FARA Friedreich's Ataxia Research Alliance

Take Action to Help Pass Crucial Pieces of Rare Disease Legislation!

The clock is ticking on this year's Congressional session and many of our advocacy partners are working to get important legislation across the finish line! If Congress isn't able to get these crucial pieces of legislation across the finish line before December 20, they will have to start from scratch and reintroduce each of these bills in the next Congress. An end-of-year health care package is being considered so now is the time to make your voice heard on pressing issues impacting the disease community. Join FARA in asking Congress to pass:

- **The Creating Hope Reauthorization Act (H.R. 7384/S. 4583)** reauthorizes the Rare Pediatric Disease Priority Review voucher program, a crucial incentive for pediatric drug development, for at least five years. This program helped make the first approved treatment for FA a reality, and it could bring more treatments in the future. Without action, it will expire on December 20. **Click HERE to urge your members of Congress to support the reauthorization of the PRV program.**

- **The RARE Act (H.R. 7383/S. 1214)** cements the FDA's long-standing interpretation of how to properly award orphan drug exclusivity, a key drug development incentive established by the life-changing Orphan Drug Act.

- **The Accelerating Kids' Access to Care Act (H.R. 4758/S. 2372)** seeks to address harmful barriers to out-of-state medical care for children with health coverage through Medicaid or the Children's Health Insurance Program (CHIP) by streamlining the provider enrollment process across state lines.

- **The Telehealth Modernization Act (H.R.7623/S. 3967)** extends the Medicare telehealth flexibilities granted during the pandemic, which are set to expire at the end of this year.

To encourage your elected officials to vote YES on the year-end package, the following action alerts have been set up for individuals to fill out for one final push on this important piece of legislation.

NORD Alert

EveryLife Foundation Alert

Capitol Hill Updates

Post-Election Updates

The U.S. 2024 Election occurred on Tuesday, November 5, solidifying an incoming change in leadership in DC. Donald Trump, the Republican candidate, will be sworn in as President, and the Senate will flip control over to the Republican Party by a thin margin (53-47). With the final House races being called earlier this month, it's clear that the Republican Party also narrowly retained the House of Representatives (220-215).

The Senate and the House convened on November 13 to discuss the incoming 119th Congress's priorities and elect leadership. Sen. John Thune (R-SD) was elected as the Senate Majority Leader, who will serve as the chief representative of the Republican Party in the Senate. Similarly, Sen. Tom Cotton (R-AR) was elected as the Majority Whip, and Sen. Shelley Moore Capito (R-WV) was elected as the Republican Policy Committee Chairwoman, both of which will assist in setting the priorities for the Senate in the 119th Congress. Additionally, while the Speaker of the House isn't selected until January, it seems likely that current Speaker Mike Johnson (R-LA) will retain the position.

Finally, many political appointees have been announced by President-elect Donald Trump, leading to speculation about Senate confirmation. Traditionally, the majority of high-level political appointees require vetting and confirmation by the Senate by a simple majority vote. However, recently there have been conversations around recess appointments. **Recess appointments** are allowed under Article II of the Constitution and allow the President to make appointments without confirmation when Congress is on recess. Recess is any period of time when Congress is not in session (ex. holidays, August recess, etc.). These

appointments do not require Senate action, meaning the President can unilaterally make these appointments without Congressional oversight. Once appointed, the person assumes the position for the Congressional term. A Congressional term is two years, with the new term starting in January 2025. So, if a recess appointment was successful, the candidate could hold the office for two years without Congressional review.

Why do recess appointments matter? Cabinet choices have the ability to greatly influence the interpretation and enforcement of policies, as well as priorities for grant funding and the federal budget. These things can all impact the pace of and investment in research and drug development, as well as coverage and access to existing treatments. Therefore, these appointments could significantly impact patients across the country. Additionally, if recess appointments are utilized, there is no way for constituent voices to be represented in the confirmation process, and there is no Congressional oversight to address bipartisan concerns regarding certain appointees.

In the meantime, the 118th Congress is coming to a close, with December 20 being Congress's last day in session before the 119th Congress is sworn into office. There are a number of crucial things that Congress needs to do before the end of the session, including passing a budget or another continuing resolution (CR) and passing crucial pieces of legislation that are very important to the rare disease community.

Ask Congress to Adequately Fund the NIH for FY25!

NIH funding is crucial to driving research forward and bringing new treatments to patients. Every fiscal year, Congress must pass a budget to allocate spending, including funding for the NIH. Current budget proposals are set to cut funding for the NIH, and, as you know, Congress only has until December 20 to pass a budget.

The Endocrine Society has created a call-to-action form for researchers, but you can submit it too! Ask your members of Congress to adequately fund the NIH to continue to advance research. Your voices matter and can make a huge difference in the budget process! Help us ensure that research funding remains available to the FA community!

Temporary Extension of Telemedicine Flexibilities

The Drug Enforcement Administration DEA and the Department of Health and Human Services (HHS) have extended telemedicine flexibilities for prescribing controlled medications through December 31, 2025, continuing the exceptions initially granted during the COVID-19 emergency. This extension allows only for the prescribing of controlled medications (i.e. opioids/narcotics, stimulants, etc.).

There are still a number of telehealth flexibilities that need to be reauthorized, such as those set out in the Telehealth Modernization Act (H.R.7623/S. 3967) listed above. These flexibilities include allowing Medicare beneficiaries to receive care through telehealth for all services (rather than specific services) and

allowing all providers recognized by the Centers for Medicare and Medicaid Services (CMS) to provide telehealth services to their patients.

Upcoming Advocacy Events

RDLA Virginia State Advocacy Day

Registration is open until **Friday, January 10.**

What: A day of action for Virginia residents impacted by rare disease to join together and meet your state legislators, share your stories, and help advance the policy priorities of the rare disease community.

Why: Many healthcare policies that affect the rare disease community are made at the state level. These policies impact diagnosis, treatment, access to care and more. One of the most powerful ways that you can influence policymakers is by meeting with them in person.

Who: This event is open to Virginia residents only. No prior advocacy experience is necessary. Registration for this event and all RDLA events are free for all rare disease advocates. Registration is required for all participants, including spouses and children.

When: January 28 and January 29, 2025. Attendance at the legislative conference on January 28 is required to attend meetings with legislators on January 29.

Mandatory Webinar Training: Tuesday, January 14 at 1:00 to 2:00 PM ET.

Register and Learn More

Announcements

Congratulations to Dr. Shandra Trantham for being named to the EveryLife Foundation Board of Directors!

FARA would like to congratulate Dr. Shandra Trantham on her appointment to the EveryLife Foundation Board of

Directors. Shandra holds the young adult board seat which is only available to graduates of the YARR leadership academy. Shandra has been an FARA Ambassador since 2018 and recently received her PhD in Genetics and Genomics from the University of Florida.

Congratulations to FARA Communications & Social Media Coordinator, Megan Wolf, on her acceptance to EveryLife's YARR Leadership Academy!

The YARR Leadership Academy, overseen by FARA's partner in advocacy, EveryLife, is a series of collaborative online courses that are offered to a select group of young adults (aged 18-30) in the rare disease community. During the 8-week program, participants will learn about the roles and opportunities for patient representation in policy-making, drug development, and the regulatory process and the steps it takes to enter those roles. To end the program, each participant will conduct and present a capstone project that will prepare them to enter rare disease leadership roles in the future.

For more information about the YARR Leadership Academy,click here.

FA Community Member Jake Juip Speaks at Rare Disease Congressional Caucus Briefing

On December 10, Jake Juip delivered a very well-prepared statement at the briefing: Cures and Care for Kids: Game Changing Legislation, on why the Pediatric Priority Review Voucher Program matters. You can view the briefing here.

GAO Study Highlights FDA's Efforts to Advance Rare Disease Drug Development

The US Government Accountability Office's (GAO) newly released study, mandated in the last User Fee reauthorization, examines FDA's rare disease

programs, including its 18 initiatives and the upcoming Rare Disease Innovation Hub to enhance cross-center coordination and expedite drug approvals.

FDA CBER Patient Listening Meeting

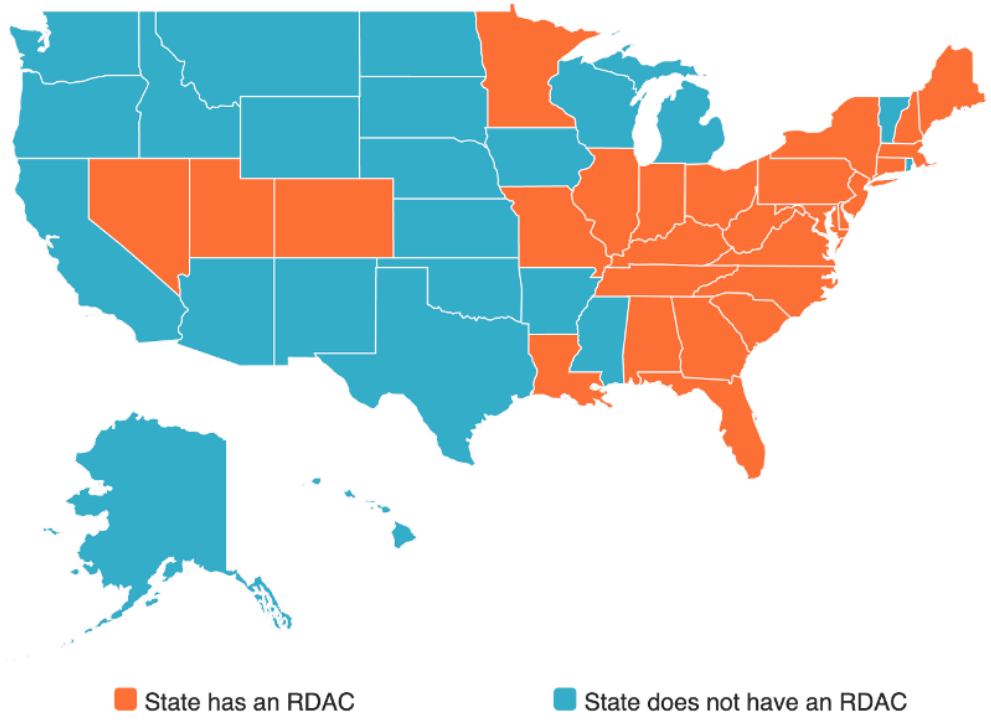
The FDA Center for Biologics Evaluation and Research (CBER) hosted a virtual public patient listening meeting on **Wednesday, December 4, 2024,** to better understand patient and care partner perspectives on enrollment of rare disease patients into gene therapy clinical trials in the pre-symptomatic or early symptomatic stages of their disease. The objective of this meeting was to understand what patients, and caregivers of patients, take into consideration when deciding whether to enroll in a gene therapy clinical trial and potentially receive an investigational gene therapy product. FARA President and Founder, Ron Bartek, spoke at the meeting to discuss perspectives from the FA community, specifically pediatric inclusion in clinical trials.

Click here to learn more about the meeting.

Nearly 2,500 people participated in Rare Artist Public Voting to select the ten Rare Artist Awardees. Congratulations to the 2024 Rare Artist Awardees!

View Awardees

State News



Upcoming Rare Disease Advisory Council (RDAC) Meetings

- **Nevada:** The Nevada RDAC meets on the first Friday of even-numbered months at 9:30 a.m. For more information, click here.
- **Tennessee:** The Tennessee RDAC meets on the fourth Wednesday of every other month 8:00 a.m. to 9:30 a.m. CST. If you are interested in joining the meeting, please email info@tnrdac.org for instructions on attending. Additional meeting information can be found here.
- **Vermont:** Join the coalition of rare disease patients, experts and advocates to encourage the creation of an RDAC in Vermont. The next coalition meeting is being held virtually on Monday, January 13 from 6:00 p.m. to 6:45 p.m. This is a great opportunity to meet other rare disease community members in your state and become an active participant in rare disease legislative advocacy efforts! Please email rdac@rarediseases.org to request the link for the Vermont RDAC Coalition meeting.

Would you like an RDAC in your state? Take action with NORD!

NORD is looking for individuals to submit an RDAC Supporter Statement. This would include a short testimonial (3-5 sentences) about why you support a state RDAC. Please also submit a photo alongside your statement 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.**