

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.

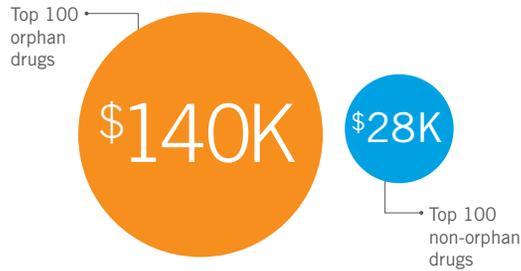
Alarming realities

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

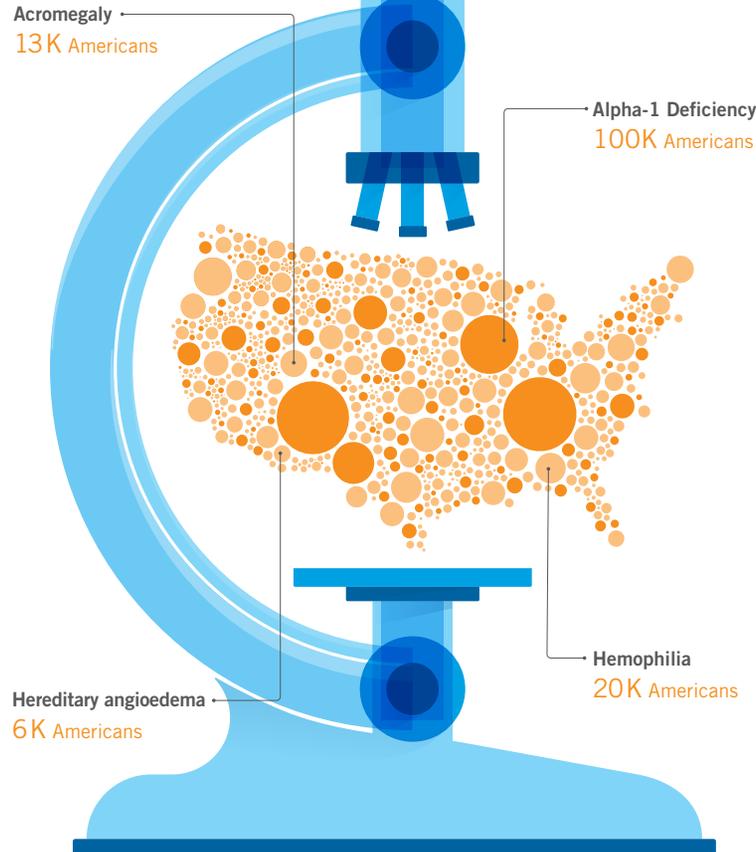
Average cost per patient per year



Some treatments cost plans **\$2.2 million** a year for one patient.

7K Rare diseases

30M Patients



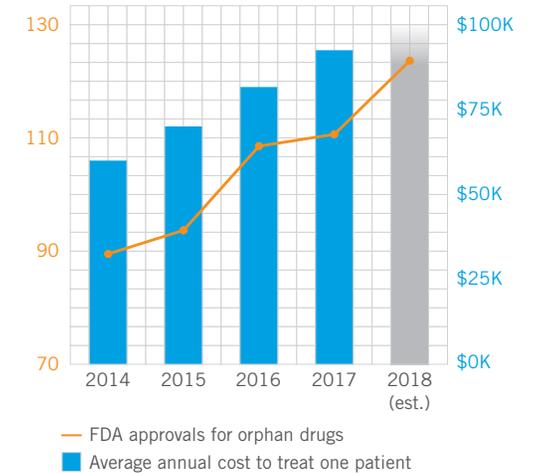
Growing pipeline of orphan drugs

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

- 600+** orphan drug indications approved since 1983
- 1 out of every 3** new FDA-approved drugs is for an orphan designation
- 95%** of rare diseases don't yet have an FDA-approved therapy

Rising approvals and costs

The 1983 Orphan Drug Act created financial incentives for breakthrough therapies. Some manufacturers use those incentives to drive up prices dramatically.



The **Rare Conditions Care ValueSM** program proactively guides patients to the most effective care while protecting your plan against unexpected, staggering costs. It adds to our market-leading offerings for treating rare diseases, including Medical Channel Management and Rare Advanced Utilization Management.

Another powerful solution in the Express Scripts SafeGuardRx[®] suite | express-scripts.com/corporate

Sources: IOM (Institute of Medicine). 2010. Rare Diseases and Orphan Products: Accelerating Research and Development. Washington, D.C.: The National Academies Press. Pp.1, 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): archdischild.193664. 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/rare-daily/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

