Specialty Pharmacy Channel Strategy Evolves as Rare Disease Drug Launches Become More Prevalent

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Channel management for traditional specialty pharmaceuticals has historically been straightforward. Manufacturers considered a specialty pharmacy networks' size, scale, and perhaps most importantly, their established relationships with payors that may influence market access. Since the majority of Food and Drug Administration (FDA) approved products over the previous decade were being commercialized for larger addressable patient populations and not for rare diseases, this strategy was effective.

Today however, products holding orphan drug designations* continue to account for a growing percentage of the novel agents approved by the Center for Drug Evaluation and Research (CDER). In fact, products holding orphan drug designation accounted for 59%, 44%, and 58% of drug approvals in 2018, 2019, and 2020 respectively.[1] With a strong pipeline of agents holding orphan designation in late-stage clinical trials, that trend is expected to continue.

Manufacturers have pivoted their attention to the orphan drug market, in part due to the ratification of favorable legislation. Such legislation includes the Orphan Drug Act, the Rare Disease Voucher Program, and the Orphan Drug Modernization Plan of 2017 which aims to create a more efficient, scientifically advanced, predictable, and modern approach to the approval of safe and effective treatments for rare diseases.[2]

*Orphan drug designation is granted by FDA for products developed for rare diseases or conditions that affect less than 200,000 persons in the United States.

Rare disease therapies and the patient populations they treat differ from treatments indicated for larger, more prevalent conditions in that:

- The patient populations are small, geographically dispersed, and hard to identify
- Most rare diseases have a genetic origin, manifest early in life, and as a result, disproportionately affect pediatric patients
- Patients often experience a diagnostic odyssey, going years or sometimes decades without an accurate diagnosis
- The length of time to diagnosis drives patients with rare diseases and caregivers to become their own "experts" and condition advocates
- Few providers are familiar with rare diseases and diagnostic criteria is often lacking or unavailable
- A limited number of providers are equipped to accurately diagnose and treat rare conditions requiring patients to travel long distances for adequate care
- Payors lack familiarity with many rare diseases and the pipeline of products in clinical trials
- Very often there is a high unmet need when a new therapy is approved because there were previously limited or no FDA approved treatments
  - Only 5% of rare diseases have FDA approved treatment options
  - For approved treatments, broad patient access remains a challenge[3] due to multiple circumstances including cost, strict prior authorization criteria, and small subset of specialists who see and treat the condition

Considering each of these factors, the launch of a rare disease therapy can in many ways be positively impacted by an experienced, rare disease focused specialty pharmacy.

As manufacturers have gained experience launching rare disease therapies, their approach for pharmacy selection has evolved. More than ever, manufacturers appreciate the importance of meeting the unique needs of their patient populations and provider base. While historically a high degree of emphasis was placed on size, scale, and the payor networks accessed through specialty pharmacy partners, these criteria are no longer the only differentiators for pharmacy selection.

To select an effective pharmacy network, manufacturers seek pharmacies that understand the needs of the patients they will serve who are afflicted with rare diseases. This understanding of and attention to patient needs is often accomplished by establishing strong relationships with patients and providers through high-touch, white glove services, and providing customized services built around individualized rare therapies. Manufacturers also prefer to work with highly collaborative specialty pharmacies that can further tailor program-specific offerings to meet individual patient needs and who are devoted to proactive programs to improve the patient and provider experience. Because rare therapy dispensing has become firmly concentrated within highly limited or exclusive networks, specialty pharmacies also play a central role in overall brand perception. As a result, manufacturers search for specialty pharmacies that have similar cultures and shared values as strategic partners for their therapy's launch.

In addition to seeking specialty pharmacy partners with shared cultures and values, manufacturers critically evaluate the number of specialty pharmacies they contract with to launch rare therapies. Over the past several years, there has been an acceleration in the utilization of and interest in exclusive distribution models for orphan and ultra-orphan products. Manufacturers recognize several advantages of working with a proven, rare disease focused, specialty pharmacy partner on an exclusive basis including both financial and programmatic benefits. Financial advantages include singular contracts, integrated hub service offerings, efficient use of launch resources, and supply chain savings. Programmatic benefits include consistent patient and provider experiences, comprehensive data capture, and program design flexibility, among others.

Clinical stage manufacturers have especially embraced exclusive specialty pharmacy distribution networks as they have greater ease designing and adapting programs specific to their therapy launch without regard to legacy networks used in prior drug launches. By limiting the network, manufacturers may collaborate more closely with specialty pharmacies to develop targeted clinical services that are tailored to providing care to unique patient populations and individualized product profiles. Limiting the network also allows manufacturers to have fewer concerns about existing channel strategy impacting already marketed products. Additionally, the financial benefits and overall model simplicity of limiting the network are attractive for these manufacturers who are characteristically cost conscientious and lean organizations.

In recent years, manufacturers of rare disease therapies have pivoted from large specialty pharmacy networks to smaller and in many cases, exclusive specialty pharmacy models. This shift is due to the quality and consistency of services in addition to material cost savings recognized at launch and throughout the product's life cycle. The expertise of smaller, highly focused, and nimble specialty pharmacy providers, rare pharmacies, capable of delivering tailored solutions for rare populations, is now the gold standard in the delivery of novel agents for rare diseases.

References:

1. Moritz, D. How are Rare Diseases Different from Rare Oncology and Specialty Diseases and What are the Implications for Companies? February, 2020. Web 4 May, 2021. https://bluematterconsulting.com/rare-diseases-vs-specialty/
2. PANTHERx Rare White Paper: Rare Pharmacy Emerges from Specialty (Pharmacy). Web 4 May, 2021.
3. Silverman, B. Breakthroughs, Orphans Hit High Notes As US FDA's 2020 Novel Approvals Play A Familiar Tune. January, 2021. Web 4 May, 2021. https://pink.pharmaintelligence.informa.com/PS143624/Breakthroughs-Orphans-Hit-High-Notes-As-US-FDAs-2020-Novel-Approvals-Play-A-Familiar-Tune

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