Patients Are Waiting: Speeding Time to Treatment in Rare Disease

Companies are overcoming challenges to develop, launch, and educate on new rare disease medicines faster.

Rare diseases are no longer rare. Every year, people's lives are upended by a diagnosis of one of 6,000–8,000 identified rare conditions, which collectively affect one in 17 people. Because a majority are genetic and appear early, more than half of these patients are children, and many are not expected to reach their fifth birthday.

For patients left waiting, the stakes for new medicines are high. Yet, less than five percent of rare diseases have at least one approved treatment. Even when effective therapies and medicines exist, reaching the right patients in need is challenging. Survey data depicts a long and emotionally gruelling journey for those awaiting a diagnosis. For adults, it can take up to five years, and half will receive a misdiagnosis.[1]

Rare diseases challenge traditional ways of doing business. They require the industry to better identify target patient populations for trials and then keep them engaged during the course of the study, even across geographies. Once a medicine is approved, it is crucial to seamlessly transition from trials to treatment given the smaller patient cohort, as the physicians who conduct the trials often become prescribers.

The good news is that the industry is making great progress in how medicines for rare diseases are developed and brought to market. From delivering better site support during clinical studies to greater connectivity between medical and commercial teams, companies are breaking down historic silos and cutting the time to treatment. Along with richer healthcare data and effective medical education for physicians on symptoms and treatment options, these advances will help those undergoing complex patient journeys.

Greater Focus on Patient Experience During Studies

Over half of orphan drug trials are eventually discontinued or fail to publish results after completion.[2] Often, studies can't recruit or lead to inconclusive results. When a trial gets off the ground, sites have to invest significant time in keeping a small number of participants engaged.

Removing excessive system and process complexity will improve their efforts. For years, sites have voiced their concerns about the multiple disconnected tools they must navigate just to keep a study going. As one site leader explains, navigating unintuitive technology absorbs time from trial activities, and can make them feel like they're asking too much from patients: "The technology shouldn't be the trial itself, it should support the trial. It's taking time away from what we want to do, which is taking care of our patients." Simplifying the technology

experience and reducing the admin burden will better meet sites' and patients' needs.

The knock-on effects could include better participant retention during rare disease trials. This may widen patient access to life-enhancing new treatments. Reflecting on her own experiences as a rare disease patient, Helen Shaw, co-founder of the virtual site VCTC, observes: "I see how hard it is to take part in a clinical trial. But patients do want that opportunity to be offered something that they wouldn't get in their standard care, whether additional MRIs or new medicines."

Data Unlocks Effective Commercial Execution

Data is core to understanding where the greatest patient needs are ahead of a launch. ADVANZ PHARMA is a global biopharma company focused on specialty, hospital, and rare disease medicines. Andy Eeckhout, head of CRM and digital solutions for global commercial excellence, explains: "It's crucial to take a patient perspective. This means using real-time data to understand the patient journey and identify the two or three most influential key opinion leaders (KOLs) per country, so we can best communicate the product across markets."

An added complexity is that rare diseases often involve multiple specialties. Medical science liaison (MSL) teams have to get up to speed quickly on complex science before meetings with relevant experts – while also being responsible for other (more mainstream) disease areas. "Our customer-facing teams need to be agile communicators and effectively switch to a more patient-oriented, in-depth scientific discussion than with generics," Eeckhout explains. Access to scientific resources and activity data in one place leads to higher-quality conversations. "Pre-call planning is crucial for MSLs before and after launch. The more data they can find, including on past interactions, the better," Eeckhout adds.

Depending on the disease, there could be a long gap before a patient presents with potential symptoms, at which point HCPs will want timely access to experts and resources. Smoother handovers between field medical and sales ensure HCPs can find answers quickly and connect to MSLs if needed. ADVANZ, for example, has launched a pre-launch module in

its CRM so that market access, medical, and commercial teams can share information compliantly and effectively. Eeckhout notes: "Physicians need a direct line to the industry so they know who to contact when they have questions. Medical and commercial teams need to talk to each other and remain agile across customer conversations."

Highly personalised content for HCPs is also key when targeting diseases with small patient populations. To be effective, marketing teams need clarity on the most impactful content to recommend for field visits across markets. For example, Recordati Rare Disease uses data analytics on its global repository of promotional and medical engagement tools, so it can support content use across a portfolio of 17 rare disease medicines of focus (and a growing global footprint). As its head of marketing and customer engagement, Gordon Daniels, notes, "How we engage with HCPs is critical. We need to know what percentage of our content is being developed and relevant to support different HCPs, whose patients rely on them for their rare disease diagnosis and management."

Defy the Odds, Then Beat Them

Scientific breakthroughs that could bring hope to rare disease patients and their families come from organisations willing to embark on a long and risky trial-to-treatment pathway.

Companies are reducing the time to treatment through better support for sites, freeing them up to focus on patient recruitment and retention during trials. Once a drug is approved,

connected technology and deep data can facilitate collaboration between functions that have not historically worked together so they can identify, engage, and provide medical education to relevant HCPs and KOLs.

Every rare disease patient faces a daunting journey. As one biotech leader points out: "Their diagnosis is with them every day. Often, they can't pronounce the condition, and don't know anyone else who has it." Life sciences will do its part to help these patients defy the odds – and eventually beat them.

Chris Moore

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