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# Regulatory, Legal Uncertainties Are Barriers To Value-Based Agreements For Drugs

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☰ SECTIONS



The past several years have seen an [increasing number of new and innovative therapies](#) entering the drug market. Many of these are [precision medicines](#) developed to treat a narrowly defined patient population, often with a previously unmet need. These treatments have demonstrated success in improving quality of life and other important health outcomes among indicated patients in clinical trials, but there is uncertainty about patient response rates in real-world settings. These uncertainties have led payers to express concerns about the costs of some new medicines and to implement policies to control patients' access to those medicines, such as higher cost sharing, [health technology assessments](#), and step therapy (which requires

providers, and biopharmaceutical companies to better understand the value of new medicines and align payment with it. By tying payment to real-world outcomes, these arrangements—collectively referred to in this post as VBAs—have the potential to support patients' prompt and affordable access to new, innovative treatments while also addressing payers' cost concerns. Despite [considerable interest](#) from stakeholders on both sides of the negotiations, there were few successful examples of VBAs in the U.S. until very recently: Between 1993 and 2013, there were [fewer than 20](#) VBAs executed in the U.S. However, [more of these arrangements](#) have recently been announced, although they remain rare, and payers are expressing increased interest. The academic literature provides [information about some of the existing agreements](#) and suggests possible barriers to their execution, but it leaves many questions unanswered. We conducted two-part interviews with a group of five stakeholders regarding their experience with these types of contracts. All respondents had direct experience developing and negotiating VBAs, four as representatives of private insurers and pharmacy benefit managers, and one on behalf of a large pharmaceutical firm launching branded products. The interviews focused on respondents' overall perceptions and expectations of VBAs, barriers to adoption, and possible solutions to those barriers (see [note 1](#)). In order to solicit unbiased responses, the interviews were double blinded: the sponsor of the research was not revealed to interviewees, and the identity of respondents is not known by the sponsor. We conducted the initial interviews during the summer of 2015 and followed up with the respondents this fall (2016) to understand how perception of VBAs and the barriers to them may have shifted.

#### **INTERVIEWS IDENTIFIED THREE KEY BARRIERS AND POSSIBLE SOLUTIONS**

We learned that while most stakeholders are excited about the potential of VBAs to manage costs and encourage improved real-world health outcomes, they believe that substantial external barriers have limited the use of VBAs. Respondents identified a number of barriers to implementing VBAs, including data collection and availability, timing of outcomes measurement, and the need for institutional champions in each organization. However, there was consensus among our interviewees that the data infrastructure is constantly improving, largely due to the adoption of evidence-based medicine and the improved availability of real-world data in clinical practice. When asked to rank the barriers to VBAs, respondents unanimously indicated that the most critical barriers are legal and regulatory concerns -- specifically, uncertainty about implications of VBAs for federal anti-kickback statutes, Food and Drug Administration (FDA) regulations of manufacturer communications, and Medicare and Medicaid price reporting requirements. Notably, the degree to which respondents viewed these legal uncertainties as obstacles varied: Some participants expressed substantial uncertainty about if and how these statutes and regulations would apply to VBAs, but they thought these issues would not prohibit VBA execution altogether, while others saw the same statutes and regulations as definitive obstacles that trump all incentives to negotiate VBAs. Additionally, the concerns about the legal and regulatory barriers persisted across both the initial and follow-up interviews, even as concerns about other barriers, such as data collection and availability, diminished.

#### **ANTI-KICKBACK STATUTE**

The anti-kickback statute is a federal criminal law that prohibits the exchange of, or the offer to exchange, anything of value with the intent to influence the use of products or services paid for by federal health care programs. It is designed to broadly protect the government, patients, health plans, and other stakeholders from fraud and abuse. The law is vague and the key safe harbors relevant to manufacturers were created prior to the implementation of Medicare Part D. In our interviews, the most common concern expressed

regarding information not included in the product labeling. These regulations preclude manufacturers from proactively communicating economic evidence not contained in the FDA-approved label, preventing or limiting potentially beneficial VBAs. The primary issue with these FDA regulations identified in our interviews relates to endpoints included on the label. The FDA-approved labeling usually contains intermediate endpoints (such as lab values) measured in clinical trials; however, real-world, patient-centered outcomes that impact quality of life, such as hospitalizations or readmissions, are arguably more meaningful and could be collected more reliably, and are thus more logical focuses for VBA contracts. For example, the FDA may approve a new treatment for diabetes based on clinical trial data showing significant improvements in A1C levels among treated patients. The FDA-approved label would reflect this trial outcome, but the A1C improvement is an intermediate outcome that is difficult for payers to quantify and thus contract on. Payers would more likely be interested in the reduction of diabetes-related complications such as strokes, kidney disease, and hypertension. These real-world outcomes are also clearer endpoints to use as the basis for contracting and more evidently reflect improvements in patients' health and quality of life. In this example, the insurer might agree to pay the manufacturer more for patients that go one year without one of these diabetes complications. However, uncertainty about FDA regulations allowing communication of real-world evidence may limit manufacturers' ability to enter into this type of contract.

#### **PRICING LAWS FOR MEDICARE AND MEDICAID**

The third regulatory concern consistently mentioned in our interviews was Medicare/Medicaid price reporting requirements, specifically the Medicare Part B average sales price (ASP) and the Medicaid best-price rules. Manufacturers are required to report their drug sales to all U.S. purchasers—except certain exempt sales, such as the Veterans Health Administration—to a government agency, the Centers for Medicare and Medicaid Services, which calculates the ASP for the Medicare Part B program and a “best price” for the Medicaid program. The Medicaid best price is typically the manufacturer's lowest price offered to non-exempt purchasers. If performance-based discounts tied to VBAs drive the contracted prices low enough, they may affect the prices paid by Medicare Part B or Medicaid. In addition, manufacturers are able to make reasonable assumptions about how to report their sales for ASP and best-price under VBAs, but a lack of clarity about how these laws apply to new value-based arrangements may deter manufacturers.

#### **RECOMMENDED SOLUTIONS**

Given the enthusiasm about VBAs expressed in all of our interviews and the literature-based evidence that these contracts can improve outcomes through access to innovative therapies, we also solicited feedback on potential solutions to the various barriers mentioned. For all three of the barriers discussed above, the interviewees identified regulatory uncertainty, rather than any known conflict, as the primary obstacle to implementing VBAs. Although they suggested that education regarding the regulations would benefit some in the industry, they stated that the best way to encourage the execution of VBAs would be to create safe harbors for these types of contracts in existing laws. Above all, the interviews emphasized that VBAs should be protected from the consequences of the anti-kickback statute and the FDA's off-label communication regulations. The potential for VBAs and similar arrangements to improve outcomes and access has not been realized to date due to the barriers and concerns identified by stakeholders in our interviews. Until measures are taken to directly address uncertainty around the relationship of VBAs to the regulatory environment—especially in the areas described above—innovative and creative approaches to contracting based on the outcomes of treatments are likely to remain rare.

#### **NOTE 1**

## AUTHORS' NOTE

Funding for this research was provided by PhRMA.

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**Arvind Cavale** · a year ago

VBA is a concept only good in the minds of those who seldom touch a patient or care for one. Taking your example of diabetes drugs, one can get an idea of the narrow viewpoint expressed here. Diabetes is a multifactorial chronic illness, the management of which depends of various factors and combination of interventions. Attributing the lowering of A1c or reduction of complications to a single drug alone, is an error of astronomical proportions. Anybody who manages diabetes can vouch for the fact that lowering a1c or reducing complications can be achieved in various ways, with and without medications, and that the same drug while highly effective for one patient, may be ineffective for another. Also, completely ignored in this commentary is consideration of side effects of a drug, which may make an effective drug unusable. Finally, the concept of evidence-based medicine has been discarded by most clinicians. Perhaps evidence-informed, practice-guided medicine might be more appropriate, unless the authors want to prescribe a cookbook for medical practice. It is time to dump the idea of VBA, as it will not serve patient's interests and can be an ethical conflict for the clinician.

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