Report abstract

High orphan drug costs a continuing concern

Rare-disease medications bring hope to many

Orphan drugs represent lifelines for people who suffer from serious and life-threatening rare diseases. An orphan drug is used to prevent, diagnose or treat a disease or condition that affects fewer than 200,000 Americans.[1] Examples of these rare diseases include Gaucher's disease, cystic fibrosis and Duchenne muscular dystrophy. These diseases are often chronic, progressive, degenerative, and it is estimated that 85 to 90 percent are serious or life threatening.[2] Eighty percent of rare diseases are genetic and half are diagnosed in children.[3]

The total number of Americans living with a rare disease is estimated at between 25 and 30 million, and there are an estimated 7,000 types of rare diseases.[1] Of those 7,000, 95 percent lack an FDA-approved treatment.[3]

Law enacted to encourage development

The Orphan Drug Act was passed by the U.S. Congress in 1983 to spur research and development of treatments for rare diseases. This was in answer to there being "no reasonable expectation" that sales in the U.S. could support the development and marketing costs of developing rare disease treatments. At the time of its passage, only 38 orphan drugs were available. In large part due to incentives in the orphan drug law, that number now exceeds 750.[4] In 2018, 58 percent of all new drugs approved by the Federal Food & Drug Administration (FDA) had an orphan designation.[5]

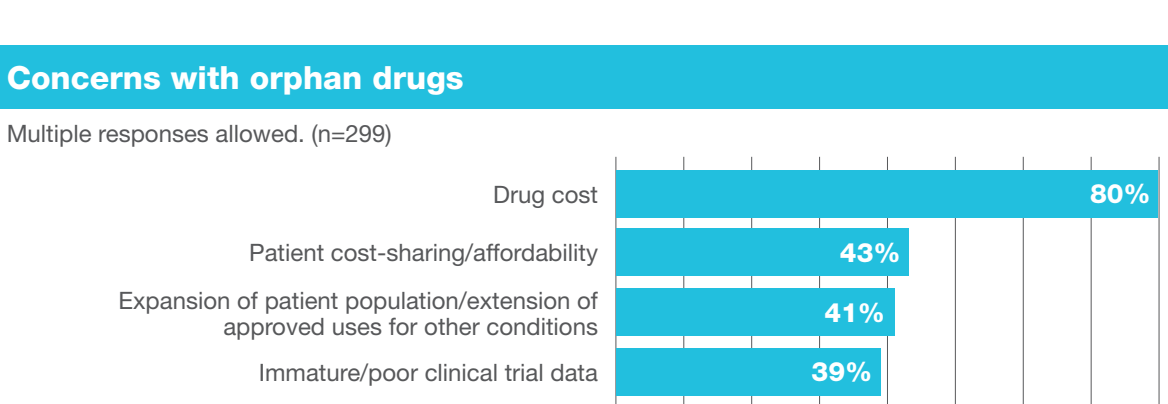
Cost burdens remain high

Orphan drugs can be life-extending or lifesaving for patients suffering from conditions that previously had no treatment, but they also come with a steep price. The median price for an orphan drug in 2017 was over $46,800.[6] However, as a percentage of national drug spending, orphan drugs make up less than 10 percent.[6]

Research published by the Pharmacy Benefit Management Institute (PBMI) found that plan sponsors are concerned about the cost of orphan drugs—with the highest concern associated with plan drug costs (80 percent) and patient cost-sharing and affordability (43 percent).[7] Seventy-one percent of employers surveyed said that they are not confident that the current prices of orphan drugs are sustainable.[7]

Concerns with orphan drugs

Multiple responses allowed. (n=299)



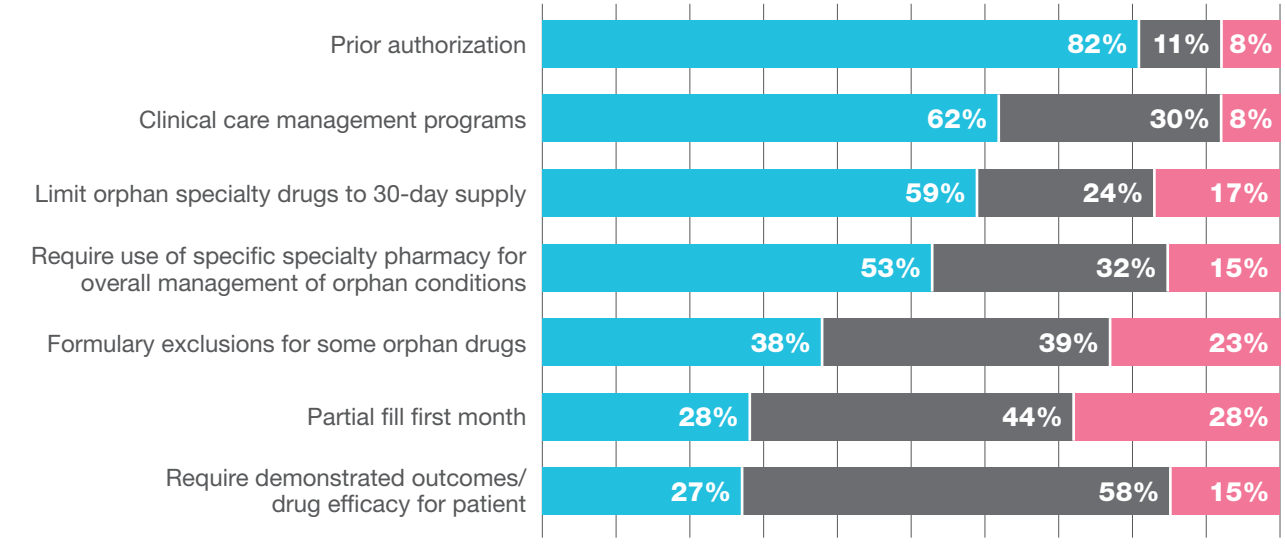
More information ▶

Cost burdens remain high (cont.)

While much continues to be done to encourage research into treatments for rare diseases, the cost of these specialty products has been under the spotlight. Even with programs such as prior authorization in place to help control costs, rare diseases often are associated with very high cost-of-care, and patients may require a large medical support team and frequent hospitalization as well as significant loss of school/job productivity for the patient and caregivers.

Cost control programs available but often underutilized

Programs in place for orphan drugs (n=299)



When it comes to covering orphan drugs, 70 percent of employers feel that they lack enough information to help them make actionable decisions for their drug benefit plans.[7] Employers expressed interest for more information on topics including cost effectiveness, clinical efficacy, available alternative treatment options, patient benefits and cost transparency.[7]

This information is summarized from the *PBMI 2019 Trends in Specialty Drug Benefits* report proudly sponsored by Walgreens and AllianceRx Walgreens Prime. To access the full report, visit PBMI.com/specialtyreports.

1. U. S. Department of Health & Human Services. National Institutes of Health. FAQs about Rare Diseases. https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases. Updated November 30, 2017. Accessed March 8, 2019. **2.** U.S. Food & Drug Administration. Orphan Drug Modernization Plan. https://www.fda.gov/downloads/ForIndustry/DevelopingProductsforRareDiseasesConditions/HowtoapplyforOrphanProductDesignation/UCM565068.pdf. Accessed April 10, 2019 **3.** Global Genes. Rare Facts. https://globalgenes.org/rare-facts/. Accessed March 8, 2019. **4.** U.S. Food & Drug Administration. Search Orphan Drug Designations and Approvals. https://www.accessdata.fda.gov/scripts/opdlisting/oopd/listResult.cfm. Accessed March 25, 2019. **5.** Regulatory Affairs Professionals Society (RAPS). FDA's Record Year: A Look at 2018 New Drug Approvals. https://www.raps.org/news-and-articles/news-articles/2019/1/fdas-record-year-a-look-at-2018-new-drug-approva. Updated January 7, 2019. Accessed March 22, 2019. **6.** IQVIA Institute for Human Data Science. Orphan Drugs in the United States. October 2018. https://www.iqvia.com/-/media/iqvia/pdfs/institute-reports/orphan-drugs-in-the-united-states-growth-trends-in-rare-disease-treatments.pdf?_=1549028146081. Accessed March 28, 2019. **7.** Pharmacy Benefit Management Institute. 2018. Trends in Specialty Drug Benefits. Plano, TX: PBMI. Available from www.pbmi.com/specialtyreports.

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