Duke
MARGOLIS CENTER for Health Policy

February 2023

Performance Measurement Strategies in Value-Based Payment Arrangements

WHITE PAPER

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KEY TAKEAWAYS

- Value-based payment (VBP) arrangements offer an opportunity to facilitate access to innovative medical products while collecting the long-term data necessary to demonstrate durable clinical benefit of these products.
- Partnerships between payer organizations, providers, and manufacturers can foster greater use of centralized registries to capture long term data. These partnerships can mitigate challenges of patient portability and data infrastructure that currently limit the scope and scale of VBP arrangements.
- Advanced performance measurement strategies for VBP arrangements should consider broader agreed-upon dimensions of value to manufacturers and payers, including overall clinical impact, how multi-year benefits should be quantified, and how to easily build and implement new outcome measures.

INTRODUCTION

Advances in biomedical innovation and expanded regulatory pathways have resulted in the proliferation of transformative therapies that have the potential to halt or reverse disease states and significantly improve a patient's overall health.[1] Transformative therapies are interventions that are designed to provide a durable response for disease complications, halt disease progression, or restore functionality for patients with conditions for which there are no comparable or available interventions. These therapies promote a shift away from traditional episodic treatments towards long-term disease management. This category of therapy includes specialty drugs, small molecule agents, and cell and gene therapies. To date, most transformative therapies have high upfront costs. Furthermore, as these therapies are fairly new (the first cell or gene therapy was approved in 2017)[2], they may have outstanding evidentiary questions about their long-term durability. For stakeholders including physicians, payers, and patients, these questions lead to uncertainty regarding the overall value of such therapies. Moreover, the combination of high short-term costs and long-term evidence questions presents a challenge for payers as they evaluate coverage and payment options for such products. These struggles may delay the use of these transformative therapies, which can further delay the development of additional evidence to inform the long-term value of such therapies.

Determining the value of these transformative therapies will require support for more thorough long-term evidence generation. Due to the increasing number of transformative therapies expected to enter the market in

the near term and questions around their value[3], stakeholders have underscored the need for policy solutions to facilitate long-term data collection and promote payment structures that balance patient access with uncertainties around the product's value. Value-based payment (VBP) arrangements have emerged as one strategy to address these evidentiary and access issues. A VBP arrangement is a contracting agreement between stakeholders where payment is tied to performance measures that act as proxies for improved health status or outcomes. These arrangements promote alignment of cost with the value of a product by supporting further evidence generation, ensuring appropriate use of products, and encouraging downstream product and payment refinements, all while facilitating patient access to transformative therapies.[4]

Evidence to-date suggests modest adoption of VBP arrangements by payers and manufacturers. In a Duke-Margolis study, manufacturers and payers reported to have implemented 88 to 122 models, respectively, between 2014 and 2017.[5] Other surveys suggest that more than 50% of payers have at least one VBP arrangements in place, with around 10% of payers have instituted more than 10 arrangements.[6] Because of the proprietary nature of these arrangements, much of the information about these arrangements is not publicly available.

VBP arrangements can be difficult to negotiate and implement and require alignment on a number of parameters, including therapy performance strategies and data infrastructure. It is common for payers and manufacturers to fail to come to an agreement, despite interest from both parties. In previous Duke-Margolis research, only one to two-thirds of early dialogue among stakeholders led to VBP implementation. Challenges can include:

1. *Stakeholder disagreement over the outcome measure(s) that most appropriately capture a treatment's intended benefit*. Payers and manufacturers may differ in their preferences for performance measures. Manufacturers focus on measuring the clinical trial endpoints with which the therapy reached FDA approval. In contrast, payers are interested in other domains such as treatment impact on health care utilization, functional status, and direct measures of clinical benefit. Misalignment on performance measurement strategies have been found as the leading cause of negotiation failure between manufacturers and payers, with manufacturers reporting that approximately two-thirds of all feasibility discussions ended without a contract for a VBP model.[6]

2. *Stakeholder disagreement over the methods or data sources from which to collect outcome measures*. Payers and manufacturers may differ on the methods to calculate an outcome measure even if there is conceptual alignment on the outcome itself. For example, payers generally only have access to medical or pharmacy claims data and may not have access to other sources of clinical data such as electronic health records (EHRs). In contrast, manufacturers expressed a preference for using clinical outcomes measures as indicators for treatment effects aligned with clinical studies, which may not be available in claims data.

3. *Longitudinal outcome data tracking.* Many transformative therapies are developed with the intention to have multi-year or lifelong benefits, and the anticipated duration of benefits may exceed the time a patient is under a specific health plan. VBP arrangements may be constructed for a shorter duration than the time length that captures all possible treatment effects for a given condition.

Policies Promoting Value-Based Payment Arrangements for Transformative Therapies

Despite the stated challenges in designing VBP arrangements, support for these types of arrangements as well as value-based care delivery models are growing on the national scale. In its report to the White House Competition Council, the U.S. Department of Health and Human Services (HHS) identified tying payments to clinical value in Medicare Part B as an "administrative lever" to address gaps between a product's efficacy and price.[7] HHS also more broadly stated that other federal authorities, such as those that allow the department to test payment models through the Centers for Medicare and Medicaid Services (CMS) Innovation Center, may be potential levers to facilitate linking payments to observed benefits. In October 2021, the Innovation Center published a white paper reflecting on the key lessons learned from experimenting with VBP arrangements over the past decade.[8] Based on these lessons, CMS is launching a reframed strategy for managing total cost of care, including the cost of medical products such as drugs and therapeutics. The Innovation Center plans to spend the next decade working with external stakeholders to test innovative VBP arrangements designed to lower beneficiary spending on drugs, incentivize use of biosimilars and generics, align stakeholder incentives, and streamline payment processes.

Another effort to facilitate the adoption of VBP arrangements includes a new CMS policy to support greater flexibility for stakeholders when entering into value-based purchasing negotiations in the private markets.[9] Previously, under the Medicaid Best Price (MBP) rule, manufacturers were required to offer Medicaid organizations the lowest price for a drug or therapeutic.[10] An unintended consequence of this rule was that it created a disincentive for manufacturers to engage in VBP arrangements that could potentially lower and reset the "best price" of a drug or therapeutic. In July 2022 the Centers for Medicare and Medicaid Services (CMS) implemented the new Medicaid multiple best prices policy[9] (MBPP) which allows manufacturers to report multiple Medicaid best prices based on VBP arrangements offered to private payers as well as Medicaid programs. The new rule offers manufacturers greater flexibility to engage in VBP arrangements without assuming the additional financial risk of resetting the Medicaid best price.

Study Objective

The objective of our study was to examine the performance measurement strategies used by payers and manufacturers in the design of current VBP arrangements for transformative therapies. We assessed stakeholders' impressions of whether current measurement strategies are adequate to track product benefits over a longer duration of time.

METHODS

Study Participants

A purposive sampling approach was used to gather broad perspectives from organizations representing three types of stakeholders. All organizations were selected because they are part of a large, multi-stakeholder consortium focused on sharing best practices and advancing research related to VBP arrangements for medical technologies. A total of 16 organizations were selected for outreach: six private payers, seven manufacturers, and three integrated health systems. We targeted manufacturer and private payer perspectives as VBP arrangements have primarily been implemented between these two stakeholder groups. We also included integrated health systems to understand how access to multiple data sources may impact performance measurement strategies.

We conducted outreach to organizational representatives that were involved in providing strategic guidance on coverage and access policies for new technologies, serving as health economic and outcomes research experts, or are directly involved in the negotiation of VBP arrangements. Semi-structured hour-long interviews were conducted via voice and video platforms. Study participants were not compensated for their participation.

Data Collection and Analysis

The interview questions were informed by a review of the literature and expert opinions around VBP arrangements and performance measures. Interview questions corresponded to two main domains: 1) general experience with VBP arrangements and 2) existing performance measurement strategies. After each interview, two trained researchers separately analyzed interviews to identify major themes. The research team then collated the key themes from each interview across stakeholder type in order to maintain anonymity of individual respondents.

RESULTS

Respondent Characteristics

Of the 16 stakeholders invited to participate, ten completed interviews: four manufacturers, three payers, and three integrated health systems. Of the manufacturers, two were from large firms (more than 10,000 employees and annual revenues greater than $15 billion) and two were from small firms (less than 4,999 employees and under $2 billion in annual revenue). Two of the payer organizations were regional, covering around three million members each, and one of the payer organization was national, covering around 15 million individuals. The three integrated health systems have between one to 12 million members in different geographic regions. Interviewees were senior leaders in business strategy, pharmacy and drug evaluation, medical and regulatory policy (US and global), and pricing.

General VBP Experience

All stakeholders stated that they engaged in feasibility discussions to design and implement a VBP arrangement. The manufacturers interviewed had moderate experience with VBP arrangements. Two manufacturers had implemented several arrangements, and all others have participated in early feasibility discussions. The interviewed payers had collectively the most experience and had implemented the highest number of VBP arrangements. The number of VBP arrangements reported by two of the payers was two to three times that reported by manufacturers. One payer indicated they had multiple VBP arrangements for the same product for different indicated populations. The integrated health systems had implemented the fewest number of VBP arrangements. Of all stakeholders interviewed, six shared that they have not implemented a VBP arrangement: one payer, two manufacturers, and all three integrated health systems. Instead, each of these same respondents reached an agreement with the manufacturer on a net price for a product given the uncertainty about its long-term effectiveness.

Measurement Strategies

The most commonly used performance measures were claims-based measures (e.g. emergency department visits, hospitalizations, total cost of care) and more process-oriented measures (such as treatment discontinuation). All stakeholders reported having experience with these measures. Claims-based measures are

better suited to determine discrete event occurrence and to capture changes in utilization trends. These measures are less suited for assessing direct clinical outcomes.

All stakeholders expressed a preference to move beyond the current measurement landscape to measures that estimate a change in overall disease burden over time, as this aligns with the anticipated benefits of many transformative therapies. Stakeholders expressed interest in exploring how to quickly develop and deploy condition-specific, patient-reported or functional status measures. Stakeholders commented that their ideal data infrastructure capabilities would include data access shared between providers and payers, limited reporting burden for providers and patients, and greater alignment on the outcome measures used.

DISCUSSION

Evidence Goals by Stakeholder | Outcomes of Interest

The interviews suggest that stakeholders' respective goals for evidence generation inform how they substantiate the value of a transformative therapy. Most manufacturers described a preference for a sequential evidence generation process with an initial focus on measures of safety and effectiveness to support regulatory approval. Payers and integrated health systems expressed greater interest in evidence of treatment durability, overall health outcomes, total cost of care, and impact on healthcare utilization. Thus, while VBP arrangements can serve to increase access to costly transformative therapies while also generating evidence on long-term clinical benefit, the varying evidentiary goals among stakeholders have proven a challenge for alignment on outcomes upon which to base the arrangement. For example, manufacturers expressed concerns over being held accountable to measures that were not required for FDA approval without the opportunity to first study these outcomes. Some manufacturers also expressed concern about focusing the performance measurement strategy for a product on the impact on utilization or total cost of care, especially in conditions where the measures may not be indicative of benefit to the patient. For instance, some transformative therapies have positive impacts on functional status or sensory conditions, such as restoration of sight or hearing, that may not affect the patient's overall level of health care utilization.

All stakeholders expressed a preference for greater use of endpoints that are a direct measure of clinical benefit as the basis for VBP arrangements. A number of transformative therapies have received expedited FDA approval in which evidence of effectiveness is inferred through use of surrogate endpoints, or proxy measures, rather than measures of direct clinical benefit. The use of surrogate endpoints is in part due to known challenges with the use of traditional clinical trial designs to study products for diseases with small patient populations, or products for diseases with long timescales of progression. These surrogate measures often are biomarkers or other lab values that may not be readily available outside of EHR records or that may not be captured during the course of routine care. Thus, while surrogate endpoints selected for the clinical trial are useful for generating the evidence required for regulatory approval under the accelerated pathway, such measures may not be considered outcomes of interest for payers nor be conducive to supporting VBP arrangements. Treatment of the broader patient population and longer-term data collection that occurs over the course of a VBP arrangement offers opportunities to leverage other measures of clinical benefit that may not have been feasible to collect during the course of clinical trials.

Finally, there are different perceptions both within and across stakeholder groups about what types of performance measures can legally be used in VBP arrangements. Some stakeholders stated that based on their understanding of the False Claims Act[11] that outlines acceptable communication, manufacturers can design a VBP arrangement on the exact outcomes listed on the FDA label. Other stakeholders seemed to suggest a different understanding of regulations, whereby manufacturers and payers can link payment to agreed-upon outcomes. These differing interpretations of what is permissible in the current regulatory structure can impede payers and manufacturers from experimenting with implementing a broader range of performance strategies. Further clarification from CMS on what types of performance measures are permissible would help mitigate these challenges.

Patient Portability

A key challenge raised in almost all interviews was patient portability. For payers, a patient leaving the health plan can negate a manufacturer's overall obligation to that payer in a VBP arrangement. Stakeholders are affected by the challenges of seeking to measure longer-term, multi-year clinical benefits that require regular interaction with the health care system to produce accurate estimates. Payers may not be keen on a VBP arrangement when the outcome timespan is expected to exceed the average duration a patient is covered in a health plan, as payers may bear the upfront costs while being unable to capture the long-term benefits of these treatments. As a means to mitigate the lost costs to plan attrition, some payers indicated that they may enter into agreements with providers directly for specific disease areas with small patient populations.[12]

Integrated health systems were the least impacted by patient portability. The general length of coverage for their beneficiaries tends to be longer than national or local market averages, allowing them to more easily capture multi-year benefits of new technologies even if there are high initial upfront costs. Further, the close relationship between providers and patients over several years and payer and provider business extensions make integrated health systems more capable of tracking patient care over time, even if some of it is received outside the health system.

Administrative Burden

Another challenge raised in all interviews with payers and manufacturers was the administrative burden to maintaining a VBP arrangement. A VBP arrangement requires the payer (or integrated health system) and the manufacturer to review patient-level data per the terms of the risk-based agreement. For the payer, this will involve timely reviews of claims data and other data sources as needed. For manufacturers this will involve reviewing the data received and issuing payments as applicable. Occasionally, measurement strategies that include patient-reported outcomes will involve additional data collection by providers which can also incur additional costs for payers. Consequently, although there was a general preference of moving beyond process-oriented measures to outcomes-based measures, stakeholders also expressed concern that an increased use of clinical, patient-reported or functional status measures would place additional burden on providers, patients, and caregivers. Further, any new provider reporting activities may require financial support. All stakeholders expressed the desire to minimize any additional reporting burden on providers, patients, and caregivers and often did not use many measures that required additional follow-up or collection of data that would not otherwise be collected during routine patient care.

Stakeholders expressed that the administrative burden of maintaining a VBP arrangement impacts the marginal cost of implementing an additional VBP agreement. For example, using multiple data types adds to the costs of developing, tracking, and reporting data in a given arrangement. Specifically, the costs of new data acquisition may exceed the benefits of participating in the contract. Some stakeholders reported attempting to steer transformative therapy patients to specialized centers in order to minimize the risk of not being able to access clinical data at other sites of care. These challenges are further compounded by the lack of infrastructure to collect and store data and the lack of personnel and resources to analyze the data properly. Consequently, stakeholders can struggle to derive reliable estimates of performance for functional status or patient-reported outcomes. Payers expressed that even extracting clinical data in cases where they easily have access to the underlying records adds to increased overhead costs. Finally, stakeholders reported that the VBP negotiation process itself can be resource intensive. Stakeholders shared that negotiation processes can take months or years and require input from data analysts and legal teams.

Data Governance | Third-Party Tracking

Given the administrative burdens and resource intensities of both negotiating and implementing a VBP arrangement, there was some interest across stakeholders for a third-party contracted set of services managing operations, including data tracking. This third party would function as a data aggregator that could mitigate the key challenges identified, namely access to specific outcomes of interest, patient portability, and administrative burden. First, a data aggregator could be contracted to access data that may not be readily available in payer claims data, such as EHR data and clinical registry data for example. Access to more sources of data could then allow for more advanced performance measurement strategies, beyond the process and utilization measures that can be easily derived from payer claims data. As part of this agreement, stakeholders expressed interest in establishing governance standards that would allow for equal access and audits on the collected data. Second, data aggregators could address patient portability by following patients across health plans, which would give payers more certainty that a VBP arrangement will be completed and not disrupted earlier due to patient attrition. Third, as the data aggregator would be the entity tracking outcomes, neither payers nor manufacturers would have the added administrative burden of tracking patient level data and reporting it. The only significant costs for the arrangement would be the negotiation process, which could presumably also be offset by the data aggregator if they are able to facilitate negotiation. Ideally, with multiple arrangements, manufacturers and payers would receive more favorable prices with a third-party data tracking and outcome aggregators.

POLICY RECOMMENDATIONS

Although stakeholders stated challenges in designing VBP arrangements, they still continue to engage in these types of arrangements as well as value-based care delivery models. Interviewees identified several approaches that could facilitate greater adoption of VBP arrangements. Recommendations range from greater clarity from CMS to expanded data infrastructure to support tracking long-term outcomes. Provisions in the new MBPP include more transparency on the type of VBP arrangements available. Under this policy, manufacturers are required to report to CMS on the VBP arrangements they maintain, including information such as the disease area, therapeutic type, and outcomes captured. This information by itself will be informative for stakeholders to understand what type of VBP arrangements are feasible.

There are additional policies that could further support the development of VBP arrangements.

Evidence Thresholds for Disease Areas

CMS as well as other payers can inform evidence thresholds for disease areas that can then inform clinical trial strategies for manufacturers. A defined evidence threshold will also allow CMS and payers to develop standard criteria or best practices for measure development, such as the use of different data sources, the definition of benchmarks for small populations, and common data elements used in assessing disease progression. If payers articulate evidence thresholds or even outcomes of interest specific to disease areas, manufactures have more information to inform their evidence generation strategy and can better facilitate patient access to new therapies.

Greater Stakeholder Engagement Prior to FDA Approval

One of the most notable findings from the interviews was the extent to which the different evidentiary goals between the FDA and payers can limit VBP arrangements. The evidence generation strategy for manufactures is largely driven by FDA requirements for regulatory approval and may not be aligned with payers' outcomes of interest. Similar to the concerted collaboration with the FDA, manufacturers could engage with payers during the clinical trial development process to ensure payer outcomes of interest are captured in the pivotal studies. Engaging with payers early in the evidence generation process will support earlier payer valuation of the therapy and facilitate increased patient access as well as VBP arrangement implementation.

Greater Use of Registries to Support Long-Term Data Collection

For payers and manufacturers, lack of a broad data infrastructure and patient portability were the most significant deterrents to VBP arrangements. National registries for a disease area were seen as an effective approach to mitigate these issues by providing data infrastructure independent of payer coverage. Further, it would allow comparisons of interventions which could broaden the scope of VBP arrangement options for a given therapy. This mechanism would require coordinating with providers to capture patient level data. A national registry would be preferred over manufacturer registry as it would leverage an existing data infrastructure. Manufacturers felt that it was difficult to expand registries in this way without additional cost and have looked to expand registries they maintain or link clinical information they own to national registries. The manufacturers noted how in other contexts, such as the UK or countries in the EU, there are national databases of patient data linked at the individual level, allowing significantly easier reporting of longitudinal outcomes with greater precision than is often possible in the United States.

An alternative to a single national registry is to expand the use of decentralized data networks. The federal government has supported variations on real-world evidence reporting models, including FDA's Sentinel and NEST systems and PCORnet. A single decentralized data network that can facilitate outcomes reporting for multiple therapies may be an appropriate platform to support performance data reporting that may be held at different sites.

As more stakeholders engage with VBP arrangements there will be a need for more sophisticated capabilities for outcome tracking. The success and ongoing adoption of VBP arrangements depends upon availability and use of measures that can meaningfully assess performance of therapies in payers' covered populations.

STUDY LIMITATIONS

From our limited sample, it is difficult to conclusively point to causal factors that may be driving the overall trends in VBP arrangement design and implementation. Our sample was selected based on historical engagement in VBP arrangements and participation in a multi-stakeholder group focused on sharing best practices about these arrangements. The sample therefore reflects the range of organizations that are already committed to pursuing VBP arrangements as a strategy for coverage and payment for transformational therapies. Further research should involve organizations that are not currently planning to implement VBP arrangements, or that may not be operationally equipped to implement VBP arrangements to gauge other challenges and opportunities for increasing VBP arrangement adoption.

CONCLUSION

Developing performance measures to capture relatively novel long-term clinical benefits of transformational therapies is challenging. It is more challenging when these measures must also be suited for determining payment in VBP arrangements. Despite the challenges in designing VBP arrangements, support for these types of arrangements as well as value-based care delivery models are growing on the national scale. Performance measurement for transformative therapies should capture broader agreed-upon dimensions of value to manufacturers and payers, including overall clinical impact, how multi-year benefits should be quantified, and how to easily build and implement new outcome measures. As the number of transformative therapies continues to increase, precise measurement and evaluation of these therapies in practice should continue to evolve as well.

REFERENCES

1. H.R.34 - 114th Congress (2015-2016): 21st Century Cures Act.
2. FDA approval brings first gene therapy to the United States. U.S. Food and Drug Administration; 2017.
3. Martin K. Reducing Spending on Prescription Drugs with Limited Clinical Evidence. To The Point. Commonwealth Fund2021.
4. Lopez MH, Daniel GW, Fiore NC, Higgins A, McClellan MB. Paying For Value From Costly Medical Technologies: A Framework For Applying Value-Based Payment Reforms. Health Aff (Millwood) 2020;39(6):1018-1025. DOI: 10.1377/hlthaff.2019.00771.
5. Mahendraratnam N, Sorenson C, Richardson E, et al. Value-based arrangements may be more prevalent than assumed. Am J Manag Care 2019;25(2):70-76. (https://www.ncbi.nlm.nih.gov/pubmed/30763037).
6. McCarthy KF, Cricchi L, Shvets E, Santiesteban D. Avalere Survey: Over Half of Health Plans Use Outcomes-Based Contracts. Avalere. Nov 4, 2021 (https://avalere.com/insights/avalere-survey-over-half-of-health-plans-use-outcomes-based-contracts).
7. Comprehensive Plan for Addressing High Drug Prices: A Report in Response to the Executive Order on Competition in the American Economy. September 2021. . In: Office of the Assistant Secretary for Planning and Evaluation USDoHHS, ed.2021.
8. Innovation Center Strategy Refresh. In: Centers for Medicare and Medicaid Services UDoHaHS, ed.2021.
9. Centers for Medicare and Medicaid Services UDoHaHS. Relating to Manufacturer Reporting of Multiple Best Prices Connected to a Value Based Purchasing Arrangement; Delay of Inclusion of Territories in

Definition of States and United States. 2021 (https://www.federalregister.gov/documents/2021/11/19/2021-25009/medicaid-program-delay-of-effective-date-for-provision-relating-to-manufacturer-reporting-of).
10. Technical Guidance - Value-Based Purchasing (VBP) Arrangements for Drug Therapies using Multiple Best Prices. In: HHS D, ed.: Centers for Medicare & Medicaid Services; 2022.
11. : False Claims Act. 31 U.S.C. 3729 et seq. January 13, 2021.
12. Embarc Benefit Protection | Cigna. Cigna. (https://www.cigna.com/employers/cost-control/embarc-benefit-protection).

ACKNOWLEDGEMENTS

Duke-Margolis would like to thank the participants of this study from the Value for Medical Products Consortium for providing their time and expertise. The Consortium—composed of patient advocates, payers, manufacturers, and providers, as well as experts on regulatory affairs, law, and policy—is developing approaches to payment reform that support better outcomes for patients and better value across the system. We would additionally like to acknowledge the contributions of Nicholas Fiore, Hannah Graunke, and Marianne Hamilton Lopez to this project.

Funding for this work is made possible through the Value for Medical Products Consortium. For more information on the Consortium, visit https://healthpolicy.duke.edu/projects/value-medical-products-consortium.

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