



INSIGHTS FOR BIOPHARMA LEADERS
LAUNCHING RARE & ORPHAN THERAPIES

Selecting the Right **SPECIALTY PHARMACY PARTNER** for a Successful Launch



The expert teams at PANTHERx Rare have launched over three dozen rare disease therapies, partnering with both emerging and established biopharma companies. They've learned from experience that an essential element of a

successful launch is picking the right specialty pharmacy (SP) partner for your brand, and the right SP distribution model to ensure that your healthcare professionals (HCPs) and patients get the support they need, precisely when they need it.



It is a rare opportunity to serve those with rare conditions.

The Orphan Drug Act defines a rare disease as a condition that affects fewer than 200,000 people in the U.S., or 60 per 100,000 people. In the European Union, an orphan disease is defined as a disorder affecting less than one in 2,000 individuals, and the ultra-orphan designation is used for diseases with a prevalence of less than one in 50,000 people.

You only get one chance to launch.

Leading biopharmaceutical companies invest incredible amounts of money, time, technology, and human ingenuity researching, discovering, developing, and testing innovative and emerging treatments for a wide range of rare health conditions. So it makes sense that these same companies also invest appropriately to design the right commercial distribution strategy, including the right specialty pharmacy partner to successfully launch, deliver, and support their life-saving treatments for the people who need them most.

Choosing the right partners is essential to your brand's success.

Selecting the right SP for a rare treatment can positively impact getting appropriate patients onto commercial therapy, having patients experience the best outcomes from their therapy, and gaining insights into how to best position the therapy to navigate the competitive marketplace that surrounds it. This is an important decision, and can influence the gain or loss of millions of dollars post-launch, simply by choosing an SP partner that can also deliver actionable insights and adapt as additional real-world information is gathered and market dynamics evolve.

The PANTHERx experts are here to help.

We developed this report to provide biopharma decision makers with the information and tools they need to make the best decision for their treatment—and the patients and HCPs they serve. While each rare disease treatment has unique support needs and challenges, the framework below surfaces key concepts and questions that you and your team must consider as you make this very important decision that will affect your brand at launch and throughout its lifecycle.



Your brand—and the people who need it—deserve the right specialty pharmacy partner.

To create the right SP network, pharmaceutical manufacturers seek pharmacies that understand the needs of the patients they will serve who live with rare diseases. This understanding of and attention to the patient journey and their needs is often accomplished by establishing strong relationships with patients and HCPs through high-touch, data-informed, personalized services built around a specific rare therapy.

Manufacturers often prefer to partner with highly collaborative and agile SPs that can further tailor program-specific offerings to meet individual patient needs, design proactive programs to improve the experiences of patients and HCPs, and

provide insights to manufacturers to guide treatment uptake and evolution. Because rare therapy dispensing has become concentrated within exclusive or highly limited networks (e.g., networks with four or fewer SPs), SPs also play a central role in how patients and HCPs think and feel about the branded therapy and the pharma company that provides it. As a result, manufacturers search for SPs that have similar cultures and shared values as strategic partners for their therapy's launch and continued support.

When launching rare therapies, manufacturers also critically evaluate the number of specialty pharmacies they contract with. The past several years have seen an acceleration in the use of exclusive distribution models for orphan and ultra-orphan products. According to Dr. Richard Faris, Chief Strategy and Growth Officer at PANTHERx Rare, "Our team's experience of launching more than three dozen rare disease therapies during the past few years confirms that early, cross-functional planning in this area is critical. Selecting the right distribution model—and the right partner—for your brand is an essential element of your patient and HCP support strategy, launch excellence, and successful product lifecycle management."

To learn more about how PANTHERx Rare can support your brand and your patients at launch—and throughout the lifecycle—email us at TheRareSP@pantherxrare.com today.

Building the right support and distribution system takes time.

No matter which phase of the product lifecycle your rare or orphan therapy currently sits, finding an SP partner that understands the unique requirements of these types of therapies and the needs of the people who take them is critical to your brand's success. If you're planning for a new commercial launch, it's best to start building your SP distribution model and speaking to potential partners 18-24 months prior to launch.

This gives internal teams and external partners the time they need to plan, design, develop and refine standard operating procedures (SOPs) and training; a risk evaluation and mitigation strategy (REMS) if applicable; diagnostic and other testing if applicable; patient reimbursement and access strategies; personalized patient education and support programs; necessary technology integrations and secure data transfer among partners; and optimal inventory management and national distribution all finely tuned to support your product, the HCPs who prescribe it, and the patients who need it most.

Start with these considerations when selecting the rare and orphan pharmacy distribution model that's right for your brand.

Just as each patient has unique needs, so does each rare disease treatment. The information below can help you see which of the product's characteristics may have an impact on your options for specialty pharmacy distribution. First, we will review some general considerations, and next we'll see how the addition of a patient services HUB increases the complexity of this important decision.

Figure 1 (next page) includes some important considerations when you're evaluating the right specialty pharmacy distribution model for your treatment. For example, manufacturers can choose to partner with PANTHERx Rare either as their Exclusive SP, or as part of a highly Limited Network of a few SPs. However, each choice requires a careful assessment to ensure alignment with the unique needs of your product, prescribers, and patients.



Dr. Richard Faris
Chief Strategy and Growth Officer
PANTHERx Rare

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	EXCLUSIVE SP	LIMITED SP NETWORK
Treatment Complexity	High	High
Patient Population Size	Small to medium	Large to very large
Payer Contracting	Streamlined	SPs require greater discount off invoice due to lower payer reimbursement in limited network. Payers can choose preferred pharmacy based on network composition; SP partners may be 'squeezed' by payers and may lead to patient transfers.
Geographic Coverage	National	National
Communications	Communications between the manufacturer and one SP are streamlined & efficient, with a single point-of-contact.	Manufacturer must communicate with multiple partners, including other SPs, HUB, copay card vendor, etc.
Data & Reporting	Report direct to manufacturer or data warehouse without need for data aggregator.	Data aggregator required to manage feeds from multiple SPs.

Figure 1: Key factors when considering an SP such as PANTHERx Rare as either an Exclusive partner or as one of several partners in a highly Limited Distribution Network.

Take a deeper dive into the factors that can support your decision making.

Below are some key questions for each of the attributes in Figure 1 that may give you more insight into selecting the best SP partner for your rare disease therapy:



Treatment complexity

- What specific needs and accommodations do people with this high-complexity condition have?
- What specific needs and support do people taking this high-complexity treatment have?
- Which SPs that I'm considering have experience with similar conditions and therapies?
- Which SPs that I'm considering have accreditation in the care and management of rare and orphan diseases?



Patient population size

In rare and orphan conditions as defined in the U.S. and E.U., population sizes of “small, medium, or large” are relative—much smaller than those for traditional pharma blockbuster drugs. An exclusive SP partner can usually support up to 25,000 patients for a single condition.

- What specific needs does this patient population have?
- Which SPs that I'm considering have experience with similar patient populations—both size and needs?



Payer contracting

It's important to note that many payers immediately place a Limited Distribution Network into a different payment schedule than an Exclusive SP, often at break-even or worse reimbursement terms for the SPs.

- If I go with a Limited Distribution Network, will the service levels provided by the SPs in the network go down because of lower reimbursement?
- How will the selected SP overcome any anticipated barriers to access?
- Which SPs that I'm considering have experience contracting as an Exclusive SP? As part of a Limited Network?



Geographic coverage

- Is the SP's distribution reach regional or national? Does the SP have redundant facilities in different parts of the U.S.?
- What is the average speed of delivery to a patient?
- Does the SP have cold-chain capabilities?
- What is this potential SP partner's expertise and experience in logistics?



Communications

- What engagement options are there for patients and prescribers? Does the SP have capabilities to provide different options to meet patients where they "Work, Eat and Play"?
- What evidence does an SP have that it can simplify communications and coordination for the manufacturer? Can they share examples?
- What are some communications and coordination issues if I select a highly Limited Network of several SPs instead of a single, Exclusive one?



Data and reporting

- How well does the SP integrate its patient support program with its pharmacy dispensing and adjudication system? Can it provide sample documentation?
- In addition to program reports, can this SP provide insights for individual patient care, population health management, and evaluating product performance?

To learn how PANTHERx Rare can support your brand as its exclusive SP partner, email us at TheRareSP@pantherxrare.com today.



A HUB adds capabilities, complexity and cost to your brand's patient support and distribution system.

Due to the high complexity and cost of rare and orphan treatments, many manufacturers decide to offer HUB services to their patients. A HUB provides capabilities that enable a manufacturer to have a single point of contact with patients taking their therapies, delivers more comprehensive patient services, may improve adherence to therapy, and may increase patient and prescriber satisfaction.

However, the addition of HUB services also adds complexity and costs to your evaluation of and decision about whether to support your launch using an Exclusive SP or a Limited Distribution Network. And even if you choose to partner with an Exclusive SP, you'll need to decide if HUB services will be provided by the SP or by an external HUB vendor or internal manufacturer HUB.

As inputs for your decision making, Figure 2 compares additional attributes when incorporating a HUB.

HUB OPTIONS ►	PANTHERx as EXCLUSIVE SP		PANTHERx in LIMITED SP NETWORK
	INTEGRATED HUB SERVICES	INTERNAL MANUFACTURER HUB	INTERNAL MANUFACTURER OR EXTERNAL HUB
Patient & Prescriber Experience	Seamless & consistent All prescriptions from HCP directly to PANTHERx PANTHERx RxARECARE™ case managers handle referrals start to finish	Seamless & consistent All prescriptions from HCP directly to PANTHERx Coordination between internal HUB and PANTHERx required	Variable, with no consistent systems or level of service among pharmacies and HUB vendor Non-mandatory HUB model: Prescriptions from HCP to HUB, PANTHERx, other SPs, or sometimes multiple entities Referral triage business rules and services required
Manufacturer Control	Full visibility into patient journey from one reliable source, providing increased consistency and oversight	Manufacturer owns some patient services Visibility into patient journey from two reliable sources	Fragmented understanding of patient journey This option may be preferred for Manufacturers preparing for additional/expanded indications or wanting to reduce perceived risks of partnering with a single SP
Cost	Savings without compromising program quality	Need to hire internal resources for both program operations and oversight	Increased administration costs due to multiple fixed-cost vendors
Staffing Expertise & Continuity	HUB and SP services performed by same PANTHERx RxARECARE™ people: <ul style="list-style-type: none"> • Case managers 100% dedicated • Clinical pharmacists 90% designated 	Internal HUB & PANTHERx resources collaborate and coordinate services	PANTHERx RxARECARE™ team 90% designated for your product

Figure 2: Impact of adding a HUB when deciding on the most appropriate SP distribution model for your brand.

HUB considerations may affect your SP decision.

Let's take a look at some key questions when adding a HUB:



Patient and prescriber experience

Rare disease therapies can be complex for HCPs and perplexing for patients, so it's critical to align care using clear communications with the appropriate language for each audience and at the right frequency (not too much as to be confusing).

- How will I ensure a high level of consistency for the services associated with my brand, from patient to patient and interaction to interaction?
- How will prescriptions be routed? What are the risks if prescription routing varies depending on the prescriber?
- Who will be responsible for HUB coordination?
- Will the HUB connect patients with foundations, manufacturer copay assistance, and other financial support options?
- Has the HUB earned industry recognition for its record of patient satisfaction?



Manufacturer control

- As a manufacturer, what tradeoffs will I need to make if my company partners with one SP vs. several? How do I mitigate the risks of "putting all of my eggs in one basket"?
- If more than one entity "owns" and manages the patient journey, how can I ensure an accurate representation of it? Will I be responsible to consolidate data from different partners into a cohesive understanding of the patient?
- Am I confident that my company's HUB capabilities are on par with those of an experienced SP partner?



Additional HUB considerations that may affect your SP decision.

Below are important questions about costs and staffing when adding a HUB:



Cost

It's important to note that HUBs often charge for "add-on" services that are core services at SPs, another reason to carefully consider the tradeoffs and costs of adding complexity to your product's distribution and support solution.

- How much will my administration costs increase if I take on the management of several SPs instead of just one?
- Will I save money by using my company's HUB capabilities instead of using the HUB capabilities from an experienced Exclusive SP?



Staffing expertise and continuity

- Will the SPs and HUBs I'm considering guarantee a specific level of staff allocation and continuity for my brand?
- How important is staff continuity in patient satisfaction?
- How does staff continuity impact patient adherence?
- How much relevant disease-state expertise does the SP have?
- How much relevant treatment expertise does the SP have in this category?

To find out more about how PANTHERx Rare can help you decide on the SP model that best suits your needs, email us at TheRareSP@pantherxrare.com today.



What are YOUR most important decision criteria when selecting a specialty pharmacy distribution model?

As you know, specialty products—and particularly rare and orphan therapies—have complexity embedded in their DNA. This complexity extends to access, dispensing, delivery, education and support for the people living with rare and devastating conditions who take your therapy.

Setting up the right SP distribution model well before launch is critical for the success of your product, ensuring comprehensive and personalized support services, deep therapeutic and rare condition expertise, and optimal patient access aligned with your strategy. Your SP partner(s) will likely be with you for the long run, so it's critical that your due diligence enables you to objectively assess their strengths and shortcomings in the areas that are most important to you and your brand.

Your launch success requires the right SP partner—and all are not equal.

For manufacturers of rare disease treatments, thoroughly evaluating your SP options is a critical part of launch planning, and selecting the right one is a critical part of launch (and lifecycle) success. The size and characteristics of the patient population, alignment with other products in your portfolio, your internal capabilities, and other factors will affect your decision.

Here are a few more questions to help sharpen your focus:

- For my company, how should I structure the due diligence and decision processes to determine the best SP distribution model for my brand?
- Who are the key stakeholders within my organization to participate in the SP evaluation and decision?
- How will this SP decision support my strategic goals and key business drivers related to differentiation, market share, industry reputation, patient access, and adherence?
- Will we seek additional indications for this treatment over the next few years?
- Does my company plan to launch other therapies in this category in the coming years?
- How do I communicate the SP distribution network to HCPs at launch?
- Are there industry consultants that can help me make this decision?

Understanding trends, complexity, and cost can help guide your decision.

In recent years, many manufacturers of rare disease therapies have pivoted from large SP networks to smaller and in many cases, Exclusive SP models. This shift is due to the quality and consistency of services in addition to cost savings recognized at launch and throughout the

product's lifecycle. The expertise of smaller, highly focused, and nimble SP providers—rare-only pharmacies, capable of delivering tailored solutions for rare populations—is now the gold standard in the delivery of novel agents for rare diseases.

LEVERAGE THE POWER OF ONE:



Financial advantages of having only one SP partner include singular contracts, integrated HUB service offerings, efficient use of launch resources, and supply chain savings.

Programmatic benefits include consistent patient and provider experiences, comprehensive data capture, and program design flexibility, among others.

The Power of One: Selecting a single rare SP partner

Manufacturers recognize several advantages of working with a proven, rare-disease focused, SP partner on an exclusive basis including both financial and programmatic benefits. Financial advantages include singular contracts, integrated HUB service offerings, efficient use of launch resources, and supply chain savings. Programmatic benefits include consistent patient and provider experiences, comprehensive data capture, and program design flexibility, among others.

Clinical-stage manufacturers have especially embraced exclusive SP distribution partners as they have greater ease designing and adapting

programs specific to their therapy launch without regard to legacy networks used in prior drug launches. By limiting the network, manufacturers may collaborate more closely with an SP to develop targeted clinical services tailored to provide care to unique patient populations and individualized product profiles.

Limiting the network also enables manufacturers to have fewer concerns about existing channel strategy impacting already marketed products. Additionally, the financial benefits and overall simplicity of limiting the network are attractive for lean, cost-conscious manufacturers.



In 2024 PANTHERx Rare was **recognized for the 7th time** as the winner of the annual Independent Specialty Pharmacy Patient Choice Award™.

PANTHERx Rare is THE rare disease pharmacy expert, from launch through lifecycle management.

Since PANTHERx Rare was founded in 2011, it has been one of THE leading specialty pharmacies for rare and orphan diseases. We proudly distribute rare and orphan therapies nationally and focus on providing access and support services to the people who need them most. And we're recognized as the expert in patient experience and care, earning seven Patient Choice Awards over the past several years.

Known as THE Rare Disease Pharmacy®, PANTHERx transforms lives by delivering therapeutic breakthroughs, clinical excellence, personalized support, and a range of access solutions to people living with rare and devastating conditions. Our experts have helped both emerging and established manufacturers launch and support more than three dozen rare disease treatments and the patients who benefit from them.

In its April 24, 2024 post Drug Channels Institute examined how manufacturers' specialty networks contribute to the substantial concentration of dispensing revenues for specialty drugs. It found that "one in three specialty drugs (33%) have exclusive dispensing networks with only a single specialty pharmacy," and "building the capabilities and strategies to service an exclusive network has allowed some smaller specialty pharmacies to retain network position and grow more quickly than the overall market." As evidence of our strong and continuing growth, the researchers found that PANTHERx Rare Pharmacy handles 11% of exclusive-network drugs, the most of any non-oncology SP.

Our RxARECARE™ approach redefines patient centricity with a product-specific, highly personalized care program for each patient.

Our RxARECARE™ teams at PANTHERx offer fully coordinated clinical services that align with our partners and accreditation requirements, patient preferences, and the unique profiles specific to complex disease states.

Specialty Workflow Technology, or SWFT, is our custom-built clinical platform that integrates with PANTHERx Rare's dispensing system and manufacturers' HUB client resource management systems

to enable dynamic patient case management. SWFT ensures precision, accuracy, and patient safety, and utilizes innovative technology to provide actionable insights including:

- Population health management
- Product performance evaluation
- Adverse event and product discontinuation oversight

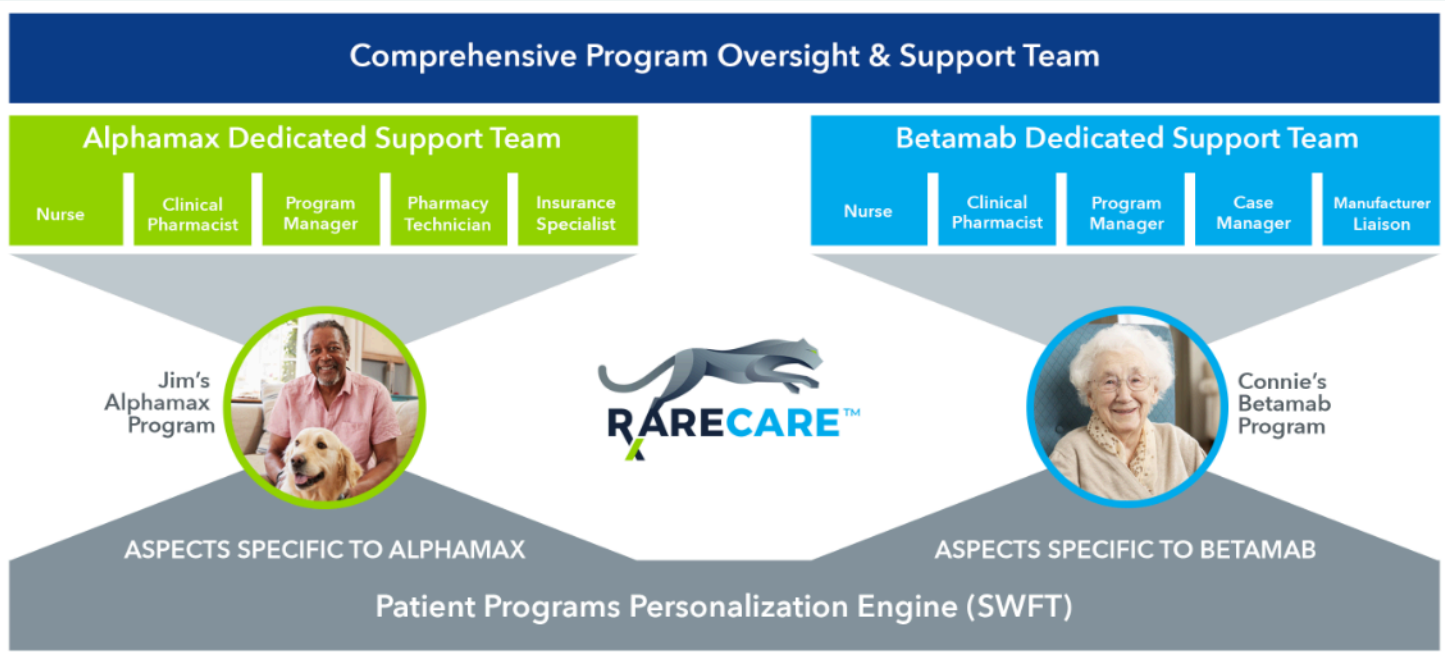


Figure 3: PANTHERx Rare combines rare-disease clinical expertise and experience with innovative technology to personalize each patient's care, consistently improving adherence and outcomes.

To learn more about how PANTHERx Rare can help support your brand and your patients, email us at TheRareSP@pantherxrare.com today.



Ready to take the next step?

We hope you found this report valuable and understand that you may still have questions about planning, designing, contracting, and operationalizing the right SP distribution solution that addresses the unique requirements of your product and best supports your patients and those who care for them.

Whether you are actively seeking an SP to help launch a new rare or orphan treatment, or are managing an inline product and are not completely satisfied with your current SP arrangement, please email us today at TheRareSP@pantherxrare.com to schedule a conversation about how PANTHERx Rare may be the right SP partner for you.



Valued by patients.
Respected by providers.
Trusted by partners.



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DISTINCTION IN ORPHAN DRUGS

