

A rare perspective

Providing adequate coverage and care for rare-condition patients is challenging from all points of view.

A condition is commonly classified as “rare” if it affects fewer than 200,000¹ people. But if we only see rare conditions from this perspective — a class of diseases that affect small patient populations — we risk missing out on the big picture.

30 million people in the U.S. are living with a rare condition.² Prevalence and costs are on the rise as the number of known rare conditions and available therapies continue to grow. Are you prepared?



Orphan drugs cost \$140,000 on average per patient, per year.^{3*}

The 10 most expensive drugs in the world are for rare conditions⁴, and costs can skyrocket depending on the condition. The average plan spend for hereditary angioedema, for example, can be \$2.2 million a patient for a single year.⁵

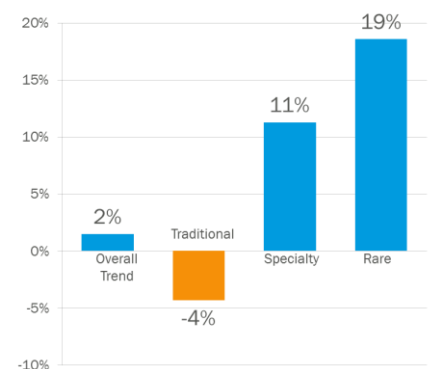
It takes an average of 7.6 years to diagnose a rare condition.²

Patients visit an average of 7 physicians on their path toward an accurate diagnosis.² They often experience conflicting prognoses and require dosing adjustments to find the right balance of medication, further driving up plan and out-of-pocket expenses.

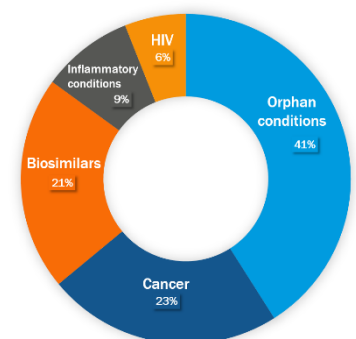
One-third of new FDA-approved drugs are for an orphan designation.^{1*}

Products for rare conditions are coming to market faster than ever, giving hope to millions of patients but increasing the likelihood that your plan will need to cover a very costly therapy.

Rare condition trend⁶



Proportion of specialty pipeline drugs pending approval⁷



1. National Organization for Rare Disorders.
2. Global Genes: Rare Disease Impact Report
3. EvaluatePharma 2017 Orphan Drug Report (Cost per patient is an estimate for the retail cost of a drug to a patient, for a given year, based on a 100% compliance to the treatment guidelines outlined in the FDA label)
4. Medscape, “Rare Disease Treatments Make Up Top 10 Most Costly Drugs”
5. Accredo Book of Business claims data for 2017 calendar year
6. Express Scripts 2017 Drug Trend Report
7. Based on novel drugs in development for specialty conditions as represented on the Specialty Top 20 report and Supplement List that are currently pending approval at the FDA

*The Orphan Drug Designation program provides orphan status to drugs and biologics intended for the safe and effective treatment, diagnosis or prevention of rare conditions affecting <200,000 people in the U.S., or >200,000 people but are not expected to recover the costs of development and marketing.

Rare Conditions Care ValueSM Program

To guide patients to the most effective care pathway and protect your plan from unexpected, staggering costs.

The SafeGuardRx[®] suite of solutions has proven that by combining specialized care with value-based strategies, we can put the right care within reach and contain costs. Now, we're applying that model to rare conditions.

The **Rare Conditions Care ValueSM** program will combine **clinical specialization** and **cost containment** tools for conditions that pose the greatest challenges, such as acromegaly, alpha-1 deficiency, Gaucher's disease, hemophilia, hereditary angioedema, Huntington's disease and idiopathic pulmonary fibrosis. The program is designed to adapt to a rapidly changing landscape, and whenever we need to expand to additional rare conditions, we will.



Specialized support for rare-condition patients.

Accredo[®] specialty pharmacy delivers enhanced care to patients taking rare-condition medications. Our Therapeutic Resource CentersSM offer support from specialist pharmacists, nurses and other pharmacy experts who have **extensive training in rare conditions and medications**. They provide:

- One-on-one patient counseling and education
- In-home nursing services for products requiring extensive training, administration or infusion assistance
- Proprietary drug-specific protocols and patient management programs to ensure safe and appropriate use of medications
- Personalized support in managing other health-related challenges, including resources to support the patient's financial, emotional and social well-being



Cost containment strategies for an evolving rare-conditions landscape.

Rare Conditions Care Value will proactively limit your plan's financial exposure through:

- **Financial protection** for high-cost utilizers in select therapy classes
- **Cost savings** when prescriptions for preferred products are filled at the Accredo specialty pharmacy
- **Driving to the lowest-cost drugs** through the National Preferred Formulary and/or Utilization Management tools



A second opinion provides additional protection.

Managed through PinnacleCare, our Second Opinion service objectively guides patients to top medical specialists at nationally acclaimed Centers of Excellence. These experts conduct medical reviews of the rare-condition patient's entire health profile to help **ensure proper diagnosis and treatment path**. This additional service provides a personal health advisor to the patient to coordinate care and decision support between their team of providers and top specialists throughout the country.

Investing in this additional service keeps patients safe and protects your plan from clinically unnecessary pharmacy and medical costs. **77% of engagements with PinnacleCare result in a change in diagnosis, therapy regimen or a treating physician.**⁸ Nine out of 10 members accept the new recommendation and report a **99% overall satisfaction rate.**⁸ This service is offered on a per-review cost basis; there is no cost to the patient.



For more information on the Rare Conditions Care Value program and SafeGuardRx, please contact your Account Executive.

⁸. PinnacleCare data