

RARE DISEASE FACTS

Learn more at rarediseases.org



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

Most rare diseases are **genetic** or have a genetic component.³



MORE THAN 90% of rare diseases are **without** an FDA-approved treatment.⁹



For many rare diseases, signs may be observed at birth or in childhood.⁵



MANY RARE DISEASES result in premature deaths of infants and young children, or are fatal in early childhood.⁴

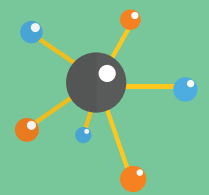


There are approximately **7,000** RARE DISEASES²

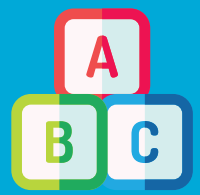
It's estimated that **25-30** MILLION AMERICANS (almost **1 in 10**) have rare diseases.⁷



There are more than **500** types of rare cancers.⁶



ALL PEDIATRIC CANCERS are rare.⁸



Sources: 1.) CFR 316.20 or Sec 526 of the Orphan Drug Act. <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; 11/30/2017. <https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases>. 3.) Institute of Medicine (US) Committee on Accelerating Rare Diseases Research and Orphan Product Development; Field MJ, Boat TF, Washington (DC): National Academies Press (US); 2010. <https://www.ncbi.nlm.nih.gov/pubmed/21796826> <https://www.omim.org/statistics/geneMap>. 4.) Institute of Medicine (US) Committee on Accelerating Rare Diseases Research and Orphan Product Development; Field MJ, Boat TF, Washington (DC): National Academies Press (US); 2010. <https://www.ncbi.nlm.nih.gov/pubmed/21796826>. 5.) Orphanet; About Rare Diseases. Last Updated 10/25/12. https://www.orpha.net/consor/cgi-bin/Education_AboutRareDiseases.php?lng=EN. 6.) Genetic and Rare Diseases Information Center; National Center for Advancing Translational Sciences; Rare Cancers; 1/25/2019. <https://www.youtube.com/watch?v=E55KyRT1qY> | <https://rarediseases.info.nih.gov/diseases/diseases-by-category/1/rare-cancers> or <https://rarediseases.info.nih.gov/diseases/diseases-by-category/1/rare-cancers>. 7.) Genetic and Rare Diseases Information Center; National Center for Advancing Translational Sciences; FAQs About Rare Diseases; Last updated 11/30/2017. <https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases>. 8.) National Cancer Institute, National Institutes of Health: Unusual Cancers of Childhood Treatment (PDQ®)—Health Professional Version was originally published by the National Cancer Institute; August 2018. <https://www.cancer.gov/types/childhood-cancers/hp/unusual-cancers-childhood-pdq>. 9.) IQVIA Institute for Human Data Science. Orphan Drugs in the United States: Exclusivity, Pricing and Treated Populations. 2018 Dec. https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/orphan-drugs-in-the-united-states-exclusivity-pricing-and-treated-populations.pdf?_=1548445322680.

