

We have a BEAUTIFUL. & RARE Chance



Reimagine a Future Where Every Person With a Rare Disease, and Their Families, Live Their Best Lives.

RARE DISEASE FACTS



RARE DISEASE:

Any disease, disorder, illness, or condition affecting fewer than 200,000 people in the United States is considered rare.¹



There are approximately **7000** known rare diseases.²



MORE THAN 90% of rare diseases are **WITHOUT** an FDA-approved treatment.³



Most rare diseases are **GENETIC** or have a genetic component.⁴



MORE THAN HALF of those affected by rare diseases are children.⁵



ALL PEDIATRIC CANCERS are rare.⁶



There are more than **500 TYPES** of rare cancers.⁷



More than **25 MILLION AMERICANS** are living with a rare disease.⁸



FOR MANY RARE DISEASES, signs may be observed at birth or in childhood.⁹

References: 1. US Food and Drug Administration. Orphan drug act - relevant examples. Updated March 9, 2018. Accessed November 8, 2022. <https://www.fda.gov/forindustry/developingproductsforrareconditions/howtoapplyfororphanproductdesignation/ucm364750.htm>. 2. Genetic and Rare Diseases Information Center, National Center for Advancing Translational Sciences. FAQs about rare diseases. Accessed November 8, 2022. <https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases>. 3. IQVIA Institute for Human Data Science. Orphan drugs in the United States: exclusivity, pricing and treated populations. 2018. Accessed November 8, 2022. https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/orphan-drugs-in-the-united-states-exclusivity-pricing-and-treated-populations.pdf?_ga=2.1548445322680. 4. Institute of Medicine (US) Committee on Accelerating Rare Diseases Research and Orphan Product Development. Field MJ, Boat TF, eds. *Rare Diseases and Orphan Products: Accelerating Research and Development*. National Academies Press (US); 2010. 5. US Government Accountability Office. Rare diseases: although limited, available evidence suggests medical and other costs can be substantial. Updated October 18, 2021. Accessed January 23, 2023. <https://www.gao.gov/products/gao-22-104235>. 6. National Cancer Institute, National Institutes of Health. Rare cancers of childhood treatment (PDQ®)—health professional version. Updated May 25, 2022. Accessed November 8, 2022. <https://www.cancer.gov/types/childhood-cancers/hp/unusual-cancers-childhood-pdq>. 7. Genetic and Rare Diseases Information Center, National Center for Advancing Translational Sciences. Rare cancers. Accessed November 8, 2022. <https://rarediseases.info.nih.gov/diseases?search=cancer>. 8. National Center for Advancing Translational Sciences, National Institutes of Health. NIH study suggests people with rare diseases face significantly higher health care costs. Updated March 23, 2022. Accessed January 23, 2023. <https://ncats.nih.gov/news/releases/2021/nih-study-suggests-people-with-rare-diseases-face-significantly-higher-health-care-costs>. 9. Orphanet. About rare diseases. Updated October 25, 2012. Accessed November 8, 2022. https://www.orpha.net/consor/cgi-bin/Education_AboutRareDiseases.php?lng=EN.

Join us in our quest to reimagine what's possible and transform lives. Visit rarediseases.org

Alone we are RARE.
Together we are STRONG.®

©2023 NORD. All rights reserved. NORD®, its icon, and tagline are registered trademarks of the National Organization for Rare Disorders. NORD is a registered 501(c)(3) charity organization. For more information, visit: rarediseases.org