FARA Friedreich's Ataxia Research Alliance


# The 6th United Against Ataxia Hill Day Was a Huge Success Thanks to Our Advocates!


On September 25, 2024, which was National Ataxia Awareness Day, members of the Ataxia community came together from across the U.S. to participate in the 6th Annual United Against Ataxia Hill Day. In all, we had **113 advocates** from **32 states** participate in **94 Congressional meetings** where they advocated for funding and legislation that would help support patients and families impacted by ataxia.

Advocates urged action, and Congress heard their voice, on several important issues including:

· **The continued inclusion of "Hereditary Ataxia" in the Peer Reviewed Medical Research Program (PRMRP), within the Congressionally Directed Medical Research Programs (CDMRP).** This program has been a game changer for ataxia research generating over $25 million in funding for ataxia research in FY22 & FY23. FY24 results have started to be announced, and two "Hereditary Ataxia" grants have been recommended for funding so far totaling over $2 million. To remain on the CDMRP/PRMRP list, advocates need to make a request to Congress each year during the budget process. NAF and FARA, with the help of many advocates, did that in the spring and "Hereditary Ataxia" is on the draft list for FY25! Now, we just need Congress to pass the budget!

· **Pass the FY25 budget with robust funding for the NIH & FDA.** Timely passage of the federal budget allows these critical research institutions to develop programs, recruit leading scientists, and expedite research. Repeated delays and threats of government shutdown leave these agencies scrambling to respond to financial insecurity instead of focusing on bringing potential treatments to patients as soon as possible.

· **Pass the Creating Hope Reauthorization Act (H.R. 7384 / S. 4583).** This bill

would continue the Pediatric Priority Review Vouchers (PRV) Program, an important incentive for pediatric drug development. It was incorporated into Give Kids a Chance Act (H.R. 3433) the day before our Hill Day. Congress included an extension of this program until Dec 20 when they passed the Continuing Resolution (CR) on September 25.

· **Pass the Accelerating Kids' Access to Care Act (H.R. 4758 / S. 2372).** This bill would provide a streamlined process for pediatric providers to enroll in out-of-state Medicaid programs, improving access to out-of-state care for patients. It passed unanimously in the House on September 17. No Senate action has occurred yet.

· **Pass the RARE Act (S. 1214 / H.R. 7383).** The RARE Act would codify the FDA practice of recognizing exclusivity on approval which would incentivize companies to continue investing in therapeutics across the disease population. This bill was incorporated into the Give Kids a Chance Act (H.R. 3433) and passed the House on September 24. No Senate action yet.

· **Pass the BENEFIT Act (S. 526 / H.R. 1092).** This bill would ensure that the FDA considers patient perspectives when performing risk/benefit assessments for proposed products. No action yet in either the House or Senate.

The success of these meetings was a direct result of the time and commitment our advocates took to prepare and deliver the message. By sharing their lived experiences, Congressional Members and their staffers gained a perspective on ataxia and the high unmet needs of our community. Thank you to all who participated! Your voice brought this action!

But our work is not done yet. Congress will return to work shortly after the 2024 election and will need to address the FY25 budget before December 20 when the CR expires. Additionally, deliberation on a number of these bills could happen before the end of the term on January 3, 2025. Keep an eye out for advocacy opportunities in the coming weeks!

## Capitol Hill Updates

## Congress Passes a Continuing Resolution (CR), Avoiding a Government Shutdown until December 20

Every year, Congress must pass the federal budget before the fiscal year ends on September 30. Without a federal budget, the government is forced to shut down, threatening progress being made on federal research priorities, drug approvals, and more. If the government cannot decide on a budget, they are able to pass something called a continuing resolution (CR), which instead maintains current funding levels until Congress can agree on a full federal budget.

On September 25, Congress passed a CR to avoid a government shutdown. While funding is available for now, it is crucial that Congress pass a budget by December 20, when the CR is set to expire. If they are unable to do so, they can extend the CR or allow the CR to expire which would lead to a government shutdown. Failure to pass a timely budget causes significant setback to crucial research, drug approvals, and other programs that help the rare disease community.

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## FDA Creates a Rare Disease Innovation Hub and Announces Inaugural Director

On October 16, the FDA hosted a public meeting titled, “Advancing Rare Disease Therapies Through an FDA Rare Disease Innovation Hub.” The purpose of the public meeting was to discuss the establishment of a Rare Disease Innovation Hub, which will enhance collaboration and cooperation across the Center for Biologics Evaluation and Research (CBER) and the Center for Drug Evaluation and Research (CDER), as well as other centers and offices across FDA, to advance rare disease therapies. FARA was in attendance and provided input on how the Innovation Hub can best support the FA community. You can read FARA’s public comment here.

On November 1, the FDA announced Amy Comstock Rick, JD as the inaugural Director of the new Rare Disease Innovation Hub. Ms. Rick has over 20 years of experience and a strong track record of working closely with the rare disease community on various health policy issues, primarily focusing on issues pertaining to the FDA. She will be joining Dr. Peter Marks, CBER, and Dr. Patrizia Cavazzoni, CDER to lead the Hub. For more information about the Rare Disease Innovation Hub or Amy Comstock Rick, click here.

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## FDA Appoints New Head of the CDRH

The FDA announced that it has appointed Dr. Michelle Tarver to head its division that oversees medical devices, the Center for Devices and Radiological Health (CDRH).

## Upcoming Advocacy Events


### Apply for Travel Reimbursement for 2025 Rare Disease Week in Washington, D.C.!

Rare Disease Week will bring rare disease advocates together on February 24-26, 2025 to make our voices heard by our Members of Congress. If you or a family member would like financial assistance to attend Rare Disease Week on Capitol Hill, the EveryLife Foundation for Rare Diseases, is offering a limited number of travel reimbursements.

The reimbursements are offered to offset the costs of travel and lodging. Your geographic location determines the amount you are eligible to receive. If you receive a travel reimbursement, you will still be responsible for booking your own travel and lodging. After doing so, you will share your receipts with the EveryLife team to be reimbursed while in Washington D.C. for Rare Disease Week.

For more information and to apply for a travel reimbursement, please click the button below.

**Applications for funding through EveryLife will close Friday, November 8.**

**Apply for a Rare Disease Week Travel Reimbursement**

# Announcements

## FARA President Ron Bartek serves on National Academies Panel offering Recommendations on Rare Disease Drugs

Congress called on the FDA to sponsor a National Academies of Sciences, Engineering, and Medicine (National Academies) study on processes for evaluating the safety and efficacy of drugs for rare diseases or conditions in the United States and the European Union. The resulting report, "Regulatory Processes for Rare Disease Drugs in the United States and European Union: Flexibilities and Collaborative Opportunities," recommends approaches for increasing engagement with people affected by a rare disease, advancing regulatory science, and improving information sharing and collaboration.

## National Academies Newborn Screening Study Releases Engagement Summary

The National Academies has also been working on a study examining the current landscape of newborn screening systems, processes, and research in the United States. As part of their efforts, the National Academies invited members of the rare disease community to provide feedback to the study committee through online listening sessions and an online questionnaire. More than 600 people participated in their engagement efforts, sharing their views on how to strengthen newborn screening programs and improve them in the future. The National Academies released a paper detailing their findings from the listening sessions and questionnaire in a paper titled, "What We Heard: Engagement Summary on Newborn Screening in the United States."

# State News

## FARA Joins the PA State Advocacy Day

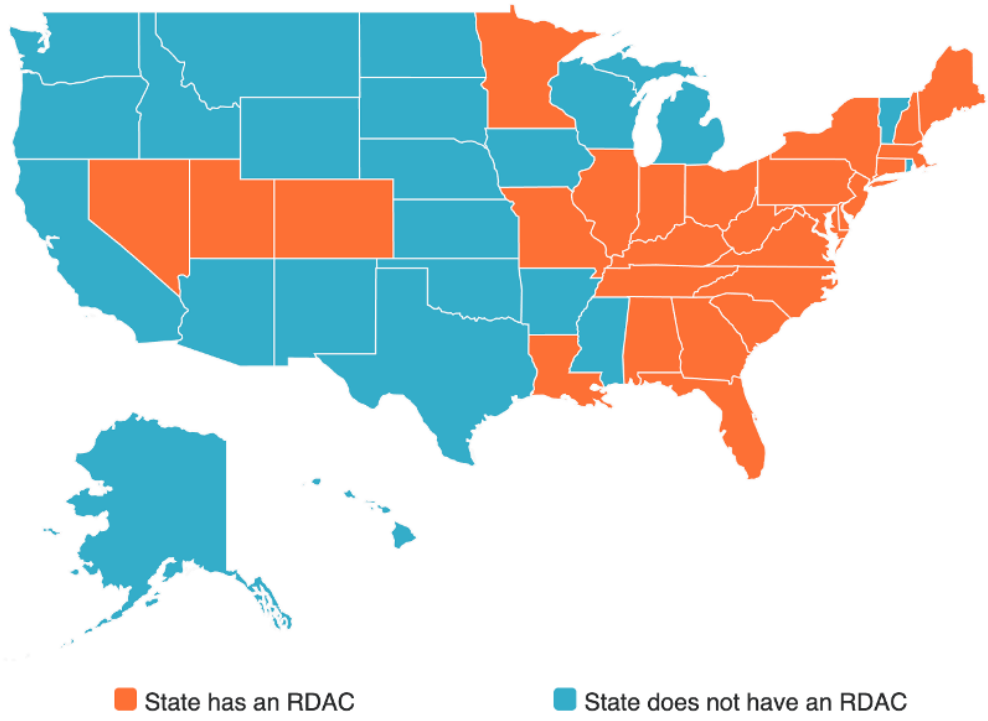
On October 1, FARA joined rare disease advocates from Pennsylvania to participate in the EveryLife Foundation's PA State Advocacy Day at the State Capitol in Harrisburg. Advocates shared their experiences with rare diseases and discussed a number of bills that have been introduced into the Pennsylvania state legislature. More information on the EveryLife Foundation's state advocacy program can be found here.

## FARA Ambassador Mary Nadon Scott Attends NORD's Vermont Rare Action on the Road Event

On November 2, Mary Nadon Scott convened with other rare disease advocates for NORD's Rare Action on the Road event in Burlington, VT. The event was an in-person workshop to help attendees develop their advocacy skills and learn more about Rare Disease Advisory Councils (RDACs) program.

## Delaware Governor Signs Bill to Extend Genetic Privacy Protections

On October 9, Delaware Governor, John Carney, signed HB 268-1 into law. This new law expands on the federal protections established through the Genetic Information Nondiscrimination Act (GINA) by prohibiting life insurers from discriminating against individuals based on information enclosed in genetic test results (excluding information in a patient's medical records or family history). Lawmakers and advocates hope that this bill will prevent any discrimination that may deter individuals from seeking genetic tests.




## California Officially Establishes a Rare Disease Advisory Council (RDAC)

Governor Gavin Newsom has signed AB2613 into law, making California the 29th state to establish a Rare Disease Advisory Council!

## Would you like an RDAC in your state? Take action with NORD!

NORD is looking for individuals to submit a RDAC Supporter Statement. This would include a short testimonial (3-5 sentences) about why you support a state RDAC. Please also submit a photo alongside your statement so that legislators can put a face to your story! They will share the statements and photos directly with State Senators and Assemblymembers. **How to submit? Fill out this form and then email your photo to csheridan@rarediseases.org. Learn more about RDACs here.**

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## State Newborn Screening Updates

### Massachusetts Becomes Fourth State to Include Duchenne Muscular Dystrophy in Newborn Screening Panel

Parent Project Muscular Dystrophy announced that Massachusetts has approved the addition of Duchenne muscular dystrophy to their newborn screening panel. Massachusetts becomes the fourth state to do so, behind Ohio, New York, and Minnesota.

### Texas Department of State Health Services Releases Report on Newborn

**Screening Expansion**

The Texas Department of State Health Services (DSHS) released a[report](#) in compliance with 2023 HB 2475, [a newborn screening RUSP alignment bill](#) led by the EveryLife Foundation and Texas Rare Alliance, and in partnership with rare disease advocates. This 2024 report highlights the progress and challenges in expanding newborn screening in Texas.

**Local Wisconsin News Station Highlights Newborn Screening RUSP Alignment Legislation**

A recent [article](#) highlights the need for RUSP alignment legislation in Wisconsin. With the recent addition of Krabbe to the Recommended Uniform Screening Panel (RUSP), Krabbe advocates in the state are calling for a streamlined process to add conditions to its screening panel. To learn more about this effort, click [here](#).