

# Trends in US payer management of competitive rare disease

In recent years, the number of rare diseases with multiple approved therapies has skyrocketed. In 2015, there were **seven rare diseases with >three approved therapies**; in 2025, the number has **increased to 54**. In some categories, the competition for a relatively small number of potential patients is significant; for example, HAE has **six approved brands for prophylaxis and only ~9,000 US patients**.

## Increasing spend on rare disease


- In 2024, 72% of FDA-approved drugs had Orphan Drug Designation
- Average launch prices for orphan drugs are 7x higher than non-orphan drugs
- In 2022, 19 of the 100 Rx drugs with the highest annual net sales were approved only for rare diseases
- 1 in 10 Americans suffers from a rare disease, ~30M
- Increasing rare disease category spend, seen as unsustainable
- Payers opting to manage categories with even modest competition
- Increased contracting in rare disease
- More restrictive management, including PA to trial inclusion/exclusion criteria and exclusions


To understand predictors of differential payer management and identify strategies manufacturers can deploy to ensure favorable access, CRA examined several competitive rare disease analogues


## Summary of payer management of select rare disease classes

Class	Est. # of US patients	No. of approved brands	Extent of clinical differentiation	Rx annual price range	Extent of differential management
HAE	9,000	6 (prophy)	Low-moderate	\$600-700K	HIGH
NMOSD	22,000	4 + biosim	Moderate	\$125-750K	HIGH
DMD	15,000	8	High	<\$100K - \$3.2M	LOW
Gaucher	6,000	5	Low	\$175-600K	HIGH

### Drivers of favorable access

 Pediatric indication

 Well-defined, distinct patient subpopulations

 First or second to market

### Potential manufacturer strategies

**Clinical differentiation**

Clinically meaningful improvements vs. SoC are key

**KOL advocacy & value communication**

Invest in value communication and KOL relationships

**Pricing & contracting**

Significant premiums likely to result in disadvantaged access

## Sources

<https://www.ajmc.com/view/us-spending-on-high-revenue-rare-disease-drugs-in-2022>; <https://www.nih.gov/news-events/news-releases/nih-study-suggests-people-rare-diseases-face-significantly-higher-health-care-costs#:~:text=The%20team%20reported%20that%20extrapolating%20heart%20failure%20and%20Alzheimer's%20disease>; <https://www.reuters.com/business/healthcare-pharmaceuticals/prices-new-us-drugs-doubled-4-years-focus-rare-disease-grows-2025-05-22/#:~:text=%22For%20years%20we've%20had,FOLLOWING%20THE%20SCIENCE>; [https://www.annallergy.org/article/S1081-1206\(25\)00258-3/fulltext](https://www.annallergy.org/article/S1081-1206(25)00258-3/fulltext); <https://pmc.ncbi.nlm.nih.gov/articles/PMC11282172/>; <https://cureduchenne.org/about/what-is-duchenne/>; <https://www.pfizer.com/disease-and-conditions/gaucher-disease#:~:text=Prevalence%20of%20Gaucher%20Disease,to%201%20in%20100%20C000%20births.&text=As%20of%202018%20approximately%206%20C000,were%20diagnosed%20with%20Gaucher%20Disease.&text=It%20most%20commonly%20affects%20people%20of%20Ashkenazi%20Jewish%20descent>.

## Contact

Joseph Black, Principal  
+1-212-520-7287 | jblack@crai.com