

RARE CONDITIONS

Are rare diseases really that rare?

While the diseases are rare, 30 million Americans are living with one, which presents **critical cost and care challenges** for payers and patients.

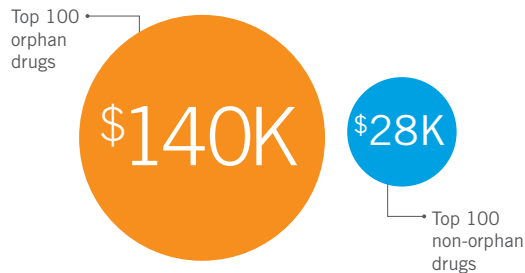
Alarming realities

These diseases cause **catastrophic social, emotional and financial burdens** for patients and their families.

- Difficult to diagnose** – It takes, on average, 7.6 years to correctly diagnose a rare condition.
- High mortality rate** – 30% of children with rare diseases won't live to see their 5th birthday.
- Extreme costs** – Top 10 most expensive drugs in the world are for rare conditions.

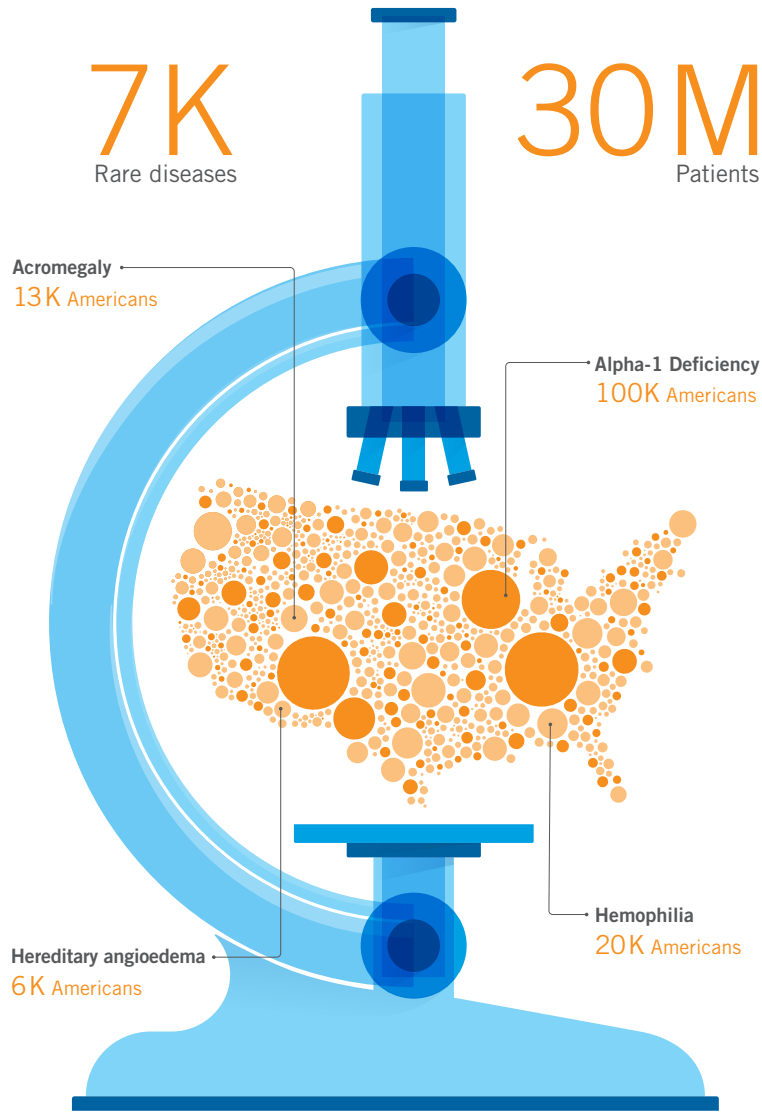
Mounting financial risk

Average cost per patient per year



Some treatments cost plans

\$2.2 million
a year for one patient.



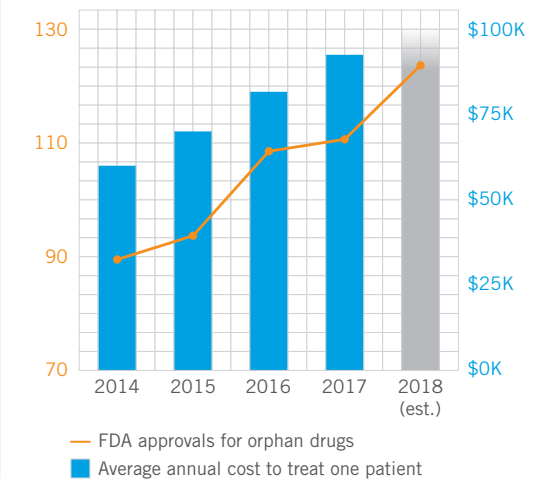
Growing pipeline of orphan drugs

Conditions that **affect fewer than 200K people** in the U.S. are called "orphan" or rare diseases.



Rising approvals and costs

The 1983 Orphan Drug Act created financial incentives for breakthrough therapies. Some manufacturers used those incentives to capitalize on the profitability of orphan drugs, which led to broader investigations regarding the act.



Sources: IOM (Institute of Medicine). 2010. Rare Diseases and Orphan Products: Accelerating Research and Development. Washington, D.C.: The National Academies Press. Pp1, 15, 51 Melnikova, Irena. "Rare diseases and orphan drugs." Nature Reviews Drug Discovery 11.4 (2012): 267-268. Thorat C et. Al. What the orphan drug act has done lately for children with rare diseases: a 10-year analysis. Pediatrics. 2012; 129:516-521 Dodge, John A., et al. "The importance of rare diseases: from the gene to society." Archives of disease in childhood (2010): archdischild193664. Schieppati, Arrigo, et al. "Why rare diseases are an important medical and social issue." The Lancet 371.9629 (2008): 2039-2041. Express Scripts/Accredo Book-of-business data 2016-2017 2017 Orphan Drug Report NORD Fact Sheet: <http://rare-diseaseday.us/about/what-is-a-rare-disease/> <https://globalgenes.org/raredaily/accurate-diagnosis-of-rare-diseases-remains-difficult-despite-strong-physician-interest/> Accredo book of business claims data. Global Genes: Rare Disease Impact Report U.S. Food and Drug Administration; <https://www.fda.gov/downloads/ForIndustry/DevelopingProductsforRareDiseasesConditions/UCM581335.pdf> 2017 Orphan Drug Report, National Organization for Rare Disorders (NORD) Fact Sheet, Express Scripts/Accredo BoB data 2016-2017 Medscape Medical News - <http://www.medscape.com/viewarticle/879422> National Organization for Rare Disorders