Stephanie Fischer
Senior Director of Patient Engagement and Communication
EveryLife Foundation for Rare Diseases

@RarePOV of @EveryLifeOrg
The mission of the EveryLife Foundation is to accelerate biotech innovation through science-driven public policy.

We seek to achieve our goals by advocating practical and scientifically-sound policies to increase the predictability of the regulatory process through scientific analysis and dialogue, grassroots support and expert-led workshops.
We Believe:

• No disease is too rare to deserve treatment.
• All new drugs for rare diseases should be safe and effective.
• We could be doing more with the science we already have.
1) Serve and Support Rare Disease Patients
2) Promote Awareness about Rare Diseases
3) Advance Regulatory Science and Policy
4) Drive Public Policy and Legislative Change
5) Build A Grassroots Advocacy Community
Community Support

**Rare Giving** provides $100,000+ in funding to the community in grants and scholarships to ensure Congress and FDA hear directly from patients and caregivers.

**Rare Artist** promotes awareness of rare diseases and highlights the talent of the rare community. The 2016 contest is accepting entries through December 5th.

We provide financial support to the **North American Metabolic Academy** which trains and encourages the next generation of rare disease physicians and scientists.
Each artist may enter one piece in the contest, and **two awards will be given out in each category**: one by popular vote and one decided by a panel of rare disease community leaders.

**The awards for each category are as follows:**
- Children (4-11): $100 gift card
- Teens (12-17): $250 gift card
- Adults (18+): $500 gift card
- Adults (18+): Photography/Digital Art: $500 gift card

**We accept photos of art in the following forms:**
- Painting
- Photography
- Mixed Media
- Collage
- Pottery
- Sculpture
Public and Scientific Policy Initiatives

We bring patient organizations, industry leaders, and other rare disease stakeholder organizations together to provide valuable insight on prioritizing future initiatives.

We convene leaders from FDA, NIH, patient advocacy organizations, and the biopharmaceutical industry to build the science to improve the clinical development process for rare diseases. The topic this year was expanded access.

We sponsored pilot legislation in California (SB 1095) that will require the state to screen for a disease once it’s on the federal Recommended Uniform Screening Panel. The legislation was signed into law on September 16th.

We are the lead supporter of the OPEN ACT (Orphan Products Extensions Now, Accelerating Cures and Treatments) to encourage biopharmaceutical companies to repurpose approved therapies for rare disease.
Rare Disease Legislative Advocates

• Educates patient advocates about how public policy impacts availability and access to treatments.
• Provides resources to patients, caregivers, physicians and others so they can be successful legislative advocates.
• Provides an online advocacy center and legislative clearinghouse for all rare disease legislation at the state and federal level.
• Builds awareness on Capitol Hill and ensures Congress hears directly from patients and others in the rare community.
Advocacy

Brings 350+ patients to Washington, DC to learn how to build effective relationship with Congress and partner with federal agencies.

Empowers advocates to meet with their Members of Congress during summer recess and hosts Regional Legislative Conferences help prepare advocates.

Hosts quarterly briefings to educate Members of Congress and their staff on issues of importance to the rare disease community.

Recognizes advocates and Congressional aides making a difference in DC and state capitols.
Setting the Stage

• 1 in 10 Americans has a rare disease
  – 30 million Americans
  – 350 million rare disease patients worldwide

• More than 7,000 rare diseases have been identified (so far)

• More than 50% of rare disease patients are children
  – 30% of those children will not live to age 5

• Average diagnostic odyssey is 7 years in the U.S.
  – This includes visits with up to 8 physicians and an average of 2-3 misdiagnoses
The Need and the Challenges

Only 289 of the 7,000 rare diseases have a treatment approved by the U.S. Food and Drug Administration (FDA)
– Less than 5%

Development of treatments is challenging
• Complicated regulatory environment
• Limited number of patients for clinical research
• Difficult to get investment for treatments for very small patient populations
So, You Want to Be an Advocate?

Sign up for monthly newsletters, action alerts and invitations to the monthly webinars and other FREE events at RareAdvocates.org.
Monthly RDLA Webinars

• Any individual or organization is welcome to contribute agenda items, from pending legislation of interest to the rare disease community to new resources (such as NORD’s state impact report) to new policy papers.

• Webinars are available online afterwards as a resource.

http://rareadvocates.org/webinars/
Establish a Relationship with Your Elected Officials

• Call, email AND meet with your U.S. Representative and Senators on a regular basis.
  – Let them know rare disease issues are important to you, and why.
  – Thank them for cosponsoring and/or voting for legislation you support.
  – Attend their town halls and other events.

• Developing a relationship with their staff is equally important.

• We can help you with this effort!
2016 was our fifth and most successful year!

- 330+ patient advocates registered
- 130+ patient organizations represented
- Leaders from FDA and National Institutes of Health (NIH) participated in a panel at the Legislative Conference, Rare Disease Congressional Caucus briefing and Lobby Day breakfast.

[http://rareadvocates.org/rdw/](http://rareadvocates.org/rdw/)
Rare Disease Week on Capitol Hill 2017

**When:** February 27\(^{\text{th}}\) through March 2\(^{\text{nd}}\)

**Who:** Rare disease patients, caregivers and other advocates including physicians

**What:** Series of events aimed at empowering patients

**Where:** Washington, D.C.

**Cost:** FREE for advocates to attend
Rare Disease Week on Capitol Hill 2017

Monday: Cocktail Reception and Film Screening
Tuesday: Legislative Conference
Wednesday: Lobby Day Breakfast
Wednesday: Lobby Day
Wednesday: Rare Artist Reception
Thursday: Congressional Caucus Briefing

All events are free for advocates, but advance registration is required. It will open on January 4, 2017.
The Legislative Conference trains advocates how to have a successful Lobby Day and to build effective relationships with elected officials.

**Past topics included:**
- What to Expect from Congress in an Election Year
- Why and How to Talk to Regulators
- Key Policy Initiatives
- How to Prepare for Hill Meetings
- Pitching Stories and Submitting Letters to the Editor

Two sets of break-out sessions provided the opportunity for advocates to learn more about working with industry, social media, FDA and key state issues such as newborn screening.
Lobby Day

This year, we scheduled 228 meetings for 236 advocates!
In 2016, we awarded more than $55k in travel stipends to offset travel expenses for advocates from 39 states and Puerto Rico. We hope to surpass this next year!

Stipends range from $300 for advocates in Maryland and Virginia to $1000 for Alaska, Hawaii and Puerto Rico. Advocates in other states receive $600.

We’re currently accepting applications for travel stipends for 2017. The deadline to apply is December 18th.

Priority is given to advocates who haven’t received a stipend in the past – so apply!
It will be critically important for the new Members of Congress to hear from patients and caregivers in their communities.

If you cannot attend, you can still ensure that Congress hears from you. We will hand-deliver statements submitted through the website to the author’s U.S. Representative and Senators.

The deadline to submit your perspective is February 12th.

http://rareadvocates.org/rdw-old/patientstories/
In order to help rare advocates build on their relationships with federal elected officials, we schedule meetings for them during the summer district work period in their home states.
This year, we hosted three regional Legislative Conferences (in Boston, Chicago and Seattle) to help prepare advocates for In-District Lobby Days.

They were also a great opportunity to meet other local rare disease advocates!
5th Annual RareVoice Awards: November 16th

• The RareVoice Awards recognize Congressional staff, rare disease advocates, state legislators, Members of Congress and Agency leaders.

• **Join us!** The event celebrates the community so there is no charge to attend.

• It is a great networking opportunity! More than 70 Congressional staffers joined us last year in addition to representatives of government agencies and biopharmaceutical companies.
5th Annual RareVoice Awards

6pm Cocktail Reception
8pm Awards Ceremony
9pm After-Party Celebration

Save the date:
November 16, 2016
Arena Stage, Washington DC

http://rareadvocates.org/rarevoice-awards/
The 21st Century Cures Act (HR 6) includes numerous provisions of critical importance to the rare disease community.

- More funding for FDA and NIH
- Workforce enhancement for FDA and NIH
- Support for the Precision Medicine Initiative
- Reauthorization of Priority Review Vouchers for pediatric rare diseases
- OPEN ACT, to encourage the repurposing of medicines for rare disease

It was passed by the House of Representatives by a broad bipartisan majority (344-77) in July 2015.
Passing 'cures' bill means better health possible for virtually every American

By Sen. Lamar Alexander (R-Tenn.)

Innovation is needed in the fight against rare diseases

Congress should encourage companies to repurpose existing drugs for rare diseases, potentially doubling the number of treatment options for patients.

By Klane K. White and David Fajgenbaum
We need YOUR help! Congress is running out of time to pass 21st Century Cures and send it to the President to be signed into law.
Take Action to support #CuresNow!

1. Make your sign using the handout
2. Snap a photo
3. Post your photo on Twitter using your Senators’ handles with the hashtag #CuresNOW
Take Action to support #CuresNow!

Please also submit your photo through our website to help with an advocacy campaign when Congress convenes in a lame duck session – their LAST CHANCE to pass Cures.

http://everylifefoundation.org/cures-now-photos/
Take Action to support #CuresNow!

You can also call your Senators to encourage them to prioritize 21st Century Cures (HR6).

- Find, write, and call your elected officials
- Map of United States Congressional Districts
- Find information on the elections in your area

http://rareadvocates.org/advocacy-tools/
For those of you based in Canada...

Check out the Canadian Organization for Rare Disorders (CORD).

CORD is Canada's national network for organizations representing all those with rare disorders.

CORD provides a strong common voice to advocate for health policy and a healthcare system that works for those with rare disorders. CORD works with governments, researchers, clinicians and industry to promote research, diagnosis, treatment and services for all rare disorders in Canada.

https://www.raredisorders.ca/
Keep in Touch!

• Please sign-up for our mailing lists at EveryLifeFoundation.org/contact-us and RareAdvocates.org/contact.

• Like us on Facebook as both the EveryLife Foundation for Rare Diseases and Rare Disease Legislative Advocates.

• Follow us on Twitter as @EveryLifeOrg and @RareAdvocates.

• You can reach me at (202) 531-3812 and sfischer@everylifefoundation.org. And find me on Twitter as @RarePOV!