

Despite having identified over 7,000 rare diseases, less than 5% have an approved effective therapy. As biopharma works to address this gap, companies aim to elevate the support provided to patients and HCPs. For the majority of rare and orphan disease launches today, Clinical Nurse Educators are being leveraged in diverse ways to accelerate market success.

RARE DISEASE MARKET MOMENTUM

Market Opportunity

Unmet need

Effective therapies exist for **<5% of the 7000+** known rare diseases.¹

Transformational patient benefits

85% of the identified rare diseases are very serious or life-threatening.¹

Significant potential

While each disease may only impact up to 200,000 patients, over **30 million patients** in the US are affected by rare disease.¹

Favorable environment

Access to **R&D tax credits, grants and attractive market exclusivity terms** through government programs and other entities.²

Market Activity

Fast-growing market

\$144B in the US in 2019, expected to grow at 12.2% CAGR through 2026.³

Extensive R&D pipeline

US leads in development with **300+ drugs** in clinical trials.⁴

Many therapeutic areas of focus

Drugs being developed in **13+ TAs**, with cancer and infectious disease being the largest categories.⁵

Several new product launches

In 2019 the FDA approved **76 orphan indications**, which included 22 novel drugs with orphan designation.¹

Growing Need for Enhanced HCP and Patient Support



"The basic patient support services used in traditional launches just don't cut it in rare disease."⁶

"Physicians are also now being asked to understand, diagnose, and treat many more diseases, including diseases that they might have heard mentioned once at a lecture in medical school."⁶

CRITICAL SUCCESS FACTORS FOR ENHANCED HCP AND PATIENT SUPPORT IN RARE DISEASE



Need for holistic patient support. Patients need significant emotional support in addition to personalized clinical support to ensure successful start and stay on therapy.

84% of patients with a treatable rare disease report feeling anxiety and stress, and **89%** worry about how their health will change in the future.⁷



Need for HCP education and training. Limited patient interactions, combined with little knowledge and resources, lead to HCP challenges in diagnosis and treatment management.

The mean length of time from symptom onset to accurate diagnosis for a rare disease patient is **4.8 years**.⁸



Need for an in-person support model. Given the importance of building real human connections.

67% of people with rare disease feel they do not have sufficient support through the healthcare system.⁹



Need for real-time learnings. Many companies launching products in rare disease are doing it for the first time, and there are few analogs to learn from.

Big data is not always available, especially in the study of rare diseases. **Small samples sizes are inevitable**.¹⁰



THE GROWING ROLE OF CLINICAL NURSE EDUCATORS IN RARE DISEASE



Use of On-Demand Clinical Nurse Educator teams, to efficiently get locally based, clinical resources in front of HCPs for education and training.



Clinical Nurse Educator playing the role of the patient's primary point of contact, providing holistic support from the time of Rx onward.



Combination of high-tech with high-touch, using digital communications to fill the gaps between nurse touchpoints and sustain the relationship long term.



Identification of ways to capture patient insights and patient reported outcomes, to enhance programs, inform brand strategy and guide other key brand initiatives.



Robust use of skilled nursing services to conduct in-home product administration, diagnostic testing, first-dose monitoring programs and more.