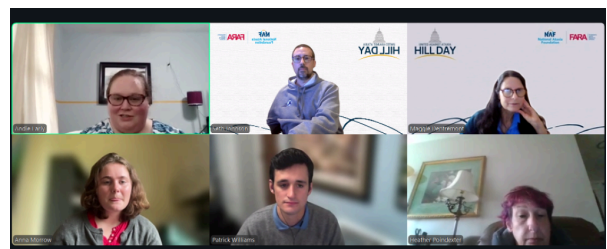
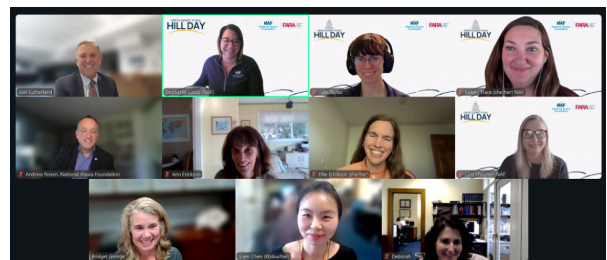




On September 23, 168 advocates from 35 states came together to educate lawmakers about Ataxia through 112 Congressional meetings! Advocates shared firsthand experiences about the challenges of being diagnosed with and living with Ataxia, as well as the urgent need for treatment options. Together, our voices made a powerful impact. The Ataxia community's advocacy on Hill Day not only helps lawmakers understand how their decisions affect families in their districts but allows FARA and NAF to sustain Congressional relationships year after year.



Advocates urged Congress to protect fair, science-based research funding, preserve Hereditary Ataxia's eligibility for CDMRP funding, and invest in rare disease research and drug development. The success of these meetings was made possible by the dedication, preparation, and passion of every advocate who took the time to share this important message. Thank you to everyone who participated in the 8th annual United Against Ataxia Hill Day!



Capitol Hill Updates



Newly Introduced Bill Would Make Rare Pediatric Disease PRV Program Permanent

A bipartisan group of lawmakers led by Representative Gus Bilirakis (FL) has introduced the Priority for Pediatric Cures Act, which would permanently authorize the Rare Pediatric Disease Priority Review Voucher (PRV) Program. A permanent authorization would provide greater long-term certainty for developers investing in rare pediatric drug development.



[Read More](#)

Senate Confirmation Hearings on HHS Leadership

The Senate HELP Committee held a confirmation hearing on the nomination of Heidi Overton, M.D., Ph.D, to serve as Commissioner of the Food and

Drug Administration (FDA) ([Watch here](#)). Overton is a physician who currently serves as Deputy Director of the White House Domestic Policy Council. The committee has not yet scheduled a vote on her nomination.



Additionally, the Senate Finance Committee voted 15-12 to advance the nomination of Chris Klomp to serve as Deputy Secretary of HHS ([watch here](#)).

2026 National Ataxia Awareness Day Resolution

We're thrilled to share some exciting news—our 2026 National Ataxia Awareness Day Resolution (S.Res. 894) 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, 2026, as National Ataxia Awareness Day, helping to elevate national recognition and understanding of Ataxia.



Each year, NAF and FARA, along with dedicated advocates, work with members of Congress to make this possible. We are deeply grateful to Senator Hyde-Smith for her leadership and to Senators Murphy and Capito for their support on this important resolution.



Research Advocates Speak Out Against a Harmful Executive Order

Recently, news broke that the administration was considering an executive order that would establish a new board of politically appointed officials to review and approve individual NIH grants. This executive order would have overburdened the scientific review process at NIH and dramatically slowed medical research progress. Patient and research advocacy organizations mobilized against the EO; Congressional champions [took action](#), and it appears that it [will not come to pass](#). This effort demonstrates that when organizations, institutions, and individuals unite, we can make a real difference.

SURGE Act Introduced to Fund State Implementation of Newborn Screening

Representatives Nick Langworthy (R-NY) and Kim Schrier (D-WA) introduced the Surge to Save Newborns Act, which would establish a grant program to provide funding for states to implement one of the 40 conditions currently on the RUSP as well as those added through the program's 5-year timeline.



Currently, when a state decides to implement a new condition after it has been added to the RUSP, they must identify new resources to cover the added costs, which can result in delays and ultimately differences from state to state in what conditions are screened for at birth.



NIH-DoW Research Agreement Draws Congressional Scrutiny

NIH and the Department of War have entered into an interagency agreement that could allow certain biodefense and infectious disease research projects to be transferred to DoW. The agreement has prompted congressional scrutiny, while NIH and DoW have stated that any collaboration would occur on a project-by-project basis and remain within NIH's public health mission.

[Read More](#)

Upcoming Advocacy Events



While there are no major advocacy events on the calendar this month, it's a good time to stay engaged and keep rare disease issues part of the conversation. Over the coming weeks, many elected officials and candidates will be attending local community events, town halls, and public forums. These gatherings can provide an opportunity to introduce yourself, share your connection to FA, and ask broad

questions about their support for medical research funding and rare disease communities.

Announcements



New Medicaid Guidance Allows ‘Tiered’ Approach to Medical Frailty Determinations

CMS released new guidance outlining how states can implement medical frailty exemptions from upcoming Medicaid community engagement requirements. The guidance allows states to use tiered approaches to identify qualifying conditions, clarifies self-attestation policies, and provides additional flexibility as states prepare for the January 1, 2027 implementation deadline.

[Read More](#)

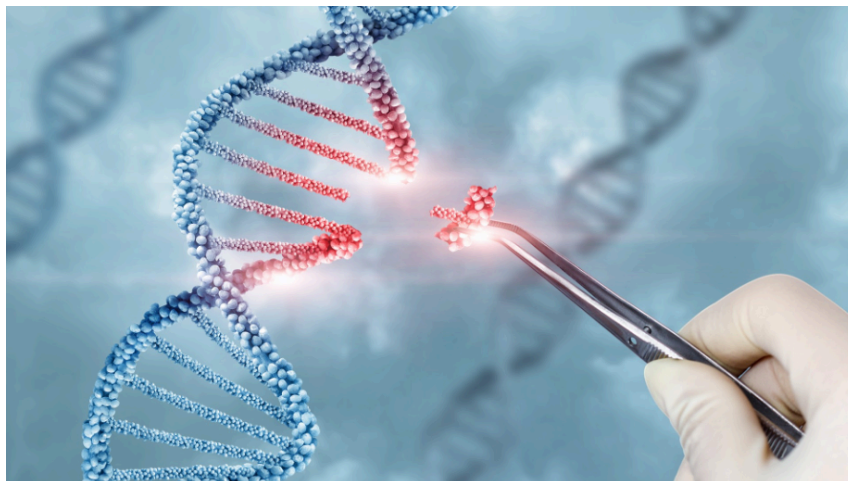
New CBER Director Highlights Patient-Centered Rare Disease Development

Newly appointed FDA Center for Biologics Evaluation and Research (CBER) Director Karim Mikhail outlined his vision for the Center, emphasizing timely patient access to innovative therapies, engagement with the rare disease community, and regulatory approaches that keep patients at the center of product development.

"Frontier science only matters when it reaches the patients," Mikhail wrote.



[Read More](#)



FDA Finalizes Cellular and Gene Therapy Guidance

FDA finalized its guidance on developing cellular and gene therapy products, incorporating several updates following stakeholder feedback. Changes include greater flexibility in correlating product characterization data with clinical outcomes, additional recommendations for generating evidence when animal models are unavailable, and expanded discussion of clinical endpoints, surrogate endpoints, and biomarkers to support product development.

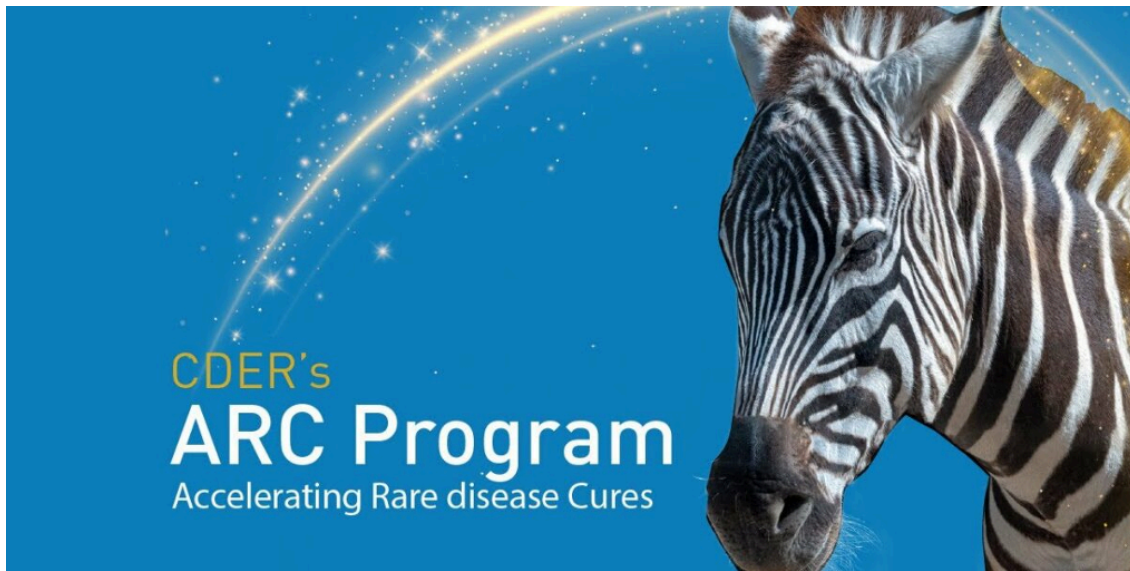
[Read More](#)



FDA Launches CDRH Rare Disease Impact Initiative

FDA's Center for Devices and Radiological Health (CDRH) has launched the Rare Disease Impact Initiative to support the development and timely authorization of safe and effective medical devices for people living with rare diseases. The initiative serves as a central hub for rare disease medical device resources, including patient engagement opportunities, regulatory information, a new Rare Disease Medical Device List, and upcoming workshops focused on innovation and device development.

[Read More](#)



FDA Releases 2025–2030 ARC Strategic Plan

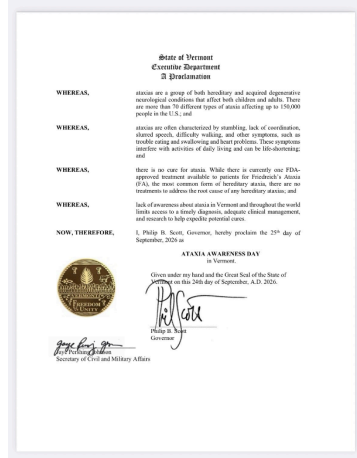
FDA's Center for Drug Evaluation and Research (CDER) has released the Accelerating Rare Disease Cures (ARC) Program 2025–2030 Strategic Plan, outlining its priorities for advancing rare disease drug development over the next five years. The plan focuses on scientific and regulatory innovation, stakeholder engagement and education, and strengthening internal operations to accelerate the development of safe and effective treatments for people living with rare diseases.

[Read More](#)

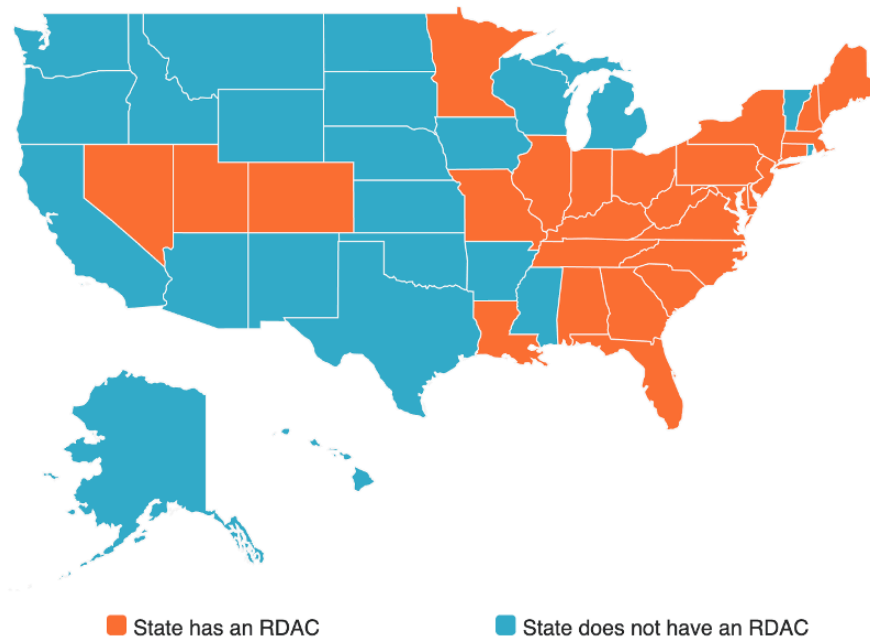
State News

State Ataxia Awareness Day Resolutions

September 25 was National Ataxia Awareness Day, and several ataxia advocates went above and beyond by introducing state Ataxia Awareness Day Resolutions.



FARA Ambassador Mary Nadon Scott introduced a resolution in her home state of Vermont. Other ataxia advocates from around the country introduced resolutions in Colorado, Virginia, South Carolina, and Michigan!



Upcoming Rare Disease Advisory Council (RDAC) Meetings

Georgia: October 21 (virtual). Meetings are held monthly.

Tennessee: November 25 (virtual). Meetings are held every other month.

Nevada: October 2 (virtual). Meetings are held every other month.

New Jersey: October 6 (virtual). Meetings are held quarterly.

Michigan: October 7 (virtual). Meetings are held quarterly.

Colorado: October 12 (virtual). Meetings are held monthly.

Maryland: October 13 (virtual). Meetings are held monthly.

Illinois: October 14 (virtual). Meetings are held monthly.

Indiana: October 23 (virtual). Meetings are held every other month.

Connecticut: October 27 (virtual). Meetings are held monthly.

Massachusetts: November 19 (virtual). Meetings are held every other month.

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.

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