



A Best-in-Class Approach from PANTHERx Rare

Harnessing Talent and Technology to Improve Service to Patients Living with Rare Disease

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The Orphan Drug Act of 1983 defines a rare disease as one that affects fewer than 200,000 individuals in the United States.¹ While each condition affects few people, together all rare diseases impact an estimated 25 to 30 million Americans.¹ Patients with rare diseases experience unique challenges, such as coping with the rarity of their disease, finding a provider who can best manage their condition, and navigating access to high-cost therapies.² Considering these challenges, patients with rare diseases turn not only to their providers, but also to the pharmaceutical industry, health plans, and rare disease pharmacies throughout their journey for education, assistance, and support.

Patients Living with Rare Diseases Look for Assistance from Key Industry Stakeholders:

- Pharmaceutical Industry – to develop treatments for their rare diseases
- Providers – to form an accurate diagnosis and prescribe the most appropriate treatment
- Payors – to provide access to rare disease therapies at a reasonable out-of-pocket cost
- Pharmacies – to dispense novel treatments that have life-changing potential and provide care throughout their entire treatment journey

Each rare disease industry stakeholder is a critical component in a patient's journey, and it is the responsibility of all parties involved to provide hope for the patients they serve.

Patients with rare diseases often feel alone throughout their journey, since another patient with their same condition may not even reside in the same state. They may spend years seeing multiple providers and practices until they are evaluated by someone who is finally able to determine the correct diagnosis. These are generally physicians who work in academic medical centers or centers of excellence across the United States. Although receiving a diagnosis is a significant milestone for patients with rare diseases, navigating the road ahead can be difficult due to limited treatment options and clinical practice guidelines. Professional opinions from key opinion leaders (KOLs), therefore, play a central role in the management of each rare and potentially devastating condition.

The journey to diagnosis and treatment can be a long and grueling process for patients with rare diseases. Support from other key industry stakeholders who can assist patients with the challenges associated with their healthcare journey is critical to achieving improved health outcomes. Rare Pharmacy, a new and unique pharmacy domain pioneered by PANTHERx Rare, was created to address unmet needs for patients with rare diseases.

Utilizing specialized, highly skilled teams and innovative technology, a new industry standard has emerged in order to provide the highest level of care to patients.

Supporting Rare Disease Patients with Highly Skilled Talent

The understanding that patients with rare diseases experience unique challenges others may never face has created a need for a differentiated and customized care model that individualizes each patient's journey. As the specialty pharmacy industry approaches over four decades of utilization, rare pharmacy has emerged with the intent to provide dedicated support to some of the smallest patient populations that may only have one treatment option.² Recognizing the level of support patients with rare diseases may need from diagnosis through initiation of treatment and beyond, highlights a need for highly skilled individuals focused on one rare disease and treatment to optimize patient outcomes. As a result, PANTHERx has created RxARECARE™ to provide a deeper focus on specialization, expert training, and intelligent design. Coupling RxARECARE with the use of agile technology drives positive outcomes for patients and all stakeholders involved.

RxARECARE was designed with the individual patient as the focus. Each patient is supported by team members who have undergone extensive training in a specific rare disease and treatment. All patient-facing staff, from reimbursement specialists to clinicians, undergo empathy, sensitivity, and motivational interviewing training to provide dedicated support in real-time. Although each staff member serves a unique role in their skill area, navigating patients through treatment barriers, each staff member is cross-functionally trained to resolve any immediate needs or triage to the appropriate clinician when necessary. It is critical that staff understand all dimensions of the patient journey, inclusive of patient advocacy, in order to offer guided support.

By embedding compassionate, highly trained associates into RxARECARE teams, PANTHERx is better able to develop patient/caregiver trust. Patients with rare diseases benefit by no longer feeling alone, knowing they have a dedicated care team to support them that understands their unique challenges. With trust established, PANTHERx RxARECARE teams can further enhance patient motivation and engage patient participation in their own personalized treatment plan, driving positive patient outcomes.

Supporting PANTHERx RxARECARE Teams with Innovative Technology

Once positive rapport is established between a patient and their RxARECARE team, our expert staff utilizes the PANTHERx proprietary patient engagement platform, Specialty Workflow Technology (SWFT™). SWFT is used to further enhance the level of care and the overall experience of patients with rare diseases. SWFT is a fully customizable solution, driving key processes that enable skilled staff members to spend more time focusing on patient/stakeholder engagement rather than operational processes and tasks. Additionally, SWFT allows us to perform intelligent patient outreach; connecting with patients at their preferred time and capturing insightful patient-reported information. Our system enables us to aggregate data to further understand the patient experience and guide clinical practice, a benefit in the rare disease landscape as evidence-based medicine is often not available for niche rare patient populations.

PANTHERx also benefits from employing highly experienced clinical advisors, often pharmacists holding Board Certified Pharmacotherapy Specialist (BCPS) credentials or Board-Certified Oncology Pharmacist (BCOP) credentials, who are able to pair robust data analyses with their clinical decision-making to evolve each rare program. They determine appropriate targeted interventions to incorporate into the program design, that may target either the entire population or individual patients requiring intervention. Utilizing SWFT for ongoing program customization and provision of extractable data, in discrete variable format, enables customized, program-specific, clinical dashboards to be created and continuously analyzed. The patient experience is evaluated for both the aggregate population and the individual patient, providing insights that lead to the creation of meaningful, targeted interventions and a greater level of care.

PANTHERx remains nimble and flexible by continuously adapting our clinical programs and facilitating changes within our SWFT platform as more information becomes available about the patient population and their progress on therapy. For patients with rare diseases, the right intervention made at the right time is critical in a patient's journey. Proper timing of program changes paired with an adaptable and customizable clinical system affords PANTHERx the opportunity to make a large impact, even on the smallest patient populations.

While the rare pharmacy industry continues to emerge and evolve, PANTHERx places a substantial investment in talented teams and our proprietary SWFT system. This commitment to our patients drives patient engagement in a more dynamic manner, providing a valuable framework to successfully deliver care to patients afflicted with rare and devastating conditions. Over the next decade, with an expected surge in the number of pharmaceutical products approved by the FDA to treat rare diseases, thought leaders and experts in rare disease will be challenged to further develop innovative solutions and novel care models to serve those living with rare and devastating conditions.

References:

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2. Rare Pharmacy Emerges from Specialty (Pharmacy). PANTHERx Rare. Web. 5 May 2021. White Paper "Rare Pharmacy Emerges from Specialty (Pharmacy)" Published - PANTHERx Rare



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