EVERSANA™

MARKET ACCESS TO CURRENT AND FUTURE RARE DISEASE TREATMENTS

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Access to treatments for rare diseases depends on a delicate balance of a price that allows for the development of innovative therapies while also being considered "affordable." A low price for a manufacturer's therapy would discourage potential developers from focusing their future efforts on new therapies because of a limited return on investments. At the opposite end of the spectrum are barriers to access based on a price that is considered cost prohibitive; utilization is limited in an effort to control costs. The balance between these 2 opposing access decision-making contingencies will determine the degree to which patients with rare diseases will receive the treatments they need.

CURRENT MARKET

For payers, budget impact is one of the most important considerations when deciding how to cover a product. The highest-cost rare disease products usually have a limited impact on budget due to the small number of eligible patients being prescribed the drug; however, several rare disease therapies have been approved by the US Food and Drug Administration in the past few years, with more approvals on the horizon. Payers are increasingly concerned about specialty drug spending, with about one-fifth of specialty spending attributed to rare disease products. Payer management regarding rare disease products focuses on prior authorizations and reauthorizations for product indications. Other utilization management tools, such as step therapy and quantity limits, are used sparingly in this area due to push back from patient advocacy groups. In terms of formulary placement, almost all novel rare disease products are covered by payers, but most are on a high or specialty tier with high cost-sharing requirements. This type of formulary placement, along with the growing trend of shifting insurance plans from a fixed co-payment to a percentage coinsurance payment method, has caused a considerable rise in patient cost-sharing.

From a patient perspective, health insurance and payer coverage guidelines surrounding rare disease treatments restrict broad access to these treatments, leaving only a small number of patients who can bypass insurance and pay for products on their own. Insured patients in the United States are typically granted access to rare disease drugs. This is due to several factors, including:

- About half of rare diseases affect children, which makes management politically difficult
- Aggressive patient advocacy groups
- Without coverage after approval, manufacturers would have no incentive to develop novel products for rare diseases

Manufacturers' value propositions are becoming increasingly important for rare disease products. These value propositions need to justify product pricing by focusing on the burden of severe illnesses and how the products address unmet needs. Rare disease drug manufacturers' role beyond gaining coverage for their products includes providing patient assistance programs to help uninsured and underinsured patients who face out-of-pocket drug costs. These programs are becoming more important as payers push more rare disease costs onto patients.

Policymakers are facing increased pressure to lower the cost of prescription drugs across the board. Rare disease treatments often have the highest price tags among prescription products, making these products a target of harsh criticism and future policies. Some of these policies include:

- Expanding Medicare eligibility to rare disease patient populations, as has been done in the past for amyothrophic lateral sclerosis and end-stage renal disease
- Controlling drug prices through price controls
- Revamping the Orphan Drug Act

These policies would have a tremendous impact on manufacturers' incentives to bring rare disease products to market. Depending on where these policies ultimately land, there could be negative implications for future rare disease products. As the government moves toward bundled payments and other models that shift pharmaceutical costs onto providers, access to treatments could be further restricted. Manufacturers will need to partner with key stakeholders to assure balance is maintained to provide uninterrupted access to treatments.

Future Market-Based Solutions

Several future market-based solutions are being discussed to ensure access. These include the development of an orphan drug reinsurance model, so that these rare disease treatment occurrences can be spread among a larger pool to prevent too great a burden being inflicted on a single payer group. This model provides pharmacy benefit managers with a proactive specialty benefit management approach to pay for innovative therapies. Some of these therapies with a short treatment course may provide long-term efficacy or a cure to a rare disease, which can help mitigate downstream costs.

Access for long-term efficacy can also be accomplished through the development of long-term, annuity-like payments that can help spread costs over a period of time, again easing the blow to a single payer. This may also present the ideal situation for value-based contracts, whereby pharmaceutical manufacturers share in the risk of treatment success, permitting patients on the fringe of treatment guidelines to gain access. This is only possible if payers are protected against failed treatments by identifying the correct endpoint for contracting.

Innovative partnership between stakeholders is thus required to ensure appropriate access for rare disease treatments. However, an example of a successful partnership is the CF Foundation work in pioneered venture philanthropy, which is leading the movement of voluntary health organizations funding drug development with for-profit companies. Future success in accessing rare disease treatments will require these types of innovative partnerships and achieving a pricing balance that provides access to today's treatments and incentives to create tomorrow's cures.

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