TESTIMONY IN RECOGNITION OF RARE DISEASE DAY

VT Senate Committee on Health and Welfare
February 19, 2026

Presented by:
Carolyn G. Sheridan, MPH
Associate Director of State Policy
National Organization for Rare Disorders® (NORD)

Good morning, Chair Lyons and Members of the Committee,

My name is Carolyn Sheridan, and I serve as Associate Director of State Policy at the National Organization for Rare Disorders, or NORD. In this role, I have the privilege of coming before state governments across the country to advocate alongside, and on behalf of, patients, caregivers, and providers in the rare disease community to advance policies that strengthen care, access, and coordination.

I am grateful for the opportunity to join you in recognition of Rare Disease Day and to share more about the rare disease community I serve and the work of NORD.

Rare disease is defined under federal law within the Orphan Drug Act as a condition affecting fewer than 200,000 people in the United States.

While each condition is rare individually, collectively rare diseases are not.

- NIH statistics report 1 in 10 Americans lives with a rare disease
    - That represents more than 30 million people nationwide
- There are more than 10,000 known rare diseases
    - Fewer than 5% have an FDA-approved treatment

Many rare diseases are chronic, complex, genetic, and life-threatening or severely debilitating. Patients frequently experience prolonged diagnostic delays, difficulty accessing specialists, fragmented systems of care, and significantly higher medical costs, sometimes 3–5 times higher than non-rare conditions.

Rare disease is not confined to one policy area. It intersects with newborn screening, Medicaid, commercial insurance, mental health, disability services, aging services, and public health infrastructure.

Thousands of Vermonters are living with rare diseases today

NORD was founded in 1983 by patient advocates who successfully fought for passage of the Orphan Drug Act.

Today, NORD is a national nonprofit organization and serves as an umbrella federation of more than 350 disease-specific patient organizations. While those organizations focus on individual rare conditions, NORD represents the rare disease community as a whole.

Main pillars of our work include:

- **Patient Assistance Program (PAP):** NORD administers one of the largest rare disease patient assistance programs in the country. Through these programs, we provide direct financial support to patients to help cover the cost of medications, travel to see specialists, and other disease-related healthcare needs. These funds are restricted and flow directly to patients; they do not support NORD's operational expenses. Similarly, a portion of our revenue supports research grants awarded directly to investigators and does not fund NORD operations.

- **Education and Information:** Our education team maintains a growing, medically reviewed rare disease database that provides evidence-based information for patients, caregivers, and healthcare professionals.

- **Clinical Excellence:** Our clinical team supports a nationwide Rare Disease Centers of Excellence network — a healthcare facility recognition program designed to elevate best practices in rare disease diagnosis and multidisciplinary care.

- **Public Policy:** Consistent with our origins in the Orphan Drug Act, our policy team works at both the federal and state levels to support legislative and regulatory solutions that improve access, affordability, research infrastructure, and care coordination for rare disease patients.

NORD operates with a fully remote workforce, allowing us to support communities across the country while remaining responsive to state-specific needs.

Rare Disease Day is observed annually on the last day of February; chosen because February 29th is the rarest day of the year. The observance began in Europe in 2008 and became a global movement in 2009 when NORD mobilized U.S. patient advocacy organizations.

The purpose of Rare Disease Day is to:
- Raise awareness
- Improve access to diagnosis and treatment
- Promote equity and representation in policy
- Highlight the lived experiences of patients and caregivers

Across the country and around the world:
- Landmarks “Light Up for Rare” in pink, purple, green, and blue
- Patients and families share their stories
- Communities gather to educate policymakers and the public

You may also see the “Show Your Stripes” campaign. The zebra is the symbol of rare disease. In medical training, physicians are often told: *“When you hear hoofbeats, think horses, not zebras.”* But for families living with rare diseases, they are the zebras.

Finally, Rare Disease Day provides a moment of visibility.

But what patients need is year-round structural attention.

As you may know, Vermont is currently considering H.46, which would establish a Rare Disease Advisory Council within the Department of Health

I look forward to returning later this session in April, joining our Vermont advocates in raising awareness of the bill.

It is our hope that by that time, H.46 will be up for discussion here in Committee so that you all might meet them and we can explore together how Vermont can continue strengthening its support for the rare disease community.

Thank you for providing me with this forum to share more about Rare Disease Day, NORD, and the rare community.

NORD stands ready to serve as a resource to this Committee at any time.

Respectfully submitted,

C Sheridan

Carolyn G. Sheridan, MPH
Associate Director of State Policy
National Organization for Rare Disorders (NORD®)
p: 203-241-0588 e: csheridan@rarediseases.org

RARE DISEASE DAY RESOURCES

For those interested in learning more or participating, RareDiseaseDay.us offers downloadable toolkits, social media graphics, official hashtags, and sample messaging. The site also provides "playbooks" tailored for homes, workplaces, schools, and communities, with practical ideas for hosting events, engaging partners, and raising awareness at the local level.

Support VT's Effort to Establish a Rare Disease Advisory Council

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NORD is working to establish well-organized, high-functioning Rare Disease Advisory Councils in every state to support the needs of the rare disease community.

More than 30 million Americans are living with a rare disease; more than half of which are children.

Rare disease patients face unique challenges such as obtaining an accurate diagnosis, accessing medical specialists with knowledge of their condition, battling for access to quality care and therapies, and receiving fair insurance coverage of their treatment and care.

With over 10,000 known rare diseases, it is difficult for state policymakers and government officials to have an in-depth understanding of the issues faced by rare disease community members, and the resources needed to support them.

With diverse membership and broad community support, a VT RDAC will serve as a valuable resource as our health care system evolves. Without an RDAC, policies that affect the rare community will be developed without the consultation of rare disease stakeholders, leaving this vulnerable population at greater risk for poor health and economic outcomes. A strong RDAC can help relieve burdens on the state by expeditiously delivering direct feedback, solutions, and resources to government decision makers with one community voice.

Email us at RDAC@rarediseases.org to get involved in the effort!

In 2015, the first RDAC was created in North Carolina by patients, caregivers, families, and providers. Since then, rare disease advocates and stakeholders have sought councils in other states to help better represent their communities.

What You Should Know About Vermont's RDAC Effort

House Bill 46 was introduced by Representative Mary-Katherine Stone. This legislation would establish a RDAC to:

- Convene public hearings and solicit comments from individuals impacted by rare diseases to create a needs assessment identifying gaps in services and the needs of caregivers and providers.
- Provide testimony and comments on pending legislation and rules that impact Vermont's rare disease community before the General Assembly and other State agencies.
- Consult with experts to develop policy recommendations that identify conditions to recommend to the Newborn Screening Advisory Committee and support timely patient access to diagnostic services and treatment
- Maintain a web page on the Department of Health's website to serve as a resource for individuals with a rare disease, including meeting notices, minutes, and annual reports
- Pursue any other activities identified by a majority of the Advisory Council.

The Council will be housed within the Department of Health and include patients (with a reserved seat for an older Vermonter), caregivers, a physician, a nurse, a pharmacist, a geneticist or genetic counselor, an academic researcher, and designees from key state agencies.

It's estimated that

30 million

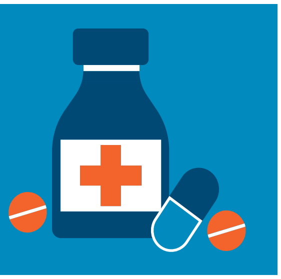
AMERICANS

(almost 1 in 10) have rare diseases.

More than

95%

of rare diseases are without an FDA-approved treatment.



Alone we are rare. Together we are strong.®

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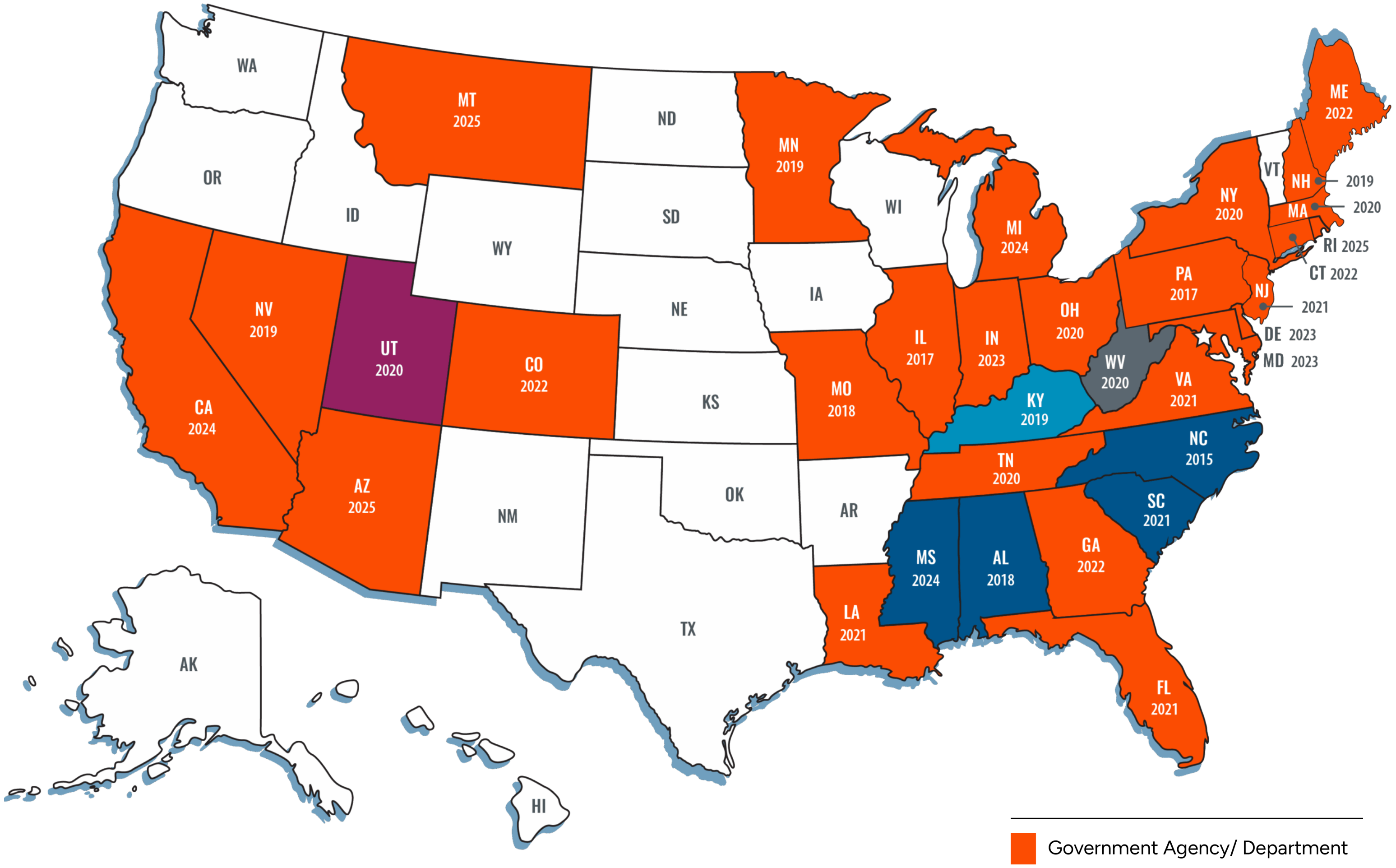
States That Have Established RDACs *(As of December 2025)*



🟧 Government Agency/ Department

🟦 University

🟦 Nonprofit

🟪 Grantee from Department of Health

⬛ Expired

NORD launched Project RDAC in November 2020 to optimize existing RDACs and help more states enact RDAC legislation. Through this work, NORD will develop tools and resources to help RDACs at every step of their journey based on feedback from the existing RDACs and rare disease community. Please check back here for additional information and updates on the project. For more, visit: **rarediseases.org/rdac-overview**

NORD: Fighting for the rare community every day for more than 40 years.

NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 300 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

The mission of NORD's Rare Action Network® (RAN) is to connect and empower a unified network of individuals and organizations with the tools, training, and resources to become effective advocates for rare diseases through national and state based initiatives across the United States. To join RAN, go to **rareaction.org.**

*For more information on Rare Disease Advisory Councils or NORD, please contact **RDAC@rarediseases.org.***

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Ways State Lawmakers Can Engage with Their Rare Disease Community

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1. Share Information on NORD's Patient Assistance Programs

Share NORD's Helpline to connect patients and caregivers with RareCare® Patient Assistance Programs and other general and disease-specific resources.

Phone: 1-800-999-6673

Website: rarediseases.org/patient-assistance-programs

2. Engage Directly with Patients and Advocates

- **Host Town Halls or Roundtables** – Engage directly with rare disease patients and caregivers to hear their challenges and policy needs.
- **Meet One-on-One with Rare Disease Advocates** – Set aside time for constituents affected by rare diseases to share their experiences and policy requests.
- **Tour a Local Rare Disease Treatment Center or Research Facility** – Visit a hospital, research center, or NORD® Rare Disease Center of Excellence to see firsthand the challenges and advancements in rare disease care (Find a NORD Rare Disease Center of Excellence in Your State).
- **Recognize a Rare Disease Advocate in Your District** – Honor patients, caregivers, or researchers making a difference in rare disease care.
- **Engage Positively with NORD and Local Rare Disease Organizations on Social Media** – Amplify awareness and show commitment to the rare disease community. (FB/Insta/LinkedIn/Twitter/Bluesky/YouTube)

3. Support Rare Disease Policy and Legislative Efforts

- **Support Establishing Rare Disease Advisory Council (RDAC) Legislation OR Connect with an Existing Council** – Strengthen patient representation in policy discussions by creating or working with a RDAC.
- **Introduce Legislation to Improve the Rare Disease Experience in Your State** – Expand newborn screening, Medicaid coverage, insurance protections, and policies to reduce out-of-pocket costs while ensuring your state is a welcoming place for specialist healthcare providers. View NORD's State Report Card to see how your state measures up.
- **Push for Stronger Step Therapy and Prior Authorization Protections** – Work on legislation that prevents unnecessary delays in treatment. View NORD's State Report Card to learn more.
- **Expand Telehealth and Interstate Licensure Compact Membership** – Support policies that improve access to specialists, especially for rural and underserved areas. View NORD's State Report Card to learn more.
- **Advocate for Better Data Collection and Research** – Champion state funding for patient registries and rare disease research grants to improve long-term care solutions.
- **Strengthen Rare Disease Education in Medical Training** – Introduce policies requiring medical schools and continuing education programs to include rare disease education. Share Continuing Medical Education Opportunities.
- **Encourage State Agencies to Improve Public Health Communication** – Ensure that state Medicaid offices, public health departments, and insurance regulators have rare disease resources available.

4. Increase Awareness and Public Engagement

- **Participate in Rare Disease Day®** – Show your commitment to the community through events, statements, or legislative action. Learn how you can Show Your Stripes® at rarediseaseday.us.
- **Carry Resolutions in Your Chamber Acknowledging Rare Disease Day** – Sponsor a resolution annually on the last day of February to recognize the rare disease community. Check out the [Proclamations Toolkit]().
- **Host a Rare Disease Day at the State Capitol** – Invite advocates, researchers, and clinicians for a public event to highlight state-specific challenges and progress.
- **Support Constituents [Running for Rare®]()** – Get involved with NORD's signature charity running team to generate awareness for rare diseases, connect the rare community and raise funds to further NORD's mission.

5. Leverage Leadership Influence and Build Coalitions

- **Encourage Other Lawmakers to Join the Effort** – Help build a bipartisan coalition for rare disease policy within the legislature.
- **Collaborate with Healthcare & Business Leaders** – Work with hospitals, biotech companies, and insurers to expand support and resources for rare disease patients.
- **Advocate for Federal-State Collaboration** – Push for federal grant opportunities that help state programs address rare disease needs. Learn more about [FDA's Office of Orphan Products Development]().
- **Appoint a Legislative Liaison for Rare Disease Issues** – Designate a staff member to be a point of contact for rare disease advocates and organizations.

Alone we are rare. Together we are strong.®

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