FARA Friedreich's Ataxia Research Alliance



Why PDUFA Matters for Rare Disease Advocacy

The Prescription Drug User Fee Act (PDUFA) was created in 1992 following strong advocacy from the HIV/AIDS community, which pushed for faster access to potentially lifesaving therapies. At the time, reviewing a new drug application took an average of 27 months, and 70% of new medicines were becoming available outside the United States before they reached American patients!

PDUFA gave the FDA additional resources by allowing the agency to collect user fees from pharmaceutical companies when they submit applications for new therapies. These resources help the FDA hire staff, improve review processes, and shorten the time it takes to evaluate new treatments. In return, the FDA promised faster timelines for drug approval.

Every five years, Congress must reauthorize PDUFA. This means that Congress must draft a "must-pass" piece of legislation that outlines how the user fees will be used and what programs or initiatives the agency will undertake over the next five years.

PDUFA reauthorization is particularly important for the rare disease community because it often serves as a vehicle for broader policy improvements that affect how therapies are developed and reviewed. Previous reauthorizations helped establish the FDA's Patient-Focused Drug Development efforts, strengthened pathways such as Accelerated Approval, expanded incentives for pediatric rare disease drug development, and created programs to support innovative therapies.

The next reauthorization, known as PDUFA VIII, is scheduled for September 2027, and Capitol Hill discussions on the legislation are beginning now. Rare disease advocates have an important opportunity to help shape the policies and programs that influence future treatment development. FARA will continue to monitor the process and share opportunities for engagement as they arise, ensuring that the needs and perspectives of the FA community remain part of the conversation.

Capitol Hill Updates



Bipartisan Faster Clinical Trials Act Aims to Accelerate Rare Disease Drug Development





Reps. Jake Auchincloss (D-MA) and Morgan Griffith (R-VA) introduced the Faster Clinical Trials Act to expand patient participation and reduce delays in clinical trials, particularly for rare and chronic diseases. The legislation would establish a Center for Clinical Trial Innovation at FDA, strengthen the role of the Rare Disease Innovation Hub, and support decentralized trials and innovative approaches to drug development.

Read More

Congressional Proposals to Promote Biosimilar Competition

A new Health Affairs Forefront article examines several bipartisan proposals under consideration in Congress aimed at increasing biosimilar competition, including changes to interchangeability requirements, patent litigation, and patent review. The analysis explores how these proposals could affect biosimilar market entry, competition, and prescription drug costs.

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Regulatory Updates



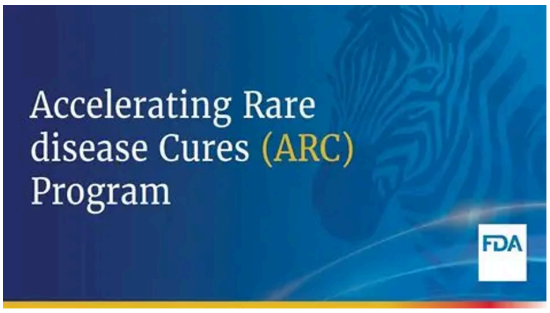
New FDA Rare Disease Framework Announced

FDA has announced the Rare Disease Framework Initiative to reimagine how drugs and biologics for rare diseases are developed and reviewed by FDA. As detailed in FDA's accompanying white paper, this initiative will build a fit-for-purpose framework that reflects the realities of rare disease drug development accounting for the unique nuances of each disease, patient needs, product type, and the evidence that can realistically be generated in small patient populations.

On October 28, FDA hosted a public meeting to solicit feedback on the initiative from patients, disease advocates, industry, and other stakeholders. Ron Bartek delivered a public comment on FARA's behalf covering the need for internal collaboration, patient voices within the regulatory process, and updated pediatric guidance.

FDA Releases 2026 ARC Program Annual Report

FDA's Center for Drug Evaluation and Research (CDER) released its 2026 Accelerating Rare disease Cures (ARC) Program Annual Report, highlighting progress in scientific and regulatory innovation, community engagement and education, and operational improvements.



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FARA's Advocacy Impact

NORD Breakthrough Summit

At the 2026 NORD Breakthrough Summit, FARA Advocacy Program Coordinator Berkley Bell and NAF Community Services Associate Courtney Bockhop presented a poster they authored with FARA Advocacy Director Brigid Brennan and NAF Community Services Director Lori Shogren, titled "United Against Ataxia: Building an Accessible and Inclusive Model for Federal Advocacy."



The poster highlighted how transitioning United Against Ataxia Hill Day from an in-person to a virtual event expanded participation by removing barriers such as travel, fatigue, and inaccessible buildings. As a result, congressional meetings increased

from 13 to 101, and a more diverse and representative community of advocates was able to participate.

FA Community Advocacy Opportunities



Keep up the Pressure to Stop OMB's Proposed Rule

A proposed federal regulation from the White House Office of Management and Budget (OMB) threatens to undermine research progress by making grants vulnerable to abrupt cancellation, political interference, and unpredictable changes in funding requirements. The far-reaching regulation has been delayed until Dec. 11, but it is not gone. You are what stands in the way of it becoming final on Dec. 12.

Research!America put together a new, quick way for folks to make a difference. All you need to do: when you see a news article, editorial, or op-ed that is connected to health, such as a research breakthrough or new data, follow these three easy steps. They'll make it easy for you to produce a compelling letter to the editor that highlights why research is important and why the proposed rule is bad for patients, bad for our communities, and bad for the nation. If you need help writing your letter to the editor, please reach out to FARA's advocacy team!

Additionally, the National Council of Nonprofits is organizing a national Day of Action on **Nov. 10**. The day unites research allies to contact their members of Congress and post on social media about OMB's proposed rule and its impact on federal grantmaking.

Learn More

SNAP
Scientist Network for Advancing Policy

Science Policy for Early-Career Scientists

The Scientist Network for Advancing Policy (SNAP) Coalition has launched SciPol 101, a free, open-access curriculum designed to educate and empower scientists to engage in science policy and advocacy. The five-module course covers the basics of science policy, how government works, writing for science policy, science communication, and science diplomacy. The materials are designed for students and emerging university science policy groups but are available to anyone looking to strengthen their science policy and advocacy skills.

Announcements



Update to Upcoming Medicaid Work Requirements

On October 29, a federal judge struck down the portion of the new Medicaid rule that requires Medicaid recipients to prove that their medical condition "significantly impairs" their ability to comply with new community engagement requirements. While this does not halt the new requirements, it should make it less burdensome for people with serious conditions, like FA, to be considered exempt from these requirements.

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Florida Living Rare, Living Stronger

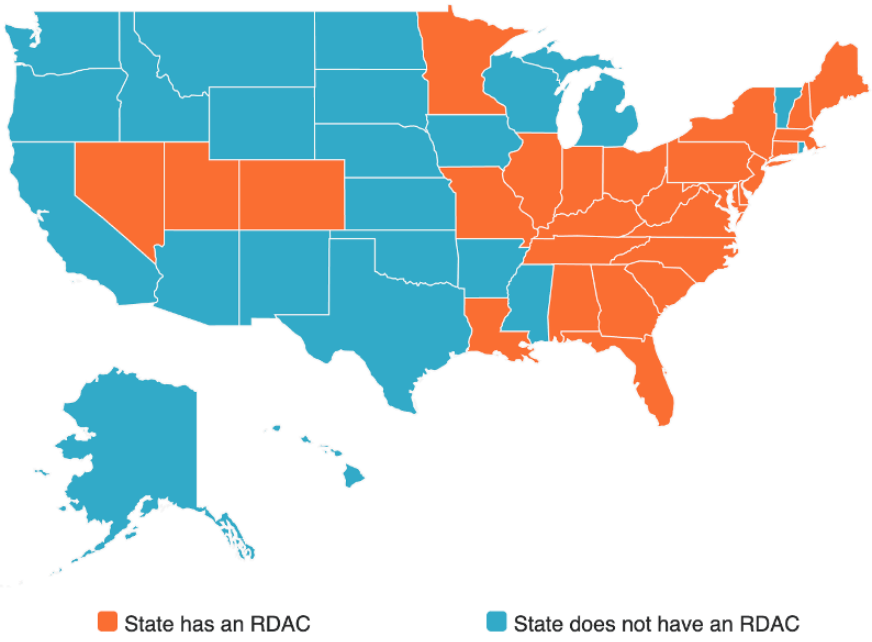
NORD is hosting a Living Rare, Living Stronger event in Fort Lauderdale, Florida on December 4, 2026. The event is free for rare disease patients and caregivers and will:

- Feature educational sessions designed to help attendees navigate life with a rare disease or as a rare caregiver.
- Help alleviate feelings of isolation by connecting caregivers and those living with rare diseases to each other.
- Introduce attendees to local advocates and present opportunities to engage in state/federal grassroots advocacy efforts.
- Highlight community-based organizations that have resources for people with rare diseases.
- Showcase how designated NORD® Rare Disease Centers of Excellence are advancing rare disease care and research.

Learn More

State News

Michigan, New Jersey, North Carolina and Pennsylvania are currently in regular session. The District of Columbia Council, Puerto Rico and U.S. Congress are also in regular session. Ohio is in recess until November 9.



Upcoming Rare Disease Advisory Council (RDAC) Meetings

Colorado: **November 9** (virtual). Meetings are held monthly.
Maryland: **November 10** (virtual). Meetings are held monthly.
Illinois: **November 11** (virtual). Meetings are held monthly.
Louisiana: **November 17** (in-person). Meets four times per year.
Virginia: **November 17** (in-person). Meets quarterly.
Georgia: **November 18** (virtual). Meetings are held monthly.
Massachusetts: **November 19** (virtual). Meetings are held every other month.
Connecticut: **November 24** (virtual). Meetings are held monthly.
Florida: **December 1** (virtual). Meets sporadically.
Tennessee: **December 2** (virtual). Meetings are held every other month.
Nevada: **December 4** (virtual). Meetings are held every other month.
Delaware: **December 8** (in person or virtual). Meetings are held quarterly.
Indiana: **December 11** (virtual).
New Jersey: **December 15** (virtual). Meetings are held quarterly.

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 Berkley Bell, berkley.bell@curefa.org.

Thank you to our Advocacy Ally Sponsor: Biogen

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FARA Friedreich's Ataxia Research Alliance

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