PERSPECTIVE ON THE RXPIPELINE

Understanding changes in the medication market and their impact on cost and care.

EnvisionRx continuously monitors the drug pipeline. As treatment options change, we evaluate and share our perspective on the clinical benefits, cost-effectiveness and overall impact to payers and patients. Our Perspective on the Rx Pipeline report provides ongoing actionable insights from our team of clinical experts and the steps we are taking to protect and improve plan performance.

Included in this Edition

- Orphan Drug Debate: Understanding the Increasingly Popular and Competitive Orphan Drug Market While Balancing this Costly Trend

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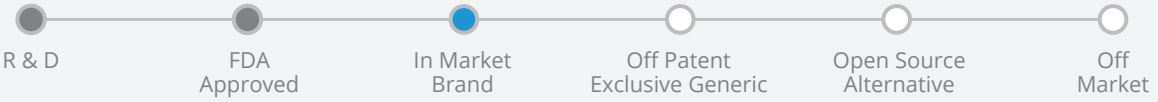
ORPHAN DRUGS

Orphan Drug Debate: Understanding the Increasingly Popular and Competitive Orphan Drug Market While Balancing This Costly Trend

PIPELINE STAGE



TIMING

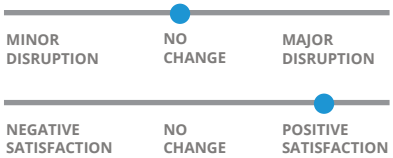
When will payers be impacted?

CLINICAL EFFECTIVENESS

Compared to available options, is this drug better for treatment?

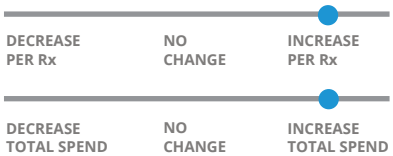
PATIENT EXPERIENCE

Will this positively or negatively impact members?



PAYER IMPACT

How will this influence Rx spend?



Situation Summary

The Orphan Drug Act of 1983 created financial incentives to investigate and launch products targeted at rare diseases or disorders, defined as affecting less than 200,000 patients. The act was created to offset the traditionally high development costs associated with bringing a drug to market with limited potential revenue because it treats diseases that impact a small number of patients. The National Organization for Rare Disorders (NORD), a key participant in establishing the act, estimates that currently about 30 million Americans are afflicted with over 7,000 rare diseases.

The act helps increase development of medications to treat these diseases by granting seven years of marketing exclusivity from the approval date, tax credits, and a shorter Food and Drug Administration (FDA) approval period. The law has been successful, as there were only 34 orphan products marketed in 1983 and over 400 FDA-approved drugs designated for orphan indications by January 2018.

There have been many commentaries published that argue pharmaceutical companies are exploiting gaps in the orphan drug status. An example of this is when a company claims "orphan" status for a drug, but ends up marketing the medication for other, more common conditions to capture larger profits.

Historically, payers have covered orphan drugs because they are frequently the only treatment option for patients. Orphan drugs, often associated with higher costs, have not had a strong impact on plan sponsors in the past because there were so few of these medications. However, as the number of orphan drugs increase, associated costs in this area also increase. These cost concerns raise new coverage issues for plan sponsors around how best to control spend for these expensive medications while providing quality patient care.

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ORPHAN DRUGS

To help plan sponsors control costs and provide quality care, EnvisionRx created an orphan drug classification that uses multiple eligibility criteria to determine true indicators of an orphan drug. The criteria includes:

- FDA orphan indication designation
- Phase three clinical trial population size
- NORD data
- The competitive landscape
- Insight from the National Institute of Health (NIH) Genetic and Rare Diseases Information Center

As new indications are approved for products, inclusion on the orphan drug classification is reviewed for appropriateness. Starting with the proper designation allows the clinical experts at EnvisionRx to remain focused on top drug targets to monitor and analyze for trends, and helps us better evaluate appropriate strategies.

Impact to the Pharmacy Care Experience

EnvisionRx has implemented a process to effectively manage orphan drug spend by taking a comprehensive approach to the management of drugs related to orphan conditions. Strategies to control spend and impact care include:

Pipeline Monitoring: We monitor and report on indication-specific pipeline activity, with an additional focus on these rare conditions. This ensures proactive and timely responses to upcoming drug trends, whether drugs will ultimately be placed on the medical or pharmacy benefit, and forecasting of potential impact. Part of our pipeline drug information services include using tools and clinical resources to identify and predict utilization prior to a drug's approval.

Pharmacy & Therapeutics Review: Our Pharmacy & Therapeutics (P&T) Committee, which helps determine a drug's formulary placement, has a robust evaluation process that is sensitive to the nature of orphan drugs. During the review process the following parameters are considered:

- In-depth review of clinical trial data, taking into account the levels of uncertainty or assumptions being made, ensuring there is an adjustment for population size.
- Evaluation of current practice guidelines, disease severity, available alternatives, level of impact the drug has on disease modification, upcoming pipeline review by indication, and consulting with specialized practitioners in the respective field as needed.

PAYER ACTION PLAN

- **Stay Informed**
  Make sure you are receiving critical drug pipeline information.
- **Determine Impact**
  Orphan drugs may or may not impact your plan. We recommend evaluating your members' diagnosis information and conducting impact modeling with your PBM and/or medical provider.
- **Develop a New-to-Market Process**
  New-to-market blocks are not used to delay approval, but allow for a disciplined strategy and development of authorization criteria. Be sure to understand your formulary choices and what rules are available for coverage exceptions
- **Plan Ahead**
  Start planning for the impact as early as possible.
- **Reassess the Market**
  Continually reassess the orphan drug space to evaluate areas for added efficiency and cost controls.

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ORPHAN DRUGS

Formulary: Overall formulary design is crucial to manage orphan drugs. Our formularies are tightly managed by utilizing a new-to-market claim block that allows time to fully evaluate adoption to a plan's formulary and develop authorization criteria. Additionally, we ensure that orphan drugs are placed on a specialty tier when appropriate.

There is also thoughtful consideration of administration and appropriate use. For instance, orphan-designated gene therapies, such as Kymriah™ (tisagenlecleucel), an infusion therapy for the treatment of B-cell lymphoma, do not make sense on a pharmacy benefit and are excluded. When implementing utilization management criteria around orphan drugs, we are sensitive to ensure access to the appropriate patient population for which there may not be alternatives, while monitoring for off-label or unsafe uses.

Pharmacy Network: As a pharmacy benefit manager (PBM), EnvisionRx identifies those orphan drugs that are best managed by a specialty pharmacy or special site of care. Our pharmacy partners utilize and connect patients to the National Organization for Rare Diseases (NORD). In fact, patients at EnvisionSpecialty are educated about services available to them prior to the first shipment. Pharmacists help support patients with accessing financial support from an organization such as NORD, as well as manufacturer-funded programs.

Plan Design: Determining the appropriate copays and deductibles for expensive orphan drugs can be complex due to the already high financial burden an orphan disease can create for patients. The benefit design needs to be well thought out with a disciplined approach to ensure cost effective, quality care.

EnvisionRx is sensitive to the population in need of orphan drugs. We apply defined authorization and re-authorization criteria to ensure clinical appropriateness and ongoing effectiveness. Authorization criteria may be appropriate for some orphan drugs, while for other therapies it may be more appropriate to manage with quantity limits and step edits. By focusing on a therapy-specific approach, we are able to tailor utilization management closely to the needs of the patient population of the orphan disease or disorder.

Our Clinical Steering Committee

The Envision Clinical Steering Committee brings together leaders from across our national pharmacy care company to monitor the drug landscape, provide recommendations on how to address changes, and to ensure our clients and patients are prepared—in advance.

With any new development, we partner with our Pharmacy & Therapeutics (P&T) Committee and consult with our best-in-class specialty pharmacy, to provide a balanced perspective on the clinical effectiveness of all available options, the cost impact to our plan sponsors and patients, and the impact on the overall patient experience.

Kel Riley, MD
Chief Medical Officer

Learn more ways to improve patient and plan outcomes

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EnvisionRx is a Pharmacy Benefit Manager (PBM), providing affordable and effective prescription drug coverage for employers and health plans. Using its proprietary EnvisionCare model, EnvisionRx optimizes all aspects of the pharmacy care experience to consistently achieve better patient and plan outcomes.

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