FARA Friedreich's Ataxia Research Alliance

Advocacy News

On Friday April 2, the White House released the President's proposed budget for Fiscal Year 2027. The President's Budget proposes $15.8 Billion in reductions to Health and Human Services (HHS), including funding reductions of 12% to the National Institutes of Health (NIH), 42% to the Centers for Disease Control and Prevention (CDC), 37% to the Advanced Research Projects Agency for Health (ARPA-H). The proposal also includes a 3.9% increase for the Food and Drug Administration (FDA).

Understanding how the federal budgeting process works — and why it matters — is essential to protecting funding for FA research and ensuring that our community's priorities are heard. The process begins when agencies submit funding requests to the Office of Management and Budget (OMB), which is in the executive branch of government. OMB reviews those requests and compiles them into a President's Budget that reflects the administration's funding priorities.

At the same time, Members of Congress are developing their own priorities. A big part of that process is learning about what is important to their constituents. That is why FARA mobilizes the FA community to ask their Congressional members to support the continued inclusion of "Hereditary Ataxia" in the CDMRP program each year during the budget cycle. By sharing your story with your members, you help to ensure that "Hereditary Ataxia" remains a vital funding opportunity for FA researchers.

Next, the House and Senate appropriations committees hold hearings to evaluate federal funding needs. Once approved by committee, appropriations bills move to the full House and Senate for debate, amendment, and passage. Because the bills passed in each chamber often differ, lawmakers must negotiate a final agreement before sending the legislation to the President for signature. If Congress is unable to complete this process by October 1, a continuing resolution is required to keep the government funded.

Congress is not required to adopt the President's Budget, and how closely it is followed varies from year to year. In essence, the President's Budget is both a financial plan and a policy statement, reflecting the administration's priorities, helping to guide the funding negotiations. The President's Budget is just one of the many factors Congress considers in the budgeting process—and another one is your input!

Capitol Hill Updates

Agency Congressional Justifications Released for FY27 Budget

Federal agencies have submitted their congressional budget justifications to Congress, outlining how they propose to use appropriated resources to achieve their missions. These justifications are crucial for Congress to understand the rationale behind agency budget requests and to make informed funding decisions. Below are key highlights:

ARPA-H:

The MATRIX program, first funded in 2024, focuses on drug repurposing to identify treatments for rare diseases that do not have therapies. This platform approach hopes to drastically accelerate the identification of potential new uses for existing new therapies.

CDC:
Funding the vital newborn screening program.

FDA:
Continuing the work of the Rare Disease Innovation Hub. The Justification also includes multiple legislative proposals that would impact the rare disease community:

- Enhance FDA's authority to publicly disclose information regarding their decisions
- Provide FDA with an increased control in appointing representatives to Advisory Panels
- Create an additional abbreviated licensure pathway for biological products
- Create an expediated phase I clinical trial pathway for certain therapies
- Permanently authorize the rare pediatric PRV program

NIH:
The NIH releases a general Justification for the entire agency and individual ones for each center. Rare disease was highlighted overall, discussing the important work of the Rare Diseases Clinical Research Network (RDCRN) and the agency's overall support for developing new gene therapies. The National Center for Advancing Translational Sciences (NCATS) Justification included a $5 million increase over FY25 levels for the Rare Disease Research and Therapeutics program. The Justification highlighted the role of NCATS in supporting the treatment for Baby KJ as well as the BEACONS study to assess the feasibility of incorporating genome sequencing into newborn screening.

NIH Director Testifies Before House Appropriations LHHS Subcommittee

The House Appropriations Subcommittee on Labor, Health and Human Services, Education, and Related Agencies held a March 17 oversight hearing on the NIH, where Director Jay Bhattacharya, M.D., M.P.H., Ph.D.,

discussed funding delays, early-career scientist retention, and proposed reforms to the agency's grantmaking and research approach. Lawmakers emphasized maintaining funding stability and restoring public trust in science, while highlighting the growing role of AI and the need for U.S. competitiveness in biomedical research.

FARA Co-Founder Ron Bartek Speaks at RISE Workshop on Data Sharing

At the April 2 "RISE Together: Data Sharing Across the Rare Disease Ecosystem" workshop hosted by the FDA Rare Disease Innovation Hub and Duke-Margolis Institute for Health Policy, stakeholders emphasized the importance of scalable, patient-centered data collection to advance rare disease research and therapy development. Discussions highlighted the need for improved infrastructure, cross-sector collaboration, and patient-centered approaches to make data sharing more consistent, scalable, and less burdensome across the ecosystem. Ron Bartek told the story of Skyclarys' approval and highlighting the importance of natural history studies.

Watch the Recording

FARA Co-Founder Ron Bartek Speaks at FDA Workshop on Pediatric Cell and Gene Therapy Trials

On April 9, The FDA's Center for Biologics Evaluation and Research (CBER), Office of Therapeutic Products (OTP) and the Alliance for Regenerative Medicine (ARM) co-hosted a hybrid workshop titled "Advancing Pediatric Cell and Gene Therapy Clinical Trials." The workshop explored the issues surrounding clinical trials for cell and gene therapies in pediatric populations, particularly for diseases where earlier intervention may result in greater therapeutic benefit. FARA's co-founder Ron Bartek participated in a panel on regulatory and scientific challenges and opportunities in pediatric cell and gene therapy (CGT) development. Visit the link below for some key takeaways and a recording of the event.

View Recording

CDC Director Nominee Announced

On April 16, President Trump has announced the nomination of Dr. Erica Schwartz as director of the Centers for Disease Control and Prevention. Dr. Schwartz, who previously served in the U.S. military and as Deputy Surgeon General during the first Trump Administration, holds degrees in medicine, law, and public health. If confirmed by the Senate, Schwartz

would replace Dr. Jay Bhattacharya, director of the National Institutes of Health, who took over as acting CDC director in February. Many in the medical community applauded the nomination, saying Schwartz brings decades of medical experience that will serve the agency well.

Secretary Kennedy Testifies Before House Committees on Department of Health and Human Services Priorities and Budget

On April 16, Health Secretary Robert F. Kennedy Jr. testified before both the House Ways and Means Committee and the House Appropriations Subcommittee, outlining HHS priorities while addressing questions on agency actions, public health policies, and proposed budget changes.

Lawmakers from both parties raised concerns and perspectives on issues including funding levels, research priorities, and public health strategy, reflecting ongoing congressional oversight of HHS direction and operations. Watch the hearings at the links below.

Appropriations

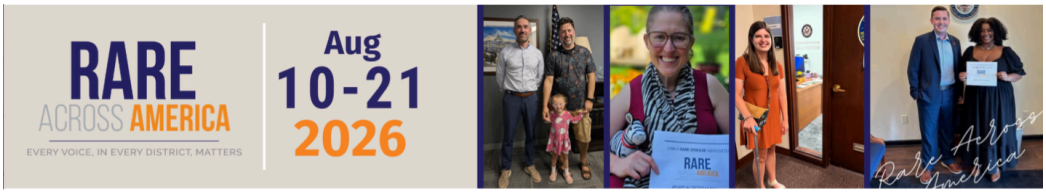
Ways and Means

Upcoming Advocacy Events

Rare Disease Legislative Advocates (RDLA) invites members of the rare disease community between 10 and 18 years old to participate in our second Virtual Youth & Teen Advocacy Day.

Advocates will have the opportunity to virtually meet with their Members of Congress and share their rare disease story. Prior to meetings with Members of Congress, advocates will attend virtual trainings on how Congress creates laws, how to communicate with your Representatives and Senators, and how to understand key policies affecting the rare disease community, designed for youth and teens. Registration is open from now until May 22.

Register Now



Rare Across America is the opportunity to meet with your Members of Congress at their in-district offices and educate them on the issues that are most important to the rare community by sharing your story. Registration opens on May 11.

The EveryLife Foundation for Rare Diseases will host a North Carolina State Advocacy Day on May 18-19, bringing together advocates to engage with local organizations, learn about state policy priorities, and meet with legislators at the Capitol to elevate rare disease issues.

Register Here

Announcements

Medicaid Story Collection

As part of the EveryLife Foundation for Rare Diseases' initiative to protect the rare disease community's access to Medicaid, they are asking advocates to share their Medicaid stories. To ensure representation from each state, EveryLife is in need of Medicaid stories from the following states: AL, AK, AR, HI, ID, IL, IA, KS, KY, LA, ME, MD, MN, MT, NE, NV, NH, NM, ND, RI, SC, SD, UT, WV, WY.

The stories you share will power advocacy efforts on social media, be shared directly with state officials, and be featured in media outreach as opportunities arise. As we begin to see the impacts of these cuts on state budgets, the rare disease community must make its voice heard. When you share your story, you put a face to a budget line. Your voice can help prioritize Medicaid funding in your state. Use this link to find more information and to submit your story!

NORD Living Rare, Living Stronger Events

Registration is officially open for two upcoming NORD® Living Rare, Living Stronger (LRLS) forums! Whether you are in the desert or the mountains, these FREE events are designed specifically for patients, families, and caregivers to come together for candid conversations, peer-to-peer education, and community-building:

Living Rare Arizona

- Date: Friday, May 8, 2026
- Location: Grand Canyon University Education Building, 2600 W Camelback Rd., Phoenix, AZ 85017
- Time: 10 a.m.- 2 p.m.

Living Rare Colorado

- Date: Friday, May 15, 2026
- Location: Auraria Campus, Tivoli – Turnhalle, 900 Auraria Parkway, Denver, CO 80204
- Time: 9 a.m. – 1 p.m.

Rare Disease Art Opportunity

Help amplify rare disease voices through art! Medical students, in collaboration with SketchNF, Rare Square, and Positive Exposure, are seeking artwork from patients, families, providers, and community members for a July exhibit in New York City. Submit artwork by June 1 for your piece to be considered for exhibition at Positive Exposure's NYC gallery—an impactful way to elevate lived

experiences and raise awareness through creativity.

More Information

#RAREis Scholarship Applications Open

The EveryLife Foundation's #RAREis Scholarship Fund has opened applications for Fall 2026, marking a milestone of over 500 scholarships awarded and more than $2.7 million in funding to support students living with rare diseases. The program continues to provide both financial support and opportunities for mentorship, advocacy, and community engagement, with applications open through April 28, 2026. Read more.

"The Economic Impact of Proposed Cuts to the NIH Budget" Report Released

United for Medical Research released a report, *"The Economic Impact of Proposed Cuts to the NIH Budget,"* analyzing the potential economic effects of the White House's FY 2027 budget proposal. Using its FY 2025 analysis as a baseline, the report estimates the impact of a 12.3% reduction in NIH funding on jobs and broader economic activity supported by NIH-funded research.

State News

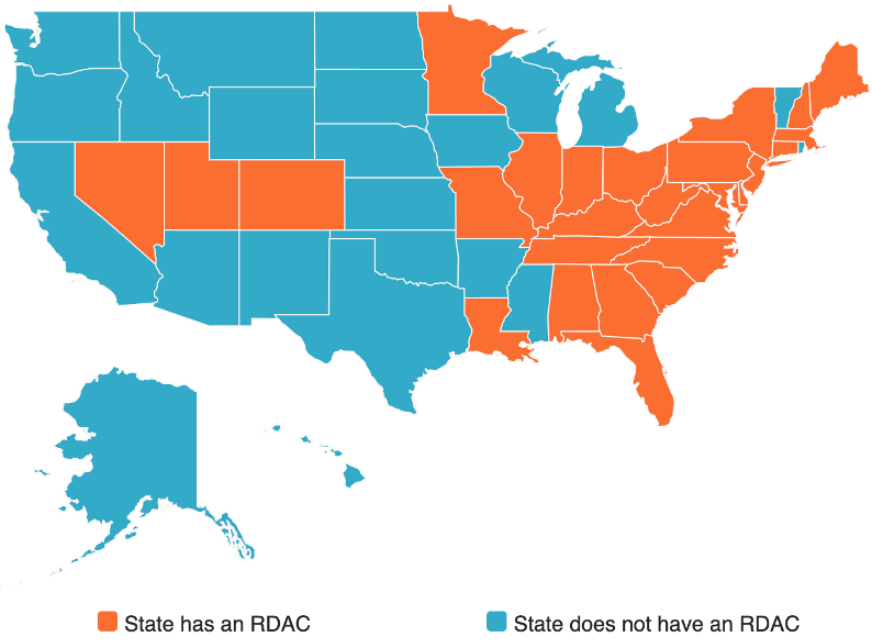
Vermont Advocacy Day

On April 8, NORD's Vermont Rare Action Network (RAN) met at the Vermont State House. Advocates met with legislators to advocate for H.46 – a bill to create a Rare Disease Advisory Council (RDAC) in Vermont. Mary Nadon Scott represented FARA at the event and gave testimony in support of the bill to the Senate Committee on Health and Welfare.

CMS Announces ASPIRE Model to Improve Care for Children with Complex Needs

The Centers for Medicare & Medicaid Services announced the ASPIRE (Accelerating State Pediatric Innovation Readiness and Effectiveness) Model, a new state-based initiative designed to improve care coordination and outcomes for children with complex medical and behavioral health needs enrolled in Medicaid and CHIP. The 10-year voluntary model will support up to five states and aims to advance early intervention, whole-person care, and value-based approaches, with a funding opportunity expected in 2026.

Read More



Upcoming Rare Disease Advisory Council (RDAC) Meetings

- **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**.
- **Connecticut:** 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**.
- **Maryland:** The Maryland RDAC meets on the second Tuesday of every month from 4:00 p.m. to 5:00 p.m. 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 will be able to find meeting information here, at a later date.
- **Michigan:** The Michigan RDAC will meet virtually on Tuesday, April 28 from 10:00 a.m. to 12:00 p.m. CT. You can learn more about joining the meeting here.

Newborn Screening Laws

The Virginia Governor signed a bill (H.B. 433) that directs the Department of Health to establish a process for adding new disorders to the state's newborn screening program.

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.

View this email in your browser

FARA Friedreich's Ataxia Research Alliance

Our mailing address is: