NORD® Advocacy Priorities for 2025

National Organization for Rare Disorders (NORD®)

DRIVING POLICIES THAT MAKE A DIFFERENCE

NORD is the only U.S. organization working at the intersection of care, research and policy for all rare diseases and the more than 30 million Americans living with a rare disease. We are rooted in the advocacy that spurred a movement resulting in the passage of the world's first law incentivizing drug development for rare diseases—the Orphan Drug Act (ODA) of 1983. We continue to fight until every person living with a rare disease has access to affordable and life-changing care and treatment.

In 2025, we are working with federal and state decision-makers to advance the following key priorities:

New and Better Therapies

NORD advocates for the development of safe and effective therapies, supporting policies that strengthen rare disease research, improving regulatory approval processes and protecting incentives for the development of rare disease treatments.

- Maintain vital incentives for rare disease drug development by reauthorizing the Rare Pediatric Disease Priority Review Voucher program and protecting the Orphan Drug Tax Credit.
- Protect the intent and integrity of the ODA in awarding orphan drug exclusivity by passing the RARE Act.
- Support strong regulatory pathways at the FDA to enable robust patient access to safe and effective therapies.
- Reduce barriers to clinical trial participation and give patients a voice through patient listening sessions, patient-focused drug development meetings, and at every step of drug development.
- Protect crucial funding for National Institutes of Health (NIH) and Food and Drug Administration (FDA) needed to advance discovery and development of rare disease treatments.

Access to Affordable Health Care and Treatment

NORD believes that all rare disease patients should have access to affordable, high-quality, comprehensive health care that best meets their complex medical needs.

- Protecting patient access to Medicaid services and extending the ACA's enhanced premium tax credits.
- Ensure drug pricing efforts related to the Inflation Reduction Act (IRA) and pharmacy benefits managers (PBM) reform truly benefit rare disease patients through reduced out-of-pocket prescription drug costs, while appropriately protecting continued innovation.
- Improve patient access to out-of-state health care providers by passing the Accelerating Kids Access to Care Act and pushing additional states to join the Interstate Medical Licensure Compact.
- Increase the number of states with high functioning Rare Disease Advisory Councils (RDACs) to give the rare disease community a voice in state government.

Reducing the Diagnostic Odyssey

It can take years to get an accurate rare disease diagnosis, which can take a significant toll on an individual's physical and mental health and contribute to significant financial burdens

- Expand availability and coverage of necessary genetic testing.
- Support robust, well-funded newborn screening programs in every state.
- Protect patient access to accurate diagnostic tools, including lab developed tests.

WHY WE FIGHT

Rare diseases are not, in fact, rare. Together, they affect 1 in 10 Americans. The legislation and policies we help advance profoundly impact these patients, their families, caregivers and medical professionals today and for years to come.

There are more than

10,000

known rare diseases.

However, **less than 5% have FDA-approved treatments.**

Source: Orphanet Journal of Rare Diseases

Rare disease patients' direct medical costs are

3-5 times higher

than non-rare disease patients.

Source: Orphanet Journal of Rare Diseases

880+

orphan drugs have been FDA-approved since the passage of the Orphan Drug Act.

Source: Orphanet Journal of Rare Diseases

Looking for more information on NORD's policy work?

Visit:
rarediseases.org/driving-policy

Email: **policy@rarediseases.org**

NORD®
National Organization for Rare Disorders

Alone we are rare.
Together we are strong.®