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Specialty Pharmaceuticals

Rare Disease & Gene Therapy: Commercial Health Plan Management, Financing, and Manufacturer Engagement

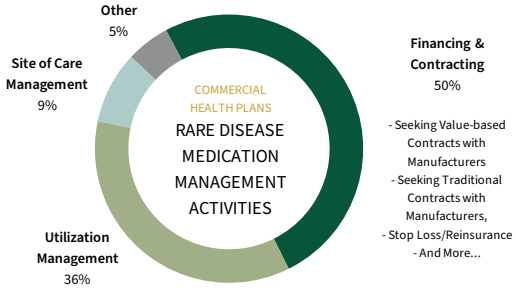
A number of innovative rare disease and gene therapy treatments have entered the market, requiring novel utilization management, financing, and contracting approaches. HIRC's report, *Rare Disease & Gene Therapy: Commercial Health Plan Management, Financing, and Manufacturer Engagement*, examines plans' handling of high and ultra high-cost therapies across 18 rare disease states and evaluates best-in-class manufacturer support. The report addresses the following questions:

- Which rare disease states and therapy types are highest priority for commercial MCO management? How are plans working to manage the cost and utilization of rare disease and gene therapies in 2023/2024?
- Which financing mechanisms are plans using/exploring for rare disease medications?
- What is the nature of the contracting environment across 18 broad rare disease states?
- How interested are commercial MCOs in novel contracting arrangements for rare disease & gene therapies?
- How are manufacturers supporting commercial plans and patients in rare disease?
- What do commercial health plans, and their members, need going forward to best ensure access and adherence to rare disease and gene therapy treatments?

Key Finding: Prior authorization is among the primary tools used by commercial plans to manage access and appropriate use of rare disease medications, though in some classes there are now enough products for plans to designate preferred products and begin to leverage step therapy.

Value-based Contracting & Prior Authorization Top List of Commercial MCOs Efforts to Manage Rare Disease Spend

Commercial MCOs are managing the cost of rare disease and gene therapies mostly through a combination of novel financing/contracting mechanisms and traditional utilization management tactics.

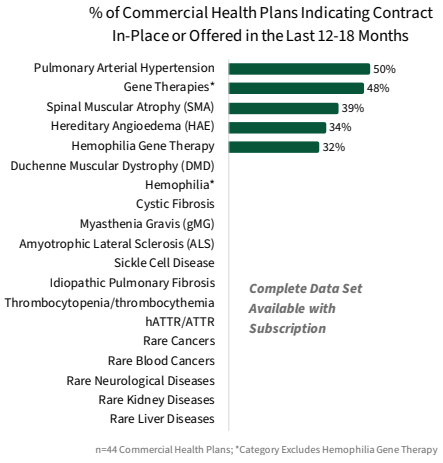


Specifically, plans are interested in and actively seeking value/outcomes-based contracts and have come to expected them to accompany ultra-high cost therapies through the form of warranties, rebates, or reimbursement adjustments.

Plans are also implementing strong PA clinical requirements, often considering the clinical trial inclusion/exclusion criteria and focusing on reauthorizations to ensure response to therapy. The full report examines management activities in detail across 18 rare disease categories.

Financing & Contracting Considerations in Rare Disease

Commercial MCOs are evaluating a variety unique benefit designs and other methods to finance rare disease & gene therapies, discussed in the complete report.



When it comes to contracting with manufacturers, the approach varies by therapy type. Traditional access-based contracts are used, and a higher prevalence of risk/outcomes-based contracts are observed compared to conventional specialty drugs. Contracts are most often reported in pulmonary arterial hypertension (PAH), gene therapies, spinal muscular atrophy (SMA), and hereditary angioedema (HAE).

The full report examines the rare disease contracting environment in detail, as well as plans' interest in prevalence-based rebates, value/outcomes-based contracts, and pay-over-time arrangements.

Genentech and Novartis Are Among Leaders in Rare Disease Engagement with Commercial MCOs

Plans were asked to consider and provide a best-in-class manufacturer nomination across three rare disease engagement parameters as noted below. Genentech, Novartis, bluebird bio, CSL Behring, & BioMarin are consistently among those nominated as best across categories.

TOP MANUFACTURERS IN RARE DISEASE & GENE THERAPY SUPPORT,

Ranked by Number of Nominations in Each Category

Overall Partner of Choice	Best-in-Class Programs/Resources	Best Product Launch
• Genentech • Novartis • bluebird bio, CSL Behring, Sarepta*	• Genentech • Novartis • BioMarin	• BioMarin • bluebird bio • CSL Behring, Sanofi*

*Indicates a tie

Many of the leading manufacturers in rare disease engagement in 2023 had recent launches in the gene therapy space, where products are accompanied by significant support services, novel contracting arrangements, and site of care assistance. The complete report provides the full listing of manufacturers recognized, drivers of best-in-class nominations, and a commercial MCO needs assessment.

Research Methodology and Report Availability

In October, HIRC surveyed 44 pharmacy and medical directors from national, regional, and BCBS plans representing 109 million lives. Online surveys and follow-up telephone interviews were used to gather information. The *Rare Disease & Gene Therapy: Commercial Health Plan Management, Financing, and Manufacturer Engagement* report is part of the Specialty Pharmaceuticals Service, and is now available to subscribers at www.hirc.com.

The **Specialty Pharmaceuticals Service** monitors managed care trends related to specialty medication management, such as payers' formulary & utilization management approaches and trends in contracting. Issues in specialty pharmacy distribution and engagement are also reviewed. The service benchmarks manufacturers across several high profile therapeutic classes. For subscription information please contact:

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