



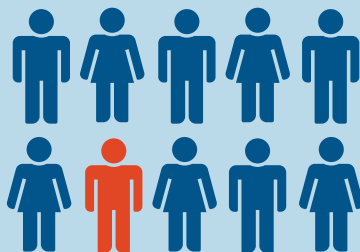
What is a RARE DISEASE ?

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

1 in 10

Americans has a RARE DISEASE

30 million people have a serious, lifelong condition.



More than half are children¹

Holding hands, they would circle the globe about **1.5 times**



7,000

RARE DISEASES exist, with less than 500 FDA-approved treatments²

ONLY 5% of RARE DISEASES have treatments.²

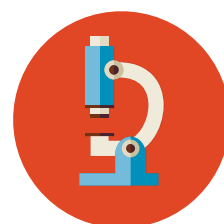
Patients with RARE DISEASES are frequently misdiagnosed or undiagnosed.

80% of RARE DISEASES ARE GENETICALLY BASED.²



Many RARE DISEASES result in premature death of infants & young children or are fatal in early adulthood.²

Families & private foundations provide about **3%** of ALL medical research funding in the U.S.⁶





Recently, more than 1/3 of all NEW drugs approved by FDA have been for RARE DISEASES; and 47% in 2015.³

and yet... 90%



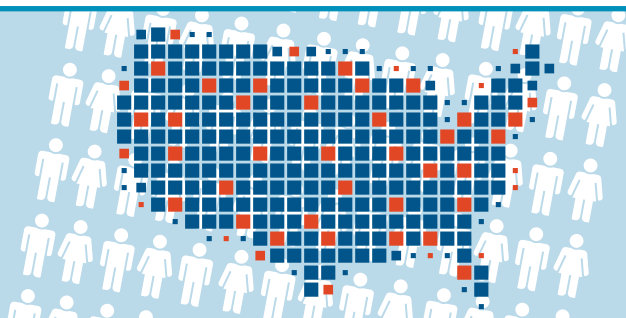
of healthcare providers must treat the majority of RARE DISEASE patients with non-FDA approved drugs.⁴



There are 100 types of cancer. Approximately 50% of people with cancer are battling a RARE cancer.

RARE cancers include brain, pancreatic, ovarian, thyroid, and stomach cancers; leukemia and lymphoma; and all pediatric cancers.⁵

There are more Americans who live with a RARE DISEASE than ALL of those who have either HIV, Heart Disease or Stroke.



75%



of NORD Members are actively funding RARE DISEASE medical research.⁷

What can you do to help?

Visit us and
Donate at: 
rarediseases.org

The content of this report has been obtained through government: FDA's Orphan Drug Product designation database, the National Institutes of Health (NIH), and Institute of Medicine (IOM) and industry sources based on the latest information. Report current as of 6/14/2016. 1 FDA Office of Orphan Products Development (OOPD). 2 "Rare Diseases and Orphan Products: Accelerating Research and Development" Committee on Accelerating Rare Diseases Research and Orphan Product Development Board on Health Sciences Policy. 3 U.S. FDA Novel New Drug Summaries (2011-2015); calculated average from each report's data. 4 Frattarelli, D.A., et al., Off-label use of drugs in children. Pediatrics, 2014. 133(3): p. 563-7. 5 Memorial Sloan Kettering Cancer Center's Cycle for Survival website; <https://www.cycleforsurvival.org/about-cycle-survival>; June 2016. 6 The Financial Times Limited 2016; "Private foundations: Searching for the sweet spot" by Sarah Murray, 6/24/2016, <http://www.ft.com/cms/s/0/57c46446-2d51-11e6-bf8d-26294ad519fc.html#axzz4DjtDphuB>. 7 NORD Membership Survey; taken 2014.