FARA Friedreich's Ataxia Research Alliance

ADVOCATING ALL YEAR LONG

The last two FARA Advocacy newsletters were filled with opportunities to get involved around Rare Disease Day. The FA community was engaged and the stories shared were incredibly impactful. Let's keep that momentum going! *Your voice is needed throughout the year!*

Advocacy can happen in many ways: testifying at a hearing, speaking to your legislators, attending events, writing or speaking about your life experiences, creating art, and more! All of these actions raise awareness of FA and the unmet medical need our community faces. Take advantage of the numerous programs and events throughout the year that give you the chance to educate others and ask them to take action! Here is a great example of FARA Ambassador Mary Nadon Scott changing VT policy.

Montpelier, VT April 10, 2024 | photo courtesy of NORD

FARA Ambassador Mary Nadon Scott helps Pass Step Therapy Legislation in Vermont

NORD and its Rare Action Network (RAN) advocates commend Vermont for enacting step therapy reform as part of H.766, signed into law by Governor Phil Scott on May 21, 2024.

Step therapy policies, also known as fail first, are used by insurance companies in an attempt to control costs by forcing patients to “fail first” on an alternative medication before accessing the medication originally prescribed by their doctor. This can cause delays to necessary care and can be harmful to patients. H.766 establishes categories of exemptions from step therapy protocols, modifies timelines within which health plans must respond to prior authorization requests, and makes responses to requests for a step therapy protocol exemption subject to the same timelines.

FARA Ambassador Mary Nadon Scott said: “How exciting! I have really enjoyed my engagement on the advocacy front with step therapy reform. Having the opportunity to speak as a patient advocate for the Senate Hearing Committee was an incredible way to share my story and rare disease patient perspective. I know my body better than my insurance company does.”

Are you ready to take action, too? In August, when Congressional Members return home for recess, the EveryLife Foundation will be running Rare Across America. All Senate meetings will be scheduled virtually and House meetings will be in person at your Member’s in-state, in-district office (more details below). Don’t miss this wonderful opportunity to educate and advocate for FA!

Capitol Hill Updates

House Energy & Commerce Health Subcommittee

Advance Critical Bills

On May 16, the House Energy and Commerce Health Subcommittee voted to advance several bipartisan bills important to the rare disease community including:

- Creating Hope Reauthorization Act (H.R. 7384)
- RARE Act (H.R. 7383)
- Accelerating Kids' Access to Care Act (H.R. 4758)
- Telehealth Modernization Act (H.R. 7623)

The bills now await votes before the full Energy and Commerce Committee.

Medicaid VBPs for Patients (MVP) Act Update

The Medicaid VBPs for Patients (MVP) Act, recently introduced by Senators Mullin (R-OK), Sinema (I-AZ), Tim Scott (R-SC), and Hassan (D-NH), supplements a Centers for Medicare & Medicaid Services rule allowing public and private payers to voluntarily enter value-based (VBA) arrangements with drug manufacturers. The Senate bill is a companion to the House's MVP Act, sponsored by Representatives Guthrie and Eshoo. The House bill passed out of the Energy & Commerce Committee in 2023.

Innovation in Pediatric Drugs Act of 2023: H.R 6664

The Innovation in Pediatric Drugs Act of 2023, spearheaded by the Children's Cancer Cause and led by Representatives Eshoo and McCaul, builds upon existing legislation such as the Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equality Act (PREA). Introduced in the House on 12/07/2023 aims to enhance pediatric drug research. It proposes removing the current exemption to the requirement for studies on pediatric populations for drugs treating rare diseases, empowering the FDA to penalize companies failing to conduct required pediatric studies promptly, and doubling the funding for the NIH BCPA Program from $25 million to $50 million. More information here.

Biomedical Research Act (H.R. 7539) Introduced

On March 5, Rep. Brian Fitzpatrick (R-PA-1) and Rep. Sanford Bishop (D-GA-2) introduced the Biomedical Research Act (H.R. 7539). This bill

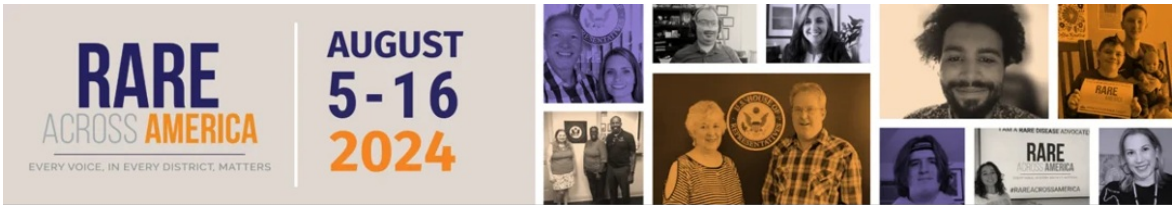
establishes the BioBonds Program that will provide loan funding to eligible biomedical companies and universities to conduct clinical trials approved by the FDA. For more information, click here.

Upcoming Advocacy Events

Virtual Youth and Teen Hill Advocacy Day

Registration has closed for this event but advocates between the ages of 10-18 can still participate by emailing their Members on June 18…or any other day!

You can find who your Members are here. Tell them about yourself and always have an "ask" — what do you want them to do. For example, you can advocate for robust funding for the NIH & FDA in the Fiscal Year 25 budget. Rare Disease Legislative Advocates (RDLA) has also prepared fact sheets on their two other asks here.



Register to Participate in Rare Across America!

Rare Across America 2024 is the opportunity to meet with your Members of Congress at their in-district offices and educate them on the issues that are most important to the rare community by sharing your story.

CLICK HERE TO REGISTER

Where?
All Senate meetings will be scheduled virtually and House meetings will be in person at your Member's in-state, in-district office. Rare Disease Legislative Advocates (RDLA) will schedule meetings for you and help

you to prepare. No prior advocacy experience is necessary.

When?

Meetings with Members of Congress will take place between August 5th and August 16th, and will be scheduled based on your availability. We will be offering training webinars to help advocates prepare for their meetings.

- July 16th, 12pm-1pm ET: **General Training Webinar**
- July 24th, 12pm-1pm ET: **Team Coordinator Training Webinar:** Meetings with 5 or more advocates will have an assigned Team Coordinator to organize the meeting agenda. A Team Coordinator is an advocate who has participated in the event before! Interested in this role? Be sure to volunteer when you register. A link to attend this training will be sent to all confirmed Team Coordinators.
- July 25th, 12pm-1pm ET: **Share Your Story with Policymakers**

State News

NORD Rare Disease Advisory Council Updates

People with rare disorders face unique challenges every day, but lawmakers often struggle to understand their needs. Rare Disease Advisory Councils (RDACs) provide opportunities for patients and advocates to educate their state lawmakers and make formal policy recommendations about pressing issues. To date, **28 states** have signed RDAC legislation into law. To learn more about NORD's Project RDAC, click here.

California: AB 2613 (Zbur), California's Rare Disease Advisory Council (RDAC) bill, has passed without opposition from the Assembly and awaits referral in the Senate. We expect it will be referred in early June and look forward to its advancement through the Senate. NORD Policy staff are organizing a **lobby day on June 27 in Sacramento** and would love to have all members of our California rare disease community join. **To register, sign up here by June 14**.

New York: Legislation to create a permanent Rare Disease Advisory Council in New York, A10292-A / S9724 was recently introduced by Assemblymember Paulin and Senator Persaud.

EveryLife announces that Tennessee Becomes 12th RUSP Aligned State

On Tuesday, April 23 Governor Lee signed SB 1791 into law, codifying the great work the Tennessee Department of Health and Public Health Lab has been doing to keep up with the RUSP and makes Tennessee the 12th state to be RUSP-aligned. Now, over 1.8 million babies are born in RUSP-aligned states each year, 49.7% of all U.S. newborns. To learn more, click here.

Florida Joins the Interstate Medical Licensure Compact

Florida Governor DeSantis signed into law Florida SB7016 making Florida the 42nd member jurisdiction of the Interstate Medical Licensure Compact (IMLC). The compact is an agreement among participating states to streamline the licensing process for physicians. It is a way for states to increase patient access to expert care in person and via telemedicine, which greatly benefits those in underserved or rural communities, individuals attempting to reach expert care from a distance, and patients with complex medical conditions who are unable to travel. To learn more from the EveryLife Foundation, click here.

Announcements

Albany, NY — NY Rare Disease Day April 16, 2024 | photo courtesy of NORD

FARA joins advocates in Albany for NY Rare Disease Day, April 16, 2024

FARA Director of Advocacy Brigid Brennan and FA community member Sarah DuVall join advocates from around the state to encourage NY Senators and Assemble Members to include funding for rare disease research in the NYS budget.

FARA speaks at the World Orphan Drug Congress in Boston, April 23-25, 2024

FARA President Ron Bartek shared the Skyclarys journey in a panel discussion on Conducting Clinical Trials in Rare Diseases.

FA Awareness Day 2024

In honor of FA Awareness Day, which fell on May 18 this year, we asked the FA community to share one way they contribute to the #CommunityToCureFA (participating in research, fundraising, or advocating) and encourage others to do the same.

We also asked the community to share #cureFA photos with us, and wow! You really came through. So many people got creative to spread awareness this year — we can't wait to see what you come up with next year!

#RAREis Scholarship Fund Grows in 2024

Thanks to Amgen's incredible support and increased funding, the EveryLife Foundation is thrilled to share the expansion of the #RAREis Scholarship Fund in 2024, now awarding up to 123 scholarships, compared to the originally anticipated 35. All scholarship applicants will be notified by July.

#RAREis Scholarship Fund
Powered by the EveryLife Foundation

Introducing: Pride in Rare!

The EveryLife Foundation for Rare Diseases is excited to introduce the Pride in Rare program to the rare disease community which will:

- **Celebrate Diversity: Acknowledge and support the unique challenges at the intersection of LGBTQIA+ identity and rare diseases.**
- **Foster Community: Provide a platform for resource sharing and discussions on issues affecting the community.**
- **Advocate for Change: Drive legislative and educational initiatives that impact both LGBTQIA+ and rare disease communities.**

EveryLife is conducting the first-ever Pride in Rare survey. The purpose of the survey is to support internal program planning and development by collecting information on experiences, resource needs, and opportunities to better serve the LGBTQIA+ rare disease community. This online survey is open to those who are living in the United States, ages 18 and older, have been diagnosed with at least one rare disease, and identify as LGBTQIA+. Data collected through the survey will remain anonymous.

If you have any questions, please reach out to Priscilla Rodriguez at prodriguez@everylifefoundation.org.

Take the Survey