

## Orphan Drug program

Manage high-cost therapies for rare conditions



Orphan drugs are often fast-tracked to market to treat cystic fibrosis, Lou Gehrig's disease and around 7,000 other rare conditions. And at an average cost of \$147,000 per year, these medications are the best hope for the 10 percent of Americans who have a rare condition.<sup>1,2</sup>

### Orphan drugs today

**\$147,000**  
average  
annual cost

Up to  
**\$750,000**  
annual cost<sup>3</sup>

Account for  
**17%** of total  
worldwide  
pharmacy sales<sup>4</sup>

Expected to  
account for  
**22%** of drug  
sales by 2024<sup>5</sup>

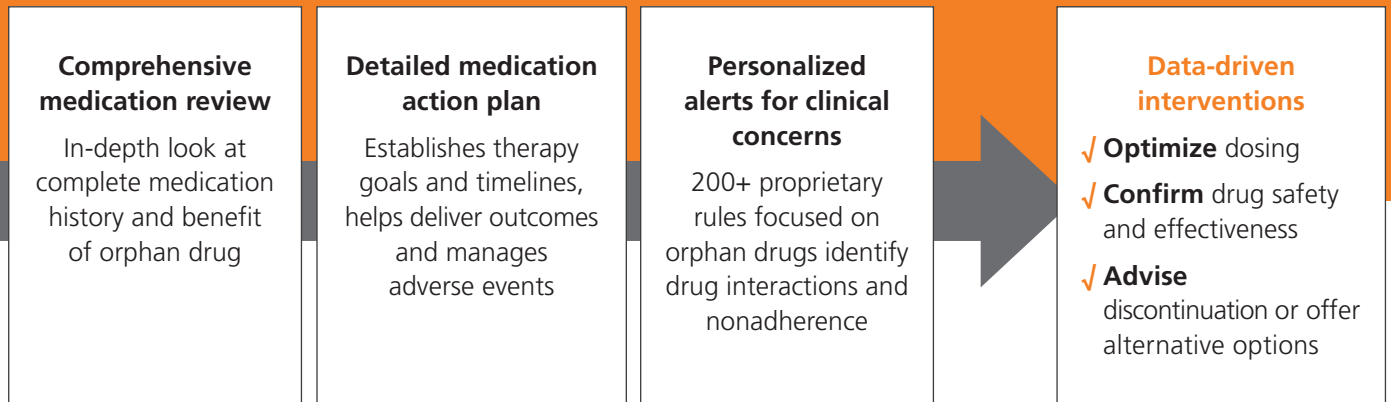
So how do you best manage costs while making sure members get safe, effective and highly specialized medication they need? The OptumRx Orphan Drug program gives you insight into appropriate drug therapy, achieving optimal health outcomes through expert support.

## Personalize clinical support

Orphan drugs may be effective for some people and not others because of differences in response and tolerability. That sensitivity, combined with often complicated dosing, opens up opportunities for pharmacists to optimize care.

With the Orphan Drug program, we provide ongoing personalized support for members who take one of 19 orphan drugs we've identified as having the greatest opportunity for clinical intervention and cost savings.<sup>6</sup> OptumRx pharmacists who specialize in orphan drugs tailor one-on-one experiences for these members, offering advanced clinical counseling to address unique medication needs.

Our rules-driven platform enables clinical pharmacists to tailor optimal orphan drug therapy for its members:



## Member experience

Early detection of ineffective therapy

Here's an example of how the Orphan Drug program can help guide members and their care providers to optimal treatment and support.

### Meet Pam, age 62

Parkinson's disease, high blood pressure



**1 First orphan drug fill:** Pam's neurologist prescribes Northera to treat dizziness. She fills it at a specialty pharmacy and receives prior authorization.



**2 Claims identification:** Our claims system identifies Pam as filling a prescription for a drug included in the Orphan Drug program. She is automatically enrolled in the program and receives a welcome letter.



## Optimize care

By investing in clinical expertise and careful monitoring of each member's experience with an orphan drug, we can quickly identify cases of potentially ineffective therapy and work with physicians to make informed decisions about next steps.

For example, orphan drug Northera® costs more than \$300 per day and may actually worsen some conditions. The Orphan Drug program is only a fraction of that cost, at around \$300 per engaged member per year. Early discontinuation of ineffective therapy can lead to better health outcomes as well as significant overall savings.



### Program cost:

\$300 per engaged member per year

- Expands beyond prior authorization to continuously monitor and validate therapeutic value
- Provides quality assessment and clinical support for busy physicians and care specialists
- Avoids prolonged drug exposure, risks and costs if the therapy is not achieving adequate outcomes
- Engages and informs members through ongoing personalized consultations to optimize adherence and clinical outcomes



### Drug cost:

>\$300 per day for Northera®

- Treats dizziness and lightheadedness associated with Parkinson's disease
- FDA approved based on limited safety and efficacy data
- Includes black box warning in the labeling
- May worsen coronary artery disease, arrhythmias and congestive heart failure
- Requires frequent assessment to verify benefit beyond two weeks of treatment

**3 3–4 days after fill:** An OptumRx pharmacist who specializes in orphan drugs calls Pam for her first counseling session. They review her medications, establish date-specific therapy goals, and develop a medication-specific action plan with blood pressure monitoring.



**4 3 weeks later:** The pharmacist sees that Pam has a new blood pressure medication and asks her about it during their next session. Her dizziness has worsened, so the pharmacist contacts Pam's neurologist. After reviewing options and supporting evidence, they switch from Northera to a new therapy.



**5 3 months later:** Pam continues on the new therapy after doing well with it for a few months. The pharmacist contacts Pam regularly to monitor her blood pressure and continue managing her Parkinson's disease.



## About OptumRx

OptumRx is a pharmacy care services company helping clients and more than 65 million members achieve better health outcomes and lower overall costs through innovative prescription drug benefits services. OptumRx is part of Optum, a leading information and technology-enabled health services business dedicated to making the health system work better for everyone. For more information [optum.com/optumrx](https://optum.com/optumrx).

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To learn more about the OptumRx Orphan Drug program and our clinical strategy, contact your OptumRx representative or send an email to [optumrx@optum.com](mailto:optumrx@optum.com).

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1. EvaluatePharma. Orphan Drug Report 2018. Published May 2018. Accessed Jan. 17, 2019.
2. Global Genes. Rare Diseases Facts and Statistics. [globalgenes.org/rare-diseases-facts-statistics/](https://globalgenes.org/rare-diseases-facts-statistics/). Accessed Jan. 17, 2019.
3. Managed Care. Orphan Drugs: Way Too Many, Way Too Expensive. Published June 4, 2017. Accessed Jan. 17, 2019.
4. EvaluatePharma. Orphan Drug Report 2018. Published May 2018. Accessed Jan. 17, 2019.
5. Ibid.
6. OptumRx internal analysis of utilization data. 2018. We will continue to assess the market and add medications to the program as warranted.



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