

Modernizing Reimbursement for Gene Therapies

After more than 20 years of careful study, gene therapies are making their way through the FDA approval process and to patients who need them. By targeting the cause of disease directly, gene therapies have the potential to fundamentally reshape the trajectory of disease. Gene therapies are different from traditional pharmaceutical and biologic medicines in that they are delivered as one-time treatments that can offer long-lasting – sometimes lifelong – benefits for patients and the healthcare system alike. We must think anew about how they are covered and paid for to ensure these therapies reach patients.

In many cases, gene therapies halt but cannot reverse the effects of a disease by addressing the underlying genetic cause. Delays in access to approved gene therapies can result in patients suffering irreversible damage caused by their disease. Increased access to gene therapies benefits patients' short- and long-term health, their caregivers, the healthcare system, and society.

Gene Therapies and Value-based Payment

We must develop a reimbursement system equipped to adequately reimburse providers for administering groundbreaking gene therapies. A novel reimbursement pathway will drive sustainability for gene therapies, while enabling patient access and provider payment.

Value-based payment (VBP) arrangements, in which payment is tied to a patient's actual outcomes after receiving a therapy, are a critical component of a modern reimbursement system. VBPs are a vital tool that can support the flexibility that manufacturers, payers, and providers need to define and agree upon payment models that will work best in the context of a given disease and patient.



Alternative Price Reporting Approaches:

We support development of alternative price reporting mechanisms for Best Price and Average Manufacturer Price (AMP) for transformative therapies and clarifying guidance on how manufacturers can incorporate VBPs in their calculations and satisfy financial obligations of federal healthcare programs, such as payment of mandatory rebates.



Compliance Clarifications:

The Department of Health and Human Services' Office of Inspector General should clarify compliance-related issues that have posed barriers to VBP advancement, such as how safe harbors can accommodate for, among other issues, the collection and sharing of data to adjudicate a contract and VBP inclusion of outcome measures that are meaningful to manufacturers, payers, and patients.

Modernizing Reimbursement for Gene Therapies



Through a final rule titled Establishing Minimum Standards in Medicaid State Drug Utilization Review (DUR) and

Supporting VBP for Drugs Covered in Medicaid, the Centers for Medicare and Medicaid Services (CMS) is taking action to provide mechanisms to address Medicaid Drug Rebate Program price reporting as it pertains to VBPs. This action presents a significant first step in clearing certain hurdles to implementing VBPs. IGT looks forward to working with the Administration on VBP-related policies and engaging in next steps to ensure a workable system for supporting a robust array of VBPs.



Congress has also taken interest in this issue. Legislative language delineating a pathway for VBP appeared in several bills and amendments as Congress

worked on comprehensive drug pricing reforms in 2019 and 2020. More recently, the Cures 2.0 initiative and the House Innovation Caucus have expressed interest in working on these issues, especially in light of the recent Medicaid rule changes, which further opened up the possibility to operationalize VBPs. IGT is actively engaging with many of these efforts to continue the progress being made to ensure patient access to gene therapies. We look forward to working with Congress to identify additional opportunities to advance VBPs on a bipartisan basis.

“ Until now, clear pathways for enabling the development of these innovative payment mechanisms did not exist. The CMS Final Rule...is a significant outcome for facilitating VBP arrangements as it bolsters transparency, flexibility, and innovation in drug pricing.

- IGT statement, December 2020



IGT works with policymakers and stakeholders to develop a sustainable, flexible, and permanent payment pathway reflective of the scientific advancements resulting in gene therapy breakthroughs.

By modernizing our payment system we can ensure patients have access to these innovative, life-changing therapies.

LEARN MORE AT [GENE-THERAPIES.ORG](https://www.gene-therapies.org)