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Rare Disease Service

Commercial Health Plans: Rare Disease & Advanced Therapy Management, Financing, and Manufacturer Engagement

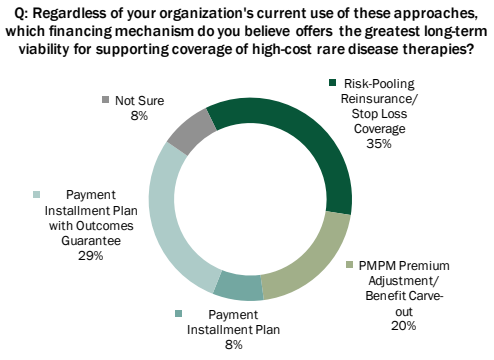
Rare disease and advanced therapy markets continue to expand as robust pipelines, accelerated innovation, and a growing number of high-cost therapies reshape the treatment landscape. HIRC's report, *Commercial Health Plans: Rare Disease & Advanced Therapy Management, Financing, and Manufacturer Engagement*, examines plans' handling of high and ultra high-cost therapies across 20+ therapeutic categories and evaluates best-in-class manufacturer support. The report addresses the following questions:

- What has changed for plans in the last 12-18 months in rare disease and how are they adapting in response? What are plans most focused on in 2026-2027 to better manage rare disease and advanced cell & gene therapies (CGTs)?
- What specific utilization and formulary management tactics are applied across 20+ unique rare disease and advanced therapy categories?
- Which financing mechanisms and protection programs are plans using/exploring for rare disease medications? Which do they view as having the most potential for sustainable long-term viability?
- What is the nature of the manufacturer contracting environment across 20+ broad rare disease and CGT categories?
- How are manufacturers supporting commercial plans and patients in rare disease?

Key Finding: Commercial plans are becoming more active in rare disease management as pipelines, costs, and competition expand, but their clinical and formulary capabilities are advancing faster than the contracting and financial solutions available to manage growing exposure.

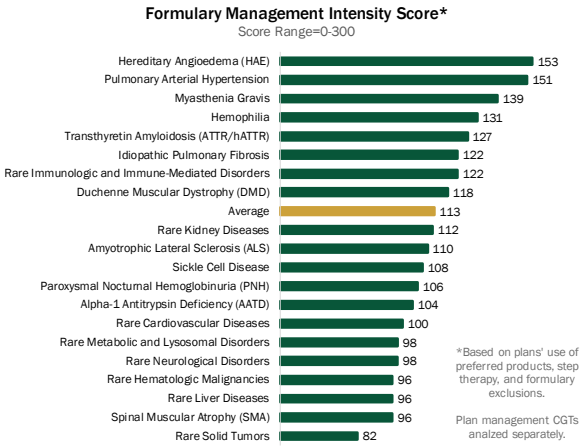
Evolving Financial Protection & Contracting Models

While payer management is maturing, the the financing models and mechanisms for absorbing increased costs haven't matured at the same pace. When it comes to **financial protection programs**, plans are turning primarily to established tools such as risk-pooling reinsurance/stop loss, while viewing outcomes-linked installment payments as having strong long-term viability (though largely not in use yet). **Manufacturer contracting** in rare disease lags comparatively to conventional specialty categories overall. Traditional contracting approaches are observed in select rare disease areas with competition while risk/outcomes-based agreements are most often reported in gene therapy. The full report examines financial protection program use and the contracting environment extensively.



Competition Expands Formulary Management Opportunities in Select Areas

Rigorous clinical eligibility remains foundational to rare disease management, but expanding treatment options are giving plans more opportunity to actively manage formulary access. Across higher-intensity categories, commercial plans are layering preferred products, step therapy, and selective exclusions onto established prior authorization requirements as therapeutic competition grows.



Plans report exerting the most formulary management intensity in pulmonary arterial hypertension, hereditary angioedema, and myasthenia gravis in 2026. The full report reviews the payer management landscape across 20+ rare disease and CGT categories in detail.

Manufacturer Benchmarks Highlight Leaders in Rare Disease Engagement

Plans were asked to consider and provide a best-in-class manufacturer nomination across several rare disease engagement parameters. Genentech, Novartis, Sanofi, and Vertex are among those nominated as best across categories.

Top Manufacturers in Rare Disease & Advanced Therapy Support, 2026
Ranked by Number of Nominations in Each Category

Overall Partner of Choice	Best-in-Class Programs/Resources	Best Product Launch
• Genentech • Novartis • CSL Behring* • Sanofi* • Vertex*	• Sanofi • J&J Innovative Medicine* • Novartis* • Pfizer* • Vertex* • Alexion	• Vertex • Sarepta Therapeutics • BridgeBio* • CSL Behring* • Genentech* • Regeneron*

*Indicates a tie

Payers increasingly define strong manufacturer engagement through knowledgeable account support, early and transparent information exchange, and resources that complement existing plan workflows. The complete report provides the full listing of manufacturers recognized and drivers of nominations accompanied by in-depth qualitative insights.

Research Methodology and Report Availability

In July and August, HIRC surveyed 49 pharmacy and medical directors from national, regional, and BCBS plans representing 142 million lives. Online surveys and follow-up telephone interviews were used to gather information. The *Commercial Health Plans: Rare Disease & Advanced Therapy Management, Financing, and Manufacturer Engagement* report is part of HIRC's Rare Disease Service, and is available to subscribers at www.hirc.com.

HIRC's Rare Disease Service examines the evolving stakeholder perspectives, financing approaches, and distribution models shaping access to rare disease and advanced cell & gene therapies. Benchmarks manufacturer engagement and contracting across commercial payer and specialty pharmacy segments. To subscribe to, contact:

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