



NORD Rare Disease Centers of Excellence

FACT SHEET

The Facts: The Urgency of Rare Disease in America

- 1 in 10 Americans suffer from a rare disease, equating to more than 30 million in the U.S.¹
- Collectively, rare disease is more prevalent than Alzheimer's and COPD and has nearly the same incidence as diabetes.²
- Half of rare disease patients are children and 30% will not live to see their fifth birthday.^{3,4}
- Rare disease is defined as any disorder, illness or condition affecting fewer than 200,000 people in the U.S.⁵ Yet, there are significantly more types of rare disease than there are types of cancer, heart disease, dementia, diabetes and other diseases.
- Less than 5% of the estimated 7,000 rare diseases have treatments and even fewer have cures.⁶
- Rare disease costs the U.S. \$400 billion annually in medical expenses which is higher than the individual costs of cancer, heart disease and obesity.⁷
- The average timeline for a rare disease diagnosis is five years and many cases take more than 10 years in comparison to days, weeks or months for other diseases like cancer, heart disease, dementia and diabetes.^{8,9}
- Unlike well-known diseases such as, cancer, heart disease, diabetes and others, rare diseases have little to no standards of care, treatment guidelines, physician expertise, widespread R&D, educational resources and community support.
- Anyone can be impacted by a rare disease at any point in their life.

About: NORD Rare Disease Centers of Excellence (NORD RDCoE)

- NORD® RD CoE is the first and largest network of U.S. medical institutions and experts dedicated to patient clinical care and research for all rare diseases.
- NORD® RD CoE provides all rare diseases a designated home within America's healthcare system; giving patients, physicians and caregivers more readily accessible medical expertise and an engaged healthcare community.
- NORD® RD CoE have a national footprint:
 - 31 designated NORD® RD CoE comprised of multiple leading medical centers and healthcare institutions
 - Spanning across 22 states and all four regions of the U.S.
 - Continued growth and expansion of sites in 2023

- NORD[®] RD CoE are creating a patient-centric model that focuses on:

<ol style="list-style-type: none"> 1. Reducing time to diagnosis 2. Improving patient access to care, treatments, and resources 3. Providing standards of specialized care 	<ol style="list-style-type: none"> 4. Accelerating R&D 5. Establishing treatment guidelines 6. Centralizing a national network of rare disease experts and researchers
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- NORD[®] RD CoE boasts an active volunteer network made up of 250 medical professionals and others that touch the rare disease patient journey.
 - Volunteers include world-class clinicians, researchers, advanced trainees, allied health professionals and support staff from across 20 areas of specialty, including pediatrics.
- NORD[®] RD CoE leadership and volunteers have identified 10 top unmet needs and challenges faced by the rare disease patient, medical professional and research community and are working to solve and improve these barriers to care:

<ol style="list-style-type: none"> 1. Long, costly path to diagnosis 2. Scattered patients and expertise 3. Limited and restricted clinical data 4. Lack of drug development & research 5. Few treatment & compliance guidelines 6. Scarce clinician and researcher training pipeline 	<ol style="list-style-type: none"> 7. Lack of guidance for clinical application of newly approved treatments 8. Access inequities to care, education and clinical trials 9. Gaps in insurance coverage for testing, procedures, treatments 10. Limited patient multi-disciplinary specialty care integration
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- NORD[®] RD CoE are at the heart of rare disease patient care. Every NORD[®] RD CoE:
 - Offers multi-specialty clinical care coordination which is essential for this complex patient population
 - Has an extensive array of experts covering all medical specialties required for the diagnosis and treatment of a broad range of rare diseases
 - Supports all stages of rare disease research, with the goals of increasing knowledge of disease etiology and pathophysiology, improving diagnostic techniques and developing rare disease treatments and cures
 - Dedicated to mentoring and training the next generation of rare disease clinician and researchers
- There is no fee associated with being a NORD-designated RD CoE nor does any NORD[®] RD CoE receive any funding from NORD for its participation.



Sources:

¹ U.S. Department of Health and Human Services; National Institute of Health (NIH); National Center for Advancing Translational Sciences (NCATS); [Rare Disease FACT Sheet](#) (January 2022).

² National Center for Advancing Translational Sciences (NCATS); [Rare Disease FACT Sheet](#) (January 2022).

³ National Institute of Health (NIH); National Library of Medicine – National Center for Biotechnology Information; <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC6185816/>

⁴ [Critical Path Institute; Press Releases](#); <https://c-path.org/c-path-launches-rare-disease-clinical-outcome-assessment-coa-consortium/>

⁵ CFR 316.20 or Sec 526 of the Orphan Drug Act; <https://www.fda.gov/patients/rare-diseases-fda>

⁶ National Center for Advancing Translational Sciences (NCATS); [Rare Disease FACT Sheet](#) (January 2022).

⁷ [CDC; National Center for Chronic Disease Prevention and Health Promotion \(NCCDPHP\); Health Economic Costs of Chronic Diseases](#)

⁸ Global Rare Disease Commission Report; <https://www.globalrare-disease-commission.com>

⁹ Genome Medicine; <https://genomemedicine.biomedcentral.com/articles/10.1186/s13073-022-01026-w>

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