Modernizing Reimbursement for Gene Therapies

IGT
Institute for Gene Therapies

Gene therapies are different from traditional pharmaceutical and biologic medicines as they target the cause of disease directly and are potential one-time treatments that offer long-lasting, even lifelong, benefits for patients. Because of their unique nature, we must think anew about how they are covered and paid for and develop a reimbursement system equipped to adequately reimburse providers for administering groundbreaking gene therapies.

A modernized and flexible reimbursement system is needed to promote and safeguard the promise that gene therapies offer to patients, families, and the US healthcare system. A novel reimbursement pathway that truly reflects the transformative nature of gene therapies will drive sustainability, enable patient access and sufficient provider payment, and keep the promise of gene therapies alive for future generations and diseases.

Alternative Payment Arrangements

VALUE-BASED PAYMENT ARRANGEMENTS – OR VBPS – in which the maker of a gene therapy receives payment based on a patient's ability to meet certain outcome measures, are a critical component of this novel pathway. IGT supports flexibility in defining VBP arrangements to accommodate the expected growth in gene therapies approved by the FDA, including policy changes such as those below to facilitate VBPs and ensure appropriate access to gene therapies:

ALTERNATIVE PRICE REPORTING REQUIREMENTS: 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 VBAs 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 but not included in a drug's FDA-approved label.

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The Centers for Medicare and Medicaid Services (CMS) and Congress have taken interest in this issue in recent years:

2020:
A CMS rule - known as the Multiple Best Prices Rule - to provide mechanisms for addressing Medicaid Drug Rebate Program price reporting metrics as they pertain to VBPs marked a significant first step in facilitating VBPs.

2022:
Congress introduced the Medicaid VBPs for Patients (the MVP) Act, which builds on the work of CMS and establishes clear pathways to VBP arrangements for manufacturers, payers, and providers.

2023:
The Center for Medicare and Medicaid Innovation released new payment models, including one to test strategies to expand access to gene therapies for Medicaid beneficiaries.

Not all disease states that may be treated with a gene therapy are amenable to VBP arrangements, such as those for rapidly progressive, degenerative diseases. IGT supports development of mechanisms to facilitate alternative payment arrangements for gene therapies, such as subscription-based coverage or annuity payments, and supports essential efforts to:

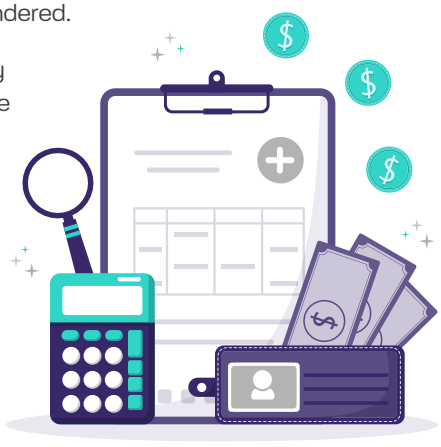
- **CARVE-OUT PAYMENTS:** IGT also advocates for granting Medicaid programs authority to carve-out payment for gene therapies from inpatient payment bundles to sufficiently cover the cost of a gene therapy and developing add-on payment constructs that exist in Medicare.

- **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:

  - Establishing an MS-DRG for Each Approved Gene Therapy – the IPPS and MS-DRG system ("a system of averages") was designed before transformative therapies like gene therapies became a reality – IGT encourages CMS to view gene therapies in a different light due to their transformational nature and to use whatever tools necessary to ensure that access is not hindered.

  - Establishing an Enhanced NTAP Pathway for Gene Therapies – CMS should provide an NTAP payment for approved gene therapies for at least five years to ensure that claims volumes reach a satisfactory level to properly inform rate setting and to facilitate continued gene therapy innovation.

  - Increased Add-on Payment – CMS should increase the add-on payment for qualifying gene therapies to 100%.



IGT ALSO ENCOURAGES TIMELY COVERAGE BASED ON THE FDA-APPROVED LABEL AND EXPEDITE ICD-10 DIAGNOSIS CODE CREATION FOR RARE DISEASESE.

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.

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