FARA Friedreich's Ataxia Research Alliance

2024 United Against Ataxia Hill Day

On September 25, 2024, on International Ataxia Awareness Day, FARA and the National Ataxia Foundation (NAF) will host the 6th United Against Ataxia Hill Day!

This is a wonderful opportunity for patients and families to meet with their legislators and advocate for policies that will affect them and promote ataxia research. Using your voice to advocate can have a huge impact! Your story can help garner support for policies that will ensure research funding, help patients and families access clinical trials, ensure that patients can access specialists and treatments, and incentivize the development and approval of potential treatments.

In prior Hill Days, FA advocates have successfully advocated for:

- a Senate Resolution recognizing September 25 as National Ataxia Awareness Day,
- passage of numerous pieces of legislation like the Federal Aviation Reauthorization Act which included accessibility reforms ensuring that patients could access clinical trials easier, and
- the inclusion of “Hereditary Ataxia” in the Congressionally Directed Medical Research Program (CDMRP) which opened up millions in grant funding for FA researchers! The success of the CDMRP initiative alone resulted in doubling the funds available for FA research.

These successes, and future ones, are not possible without the support of the ataxia community. Legislators can only make changes when they

know and understand the issues. Sharing your lived experience can make that happen!

You can register for the 2024 United Against Ataxia Hill Day here.

- No experience is required! You will receive a link for training videos which you can watch at your convenience. The training will take less than one hour to review.
- Once your training is completed, you will return a short survey to us to let us know that we should schedule your Congressional meetings.
- Your meeting schedule will be sent on Monday, September 16.
- You will be scheduled for 2-4 meetings with other ataxia advocates, including one “Team Lead” who will coordinate the meeting. No one will be alone.
- You will also receive a zoom link to meet with your team on September 18 at 7 pm to prepare for your meetings.
- **Registration and training closes on Friday, August 30. Sign up today!**

If you have any questions, please contact Emma Potter at emma.potter@curefa.org.

Register for Hill Day 2024!

Capitol Hill Updates

FY25 Appropriations

Every year, the federal government must pass a budget. This process is called appropriations, and it is a very important time for the FA community. During this time, Congress decides how much money important agencies, like the NIH and FDA, will get for the next year. This funding affects research, drug approvals, and many other areas of policy important for the FA community.

The cycle begins when the President submits his budget to Congress. He is supposed to do that by the first Monday of February, but often it is done later. The President's budget acts as a guide for Congress, allowing them to develop a budget that aligns with or differs from that of the President. Both the Senate and the House have an Appropriations Committee, made up of twelve subcommittees, each covering a different area of funding. A bill is moved from each subcommittee to the floor for a vote. This process happens independently in both the House and the Senate. Any differences will need to be reconciled afterward. Since the federal fiscal year begins on October 1st, this process needs to be completed by Congress, and signed by the President, by September 30.

Congress is currently working to pass the Fiscal Year 2025 (FY25) budget. The House passed five of the twelve spending bills before they left for August recess. The seven remaining bills, including the one affecting the NIH (Labor, Health, and Human Services) and the FDA (Agriculture-FDA), will not see movement until the House returns in September. The Senate has not passed any appropriations bills yet. With the upcoming election in November, there is a possibility that we will not see a FY25 budget pass by September 30. Rather, Congress may pass something called a "Continuing Resolution," or CR, which is a temporary spending bill that prevents a government shutdown until a budget is passed.

A CR would hold research agencies to their budget from last year. It would not allow for inflation or permit new programs to start. Research agencies are hampered when funding is insecure in both recruiting new talent and competing with industry positions. This budget bandaid jeopardizes advancing research and delays potential treatments.

For more information on how appropriations work, watch this short video from the Coalition for Health Funding.

The Harley Jacobsen Clinical Trial Participant Income Exemption Act (H.R. 7418)

Representatives Mike Kelly (R-PA-16) and Chrissy Houlahan (D-PA-6) introduced the Harley Jacobsen Clinical Trial Participant Income Exemption Act (H.R. 7418). This bill would make any compensation or

reimbursements from eligible clinical trials tax-exempt for patients and families. This will help mitigate costs associated with clinical trial participations and incentivize patients to participate in clinical trials.

Accelerating Kids Access to Care Act (H.R. 4758)

On June 12, the Accelerating Kids Access to Care Act (H.R. 4758) passed unanimously out of the House Energy and Commerce Committee. The Accelerating Kids' Access to Care Act would provide a streamlined way for pediatric providers to become participating providers in out-of-state Medicaid plans and provide for out-of-state access for pediatric patients. The bill will now head to the House floor for a chance to pass out of the House and move to the Senate, where a companion bill (S. 2372) already has 41 cosponsors.

Creating Hope Reauthorization Act Introduced in Senate (S. 4583)

On June 18, Senators Casey (PA) and Mullin (OK) introduced the Creating Hope Reauthorization Act (S. 4583). This bill, which mirrors the House Creating Hope Reauthorization Act (H.R. 7384), would extend the Priority Review Voucher program for six years. The Priority Review Voucher (PRV) Program provides vouchers to companies who gain approval for drugs targeting rare pediatric diseases. This voucher can be used by the company to expedite FDA review of a subsequent drug or be sold to another company. This is a very important incentive program which will end on September 30 if it is not reauthorized.

FDA Rare Disease Innovation Hub

Big news from the FDA! On July 17, the FDA announced plans to establish a Rare Disease Innovation Hub. (FDA press release) This exciting initiative will be jointly managed by the CBER and CDER divisions and will work across rare diseases to encourage and guide drug development. The Hub will serve three primary functions: serving as a single point of engagement with the rare disease community, enhancing inter-center collaboration on important issues impacting rare disease therapy development, and advancing regulatory science and innovating the development process for rare disease treatments.

FARA Attends Alliance for Regenerative Medicine (ARM) Congressional Fly-In

On July 15 and 16, FARA Advocacy Program Coordinator, Emma Potter, attended the ARM Congressional Fly-In, an event centered around advocating for policies that promote development of and access to cell and gene therapies. FARA joined advocates in advancing:

- The reauthorization of Pediatric Priority Review Vouchers (PRV), which incentivizes the development of pediatric rare disease treatments;
- The Accelerating Kids' Access to Care Act, which would make it easier for patients on Medicaid to access pediatric specialists out of state;
- The MVP Act, which would allow for value-based payments for cell and gene therapies for patients on Medicaid.

State News

California Rare Disease Advisory Committee (RDAC) Progress

Earlier this summer, the California State Assembly passed AB2613, a bill that would establish a Rare Disease Advisory Committee (RDAC) in the state, without opposition. The bill has now been referred to the California State Senate. If this bill passes the Senate and is signed by the governor, rare disease patients will have a much-needed voice in policy decisions that affect the rare disease community.

On August 5, the bill was referred to the California State Senate Appropriations Committee, where it is awaiting a suspense file hearing scheduled for August 15.

Step Therapy Bill Passes Both Chambers in New York

On June 5, the New York Assembly voted to pass New York Senate Bill 1267. This bill prohibits health plans from the following:

- Requiring patients to try a drug that is not FDA approved or is not supported by current medical guidelines as a prerequisite for coverage of a prescription requested by the patient's provider.
- Requiring patients try and fail on more than two drugs within one therapeutic category as a prerequisite for coverage of a prescription requested by the patient's provider.
- Requiring the use of a step-therapy drug for longer than 30 days or a duration of treatment supported by current evidence-based treatment guidelines.

Colorado Governor Signs Prior Authorization Reform into Law

Colorado Governor Polis signed HB 24-1149, making several important changes to the laws that regulate the prior authorization practices of state-regulated health plans. The new law requires health plans to publicly post certain prior authorization data and requirements, eliminate ineffective practices, and collaborate with providers to determine ways to reduce administrative burdens.

North Carolina Comes Closer to Joining the Interstate Medical Licensure Compact (IMLC)

On May 22nd, the North Carolina Senate Committee on Health passed HB681. This legislation would authorize North Carolina to join the Interstate Medical Licensure Compact (IMLC), and it now moves to the Senate Finance Committee for consideration. The IMLC is an agreement between participating states that makes it easier for providers to practice health care in states outside of where they aren't licensed. This would greatly impact FA patients in North Carolina, as it would make it easier for patients and families to access out-of-state specialists.

NORD Issues a Call for Statements in Support of Rare Disease Advisory Councils (RDACs)

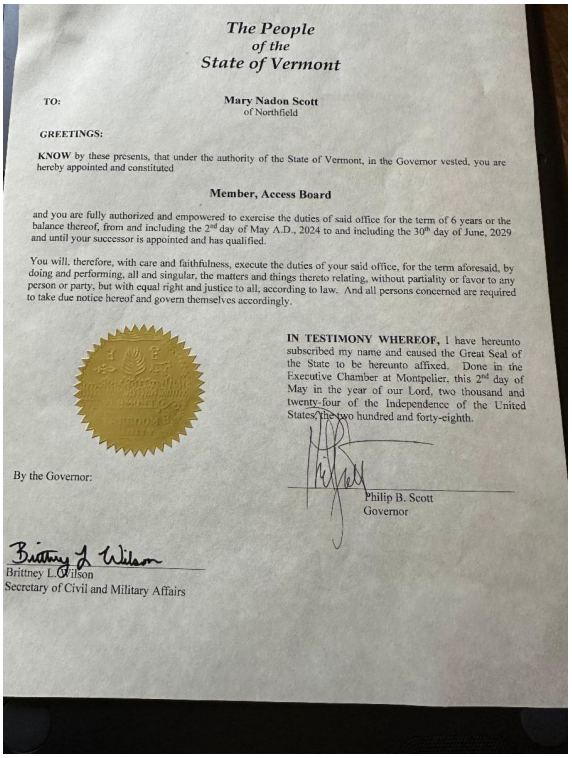
NORD is looking for individuals to submit an RDAC Supporter

Statement. This would include short testimonials (3-5 sentences) about why you support a state Rare Disease Advisory Council! They also ask that you submit a photo alongside your statement of support so that legislators can put a face to your story! They will share the statements and photos directly with State Senators and Assemblymembers. **How to submit? Fill out this form and then email your photo to csheridan@rarediseases.org.**

Announcements

FARA Ambassador Appointed to State Panel

Congratulations to FARA Ambassador Mary Nadon Scott for her appointment as a patient representative to the Vermont Access Board.



The People
of the
State of Vermont

TO: Mary Nadon Scott
of Northfield

GREETINGS:

KNOW by these presents, that under the authority of the State of Vermont, in the Governor vested, you are hereby appointed and constituted

Member, Access Board

and you are fully authorized and empowered to exercise the duties of said office for the term of 6 years or the balance thereof, from and including the 2nd day of May A.D., 2024 to and including the 30th day of June, 2029 and until your successor is appointed and has qualified.

You will, therefore, with care and faithfulness, execute the duties of your said office, for the term aforesaid, by doing and performing, all and singular, the matters and things thereto relating, without partiality or favor to any person or party, but with equal right and justice to all, according to law. And all persons concerned are required to take due notice hereof and govern themselves accordingly.

IN TESTIMONY WHEREOF, I have hereunto subscribed my name and caused the Great Seal of the State to be hereunto affixed. Done in the Executive Chamber at Montpelier, this 2nd day of May in the year of our Lord, two thousand and twenty-four of the Independence of the United States the two hundred and forty-eighth.

Philip B. Scott
Governor

By the Governor:

Brittney L. Wilson
Secretary of Civil and Military Affairs

RDLA recently hosted the second Virtual Youth and Teen Advocacy Day where 55 advocates between the ages of 10 and 18 from 23 states participated in 81 meetings with Members of Congress. The youth and teen advocates shared their rare disease stories with their Members of Congress and asked their Members to support legislation including the Accelerating Kids Access to Care Act and Creating Hope Reauthorization Act. Prior to the meetings, the

youth and teen advocates participated in trainings on how to meet with their Members and how to effectively share their story with Congress.

Rare Artist Contest is now Open for Submissions

Enter August 1 – September 13 at RareArtist.org
The Rare Artist program, powered by the EveryLife Foundation for Rare Diseases, was established in 2010 to showcase the talents of those impacted by rare diseases. The annual contest provides a national platform for artists to advocate through visual artwork and poetry.

Click here for more information about contest guidelines and awards.

Click here to enter

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