TRINITY

ADVISORY BRIEF

Calibrating a Patient-centric Approach in Rare Disease: Four Pillars to Focus Resource Allocation

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54%

Orphan drug approvals represented 54% of FDA approvals in 2022.

12%

Through 2028, orphan drug sales are expected to grow at 12%.*

Rare diseases present **unique challenges** to companies looking to commercialize treatments.

Although there is **vast potential** in rare disease, each space is different and unique.

Companies must effectively address challenges around identifying and treating patients—as well as providing long-term support. It is critical that companies understand the nuances in each disease.

*Source: https://info.evaluate.com/rs/607-YGS-364/images/Orphan%20Drug%20Report.pdf

Keeping a patient-first mentality, advancing patients' needs and providing excellent support services are mission-critical for life sciences companies touching rare disease. However, lack of disease awareness and unclear referral patterns present challenges to achieving positive outcomes. In focusing on four key pillars, manufacturers can improve patient care and differentiate themselves as a partner to patients.

1. Shape the disease ecosystem and referral pathways to ensure patients are identified, diagnosed and entered into the treatment funnel.

2. Invest in patient identification strategies and tactics, such as claims analysis using artificial intelligence and machine learning (AIML), to find and target the right patients for clinical trials and future treatments.

3. Develop ways to interact with patients, showing a commitment to their disease by elevating the patient voice and empowering patient communities.

4. Offer tailored and customized patient support services (PSS) based on deep patient insights to smooth the patient experience from diagnosis through ongoing treatment and management.

1 Shaping the disease ecosystem

THE CHALLENGE

One of the biggest—and most unique—challenges in the rare disease ecosystem is the lack of disease awareness, education and information, particularly in the physician community. This results in an unclear referral structure. Without recognizing the symptoms of a rare disease or specific symptom patterns, HCPs may not be able to provide answers to presenting patients and may not be sure where to refer the patient for other expertise. The journey from a patient presenting with symptoms to a diagnosis may therefore be long and complex, making it especially frustrating for patients, as the diagnosis itself becomes a struggle. All of these factors leave patients feeling isolated and uncertain about next steps.

THE SOLUTION

Bridging the gap between the current patient journey and the ideal serves to strengthen the manufacturer's leadership within the disease area. It is essential for HCPs to recognize presentation patterns and get patients to the specialists best equipped to diagnose and treat them. Patients are essentially required to become advocates for themselves to access the HCPs they need for treatment. This situation requires a concerted effort from pharmaceutical companies to empower patients and their communities through patient advocacy groups (PAGs). Educational initiatives can increase medical literacy and disease awareness among patients and HCPs, promoting better access to care.

Life sciences companies should invest in strategies that promote disease awareness and education early in the diagnostic process, including:

- Conducting HCP-led disease awareness programs to provide critical information to patients and other HCPs about the signs and symptoms of rare diseases, diagnostic test tools and treatment options.
- Ensuring patients receive the appropriate care they need from specialized treaters by fostering local connections among treaters.
- Collaborating with or sponsoring advocacy groups to improve disease awareness and education of lesser-known diseases through development of patient content, resources and programming with the goal of providing content that enables patients to self-advocate based on information they find online.

2 Finding and targeting patients and physician networks

THE CHALLENGE

HCPs may not suspect a rare disease when evaluating the symptoms of a patient and may struggle to identify rare disease patients within their population. HCPs may not be familiar enough with a TA to accurately identify patients—and disseminating disease state education among different HCPs with various priorities can be a challenge. Additionally, manufacturers running rare disease trials may struggle to recruit patients for clinical studies. Identifying the right patients in the rare disease space is crucial to ensure a pipeline of patients for clinical trials and future treatments.

THE SOLUTION

There are several strategies and tactics that life sciences companies can employ for patient identification:

- Finding and educating HCPs about the disease is a critical first step (but it is often not enough on its own).
  - Engaging with treating and referring HCPs can help to increase awareness of treatment options.
- Exploring patient identification through targeted investment in genetic diagnostics such as testing sponsorship (making it free to the patient to decrease testing barriers).
- Utilizing advanced analytics and machine learning tools to help understand diagnosis and thus find patients.
  - If diagnostic codes do not exist, identification may rely on using HCPs as a proxy to find patients.
  - Claims data can be analyzed for patients of interest or patients on therapy to develop an algorithm that 'learns' from current patients to understand how they present and ultimately, to find new potential patients.

AIML-Based Patient Finding—Threading the Needle in the Haystack

Explore Patient Finding Thought Leadership

3 Elevating the patient voice

THE CHALLENGE

In rare disease, physicians may lack understanding of a patient's struggles. It is important that patients feel heard and understood by physicians and pharmaceutical companies to know that their issues are being addressed. When manufacturers amplify patient stories and struggles, patients can feel heard and understood, leading to a stronger sense of community and support. The process ensures that patient support programs (PSPs) are appropriately tailored to the needs of patients and marketing materials take the right tone, as well as potentially enabling faster trial recruitment through interaction with advocacy groups.

THE SOLUTION

Patients with rare diseases often search for support systems, trusted sources and connections among others with the same disease, especially when they feel a lack of support from their immediate circle. Pharmaceutical companies can facilitate this by providing resources, education and support to patients:

- Conducting patient/caregiver advisory boards to understand the patient perspective.
- Building public relations and corporate messaging campaigns that resonate with patients' emotional and clinical needs.
- Sharing success stories and publishing real-world evidence (RWE) to show empathy for patients and their burden of illness.
- Activating patients is equally important. By engaging with advocacy groups in the early stages of drug development (e.g., through sponsorship of patient forums), manufacturers can learn about communities that have formed around rare diseases.

4 Optimizing patient support services (PSS)

THE CHALLENGE

PSPs are essential in rare disease—patients may face significant hardships from the disease and its treatment, including financial challenges, complexities of administration and the high-level burden of facing a lifetime of chronic treatment. Patients are often dissuaded by the cost of medication, logistical hurdles or lack of education about their disease and treatment options. PSS supports the three pillars above, guiding the patient journey from pre-diagnosis through adherence and long-term outcomes—and the programs support more than just the financial needs of patients. Robust patient services should be prioritized through the rest of the organization to optimally inform strategies and decisions to ensure a seamless patient experience.

THE SOLUTION

Pharmaceutical companies should support the broader patient ecosystem by focusing on high-touch support and attention for rare disease patients, as well as facilitating connections between patients through patient advocacy groups:

- Listening and learning – understanding emotions in addition to the clinical experience of patients.
  - Re-visiting and periodically reassessing the on-therapy experience to identify gaps and leakage points for patients.
- Exploring novel roles that PS can play that align with patient needs in the specific disease area (incl. travel for gene therapy, etc.).
- Fostering community and peer support if there aren't existing groups.
- Encouraging patients to remain compliant through outreach and reminders, remote monitoring and medication management

By investing in the strategies and tactics outlined above, manufacturers in the rare disease space can:

- Help shape the rare disease ecosystem by ensuring patients and healthcare providers have the latest information.
- Ensure HCPs are using a data-based approach to find, appropriately diagnose and provide appropriate care for patients.
- Highlight an ongoing commitment to patients and elevate their profile as patient-centered organizations and leaders.

Authors

Jennifer Parr | Partner & Head of Patient Centricity

Jennifer leads Trinity's Patient Centricity Center of Excellence, helping clients weave the Voice of the Patient into all facets of the product lifecycle. Jennifer's group works closely with Trinity's Strategic Advisory, PMR and RWE teams to produce deep patient insights based in real-world data that are tactically actionable for a wide range of biopharma customers. Jennifer has been involved in patient and consumer insights work for over 18 years. Having joined Trinity in 2010, Jennifer helped develop the company's primary research capabilities into a core part of the business, specifically in the areas of qualitative research and Patient Journey.

Jennifer earned a BA in Psychology from Boston University.

Akshay Mehta | Principal, Strategic Advisory

Akshay has advised numerous leading and emerging companies on a wide range of commercialization issues (strategy and operations) such as strategic marketing, commercial go-to-market planning, asset valuations, corporate strategy, sales force effectiveness, commercial analytics and organization redesign. In the last couple of years, he has also taken a leadership role in helping clients in the pre-commercial and early commercial stages. His therapy areas of focus are cell and gene therapy, oncology and rare diseases.

Akshay holds an MBA from The Wharton School (Palmer Scholar Honors) & Bachelors in Engineering from University of Pune, India (First Class with Distinction).

Daniel Foster | Engagement Manager, Strategic Advisory

Over the course of his career, Daniel has focused on market research, opportunity assessment, commercial analytics, sales force strategy, demand studies and forecasting. He has extensive experience working in rare and orphan diseases, including gene therapy.

Daniel has a BS in Neuroscience and a PhD in Molecular Biology from Harvard University.

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