FARA Friedreich's Ataxia Research Alliance

Advocacy News

There has been a lot of action happening since our last advocacy newsletter. The 5th United Against Ataxia Hill Day was held, FARA prepared for six Medicaid hearings, and the FY23 CDMRP grants were announced. As 2023 comes to a close, it is a great opportunity to review the amazing work done by the FA community over the last few months.

Hot off the presses! The FY23 grant recipients were just announced and we are thrilled to share that **seven Hereditary Ataxia grants were recommended for funding totaling almost $10.5 million!**

That brings the total recommended for two years to almost **$24.5 million.**

This funding was made possible through the time and commitment of so many advocates in the ataxia community, the support of many Congressional Members, and the dedication of outstanding researchers. Amazing work team!

CONGRATULATIONS TO THE FOLLOWING RECIPIENTS:

DAVID LYNCH AND DON JOSEPH, CHILDREN'S HOSPITAL OF PHILADELPHIA, PA

LAURA RANUM AND MONICA BANEZ-CORONEL, UNIVERSITY OF FLORIDA

ROB WILSON AND LIMING PEI, CHILDREN'S HOSPITAL OF PHILADELPHIA, PA

YINA DONG, CHILDREN'S HOSPITAL OF PHILADELPHIA, PA

YUHO KIM, THE UNIVERSITY OF MASSACHUSETTS, LOWELL, MA

SHIANG LIM, ST VINCENT'S INSTITUTE OF MEDICAL RESEARCH, AUSTRALIA

JOAN O'KEEFE, RUSH UNIVERSITY MEDICAL CENTER, CHICAGO, IL

5th United Against Ataxia Hill Day 2023

Our 5th United Against Ataxia Hill Day was impressive — 164 advocates from 33 states educated lawmakers on ataxia during 86 Congressional meetings. They urged action in funding research programs (CDMRP, NIH, & FDA) and legislation that would expedite treatments (Federal Aviation Act Reauthorization, the BENEFIT Act, & the RARE Act).

The success of these meetings was a direct result of the time and commitment each advocate took to prepare and deliver our initiatives.

Here is a quick update on where our "asks" are currently:

Appropriations

On November 15, the U.S. Senate voted 87-11 to approve a laddered continuing resolution (CR) to keep the government open and funded at 2023 levels. A laddered CR creates two separate expiration dates for two groups of annual spending bills.

- January 19, 2024, deadline includes Agriculture-Rural Development, Military Construction-Veterans Affairs, Energy & Water, and Transportation-HUD
- February 2, 2024, deadline includes Defense, Financial Services, Homeland Security, Labor-HHS-Education, and more

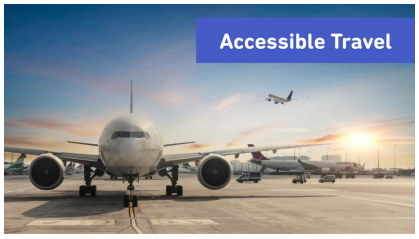
Click here to learn more from our partner the EveryLife Foundation.

The Senate is scheduled to return to session on January 8, and the House on January 9. When the two bodies return, they will have only about ten days to complete action on the Agriculture/FDA, Military Construction and Veterans Affairs, Transportation and Housing and Urban Development, and Energy and Water appropriations bills before the current CR expires on January 19. Speaker of the House Mike Johnson (R-LA) said he will not support further short-term CRs. Instead, he has suggested moving to a full-year CR which would be detrimental to federal research agencies.

CDMRP

The primary “ask” during Hill Day was the continued inclusion of "Hereditary Ataxia" in the CDMRP/PRMRP. Appropriations requests were made last spring for FY24, and the necessary language is included in the Senate draft report. While offices overwhelmingly expressed support in passing this initiative, we are still waiting for the FY24 budget to be passed! Separately, most offices also encouraged us to reach out to them when FY25 appropriations requests opened in the spring. That is huge! It is a clear example of how you are helping to grow ataxia champions on Capitol Hill.

Federal Aviation Act (FAA) Reauthorization



The FAA must be reauthorized every five years and was due to expire on September 30. The House signed off on an FAA reauthorization bill in July, but the legislation has stalled in the Senate. Since then, there have been two extensions which moved the deadline to March 8. The proposed bill includes meaningful reforms which would make air travel more accessible, an important initiative to the FA community. If you would like to contact your Senators to ask them to support the FAA reauthorization, you can do so through our partners at MDA here.

Better Empowerment Now to Enhance Framework and Improve

Treatments (BENEFIT) Act (S. 526 / H.R. 1092)

The BENEFIT Act would amend the Food, Drug and Cosmetic Act (FDCA) to ensure that patient experience, patient-focused drug development (PFDD), and related data – including information developed by a product sponsor or a third party such as a patient advocacy organization or academic institution – be considered as part of the benefit-risk assessment. Several Members have signed on as a co-sponsor of this bill since our Hill Day. If you would like to send a letter to your Representative and Senators asking for their support, you can do it here thanks to our partner Parent Project Muscular Dystrophy (PPMD).

Now that you are aware of what an incredible impact your advocacy can have on ataxia research, make sure to mark your calendar for the 6th *United Against Ataxia Hill Day on September 25, 2024,* which happens to also be International Ataxia Awareness Day (IAAD)!

Access

Medicaid Drug Utilization Review (DUR) Boards and Pharmacy & Therapeutics (P&T) Committees

With the approval of Skyclarys, FARA and the FA community have entered into a new chapter — access! States must review new medications when they come on the market to decide where the medication will be placed on the drug formulary for Medicaid. To facilitate this process, federal Medicaid rules require each state to have a comprehensive Drug Utilization Review (DUR) program that assesses the utilization, quality, medical appropriateness, and cost of prescribed medication through the evaluation of claims data.

Each state has its own process. In some states, the DUR program is run by a DUR Board, and in some states, it is run by a Pharmacy and Therapeutics (P&T) Committee. DUR programs can conduct both prospective and retrospective drug utilization reviews – they can review drugs newly on the market, or drugs that have been available on the market for some time. They only review drugs that have received FDA approval.

Since September, FARA has participated in six state Medicaid hearings

which established the prerequisites needed to approve a Skyclarys prescription. Many states started with a list that included items that you would expect such as a confirmed FA test or a neurological exam, but other states required the mFARS test, the recumbent bicycle test, or ambulation to name a few. FARA, and some amazing community advocates, shared their experiences with FA and were able to successfully persuade the DUR and P&T boards to change their initial list, thereby paving the way for increased access to Skyclarys for everyone in that state.

A huge thank you to our advocates who spoke:
Michigan: Ruth Acton & Jack DeWitt; Maureen, Jake & Claire Juip
Arkansas: Darla Sparacino
Missouri: Sean Sommerville
Colorado: Carrie Bolinger
FARA also spoke in Kentucky and Nevada without additional advocates.

As of the end of November, 28 states did not have policies in place for Skyclarys. Eight of these states do not allow any access prior to policy implementation. The year 2024 will bring many more of these meetings and your voice is needed! Meetings in Arizona, Kentucky and Iowa have already been scheduled. If you are willing to speak at your state Medicaid hearing, please email Kellyn Madden, FARA's Patient Engagement Manager, at kellyn.madden@curefa.org.

Capitol Hill Updates

Congressman Mike Johnson (LA-04) is elected Speaker of the House

On October 25, 2023, Congressman Mike Johnson (LA-04) was elected as the Speaker of the House. As a

Congressman, Speaker Johnson represents the northwest and western regions of Louisiana. Prior to his election to Congress in 2016, Speaker Johnson was an attorney and served in the Louisiana State Legislature from 2015 to 2017.

170 Patient Organizations send letter regarding the IRA

Following the passage of the Inflation Reduction Act of 2022 (IRA), the rare disease community continues to express concerns over the effect of the limited nature of the orphan drug exclusion within the Medicare Drug Price Negotiation Program (MDPNP) on the incentives to invest in pursuing additional uses for already approved rare disease therapies.

FARA joined 170 patient organizations in signing a letter of support urging Congress to consider small technical corrections to the Inflation Reduction Act's orphan drug exclusion that would benefit the rare disease community. As a reminder, the two changes the co-signing organizations are seeking to the IRA's orphan provisions are:

1. Clarify that the number of orphan designations FDA grants a product has no effect on its eligibility for the IRA's orphan drug exclusion; and
2. Maintain the purpose of the orphan drug exclusion by clarifying an orphan product becomes negotiation-eligible 7 or 11 years after it loses that exclusion.

A one-pager with additional information can be found here.

Senators Recognized as Ataxia Research Champions

NAF and FARA presented Senator Shelley Moore Capito (WV) and Senator Debbie Stabenow (MI) with the Ataxia Research Champion award. This award is given to members of Congress who have shown unwavering support for research into Ataxia treatments and cures.

"Ataxia is a heartbreaking disease for patients, families, and caregivers. NAF and FARA are incredible partners in the fight to end this disease," said Senator Stabenow. "I'm honored to receive the Ataxia Research Champion award."

"Establishing National Ataxia Awareness Day will allow us to raise awareness among the American public and shine a light on those impacted," Senator Capito said. "Moving forward, we must continue our dedication towards improving diagnosis and treatment and ultimately find a cure. "

Protect RARE Act Introduced

On October 26, 2023, Representatives Dunn (FL), Doris Matsui (CA) Mike Thompson (CA), Mike Kelly (PA), and Markwayne Mullin (OK) introduced The Providing Realistic Opportunity to Equal and Comparable Treatment for Rare (PROTECT Rare) Act, H.R 6904. This legislation would support patients with rare diseases in getting evidence-based, medically necessary care covered by their health insurance. The bill permits Medicare and Medicaid to use clinical guidelines and peer-reviewed literature to allow for coverage of rare disease treatments and requires private payers to establish an expedited pathway for formulary exceptions, reconsiderations, and appeals related to rare disease treatments. To learn more, click here.

New NIH Leader

Monica M. Bertagnolli, M.D., started on November 9 as the 17th director of the National Institutes of Health, the nation's biomedical research agency and largest public funder of biomedical research in the world. She is the first surgeon and the second woman to hold the position. Nominated by President Biden, Dr. Bertagnolli was confirmed on a bipartisan basis by the U.S. Senate on November 7. She transitioned from

her role as the 16th director of the National Cancer Institute, a position she has held since October 2022.

Upcoming Advocacy Events

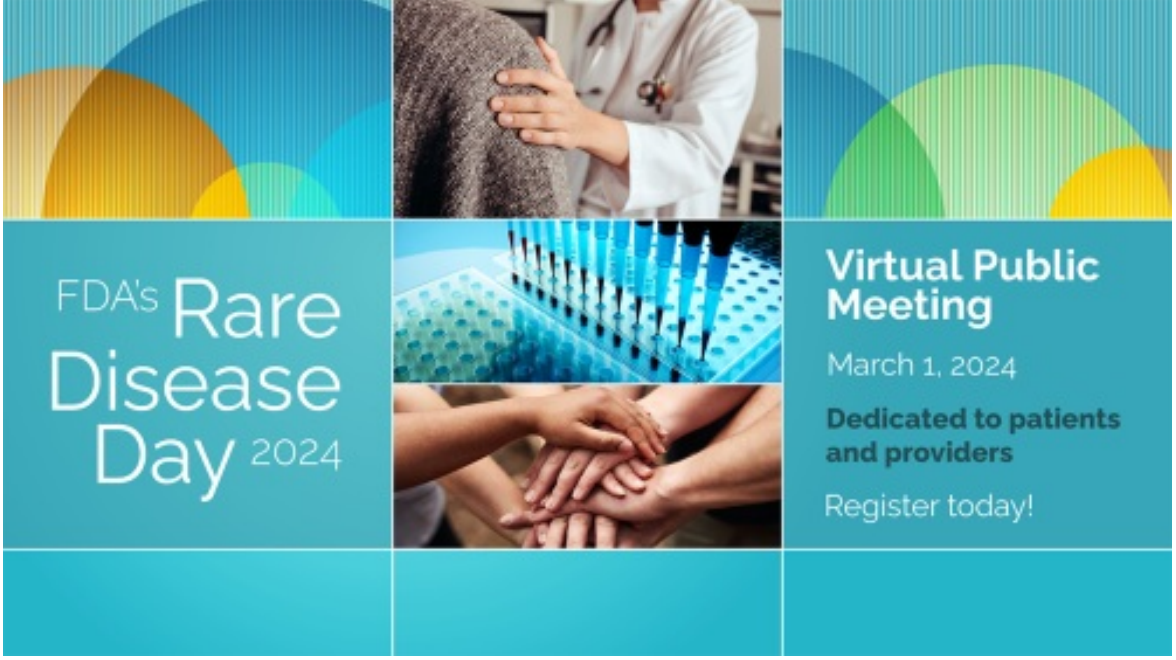
Save the date for Rare Disease Week on Capitol Hill from February 25 to February 28, 2024, in Washington, D.C. This event brings together rare disease advocates from across the country to amplify their voices to Members of Congress. Rare Disease Week includes a documentary screening, legislative conference, YARR meet-up, Rare Disease Congressional Caucus briefing, Rare Artist reception, and meetings with Members of Congress.

RARE DISEASE WEEK ON CAPITOL HILL

Registration will open on Wednesday, January 3. More information here.

RARE DISEASE DAY at NIH
Feb. 29, 2024 | #RDDNIH

Mark your calendars for Rare Disease Day at the National Institute of Health (NIH) on February 29th, 2024 – an extraordinary occasion dedicated to raising awareness for rare diseases and all those who are impacted. Registration for this event opened on December 6. Click here to secure your spot.



Join the Food and Drug Administration (FDA) for their virtual Rare Disease Day on March 1, 2024. This event is dedicated to patients and healthcare professionals all around the world and will delve into various topics addressing the FDA's role in approving studies and medical products, unraveling the intricacies of clinical trials, exploring health technologies, and shedding light on innovative health initiatives. To learn more information and register, click here.

Announcements

EveryLife Lauds Winners of 2023 RareVoice Awards

EveryLife Foundation for Rare Diseases is proud to announce the winners of the RareVoice Awards hosted by Rare Disease Legislative Advocates (RDLA). The awards recognize and celebrate exceptional advocacy efforts in the rare disease community. A full list of winners can be seen here.

FARA extends its congratulations to FA community member Jake Juip for his nomination under the Advocacy by a Youth or Teenager category. Jake's work on the National Ataxia Awareness Day Senate Resolution helped elevate the conversation on ataxia to a national level. Great job Jake!

The Cost of Delayed Diagnosis in Rare Disease – A Breakthrough Study Amplifying the Economic Impact of Delayed Diagnosis

In one of the first studies of healthcare resource utilization and costs for patients with rare diseases, the EveryLife Foundation for Rare Diseases reveals that timely diagnosis and screening can shorten and possibly eliminate the diagnostic odyssey while significantly reducing the cost impact of rare diseases for individuals, families, and the healthcare system. The report, entitled, "The Cost of Delayed Diagnosis in Rare Disease," is a follow-up to the landmark 2022 "National Economic Burden of Rare Disease Study," and provides an in-depth analysis of the avoidable costs associated with seven rare diseases.

The results of the study were unveiled during the Congressional Caucus Briefing on September 14th, titled "*Delayed Diagnosis in Rare Disease: The Economic Cost, Personal Impact, and Policy Solutions*." The study estimates that the avoidable per-patient medical costs and productivity losses attributable to delayed diagnosis of those seven rare diseases range between $86,000 and $517,000 per patient. The benefits of timely intervention are particularly significant in diseases where newborn screening has been implemented in some or all U.S. states, such as ALD, Pompe disease, and SCID. For these three rare

diseases, timely diagnosis can eliminate the diagnostic odyssey, reduce associated medical costs, and provide the opportunity for optimal intervention and improved health outcomes. For more information and to view the Caucus Briefing, you can visit this link.

FDA Launches Pilot Program to Help Further Accelerate Development of Rare Disease Therapies

The FDA is launching a pilot program called the Support for Clinical Trails Advancing Rare Disease Therapeutics (START) to expedite the development of novel drug and biological products for rare diseases. This program offers select sponsors the opportunity for more frequent communication with FDA staff to address clinical development issues, including study design and patient population selection. It's open to sponsors of products currently in clinical trials and regulated by the Center for Biologics Evaluation and Research (CBER) or the Center for Drug Evaluation and Research (CDER).

Applications for the START program will be accepted from January 2, 2024 – March 1, 2024, with up to three participants selected for each center. The agency will assess the pilot's success and may consider a second iteration in the future.

New York Passes Newborn Testing of Duchene Muscular Dystrophy

New York's Governor Kathy Hochul signed bills A 504 and S 6814 which require newborn testing of Duchenne muscular dystrophy. The acts took effect immediately. For more information, please visit here.

EveryLife Foundation Seeking Virginia Advocates to Support Newborn Screening Bill

Early next year, the EveryLife Foundation will work with Virginia patients and lawmakers to introduce legislation to encourage the state to screen newborns for conditions on the federal Recommended Uniform Screening Panel (RUSP). RUSP alignment legislation, which has passed in 11 other states, requires states to screen newborn babies for any disorder on the RUSP, implements a timeline in which the screening must begin, and ensures that resources will be available to fund all conditions added to the RUSP in the future. For more

information on RUSP alignment legislation, please visit our Newborn Screening Action Center.

Of the babies born in the United States, 48% are born in RUSP-aligned states. While great progress, the goal is to have all babies born in the U.S. have equitable access to timely diagnosis and treatment. **In the coming months, the EveryLife Foundation will create a coalition of Virginia advocates to support this bill.**

If you or someone you know is a Virginia resident and would like to participate in additional advocacy in support of this bill, please share your contact information with us by filling out this survey.