

# Driving Life-Changing Policy

National Organization for Rare Disorders (NORD®)



## A TRUSTED, INDEPENDENT VOICE

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. Our work is driven by the needs of the patient community, and our evidence-based approach establishes us as a credible resource for legislators and policymakers.

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 of 1983. We continue to fight for affordable access to life-changing care and treatment.

*We work with national and state decision-makers to advance the following priorities:*

### New and Better Therapies

NORD advocates for the development of safe and effective therapies and better diagnostics, supporting policies that strengthen rare disease research, improve regulatory approval processes and protect incentives for the development of rare disease drugs. Everyday, we are advocating to:

- Protect the intent and integrity of the Orphan Drug Act (ODA)
- Advance patient-focused drug development (PFDD)
- Ensure strong regulatory pathways, including accelerated approval for rare disease drugs
- Expand clinical trial diversity and give patients a voice in clinical trial design

### 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. To achieve this, we're fighting to:

- Ensure the Inflation Reduction Act (IRA) benefits rare disease patients through reduced out-of-pocket prescription drug costs
- Protect Affordable Care Act (ACA) requirements for patient access to comprehensive health insurance benefits
- Reduce barriers to specialists for telehealth services
- Improve patient access to out-of-state health care providers

### 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, which is why we're working to:

- Expand availability and coverage of necessary genetic testing
- Support robust, well-funded newborn screening programs in every state

## WHY WE FIGHT

Rare diseases are not, in fact, rare. Together, they affect 1 in 10 Americans – more than 30 million people. 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](https://rarediseases.org/driving-policy)**

Email: **[policy@rarediseases.org](mailto:policy@rarediseases.org)**

Alone we are rare.  
Together we are strong.®

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