



# From PDUFA to Patient: A Guide to Rare Disease Launch Success

## Why rare launches are different

Rare disease treatment launches are unlike traditional pharmaceutical rollouts. With small patient populations, complex care journeys, and high-stakes payer dynamics, success requires precision, collaboration, and a patient-first mindset.

This guide outlines the three launch levers that strongly influence success in rare disease markets, and how manufacturers can activate them to accelerate access and optimize patient outcomes.



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### Build Diverse Relationships Across the Ecosystem

- Rare therapies are often **first-in-class**, with limited prescriber experience.
- Patients face **years of misdiagnoses** and fragmented care.
- Pediatric caregivers and **advocacy groups** play a central role in support and education.

**Action:** Begin engaging rare pharmacies, hub, and advocacy partners **6-12 months before PDUFA**. Align incentives, design personalized care models, and anticipate barriers to ensure smooth patient access.



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### Align Data and Tech Infrastructure with Stakeholder Needs

- Small populations demand **nimble, real-time insights**.
- **Synchronize data flows** across manufacturers, pharmacies, and hubs.
- Leverage **patient-reported outcomes** to deepen understanding of prescriber patterns and patient challenges.

**Action:** Establish governance, cadence, and integration with data aggregators before launch. Accurate mapping and testing ensure seamless coordination, compliance, and operational excellence.



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### Engage Payers with a Data-Driven Value Story

- Payers require clear evidence of **clinical and economic value**.
- Pre-build **prior authorization** (PA) “evidence packets” to reduce delays.
- Collect **real-world evidence** to demonstrate ongoing value.

**Action:** Engage payers early through parallel tracks with market access teams and pharmacy partners. Streamlined PA processes and proactive formulary engagement mean faster approvals and fewer denials.

Ready to improve your rare disease launch readiness? Contact us at [TheRarePharmacy@pantherxrare.com](mailto:TheRarePharmacy@pantherxrare.com)