves through art. In 2015, we had a record number of entries at more than 280, and several artists generated media coverage which helped to raise awareness of rare disease and of the work of the Foundation. Rare Artist entries were showcased at a reception during Rare Disease Week on Capitol Hill, RareVoice Awards Gala and Italian Street Painting Marin.

Italian Street Painting Marin, the Foundation’s local outreach program, showcases the fine art of street painting and supports quality arts-based programs and experiences for underserved students. In 2015, it provided nearly $15,000 in scholarship awards supporting local, regional, national, and international artists. The 2015 Carnevale di Venezia event featured the stunning artwork of more than 100 Madonnari (street painters), a parade of traditional Venetian costumes, and a display of selected works from the Rare Artist contest.
The Foundation’s Board covers fundraising, lobbying and operational expenses, allowing every donation and sponsorship to directly support our programming. At the end of 2015, the Foundation’s cash reserves were approximately $400,000.

Rare Affair, the Foundation’s only fundraiser, is held annually before the J.P. Morgan Healthcare Conference at Harry Denton’s Starlight Room on the top floor of the Sir Francis Drake Hotel in San Francisco. Venture capitalists, biopharmaceutical and financial executives, and members of the rare disease community network while enjoying small-batch wines, appetizers and stunning views.
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