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Rare Disease Service

Specialty Pharmacy Providers: Rare Disease & Advanced Therapy Distribution, Contracting, and Manufacturer Engagement

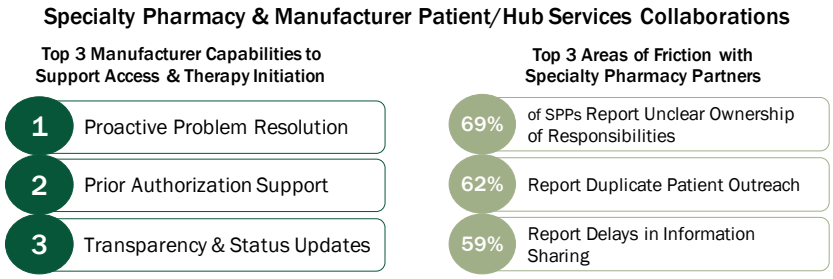
Specialty pharmacy providers (SPPs) are gaining access to a broader range of rare disease and advanced therapies, and their ongoing patient contact position them as strong partners in patient access and adherence. HIRC's report, *Specialty Pharmacy Providers: Rare Disease & Advanced Therapy Distribution, Contracting, and Manufacturer Engagement*, examines issues in distribution and manufacturer partnerships across 20+ therapeutic categories. The report addresses the following:

- What do specialty pharmacies report as their access to rare disease and cell & gene therapies (CGTs)? How does this vary by SPP ownership type? What are the trends in manufacturers' specialty pharmacy distribution networks?
- What are SPPs' unique capabilities in rare disease and how are they investing for the future? How does this differ by ownership type?
- How do specialty pharmacies evaluate manufacturer patient and hub services? What do effective specialty pharmacy-hub partnerships look like?
- Which contracting approaches are manufacturers engaging in to support the specialty pharmacy services for complex rare disease therapies?
- Which manufacturers are considered best-in-class in SPP rare disease engagement?

Key Finding: Specialty pharmacies value manufacturers that pair product access with operational, financial, and clinical support throughout treatment, including contracting arrangements that help fund the services complex rare disease therapies require.

Rare Disease Patient/Hub Services Collaborations

SPPs rate several manufacturer patient/hub services capabilities as critical to successful access and therapy initiation, with proactive problem resolution, prior authorization support, and transparency and status updates rated as the top most important program features. However, unclear ownership of responsibilities, duplicate patient outreach, and delays in information sharing are common reported sources of friction.

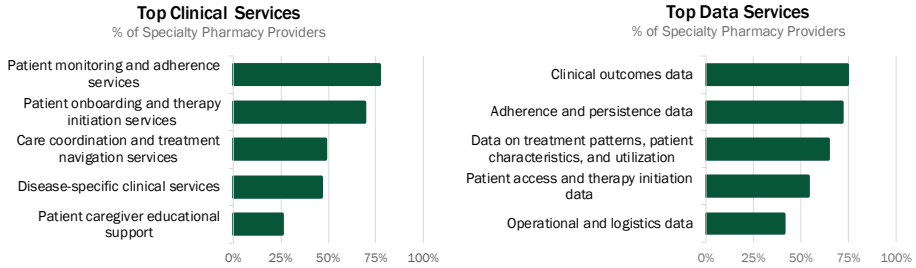


Specialty pharmacy perspectives on **division of responsibilities**, what **effective patient/hub services collaboration looks like**, and which pharmaceutical manufacturers are **nominated as best-in-class** for patient/hub services are examined in the full report.

Upfront Discounts and Enhanced Services are Central to Contracting for Rare Disease Therapies

Upfront discounts are reported as a common financial foundation of rare disease contracts, with data requirements and therapy-specific patient needs shaping enhanced services. SPPs report the strongest manufacturer interest in enhanced clinical contracts that support patients getting on and staying on therapy, while data contracts that report on clinical patient outcomes and adherence and persistency are most common.

Rare Disease Enhanced Service Contracts with Specialty Pharmacies



The full report explores the prevalence of four contracting types across 20+ rare disease and advanced therapy categories as follows: 1) Enhanced Clinical Service Contracts, 2) Enhanced Data Service Contracts, 3) Upfront/Off-Invoice Discounts, and 4) Performance-based Contracts, as well as specifics on enhanced services by therapeutic area.

Manufacturer Benchmarks Highlight Leaders in Rare Disease Specialty Pharmacy Partnerships

Specialty pharmacy executives were asked to consider and provide a best-in-class manufacturer nomination across several rare disease engagement parameters as noted below. Vertex, AstraZeneca/Alexion, and Biogen are consistently among those nominated.

Top Manufacturers in Rare Disease & Advanced Therapy Support, 2026

Ranked by Number of Nominations in Each Category

Overall Partner of Choice	Best Patient/Hub Services	Best New Product Launch	Best Clinical & Data Collaborations
▪ Vertex ▪ AZ/Alexion, Biogen, Sanofi* ▪ Genentech ▪ 14 Other Manufacturers	▪ AZ/Alexion, Biogen, BioMarin* ▪ Argenx, Sanofi* ▪ 10 Other Manufacturers	▪ Vertex ▪ 15 Other Manufacturers	▪ Vertex ▪ Pfizer ▪ 20 Other Manufacturers

*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 July and 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 *Specialty Pharmacy Providers: Rare Disease & Advanced Therapy Distribution, Contracting, and Manufacturer Engagement* report is part of HIRC's Rare Disease Service, and is now 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, contact:

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