FARA Friedreich's Ataxia Research Alliance

Rare Disease Day Advocacy!

This year, Rare Disease Day falls on the rare day of February 29, 2024!

Below is an amazing list of events that you can participate in to help raise awareness of rare disease in general, and FA specifically! Moreover, sharing your experience is the key to others understanding FA, and paves the road to actions that can expedite treatments. Don't let this Rare Disease Day pass without being part of the conversation!

RARE DISEASE DAY®

Rare Disease Day is also a wonderful opportunity to reach out to your Congressional Members. Common Cause will help you locate your Members and you can either call them, or click on their homepage to send a note. Here are a few items you could include:

1. **Pass the budget!** Federal research agencies are hampered in advancing new programs and hiring needed staff under Continuing Resolutions. Failure to pass a timely budget delays getting treatments to patients.
2. **Extend the Pediatric Priority Review Voucher Program (Creating Hope Reauthorization Act (H.R.7384)).** The PPRV program has effectively incentivized pharmaceutical companies to develop rare pediatric disease treatments. This program will sunset on September 30, 2024. Learn more here.
3. Invite your Member of Congress to join the **Rare Disease Congressional Caucus**. You can check if they are already a member here.

Rare Disease Day/Week

Unable to Join Rare Disease Week In-Person? Check out 5 ways you can participate from home!

Click the button below to register and receive links to livestreams of select events during Rare Disease Week.

REGISTER HERE

1. View the **Rare Disease Documentary** on February 25 at 5:15 pm ET.
2. Watch the live stream of the **Legislative Conference** to learn more about how rare disease advocates can impact policy on February 26 from 9:00 am–5:00 pm ET.
3. View the live stream of the **DEIA Discussions** on February 28 from 10:00 am–12:00 pm ET
4. View the live stream of the **Rare Disease Congressional Caucus Briefing** on February 28 from 1:00-2:30 pm ET
5. **Email or call your Representative and Senators to make your voice heard during Rare Disease Week on Capitol Hill 2024.**

Heading to Rare Disease Week? Check out the Share Your Story with Policymakers Webinar!

View the Share Your Story with Policymakers Training Webinar Recording

This webinar covered important Rare Disease Week information including:

- What to expect in your meetings with Members of Congress and how to prepare
- Tips on how to refine your elevator pitch and develop a clear and concise “ask” for your meetings

You can find both of the Share Your Story worksheets mentioned in the training below.

- **Tips on sharing your story and crafting your ask:** Click here
- **Worksheet to draft your pitch:** Click here

To view the webinar recording, go to the Rare Disease Week Attendee Corner where you'll find the full Rare Disease Week agenda, resources to prepare for Hill meetings, training webinar recordings, accessibility information, FAQs, and more. Simply click the button below and sign in using the password **#RareDC2024.**

Attendee Corner

NORD invites you to #ShowYourStripes at the TODAY Show Plaza!

Join us and fellow allies on Thursday, February 29, as we stripe out the TODAY Show Plaza for Rare Disease Day.

Your participation will help raise awareness of programs that advocate for faster diagnoses, accelerated research, new treatments, and patient support to help the 1 in 10 Americans living with rare disease.

NYC #ShowYourStripes
6:30 am | Thursday, February 29, 2024
Rockefeller Plaza, 35 West 48th Street
(between 5th and 6th Avenues)
New York, NY 10112

Register Now

This is a family-friendly event. Get your posters ready, dress in your most festive zebra gear and let's raise awareness! Share your photos on social media using **#ShowYourStripes** and **#RareDiseaseDay**.

Everyone can participate in the Global Chain of Lights and Share Your Colors!

In an effort of global solidarity, you are invited **to light or decorate your home** with the Rare Disease Day colors at **7 PM your local time on February 29, 2024**. Share a photo on social media with the hashtag **#rarediseaseday**!

You can use garlands, social media filters, candles, disco lamps, colorful decorations…Let your creativity shine!

Light Up for Rare on Rare Disease Day®!

On Rare Disease Day, landmarks throughout the world will be illuminated in the Rare Disease Colors of pink, green, blue, and purple. FARA has arranged for the Tower Crown Light – Capella Tower Minneapolis to light up for rare 2024! #LightUpForRare

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Rare Disease Events in Your Area

To see a **full list of Rare Disease Day events**, check out NORD's calendar to find where rare community members are hosting an event near you.

Event Calendar

NORD Resources to Download

Social Media Toolkit: Use our social media toolkit to **#ShowYourStripes** online to spread awareness to your online community!

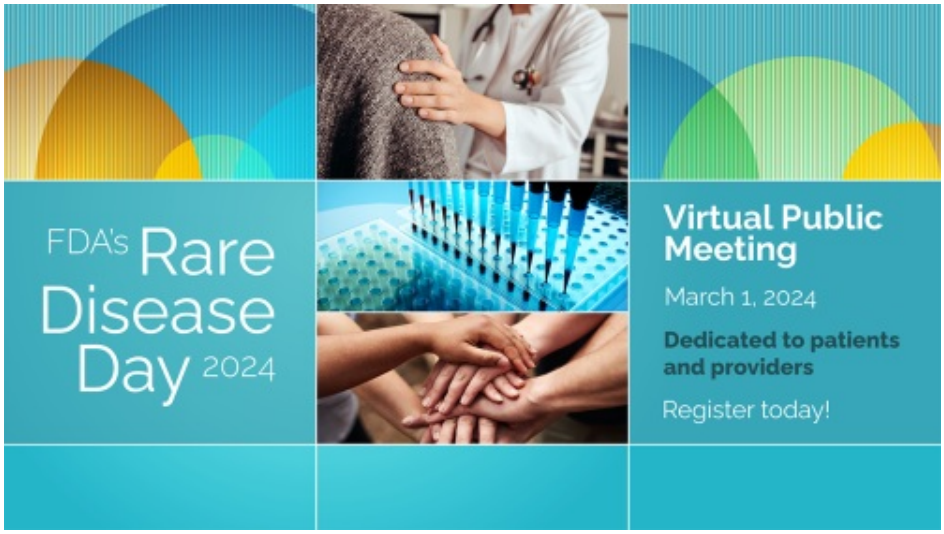
Download Toolkit

Lawn Signs, Fact Sheets, Coloring Pages, and More: Download these free resources for use in your own community, whether you want to #ShowYourStripes in your neighborhood or at a Rare Disease Day event you are hosting!

Download Resources

Rare Disease Day at the National Institute of Health (NIH) on February 29, 2024 – an extraordinary occasion dedicated to raising awareness for rare diseases and all those who are impacted. This event is free and open to the public. A virtual livestream will also be available.

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.

Register Here

Did you know an estimated 10,000 different rare diseases are impacting nearly 400 million people worldwide? Each person has a story to tell.

In honor of **Rare Disease Day on Thursday, February 29, *#RAREis* One** is hosting a global webinar featuring the diverse perspectives from the rare disease community around the world.

#RAREis...

A program by AMGEN

#RAREis One Webinar

Elevating the faces, voices, and diverse experiences of those living with or caring for someone with a rare or chronic disease

February 29, 2024 | 11:30 a.m. ET

The conversation will focus on elevating the faces, voices, and unique experiences of those living with, caring for, and advocating for someone

with a rare disease. Through the recounts of diverse storytellers, you will gain an understanding of what life is like in different parts of the globe and the challenges they've encountered and overcome.

Register Now

Capitol Hill Updates

Senate and House Approve 6-week Continuing Resolution Avoiding a Shutdown — Next deadlines are in March

On January 18, the Senate voted 77-18 to approve HR 2872, extending the Continuing Resolution (CR) by 6 weeks. The new deadline is March 1 for bills covering funding for Agriculture/FDA, Energy & Water, Military Construction/VA, and Transportation-HUD bills. The 8 other appropriations bills, including Labor-HHS, have a CR deadline of March 8.

The House approved the Senate bill by a vote of 314-108 and it was signed by the President on January 19.

Upcoming Advocacy Events

The ECRD is the **largest, patient-led, rare disease policy-shaping event** held in Europe.

By bringing together people with rare diseases and patient advocates, policymakers, healthcare industry representatives, clinicians, regulators, and Member State representatives, EURORDIS harnesses **the power of this extensive network** to shape goal-driven rare disease policies of the future.

With over 1000 participants, the Conference is an unrivaled opportunity to **network and exchange invaluable** insights within the rare disease community. Through collaborative efforts, these discussions culminate in **clear policy recommendations** that can influence both EU and national policies.

Get ready for the next ECRD, a fully hybrid conference taking place on **15 & 16 May 2024** online and at The Square in Brussels. Learn more here.

GET YOUR PASS

PROGRAMME AT A GLANCE

Announcements

Time is crucial in rare disease research. Understanding how diseases progress over time and how they impact patients at each stage is vital for setting research priorities. Rare disease experiences are unique, and it's important to listen to patient voices to shape valuable research agendas.

The EveryLife Foundation and The Innovation and Value Initiative (IVI) report aims to advance the field of patient-centered outcomes research for rare diseases. This effort was made possible through funding support from the Eugene Washington PCORI© Engagement Awards Program, an initiative of the Patient-Centered Outcomes Research Institute® (PCORI®), and Alexion, AstraZeneca Rare Disease.

Read Report

State News

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.

Wisconsin Newborn Screening Update

In photo: Kimberly Haugstad, President & Founder WI Rare, Emily Stauffer, EveryLife Foundation

This month, Wisconsin advocates joined the EveryLife Foundation team in Madison to testify in favor of WI SB 962, a bill to align the state's newborn screening program with the federal RUSP. The bill passed out of the Wisconsin Senate Health and Human Services Committee and is now under consideration by the full Senate.

Learn More