



# Special Report Series

## Rare Disease & Gene Therapy: Specialty Pharmacy Provider Distribution and Manufacturer Engagement

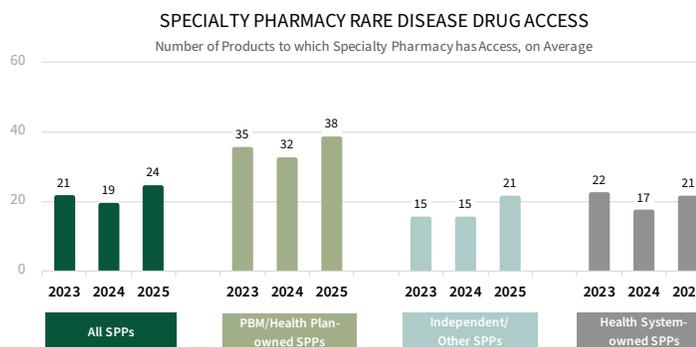
Specialty pharmacies have developed robust and unique capabilities to support the growing market of rare disease & gene therapies, and can serve as strong partners in patient access and adherence. HIRC's report, *Rare Disease & Gene Therapy: Specialty Pharmacy Provider Distribution & Manufacturer Engagement*, examines issues in dispensing and distribution networks, contracting approaches, and evaluates best-in-class manufacturer support. The report addresses the following:

- What do specialty pharmacy providers (SPPs) report as their access to rare disease & gene therapy medications? How does this vary by SPP ownership type (e.g., PBM/health plan-owned, independent, health system-owned)?
- What are the trends in manufacturer specialty pharmacy distribution networks for rare disease & gene therapies?
- What are SPPs' capabilities in rare disease dispensing? How does this differ by ownership type?
- What contracting approaches are manufacturers engaging in to support SPPs' clinical and data collection activities for rare diseases?
- Which manufacturers are considered best-in-class in SPP rare disease engagement?
- What opportunities exist for manufacturers to better support SPPs in rare disease?

**Key Finding:** The best manufacturers in rare disease specialty pharmacy engagement collaborate strategically with SPPs, enabling both partners to deliver the shared goal of getting patients on therapy, keeping them on therapy, and improving outcomes.

## Rare Disease Dispensing & Specialty Pharmacy Network Trends

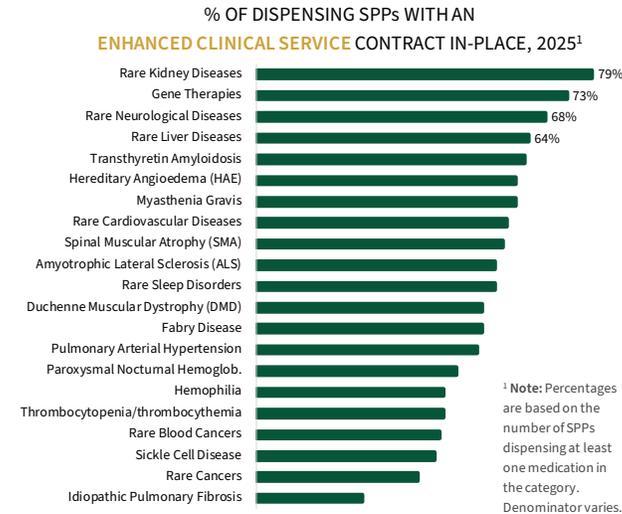
Specialty pharmacy providers report access to 24 rare disease or gene therapy products on average in 2025, up from 19 in 2024. While PBM/health plan-owned SPPs report access to a greater number of rare disease products, interview data suggest networks for rare disease medications are becoming increasingly narrow and that independent/specialized SPPs and health system-owned SPPs (when qualified) are gaining ground.



The full report examines trends in rare disease therapy dispensing, specialty pharmacy networks, and insights to help inform network selection strategy.

## Specialty Pharmacy Contracting Approaches in Rare Disease

Contracts between manufacturers and specialty pharmacies are relatively common to support rare disease and gene therapy dispensing. HIRC's research examines the prevalence of four unique enhanced services and pricing/performance contract types.



Enhanced clinical service contracts are most common overall, especially for rare kidney disease medications and gene therapy. The clinical services included are highly tailored to the therapy but are typically in the form of additional customized outreach to ensure that patients stay on therapy, or in the case of gene therapy, may be more focused on front-end

tasks. The complete report examines the contracting environment in detail across 20+ rare disease areas and by SPP ownership type.

## AstraZeneca/Alexion, Pfizer, and Bristol Myers Squibb Among Leaders in Rare Disease Engagement with SPPs

Plans were asked to consider and provide a best-in-class manufacturer nomination across several rare disease engagement parameters as noted below. AstraZeneca/Alexion, Pfizer, and Bristol Myers Squibb are consistently among those nominated as best in 2025. AstraZeneca/Alexion is recognized especially by health system-owned SPPs.

### TOP MANUFACTURERS IN RARE DISEASE & GENE THERAPY SPP ENGAGEMENT, Ranked by Number of Nominations in Each Category

Partner of Choice	Best New Product Launch	Best Rare Disease Account Managers	Best-in-Class Collaborations
<ul style="list-style-type: none"> <li>AstraZeneca/Alexion</li> <li>Bristol Myers Squibb, Pfizer Sanofi*</li> </ul>	<ul style="list-style-type: none"> <li>Madrigal</li> <li>BridgeBio, Sarepta, Vertex*</li> </ul>	<ul style="list-style-type: none"> <li>Pfizer</li> <li>Bristol Myers Squibb</li> </ul>	<ul style="list-style-type: none"> <li>AstraZeneca/Alexion</li> <li>Pfizer, Vertex*</li> </ul>

\*Indicates a tie

The full report provides additional, detailed qualitative insights into leading manufacturers' support as well as factors that drive best-in-class nominations across categories.

## Research Methodology and Report Availability

In August, HIRC surveyed 39 PBM/health plan-owned, health system-owned, and independent/other specialty pharmacy providers. Online surveys and follow-up telephone interviews were used to gather information. The *Rare Disease & Gene Therapy: Specialty Pharmacy Provider Distribution & Manufacturer Engagement* report is part of HIRC's Special Reports Series, and is now available to subscribers at [www.hirc.com](http://www.hirc.com).

HIRC's Special Reports Series is developed by a multidisciplinary team of HIRC researchers to provide comprehensive views into 2-3 high priority broad topic areas, such as contracting, regulatory issues, rare disease and more. Because insights draw upon content across HIRC's research portfolio, special report content often spans multiple market segments, product channels, and/or therapeutic areas. To subscribe to this new, unique offering, contact:



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