FARA Friedreich's Ataxia Research Alliance

The 6th United Against Ataxia Hill Day is TOMORROW! You Can Still Be a Part of It!

Advocacy is essential to our mission of mobilizing the resources and relationships required to advance treatments for Friedreich's ataxia. It is a powerful tool that amplifies our efforts and helps us reach our goals. By sharing your FA experience through advocacy efforts, you not only raise awareness but also educate lawmakers about the real-world impacts of their policies, motivating them to take decisive and meaningful action.

Even if you weren't able to participate in Hill Day, you can still use your voice to create change! Here's how:

- Find your two Senators here and your House Representative here. You should have the option to "contact" them, which will take you to a form from their office.
- Use this form to share your story and discuss how ataxia has impacted you and your family.
- Don't forget to ask them to support the initiatives listed below!
- You can use the template below or write your own message!

Our Hill Day Initiatives:

- **Pass a short-term CR and then the FY25 budget.** The deadline to pass the budget is Sep 30. A government shutdown or a long Continuing Resolution (CR) would not allow for the necessary growth, recruitment, or funding needed for research to expedite treatments for ataxia. This will slow research and significantly impact the ability for projects to

get funded. It is likely that Congress will need to pass a CR this week to stay open. Our ask: pass a short-term CR so that a FY25 budget can be passed asap.

- **Senate only: Support the continued inclusion of "Hereditary Ataxia" in the Peer Reviewed Medical Research Program (PRMRP) within the Congressionally Directed Medical Research Program (CDMRP) as listed in the draft FY25 defense appropriations bill.** While we have an approved treatment for FA, we have no treatments that address the underlying cause of FA and no treatments approved for those under 16 years of age. Furthermore, there are no treatments approved that address the underlying cause for the more than 70+ types of hereditary ataxia. The funding provided by CDMRP would expedite critical research and help bring treatments to the ataxia community. The House passed the defense appropriation bill already.

- **Senate only: Pass Creating Hope Reauthorization Act (H.R. 7384 / S. 4583)** This bill was incorporated into Give Kids a Chance Act. (H.R. 3433) and passed the House on 9/24! No Senate action yet but the Senate HELP committee is reviewing on Thursday 9/26. This bill would extend the Pediatric Priority Review Voucher (PRV) program which is a crucial incentive to get pharmaceutical companies to develop treatments for rare diseases affecting pediatric populations. The program has been successful at bringing rare disease treatments to market, but it is set to expire on September 30, 2024. A CR would also extend this program until the CR expires.

- **Senate only: Cosponsor and pass the Accelerating Kids' Access to Care Act (H.R. 4758/S.2372).** Many ataxia patients have to seek care out-of-state due to a lack of in-state providers knowledgeable about hereditary ataxia. For many patients on Medicaid, they are unable to get coverage for out-of-state care. This bill would allow states to implement a streamlined process to enroll out-of-state pediatric providers in other states' Medicaid programs, allowing patients to receive coverage for services from these providers. This bill will help patients access the care that they need without having to pay out-of-pocket or fight with insurance. The House has already passed this bill.

- **Cosponsor and pass the BENEFIT Act (H.R. 4472/S.373).**This bill will ensure the patient perspective is considered by FDA reviewers who are evaluating drugs and other medical products. The patient experience data (patient reported outcomes, patient testimonials, and natural history data) would be included in the risk-analysis when reviewing new drug applications.

- **Senate only: Pass Retaining Access and Restoring Exclusivity (RARE) Act (S. 1214 / H.R. 7383)** This bill was incorporated into Give Kids a Chance Act. (H.R. 3433) and passed the House on 9/24! No Senate action yet. This bill would codify the FDA practice of recognizing exclusivity on approval which would incentivize companies to continue investing in therapeutics across the disease population.

Here's a template to use when reaching out to your Members of Congress (you need to edit the highlighted sections). If you have any questions about reaching out to your Members of Congress, please feel free to contact Emma Potter at emma.potter@curefa.org.

Hello Rep./Sen. ___,

My name is [YOUR NAME], and I am a constituent of yours affected by hereditary ataxia. There are over 70+ types of hereditary ataxias, which are rare genetic neurological disorders affecting mobility, coordination of movement, speech, and much more.

[Insert more of your personal story here if you would like]

95% of the over 10,000 known rare diseases have no approved treatments, and many hereditary ataxias are no exception. As of now, there are no treatments addressing the root cause of the disorder, and the treatments available only help to manage symptoms and/or slow progression. This is why adequate funding for agencies like the NIH and FDA is so crucial. Funding these agencies helps accelerate potential treatments and gives patients and families hope. Thus, I ask that you do all that you can to ensure a timely FY25 budget or a short CR.

Additionally, the inclusion of hereditary ataxia in the Peer Reviewed Medical Research Program (PRMRP) within the Congressionally Directed Medical Research Program (CDMRP) has helped fund crucial research on hereditary ataxias. The continued inclusion of "Hereditary Ataxia" in the program will continue to fund research that may lead to potential treatments and help improve the lives of patients and families impacted by ataxia.

For House Members:

There are also several other pieces of legislation that would help incentivize the development of treatments for rare diseases, ensure access to care across state lines, and make sure that patient voices are represented in the research and development of treatments. First and foremost, I want to thank you for passing the Accelerating Kids' Access to Care Act (H.R. 4758/S.2372) and the Give Kids a Chance Act (H.R. 3433). These bills will have significant impacts on the ataxia community by ensuring that patients on Medicaid are able to receive coverage for out-of-state care.

However, there are still one crucial bills that have yet to be passed, namely the BENEFIT Act. Therefore, I ask that you cosponsor and/or support the BENEFIT Act (H.R. 4472/S.373). This bill would help the ataxia community by ensuring that patients are able to have their voices represented in the drug approval process.

For Senate Members:

Thank you for passing the Senate Resolution recognizing September 25 as "National Ataxia Awareness Day." Raising awareness is an important first step towards ending ataxia. There are also several other pieces of legislation that would help incentivize the development of treatments for rare diseases, ensure access to care across state lines, and make sure that patient voices are represented in the research and development of treatments. Therefore, I ask that you cosponsor and/or support the Give Kids a Chance Act (H.R. 3433), which now includes the Creating Hope Reauthorization Act (H.R. 7384/S.4583) and the RARE Act (H.R. 7383/S.1214), the Accelerating Kids' Access to Care Act (H.R. 4758/S.2372), and the BENEFIT Act (H.R. 4472/S.373). These bills would help the ataxia community by ensuring that patients and families are able to have access to care and quicker access to treatments, as well as have their voices represented in the drug approval process.

Thank you for your time and consideration, and please don't hesitate to reach out with any additional questions.

Best,

[YOUR NAME]

Capitol Hill Updates

2024 National Ataxia Awareness Day Resolution Passed

The National Ataxia Awareness Day Resolution (S.Res 794) passed the U.S. Senate with unanimous consent. The resolution designated September 25, 2024, as National Ataxia Awareness Day, and this legislation elevates the awareness needed to improve the lives of those affected by ataxia.

NAF and FARA, along with our volunteer advocates, work with members of Congress to obtain this designation each year. We'd like to thank the Resolution Sponsor Senator Hyde-Smith (MS) and Cosponsors Senators Stabenow (MI), Moore Capito (WV), and Murphy (CT) for their continued support of National Ataxia Awareness Day and for championing awareness and treatment development for Ataxia!

Accelerating Kids' Access to Care Act Passes the House

On September 17, the U.S. House of Representatives voted to pass the Accelerating Kids' Access to Care Act (H.R. 4758). This bill would allow states to implement a streamlined process for pediatric providers from out-of-state to enroll as participating providers in other states' Medicaid programs. This will allow patients and families on Medicaid to access and receive coverage for the care they need out-of-state. The bill is now in the U.S. Senate and has been

referred to the Committee on Finance for consideration.

Upcoming Advocacy Events

FARA will join RDLA and rare disease patients and families at the Pennsylvania State Advocacy Day to advocate for policies to help the Pennsylvania rare disease community. Registration for this event has closed but you can still be a part of it by emailing or calling your state members. Many healthcare policies that affect the rare disease community are made at the state level, and your advocacy can help improve the lives of many patients and families across your state.

FDA Public Meeting on Rare Disease Innovation Hub

The Food and Drug Administration (FDA) is hosting a public meeting titled "Advancing Rare Disease Therapies Through an FDA Rare Disease Innovation Hub." The purpose of the public meeting is to discuss the establishment of a Rare Disease Innovation Hub, which will enhance collaboration and cooperation across the Center for Biologics Evaluation and Research (CBER) and the Center for Drug Evaluation and Research (CDER), as well as other centers and offices across FDA, to advance rare disease therapies. The public meeting will be held on October 16 from 10:00am to 1:00pm ET. Either electronic or written comments on this public meeting must be submitted by 11:59pm ET on October 31. To learn more or to register, click here.

State News

California Rare Disease Advisory Committee (RDAC) Progress

California's RDAC bill, AB2613, passed through both the Assembly and Senate unanimously and is now headed to Governor Newsom's desk for his signature. Once signed, California will be the 29th state to establish an RDAC. You can track the bill here.

NORD has issued a call to action to help get this approved and signed into law! **Add your support by clicking HERE to send Governor Newsom a message requesting his signature for approval.** Raise your voice to encourage Governor Newsom to create CA's RDAC!

Virginia Issues 2023 Newborn Screening Report

In 2023, the Virginia General Assembly directed the Department of Health and the Department of General Services to convene a workgroup to evaluate the current funding model for Virginia's newborn screening program. Over the past year, stakeholders:

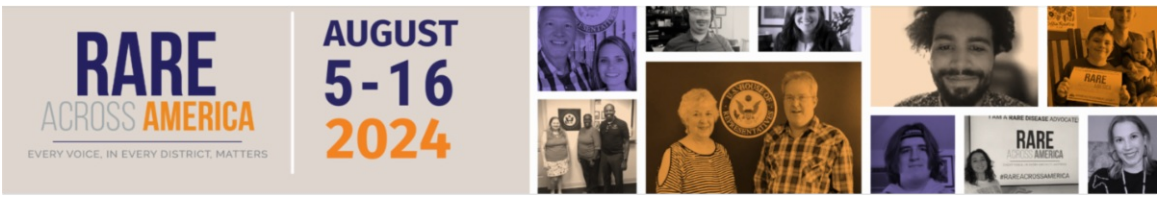
- Reviewed the current funding model for newborn screenings
- Heard presentations from other state newborn screening programs on alternative funding models
- Received public comments

The recently released workgroup report concluded that the best funding model for Virginia will depend on the priorities of the General Assembly. This report is a clear signal to the General Assembly that it is time to determine what the program should look like. For more information, read the report here and visit EveryLife's newborn screening action center here.

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. Learn more about RDACs here.**

Announcements



Rare Across America is a huge success!

FARA joined **425 rare disease advocates** from around the country to participate in Rare Across America last month! **49 states and D.C.** were represented in **242 meetings** with Members of Congress. The impact made by advocates will benefit rare disease patients and families for years to come. To

learn more about the legislative asks, visit EveryLife's website here.