AMCP
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January 22, 2024

The Honorable Bill Cassidy
520 Hart Senate Office Building
Washington, DC 20510

Dear Ranking Member Cassidy,

The Academy of Managed Care Pharmacy (AMCP) thanks you for the opportunity to respond to your request for information on "Improving Americans' Access to Gene Therapies" published on December 5, 2023.

AMCP is the nation's leading professional association dedicated to increasing patient access to affordable medicines, improving health outcomes, and ensuring the wise use of health care dollars. Through evidence and value-based strategies and practices, AMCP's nearly 8,000 pharmacists, physicians, nurses, and other practitioners manage medication therapies for the 270 million Americans served by health plans, pharmacy benefit management firms, emerging care models, and government health programs.

Cell and gene therapies offer significant promise to many patients, including those with cancer and certain rare diseases. There have been 34 treatments considered to be cell and gene therapies approved by the Food & Drug Administration (FDA) as of January 2024, including Casgevy — the first approved treatment to use CRISPR genome editing technology — in December.[1] These therapies treat a variety of serious conditions, including cancer, sickle cell disease, hemophilia, and spinal muscular atrophy. While these therapies are innovative and frequently curative, they are also costly. Casgevy, which treats sickle cell disease, costs $2.2 million for a course of treatment. Cell and gene therapies can cost millions of dollars on the high end, but the low end is still hundreds of thousands of dollars. Unfortunately, this puts these potentially life-saving treatments out of reach for many patients, especially those on Medicaid.

The Medicaid VBPs for Patients (MVP) Act of 2023 (H.R. 2666) would help improve access to these therapies for America's most vulnerable patients while also protecting Medicaid programs from paying for expensive treatments that are not effective. Introduced by Representatives Brett Guthrie, Anna Eshoo, John Joyce, Jake Auchincloss, Mariannette Miller-Meeks, and Scott Peters, this bipartisan bill expands the ability of Medicaid programs and pharmaceutical manufacturers to enter into value-based purchasing agreements, where the price paid for the treatment is tied to how effective if is for the patient. This is the dominant practice in the commercial market, where parties agree to certain patient outcome benchmarks in advance. Reimbursement methods vary by contract but frequently include installment payments, partial or total refunds, and rebates. Last year, AMCP's Value-Based Contracting

[1] "Approved cellular and gene therapy products." (2023). United States Food & Drug Administration. https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products/approved-cellular-and-gene-therapy-products.

Advisory Group published a lexicon of value-based[2] contracting concepts and real-world examples to distinguish between these emerging models.[3]

The Department of Health and Human Services (HHS) finalized the Medicaid Multiple Best Price Rule under former President Trump in an effort to expand the use of outcomes-based contracting, which were then underutilized by both private and public stakeholders. The rule was then further updated by President Biden in 2022. While the rule served as an important release valve to motivate uptake of value-based contracts in the commercial market, Medicaid has not realized the same benefit. Many cell and gene therapies are only available under value-based contracts, leaving Medicaid beneficiaries without access.[4] The MVP Act provides the clarification and stability that manufacturers and Medicaid programs need to voluntarily enter into outcomes-based contracts by clarifying that the best price under a value-based contract is the highest price paid assuming that all benchmarks are satisfied.

Medicaid programs currently have the option to approximate a fee-for-service price if they choose to cover a cell or gene therapy. This approach leaves them vulnerable because cell and gene therapies can often have highly individualized outcomes for patients. What is completely curative for one patient may be totally ineffective for another, or somewhere in between. Under a fee-for-service arrangement, the Medicaid plan is simply out the cost of the treatment in the event of failure. While a high price is worth it to cure a patient's condition, Medicaid programs are not equipped to absorb the cost if treatment is ineffective.

The MVP Act addresses these problems, and the Energy & Commerce Committee voted to send the bill to the full House of Representatives for a floor vote. However, a Senate companion has not been introduced. AMCP encourages the Health, Education, Labor & Pensions (HELP) Committee to work with the House sponsors to introduce and pass a Senate version in 2024. Doing so would help address the significant disparities in access to life-saving cell and gene therapies that are out of reach for Medicaid beneficiaries.

Thank you for your time and the opportunity to respond to your request for information. AMCP looks forward to working with you to further enhance patient access to innovative therapies.

Sincerely,

Jennifer L. Mathieu
Senior Vice President, Professional & Government Affairs
Academy of Managed Care Pharmacy

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[2] Academy of Managed Care Pharmacy. (2023). "Talking the talk: In the world of value-based care, words matter." https://www.amcp.org/sites/default/files/2023-12/VBCLexicon_DEC2023_0.pdf.
[3] Academy of Managed Care Pharmacy. (2023). "Talking the talk: In the world of value-based care, words matter." https://www.amcp.org/sites/default/files/2023-12/VBCLexicon_DEC2023_0.pdf.
[4] Horrow, C., & Kesselheim, A. S. (2023). Confronting high costs and clinical uncertainty: innovative payment models for gene therapies. *Health Affairs (Project Hope)*, *42*(11), 1532–1540. https://doi.org/10.1377/hlthaff.2023.00527.