Are rare diseases really that rare?

Conditions that affect fewer than 200K people in the U.S. are called “orphan” or rare diseases. The individual diseases may be rare, but collectively affect 1 in 10 Americans and present **critical cost and care challenges** for payers as well as patients.

### Alarming realities

These diseases have a high early mortality rate and cause **catastrophic social, emotional and financial burdens** for patients and their families.

- **Difficult to diagnose** – On average, patients wait nearly 5 years for an accurate rare disease diagnosis and see 7 physicians during that time.
- **High mortality rate** – 30% of children with rare diseases won’t live to see their 5th birthday.
- **High costs** – Annual costs per patient are more than 5x higher than non-orphan drugs.

### Financial exposure

**Average cost per patient in 2016**

- **Top 100 orphan drugs**
  - $140K
- **Top 100 non-orphan drugs**
  - $28K

**441** FDA-approved orphan drugs

**35%** of specialty drugs in the pipeline are orphan

**95%** of rare diseases don’t have an FDA-approved therapy

### Good news/bad news

It’s life-changing for patients when new therapies become available, but the costs can be staggering. Here are just a few examples...

<table>
<thead>
<tr>
<th>Rare disease conditions</th>
<th>Average spend per patient</th>
</tr>
</thead>
<tbody>
<tr>
<td>Bleeding disorders (factor)</td>
<td>$1,603,783</td>
</tr>
<tr>
<td>Hereditary angioedema</td>
<td>$3,090,961</td>
</tr>
<tr>
<td>Rare congenital and acquired general lipodystrophy</td>
<td>$1,011,022</td>
</tr>
</tbody>
</table>

### Rapid increase in approvals and cost

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

**Specialty drug approvals**

- **Orphan**
- **Non-orphan**

### See how Express Scripts and Accredo can help you be prepared...
Express Scripts and Accredo help your plan ...

Identify top areas of spend for high-cost therapies, both for current patients and those new to service

Control unnecessary costs by implementing utilization management solutions

Provide exceptional patient-centered support through Accredo's specialized care model

Ask about our solutions for managing rare diseases – focused on appropriate dosing, safety and adherence

Sources:
7. 2017 Orphan Drug Report
8. NORD Fact Sheet: http://rarediseaseday.us/about/what-is-a-rare-disease/
10. Accredo book of business claims data. Spend figures represent the average spend per patient with a total spend of >=250K for the 2016 calendar year.