CAPITOL STREET

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Gene Therapy Policies Advance as HHS Finalizes IRA Negotiation Rules

CMS Cell & Gene Pilot Starts 2026

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This week, the IGT hosted its second Capitol Hill briefing focused on the impact of sickle cell disease (SCD) and the potential for gene therapies in treating patients with sickle cell disease. Panelists included Dr. James Taylor (Director of Howard University College of Medicine's Center for Sickle Cell Disease), Dr. Donna Christian- Christensen (Chair of IGT Scientific, Academic and Medical Advisory Council), Shanetta Richardson (Sickle Cell Patient Advocate), Regina Hartfield (President & CEO, Sickle Cell Disease Association of America) and John Feore (IGT Policy Director).

⋙ Our Take & Next Up

HHS/CMS may release final IRA Drug Negotiation Guidance in the next 1-2 days. See our summary of the proposed tule for the “hot topics” that will likely be addressed. Specifically, we think that (1) bona fide marketing for generics/biosimilars (2) secrecy of negotiations (3) active moiety / combination e.g. Darzalex ex) (4) orphan designation definition are likely to be discussed. **On gene therapy we await:** (1) legislation advancing Medicaid best prices as well as (2) CMS policy on a pathway for cell & gene therapy. We do not foresee legislation passing this year but the CMMI demonstration will move forward (2026 start date) The IGT Capitol Hill briefing was #2 of 3 that will be hosted by IGT. With another FDA accelerated approval in gene therapy granted for Elevydis (SRPT), we expect more gene therapies to proceed through the expedited pathway. CMMI continues to conduct stakeholder meetings on cell & gene therapies to help craft their Medicaid model (2026). We will see additional model details in 2024/2025. Cost and coverage continue to be a major barrier to access despite the enthusiasm of patients and providers for gene therapies. A gene therapy for sickle cell disease has the potential to astronomically increase state Medicaid program costs. State adoption of multiple best prices is expected to take time, but we may see quicker adoption following FDA approval of sickle disease gene therapies potentially as soon as 4Q23.

Key Points

The Institute for Gene Therapies is a stakeholder organization that advocates for a modernized regulatory and reimbursement framework for gene therapies. Members of the IGT advisory council include manufacturers (SRPT, PTCT), patient advocacy groups (Cure SMA, American Autoimmune Related Diseases Association), and various research organizations (National Genome Center).

IGT has two notable policy objectives.
(1) Passage of the *Medicaid VBPs for Patients Act*, which codifies CMS regulation that allows for multiple best prices;
(2) A CMS inpatient hospital payment gene therapy NTAP (new technology add-on payment) designation for enhanced payments.

***The Medicaid VBPs for Patients (MVP) Act* is moving through the House but may not pass in 2023,** as there is no Senate companion (or CBO score). On May 24th, the MVP Act passed the House Energy & Commerce committee with bipartisan vote of 31 yes to 19 no. The MVP Act codifies 2022 guidance that allows state Medicaid programs to voluntarily enter value-based purchasing (VBP) arrangements and allows manufacturers to use varying best price points for Medicaid. The bill also resolves technical issues like requiring CMS guidance on inpatient hospital stays related to gene therapy administration to ensure provider reimbursement. IGT notes the bill creates visibility into VBP arrangements in the future by setting up a consistent federal regulatory framework. Cosponsors include House E&C subcommittee on Health Chair Guthrie (R-KY), and Subcommittee Ranking Member Eshoo (D-CA).

A New Technology Add-on Payment (NTAP) reimbursement pathway for gene therapies is a proposed Medicare coverage solution. As a reminder, in the 2023 IPPS final rule, CMS solicited comments on how to address inpatient payment issues for rare diseases, including ways to address hospital formulary challenges for high-cost, low volume therapies. IGT requested consideration of an enhanced NTAP alternative pathway for gene therapies per what is offered for Qualified Infectious Disease Products (QIDPs) and Limited Population Pathway for Antibacterial and Antifungal Drugs (LPADs). CMS did not release any new policies to address rare disease payment issues in the 2024 IPPS, but the topic is expected to be revisited in the future.

What is an NTAP? A New Technology Add-On Payment (NTAP) is a form of reimbursement from CMS that allows for an additional payment on top of the standard Medicare Severity Diagnosis- Related Group (MS-DRG) payment to pay for the usage of innovative technologies. For a traditional NTAP, products must meet CMS guidelines on newness, cost burden, and show substantial clinical improvement. NTAP designation for innovative drugs and devices typically lasts about three years.

CMMI is embarking on a cell & gene therapy payment pilot (2026), allowing states to band together and engage in outcomes-based arrangements with CGT companies. On Feb 14, HHS released three drug pricing models. CMMI's cell & gene model would test outcomes-based agreements (OBAs) to help increase Medicaid access to high-cost specialty drugs. CMS would take the lead in coordinating multi-state OBAs with manufacturers and take responsibility for implementation. 3 potential methods include (1) Outcomes-Based Payments, with a portion of payment up front, and the remainder based on clinical milestones (2) Outcomes-Based Rebates, with payment up front and a rebate if a specific clinical outcome is not achieved (currently used by BLUE with commercial payers) and (3) Outcomes-Based Annuities, with fixed price payments spread over time if beneficiaries receiving

treatment continue to achieve specific clinical outcomes. Model development is starting this year with aims to announce in 2024-2025, and testing to start in 2026.

The impetus for the CMMI pilot is sickle cell disease, which will potentially be addressed via new therapies (BLUE, CRSP, VRTX, others) and could financially cripple state Medicaid agencies. Panelists spoke about the impact of sickle cell disease (SCD) and barriers to care. CMMI is expected to focus on sickle cell anemia therapies (BLUE, NVO, CRSP, VRTX, EDIT others), and cancer therapies (BMY, NVS, JNJ, GILD, IOVA, others). IGT panelist Regina Harfield, President of the SCD Association of America, discussed the difficulty finding specialized care and the barriers that SCD patients face, including medical discrimination. While there are 4 FDA approved drugs specifically for sickle cell disease, only 2-4% of sickle cell patients have access to therapies. Current FDA approved therapies for sickle cell disease include Droxia (BMY), Endari (Emmaus Life Sciences), Oxbryta (PFE), and Adakveo (NVS). SCD care was described as astronomically expensive and hospitals are reimbursed below cost.

50% of sickle cell patients obtain coverage through Medicaid. SCD is the most common genetic disease in the US and 2 potential SCD gene therapies are nearing FDA approval in 2023: exa-cel (VRTX & CRSP) and lovo-cel (BLUE). A gene therapy approval for sickle cell disease could potentially bankrupt state Medicaid programs as roughly 50% of patients with sickle cell disease obtain coverage through Medicaid. The trigger for CMMI gene therapy model was likely as a response to help control state healthcare costs as the gene therapy industry develops further.

Commercial reimbursement for gene therapies is advancing, but there is a significant financial impact on self-insured (ERISA) plans. Cell and gene therapies are frequently named as the top cause of catastrophic claims for self-insurance (ERISA) plans with stop-loss insurance. IGT is pushing to consider the lifetime value and curative nature of gene therapies in determining reimbursement to justify the costs for treatments. Gene therapies cost millions with two of the costliest being Hemgenix (CSL Behring), a hemophilia B therapy, priced at $3.5 M and the recently accelerated approved Elevydis (SRPT), a Duchenne muscular dystrophy (DMD) treatment, priced at $3.2 M.

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