FARA Friedreich's Ataxia Research Alliance

RARE DISEASE DAY®

Rare Disease Day 2025

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. 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 need of this community.

Participation in Rare Disease Day events is crucially important to raising awareness of FA, advancing research, and supporting access for the FA community. By raising our collective voice, FA advocates have successfully doubled the funding for FA research through the Congressionally Directed Medical Research Program (CDMRP), helped pass numerous bills that expedite research, called for adequate funding and increased flexibility for the FDA and NIH, and more. However, there is still more work that needs to be done. Many crucial initiatives, such as the Rare Pediatric Disease Priority Review Voucher program and the Accelerating Kids' Access to Care Act, were not passed before the end of the 118th Congress in December even with strong bipartisan support. The rare disease community will now need to start over in educating and engaging Congressional Members of the 119th Congress, 77 of which are new to the job! In addition, with the new Congress we welcome new chairs and members of the Finance, Energy and Commerce, Health, Education, Labor and Pensions, and Ways and Means Committees, who will lead work on rare disease related legislation. Sharing your life experiences matters now more than ever to get important initiatives like these across the finish line!

With a new Congressional session starting, it is the perfect time to reach out to your legislators. Members of Congress are especially receptive to meetings with constituents and, as many have never heard of FA before, sharing your story

would give them the information needed on how to help. Your life experiences could be the reason that one of your representatives prioritizes rare disease legislation or votes “yes” on one of our initiatives. Every voice matters, and Rare Disease Day is the perfect opportunity join the conversation!

This year Rare Disease Day is February 28, 2025, but organizations are celebrating throughout the month! Below is an amazing list of events that you can participate in to help raise awareness of rare disease in general, and FA specifically! Don’t let this Rare Disease Day pass without you!

Rare Disease Day Events

Rare Disease Week on Capitol Hill

Join FARA and the EveryLife Foundation **in Washington D.C.** from **Monday, February 24 to Wednesday, February 26** for a chance to engage with the rare community, learn about legislative priorities, and share your story with your Members of Congress! The event is free to attend, and EveryLife will coordinate all training, meetings, and events for you. Travel and lodging are your responsibility.

- **Monday, February 24:** Documentary screening and a reception

While this event is not mandatory, it is a lot of fun to attend! The screening and reception is a great way to meet other rare disease advocates and learn more about some of the important work happening in the rare community!

- **Tuesday, February 25:** Legislative Conference

This event will feature a number of policy experts who will run through current legislative priorities for the rare community, help you understand the legislative asks for the meetings on Capitol Hill the following day, and provide tips and tricks for meeting with your legislators. During this event, you’ll also meet other advocates from your district who will join you in the meetings and coordinate logistics for the next day’s activities. ***Attendance at the legislative conference is mandatory if you want to attend Congressional meetings.***

- **Wednesday, February 26:** Meetings with your Members of Congress

For the final day, you will head to Capitol Hill with your team of advocates to meet with your legislators! You will have a series of meetings with Representatives and Senators from your state. You will be with other advocates for all meetings. EveryLife will provide you with a map, schedule, and one-pagers on the legislative asks so that you can navigate the day with ease.

EveryLife will also provide a series of webinars and office hours prior to the event to help provide additional information and allow you practice before you arrive in D.C. You can find the dates and times for webinars and office hours here.

This event is a great opportunity to see democracy in action! Join the hundreds of rare disease advocates coming to DC to advocate for policies that support research, treatments, and access.

Registration is open NOW!

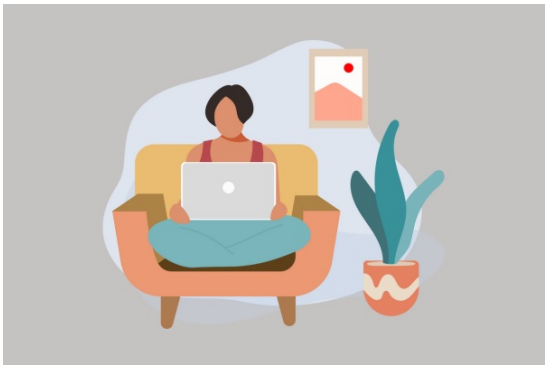
The FDA and NIH will be holding a joint event for Rare Disease Day from **Thursday, February 27 and Friday, February 28.** This event is free to attend and has both an **in-person and virtual** option, allowing you to attend even if you can't make it to DC! This event will feature patient stories, highlight recent rare disease research supported by the FDA and NIH, and provide additional updates to the rare disease community.

Register Now

If you can't make it to DC for Rare Disease Week this year, there are a number of other events happening across the country, including:

- **Goodwin's Annual Rare Disease Symposium**, February 5, 2025, in Boston, Massachusetts
- **Rare Disease Symposium**, February 20, 2025, at Washington University Medical Center in St. Louis, Missouri, or via Zoom
- **Oklahoma Rare Disease Day**, February 20, 2025, at the Oklahoma State Capitol in Oklahoma City
- **The Boston Globe's Rare Disease Summit**, February 25, 2025, at Big Night Live in Boston, Massachusetts
- **Albany Rare Disease Day**, February 25, 2025, at the New York State Capitol in Albany, New York
- **CHOC & UCI Rare Disease Symposium & Family Conference**, February 26, 2025, and February 28, 2025, at the Beckman Center in Irvine, California
- **Rare Disease Day 2025 at the University of Minnesota**, March 6, 2025, at the McNamara Alumni Center in Minneapolis, Minnesota

NORD also has an interactive page listing local Rare Disease Day eventshere.



Rare Disease Activities from Home!

If you cannot travel, there are still plenty of ways that you can get involved at home!

Contact Your Legislators!

Contacting your Members and sharing your story can still be incredibly impactful even if shared via email or phone. First, look up your legislators here by entering your home address. Then, contact them through their websites. Here is a template to use when reaching out to your Members:

> Hello Rep./Sen. [Insert name of Representative/Senator],
>
> My name is [YOUR NAME], and I am your constituent from [CITY, STATE]. I am reaching out to you today to share my story with rare disease and encourage you to take action to support the needs of the rare community. I have a rare disease called Friedreich's Ataxia (FA), which is

one of over 70+ forms of heredity ataxia. FA is a rare degenerative neuromuscular disorder that affects my mobility, coordination of movement, speech, and more including premature death from cardiomyopathy.

[You can insert more of your personal story here to help educate your legislators!]

There are over 10,000 known rare diseases, of which 95% have no approved treatments. Hereditary ataxias are no exception. As of now, there are no treatments addressing the root cause of the disorder, and the treatments available only help to manage symptoms and/or slow progression. This is why adequate funding for agencies like the NIH and FDA is so crucial. Funding these agencies helps accelerate potential treatments and gives patients and families hope. I respectfully request that you do all that you can to pass the FY25 budget by the March 14 deadline. Agencies cannot recruit or retain the best doctors and scientists when funding is not robust or timely.

I also ask that you work to reinstate the Rare Pediatric Disease Priority Review Voucher (PRV) Program. This program serves as a crucial incentive for drug development for pediatric-onset rare diseases like FA. With over 70% of rare diseases presenting in childhood and 30% of those children dying before the age of five, we need more incentives for pediatric drug development, not less. Moreover, the program presents no cost to taxpayers, making it a budget-neutral program with monumental impact.

Thank you for your time and consideration, and please don't hesitate to reach out with any additional questions.

Best,
[YOUR NAME]

NORD®
National Organization for Rare Disorders

NORD is running the following Rare Disease Day programs. Get creative! Have fun!

Raise Awareness and Show Your Stripes®
Show your support for Rare Disease Day and raise awareness for rare disease by decorating your home, neighborhood, school, or town with zebra stripes. Click here for more information on how you can Show Your Stripes® this Rare Disease Day.

If you are interested in free promotional materials and other Rare Disease Day activities, you can find those here.

Light Up for Rare on Rare Disease Day®
Light Up for Rare® is an easy way to get involved in rare disease day in your community. Typically, you will need to get in touch with the landmark and may have to fill out a request form. It's that easy to raise awareness for rare disease!

Global Chain of Lights
In an effort of global solidarity, you are invited **to light or decorate your home** with the Rare Disease Day colours at **7 PM your local time on 28 February 2025**.
You can use garlands, social media filters, candles, disco lamps, colorful decorations… Let your creativity shine!
If you choose to participate, take a picture of yourself with the illuminations or decorations you chose and post it on your social media using a **"Global Chain of Lights" filter** and **#rarediseaseday**.

Raise Awareness on Social Media
There are so many ways to raise awareness about rare disease on social media! This could include:

- Sharing your rare disease story
- Posting a selfie in stripes and encouraging your friends and family to do the same
- Repost infographics and photos from other members of the rare disease community
- Or making other posts about rare disease and FA!

If you intend to raise awareness on social media, make sure to use **#rarediseaseday** in your post! You can find more information and templates for posts in this social media toolkit!

For more information on how you can get involved on Rare Disease Day and additional resources to help you get started, take a look at NORD's Rare Disease Day Community Playbook.

Capitol Hill Updates

Congress Passes Continuing Resolution

On December 20, 2024, Congress prevented a government shutdown by passing a second continuing resolution extending federal funding until March 14, 2025. While this continuing resolution reauthorized many programs and authorized new funding for things like disaster relief, it notably excluded several crucial programs, including the Pediatric Priority Review Voucher (PRV) Program, which has been a focal point of FARA's advocacy over the last year. In 2025, FARA will continue to advocate for the reauthorization of PRV. Your advocacy matters now more than ever to ensure that this crucial program continues to bring treatments to the rare disease community!

On January 3, 2025, the 119th Congress was officially sworn in, signaling the beginning of the new session which will last for the next two years. Among the ranks of the 119th Congress, there are 77 new Members. Overall, the 119th Congress will have a split of 220 Republicans and 215 Democrats in the House and a split of 53 Republicans and 47 Democrats in the Senate.
For more information on the 119th Congress, visit thisCBS news article.

Rep. Mike Johnson Re-Elected as the Speaker of the House

On January 4, Rep. Mike Johnson was re-elected as Speaker of the House, allowing him to retain his position into the 119th Congress. The Speaker of the House is the most important position within the House of Representatives, responsible for giving permission to speak on the House floor, counting and declaring votes,

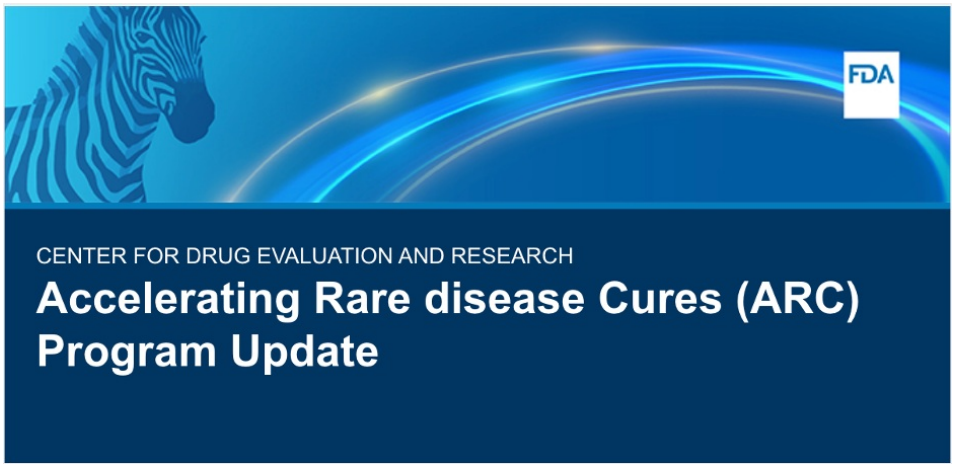
appointing Members to committees, sending House bills to committees, and signing all bills and resolutions that pass the House. Therefore, the Speaker has a good deal of discretion over the agenda in the House during a given session.

Announcements

Senators Bob Casey Jr. and Amy Klobuchar Receive the 2024 ARC Award

The National Ataxia Foundation (NAF) and the Friedreich's Ataxia Research Alliance (FARA) have jointly awarded the Ataxia Research Champion (ARC) Award to **Senators Amy Klobuchar (MN) and Bob Casey (PA)**.

This award signifies our heartfelt appreciation for their continuous support of individuals affected by Ataxia. Their ongoing commitment to raise awareness of this devastating disease and the unmet medical need of the Ataxia community is commendable. Congratulations to both Senators on receiving the ARC Award!



The FDA's Rare Disease Innovation Hub (the Hub) released its Strategic

Agenda, which outlines the actions the Hub plans to undertake during its first year with extensive involvement from the rare disease community, while also addressing questions about the ultimate structure and programs of the Hub. The Strategic Agenda reflects extensive input from and partnership with the larger rare disease community, and identifies the following goals for the Hub:

- Goal 1 – Further Advance Regulatory Science of Rare Disease Therapies
- Goal 2 – Enhance and Strengthen Coordination and Alignment Between FDA's Medical Product Centers, with Particular Focus on the Center for Biologics Evaluation and Research (CBER) and the Center for Drug Evaluation and Research (CDER)
- Goal 3 – Create a Centralized Point of Contact for External Partners

It is the hope and expectation of FDA leadership and the Hub that this Agenda will evolve as the Hub evolves. It is also the expectation that the rare disease community will remain heavily engaged in this evolution. Throughout the year, there will be multiple ways for the community to engage with and offer suggestions to the Hub, both for immediate use and for consideration for the Hub's 2026 Strategic Agenda.

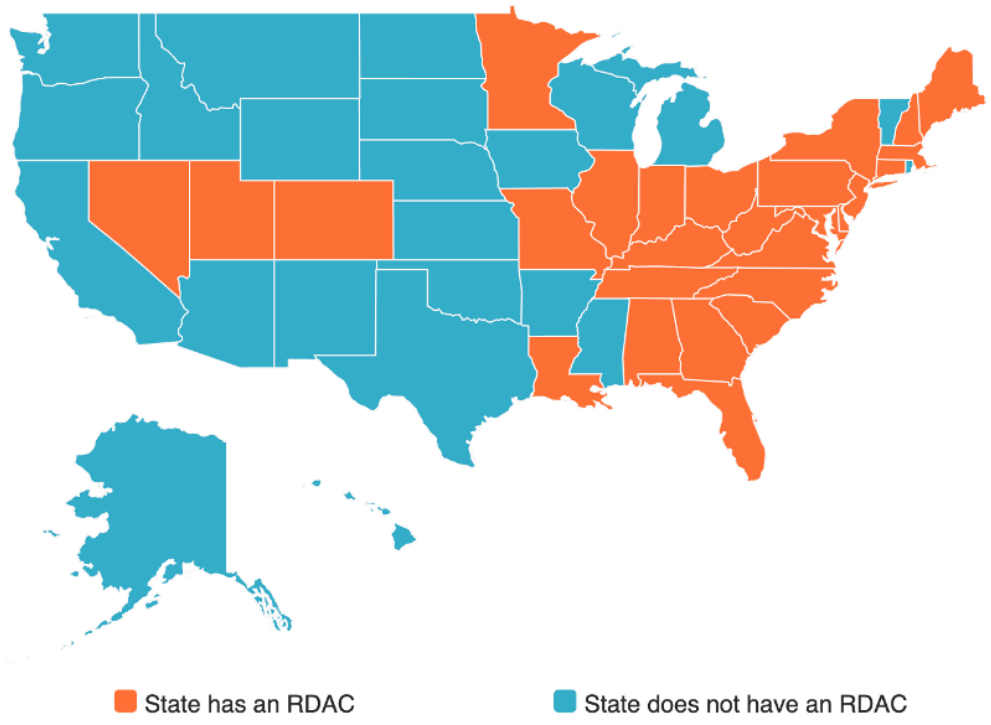
ARPA-H announces new program to accelerate rare disease diagnosis

ARPA-H has launched the RAPID program to revolutionize rare disease diagnosis using AI-enabled tools, aiming to reduce the multi-year diagnostic journey to months or even days through advanced data integration and innovative detection models. Find more information here.

CFPB Final Rule Prevents Medical Debt from Affecting Credit Reports

On January 7, 2025, the Consumer Financial Protection Bureau (CFPB) finalized a rule that will remove an estimated $49 billion from Americans' credit reports, impacting approximately 15 million Americans. The rule also bans the inclusion of medical debt in subsequent credit reports used by lenders and prohibits lenders from using medical information when rendering decisions. To read more about the final rule, you can visit the CFPB announcement.

State News



Upcoming Rare Disease Advisory Council (RDAC) Meetings

Colorado: The Colorado RDAC is meeting virtually on Tuesday, February 11 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**.

Vermont: Join the coalition of rare disease patients, experts and advocates to encourage the creation of an RDAC in Vermont. The next coalition meeting is being held virtually on Thursday, February 13 from 6:00 p.m. to 6:45 p.m. ET. Please email rdac@rarediseases.org to request the link for the Vermont RDAC Coalition meeting.