Patient-Centricity in Rare Disease:
Accelerating the Path to Treatment

More than 300 million people worldwide live with a rare disease, representing up to 5.9% of the global population. With over 6,000 distinct conditions, 72% of which are genetic, the therapeutic landscape is uniquely complex. For these patients, the primary barrier to life-changing care is not simply a lack of medicine, but a fragmented healthcare system that often fails to connect them with the right specialists in time.

Rare disease patients are frequently isolated, navigating a disjointed path before receiving a correct diagnosis, joining a clinical trial, or accessing the right therapy. *'Diagnosed with a rare cancer at 28, I learned the hard way how disconnected healthcare is. I had to consult five different specialists just to understand my options and drive 800 kilometres a week to be on an early-phase clinical trial. It's a stark reminder of how difficult it remains for patients to access the expertise, information and care they need,'* explains Jan Geissler, founder and CEO of Patvocates.

Patients' access to treatment depends heavily on the industry's ability to find, educate and support healthcare professionals (HCPs) who care for them. Yet, functional silos often prevent critical clinical insights from reaching field teams, meaning relevant HCPs may not receive the timely, consistent information they need to confidently diagnose and treat patients.

Breaking down these silos is essential to helping patients receive the treatment they need faster. This starts by unifying clinical, medical and commercial teams on a single platform with a shared, trusted data foundation. An integrated ecosystem of connected data and software enables seamless collaboration across functions, more consistent engagement with key stakeholders and an accelerated path from insight to treatment to improve patient outcomes.

Identifying the Right Specialists with Precision Data

In rare disease, access to treatment starts long before launch. Patient populations are small and dispersed and the physicians who treat them often remain hidden from traditional mapping. Early visibility and scientific engagement with these HCPs can accelerate treatment adoption by up to 40%.

One leading rare disease biopharma is addressing this by using high-quality reference data to gain a complete view of the healthcare ecosystem and find key specialists. Comprehensive HCP profiles including affiliations and networks such as the NHS Boards in the UK or reimbursement statuses in Italy offer the company a deeper understanding of the healthcare system and access to hard-to-reach HCPs.

But knowing where the experts are in a complex ecosystem only solves part of the equation. Driving meaningful engagement requires understanding who those experts are and what they care about. HCPs treating rare diseases often have a deeply personal connection to the condition and expect an elevated scientific dialogue with biopharmas. By using deep data to surface insights into an HCP's specific interests, clinical trials, niche expertise and recent publications, commercial teams can provide high-value, personalised conversations to become true partners in patient care.

Increasingly, biopharma companies are rethinking how they capture and operationalise medical insights to move from observation to understanding. By applying AI to translate, tag and analyse fragmented field insights at scale, organisations can identify emerging patterns and build a clearer, shared view of medical need, turning insights into measurable medical impact for patients.

Delivering Scientific Evidence at the Speed of Patient Care

Scientific education is the primary force evolving clinical practice and shaping care delivery. Research shows that 94% of key opinion leaders (KOLs) value scientific exchange with biopharmas – especially in the rare disease space, where clinicians rely on the latest data to navigate the complexity of treating these conditions.

Recordati provides a powerful example on how medical information teams are transforming into strategic, tech-enabled partners supporting HCPs. Michelle Bridenbaker, head of global medical excellence and communications at Recordati and vice president of Medical Information Leaders in Europe (MILE) Association, explains how they use AI to upskill professionals and lower barriers to scientific evidence: *'A clinician reached out regarding a child in palliative care with a complex medical situation. By leveraging AI to synthesise internal product data and published evidence, we were able to deliver a response in just three hours, a task that previously took more than a day, allowing the physician to start treatment the same day.'*

By using a tightly governed framework where AI handles the searching while humans retain control over authorship, Recordati ensures full traceability for regulators while delivering scientific answers at the speed of patient care.

Removing Blind Spots for a Seamless Patient Pathway

While medical and field teams operate on the front lines with HCPs, capturing the voice of the patient and the reality of the clinic remains a significant hurdle. Too often, these insights are lost in transit or trapped in disconnected tools, creating blind spots that prevent biopharma from refining engagement according to actual needs. This fragmentation affects the patient by creating knowledge gaps that delay access to life-changing therapies.

Bringing all functions on a single CRM enables a connected flow of information, where medical insights can instantly inform a commercial strategy or a clinical trial design. Each interaction is built upon the last

one, ensuring coordinated engagement for better HCP and patient experiences.

When Italfarmaco launched a new rare disease division, it established a strong operational foundation to enter a complex, highly specialised market with speed and accuracy. By unifying its commercial and medical teams on a single platform, the company was able to balance global standardisation for efficiency with country-specific requirements to scale across Europe in record time. Simona Gay, European customer excellence lead for rare disease at Italfarmaco, explains: 'Implementing a CRM means transforming the great potential for knowledge that resides in local teams into structured information: it is the starting point for building value.' The goal, she adds, is to offer the team a 'level of coordination and access to information that is fast, secure and complete as possible.'

Today, the company uses this infrastructure to map centre readiness, orchestrate compliant engagement and enrich hospital profiles with real-time insights from the field. Simplifying these internal processes allows teams to focus on the external interactions that change lives, ensuring the patient no longer carries the burden of navigating a fragmented system alone. As Gay emphasises, 'the ultimate mission is to bring concrete results to patients and families by listening to their voices.'

Measuring Success through Patient Outcomes

Digital transformation is not the end goal of the life sciences industry patient, impact is. By connecting software and data and simplifying processes, biopharma leaders enable commercial teams to focus on the high-value human interactions that truly change lives.

Every professional in the industry must act as a patient advocate and interact with patient communities to inform their work. As Jan Geissler says: '*When you get up in the morning, think about how you can, in your own sphere of influence, deliver patient impact. There are strong patient communities out there that can help you truly understand what patients want. Don't just make assumptions, work with patient organisations to co-design healthcare solutions to deliver patients' pressing unmet needs.*'

With the right data and technology, the industry can bridge the gaps in a fragmented healthcare system and transform the patient pathway, accelerating access to treatments and healthcare services patients so urgently need.

Chris Moore

Chris Moore, President Europe at Veeva Systems joined Veeva in 2018 as President of Veeva Europe to enable the European team to establish Veeva as the cloud platform of choice for the Life Sciences industry for its R&D and Commercial activities. Chris holds a bachelor of science degree in information technology from the University of Salford.