FARA Friedreich's Ataxia Research Alliance

FY26 Budget Updates

With the U.S. government shutting down on October 1, 2025, the country is now in the second longest shutdown in history with **35 days** without federal appropriations. Negotiations to end the shutdown are at a standstill due to a disagreement within Congress about the extension of Enhanced Premium Tax Credits (PTCs) for Marketplace plans. These enhanced PTCs were premium subsidies enacted during the COVID-19 pandemic to make insurance coverage more affordable and accessible for Americans. The enhanced PTCs are set to expire in December, and if not extended, estimates suggest that premium costs will significantly rise, leading to over 4 million people to lose medical insurance coverage.

You may ask, “How does the shutdown impact the FA community?” Without appropriated funding, around 670,000 federal workers have been furloughed, 4,100 have been fired, and 730,000 have been working without pay, and this has led many critical government services to be stalled. For instance, the FDA can continue to review applications that were already submitted but would not be able to accept a new drug application for FA. Similarly, while FA researchers can still submit grants to the NIH, no new grants will be reviewed or awarded until the shutdown ends.

We don't know how long the shutdown will continue, but the longer it continues, the more delays patients will face. Therefore, it's important that advocates inform Members of Congress how this is impacting the FA community by reaching out to their offices. You can do so by either emailing or calling their offices, and you can get started by looking up your Members using this website. All you need to do is input your address, and the website will provide you with their names and ways to contact them. From there, you can share your story, how the community has been affected by the shutdown, and why you think it's important to reopen the government. There's a template below to get you started.

Dear **[Representative/Senator]**,

My name is **[your name]**, and I am a constituent of yours from **[insert city, state]**. I am writing to you today to urge you to end the government shutdown as soon as possible. As someone impacted by Friedreich's Ataxia (FA), a rare genetic neuromuscular disorder, biomedical research funding and regulatory agencies are a beacon of hope—one that has been deeply affected by the current shutdown. Patients and families impacted by FA do not have time to waste. FA is progressive and will inevitably lead to life-altering loss of mobility and often other complications without therapeutic intervention. Our community needs treatments, and hopefully someday, a cure. That is not possible without the support of the federal government's biomedical research and regulatory infrastructure.

Due to the shutdown, the NIH cannot review or award new grants, stalling research studies that could yield scientific breakthroughs. Moreover, the FDA cannot accept or review new drug applications, which may delay patient access to promising therapies. While a month may not seem like a significant delay to you, for a community dealing with a progressive neuromuscular disease, every single day matters, and continued delays could cause irreparable harm to patients. This is why I am deeply concerned about the present shutdown and hope that you will vote to reopen the government to ensure that government services can continue to serve patients and families like mine.
I am grateful for your time and consideration, and thank you for your commitment to serving the people of **[insert district/state]**.

Best,
[your name]

Capitol Hill Updates

Trump Administration Changes Course on Accessible Travel

In early October, the Department of Transportation (DOT) announced that it would be delaying enforcement of four key provisions of the "Wheelchair Rule."

This federal rule, which was originally supposed to take effect in January 2026, requires training and heightened standards for airline staff and contractors who assist disabled passengers or handle mobility aids. It also establishes a formal process for passengers whose mobility aids were damaged to seek recourse.

The DOT announcement is looking to make changes to the following: whether airlines can be held liable for damaged mobility aids, how often airline staff should be trained, and when passengers can be notified of higher fees because of accessibility needs. Given the importance of air travel to clinical trial participation and access to care, FARA is committed to advocating for better airline accessibility.

Trump Administration Pursues More Layoffs As Government Shutdown Grows Longer

Earlier in October, the Trump Administration took unprecedented steps to reduce the government workforce by terminating federal employees during the shutdown. These

layoffs affected approximately 4,100 federal workers, including approximately 1,200 employed in the Department of Health and Human Services (HHS).

A union representing federal workers filed a lawsuit against the government, asserting that the terminations were illegal, and as a result, a judge issued a temporary restraining order (TRO) blocking the layoffs from going into effect. Additionally, some workers were reinstated due to the essential nature of their positions.

Senate HELP Committee Holds Hearing On The Future of Biotechnology

On October 29, the Senate Health, Education, Labor, and Pensions (HELP) Committee held a hearing titled "The Future of Biotech: Maintaining U.S. Competitiveness and Delivering Lifesaving Cures to Patients" to hear from experts regarding how Congress should act to ensure that biotechnological innovation continues.

Experts on the panel discussed important issues like the impact of federal investments in research, the need for flexibilities for rare disease indications, and how to ensure patients get access to scientific breakthroughs.

Top FDA Orphan Drug Regulator Is Reassigned

Sandra Retzkey, DO, JD, MPH, formerly the head of the FDA's Office of Orphan Products Development, has been removed from that role. Dr. Retzkey has led the Office of Orphan Products Development since 2021 and was a leading advocate for rare disease drug development and patient

engagement. Her new role and replacement are currently unknown.

Newborn Screening Saves Lives Reauthorization Act Gains Momentum

On September 10, the House Energy & Commerce Committee's Subcommittee on Health voted to advance the Newborn Screening Saves Lives Reauthorization Act (H.R. 4709) to the full committee—advancing a critical public health program that allows for screening and early diagnosis of life-limiting diseases.

Part of this legislation would restore the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC), the advisory panel that determines what conditions are added to the Recommended Uniform Screening Panel (RUSP). The ACHDNC was dismantled earlier this year by the Trump Administration.

FDA Publishes Internal Filing Checklist

The FDA's Center for Drug Evaluation and Research (CDER) recently released its internal filing checklist used to determine if a new drug application is complete and reviewable to the public. This move comes as a

larger push to ensure transparency in FDA processes and procedures.

Upcoming Advocacy Events

CLOSING SOON: Apply for Funding to Come to Rare Disease Week 2026!

Applications are closing soon for travel reimbursement for Rare Disease Week 2026! Rare Disease Week 2026 will take place in Washington, D.C. from February 24-26, 2026 and is a fantastic opportunity to educate your legislators, learn about issues affecting the rare disease community, and meet other patients and families impacted by rare diseases. To support participation, the EveryLife Foundation provides travel reimbursement to help cover the cost of transportation, lodging, and other expenses.

Anyone interested in attending Rare Disease Week on Capitol Hill should apply for the EveryLife travel reimbursement before the November 7th deadline!

Apply for EveryLife Travel Reimbursement

Announcements

FARA Has Strong Presence At Major Rare Disease Events

October was a busy month in the rare disease community, and FARA made a strong showing at a number of events

—including the NORD Breakthrough Summit, the BIO Patient Advocacy Changemakers Event, an EveryLife Share Your Story Webinar, and more. Not only did FARA attend these events, but the FARA Advocacy Team contributed important insights and ensured that the FA community's needs and experiences were represented.

At the EveryLife Share Your Story Webinar, FARA Director of Advocacy, Brigid Brennan, shared more about FARA's new FA & Speech Toolkit and how it was created to help empower people with FA to feel more comfortable speaking and engaging in advocacy. She also spoke during the Association of Managed Care Providers (AMCP) conference on the importance of including the patient voice in access decisions.

Additionally, at the NORD Breakthrough Summit, FARA Co-Founder and President Ron Bartek was asked to serve on a panel of experts discussing the challenges of navigating the federal regulatory process. He also provided important insights into the importance of pediatric inclusion and flexibilities that allow children and adolescents to engage in the drug development process soon.

United Against Ataxia Hill Day In The News

Healio, a medical media organization providing news and continuing

education for physicians, recently featured an article about NAF and FARA's 7th Annual United Against Ataxia Hill Day. The article is an interview style Q&A with Brigid Brennan, FARA's Director of Advocacy, and features a discussion of why advocacy is important, the history of the event, the legislative asks, and things physicians can do to support patients.

Read the Healio Article

New Analysis Shows FDA Approvals Are Slowing

A recent analysis by RBC Capital Markets found that FDA drug approvals dropped 14% in the last quarter compared to averages from the previous six quarters—falling from an average of 87% to just 73%. The authors also found that delays in meeting review deadlines also rose and that there was a significantly higher number of rejections.

State News

Advocate in Your State—Apply for Your Medicaid Beneficiary Advisory Council!

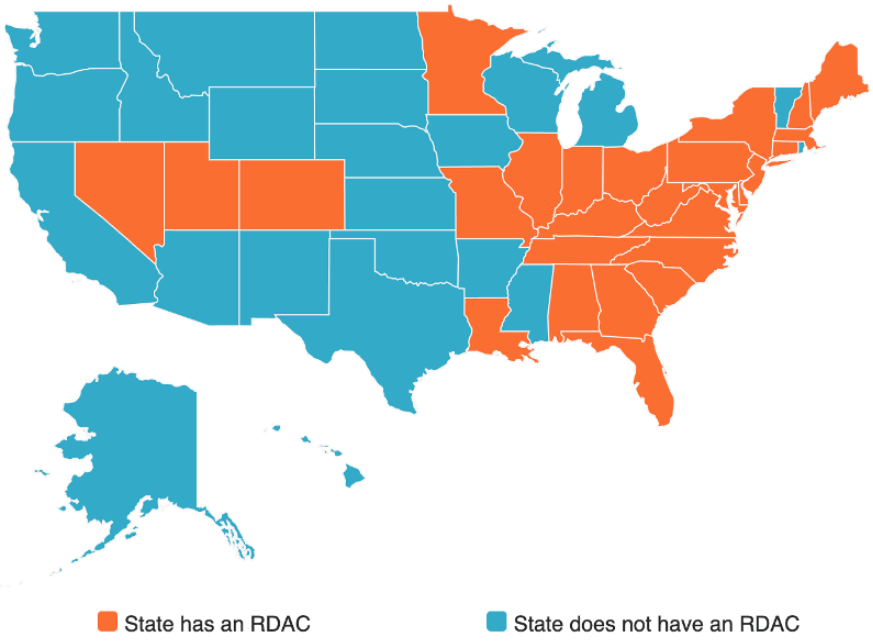
Numerous states are currently accepting applications for their Medicaid Beneficiary Advisory Councils (BAC), and this is a great opportunity to make your voice heard and advocate for patients and families in your state.

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. FARA will publish additional states opening their applications later this fall.

- **New York**—Applications are being accepted on a rolling basis with no deadline!
- **Florida**—Applications are currently open!
- **Maryland**—Applications are currently open! Email mdh.mmacbac@maryland.gov to apply!
- **Oklahoma**—Applications are currently open with no deadline!
- Washington—Applications are currently open! Email ahcommunications@hca.wa.gov to apply! Make sure you include your name, client ID, contact information, and your preferred time to be contacted in your message.



Upcoming Rare Disease Advisory Council (RDAC) Meetings

Colorado: The Colorado RDAC is meeting virtually on Monday, November 10 from 9:00 a.m. - 12:00 p.m. Additional meeting information can be found **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**.

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**.

Ohio: The Ohio RDAC is meeting virtually on Thursday, November 6 from 2:00 p.m. to 4 p.m. ET. Additional meeting information can be found **here**.

Massachusetts: The Massachusetts RDAC is meeting on Thursday, November 20 from 9:00 a.m. to 11:00 a.m. ET. Additional information can be found **here**.

Virginia: The Virginia Rare Disease Council is meeting virtually on Wednesday, November 19 at 10:00 a.m. ET. You can find additional meeting information **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 on FA or rare disease? FARA would love to share the amazing advocacy work our community members are doing. Please send updates to Emma Potter, emma.potter@curefa.org.

Our mailing address is:

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