



Rare Disease Day is a global movement that shines a light on the [more than 300 million people](#) worldwide living with a rare disease and pushes for greater equity in health care, including access to timely diagnoses and approved treatments. Since the launch of Rare Disease Day, thousands of people have mobilized across the world in support of finding rare disease treatments, expanding public understanding and highlighting the urgent unmet needs of our community.

For those of us in the FA community, participation in Rare Disease Day is more than symbolic; it's strategic. When we show up, share our stories, and speak with a unified voice, we make measurable progress. Together, FA advocates have doubled federal FA research funding through the Congressionally Directed Medical Research Program (CDMRP), passed laws that accelerate research, and ushered in the approval of our first FA treatment! The momentum is incredible, but our work isn't finished.

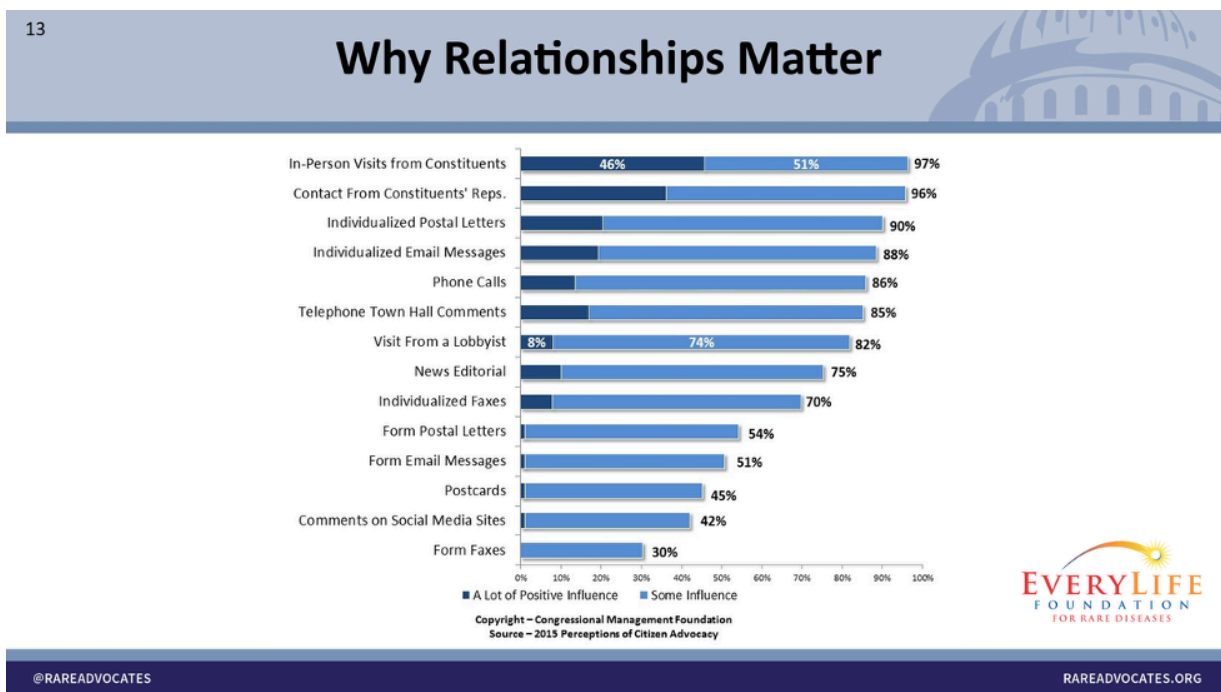
Since the passage of the Orphan Drug Act in 1983, [more than 1,300](#) orphan drugs have been approved, demonstrating remarkable scientific progress for rare disease patients. Increased funding, innovative incentives, and patient-centered policies can help unlock the next generation of breakthroughs.

Members of Congress are especially responsive to the people they represent, and many have never heard of FA until meeting someone in our community. When you share what it's like to live with FA, care for someone with it, or navigate the health-care system, you provide lawmakers with the context they need to champion

rare disease research. Your story could be the reason someone supports a law that leads to crucial scientific discoveries.

This year, Rare Disease Day falls on Saturday, February 28, 2026, and organizations are celebrating all month long. Below, you'll find a schedule of events where you can help raise awareness. Whether you participate online or in person, your involvement amplifies our collective impact.

And as seen in this slide recently shared by the EveryLife Foundation, sharing your story with your legislators—in person, by email, on social media, or within your community—is one of the most powerful tools we have. Let's make sure Rare Disease Day doesn't pass without you!



Rare Disease Day Events



Join the FDA for a **virtual public meeting** on Monday, February 23, 2026, in global observance of Rare Disease Week.

The theme is: **“Moving Forward. Looking Ahead. An Event for Patients.”** The goal of this year’s Rare Disease Day is to explore ways to engage and collaborate with patients and their communities to support and accelerate the development of medical products for rare diseases. We want patients to connect with FDA and stay involved with our outreach and engagement programs to ensure the patient voice informs medical product development and regulation throughout FDA.

Panels will discuss:



- Patient Focused FDA Initiatives
- Patient Engagement Opportunities
- Addressing Challenges and Opportunities with AI Technology
- Utilizing Real World Data and Real World Evidence at FDA

[Register Now](#)

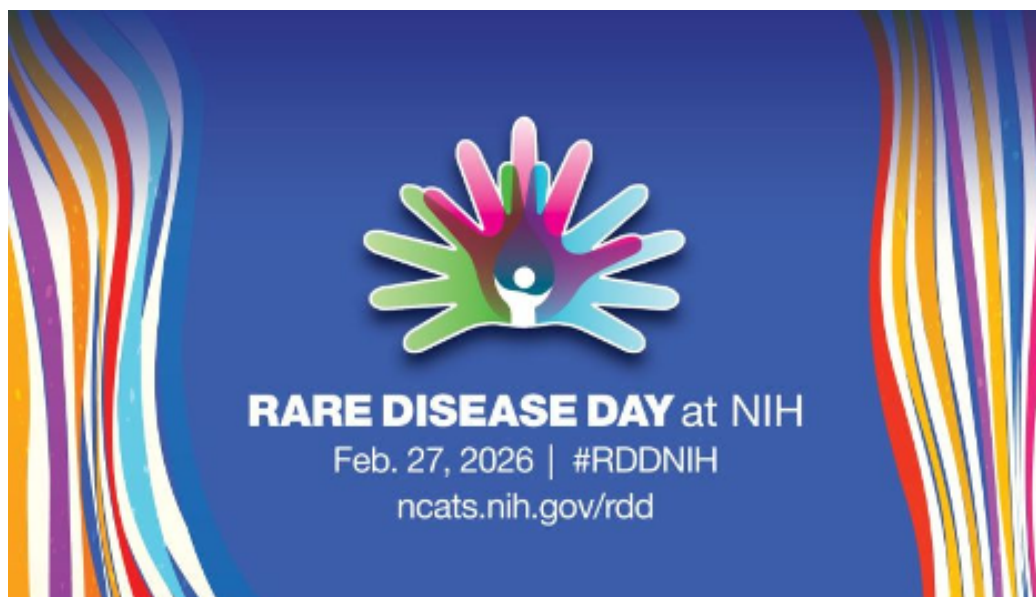


This free multi-day event, hosted by the Rare Disease Legislative Advocates, a program of the EveryLife Foundation for Rare Diseases, brings together rare disease advocates from across the country to make their voices heard with their Members of Congress. Participants are educated on policy proposals impacting the rare disease

community and provided opportunities to advocate directly for policy change with their Members of Congress.

 When? February 24 - 26, 2026  Where: Washington, D.C.

Registration for this event has closed, but some of the programming will be livestreamed. Click [here](#) for more information.



This **free, hybrid event** is an opportunity to hear directly from the world's leading researchers about the current challenges and emerging opportunities in the field of rare disease research.

[Register Now](#)

NORD and EURORDIS Create Rare Disease Resources

NORD has curated [Rare Disease Day resources](#) — including social media assets, messaging toolkits, downloadable activities, and opportunities like *Show Your Stripes* and *Light Up for Rare* — to make participation easy for organizations of all sizes.



For international information, Rare Disease Day is led by EURORDIS – Rare Diseases Europe, and they have developed a [Rare Disease Day website!](#)



Capitol Hill Updates



The Senate passed [H.R.7148](#), the Consolidated Appropriations Act, 2026, on January 30, 2026, with a 71-29 vote. The bill, which passed with amendments, was subsequently agreed to by the House on February 3, 2026, by a vote of [217 to 214](#), and signed into law that same day, marking a resolution to the federal budget process.

This law includes:

- \$47.216 billion for the NIH base budget, representing a \$415 million (0.9%) increase, relative to the total for the agency in FY25
 - o The National Center for Advancing Translational Sciences (NCATS), the NIH's home for rare disease research, will receive a \$10 million increase dedicated to rare disease research.
 - o Protections against drastic cuts to the NIH's indirect cost rate
 - o Blocks a planned change to grant funding that would have reduced the number of grants awarded each year.
- Approximately \$6.957 billion in funding for the FDA for FY26. This includes \$3.422 billion in discretionary funding.

- CDMRP/PRMRP funding restored
- Accelerating Kids' Access to Care Act which streamlines Medicaid and CHIP enrollment for out-of-state providers
- *Mikaela Naylor Give Kids a Chance Act* (originally H.R. 1262), which reauthorizes the FDA's Rare Pediatric Disease Priority Review Voucher (RPD PRV) program through September 30, 2029!

FARA and the FA community have followed and supported all of these issues, but one brings particular joy and satisfaction. The RPD PRV Program, established by the FDA over a decade ago, has been instrumental in advancing therapies for more than 40 rare pediatric conditions, many of which would likely never attract investment without this program...including Skyclarys! *This important and successful program costs the taxpayers nothing but offers immense benefits!* Its reauthorization, after more than two years of your advocacy, means the RPD PRV can continue to encourage pediatric therapy development and clinical trials.

The federal government is now funded through September 30 ***except for DHS, which is only funded through Feb. 13.*** The DHS budget includes funding for major operational components focused on border security, immigration, transportation security, and emergency management. Key agencies include:

U.S. Customs and Border Protection (CBP)

U.S. Immigration and Customs Enforcement (ICE)

U.S. Coast Guard (USCG)

Transportation Security Administration (TSA)

- NOTE: if you are traveling after Feb 13, TSA will be working but delays possible

Federal Emergency Management Agency (FEMA)

Cybersecurity and Infrastructure Security Agency (CISA)

U.S. Secret Service (USSS)

U.S. Citizenship and Immigration Services (USCIS)



On Tuesday, NIH Director Dr. Jay Bhattacharya testified before the

Senate Health, Education, Labor, and Pensions (HELP) Committee.



The focus of the hearing was NIH modernization, but a range of topics were addressed including vaccine policy, clinical trial disruptions, and multi-year funding. Watch the recording [here](#).

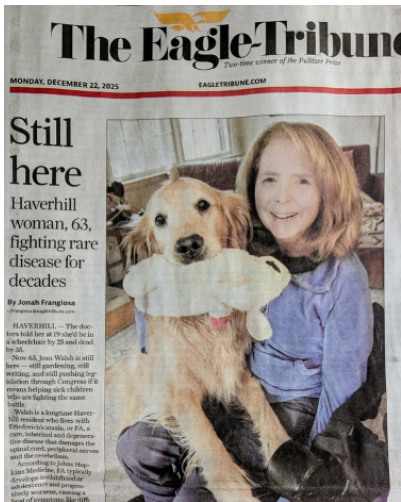


Secretary Kennedy Adds Two New Conditions to the Newborn Screening Panel!

The U.S. Department of Health and Human Services (HHS) has added Duchenne muscular dystrophy (Duchenne) and Metachromatic Leukodystrophy (MLD) to the federal Recommended Uniform Screening Panel (RUSP). Congratulations to both communities for successfully advocating for this significant milestone.

FA is not eligible for the RUSP as there are no approved treatments for newborns...YET!

Announcements



FARA Ambassador Jean Walsh in the News!

Thank you, Ambassador Walsh, for spreading the word on rare disease and the importance of the Rare Pediatric Disease Priority Review Voucher (RPD PRV) program! By writing an [Op-ed piece](#), her story ended up on the [front of her local paper](#). One person can make a difference!

#RAREis Scholarship Fund



Thanks to the support of Amgen, the #RAREis Scholarship will be offered in 2026 for individuals ages 17+ with a rare disease who are enrolling in undergraduate or graduate school, trade school, or any accredited course in Fall 2026.

The application will be open from March 17 through April 28, 2026 (3 PM CT). Up to 58 \$5,000 scholarships will be awarded for the fall semester. Click [here](#) to learn more.

State News



Your voice is needed at the State Capitol! Please join the **National Organization for Rare Disorders (NORD)** for **Rare Disease Day in Albany, New York** on **Tuesday, February 24, 2026**.

This is your opportunity to meet face-to-face with your state legislators and urge them to support **Senate Bill S1287B / Assembly Bill A1296B**, a critical bill that would codify a **Rare Disease Advisory Council (RDAC)** into law. An RDAC ensures that the rare disease community has a seat at the table when the state makes decisions regarding healthcare access and policy.

Event Details

When: Tuesday, February 24, 2026

Time: 9:30 AM – 4:00 PM

Where: State Street and Washington Avenue, Albany, NY 12224

Food: Complimentary food and drinks will be provided.

Register Now!

Join Your State's Medicaid Beneficiary Advisory Council!

Numerous states are currently accepting applications for their Medicaid Beneficiary Advisory Councils (BAC), and this is a great opportunity to make your voice heard and advocate for patients and families in your state. Members of these advisory councils will influence Medicaid policy and program implementation, including ways to ensure that patients have access to providers and are able to navigate upcoming Medicaid changes mandated by the One Big Beautiful Bill Act. The time commitment should be minimal, and some states offer modest compensation for advisory council members. Applications are often short and simple to complete.

If you are a current or former Medicaid beneficiary or caregiver of a Medicaid beneficiary and reside in one of the states below, consider applying and helping ensure that Medicaid best serves the FA community.

- Connecticut is now accepting applications with no deadline indicated.
- [Florida](#) is now accepting applications.
- Maryland is accepting applications via email with no specific deadline. For more information, please email mdh.mmacbac@maryland.gov.
- [Montana](#) is accepting applications with no specific deadline.
- [Ohio](#) is accepting applications with no deadline.
- [Washington](#) is accepting applications by email at ahcommunications@hca.wa.gov.

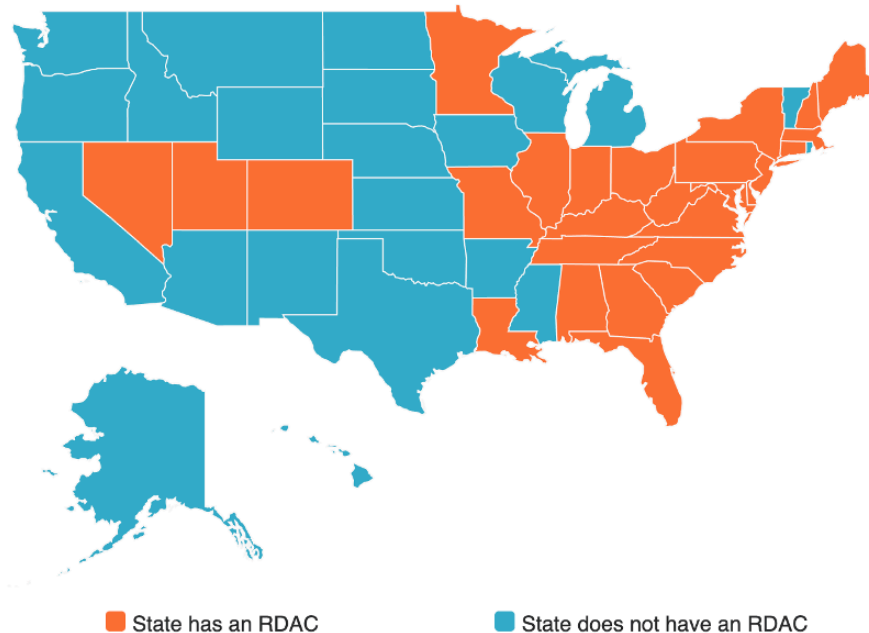
Include your name, client ID, contact information, and your preferred time to be contacted in the email.

- [Idaho](#) is accepting applications

- [New York](#) is accepting applications

States that will open applications soon:

- [Hawaii](#) and [North Carolina](#) will begin a new round of applications in the fall.



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](#).

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.



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