PATHWAYS FOR PAYING FOR RARE DISEASE TREATMENTS

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Determining how to pay for the treatment of uncommon yet serious diseases is an important consideration in terms of sustainability and patient access. Novel and expensive therapies will create financial pressure for payers' drug budgets. Clinical pathways have demonstrated success at determining the "right treatment for the right patient at the right time," but they also have the potential to be used to provide guidance on the "right" payment path or alternative payment models to ensure access and to decrease financial burdens for patients and strain on payers and providers.

Payment for rare diseases has become an important focus in ensuring the sustainability of health care. Payers' drug budgets are facing financial pressure because of very high treatment expenses that are often paid all at once, despite the fact that the benefit is received over many years.

Many novel therapies show efficacy in populations with rare diseases that have no previous US Food and Drug Administration (FDA)–approved treatments. These novel therapies often carry a relatively high price tag that can be a financial burden for both patients and payers. Many view high-cost drug therapies as a strain on the US health care budget, and the advent of chimeric antigen receptor therapy (CAR-T), RNA interference (RNAi), and gene therapies for the treatment of rare diseases could increase that cost burden substantially. Even if such drugs do prove to be cost-effective in providing patients with much-needed treatments for genetic and other rare diseases, the question of budget impact and of how these therapies will be paid for remains largely unanswered. Although clinical pathways are designed to identify the "right" treatment, there appears to be an opportunity for pathways to also provide guidance on the "right" payment approach that will ensure access to rare disease treatments for both patients and society.

What Novel Therapies Mean to Each Stakeholder

Experts expect dozens of novel therapies to come to market over the next decade. Many of these will carry astronomical prices, with some topping $1 million per patient, while treating rare diseases that affect approximately 10%– or less–of the US population. According to the National Institutes of Health, more than 7000 rare diseases have been identified, with 95% lacking an FDA-approved treatment.[1,2] With such promising therapies, yet daunting prices, each stakeholder involved will have varying perspectives and interests as these therapies become available—all of which need to be considered and integrated into payment solutions for therapies.

Patient and Caregiver Perspective

Many rare diseases can be life-limiting and even life-threatening. It is difficult or even impossible to prevent most rare diseases without treatment because the majority are caused by changes in genes.[1] Patients and caregivers are responsible for medical bills and treatment-related costs that can create financial toxicity. For caregivers, a family member

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with a rare disease can have a deep impact on their own health and productivity at work. Working with advocacy and patient groups can be helpful in guiding patients to clinical trials, social support, and financial assistance. New therapies provide hope to patients and caregivers and can improve patients' quality of life if they respond to treatment. Pathways that provide guidance on evidence-based options should also integrate patient support options in terms of clinical and financial challenges, as well as quality-of-life concerns.

Manufacturer Perspective

The central challenge for the rare disease drug market is that, although the patient population that benefits from a particular rare disease therapy is generally very small, billions of dollars go into clinical trials, regulatory approval, and commercialization of that single new product. This dynamic results in extremely high prices for drugs that treat rare diseases in order to justify to investors the costs associated with research, regulatory approval, and commercialization.

A pharmaceutical manufacturer recently told investors that it may be able to charge $4 million to $5 million for a potential gene therapy product that treats a life-threatening rare disease called spinal muscular atrophy (SMA).[3] A therapy that could correct a genetic flaw over a lifetime may be economically viable over a span of a couple years. Similar examples could be applicable to many other high-cost diseases, such as hemophilia, rare malignancies with specific genetic defects, and transfusion-dependent ß-thalassemia.

One solution to this problem is to create value-based contracts that permit payment for these therapies over several years. These value-based pharmaceutical contracts would be tied to outcomes such that payers are not financially responsible for treatment failures.[4] Since patients can easily switch health plans each year, this proposal is unlikely to be adopted by payers for treatments with short regimens and multi-year benefits. Manufacturers may need to develop contracts requiring that the payment for cost of therapy follow the patient over time and through other potential payers should he or she switch health plans. This path would improve cash flow issues for payers because the payment would be spread over the benefit period for the treatment. Recently, two US Senators introduced a bill that would lay a legal foundation for pharmaceutical and medical device companies to strike payment installment deals with insurers that are linked to the performance of their products.

Manufacturers may consider another solution to this problem by partnering with financial institutions that provide patients with long-term loans for treatments. This solution would bypass payer approval for products and provide patients with innovative therapies that are priced based on their long-term benefits. Combining any solution with a patient assistance program will be vital to elevating a new treatment to standard of care.

Payer and Clinical Pathways Perspective

According to a 2018 EvaluatePharma Orphan Drug Report, sales of orphan drugs are set to climb by 11% a year through 2024, eventually reaching $262 billion.[5] With a growth rate double that of the overall pharmaceutical market, rare diseases have caught the eye of payers. Payers with a few more rare disease patients than anticipated can see challenging increases in their budgets with the approval of a new expensive treatment. Small payers can become financially insolvent with large costs from a few patients with a rare disease, while some large payers may also see 10% to 20% specialty spending on rare diseases.[6]

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Managing costs related to rare diseases can be a challenge when efficacy of new products is better than that of existing therapies or in rare diseases with no established standard of care. Fifty percent of patients with rare diseases are children, making it difficult to manage treatments for these disease states.[2] Managing costs in rare disease populations may be appropriate

in certain scenarios. Creating a rare disease utilization specialist with experience in several rare diseases can help manage treatment. Rare disease utilization specialists can be guided by clinical pathways in rare diseases with several treatment options. These specialists should also work closely with case managers who prevent direct costs such as hospitalizations and guide patients to appropriate specialists to curtail extra costs.

Predicting the effects of a new rare disease product on budgets can also be a challenge because of the risk of year-to-year variability. Innovative contracts with manufacturers that limit exposure to this variability can be a potential solution that payers should explore in the initial years after a rare disease product has been approved. Additionally, there should be a shared responsibility between manufacturers and payers to examine the epidemiology of rare populations and extrapolate findings to insured populations.

Pathways for Coverage

There are a range of different paths when it comes to coverage for rare disease treatments. These paths have different impacts depending on the level of payer responsibility and pricing. At the far end of the spectrum is the current path, where treatments are paid for in full at the time of administration, which is the sole responsibility of the payer. At the other end of the spectrum would be a situation where Medicare takes on full responsibility for care of patients with rare diseases. There is precedence for this path.

One model for paying for rare diseases is Medicare coverage for end-stage renal disease (ESRD) and amyotrophic lateral sclerosis (ALS, also known as Lou Gehrig's disease).Patients with ALS or ESRD gain the benefit of essential health care coverage through Medicare months after being diagnosed, rather than waiting 2 years for qualification with disability.[7-9] Patients with ESRD require dialysis to manage their disease state, which is paid for by Medicare through a bundled payment to manage costs. This program has reduced overall costs for Medicare, but some stakeholders have raised concerns that bundled payments may limit innovation in the ESRD market or that physicians may be reluctant to use any new ESRD drugs that facilities would find too costly to cover within the payment bundle.[10]

One of 2 other paths include the use of federal reinsurance and subsidies, which, again, has precedence in the use of the Health Insurance Marketplace following passage of the Affordable Care Act. The second is reference pricing, which has been proposed as a method to reduce Medicare Part B drug prices by indexing them to the pricing of treatments outside of the United States. The international pricing index can be applied to rare disease products once they are launched outside of the United States. This would lower prices for high-priced treatments, since single-payer countries use price controls and monopsony power to procure drugs at a fraction of the US cost.[11]

Conclusion

High-cost drug therapies have long strained the US health care budget, and the advent of CAR-T, RNAi, and gene therapies for treating rare diseases could increase that cost burden substantially. Even if such drugs do prove to be cost effective in providing patients with much-needed treatments for genetic and other diseases, the question of budget impact and of how to pay for these therapies remains largely unanswered. The path going forward may include pathways that provide guidance on the "right" payment approach to ensure patient access to potentially life-saving treatments.

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REFERENCES

1. National Institutes of Health (NIH), Genetic and Rare Disease Information Center. FAQs about rare diseases. https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases. Updated November 30, 2017. Accessed January 30, 2019.
2. Rare diseases: facts and statistics. Global Genes website. https://globalgenes.org/rare-diseases-facts-statistics/. Accessed January 30, 2019.
3. Novartis says SMA gene therapy is cost-effective at $4-5 mln per patient [news release]. Zurich, Switzerland: Novartis; November 5, 2018. https://www.reuters.com/article/novartis-gene-therapy/novartis-says-sma-gene-therapy-is-cost-effective-at-4-5-mln-per-patient-idUSFWN1XG0OD. Accessed January 30, 2019.
4. Walker J. Biotech proposes paying for pricey drugs by installment. *The Wall Street Journal*. January 8, 2019. https://www.wsj.com/articles/biotech-proposes-paying-for-pricey-drugs-by-installment-11546952520. Accessed January 30, 2019.
5. *EvaluatePharma Orphan Drug Report* 2018. Evaluate website. http://info.evaluategroup.com/rs/607-YGS-364/images/OD18.pdf. Published May 2018. Accessed January 30, 2019.
6. Silverman E. No price pressure on orphan drugs (yet). *Managed Care* website. https://www.managedcaremag.com/archives/2017/6/no-price-pressure-orphan-drugs-yet. Published June 8, 2017. Accessed January 30, 2019.
7. Signing up for Medicare if you have ESRD. Medicare.gov website. https://www. medicare.gov/information-for-my-situation/signing-up-for-medicare-if-you-have-esrd. Accessed January 30, 2019.
8. Norris L. Medicare eligibility for ALS and ESRD patients. Medicareresources.org website. https://www.medicareresources.org/medicare-eligibility-and-enrollment/medicare-eligibility-for-als-and-esrd-patients/. Published October 1, 2018. Accessed January 30, 2019.
9. A professional's guide to assisting families with obtaining government benefits. The ALS Association website. http://www.alsa.org/als-care/resources/publicationsvideos/factsheets/professionals-guide-govt-benefits.html. Accessed January 30, 2019.
10. US Government Accountability Office (GAO). *End-Stage Renal Disease: Bundling Medicare's Payment for Drug With Payment for All ESRD Services Would Promote Efficiency and Clinical Flexibility*. Report no. GAO-07-77. https://www.gao.gov/assets/260/253353.html. Published December 6, 2006. Accessed January 30, 2019.
11. Pakizegee M, Stefanacci RG. Value-based clinical pathways in the world of reference-priced Medicare Part B drugs. *J Clin Pathways*. 2018;4(10):38-39. doi:10.25270/jcp.2018.12.00052