

Gene therapy and US healthcare: Rationalizing the price of promise

An orphan disease is defined as a condition that affects fewer than 200,000 people nationwide. Collectively, these rare diseases may affect as many as 25 million Americans, according to the National Institutes of Health, making these diseases a public health priority for identifying treatments, including curative treatments. For many people living with an orphan disease, the prognosis has been equated to a life sentence; yet the promise of cell and gene therapies is providing many with hope of a different future. No doubt these innovations have the potential to offer life-changing hope for many rare disease patients; but for many health plans, these innovative treatments and therapies are causing much anguish.

First, the current remuneration system for healthcare in the United States is a fee-for-service model, with health plans reimbursing for a patient's medical care, testing, treatments, etc. as they receive them. These treatments and/or services are paid by the member's health plan for the duration that the member remains on that plan. The costs incurred are then theoretically offset by any benefits that would accrue. When a patient changes health plans, the new insurer begins to assume any of the costs associated for treatments and/or therapies the member receives for however long that member remains on their health plan, which can be likened to a pay-as-you-go model.

The current payment model was never meant to cover one-time curative therapies. The anticipated one-time multi-million-dollar price tag associated with these new cell and gene therapies has the potential to create a much more concentrated burden on health plans, which may be untenable for smaller plans. Administered as a one-time treatment that is expected to offer long-term, even curative, benefits, gene therapy presents challenges considering the number of times patients switch health plans. Patient mobility creates the potential for misaligned cost/benefit incentives for the health plan that picks up the one-time high cost associated with these treatments without getting an opportunity to realize the savings because patients often change health insurers.

In addition, the full benefit and risks associated with these treatments remain unclear, making the combination of concentrated up-front costs and uncertainty regarding real-world clinical benefit and long-term durability of the therapeutic benefits challenging for payers. Given that the current treatments have been studied in a relatively small number of patients, there is concern that payers might eventually have to face additional costs.

The US Food and Drug Administration (FDA) estimates that, by 2025, they will be approving 10–20 cell and gene therapy products a year based on an assessment of the current pipeline and the clinical success rates of these products (<https://www.fda.gov/news-events/press-announcements/statement-fda-commissioner-scott-gottlieb-md-and-peter-marks-md-phd-director-center-biologics>). With so many cell and gene therapies in the pipeline, payers have grappled with underwriting the risk of full payment for the entire range of gene therapies coming to market. I rarely hear payers say they would not consider covering treatments for conditions where there are no current treatments available, such as spinal muscular atrophy. However, many payers have expressed the need to justify a return on investment for any new and innovative treatments for conditions that currently have effective treatment options available, such as hemophilia.

At this point, you may be asking whether health plans see anything positive about cell and gene therapies coming to market. I do believe that health insurers want patients to achieve optimal outcomes, and if these new innovative treatments and therapies are the answer, then they want to do all they can to provide access to them. That is why they are working to identify the issues and challenges to the status quo these new therapies present and, in turn, striving to find solutions. Many payers have reached out to us at the National Hemophilia Foundation to discuss the challenges and identify potential solutions. They are also engaging in collaboratives, such as the Massachusetts Institute of Technology (MIT) New Drug Development Paradigms (NEWDIGS), and meeting with manufacturers and vendors that have developed various solutions to address the challenges.

It is my belief that all stakeholders have the responsibility to reflect on the promise as well as the challenges these innovative therapies present, to collaborate to identify solutions, and to set expectations accordingly. If I had a crystal ball, I believe it would indicate that the greatest chance for success will include manufacturers bringing these products to market at reasonable prices (not what the market will bear) and transitioning from the current fee-for-service payment model to one or more solutions that ties payment to performance. Whether paid in a lump-sum, up-front payment or paid over a period of time when performance metrics are met, health plans will need some assurance that they will not be paying several million dollars for a new treatment if the response or durability is not guaranteed.

Editorial

Of course, there is no one-size-fits-all option, so health plan sponsors will do best to frequently engage with their vendor partners to stay on top of all current and new solution offerings as the gene and cell therapy market continues to evolve.

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