

Ensuring Patient Access to Gene Therapies

After more than 20 years of careful study, gene therapies are now 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.

Ensuring and increasing patient access to gene therapies will take broad action by HHS, including CMS, and FDA, Congress, and a cross-section of healthcare stakeholders. IGT supports essential efforts to:



Encourage Greater Adoption of Value-Based Payments (VBPs):

Value-based payment 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. **See our *VBP Policy Brief for more details.***



Address Disparate Reimbursement Methodologies:

Medicare bundled payment methodologies in the inpatient setting create access issues for patients seeking gene therapies. To address these, IGT supports DRG revisions that may be necessary in the future. IGT also advocates for innovative approaches in Medicaid to alleviate access delays resulting from traditional inpatient payment bundles.

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Facilitate Payment-over-time Arrangements:

Not all disease states that may be treated with a gene therapy are amenable to VBPs, such as those for rapidly progressive, degenerative diseases. IGT supports development of mechanisms to facilitate payment-over-time arrangements for gene therapies, which may support expanded access to certain gene therapies.



Require Timely Coverage Based on the FDA-approved Label:

Many of the gene therapies in late-stage development are treatments for rare diseases. FDA recognizes that clinical trials for rare diseases may not include patients that span the entire spectrum of the disease state due to inherent variability. As such, when considering approval and crafting the indications in the label, the agency considers scientific plausibility, mechanism of action, and other factors. In contrast, payers often implement restrictive coverage policies that do not align with the FDA-approved label. IGT urges CMS to issue guidance that reiterates states' and contracted managed care organizations' obligations to cover "covered outpatient drugs" immediately upon FDA approval and aligned to the approved medically accepted indication.



Expedite Diagnosis Code Creation:

The Regenerative Medicine Advanced Therapy (RMAT) designation and accelerated approval pathway are critical to facilitate rapid review of transformative therapies. However, following FDA-approval, patients and providers may face additional hurdles due to the lack of an ICD-10 diagnosis code for these rare diseases. Inaccurate coding often leads to providers engaging in time-consuming appeals and exceptions processes, which hinders patient access and significantly impacts health outcomes. Congress should require HHS to implement expedited diagnosis code processes for transformative therapies that align with FDA approval.

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

We must ensure patients have access to these innovative, life-changing therapies.