FARA Friedreich's Ataxia Research Alliance

2025 In Review

2025 brought major changes to federal agency funding and structure, but FA advocates rose to each challenge and made their voices heard throughout the year. Let's take a look back at some of the **major events of 2025** and how the FA community helped shape the narrative.

Rare Disease Week on Capitol Hill
FA advocates joined nearly 1,000 advocates at Rare Disease Week in Washington D.C. in February. The event included over 300 Congressional meetings and included advocates from all 50 states, plus D.C., Puerto Rico, and American Samoa. FARA Ambassadors Darla Sparacino (Arkansas), Kelly Barendt (Ohio), Liam Kruesi (New Jersey), Noah Griffith (Alabama), and Alexis Baker (Tennessee), as well as FA parent Melinda Richard (Louisiana), joined FARA President Ron Bartek, FARA Director of Advocacy Brigid Brennan, and FARA Advocacy Program Coordinator Emma Potter in D.C. to

make sure that the FA community had a voice at this important event!

The Congressionally Directed Medical Research Programs (CDMRP) have been a game changer for ataxia research since it was added to the program in Fiscal Year 2022.

So, when Congress cut funding for this program by 57%, FARA joined hundreds of other groups to educate lawmakers on the importance of the CDMRP and advocated for restored funding. FA community members were instrumental in collaborating with FARA to submit appropriations requests to their members of Congress requesting that “hereditary ataxia” remain on the list of eligible conditions for FY26. This effort ensures that our researchers continue to have access to this vital source of funding.

FARA and the National Ataxia Foundation (NAF) once again partnered to host the 7th Annual United Against Ataxia Hill Day.
The virtual Hill Day garnered a record-breaking 169 advocates from 38 states, and participants attended 99 Congressional meetings where they urged Congress to adequately fund the FDA and NIH, reauthorize the Rare Pediatric Disease Priority Review Voucher (PRV) program, pass the Accelerating Kids’ Access to Care Act (H.R. 1509/S. 752), continue including “hereditary ataxia” in the CDMRP, restore funding for CDMRP, and ensure the integrity of the NIH peer-review process.

The community's asks were well-received, and advocates fostered important relationships with new offices that will support meaningful changes for patients and families affected by FA.

Senate Recognizes September 25 as National Ataxia Awareness Day

The [2025 National Ataxia Awareness Day Resolution (S.Res. 447)] passed the U.S. Senate with unanimous consent!

This resolution, led by Senator Cindy Hyde-Smith (MS) and co-sponsored by Senators Murphy (CT) and Capito (WV), designates September 25, 2025, as National Ataxia Awareness Day, helping to elevate national recognition and understanding of Ataxia. Each year, NAF and the FARA, along with dedicated advocates, work with members of Congress to make this possible.

FARA Releases New Advocacy Materials & Resources

FARA recognizes that our advocates are the building blocks of the FARA advocacy team! This year FARA developed two new resources for the community — the [FA & Speech Toolkit], a selection of resources related to public speaking and how FA affects speech, and the [Proclamation Toolkit], a guide on how to submit a proclamation request to a state or local government. The Proclamation Toolkit was inspired and guided by the work of FARA Ambassador, Mary Nadon Scott.

FARA Continues to be a Strong Presence in the Rare Disease Community

FARA staff participated in a number of events hosted by advocacy groups and other stakeholders, including the Alliance for Regenerative Medicine's Congressional Fly-In, the NORD Breakthrough Summit, the BIO Patient Summit, the inaugural Galien Patient Summit, the Academy of Managed Care Pharmacy (AMCP) Nexus Conference, and more! Beyond simple participation, FARA staff took every opportunity to raise awareness for FA and the community's needs. Ron Bartek spoke at the NORD Breakthrough Summit, where he attested to the urgent need for pediatric inclusion in clinical trials to expedite treatments for pediatric patients. FARA Director of Advocacy, Brigid Brennan spoke on critical policy issues and the need to include the patient voice during the EveryLife Foundation Share Your Story webinar and the AMCP Conference.

FA Advocates Also Impact State Policy

The FA community also found ways to raise awareness of FA by engaging in state advocacy. This included meeting with their state and local legislators, requesting proclamations for FA or rare disease awareness days, providing testimony at state legislative sessions or to Medicaid boards, serving on advisory boards or committees, and writing blogs or letters to the editor of local papers. What outstanding representation!

2025 was a challenging year for advocacy but the FA community was not deterred! By sharing their lived experience, they offered perspective on critical issues and often were the catalyst needed for action. FARA is grateful for the incredible commitment shown by all, and look forward to continuing to grow this momentum in 2026!

Capitol Hill Updates

The Federal Government Reopens After 43 Days

The government shutdown officially ended on Wednesday, November 12 after President Trump signed a bipartisan continuing resolution (CR) that would extend funding until January 31, 2026.The shutdown lasted 43 days, making it the longest in U.S. history. The federal government can now resume awarding new NIH grants and assessing New Drug Applications (NDAs). Congress must pass a full budget before the January 2026 deadline to avoid another shutdown.

CDMRP Briefing Held

On Tuesday, November 18, the Defense Health Research Consortium held a Congressional briefing on the Congressionally Directed Medical Research Programs (CDMRP).

The Defense Health Agency, represented by Col. Mark Hartell, Dr. Becky Fisher, and Dr. Kathy Lidle, discussed the importance and success of the CDMRP program, as well as the impact that reduced funding has had. Hereditary Ataxia was added to the CDMRP in FY22 as a result of the hard work of our advocates. This action resulted in many ataxia research grant awards, totaling over $35M so far.

FDA Outlines New “Plausible Mechanism” Pathway

On November 12, FDA Commissioner, Marty Makary, and CBER Director, Vinay Prasad, unveiled a new regulatory pathway aimed at expediting therapies for rare disorders in an article in the New England Journal of Medicine.

The new framework is meant to target products for which a randomized trial is not feasible, with an initial emphasis on cell and gene therapies. The “Plausible Mechanism” Pathway would allow manufacturers who have successfully treated several patients with bespoke (or individualized) therapies to leverage platform data for approval of similar treatments in other conditions. There is no official regulatory guidance as to how this new pathway will be implemented.

FDA Releases Draft Guidance on Reducing Testing on Non-Human Primates for Monoclonal Antibodies

On December 2, the FDA issued draft guidance outlining specific product types for which the FDA believes six-month non-human primate toxicity testing can be eliminated or reduced. In lieu of animal testing, the FDA is incorporating risk assessments that integrate human-relevant models — including computational toxicology, organoid systems, and real-world human safety data — into regulatory decision-making.

In just over a month, CDER Director George Tidmarsh was replaced by Dr. Richard Pazdur who was then replaced by Dr. Tracy Beth Hoeg

CDER Director George Tidmarsh resigned on November 2. On November 11, the FDA announced that it had appointed Dr. Richard Pazdur as the new CDER Director. Dr. Pazdur is a longtime veteran of the FDA where he is credited with serving as the founding director of its Oncology Center of Excellence.

On December 2, Dr. Pazdur announced that he will be leaving the FDA. On December 7, Dr Tracy Beth Hoeg, was appointed as director. Dr. Hoeg is the fifth CDER director appointed in 2025.

Upcoming Advocacy Events

Register Now: Rare Disease Day 2026

FDA will host Rare Disease Day, a virtual public meeting, on Monday, February 23, 2026 in global observance of Rare Disease Week. The theme is: “Moving Forward. Looking Ahead. An Event for Patients.” The goal of this year’s Rare Disease Day is to explore ways to engage and collaborate with patients and their communities to

support and accelerate the development of medical products for rare diseases. We want patients to connect with FDA and stay involved with our outreach and engagement programs to ensure the patient voice informs medical product development and regulation throughout FDA.

Panels will discuss:

- Patient Focused FDA Initiatives
- Patient Engagement Opportunities
- Addressing Challenges and Opportunities with AI Technology
- Utilizing Real World Data and Real World Evidence at FDA

Register Now

Nov. 24, 2025

Submit Your Artwork by Jan. 2 for the Rare Diseases Are Not Rare! 2025 Challenge Prize Competition

The deadline for the third iteration of the Rare Diseases Are Not Rare! 2025 Challenge Prize Competition is quickly approaching on Jan. 2, 2026. We are looking for creative, original digital communications and artwork that can be easily shared to:

- raise awareness of rare diseases
- highlight the people they impact
- emphasize the need for research and development of treatment strategies that address multiple rare diseases at a time.

Learn more about the **Challenge Prize Competition requirements** and view winners and honorable mentions from the **2018** and **2020** Challenges.

Rare Diseases Are Not Rare! 2025 Challenge Prize Competition *(NCATS)*

Learn More

Announcements

FARA & NAF Announce Senators Collins and Mullin as 2025 ARC Award Recipients

In November, the NAF and FARA jointly presented this year's Ataxia Research Champion (ARC) Awards to Sen. Susan Collins (R-ME) and Sen. Markwayne Mullin (R-OK). The ARC Awards are presented to members of Congress whose work has specifically addressed the needs of the ataxia

community. This year's awards were presented in-person on Capitol Hill alongside notes and stories from community members. A special thank you to the advocates that were willing to attend or write letters conveying the importance of policy to the FA community.

New Analysis Shows Many Clinical Trials Have Been Affected By NIH Funding Cuts

A new study published in JAMA sought to quantify the impacts of recent NIH funding cuts and grant terminations on clinical trials. The authors of the study found that grant terminations disrupted 1 in 30 clinical trials and affected approximately 74,000 trial participants. These results raise major concerns around the long-term impacts of terminations on research and development and the patients that rely on clinical trials.

State News

MJFF joins Pennsylvania Representative Kyle Mullins (D-112) and advocates to announce a new effort to secure state funding for neurodegenerative disease research.

Several States Announce Additional Investments In Neuroscience Research

In the face of challenges regarding federal research funding, state advocates and legislators have taken action to fund critical neuroscience research. Both Texas and Pennsylvania recently announced historic investments in neuroscience research in their states. In Texas, voters approved Proposition 14, a ballot initiative that authorizes $3B in funding for brain disease research over the next several years. The initiative was set into motion when the Texas state legislature passed Senate Bill 5, which would create a research funding program for dementias and other related diseases. After its passage, Texas Governor, Greg Abbott, signed the bill into law, sending it to voters to authorize. The initiative cleared its final hurdle this past election day and can now begin to take effect. Funding associated with Proposition 14 will total $300M each year, and 95% of the funds are required to go directly to research.

Through a different approach, Pennsylvania state legislators appropriated $5M in the state's annual budget for neurodegenerative disease research. In addition to the authorization of research funding, the state budget also authorizes the creation of the Neurodegenerative Disease Research Program, a new program operated under the Pennsylvania Department of Health that will administer the appropriated research funding.

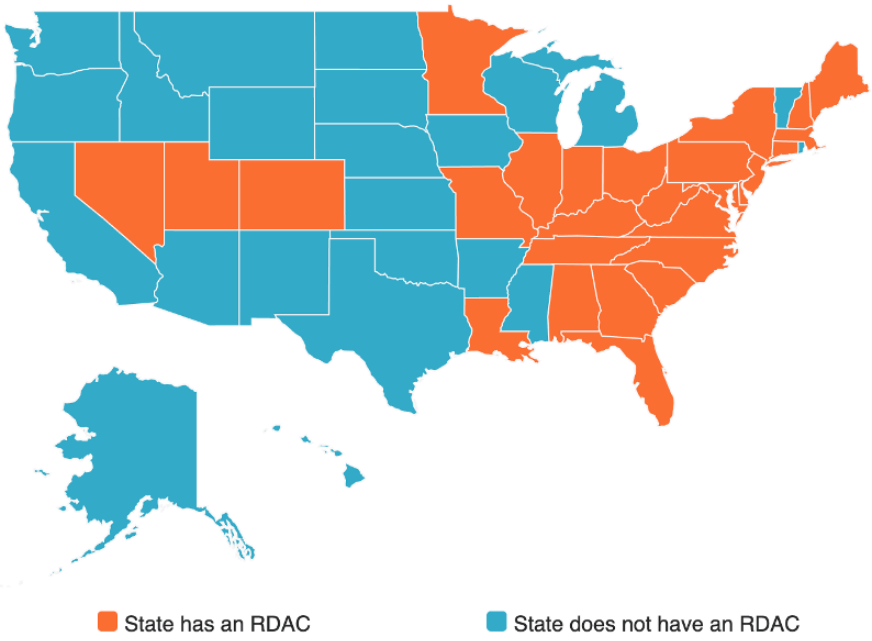
Join Your State's Medicaid Beneficiary Advisory Council!

Numerous states are currently accepting applications for their Medicaid Beneficiary Advisory Councils (BAC), and this is a great opportunity to make your voice heard and advocate for patients and families in your state. Members of these advisory councils will influence Medicaid policy and program implementation, including ways to ensure that patients have access to providers and are able to navigate upcoming Medicaid changes mandated by the One Big Beautiful Bill Act. The time commitment should be minimal, and some states offer modest compensation for advisory council members. Applications are often short and simple to complete.
If you are a current or former Medicaid beneficiary or caregiver of a Medicaid beneficiary and reside in one of the states below, consider applying and helping ensure that Medicaid best serves the FA community.

- Connecticut is now accepting applications with no deadline indicated.
- Florida is now accepting applications.
- Maryland is accepting applications via email with no specific deadline. For more information, please email mdh.mmacbac@maryland.gov.
- Montana is accepting applications with no specific deadline.
- Ohio is accepting applications with no deadline.
- Washington is accepting applications by email at ahcommunications@hca.wa.gov. Include your name, client ID, contact information, and your preferred time to be contacted in the email.

States that will open applications soon:

- Idaho and New York will launch applications in the coming months.
- Hawaii and North Carolina will begin a new round of applications in the fall.



Upcoming Rare Disease Advisory Council (RDAC) Meetings

- **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**.
- **Connecticut:** The Connecticut RDAC meets on the fourth Tuesday of every month from 2:00 p.m. to 3:00 p.m. ET, unless otherwise noted. To join the monthly meeting or see other events held by the Connecticut RDAC, click **here**.
- **Maryland:** The Maryland RDAC meets on the second Tuesday of every month from 4:00 p.m. to 5:00 p.m. Additional meeting information can be found **here**.

Update Us On Your Advocacy!

Have you engaged in advocacy recently? Met with a federal, state or local lawmaker? Participated in a public meeting or wrote about FA or rare disease? FARA would love to share the amazing advocacy work our community members are doing. So, please let FARA know by sending updates to Brigid Brennan, brigid.brennan@curefa.org.

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