Patients and the Next Generation of Value-based Arrangements: Reframing the VBA Debate Around Patient Benefit

A NEHI White Paper

NEHI Network for Excellence in Health Innovation

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Support

NEHI wishes to acknowledge the support of Eli Lilly for its VBA and patient stakes project.

Recommendations in this white paper are based on NEHI research and multi-sector expert roundtables conducted in December 2019 and April 2020. NEHI is solely responsible for findings and recommendations made in the white paper.

About NEHI

NEHI is a national nonprofit, nonpartisan organization composed of stakeholders from across all key sectors of health and health care. Its mission is to advance innovations that improve health, enhance the quality of health care, and achieve greater value for the money spent.

NEHI brings together expert stakeholder perspectives with relevant research to devise policies that speed the adoption of innovations.

Executive Summary:

Value-based arrangements (VBAs) are now seen as a "tool in the payment toolbox" for health care payers and pharmaceutical manufacturers, although a tool in limited use

VBAs are not a new tool for negotiating price, but a tool for brokering net payments that represent win-win arrangements for payers and manufacturers pursuing goals that vary from VBA to VBA.

- Examples include VBAs that link payment to replicating real world results from clinical trials; to reductions in total (medical and pharmacy) costs of care achieved through optimal use of medications; and real-world results that demonstrate the comparative effectiveness of a drug.

- Payer-manufacturer goals may vary considerably if the VBA is covering therapy for a rare disease as compared to a VBA covering a chronic disease medication.

Innovative, next generation VBAs will have greater impact if they are framed explicitly around increasing the net benefits to patients

- Next generation VBAs should be patient-focused, visible win-win-win arrangements for patients, payers, and manufacturers.

- Patient-focused VBAs are more likely to justify regulatory forbearance or exemptions that could trigger greater innovation in VBAs and offer payers and manufacturers a more compelling rationale to overcome the still-daunting operational complexities of VBAs.

- Federal agencies (HHS, CMS, OIG) should define VBAs as arrangements that not only link payments to defined outcomes, but link payments to demonstration of increased patient benefit.

To realize the full potential of next generation VBAs, federal agencies should adopt a VBA Innovation Agenda that invites payers, manufacturers and patient communities to submit VBA proposals that address –

- Serious unmet medical needs among children

- Serious unmet needs among low-income and other vulnerable populations, such as unmet needs in behavioral health

- Gaps in optimal medication use that are impeding critically needed improvements in population health, such as improvements in diabetes and behavioral health care.

Federal agencies should approve promising win-win-win proposals for demonstrations and offer support, such as forbearance on current regulatory enforcement to allow for time-limited VBA demonstration projects

Patients and the Next Generation of Value-Based Arrangements

Value-based arrangements have become an accepted method for the sale of pharmaceuticals by manufacturers to health care payers, although a method with several limitations.[1]

VBAs are not a method for setting prices; manufacturers set one list price for each drug on the market, and the list price applies whether or not the drug is sold through a VBA. VBAs must be negotiated for compliance with current federal regulations that allow payers to reach agreements with manufacturers on discounts and rebates, subject to provisions of the federal Anti-Kickback Statute. A VBA links rebates to proof that a drug's performance among patients covered by the payer lives up to certain pre-negotiated benchmarks. Like typical drug purchasing contracts VBAs are confidential business agreements between payers and manufacturers. A limited amount of publicly divulged information is available about the size of conventional discounts and rebates and the size of rebates paid when VBA outcomes are not achieved. It is likely that most current VBAs (and maybe all current VBAs) make provision for both types of rebates.

Publicly released details on recent value-based arrangements illustrate the array of goals that payers and manufacturers are pursuing through VBAs that are allowed under current federal regulation. They include:

- Prompt access to one-time, potentially curative therapy in exchange for payments linked to demonstration of the therapy's durability and its comparative effectiveness against other therapies[45]
- Reductions in total costs of care among patients with type 2 diabetes[46]
- Manufacturer rebates triggered when patients with multiple sclerosis discontinue or switch from an MS therapy covered by a VBA[47]

Assertions to the contrary [2], current VBAs are not utilized to lower list pricing. Most payers and manufacturers prefer to rely on standard discount and rebate contracting except when a VBA might be useful in arbitrating a very specific goal of either the payer or manufacturer. VBAs on standard, chronic disease medications have been negotiated to promote patient medication adherence and persistence. VBAs on complex, novel therapies (such as breakthrough drugs) have been negotiated to reduce payer costs in the event an individual patient does not respond to treatment. In some cases manufacturers and payers see a VBA as a mechanism for testing the comparative effectiveness of a drug that may otherwise not be chosen as a preferred or low-cost option for the plan's subscribers, but may improve outcomes and lower total costs of care among patients who do not respond well to other, more highly preferred drugs.

Greater innovation in VBAs -- including innovations that might lower health care spending more decisively - is constrained by barriers that NEHI and others have documented frequently.[3] VBAs are a departure from standard, tried-and-true, discount and rebate contracting and thus impose extra burdens of complexity and management on payers and manufacturers. The obligation on manufacturers to pay rebates to Medicaid programs based on Best Price reporting means that the additional cost and complexity of a VBA for any one payer is weighed against the potential added cost of Medicaid rebates. VBAs are not clearly protected by Safe Harbor status under the Anti-Kickback Statute, and thus far the HHS Office of the Inspector General has not proposed a rule that would extend Safe Harbor status to them.

NEHI conducted a series of interviews and convened expert panels in 2019-2020 on the premise that reorienting the debate on VBAs towards a more clear focus on benefits to patients will not only clarify the complex issues of regulatory reforms needed to spark a "next generation" of VBAs, but may likely be a critical step in winning support among legislators, government agencies, payers and manufacturers to negotiate fair and equitable solutions. We find a general consensus to support our premise that re-defining next generation VBAs in terms of their benefits to patients is key to catalyzing the regulator, payer, and manufacturer support necessary to overcome the still-daunting barriers to execution of VBAs.[4]

VBAs - The Stakes for Patients

What drives payers and manufacturers to undertake a VBA?

At least for now, there is little in the way of published research and evidence on the structure of, and the results from value-based arrangements. Two reasons for this may be that, like nearly all payer-manufacturer agreements on pharmaceuticals, they remain confidential business agreements. Also, while there are examples of VBA-type contracts in the U.S. that date back ten or more years[5] , the recent upswing in VBA activity among payers and manufacturers only dates to the last five or six years. Nevertheless, policy proposals from payers and manufacturers, and anecdotal evidence (such as NEHI has gleaned through several projects on VBAs) point to three major factors:

1. The introduction of highly novel, potentially transformative drugs such as gene therapies and CAR-T cancer therapies, that are administered on a one-time basis, often at high initial cost, give payers and manufacturers a shared interest in arrangements that mitigate the initial budget impact of a new therapy.[6]

2. Manufacturers enjoy leverage in negotiations on payment for newly introduced drugs that are novel, first-in-class, or approved as a superior therapeutic option for patients or representing a new standard of care. For payers, a VBA can offer a mechanism to provide patients with access to novel drugs while linking net payment for the therapy to proven results, thus mitigating the financial impact on health plan sponsors such as employers.[7]

3. Payers enjoy leverage in negotiations on payment for branded drugs that are comparable or directly compete with other branded drugs. Manufacturers have a motivation to use VBAs as a vehicle for winning better placement on payers' drug formularies if the manufacturer has strong evidence in data to suggest that a drug will perform better than the payer's preferred alternative. Innovative payers are taking this opportunity to utilize VBAs for drugs and align them with the goals of their provider payment reforms to achieve better outcomes in population health among large groups of patients, such as patients with type 2 diabetes and heart disease.

What do patients have to gain from VBAs on the drugs they use?

Patients are not direct parties to payer-manufacturer contracts on drugs. To the extent patients and patient communities have leverage it is informal and exercised through outside advocacy. Arguably, the role of patient advocacy in influencing payer coverage decisions has become more visible, if not more influential, as the FDA has approved a stream of highly-novel, high-cost therapies based on accelerated reviews from the FDA Breakthrough Therapy and Orphan Drug approval pathways. Recent examples include patient campaigns to persuade payers to cover, or to expand coverage, of therapies for Duchenne muscular dystrophy and spinal muscular atrophy.[8] Some U.S. payers have reached prompt decisions on coverage by negotiating VBAs.[9]

Yet as noted already, payer and manufacturer interest in VBAs is not confined to highly-novel, high cost drugs, and extends from coverage of medications for rare disease to coverage of branded, frequently used drugs for chronic disease in which there may be multiple, competitive options. The stakes for patients, and the implications for patient advocate groups, differ accordingly.

Patient Goals for VBA Performance: Rare Disease vs. Chronic Disease

A simplified but useful way to differentiate the stakes for patient groups in value-based arrangements is to see VBAs as falling into roughly two scenarios. In one scenario, (Scenario #1), VBAs are used by payers and manufacturers to cover drugs targeted to small patient populations, including populations with rare or ultra rare diseases. Many highly novel and high cost drugs, such as gene therapies, fall into this scenario. In another scenario, (Scenario #2), VBAS are used by payers and manufacturers to cover drugs used by patients with highly prevalent chronic diseases such as diabetes, COPD, asthma, and heart disease. Exhibit 1 provides a rough breakdown of these diverging interests.

Exhibit 1

MAJOR GOALS	Payer	Manufacturer	Patients and Patient Communities
Scenario #1: Therapies for rare diseases and other diseases of small prevalence; ordinarily classified as specialty pharmaceuticals, includes drugs with high one-time costs	• Link net payments (including rebates) to results seen in Real World use • Recover costs if therapy fails in individual patients • Cushion initial budget impact of high-cost therapy (example: enable payment-over-time)	• Expedite payer's decision to cover therapy • Adapt utilization management requirements to facilitate access to novel drugs by providers and patients	• Prompt decisions on coverage by payers after drug's initial launch • Adapt utilization management requirements to facilitate prompt time-to-therapy • Financial protection against costs of therapy if therapy fails
Scenario #2: Therapies for chronic disease and other diseases of large prevalence (example: type 2 diabetes)	• Determine comparative effectiveness vs. preferred drugs • Lower total costs of care (medical and pharmacy) by increasing drug choices on formulary that enable optimal use of medicines by prescribers and patients	• Improve formulary position, lower barriers to access (or expand access) to drugs otherwise seen as interchangeable or commoditized	• Better affordability of therapies (example: reduce or eliminate out-of-pocket cost); reduced financial barrier to adherence and persistence • Improved access to drugs generating better treatment response vs. preferred drugs • Reduced disruption from non-medical drug switching

Scenario #1 and Scenario #2 are simplifications, of course. New pharmaceuticals can be novel, potentially transformative, comparatively costly (compared to prevailing standards of care) and address a potentially large patient population; recently introduced therapies for acute migraine headache (a patient population of upwards of 38 million) are a case in point.[10] Goals for patient benefits outlined in recent payer-manufacturer VBAs on drugs of varying types can be summed up in three general categories. These three categories could be core parameters of any basic definition of "next generation" VBAs:

1. Patient access to therapy : As noted, payers and manufacturers are using VBAs to adapt, adjust or reduce utilization management requirements for use of drugs, or provide better placement on the payer's drug formulary, in exchange for rebates linked to performance, thus improving timely access to therapy for patients.[11]

2. Patient focus: Payers and manufacturers are using VBAs that link payments to patient outcome measures that would not otherwise be considered in payer-manufacturer contracting, such as utilizing patient-reported outcome measures (PROMs) to track whether a drug is slowing the progression of a degenerative disease such as multiple sclerosis,[12] or tracking how well cancer patients tolerate cancer therapy and maintain adherence, as measured by rates of patient discontinuation of therapy.[13]

3. Patient affordability: Innovative payers and manufacturers are using VBAs to reduce patient cost barriers to medication adherence and persistence by stipulating low or zero out of pocket costs to patients for use of drugs covered by VBAs.[14]

Defining Next Generation VBAs by Their Benefits to Patients

In prior reports NEHI called on federal agencies to work with key stakeholders (patients, payers, and manufacturers) to develop a consensus-based definition of VBAs.[15] A core definition of VBAs is necessary for appropriate regulatory changes that will facilitate more innovative VBAs, and it could also serve to promote standardization of metrics, data collection, analytical methods and other aspects of VBA negotiation and execution that contribute to the operational complexity of VBAs.

In June 2017 the Centers for Medicare and Medicaid Services (CMS) released its first proposal for a core VBA definition. As of now it is the only VBA definition to be offered by a federal agency. In October 2019 the HHS Office of the Inspector General deferred a decision on defining pharmaceutical VBAs for purposes of an enforcement Safe Harbor under the federal Anti-Kickback Statute.[16]

The CMS proposal defines VBAs very flexibly. VBAs are defined as meeting criteria in one or both of two categories: VBAs that "substantially link the cost" of a pharmaceutical to existing evidence of the pharmaceutical's effectiveness or value, or VBAs that "substantially (link) payment" to measures of outcomes that demonstrate actual performance of the drug in a patient or population or a reduction in other medical expenses."[17]

The CMS proposal takes a positive step by proposing that "reduction(s) in other medical expenses" will be one of several major characteristics of VBAs that meet the proposed CMS criteria. Reductions in "other medical expenses," such as reductions in total costs of care resulting from use of a new medicine, or optimal use of chronic disease medicines, is an objective of several VBAs announced by payers and manufacturers in recent years.

Perhaps more significantly, reducing overall costs of care through better coordination of pharmacy, medical and other services is an increasingly important goal of major payers throughout the U.S. health care system. Major payers have increasingly acquired (or created their own captive) pharmacy benefit managers (PBMs) to facilitate integrated medical-pharmacy management.

Cigna and its prescription benefit management unit, Express Scripts, claims to have saved $850 per insurance enrollee in a recent two-year period through integrated management of medical, drug and mental health benefits.[18] Medicare Advantage plans also have a particular incentive for managing pharmacy and medical costs as a whole since they cover their Medicare patients for both medical and drug benefits.[19] Humana, a leading Medicare Advantage insurer, reported medical savings of $3.5 billion for 2019 through its "whole person" management of medical and drug benefits. (Humana also acknowledges itself as an active party to multiple VBAs, executed every year over the last decade.)[20]

Prime Therapeutics, a pharmacy benefit manager owned by 18 of the nation's 35 Blue Cross Plan, describes its VBA strategy as "focus(ing) on partnering with combinations of stakeholders, (including) ...integrated delivery systems, payers and pharmaceutical manufacturers. As a result, our value-based approach is uniquely positioned to aspire to the triple aim of improved patient experience, improved health of populations, and lower cost of care, recognizing mutual cost risk among all participants."[21] Prime Therapeutics is also an active negotiator of value-based arrangements.[22]

However, neither of the two options offered by CMS is framed affirmatively or explicitly around criteria of net health benefit for patients. European countries that have developed definitions (or frameworks) for outcomes-based contracts in recent years have generally framed qualifying contracts as agreements that result in improvements, or at minimum stabilization of patient health in defined disease areas.[23] The UPMC Health Plan, a major national innovator in VBAs, has defined its goal for VBAs as " (f)irst and most importantly, VBPCs (value-based purchasing contracts) should focus on medications that clearly offer clinically meaningful benefits to patients."[24])

Framing VBAs around goals for improved benefits to patients could be an important factor in brokering agreement on reasonable changes in Medicaid Best Price reporting that will trigger innovative VBAs while benefiting Medicaid programs as well. Current projections suggest that state Medicaid programs will enroll as many as 12 million or more beneficiaries in coming months as a result of job losses due to the COVID crisis.[25] The increased stress on Medicaid programs will likely increase the insistence of state Medicaid programs that any regulatory changes to encourage VBAs be structured so as to hold Medicaid programs harmless, at minimum, if not improve the finances of state Medicaid programs. In commenting on CMS's proposed rule the National Association of Medicaid Directors (NAMD) urges CMS to revise its proposed definition of VBAs by adding parameters ("guardrails") that would, among other objectives, tighten the focus of VBAs on patient outcomes and " (r)equiring that the (value-based purchasing) arrangement be anticipated to generate savings larger than those available under the current MDRP framework, inclusive of administrative costs."[26]

At the same time, Medicaid programs incur substantial costs every year due to poor patient medication adherence and other forms of sub-optimal medication use. Improvements in patient adherence and in optimal medication use to treat chronic disease are already goals in VBAs executed by commercial payers under current rules, since sub-optimal medication use, and poor patient medication adherence and persistence result in substantial costs of otherwise avoidable health care utilization. A recent systematic review of medication adherence studies estimated that poor medication adherence among patients with diabetes results in a cost burden of as much as $7000 per year per patient in avoidable health costs. The same systematic review estimated that avoidable health care costs associated with sub-optimal medication adherence among patients with mental illness is in the range of $16,000 per patient per year. (Both estimates in 2015 dollars.)[27]

More innovative VBAs could be designed to demonstrate good use of medicines (in commercial agreements, or in agreements with Medicaid programs) that improve outcomes and cut costs for care for treatment of conditions that are over-represented among Medicaid patients, such as patients with diabetes and patients with serious mental illness. Average total costs of care for patients with both sets of conditions (which are often co-morbid, as well) are among the highest costs borne by Medicaid programs on a per-condition basis. A 2017 analysis from the CDC determined that Medicaid patients with these conditions have consistently poor rates of medication adherence.[28] Notably, the first value-based agreement approved by CMS for a state Medicaid program (a VBA between the State of Oklahoma and the biotech firm, Alkermes) is a VBA linked to adherence outcomes for patients with schizophrenia.[29] More innovative VBAs in the commercial sector could provide important demonstrations for translation to Medicaid when they are applied to therapies that treat conditions highly prevalent in the Medicaid population.

A Patient-focused Definition of VBAs

Payer-manufacturer agreements that qualify as a next generation VBA should conform to several parameters of patient benefits; as noted, we suggest that the three over-arching parameters of benefit should be defined as patient access, patient-focus, and patient affordability.

Specifically, the parameters should include:

Measurable improvements in patient health: Qualifying VBAs should be designed to demonstrate measurable improvements in patient health. A useful precedent is the October 2019 proposal from the HHS Office of the Inspector General regarding a Stark Law Safe Harbor to facilitate value-based payment agreements with health care providers, in which qualifying payment models must be designed to demonstrate measurable improvements in quality, health outcomes, or efficiencies in care delivery.[30]

Major categories for improvement include:

- Measurable improvement in patient treatment adherence and persistence, particularly for medications treating chronic disease
- Reduced patient utilization of health care services, provided patient outcomes are evaluated against standard measures of health care quality to prevent under-treatment
- Reduced total costs of patient care measured against a patient population, provided outcomes are evaluated against standard measures of health care quality
- Improved patient quality of life, as measured by patient-centered, clinical outcomes assessments (COAs) such as validated patient-reported outcome measures (PROMs)

Validated metrics: **Qualifying VBAs should link payment to validated outcome metrics.** Here again, the recently proposed Stark Law Safe Harbor is a useful precedent. The proposed rule calls for outcome metrics grounded in legitimate, verifiable data or other information from a credible external source (such as a medical journal, social sciences journal, or scientific study), an established industry quality standards organization, or results of a payor or CMS-sponsored model or quality program.

Monitoring of safety, quality and performance: **Qualifying VBAs should include provisions for monitoring and assessment of patient safety and care quality throughout the term of the VBA.** Safety and performance data should be available on a reasonable basis to prescribers and to patients. Drugs that fall into the Scenario #1 category may be subject to close monitoring for other reasons; FDA-required surveillance, for example. For more commonly used chronic disease medications (Scenario #2) the VBA should include a data collection program that will allow for reasonable comparisons to pertinent standards of care.

Provisions to Enable Patient Adherence and Persistence: **Qualifying VBAs should enable patients to maintain treatment adherence and persistence.** Patients will not benefit from drugs covered by VBAs if barriers to adherence get in their way. Ensuring adherence and persistence is also critical to demonstrating the usefulness of VBAs now and in the future, and the justification for extending regulatory forbearance to VBAs in the interest of proving their impact on improved patient health and lowering total costs of health care.

Payers and manufacturers can reduce the incidence of cost-related non-adherence by reducing or eliminating patient out-of-pocket costs on drugs covered by the VBA[31], or if payers stipulate that the drugs will be covered under insurance plans that reduce or eliminate out-of-pocket costs for drugs deemed to be of high value. Insurance plans that utilize value-based insurance design (VBID) have been shown to improve outcomes and decrease total costs of care for patients with chronic disease. As VBID proponents have pointed out, pharmaceutical VBAs and VBID insurance plans could be integrated and utilized more widely to improve medication use and population health management and reduce total costs of care. [32]

Other forms of support for patient adherence and persistence should be allowed as core elements of qualifying VBAs. The HHS Office of the Inspector General has expressed reservations that inclusion of manufacturer-supported adherence supports within VBAs could violate the Anti-Kickback Statute, even inadvertently. Adherence supports could have the effect of steering patients towards the manufacturer's drugs even when other drug choices are preferable, and lead to inappropriate utilization that create undue costs to federal health programs.[33] Potential harms to patients through medication adherence supports provided under a VBA could be prevented by enforcing clear guidance on patient safety and quality monitoring, as suggested above. Meanwhile, potential increases in federal spending due to VBAs can be reduced or mitigated by requiring VBAs to demonstrate a reasonable pathway towards improved outcomes that offset medical costs, along the lines of VBA-VBID integration.

Early in 2020 the Office of Inspector General issued an advisory opinion that allows patients undergoing a specific, personalized cancer treatment (reported to be Kymriah, the first U.S.-approved CAR-T cancer therapy) , to receive assistance for travel, lodging and meals associated with the hospital stays required for administration of the therapy.[34] The advisory opinion pertains only to this one, specific therapy but it underscores the principle that, within appropriate Anti-Kickback guardrails, some non-medical and non-pharmaceutical forms of support may be essential to realize successful treatment, patient benefits, and to achieve improvements in overall costs of care.

Travel assistance for Kymriah patients is an example of adherence supports for a highly specialized, "Scenario #1" type drug, but the principle extends to VBAs covering chronic disease medications as well. Social factors that prevent a patient from accessing or maintaining therapy, such as lack of access to transportation to a physician's office, or poor access to affordable , healthy food, can defeat the purpose of value-based use of chronic disease medication.[35] Barriers related to social determinants of health (SDOH) are an increasing focus of provider payment reforms, including cutting-edge innovations in Medicaid Accountable Care that are now screening Medicaid patients for their level of SDOH-related risk, and tailoring SDOH-related supports such as transportation and nutrition assistance.[36] SDOH-related support services that are directly related to assuring patient success on therapy covered by a VBA should be allowed as reasonable elements of patient-focused VBAs.

Limited or No Charge to Patients for Treatment Failure: **Qualifying VBAs that return money or void charges to payers if a treatment fails should include provisions that return money or void charges to patients as well.**

By way of background: "real world" patients treated in real world settings are always expected to respond to treatment at varying levels of response, (the so-called heterogeneity of treatment effect). With some highly novel new therapies there is a reasonable expectation that some patients will respond weakly or fail to respond altogether. A loose consensus has emerged among some manufacturers that in these circumstances manufacturers should rebate, lower or refrain from charging payers when a patient fails treatment. [37] One highly visible case of a manufacturer offering a reduced-charge-for-non-response contract is Novartis, which introduced such a contract with the approval of Kymriah, the first CAR-T cancer therapy approved for use in the United States. Nevertheless, payers and manufacturers still believe that Medicaid Best Price reporting impedes wider use of no-charge or reduced-charge contracts, since under a strict interpretation of the rules a manufacturer that rebates all or most of the price of a new therapy will be responsible for providing the therapy at a zero or greatly reduced price to all Medicaid programs regardless of whether Medicaid patients fail on the therapy or not.

The proposed CMS rule on Medicaid Best Price and Value-based Arrangements appears to address this issue by proposing new reporting rules, although many manufacturer and payer organizations, including state Medicaid directors, responded to CMS that the proposed changes in price reporting are too operationally complex to be practical.[38]

A final rule on Medicaid Best Price and Value-based Arrangements will clearly require further development, including development of a firm consensus among Medicaid programs, other payers, and manufacturers. A consensus agreement should protect patients by making them eligible for no-charge or reduced-charge protection when their response to therapy falls short of a pre-defined benchmark of treatment failure. No-charge provisions are likely to be most relevant to pharmaceuticals (such as the Kymriah CAR-T therapy) that fall in the Scenario #1 category cited above: highly-novel, most likely emerging from the FDA Breakthrough Therapy or Orphan Drug approval pathways, and so on. Earlier this year 37 rare disease organizations called on Congress to ensure that patients will be afforded this financial protection in legislation or regulation.[39]

Meaningful Duration of Contract: **Qualifying VBAs should be of sufficient duration to prove meaningful results for patients.** Some therapies may produce immediate or short-term results in improved patient health and patient quality of life, but in many other cases improvements take time. Qualifying VBAs should be of long enough duration to prove results that are measurable and significant. (For example, a 3-year VBA contract for heart failure medication that monitors a statistically meaningful reduction in reduced hospital readmissions.[40])

An Innovation Agenda for Value-based Arrangements

Under current regulation and current operational constraints, VBAs may have limited application in the years ahead. At the same time innovative payers and manufacturers are applying VBAs to coverage and utilization management of pharmaceuticals that create important benefits for patients, such as timely access to novel therapies for serious conditions with unmet medical need, and support for optimal use of therapies among patients with high prevalence chronic diseases that are major targets for improved population health management within provider payment models such as Accountable Care Organization models.

Re-framing a core definition of VBAs around benefits to patients could be an important step in the difficult process of achieving consensus on the regulatory treatment of VBAs. Even then, VBAs may remain a complex choice for payers and manufacturers. In order to fully demonstrate the capability of VBAs in meeting the needs of patients, manufacturers, and payers (including federal health programs), VBA demonstrations that address significant health needs should be encouraged by federal agencies.

For example, the CMS Innovation Center (CMMI) has statutory power to authorize time-limited demonstrations of value-based arrangements that could afford payers and manufacturers some flexibility from current Medicaid Best Price reporting, including an exemption for Medicare Advantage plans. CMMI's authority is grounded in statutory language that authorizes it to sponsor demonstration projects to address "deficits in care" or "potentially avoidable expenditures" for Medicare and Medicaid beneficiaries.[41]

There is a wide range of therapeutic areas in which private sector VBAs could demonstrate effective and optimal use of medicines for the benefit of patients in general, and beneficiaries of Medicaid and Medicare in particular.

Stakeholders offered several ideas for experiments with innovative VBAs to NEHI in the course NEHI's work on this paper, including:

1 Serious illness among children:

- Serious unmet medical needs among children, an area of importance for Medicaid, the largest insurer of children's health in the U.S.; rare disease therapies (Scenario #1-type therapies) that treat genetic diseases are often indicated for use primarily with children. Medicaid managed care plans may be particularly vulnerable to an initial budget impact of higher-cost novel drugs; demonstrating cost effective ways to utilize VBAs that mitigate budget impact and prove high-value use of novel drugs for patients, providers and Medicaid plans should be a priority.

2 Serious unmet needs among vulnerable patients:

- Serious unmet needs among low-income and other vulnerable populations, such as unmet needs in behavioral health and mental illness, conditions imposing significant lifelong burdens on patients, major costs on society. Value-based behavioral health care is a significant priority for Medicaid programs in which the application of meaningful metrics, and data collection on performance, are also priorities for demonstration.[42]

3 Gaps in optimal use of chronic disease medications:

- Gaps in optimal medication use that are impeding critically needed improvements in population health, such as improvements in diabetes and behavioral health care, major targets for improvement in Medicare and in Medicare's alternative payment models, such as Accountable Care.

4 Integration of pharmaceuticals and services:

- VBAs that integrate utilization of pharmaceuticals and supportive services, such as aids to patient medication adherence, or services that address social determinants of health (such as food insecurity, or lack of transportation to treatment) that demonstrate innovation in population health management linked to improved population outcomes and reduced total costs of care.

5 Patient input on VBA design:

- VBAs that are designed with direct input from patient communities regarding patient preferences and values in therapy; the UPMC Health Plan has been an innovator in this approach.[43]

6 Patient-centered outcome metrics:

- VBAs that utilize metrics of patient outcomes, such as new and innovative clinical outcome assessment measures (patient-reported outcome measures, caregiver-reported outcome measures, etc.) that reflect patient preferences and values but prove challenging for data collection in real world settings.

7 Payer agnostic VBAs:

- "Payer agnostic" VBAs, or standardized VBAs, designed initially by manufacturers, executed by multiple, different payers as a demonstration of patient benefit with reduced operational complexity.[44]

8 VBAs and Third-Party or Shared Data Sources:

- VBAs in which outcomes are measured through third-party data sources, or data pooled and shared among payers, as demonstrations of VBAs executed with reduced administrative expense

Conclusion

While progress has been uneven, the U.S. health care system has moved towards greater reliance on value-based models of payment to health care providers in recent years, and value-based arrangements for pharmaceuticals have become one set of strategies payers and manufacturers have used to broker agreement on the coverage and utilization management of drugs.

The future of this system-wide movement towards value-based models is in some doubt, due to the COVID-19 pandemic. However, there is good reason to believe that value-based payment models for providers may take on greater importance in the years ahead as the system struggles to meet increasing medical needs with limited dollars.

Meanwhile, should policymakers ultimately decide to replace or reduce the health care system's current reliance on discount-and-rebate contracting, VBAs will serve as a strategy to reconcile the costs of drug therapy with proven results. Difficult compromises remain to be achieved in order to forge consensus on regulatory and operational changes that will trigger more innovation and a next generation of VBAs. To achieve these compromises VBAs should be refocused and redefined in terms of their increased benefits to patients, and VBAs should be developed that will teach lessons learned on improved patient care and patient quality of life that can be applied to patients with all forms of health insurance, public and private sector alike.

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