



---

## The FDA's Accelerated Approval Pathway and FA Treatments

Accelerated approval allows the FDA to approve drugs for serious conditions that “fill an unmet need” using an intermediate or “surrogate” endpoint—a marker that is thought to predict clinical benefit, even if that benefit has not yet been fully demonstrated. The FDA created the Accelerated Approval pathway in 1992, largely in response to the HIV/AIDS epidemic, after advocates urged the FDA to streamline efficacy requirements to expedite access to innovative products that show promise for serious, incurable diseases. This pathway reflects a powerful truth: patient advocacy and participation in clinical trials can reshape how therapies are evaluated and approved.

Utilizing the accelerated approval pathway can significantly speed up access to new treatments but comes with an important requirement: companies must conduct a confirmatory trial after approval to verify that the drug truly provides the clinical benefit expected. If the companies do not conduct the trial or if the trial does not confirm the benefit, the FDA has the authority to remove the drug from the market.

A good illustration of using a marker thought to predict clinical benefit is a drug that shrinks a cancerous tumor. It is usually a good result to see a tumor get smaller, but that action may happen quicker than the sponsor can show that the proposed drug candidate has actually improved the person's health or survival. A confirmatory trial will follow patients taking the drug to show that the predicted clinical benefit, the patient got better or survived longer, actually happened.

At this moment, accelerated approval is especially relevant to the FA community. Several companies are considering new drug applications that could include accelerated approval requests based on the surrogate endpoints. Larimar Therapeutics is proposing increases in frataxin protein levels in FA patients as a novel surrogate endpoint and Lexeo Therapeutics is evaluating both frataxin levels in the heart in FA patients and changes in a cardiac biomarker as surrogate endpoints. Raising frataxin should improve neurological and cardiac function and/or slow disease progression, but confirmatory trials will be needed to prove that result.

Accelerated approval exists because of the many patients, families, and advocates who highlighted the need for urgency in getting treatments to patients. That legacy continues today, as your participation in clinical trials helps generate the data needed for approval, your voice helps define what outcomes matter most, and FARA collaboration with all stakeholders to develop meaningful endpoints expedites FA treatments.

---

## Capitol Hill Updates

---



### Office of Management and Budget Proposed Regulation for Federal Assistance

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.



Together, these changes could have wide-ranging impacts on the research ecosystem and the pace of scientific progress. FARA is closely monitoring the potential impact on the FA research community and will keep the community updated on advocacy opportunities that develop in response to this proposal. Stay tuned!

---



## **Support Rare Disease Innovation Hub at FDA**

In July 2024, the FDA formed the Rare Disease Innovation Hub to help address challenges in rare disease treatment development by improving collaboration between FDA, patients, advocates, and researchers and to facilitate communication between the three FDA research divisions. It has successfully launched multiple public meetings and is ready to grow the reach of this program. Join The EveryLife Foundation in is urging Congress to provide \$5 million in funding for the FDA's Rare Disease Innovation Hub (RDIH) for Fiscal Year 2027 [here](#).

---

## **Rare Disease Congressional Caucus Briefing**



The Rare Disease Congressional Caucus held a June 2 briefing titled “The Pathway to Cures and Treatments for Rare Diseases.” FARA’s Director of Advocacy, Brigid Brennan, and Advocacy Program Coordinator, Berkley Bell, attended with NAF partners Lauren Moore and Courney Bockhop. The event allowed patient advocacy groups to educate industry and congressional staff on current regulatory challenges and the importance of strengthening the pathways for rare disease treatments.

---

## ACT for ALS Reauthorization is Introduced



The ACT for ALS Reauthorization Act of 2026 ([H.R. 8205 / S. 4472](#)) has been introduced in the U.S. House and U.S. Senate, bringing us closer to reauthorizing a law that strives to expedite research for those living with ALS and other rare neurodegenerative diseases and their families. Since passage in December 2021, the ACT for ALS has provided over \$20 million in FDA grants for rare neurodegenerative

disease research and commissioned a regulatory action plan from FDA to chart the way forward.

ACT for ALS programs will expire on September 30, 2026, without congressional action. Visit [this link](#) to urge your Members of Congress to support H.R. 8205 / S. 4472.

---

## American Cures Act is Reintroduced

On May 12, Sen. Dick Durbin (D-Ill.) reintroduced the American Cures Act ([S. 4494](#)), legislation that would provide guaranteed steady growth in funding for research supported by the NIH, CDC, DoD, and VA. [Read the Senator's press release here.](#)

---



## FDA Advances Real-Time Clinical Trial Initiatives

The FDA announced new efforts to modernize drug development by launching “real-time” clinical trials, which allow safety data and results to be shared with the agency as they happen rather than after long delays. The agency hopes that real-time trials could make the drug development process faster and more efficient, helping promising treatments reach patients more quickly.

[Read More](#)

---

## Upcoming Advocacy Events

---

### Registration Open for Rare Across America 2026



Registration is now open for Rare Disease Legislative Advocates' Rare Across America 2026. This advocacy initiative offers rare disease advocates the opportunity to meet virtually and in-district with Members of Congress from August 10–21 to discuss community policy priorities and share personal stories. Registration closes July 17.

[Register Here](#)

---

## Announcements

---

### Pediatric Inclusion Alliance Roundtable Urges Early Inclusion=Better Outcomes



The Pediatric Inclusion Alliance, of which FARA is a founding member, is a multi-stakeholder collaboration committed to earlier inclusion of children in clinical trials. FARA's President Ron Bartek helped organize and moderate a May 5 roundtable that brought together all stakeholders: industry, FDA and NIH leadership, patient advocates, and academia, to reaffirm regulatory leadership's commitment, highlight the successes of prior programs, and collaborate on next steps to advance this initiative.

---

## **FARA's Director of Advocacy Presents at the Yale Innovation Summit**



On May 28, FARA Director of Advocacy Brigid Brennan spoke on a panel titled "How Patient Advocacy Accelerates Early-Stage R&D" at the 2026 Yale Innovation Summit. The Yale Innovation Summit is the Northeast's largest innovation event and brings together experts in arts, biotech, civic, climate, health, and tech.

---

## **EveryLife Foundation Scientific Workshop**

On May 12, The EveryLife Foundation hosted a scientific workshop titled “Making What Matters Count: Advancing Patient-Centered Rare Disease Therapy Development Methodologies, Tools, and Knowledge Management.” FARA’s Advocacy Program Coordinator, Berkley Bell, shown here with NAF partners Andrew Rosen and Courney Bockhop, attended the workshop, which brought together patient advocacy groups, industry, and the FDA to discuss patient-centered rare disease therapy development.



---

## Noah Griffith Shares His “Being Positioned” Baseball Journey to San Francisco



Last year, Noah Griffith traveled to San Francisco for an incredible, baseball-themed trip with friends and family. This adventure was the courtesy of Being Positioned, an

organization that creates unforgettable trips and retreats for people living with neuromuscular diseases.

Before the journey, Noah, an avid baseball fan, shared what the experience meant to him:

*“Visiting all 30 MLB ballparks has become my top bucket-list item, and the fact that I still have 22 parks left to see reminds me there is still a lot of good in life left ahead of me despite the ongoing effects of FA. Receiving a Being Positioned journey reminds me of the joy available on this good but hard life I’m living. When dark days come (because they will), I will look back on this trip with a heart full of gratitude.”*

The journey included stops at two new ballparks and time exploring new cities. First, Noah attended the Giants vs. Dodgers game at San Francisco’s Oracle Park, followed by a trip to Sacramento to see the Athletics play against Noah’s favorite team, the Atlanta Braves.

Noah was recently invited to speak at Being Positioned’s annual gala, where he spoke about the impact of the trip and shared a video that captured the experience. The evening was a huge success.

You can watch the video [here](#), read more about Noah’s trip [here](#), and learn how to apply for a Being Positioned journey [on their website](#).

---

## Now Open: Rare Family Day 2026 Travel Stipend Application



The EveryLife Foundation is excited to invite families to apply for travel stipends to attend Rare Family Day 2026 on October 3. Hosted by Ultragenyx at its campus in the San Francisco Bay area, this special event brings rare families together from across the country for a day of connection, celebration, and fun.

Rare Family Day features activities, food, advocate speakers, a Rare Artist display, and more, creating a fun and welcoming environment for families to connect and celebrate together. The travel stipend application is open through June 26. Visit [this link](#) to apply.

---

## State News

---

### Vermont Passes House Bill 46



Vermont [H. 46](#) was signed by Governor Phil Scott on May 18, establishing a rare disease advisory council in Vermont. This milestone is the result of two years of collective effort and support from rare disease advocates, including FARA Ambassador Mary Nadon Scott! [Read more.](#)

---

### Connecticut Passes Senate Bill 413



Peace Love ACCESSibility—led by proud members of the FA community, Mary Caruso and Alex Bode—helped pass [Connecticut Senate Bill 413](#), which will bring several important improvements to Connecticut’s accessible parking program:

- The creation of Accessible Parking Awareness Day, helping shine a light on why these spaces matter and who they are truly for which will be the second Monday in July, yearly
- Clearer expiration visibility on placards, making it easier for law enforcement to identify valid permits
- A QR code added to the medical certification form, giving providers quick access to guidance and education
- A requirement for healthcare providers to identify and circle the qualifying criteria, helping ensure clarity, consistency, and integrity in the process

---

## Vermont Issues an FA Awareness Day Proclamation

Thanks to the leadership of FARA Ambassador Mary Nadon Scott, Vermont Governor Phil Scott signed a proclamation making May 16 Friedreich’s Ataxia Awareness Day in Vermont!

For information and guidance on how you could get an Ataxia for FA Awareness Day proclamation issued in your state, visit [FARA’s Proclamation Toolkit!](#)

**State of Vermont  
Executive Department  
A Proclamation**

**WHEREAS,** Friedrich's Ataxia (FA) is a rare, progressive, inherited neurodegenerative disease that affects approximately 1 in 50,000 people worldwide, often beginning in childhood or adolescence; and

**WHEREAS,** FA causes progressive damage to the nervous system, leading to symptoms including loss of coordination, muscle weakness, fatigue, and in many cases, life-threatening heart complications; and

**WHEREAS,** there is currently no cure for Friedrich's Ataxia, and available treatments are limited to managing symptoms and improving quality of life; and

**WHEREAS,** increased awareness, research, and support are critical to finding effective treatments and a cure, as well as to improving the lives of those living with FA and their families; and

**WHEREAS,** individuals affected by FA, along with their caregivers, medical professionals, and advocates, demonstrate extraordinary strength, resilience, and commitment in the face of adversity; and

**WHEREAS,** Friedrich's Ataxia Awareness Day serves as an opportunity to educate the public, honor those affected, and commit to supporting research and advocacy efforts that will help cure for those who brave this disease every day.

**NOW, THEREFORE,** I, Philip B. Scott, Governor, hereby proclaim the 16<sup>th</sup> day of May, 2026 as

**FRIEDREICH'S ATAXIA AWARENESS DAY  
in Vermont.**

Given under my hand and the Great Seal of the State of Vermont on this 15th day of May, A.D. 2025.



*Jaye Pershing Johnson*  
Jaye Pershing Johnson  
Secretary of Civil and Military Affairs

*Philip B. Scott*  
Philip B. Scott  
Governor

---

## **Medicaid and CHIP Access Commission (MACPAC) Regulatory Recommendations on State Medicaid**



MACPAC is a legislative branch agency that makes recommendations to Congress, the Department of Health and Human Services, and states on a wide array of issues affecting Medicaid. At its May 2026 meeting, MACPAC made recommendations to Congress and CMS on how states should implement Medicaid Policy.

The recommendations emphasized that automation should not replace human judgment, calling for plans to disclose the use of automated tools and include human reviewers in Medicaid prior authorization. MACPAC also urged CMS to develop a



meeting information can be found [here](#).

- **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, July 23. Additional meeting information can be found [here](#).
- **Connecticut:** June 23 (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. To join the monthly meeting or see other events held by the Connecticut RDAC, click [here](#).
- **Tennessee:** The Tennessee RDAC meets on the fourth Wednesday of every other month. They will be meeting on Wednesday, July 22. If you are interested in joining the meeting, please email [info@tnrdac.org](mailto:info@tnrdac.org) for instructions on attending. Additional meeting information can be found [here](#).
- **Maryland:** June 9. 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, June 9. Additional meeting information can be found [here](#).
- **Nevada:** The Nevada RDAC meets on the first Friday of each even numbered month. They will be meeting on Friday, June 5 at 9:30 a.m. PT. You can find meeting information [here](#).
- **Colorado:** The Colorado RDAC will be meeting virtually on June 8 from 9:00-11:00 AM. You can find additional meeting information [here](#).
- **New Jersey:** The New Jersey RDAC will be meeting virtually on June 9 from 2:00 to 3:30 PM. You can find additional meeting information [here](#).
- **Michigan:** The Michigan RDAC will be meeting on July 22 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](mailto:berkley.bell@curefa.org).

---



[View this email in your browser](#)



*Copyright (C) 2026 FARA. All rights reserved.*

Our mailing address is:

Want to change how you receive these emails?

You can [update your preferences](#) or [unsubscribe](#)