RESEARCH SUMMARY

The State of Rare Disease Care:

Stakeholders identify clear path to faster, more personalized care

Background

PANTHERx® Rare, the largest independent specialty pharmacy (SP) focusing solely on rare and orphan therapies, released new research with Morning Consult in February 2026 that illuminates the rare disease care experiences of patients, caregivers, healthcare professionals (HCPs), and payers.

The goal of the research was to move beyond anecdotes and quantitatively document the realities of the rare disease journey, providing evidence to inform better models of communication, coordination, and support. Nearly three-quarters of patients and caregivers (72%) in the survey reported having SP programs involved in their care, yet most operate with significant limitations that leave patients wanting more.

What follows are the key findings, insights, themes, and recommendations gained from analyzing the survey data.

Many patients reported feeling responsible for self-advocacy and coordinating their own care

Patients are often forced to drive their own care. These statistics reveal system-wide challenges where patients must become their own case managers during this phase.

Nearly three-quarters of patients and caregivers (73%) and healthcare professionals (73%) agree that it's not always clear who is responsible for coordinating care for rare disease patients, compared to 28% of payers.

Key findings:

- **90%** of patients and caregivers feel personally responsible for pushing to receive tailored care.
- **54%** say they're most often the ones driving what happens next in their care journey.
- **74%** felt overwhelmed during diagnosis—yet more than **90%** faced barriers at this critical phase.

"It's not always clear who is responsible for coordinating care...."

Across patients, HCPs, and payers, the #1 agreed-upon solution to improve patient care is a single point of coordination.

Rare disease patients are often stuck in communication limbo

According to the survey results, a staggering 90% of rare disease patients and caregivers have experienced delays in care due to communication-related issues, turning urgent medical care into a frustrating waiting game.

According to the survey, the most commonly missing pieces of information in communications are the status of insurance or coverage decisions and who is responsible for moving the process forward. The information gaps hit patients hardest during their most stressful and vulnerable moments, especially during the diagnosis phase.

Key findings:
The three biggest delay culprits reveal systemic coordination failures:

- **68%** of patients and caregivers wait for updates or answers from doctors, pharmacies, or insurance.
- **61%** waste time repeating the same information to multiple people.
- **60%** face delays in hearing back about insurance coverage or approvals.

> **"PANTHERx continuously strives to better understand the patient journey. Insights from stakeholders can help improve the care that patients receive."**

Dr. Richard Faris
Chief Commercial & Clinical Officer
PANTHERx Rare

Specialty pharmacy's transformation opportunity: personalization

When describing their ideal SP, 49% want a primary partner who knows their situation, can answer questions, and manages care logistics across doctors, insurance, and pharmacy.

Two of the most valued SP functions emphasize ongoing relationships: 72% of patients and caregivers would find dedicated coordinators who stay involved over time very helpful, and an equal share (72%) would find seamless coordination without patient intervention very helpful.

Key findings:

- Currently, only **32%** of patients have dedicated coordinators.
- Only **26%** receive regular check-ins.
- **62%** have had to follow up themselves to keep processes moving forward.
- **47%** say support feels generic rather than personalized to their situation.

About the research

"The State of Rare Disease Care" survey was fielded January 27-February 11, 2026, among 226 rare disease stakeholders, including 126 patients and caregivers, 75 specialist healthcare professionals, and 25 payer decision makers who make or influence coverage decisions for rare and high-cost therapies. The research was conducted by Morning Consult in partnership with PANTHERx Rare to increase awareness of patient challenges for Rare Disease Day in 2026.

PANTHERx pioneered hyper-personalized clinical, access, and support services for people living with rare conditions, and our dedicated RxARECARE® teams know each patient by name.

About PANTHERx Rare

PANTHERx Rare makes rare disease care more hyper-personalized and less overwhelming by focusing relentlessly on each patient and each therapy. PANTHERx experts develop deep personal relationships with patients, prescribers, and pharmaceutical partners, serving as trusted advocates to ensure seamless collaboration and exceptional care. Since its founding in a garage in Pittsburgh, PA in 2011, PANTHERx has grown into the largest independent rare pharmacy in the U.S., leveraging established-company resources while maintaining small-company responsiveness, innovation, and attention to detail.

PANTHERx is licensed in all 50 states and was the first national pharmacy to achieve dual accreditations in rare disease from the Accreditation Commission for Health Care (ACHC) and Utilization Review Accreditation Commission (URAC). PANTHERx is also the eight-time winner of the prestigious MMIT Patient Choice Award for patient satisfaction, including the 2025 honor.

To learn more about how we can help you and your team improve the rare disease experience for patients and HCPs, email TheRareSP@pantherxrare.com.

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