FARA Friedreich's Ataxia Research Alliance

Rare Disease Day Recap

Rare Disease Day is a global movement to raise awareness of rare diseases and work towards equity in health care, particularly access to diagnosis and approved treatments for people living with a rare disease. It was celebrated this year on February 28. Since its inception, thousands of Rare Disease Day events have taken place across the world, reaching millions of people and fostering a growing public understanding of rare diseases and the high unmet medical needs of this community. Each year, advocates participate in a number of initiatives such as lighting up community landmarks for Light Up for Rare, dressing in zebra strips for Show Your Stripes, attending global Rare Disease Day events, meeting with legislators, and sharing stories on social media. One in ten, or over 30 million Americans are affected by rare diseases. Participation in Rare Disease Day events is a critical way to demonstrate the size and strength of the rare community and to push for progress toward a better future for patients and their families!

A growing and impactful event celebrating Rare Disease Day is the EveryLife Foundation's Rare Disease Week on Capitol Hill. Advocates come together in Washington, D.C. to connect, learn about issues impacting the rare disease community, and advocate for change. This year, it was held from February 24 to February 26, The FA community took advantage of Rare Disease Week and made their voices heard! FARA Ambassadors Darla Sparacino (Arkansas), Kelly Barendt (Ohio), Liam Kruesi (New Jersey), Noah Griffith (Alabama), and Alexis Baker (Tennessee), as well as FA mom Melinda Richard (Louisiana), joined FARA President Ron Bartek, FARA Director of Advocacy Brigid Brennan, and FARA Advocacy Program Coordinator Emma Potter for all of the Rare Disease Week events. Thank you to our advocates who came to D.C. to make sure that the FA community had a voice at this important event!

FA advocates kicked off the week with a reception and film screening about various rare diseases on Monday night before diving into a full-day legislative

conference on Tuesday in preparation for Capitol Hill meetings. At the conference, advocates met with other members of the rare community from their state, learned about various current policy issues impacting the rare disease community, and heard from experts about innovative initiatives and policies. Bright and early the next morning, they were off to Capitol Hill to attend a Congressional briefing and meet with their legislators. The FARA team joined almost 1,000 advocates in more than 300 meetings with members of Congress. They advocated for robust research funding, support for public health agencies, and several pieces of legislation, including the Give Kids a Chance Act (H.R. 1262, S. 932) which would reauthorize the Rare Pediatric Disease Priority Review Voucher program (PRV), and the Accelerating Kids' Access to Care Act (H.R. 1509). Additionally, over 10,000 members of the rare community signed a petition which was delivered to Congress on February 28 to urge robust funding and continued support for biomedical research and federal agencies.

A special Rare Disease Day shout out to FARA Ambassador Mary Nadon Scott who demonstrated that, even if you can't make it to D.C., you can make an impact! Mary scheduled meetings with the local offices of her two U.S. Senators and her US House Representative to update them on our issues. She also met with Vermont Governor Phil Scott who signed a proclamation declaring February 28 Rare Disease Day in the state of Vermont during their meeting!

Rare Disease Day was a wonderful opportunity to raise awareness of FA, but the work is not done! As you may have heard, there have been numerous changes to federal agencies and research funding recently. Now more than ever, your voice is needed. Sharing your story with lawmakers gives them direct

examples of the impact of those changes. You can do that in many ways from sending an email, stopping by their local office, or attending sponsored events. Also, stay tuned for future action alerts and advocacy opportunities shared by FARA.

Capitol Hill Updates

Help Ensure Crucial FA Research Funding Remains Available by Submitting CDMRP Appropriations Requests!

The Congressionally Directed Medical Research Program (CDMRP) is a crucial program run through the Department of Defense (DoD) that allows Congress to appropriate funding for research on conditions with significant unmet medical needs. In order to participate in the program, advocates must submit requests to their members of Congress each year requesting to be included on the list of qualifying conditions. FA has been included in the program since FY22 and, as a result, research funding for ataxia has doubled. This is a crucially important program to FA research!

FARA is currently working with community members to make these requests. Most members have a form on their website to complete and FARA has both a template to help you complete it and supporting documents to share with offices. If you would like to support this initiative, email Emma.potter@curefa.org.

Congress Passes Continuing Resolution (CR), Avoiding a Government Shutdown

Last week, Congress narrowly avoided a government shutdown by passing a third continuing resolution (CR) which extended federal funding until September 30, the end of FY25. On March 11, the U.S. House of Representatives passed their CR bill along party lines, and shortly after, on March 14, the Senate passed it in a 54-46 vote. Ten Democratic Senators joined their Republican colleagues in voting to pass the CR. The bill included cuts to some vital programs for the rare disease community. The Department of Defense's Congressionally Directed

Medical Research Program (CDMRP), a critical grant program to ataxia research, had its budget cut by 57%, with its funding dropping from $1.509 billion to $650 million. Some important federal agencies, like the NIH, also received budget cuts. In addition, key legislative provisions like the reauthorization of the Rare Pediatric Disease PRV Program were also excluded from the final CR.

Give Kids A Chance Act (H.R. 1262/S. 932) Reintroduced

On February 12, 2025, the Give Kids a Chance Act (H.R. 1262/S. 932), was reintroduced by Representatives McCaul (TX) and Dingell (MI). The Give Kids a Chance Act would expand access to life-saving therapies for children battling rare diseases as well as reauthorize the Rare Pediatric Review Voucher (PRV) program. The PRV program was created by the Creating Hope Act of 2012 to incentivize the development of drugs for rare diseases that affect children. Since then, the program has resulted in the development of 58 new treatments. Unfortunately, the PRV Program expired on December 20, 2024, and wasn't included in the most recent CR.

Accelerating Kids' Access to Care Act (H.R. 1509) Reintroduced

On February 21, Rep. Lori Trahan (MA-03) and Rep. Mariannette Miller-Meeks (IA-01) reintroduced the Accelerating Kids' Access to Care Act (H.R. 1509), which would make it easier for out-of-state pediatric providers to enroll as participating providers in state Medicaid programs. This action would facilitate pediatric patients receiving care out-of-state quicker, which is crucial for patients with rare diseases like FA that have a limited number of providers familiar with the condition and who tend to practice in a few select centers of excellence.

HELP Copays Act (S. 864) Introduced

The HELP Copays Act (S. 864) was introduced by Senators Roger Marshall and Tim Kaine and aims to ensure health plans count the value of drug copay assistance towards patient cost-sharing requirements and close a loophole regarding "non-essential" covered drugs. Insurance companies routinely penalize patients for using copay assistance programs, which are financial assistance programs from nonprofits or drug manufacturers to help lower out-of-pocket costs for patients, by preventing savings from copay assistance programs from counting towards deductibles and out-of-pocket maximums.

Orphan Cures Act (H.R. 946) Introduced

In early February, Representative John Joyce (PA-13) re-introduced the Orphan Cures Act as H.R 946. This bill would expand and clarify the Orphan Drug exclusion under the Drug Price Negotiation Program in order to incentivize research & development for drugs to treat rare diseases.

Congress Holds Hearings on Nominations for FDA & NIH Leadership

On March 5 and 6, the Senate Health, Energy, Labor and Pensions committee held two crucial hearings regarding the Trump Administration nominees for the NIH and FDA. Dr. Jay Bhattacharya, a Stanford professor of medicine and health economist, was nominated to be NIH Director, and Dr. Marty Makary, a Johns Hopkins surgeon, was selected for FDA Commissioner. These two roles are critical to research and drug development.

Both nominees are likely to be confirmed by the Senate.

Upcoming Advocacy Events

Oregon Lobby Day

Join NORD on Tuesday, April 22 from 9am to 4:30pm to advocate for legislation (HB 2457) that will establish a Rare Disease Advisory Council (RDAC). This is your chance to meet with legislators in-person in Salem and let them know an RDAC is needed in Oregon!

Location: State Library of Oregon, 250 Winter St. NE, Salem, OR 97301
Date: Tuesday, April 22
Time: 9am to 4:30pm

Register Now

Announcements

Apply for the Rare Advocacy Learning Program!

The seminar series will begin on April 21 and end on June 6.
Application closes March 21, 2025

The Rare Advocacy Learning program is a free, 6-week seminar series launched to provide in-depth education and advocacy training, developing a pathway toward year-round advocacy engagement. Advocates with prior advocacy experience are encouraged to apply. The Spring 2025 series is titled, “Understanding the Rare Disease Community’s Imperatives.”

Apply or Learn More

Are You a Student or Hoping to Return to School? Apply for the #RAREis Scholarship Fund!

Living with a rare disease means managing unique challenges, including frequent doctor visits, rigorous treatment regimens and hospitalizations, and exposure risks. While quality and duration of life continues to improve thanks to improved diagnosis and treatment approaches, individuals living with rare diseases still face disparities in achieving traditional life milestones. That’s why the EveryLife Foundation for Rare Diseases established the #RAREis Scholarship Fund – to enrich the lives of adults living with rare diseases by providing support for their educational pursuits.

Thanks to the support of Amgen, The EveryLife Foundation for Rare Diseases will provide one-time awards of $5,000 scholarships to 104 rare disease recipients in 2025.

Applications will be open from March 17 through April 28, 2025.

Apply Now

State News

On March 17, Gavin Lambert, who lives

with FA, and his mother Dawn testified before the Florida legislature in favor of a bill establishing a rare pediatric disease collaborative among Florida universities, including an emphasis on newborn screening.

Watch the Video

Richmond State Advocacy Day

On January 28 and 29, Emma Potter, FARA's Advocacy Program Coordinator, joined more than 20 rare disease patients in Richmond to advocate for crucial issues such as newborn screening RUSP alignment, prior authorization, and medical debt. Together, we held productive meetings with 45 members of the General Assembly.

Learn More

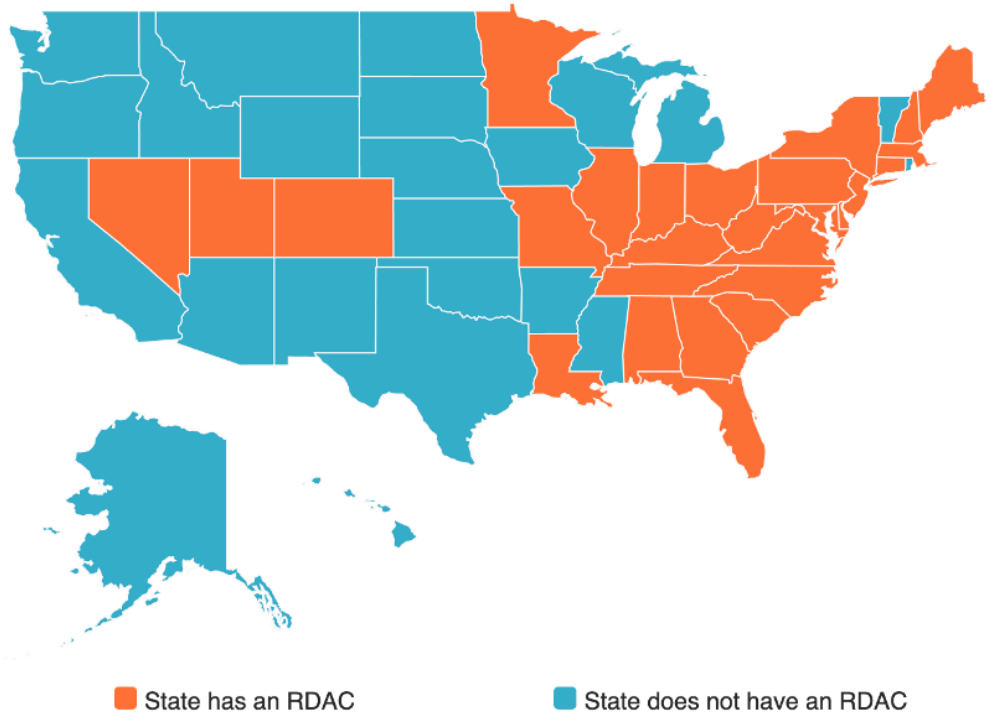
RUSP alignment Bill Passes Virginia House of Delegates

On January 31, Virginia House Bill 1782 passed in the House of Delegates, and it passed the Senate on February 21. This bill would ensure that the Commonwealth of Virginia screens newborns born in the state for all conditions on the Recommended Uniform Screening Panel (RUSP). The bill is now waiting to be signed into law by the Governor. The deadline for Governor action is March 24, meaning if the bill isn't signed into law by the 24th, it will become law without signature.

Learn More

Arkansas Passes Bill to Join the Interstate Medical Licensure Compact (IMLC)Arkansas Passes Bill to Join the Interstate Medical Licensure Compact (IMLC)

On March 10, the Arkansas legislature passed SB 119, and the bill has been transmitted to Governor Sanders for her consideration. This bill, if enacted, would make Arkansas the 41st state to join the Interstate Medical Licensure Compact (IMLC), which provides an expedited pathway for physicians to become licensed in multiple states and expands access to expert care for patients with rare diseases.



Upcoming Rare Disease Advisory Council (RDAC) Meetings

Colorado: The Colorado RDAC is meeting virtually on **Monday, April 14 from 9:00 a.m. – 12:00 p.m.** Additional meeting information can be found **here**.

Nevada: The Nevada RDAC meets on the **first Friday of even-numbered months at 9:30 a.m.** For more information, **click here**.

Tennessee: The Tennessee RDAC meets on the **fourth Wednesday of every other month 8:00 a.m. to 9:30 a.m. CST**. If you are interested in joining the meeting, please email info@tnrdac.org for instructions on attending. Additional meeting information can be found **here**.

Rhode Island: Join the coalition of rare disease patients, experts, and

advocates to encourage the creation of an RDAC in Rhode Island. The next coalition meeting is being held virtually on **Thursday, March 20 from 6:30 p.m. to 7:15 p.m. ET.** Please email csheridan@rarediseases.org to request the link for the Rhode Island RDAC Coalition meeting.

Friedreich's Ataxia Research Alliance | 533 W Uwchlan Ave | Downingtown, PA 19335 US

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