



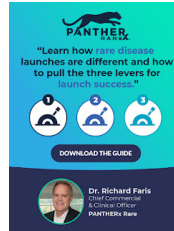
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## Targeted Reach, Tighter Relationships: Why Rare Disease Launches Are Different

Today's guest post is from Dr. Richard Faris, Chief Commercial and Clinical Officer at PANTHERx Rare.

Richard examines key considerations in launching rare disease therapies. He argues that targeted reach, tighter relationships, and data-driven collaboration can improve success for both patients and manufacturers.

To learn more, download PANTHERx Rare's free guide: [From PDUFA to Patient: A Guide to Rare Disease Launch Success.](#)



Read on for Richard's insights.

**Targeted Reach, Tighter Relationships: Why Rare Disease Launches Are Different**  
By Dr. Richard Faris, Chief Commercial and Clinical Officer, PANTHERx Rare.

The launch of a new rare disease therapy can be the bridge between hope and reality for patients. Many manufacturers have launch experience, but their familiar playbooks may not account for the unique complexities of rare diseases. The "rare journey" from development to patient access needs a fundamentally different approach, requiring precision and close collaboration among the manufacturer, pharmacy, prescribers, patients, and caregivers. This post focuses on three "launch levers" that can make or break high-stakes market entries in rare diseases.



### Launch Lever 1: Build Diverse Relationships Across the Ecosystem

Rare launches demand a nuanced understanding of the unique ecosystem surrounding the condition and its current treatment options:

- Many rare therapies are first-in-class with limited prescriber experience treating a specific condition and/or navigating access to a specific treatment.
- The patient journey often meanders through years of misdiagnoses and ineffective therapies.
- Rare diseases are often genetic, requiring pediatric caregivers.
- Patients and caregivers frequently feel isolated, turning to advocacy groups for education, support, and navigating access challenges.

Demonstrating what makes a therapy different amidst these challenges demands a forward-thinking approach to building relationships with external partners 6-12 months before PDUFA. Early engagement with the rare pharmacy and hub is vital to align objectives, develop a personalized care model that puts patients' needs at the center, identify potential barriers, and construct robust processes that adapt to complex health conditions, treatments, and access issues.

### Launch Lever 2: Align Data and Tech Infrastructure with Stakeholder Needs

Supporting small patient populations requires nimbleness as new real-world insights emerge. A clear data strategy connecting disparate sources and providing insights into market performance is key to a successful launch. To get there, the manufacturer, pharmacy, and other partners must synchronize data flows to ensure operational excellence. This includes identifying and preparing core data files to enable seamless coordination throughout the patient journey, including inventory levels, dispense records, and detailed patient status/sub-status information.

Experienced rare pharmacies with strong patient engagement programs deliver additional data, including patient-reported outcomes intentionally gathered during routine touchpoints. These endpoints can deepen the understanding of prescriber patterns and patient issues while informing payer and prescriber engagement strategies.

Prior to launch, manufacturers and their partners should align cross-partner data team cadence, definitions, and governance, and may integrate with the manufacturer's data aggregator. Data mapping and testing are critical to ensure data accuracy and compliance with manufacturer requirements, often mandating dedicated decision-making processes for integrating an internal hub, external hub, and/or patient services team.

When data flows seamlessly, manufacturers gain real-time insights, partners act decisively,

and patients receive care without unnecessary barriers.

### Launch Level 3: Engage Payers with a Data-Driven Value Story

While the access landscape in rare differs dramatically as well, linking data to clinical benefits still matters. Developing a payer launch strategy begins with articulating a clear value proposition supported by evidence of a therapy's clinical and economic value. Manufacturers should also have a plan to collect real-world evidence that demonstrates ongoing value at the individual and population levels.

Anticipating payer needs and informing prescribers about the right data to provide can reduce prior authorization (PA) approval times and improve patient experience. Prior to launch, the pharmacy's payer and clinical teams analyze pivotal trials, identifying inclusion criteria, genetic or lab requirements, and outcomes most likely to satisfy payer coverage criteria. Pre-building "evidence packets" with these data in mind can streamline PA and appeal processes significantly.

Engaging payers early also helps avoid delays in drug formulary inclusion. Parallel tracks with the market access teams at manufacturers and their pharmacy partners are essential for effective payer engagement. The pharmacy's payer relations team can simultaneously engage the clinical and pharmacy sides of pharmacy benefit managers to convey real clinical value from their frontline perspective.

Getting this right means fewer denials, patients receiving their therapies faster, and a stronger foundation for long-term, positive payer relationships.

### Launch Readiness Starts with the Patient

Successful rare disease launches require manufacturers to think beyond traditional pharmaceutical launch paradigms. Recognizing the unique characteristics of rare disease markets, treatments, and patient journeys; investing in strategic partnerships; and building comprehensive support infrastructure before launch enables manufacturers to significantly improve the likelihood that their innovations will reach those who need it most.

Selecting a rare pharmacy with deep launch expertise means hiring a trusted advisor to co-lead the process and align to best practices that increase the chances of success.

PANTHERx<sup>®</sup> Rare has launched nearly 50 rare disease therapies, offering practical guidance to navigate today's complex landscape. Our experts serve as trusted advocates to ensure seamless collaboration and exceptional care. Together, we take a customized and hyper-personalized approach that addresses a wide range of patient needs, from treatment management to accessing life-changing therapies.

To learn more about improving launch readiness for rare disease therapies, download our guide, [From PDUFA to Patient: A Guide to Rare Disease Launch Success](#).



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