Navigating competitive arenas in rare disease

Critical success factors for industry

Treatment choice is beneficial for people living with rare diseases (RDs) and competition can drive important economic advantages for payers. For drug manufacturers, however, competition in RDs poses a unique set of challenges. With an increasing number of RDs served by multiple approved therapies, we are seeing the emergence of competitive arenas and a lower return on investment for orphan drug incentives, impacting where and how industry must focus their efforts to meet payer evidence expectations and effectively position RD assets in an evolving environment.

16 Rare diseases with **>2 treatments** in **2015**

101 Rare diseases with **>2 treatments** in **2025**

7 Rare diseases with **>3 treatments** in **2015**

54 Rare diseases with **>3 treatments** in **2025**

Critical success factors and select considerations for manufacturers in competitive RDs

	New entrant	Incumbent
Evidence	Identify possible opportunities to generate evidence that differentiates vs. existing options, including evidence beyond efficacy	Focus on long-term evidence, gather/ publish available real-world evidence, and leverage existing stakeholder relationships
Positioning	Consider whether there may be room for multiple options (e.g., alternative mechanisms of action in the same line of therapy, pursuit of later line of therapy), particularly in larger rare diseases	Positioned as the established, trusted product in the rare disease space, relying on the aforementioned long-term and real-world evidence
Value communication	Prioritize stakeholders based on launch strategy (e.g., patients & providers if planning to drive uptake through treaters/patients vs. payers if planning to drive uptake through access)	Center conversations on patients that are well-managed and the potential high risk of switching them to something new (and question the value gain of doing so)
Pricing & access	Multiple options, including *parity* (seek comparable access and win in field), *discount* (disrupting market on price to secure access), and *premium* (accepting niche positioning supported by strong scientific and economic value communication)	Encourage payers not to select preferred products and use existing relationships/ evidence to maintain/ grow share rather than paying for preferential access

Increasingly, RD manufacturers will need to navigate the transition to competitive categories, creating challenges for both incumbents and new entrants to maintain and preserve value. The industry must ensure cross-functional preparedness and an agile strategy to succeed in these competitive environments.

Sources: FDA Orphan Drug Designations and Approvals Database

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