FARA Friedreich's Ataxia Research Alliance

United Against Ataxia Hill Day 2026

We're thrilled to invite you to our **8th annual United Against Ataxia Hill Day on September 23, 2026!** Hosted jointly with our partners at National Ataxia Foundation (NAF), United Against Ataxia Hill Day allows members of the ataxia community to meet virtually with members of Congress to advocate for the issues that matter most. Whether you're living with ataxia, caring for a loved one, or supporting our cause, your voice has the power to make a difference.

Meeting directly with lawmakers and their staff helps put a human face on the challenges our community faces every day. Statistics and research are important, but personal stories are what inspire action. By sharing your experiences, you can help your members of Congress better understand the urgent need for research funding, scientific collaboration, and the timely development of new treatments.

Last year, 169 attendees from 38 states joined us to speak up for the ataxia community, and all of our asks were met. We need your help to make this year an even bigger success! No previous advocacy experience is required, just a willingness to speak from the heart. We can't wait to show Congress the strength, resilience, and determination of the ataxia community.

Please save the date; registration begins on August 10!

Capitol Hill Updates

Office of Management and Budget Proposed Regulation for Federal Assistance

In late May, the Office of Management and Budget proposed a new regulation that would significantly reshape how scientific research grants are awarded, managed, and communicated, shifting decision-making power away from independent scientific peer review and toward political appointees and priorities. FARA submitted a public comment in opposition to the wide-ranging impacts this rule would have on the research ecosystem and pace of scientific progress, joining 496,774 others who commented on the proposed regulation!

Across the country, individuals and groups representing medical research, agriculture, law enforcement, transportation, housing, and others have sounded the alarm on this proposed rule's destructive impact on key priorities of the American people. On August 3, the Senate Appropriations Committee passed a budget bill that delays implementation of the rule. This legislation is a sign that lawmakers are listening to us and a testament to the overwhelming power of advocacy!

The fight is not over! We still need the Senate to approve the Appropriations Committee's budget and reconcile the bill with the House version. You can use this form to urge lawmakers to continue to stand up against this rule. You may edit your response to reflect your personal story, and some of the following points on how the rule would impede FA research:

- If grant review begins to reflect shifting policy objectives, rare disease research could be disproportionately disadvantaged because it may be viewed as less

relevant to broader political priorities across administrations.

- Restrictions on global partnerships could make it harder to recruit enough participants for research studies.
- Grants could be terminated in the middle of a study, after researchers and patients have contributed significantly to the program.
- Restrictions on publication costs, conference participation, and professional society membership would impede the exchange of scientific knowledge that is essential to research progress.

ENERGY & COMMERCE
CHAIRMAN BRETT GUTHRIE

House Energy and Commerce Committee Hearing: Maintaining US Leadership in Biomedical Research

On July 15, the House Energy and Commerce Committee held a hearing on maintaining US leadership in biomedical research. Members expressed bipartisan support for strengthening rare disease research by operationalizing the FDA's Operation TrialBlazer initiative. Watch here.

Draft Legislation: Next-Generation U.S. Clinical Development to Accelerate Cures

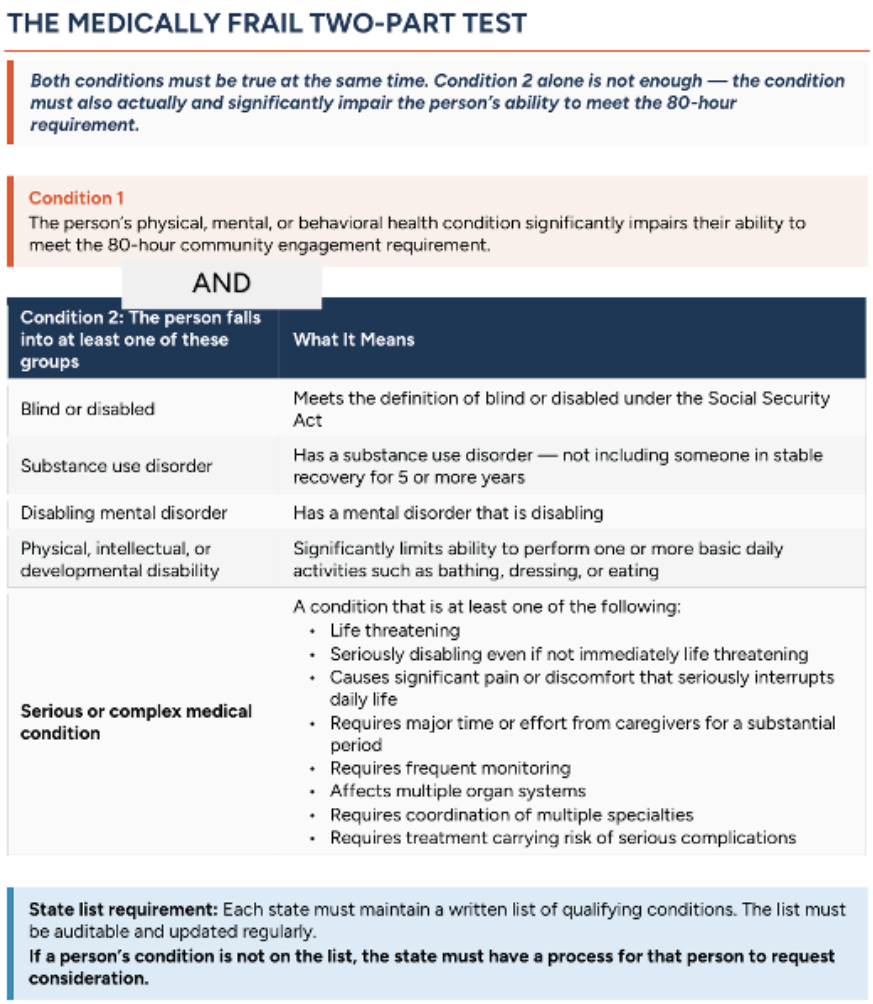
Representative Auchincloss (D-MA) released draft legislation aimed at modernizing U.S. clinical development for faster and more accessible cures. Auchincloss has said that clinical trial reform is one area where he believes there is bipartisan support to address rising development costs, slow trial timelines, limited patient access and growing concerns that the United States is losing ground to

countries with faster early-stage research systems, such as China. Read more.

FARA Advocates on Capitol Hill

FARA President Ron Bartek and community advocate Anna Morrow joined NAF Community Services Associate Courtney Bockhop on Capitol Hill on August 22. They attended a briefing hosted by the Rare Disease Congressional Caucus and met with legislative offices to discuss Ataxia Awareness Day and OMB's Regulation for Federal Financial Assistance.

Medicaid Community Engagement Requirements



THE MEDICALLY FRAIL TWO-PART TEST

Both conditions must be true at the same time. Condition 2 alone is not enough — the condition must also actually and significantly impair the person's ability to meet the 80-hour requirement.

Condition 1
The person's physical, mental, or behavioral health condition significantly impairs their ability to meet the 80-hour community engagement requirement.

AND

Condition 2: The person falls into at least one of these groups	What It Means
Blind or disabled	Meets the definition of blind or disabled under the Social Security Act
Substance use disorder	Has a substance use disorder — not including someone in stable recovery for 5 or more years
Disabling mental disorder	Has a mental disorder that is disabling
Physical, intellectual, or developmental disability	Significantly limits ability to perform one or more basic daily activities such as bathing, dressing, or eating
Serious or complex medical condition	A condition that is at least one of the following: • Life threatening • Seriously disabling even if not immediately life threatening • Causes significant pain or discomfort that seriously interrupts daily life • Requires major time or effort from caregivers for a substantial period • Requires frequent monitoring • Affects multiple organ systems • Requires coordination of multiple specialties • Requires treatment carrying risk of serious complications

State list requirement: Each state must maintain a written list of qualifying conditions. The list must be auditable and updated regularly.
If a person's condition is not on the list, the state must have a process for that person to request consideration.

As we outlined in last month's newsletter, The Centers for Medicare & Medicaid Services (CMS) has released a rule dictating how states will implement the Medicaid work/community service requirements from last year's H.R. 1. The rule requires many adults enrolled in Medicaid to complete at least 80 hours per month of work, education, job training, or community service to maintain their coverage and took effect July 31, 2026. States must implement the requirements by 2027.

Importantly, the rule outlines exemptions from the work requirements for Medicaid recipients considered "medically frail." To be considered medically frail, individuals must meet 2 requirements: 1. significantly impaired ability to work 2. a serious or complex medical condition. To determine which conditions are considered serious for criterion #2, states will be required to compile lists of conditions. Because FA has an ICD-10 diagnostic code, individuals with FA are likely to qualify for a medically frail exemption in many states.

Nevertheless, we are concerned about the first criterion, which we believe unfairly disregards the realities of progressive disease and places undue burden on

individuals already exhibiting extreme resilience. FARA submitted a public comment to CMS in opposition to the two-part medical frailty test, providing the following recommendations:

- Clarify that progressive neurodegenerative rare diseases, including Friedreich's ataxia, qualify under the medical frailty exemption.
- Expand the use of sworn self-attestation.
- Create community engagement waiver policies that capture the fluctuating realities of living with a progressive rare disease.
- Minimize unnecessary reverification requirements for individuals with permanent, progressive conditions that are unlikely to improve.
- Monitor state implementation for disparities affecting individuals living with rare diseases and publish guidance as needed to promote consistency across states.

An excerpt from FARA's public comment:

> "Requiring patients to prove that they cannot work, rather than recognizing the substantial burdens imposed by a progressive rare disease, creates a harmful paradox. Many people with FA continue working or attending school only through extraordinary effort, workplace accommodations, family support, and personal sacrifice. Their ability to engage in some level of work or education should not be interpreted as evidence that they are not medically frail. Patients should not be forced to repeatedly prove that a lifelong, progressive condition continues to exist or that it remains burdensome enough to warrant protection."

FDA Rebuilds Workforce and Takes Action to Modernize Clinical Development

FDA leadership announced that the agency has been authorized to hire approximately 2,200 employees as it works to rebuild staffing following significant workforce reductions last year. Officials reported that hiring is accelerating, employee attrition has returned to previous levels, and restoring review capacity and scientific expertise remains a top agency priority. Read more.

The agency also announced actions to accelerate and modernize clinical research across the full continuum of drug development — from the Investigational New Drug (IND) phase to late-stage pivotal trials. The FDA's work is outlined in *Operation TrialBlazer*, a U.S. Department of Health and Human Services (HHS) initiative. Read more.

Upcoming Advocacy Events

National Ataxia Foundation

Caring for Caregivers: Federal Advocacy in Action Webinar

Join National Ataxia Foundation at 2:00 PM ET on August 10 for a webinar discussing legislative proposals that would help caregivers build retirement security, save money, and earn Social Security credits for the care they provide, while exploring how members of the ataxia community can help advance these important policy solutions. Whether you are a caregiver, person with Ataxia, healthcare professional, or advocate, this session will provide practical insight into current legislative efforts and opportunities for engagement.

Register Here

Fall 2026 YARR Leadership Academy Application Open

Applications for The EveryLife Foundation's Fall 2026 YARR (Young Adult Representatives of RDLA) Leadership Academy are open through August 31. The program is a series of online classes offered to a select group of young adults in the rare disease community. It will introduce rare disease state policy priorities, legislative processes, advocacy tools, and leadership opportunities at the state level.

Apply Here

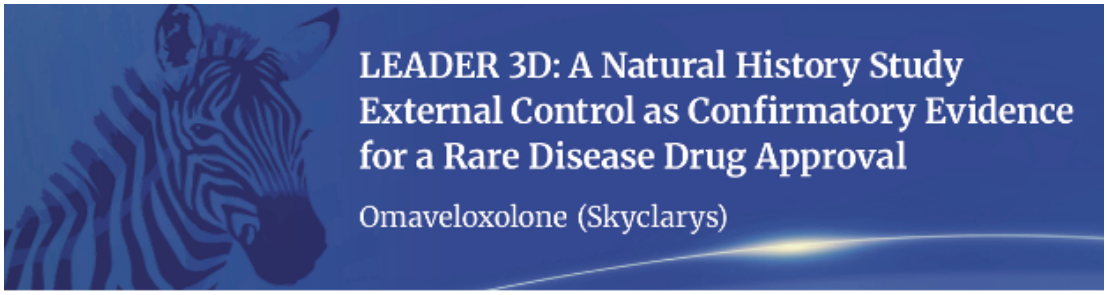
New England Rare Disease Advocacy Day

Registration for Rare Disease legislative Advocates' (RDLA) New England Rare Disease Advocacy Day is open through Friday, September 11! The event is a virtual day of action for New England (Connecticut, Maine, Massachusetts, New Hampshire, Rhode Island, Vermont) residents impacted by rare disease to meet your state legislators, share your stories, and help advance the policy priorities of the rare disease community.

Virtual meetings will be held on October 6 and 7, with mandatory attendance at the virtual legislative conference and webinar trainings the week of September 28.

Register Here

Announcements



Omaveloxolone Case Study

The FDA's LEADER 3D initiative is designed to support and inform rare disease drug development. As part of this effort, the FDA has developed a series of case studies highlighting drug approvals for rare diseases. One of these case studies features omaveloxolone and the use of FACOMS natural history data as confirmatory evidence.

Read Case Study

FDA Launches *Rare Connections* Newsletter

FDA has launched *Rare Connections*, the Agency's first centralized, agency-wide newsletter dedicated to the rare disease community. Published quarterly by the Rare Disease Innovation Hub, *Rare Connections* is designed to serve as a one-stop resource for updates from across FDA's medical product centers, bringing together information that has traditionally been spread across multiple offices and programs. Each issue will feature recent FDA approvals, guidance documents, regulatory

initiatives, patient engagement opportunities, public meetings, workshops, and other activities relevant to rare disease stakeholders.

Read the First Edition

Subscribe to Rare Connections

ARPA-H Launches THRIVE Program to Accelerate Rare Disease Gene Therapies

ARPA-H has announced up to $160 million in awards for its THRIVE program, a new initiative designed to accelerate the development of personalized genetic medicines for rare pediatric diseases. The program will support innovative platform technologies and umbrella clinical trial models intended to speed the development, testing, and delivery of treatments for multiple rare genetic conditions.

Read More

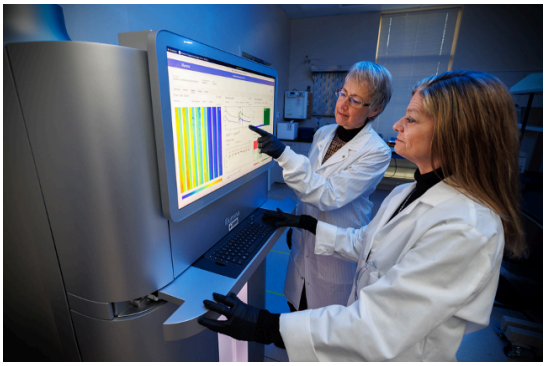
Sam Dupree Launches from Blog to Advocacy

FA Ambassador Sam Dupree's recent blog post shares his personal journey with FA and his hope for future treatments. As he worked on the piece, he realized his story needed to reach more than just readers. He then turned his experience into advocacy, reaching out to his legislators to share the urgent need for continued

research, access, and support for the FA community. Sam’s action highlights that you don’t need to wait until Hill Day to contact your lawmakers, but can be proactive about advocating for research year-round!

Read Sam’s Blog Post

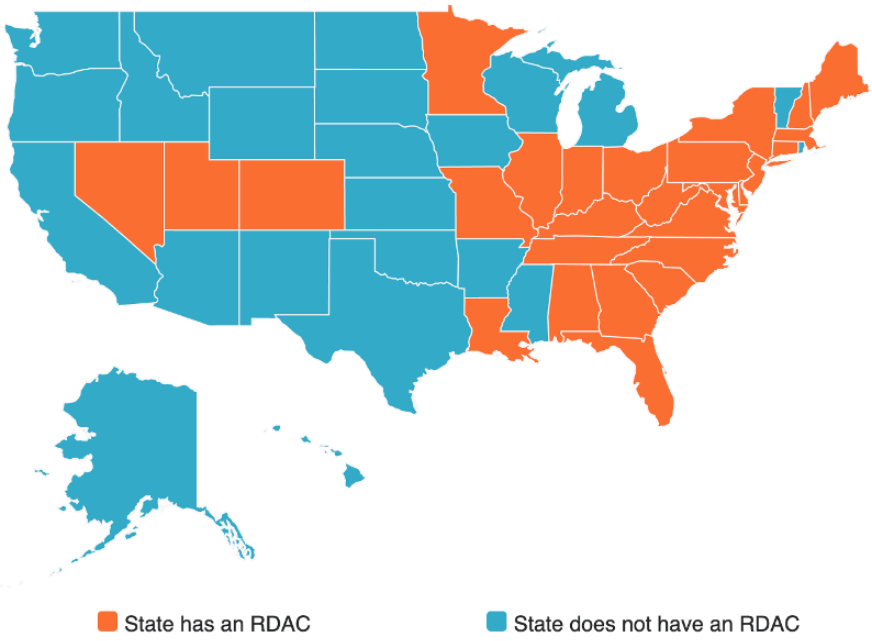
State News



Florida Advances Statewide Genomic Newborn Screening

Florida has launched enrollment for the Sunshine Genetics pilot program, one of the nation's first statewide initiatives to evaluate genomic newborn screening. The program will assess whether genomic sequencing can improve early identification of treatable rare diseases and help inform future newborn screening policy and implementation.

Read More



Upcoming Rare Disease Advisory Council (RDAC) Meetings

- **Massachusetts:** The Massachusetts RDAC meets on the third or fourth Thursday of every other month from 9:00 am to 11:00 AM. They will be meeting virtually on Thursday, September 17. Additional meeting information can be found here.

- **Connecticut:** August 25 (virtual). 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.

- **Tennessee:** The Tennessee RDAC meets on the fourth Wednesday of every other month. They will be meeting on Thursday, September 24. If you are interested in joining the meeting, please email info@tnrdac.org for instructions on attending. Additional meeting information can be found here.

- **Maryland:** The Maryland RDAC meets on the second Tuesday of every month from 4:00 p.m. to 5:00 p.m. They will be meeting on Tuesday, August 11. Additional meeting information can be found here.

- **Colorado:** The Colorado RDAC will be meeting virtually on September 14 from 9:00-11:00 AM. You can find additional meeting information here.

- **Michigan:** The Michigan RDAC will be meeting on October 7 at 10 AM. You can learn more about joining the meeting 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 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.

View this email in your browser

FARA Friedreich's Ataxia Research Alliance

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