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Payer and Pharmaceutical Manufacturer Considerations for Outcomes-Based Agreements in the United States

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ABSTRACT

Background: Considerable interest exists among health care payers and pharmaceutical manufacturers in designing outcomes-based agreements (OBAs) for medications for which evidence on real-world effectiveness is limited at product launch. **Objectives:** To build hypothetical OBA models in which both payer and manufacturer can benefit. **Methods:** Models were developed for a hypothetical hypercholesterolemia OBA, in which the OBA was assumed to increase market access for a newly marketed medication. Fixed inputs were drug and outcome event costs from the literature over a 1-year OBA period. Model estimates were developed using a range of inputs for medication effectiveness, medical cost offsets, and the treated population size. Positive or negative feedback to the manufacturer was incorporated on the basis of expectations of drug performance through changes in the reimbursement level. Model simulations demonstrated that parameters had the greatest impact on payer cost and manufacturer reimbursement. **Results:** Models suggested that changes in the size of the population treated and drug effectiveness had the largest influence on reimbursement and costs. Despite sharing risk for potential product underperformance, manufacturer reimbursement increased relative to having no OBA, if the OBA improved market access for the new product. Although reduction in medical costs did not fully offset the cost of the medication, the payer could still save on net costs per patient relative to having no OBA by tying reimbursement to drug effectiveness. **Conclusions:** Pharmaceutical manufacturers and health care payers have demonstrated interest in OBAs, and under a certain set of assumptions both may benefit.

Keywords: managed care, outcomes-based agreements, pay for performance, reimbursement.

Introduction

Growing focus on value in health care has led to a number of initiatives that are designed to shift the reimbursement system to better align costs of services to value in real-world settings [1,2]. In recent years, there has been increased interest in manufacturer and payer agreements that tie reimbursement to product performance. These are generally referred to as performance-based risk-sharing arrangements or outcomes-based agreements (OBAs) [3]. In an OBA, performance in a defined patient population is tracked over a specified period of time in a defined population or at the individual patient level, and the amount or level of reimbursement is determined on the basis of the outcomes achieved [3,4]. Both pharmaceutical manufacturers and payers have been motivated to develop such agreements to more closely align price and value. Recent headlines regarding higher cost new medicines have also likely been a catalyst for interest in OBAs [5,6].

To date, most examples of implemented OBAs come from Europe [5]. In the United States, attempts to structure OBAs have been few and far between, because the contracting parties often struggle to align on and define the core metrics used to assess health outcomes under the contract [6]. In addition, specific details on existing deals are limited because of the proprietary nature of these agreements between individual payers and manufacturers [5].

Conflicts of interest: R. Sheer, L. Sudharshan, and M. Pasquale are all employees of Comprehensive Health Insights Inc. F. Brownfield is an employee of Humana Inc. K. Axelsen, P. Subedi, D. Wiederkehr, and S. Kamal-Bahl are employees and shareholders of Pfizer Inc. There are no other perceived or actual conflicts of interest.

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Given the difficulty observed in developing successful OBAs, the primary objectives of this study were to build models to better understand key variables having the greatest impact on outcomes, costs, and cost sharing, and to propose design elements for the development of OBAs that may improve each party's willingness to negotiate such agreements in the future.

Methods

Hypercholesterolemia was chosen as a case example for the model given recent innovations in treatment paradigms that are anticipated to have a large budget impact on payers [7]. Hypercholesterolemia is associated with multiple clinical outcomes that can be measured including surrogate end points (low-density lipoprotein cholesterol [LDL-C] measurements and goals) as well as "hard" clinical outcomes (i.e., acute myocardial infarction [MI] and stroke). Appropriate outcomes and target populations for implementing a hypercholesterolemia OBA were determined on the basis of review of end points from published literature and prescribing information for available products [8–10]. Excel-based models were developed to demonstrate the impact that various parameters could have on OBAs. An internal project advisory board provided input via firsthand accounts of successes and limitations of previous OBAs from both manufacturer and payer perspectives. This input was incorporated into the model development to address areas in which OBAs may be improved.

Data on hypercholesterolemia incidence were extracted from the Humana Research Database (Louisville, KY). The patient population included patients enrolled in Medicare or commercial plans at Humana Inc. with an index diagnosis of familial hypercholesterolemia or a history of atherosclerotic cardiovascular disease (ASCVD), defined as MI, stroke, angina, peripheral arterial disease, or revascularization procedures [11]. Diagnosis and procedure codes used in the identification process are detailed in Appendix Tables 1 and 2 in Supplemental Materials found at http://dx.doi.org/10.1016/j.jval.2017.07.009. Rates of ASCVD events per 1000 person-years were established after the index diagnosis for this patient population.

Two model structures were developed. The first modeled patients reaching a goal LDL-C reduction on the basis of observed LDL-C reductions in pivotal clinical trials of proprotein convertase subtilisin/kexin type 9 (PCSK9) inhibitors, a novel class of medications indicated for hypercholesterolemia (model 1) [9,10]. As part of the hypothetical OBA structure, patients with LDL-C values at baseline would be followed up at 12 weeks after treatment initiation. Given that PCSK9 inhibitors have been shown to be highly effective at lowering the LDL-C, the percentage of patients meeting LDL-C goal reductions at 12 weeks ("first-line successes") was set as 80% (ranging from 70% to 90%) [9,10,12–14]. The patients who did not meet LDL-C goal reductions were classified into one of three groups: 1) failed goal and terminated therapy (40% of first-line failures, i.e., those 20% not meeting initial LDL-C goals), 2) met goal after a dose change and an additional 12 weeks of therapy (40% of first-line failures), and 3) failed goal after an additional 12 weeks of therapy (20% of first-line failures). For this OBA structure, the manufacturer was to absorb financial responsibility for all PCSK9 inhibitor medication costs until the LDL-C goal was achieved, whereas the payer was responsible for medication costs afterward. Thus, the manufacturer was responsible for 12 weeks of therapy costs for 80% of the population and 24 weeks for the remaining 20% of the population (those requiring additional therapy before reaching goal or terminating therapy). Thereafter, the payer would reimburse the manufacturer at the negotiated price. Total cost of participation (i.e., responsibility for medication costs) was calculated for both manufacturers and payers at a 1-year time period.

The second model structure (model 2) included ASCVD-related outcomes occurring after treatment initiation and up to 1 year of follow-up. An expected rate reduction of these events was modeled for three different scenarios: 1) when the use of the medication was associated with a predefined outcome event rate goal, 2) when the outcome event rate reduction exceeded the predefined expectations ("overperformance"), and 3) when the outcome event rate failed to meet expectations ("underperformance"). The baseline event rate was determined on the basis of the manage care organization's population. Expected reductions of 15% (range 10%–20%) in outcome events were estimated on the basis of recently published data for the Further Cardiovascular Outcomes Research with PCSK9 Inhibition in Subjects with Elevated Risk (FOURIER) trial [14]. For the payment structure in model 2, a tiered system was developed that linked reimbursement to the three outcome event rate scenarios described previously. Reimbursement for the product was reduced by 30% to 70% if the medication underperformed, and allowed for increased reimbursement of 105% to 125% if the medication overperformed. Reimbursement rates remained flat if the reduction in event rates met expectations.

Medication costs were assumed to be $1,167 per month, or $14,000 annually, on the basis of estimated prerebate costs for PCSK9 inhibitors currently on the market [15]. Costs were calculated with an assumed adherence of 100% to therapy. Although this is unlikely in real-world situations, we assumed the simplest case considering that no real-world adherence data are available for our case example of PCSK9 inhibitors. Moreover, lower adherence rates would result in proportionally lower cost estimates (e.g., 80% adherence would result in 80% of medication costs). Because of the lack of real-world evidence on the exact relationship between adherence levels and effectiveness, the effect of lower adherence on effectiveness could be assumed to be proportional as well. Many existing OBAs have reportedly used predefined cohort inclusion criteria specifying minimum adherence to therapy to assess the agreed upon outcomes of interest. Estimated medical offsets due to a reduced rate of ASCVD events were also included in the payer value calculations in model 2. These offsets were calculated on the basis of annual hospitalization rates for MI, stroke, and unstable angina, as well as coronary artery bypass graft and percutaneous coronary intervention procedures, taken from the Humana Research Database. The derived rate estimates were then multiplied by the incremental 1-year cost of care for each type of event, including follow-up outpatient care, on the basis of available literature (see Appendix Table 3 in Supplemental Materials found at http://dx.doi.org/10.1016/j.jval.2017.07.009) [8]. For each model, payer costs were calculated as total costs per treated patient, and payments to manufacturers were defined as the total reimbursement paid to the manufacturer.

In each model, two simulations were included: "without OBA" and "with OBA." We assumed that payers who implemented an OBA with a pharmaceutical manufacturer would deploy fewer barriers to access the medication because the uncertainty regarding the medication's value was shared between both parties. Thus, a scenario in which no OBA was present, formulary restrictions, previous authorizations, higher patient co-payments, or other barriers would be in place, which meant that fewer patients would have access to the medication. These considerations incorporate either of the following two general scenarios: 1) a medication is first to market or is unique in its class or 2) a medication has one or more comparators, with the manufacturer competing for market share. In scenario 1, restrictions to market access for a given patient population may be due to the nonformulary status of the drug. For scenario 2, tiered formularies may influence market share, and a relative position versus competitors is also important to consider. These models