LOS ANGELES & SAN FRANCISCO

Daily Journal

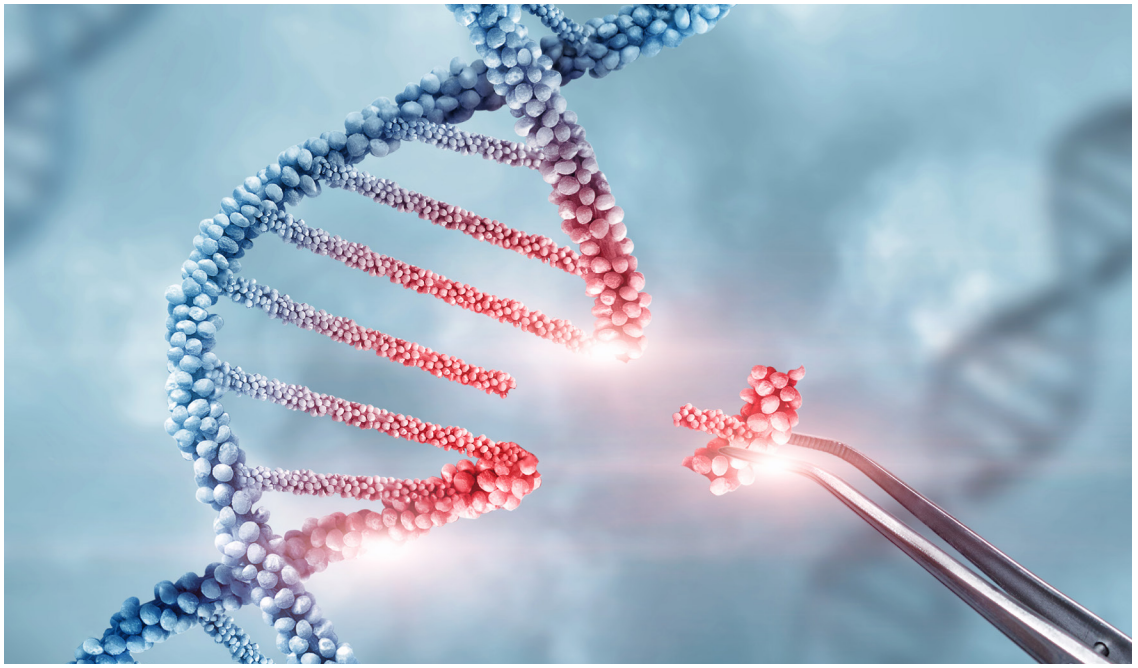
www.dailyjournal.com

TUESDAY, DECEMBER 26, 2023

PERSPECTIVE

The future is here: state payment for life-changing gene therapies

By Katie Pettibone



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The 2013 film Elysium centers over a fight between the haves and the have-nots, focusing on access to advanced Med-Bays, devices that can heal any chronic, life-threatening, or rare disease or condition, such as cancer or genetic diseases. The movie ends with a young girl who is suffering from leukemia accessing the Med-Bay, thereby curing her illness, and Med-Bays being flown to Earth for all of mankind. While the movie seemed to be fiction at the time, we are now potentially in the dawn of innovative treatments that could turn this from fiction to reality.

Over the last few years, there have been glimmers of future innovations with the approval of gene therapies such as Luxturna, Zolgensma , and others (U.S. Food and Drug Administration (FDA), Approved Cellular and Gene Therapy Products). But with the recent announcement of FDA approval of the first cell-based gene therapies for the treatment of sickle cell disease (SCD), which could help thousands, the impact of these innovations has captured the attention of healthcare payers because the cost of treatment is in the millions (CNN, "FDA approves two gene therapies for sickle cell, bringing hope to thousands with the disease" by Meg Tirrell, Dec. 8, 2023).

These transformative and potentially curative, one-time administered therapeutics are pushing governments to reimagine the old way of contracting with manufacturers for therapeutics. The federal government's Medicaid Prescription Drug Rebate Program (MDRP) and California's Medi-Cal state rebate program (WIC §14000, *et seq.*) both contract with pharmaceutical companies to secure favorable terms for the purchase of prescription drugs. Under these programs, for a manufacturer's drug to be covered under Medicaid, the company enters into a rebate agreement with the respective Health and Human Services agency to provide a rebate on a portion of the Medicaid payment for the drug to the states, who in turn share the rebates with the federal government. These rebates help reduce the overall cost of prescription drugs for the Medicaid program. Medicaid programs cover nearly all of the manufacturer's Food and Drug Administration (FDA) approved drugs, and the drugs are eligible for federal matching funds. The rebate amounts are statutorily set, vary by brand drugs, generic drugs, or specialty brand drugs, and include an inflationary component. Pursuant to the program's rules, states multiply the units of each drug purchased by the unit rebate amount (URA) and invoice the manufacturer for that amount, often quarterly. Manufac-

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turers then pay states the statutory rebate amount, a volume-based contract. For California, MediCal engages in supplemental rebate agreements with pharmaceutical manufacturers as well. While MediCal participates in the federal Medicaid Drug Rebate Program, Medi-Cal's agreements involve negotiated rebates that go beyond the federally mandated rebates provided under the Medicaid Drug Rebate Program.

Alternative payment models

With the arrival of one-time, transformative gene therapies and other innovative treatments, manufacturers and payers are seeking new models of reimbursement to facilitate patient access, manage financial risks, and maintain affordability. Therapies that are administered once, with success measured by how long a patient is free of disease, requires a new approach to contracting. "Value-based" purchasing arrangements (VBA), also known as value-based purchasing (VBP), have come to the fore, as these are designed to tie reimbursement to how a drug performs. These contracts allow manufacturers to share the financial risk of a therapy that today otherwise would be borne by the payer alone. This contract may take the form of a variety of structures, but ultimately if a drug fails to produce an agreed-to clinical outcome or benchmark, then this can trigger a refund to the state. CMS has approved state plan amendments (SPAs) for states to utilize VBAs under this framework and as part of state supplemental rebates. In 2018, Oklahoma's Medicaid program executed the first VBA, in the form of supplemental rebate agreements ("Oklahoma Signs the Nation's First State Medicaid Value-Based Contracts for Rx Drugs," National Academy for State Health Policy Blog, 09-25-18). If the drug fails to meet certain benchmarks related to effectiveness and outcomes, the manufacturer will make additional payments to the state in the form of a supplemental rebate. On Dec. 21, 2020, CMS approved a final rule to Medicaid's Drug Rebate Program to authorize more value-based payment arrangements for drugs ("Establishing Minimum Standards in Medicaid State Drug Utilization Review (DUR) and Supporting VBP for Drugs Covered in Medicaid, Revising Medicaid Drug Rebate and Third Party Liability Requirements," 85 FR 87000, 42 CFR Parts 433, 438, 447 and 456). Additionally, since Oklahoma executed the first VBA, 20 additional states have filed a state plan amendment (SPA) seeking approval of a Medicaid VBA, including: Alabama, Arizona, Arkansas, Colorado, Indiana, Louisiana, Massachusetts, Michigan, Missouri, New York, North Carolina, North Dakota, Ohio, Oregon, Pennsylvania, Tennessee, and Texas (available at: https://www.medicaid.gov/). These states are in various phases of implementation, from exploring contracts with clinical patient outcomes as benchmarks to having executed contracts.

California

California has not yet submitted an SPA for approval of a state VBA. In California, Welfare and Institutions (W&) Code § 14105.33(b) (1) mandates a limited specific method of contracting, reflected in the California Department of Health Care Service's (DHCS) two contract templates in use by Medi-Cal. They have been used for several decades and do not accommodate new treatment modalities nor VBAs. However, the work of companies like CRISPR, Vertex Pharmaceuticals, and others have begun to raise the need to rethink contracting for gene therapies and other innovations. In 2021, Senator Steven Bradford (D-Gardena) introduced Senate Bill 521 to update language in the W&I Code to ensure that DHCS had clear authority and legislative guidance encouraging DHCS to pursue a SPA for approval to enter into VBAs. However, as reported in the California State Senate Health Committee's analysis of Feb. 17, 2021, DHCS was unclear about how to implement such an arrangement and whether it needed additional authority to pursue such arrangements. Although the bill received no "No" votes, it was held in the State Assembly Appropriations Committee.