



WHITE PAPER

HIGH-INVESTMENT MEDICATIONS:
**PREDICTABILITY, AFFORDABILITY,
AND ACCESSIBILITY**

Abstract

While the Food & Drug Administration (FDA) is continuing to approve novel therapies to address important medical needs, the costs of certain therapies are raising value and affordability concerns for employers, payers, and patients. To explore solutions for improving the predictability, affordability, and accessibility of high-investment treatments, AMCP held a multi-stakeholder Partnership Forum in Arlington, Va., on April 26 and 27, 2022. Forum participants were tasked with three objectives: 1) identifying stakeholder challenges associated with high-investment medications, 2) exploring the challenges and opportunities related to financial tools to address predictability, affordability, and accessibility for high-investment medications, and 3) determining the challenges and opportunities of potential policy solutions to improve the predictability, affordability, and accessibility of high-investment treatments. Stakeholders noted that the benefits of these therapies to patients can be significant, and though the financial costs of these high-investment drugs are substantial, these products are used in a targeted and precise manner. However, the uptake of innovative payment strategies to manage them has been slow. To prevent future challenges, payers should implement incremental changes to prepare for the future impact of the growing pipeline of high-investment medications. Opportunities for process improvement include streamlining data collection, standardizing terminology, providing education on new financial tools and policy solutions, and utilizing a variety of financial tools tailored to specific needs. Engaging and partnering multiple stakeholders will be necessary to improve the predictability of outcomes and costs, affordability for patients and the health care system, and accessibility for patients.

Introduction

While the FDA continues to approve novel therapies to address important medical needs, the costs of certain therapies are raising sustainability, value, and affordability concerns for employers, payers, and patients. This is especially true of innovative medication technologies, such as cell and gene therapies, which offer potentially life-changing therapeutic advances or cures. Many of these high-cost medications are used only once or for a short duration, creating challenges for patients, health care providers, and payers. Examples include the need for complex care coordination, uncertain long-term health benefits, and potential financial losses when patients switch employers or enroll in different health plans. These challenges present opportunities to improve the predictability of outcomes and costs, affordability for patients and the health care system, and accessibility for patients.

This market is expected to grow significantly, with hundreds of new product and indication approvals expected in the United States by 2030.¹ Included in this category are cell therapy products, such as cellular immunotherapies, cancer vaccines, and other types of both autologous and allogeneic cells for certain therapeutic indications, including hematopoietic stem cells and adult and embryonic stem cells.² Cell therapy treatments are used for blood cancers, including lymphomas, some forms of leukemia, and, most recently, multiple myeloma. An example of this type of therapy are chimeric antigen receptor T-cell (CAR-T) treatments that involve harvesting patient-specific T-cells and re-engineering them in a lab before infusing them back into the patient.³ Also included in this category are human gene therapies, which work by manipulating the expression of a gene to alter the biological properties of living cells for therapeutic use.² Examples of this include treatments for hemophilia, bladder cancer, and beta-thalassemia.

Current payment models and financial tools, such as stop-loss or reinsurance, as well as emerging models like value-based solutions and newer financial payment models (subscription models, warranties, or annuities) offer potential solutions to address these challenges by aiding with risk mitigation and affordability concerns. Though numerous current, emerging, and new solutions exist throughout the health care industry, they generally fall within four broad categories: 1) insurance options to cover costs specifically related to the therapy, such as reinsurance, stop-loss, or risk carve-out, 2) third-party negotiation services that support the contracts associated with cell and gene therapies, as well as related data management, 3) services offered to assist payers in their negotiation with providers, including access to Centers of Excellence (COEs) for a specific condition, and 4) payment plans, with or without performance guarantees and warranties, available to provide protection to payers for suboptimal product performance.⁴ The results of a survey published in 2021 that asked payers, providers, and employers about their experiences affording the cost of orphan drugs found that payers and employers rely primarily on their existing partnerships to execute their financial models. Additionally, stop-loss insurance was the most favored model for managing high-investment medications, such as most orphan drugs.⁵

Methods

AMCP held a multi-stakeholder Partnership Forum in Arlington, Va., on April 26 and 27, 2022, to explore the ability of alternative payment models, financial tools, and policy solutions to improve the predictability, affordability, and accessibility of high-investment treatments. The goal of this forum was to find solutions for ensuring that patients requiring high-investment treatments have access to the medications they need at an affordable cost. This group of 31 stakeholders included representatives from pharmacy benefit management, health plans, health systems, biopharmaceutical industry, consulting, Medicaid administration, employer coalitions, and patient advocacy organizations. In addition, individuals with expertise in rare disease management and actuarial services attended.

Forum participants were tasked with three objectives: 1) identifying stakeholder challenges associated with high-investment medications, 2) exploring the challenges and opportunities related to financial tools to address predictability, affordability, and accessibility for high-investment medications, and 3) determining the challenges and opportunities of potential policy solutions to improve the predictability, affordability, and accessibility of high-investment treatments. To accomplish this, they reviewed findings from expert speakers and a pre-forum survey, engaged in panel sessions, participated in breakout groups, and discussed the opportunities and challenges for stakeholders to engage with new financial tools and policy solutions. These proceedings synthesize the discussion from the two days; however, they should not be construed as consensus nor the perspective of individual participants' organizations.

Results

Stakeholders' Challenges and Opportunities

During the discussion, participants highlighted challenges and opportunities for accessing and paying for high-investment medications across all stakeholders. Forum participants felt high-investment medications will likely

raise sustainability and affordability concerns in the coming years. Participants also highlighted challenges related to access, such as variation across states, especially if treatment at Centers of Excellence (COEs) is required. Among specific stakeholders, participants suggested that state Medicaid plans and employer groups are likely to have the most urgent need to address the predictability, affordability, and accessibility of these medications.

Several challenges for Medicaid plans were identified by participants and included their need to balance a fixed budget with competing budget priorities, siloed decision making, political environments, and difficulties in long-term enrollment and access to care that make treatment follow-up difficult. Challenges for employer groups include actuarial risk for self-insured plans, issues with visibility into data and accurate forecasting, and reliance on the transparency of their vendor partners to guide the selection of benefits. Some other challenges faced by employer groups that participants indicated were a lack of agility with outdated benefit design models, over-reliance on rebates, and limited expertise regarding the application of newer, more innovative payment and coverage solutions.

However, participants noted that all payers have significant incentives to overcome these challenges and doing so presents opportunities from which all stakeholders might benefit. For Medicaid, forum participants underscored the potential of these therapies to restore health, thereby reducing resource utilization. For employer groups, these therapies were noted for their potential to improve workforce resilience and offset future medical costs.

Stakeholders prioritize predictability, affordability, and accessibility in different ways.

Outlined in Figure 1 are the differences in how stakeholders describe the importance of predictability, affordability, and accessibility for high-investment medications. Given these differences, it is not surprising that existing financial tools address some but not all stakeholder needs. Table 1 explains the different tools and how participants of the forum ranked the potential impact each tool may have on predictability, affordability, and accessibility. Recently, stop-loss risk management strategies have been increasingly popular due to their ability to reduce risk and financial exposure to unexpected and rare high-cost events. One panelist noted the stop-loss carrier market has observed the spread of financial risk across a broader pool of payers, doubling since 2014. However, as new high-investment medications transition from unexpected to predictable costs, stop-loss policies and reinsurance become less appropriate options. Instead, value-based payment and outcome-based contracts may better address scenarios when treatment effects are more predictable, rendering stop-loss insurance unnecessary. However, as an emerging tool, value-based payment options may increase rather than decrease costs to the system.

Figure 1. Stakeholder perspectives of importance on predictability, affordability, accessibility for high-investment medications

| | <u>Predictability</u> | <u>Affordability</u> | <u>Accessibility</u> |
|--|-----------------------|----------------------|----------------------|
|  Patients | Medium | High | High |
| Employers and unions  | High | High | Medium |
|  Health systems | Medium-High | Medium | Medium-High |
| Medicaid  | Medium-High | High | Medium |
|  Medicare | High | Medium | Medium |
| Regional Commercial plans  | High | High | Medium-High |
|  National commercial plans | Low-Medium | Medium | Low-Medium |
| Pharmacy benefit managers  | Low-Medium | Low | Medium |

Table 1. Financial Tool Terms and Definitions

| Term | Description | Participant Rating of Potential Impact | | |
|---|--|--|---------------|---------------|
| | | Predictability | Affordability | Accessibility |
| Risk management strategies | | | | |
| Orphan reinsurer and benefit manager (ORBM) | A risk pooling solution to manage actuarial risk and execution challenges, including contracting, reimbursement, and care coordination. | High | Medium | High |
| Risk carve-out solutions | A benefit in which the payer delegates coverage, management, and coordination for treatment to a third party for a premium. | High | Medium-High | Medium-High |
| Stop-loss | A third party protects the employer against shock claims (high-dollar, low-frequency events) for an annual premium. | High | Medium-High | Medium-High |
| Value based solutions | | | | |
| Annuity-based contract with outcomes | A multi-year agreement that ties reimbursement installments for a medical intervention or manufactured product to predetermined outcomes or cost measures and monitors these measures over a defined period. | High | Medium-High | Medium-High |
| Annuity-based contract over time | A multi-year contract that ties a biopharmaceutical manufacturer's reimbursement to installments at specified intervals, spreading the cost over time. Installment payments are independent of meeting outcome or cost benchmarks. | Low-Medium | Low-Medium | Medium |
| Milestone-based contract | A type of performance-based contract in which a manufacturer guarantees to refund the cost of therapy (partially or fully) to the payer if an agreed milestone or outcome is not achieved. | Medium | Medium-High | Medium-High |
| Outcome-based contract | A contract between a payer, manufacturer, and/or health care provider that links payment for a treatment that meets, exceeds, or fails to meet expected patient health measures or other real-world outcomes over a defined period. | Low | Medium | Medium |
| Outcomes-based rebate | A contract between a payer, manufacturer, and/or health care provider where the payer pays the full price of the drug upfront but receives a rebate later if the drug fails to meet expected patient health measures or other real-world outcomes. | Low | Medium-High | Medium-High |

Table 1. Financial Tool Terms and Definitions (Continued)

| Term | Description | Participant Rating of Potential Impact | | |
|--|---|--|--------|-------------|
| Subscription contract | A payment contract that provides a medical intervention or a manufacturer's product for a set fee to treat a certain proportion of patients or a set price per patient. | High | Medium | High |
| Warranty contract | A policy (typically through a third-party insurer) that reimburses treatment-related costs for suboptimal performance. | Low | High | Medium |
| Contract negotiation services | | | | |
| Contract negotiation/management | A third-party negotiation of contracts for treatments that provides data and outcomes tracking. Contracts may include a performance guarantee, but the third party or service company does not assume financial risk. | Low | Medium | Medium-High |
| Provider contract negotiation services | A negotiation that offers multiple services for providers and patient care pathways, including Centers of Excellence (COEs) network creation and contract, data analytics, and cost containment. | Medium | High | High |

Uptake of new and innovative payment models has been modest but growing.

Participants conveyed the overall impression that uptake of innovative payment models has been slow although data indicate that many organizations are considering employing them. As part of this consideration, participants stressed the need to balance the use of existing tools, such as stop-loss policies, with affordability and access. Additionally, participants emphasized the need to explore alternatives when current models are insufficient. Novel tools and policies may also provide emerging options in providing affordability and access in certain therapeutic areas by helping payers mitigate some of the financial uncertainty.

Proactive preparation for high-investment medications is key.

Participants emphasized that making incremental changes to prepare for the future impact of high-investment medications is key. This may include improving data infrastructure to enable pilot programs to explore the potential of emerging financial tools. Data-related improvements discussed by participants included more robust collection of patient-reported outcomes data, better utilization and analysis of real-world evidence, and the aggregation of data on relevant, meaningful outcomes. State Medicaid plans as well as employer groups were discussed extensively as potential pilot participants gave their position to create meaningful benefit design changes to support coverage of high-investment medications.

Opportunities to manage high investment medications.

Table 2 includes potential emerging policy solutions along with forum participants' rating of the solution's potential impact on predictability, affordability, and accessibility.

Table 2. Potential Emerging Policy Solutions

| Solution | Description | Participant Rating of Potential Impact | | |
|---|---|--|---------------|---------------|
| | | Predictability | Affordability | Accessibility |
| National high-investment medication benefit ⁶ | Offering coverage outside of the MDRP in which the federal government would purchase certain high-cost products, such as cell and gene therapies. | High | Medium | High |
| High-investment medication risk pools ⁶ | Creating multistate or national purchasing pool for high-cost drugs that treat small patient populations. | High | Medium | Medium |
| Patient portability credits | Providing credit to the initial payer who covers a durable/curative therapy when the treated patient switches coverage to another payer. | Low | Medium-High | Medium |
| Alternative calculation of Medicaid Best Price ⁷ | Updating the current Medicaid Best Price calculation to prevent artificially low best prices that may result from VBAs. | Inconclusive | Inconclusive | Inconclusive |
| Patient incentives for disease registry engagement | Providing coinsurance discounts or other incentives to patients for engagement in a disease registry. | Low | Medium-High | Medium-High |
| Mandatory value-based contract | Requiring a value-based contract be in place for drugs priced above a particular threshold. | Low | High | High |
| Regulations to enable state pilots ⁸ | Updating state or federal legislation, such as the Antikickback Statute, which may limit state Medicaid plans from engaging in innovative pilots. | Low | Medium | Medium |

COE – Centers of Excellence; MDRP – Medicaid drug rebate program; VBA – value-based agreement/arrangement

Enhance data collection

Initiatives to enhance the collection, sharing, and utilization of data related to the safety and outcomes of high-investment medications were seen by participants as necessary. It was noted that the enhanced collection could be accomplished using a patient global consent, thus allowing for a comprehensive capture of patient data.

Standardize terminology and promote education to raise awareness of emerging financial models and policy solutions.

Participants urged development of standardized terminology and education across stakeholders to provide awareness of emerging models and potential solutions. Particularly, the participants felt clarification of nuances in the financial tools and better definitions of the parameters of the potential policy solutions are required. For example, there were challenges differentiating between annuity-based, milestone-based, and outcome-based arrangements. Additionally, many questions arose related to a potential federally funded high-investment medication benefit: Would it cover only the cost of the drug? Would ancillary services be covered as well? Questions also arose related to the use of patient portability credits: Who would fund the credit? How would the amount be determined and paid out? These questions will require additional research and should be included in future forums.

Tailoring the use of multiple tools

Participants recognized that all stakeholders value predictability, affordability, and accessibility but from different perspectives. Therefore, multiple financial tools and policy solutions are needed. This may include updates to existing tools and policies and development of new solutions. For instance, participants noted that traditional stop-loss and reinsurance programs may offer adequate risk management in certain therapeutic disease areas; newer strategies such as risk-carve out, orphan drug specific reinsurance, and benefit management solutions may be better in other disease states. In therapeutic areas poorly managed with currently used tools and solutions, newer strategies would be needed to address affordability, accessibility, and predictability. A policy-related example offered by participants was a recent change in how to calculate Medicaid Best Price. This change in methodology has the potential to remove a barrier when it comes to participating in value-based agreements. Continuous enhancement of regulations would enable state Medicaid plans to engage in testing innovative value-based tools in pilots.

Participants also recommended tools and policies be tailored and used together so as to more completely address predictability, affordability, and accessibility. State and federal risk pools or a national high-investment drug benefit program may address the predictability of costs with respect to patient portability, for example, while value-based contracts are assisting with the predictability of costs by relating payments to treatment response and medication durability. Another point discussed by participants was the potential policy solution of mandatory value-based contract for drugs priced above a particular threshold. Participants suggested caution as this requirement may be inappropriate for all high-investment medications.

Facilitate partnering and innovation across stakeholders.

Participants emphasized that patient engagement is crucial to improving and maintaining the predictability, affordability, and accessibility of high-investment medications. Though some patients may be highly motivated to engage in ongoing data collection, there are patients for whom potential challenges must first be overcome. Many of the patient challenges identified by participants were related to the fear of the unknown. Examples included fears the therapy will fail, concerns related to adjusting to a new normal following treatment, decreased trust in the medical system, concerns about the complexities with obtaining and receiving the medication, and worries about affording the out-of-pocket costs.

Simplifying and creating efficiencies throughout the care continuum to make access to these medications easier for patients may mitigate these challenges and increase patient engagement. As providers and patient advocacy groups are particularly influential with patients, participants thought these stakeholders might be among those best positioned to be leaders in the development of patient engagement solutions.

Participants also supported partnering and innovating between stakeholders. One suggestion was to encourage state health authorities to design Medicaid pilot programs for the emerging financial tools and policy solutions. They also proposed that regional health plans might be more open to explore collaborating with stakeholders on emerging solutions as they are often more agile than larger, national organizations. To achieve success, however, participants acknowledged the necessity of broadening stakeholder discussions to grasp different stakeholder viewpoints and identify areas where solutions can be applied, incentives can be aligned, and transparency can be enhanced to foster trust.

Future Directions

For all stakeholders, participants emphasized the importance of being proactive in planning how they will manage high-investment medications as the number and cost of high-investment medications continues to grow. Also, to improve the predictability, affordability, and accessibility of these innovative medications and technologies, participants view patient engagement and increased collaboration across stakeholders as key.

Future activities considered include continuing to engage all stakeholders on the topic of high-investment medications and the need for educational materials regarding the financial tools discussed throughout this Partnership Forum. Stakeholders should continue to explore opportunities for the private and public sector to permit, test, and encourage new and emerging financial tools and policy solutions to meet the market need.

Limitations

Limitations of this Partnership Forum included that a different mix of stakeholders or individuals might have resulted in a different set of key topics identified. Additionally, the breakout discussions considered stakeholder priorities for predictability, affordability, and accessibility and the ability for certain financial tools to address these. A different mix of stakeholders may have resulted in differences in impact ratings. The results relied on the assumptions of the broader groups of stakeholders. For each of the policy solutions, various caveats and nuances suggested that careful considerations and flexibility would be needed rather than applying a one-size-fits-all approach.

Conclusion

The often short-term, high investment required for innovative medication technologies that offer potentially life-changing therapeutic advances or cures raises sustainability, value, and affordability concerns for employers, payers, and patients. Key points that emerged from this forum discussion include that while the number and cost of high-investment medications is growing, the uptake of innovative payment strategies has been slow. However, making incremental changes to prepare for the future impact of high-investment medications is important. Opportunities to manage these technologies include improving data collection, standardizing terminology, providing education on new financial tools and policy solutions, and promoting stakeholder collaboration and trust.

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