

# Outcome-based pricing for new pharmaceuticals via rebates

Elodie Adida

School of Business, University of California Riverside, elodie.goodman@ucr.edu,

The price of new brand-name prescription drugs has been rising fast in the US. For example, the Amgen cholesterol drug Repatha had an initial list price of \$14,523 per year. Patients, even with insurance coverage, must pay out-of-pocket a significant portion of this price. The treatment might not be successful, and this possibility reduces risk-sensitive patients' incentives to purchase the drug. The high price together with the chance of negative treatment outcomes may lead payers to deny coverage for the drug. Outcome-based pricing has been proposed as a way to re-allocate the risks and improve both payer resource allocation and patient access to drugs. According to an outcome-based rebate contract between Amgen and Harvard Pilgrim Health Care, if a patient on Repatha suffers a heart attack or a stroke, both patient and insurer are refunded the cost of the drug. We use a stylized model to analyze the effect of outcome-based pricing via rebates. Our model captures the interaction between heterogeneous, price-sensitive, risk-sensitive patients who decide whether to purchase the drug; a payer deciding whether to provide coverage for the drug; and a price-setting pharmaceutical firm seeking to maximize expected profits. We find that in many cases, a pharmaceutical firm and payer cannot simultaneously benefit from outcome-based pricing, and who will benefit is determined by the probability of treatment success. Outcome-based pricing thus appears unlikely to solve the issues of high drug prices and high payer expenditures. However, supplementing outcome-based pricing with a transfer payment from firm to payer can make payer and firm (but not necessarily the patients) better off than under uniform pricing when the drug has a low chance of success.

*Key words:* Health care, Pharmaceuticals, Drug pricing, Pay-for-performance, Risk sharing.

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## 1. Introduction

### 1.1. Background and Motivation

The cost of prescription drugs has steadily risen over the past few decades. According to the Centers for Medicare & Medicaid Services (CMS) National Health Expenditure Accounts, in 2016 the US spent \$328.6 billion on retail prescription drugs, or 1.8% of GDP (CMS 2018). The US spends significantly more than other countries on pharmaceuticals (Papanicolas et al. 2018). High drug prices are gaining national attention as they impose a heavy burden on insurers and threaten access to certain drugs for many Americans, even for those with insurance coverage. According to CMS data, while private insurance and CMS programs bore a large portion of the total spending

on retail prescription drugs in 2016 (43% and 39%, respectively), patients paid out-of-pocket 14% of the total spending. High prescription-drug spending can be explained both by the increasing price of existing drugs and by the emergence of new drugs that are costly to develop. This paper focuses on new drugs. New branded medicines spending has grown by \$12 billion in 2017, more than double that generated by price increases in existing drugs (\$5.2 billion) (IQVIA 2018)[Chart 4]. Even accounting for the growth in volume of sales of existing drugs (\$1.5 billion), the effect of new drugs remains largely dominant over that of existing drugs.

For expensive drugs that offer only a marginal effectiveness improvement over existing ones, payers may be reluctant to provide coverage. Food and Drug Administration (FDA) approval requires that the drug's benefit (or potential benefit) over available treatments outweigh the risks, but the drug rarely works for all patients. Facing this trade-off between high cost and a limited gain in clinical benefit for only a fraction of patients, payers may decide to deny coverage. For example, Exondys 51, a drug indicated to treat Duchenne muscular dystrophy, was approved in 2017 based on limited evidence of efficacy and was priced at \$300,000 per year; Anthem declined to cover the drug, while Humana decided to cover it for certain patients only (Gellad and Kesselheim 2017).

The high price of drugs may cause patients to forgo taking a drug they need. In 2015, 24% of Americans taking prescription medicine did not fill a prescription because of the cost (KFF 2015). Patients often exhibit a risk-sensitive, loss-averse behavior (e.g., Rasiel et al. 2005). The risk of incurring a high out-of-pocket cost without necessarily obtaining the warranted health benefits further reduces the patients' incentive to pay for the drug (Brody 2017).

Outcome-based pricing has been proposed as a new paradigm to pay for pharmaceuticals with uncertain outcomes. The basic idea is to re-allocate the risks by making payment contingent on whether the drug works, instead of based only on volume. In 1998, Merck agreed to refund up to 6 months of treatment costs if simvastatin (Zocor) plus diet did not help patients lower LDL cholesterol to target concentrations identified by their doctors (Carlson et al. 2009). In 2006, Johnson and Johnson agreed to refund the UK National Health Services for multiple myeloma patients who fail to respond after 4 cycles of bortezomib (Velcade) (Neumann et al. 2011). In 2017, Amgen Inc. agreed to a full refund if patients taking a cholesterol-lowering drug (Repatha) suffer a heart attack or stroke. In the same year, Novartis agreed to refund CMS the price of a \$475,000 childhood leukemia drug (Kymriah) if patients do not respond within a month of treatment (Loftus 2017). Other examples of full-refund contracts can be found in Møldrup (2005)[Box 2]. Variants of this pricing model have been used in the US in a few relatively isolated instances since the mid 1990s, and more broadly in Europe. They yielded mostly disappointing results in Italy (Navarria et al. 2015, Thomas and Ornstein 2017). Yet, the current US administration is reportedly considering a similar approach to tackle high drug prices. A draft executive order prepared in June 2017

includes a value-based pricing proposal (Kaplan and Thomas 2017). CMS announced in August 2017 that it is working on innovative payment arrangements for new treatments, arrangements that “may, for example, include outcome-based pricing for medicines in relation to clinical outcomes” (CMS 2017). The Department of Health and Human Services released in May 2018 a blueprint to lower drug prices and reduce out-of-pocket costs (HHS 2018). Some of the proposals emphasize the need for a “value-based transformation of our entire healthcare system” including by “hold[ing] manufacturers accountable for outcomes”.

Outcome-based pricing offers patients and payers the advantage of holding the pharmaceutical firm accountable for the clinical outcome of the drug treatment. It shifts some of the risk of failure to the firm, reducing for patient and payer the financial risk of paying for an ineffective drug. Because of a lower risk exposure, payers may be more likely to offer coverage for new drugs despite the limited effectiveness, if the price is not too high, which could improve patients’ access to these drugs. Outcome-based pricing also offers advantages for pharmaceutical firms. Given the current growing pressure to justify the high prices, such a pricing scheme allows for more transparency on the value of new pharmaceuticals. Outcome-based pricing can also help increase a drug’s sales by improving patient adoption. However, if pharmaceutical firms set a price that is commensurate with the risk they take on, prices could rise. High prices may discourage payers from offering coverage. Even if the payer offers coverage, the high price may make the drug unaffordable to patients, affecting the patients’ access and out-of-pocket spending, as well as the payer’s expenditures.

## **1.2. A Case Study: Repatha**

To provide a concrete setting, we frame the problem studied in this paper in the context of the drug Repatha. Repatha (evolocumab) is a cholesterol drug, in the category of PCSK9 inhibitors, made by Amgen. It had an initial list price of \$14,523 per year and is generally administered for life in addition to a standard statin therapy (Fonarow et al. 2017). The drug was FDA-approved in 2015 based on clinical trials finding that the drug sharply reduced cholesterol levels. The Harvard Pilgrim Health Care insurer agreed to cover the drug in exchange for a discount and potential rebates if the treatment failed to meet performance targets. A randomized, double-blind, placebo-controlled clinical trial (“FOURIER”) was launched to evaluate the effect of PCSK9 inhibitors on hard clinical endpoints. Researchers found that using evolocumab lowered cholesterol levels but resulted in only a modest reduction in cardiovascular events; there was no overall or cardiovascular-specific mortality benefit (Sabatine et al. 2017). These results fell short of high expectations: a cost-effectiveness study found that the annual net price would need to be substantially lower to meet generally accepted cost-effectiveness thresholds (Fonarow et al. 2017). In 2017, upon publication of the FOURIER study, Amgen and Harvard Pilgrim agreed to an outcome-based refund contract

for Repatha. According to this contract, if a patient is hospitalized due to a heart attack or stroke after taking Repatha for six months or more and maintaining an appropriate level of compliance on the drug, both patient and insurer are refunded the cost of the drug (Amgen 2017).

Consider the decisions that risk-sensitive patients with a prescription for Repatha are facing. Because of the high price of the drug together with the limited clinical effectiveness, patients face a difficult dilemma: is the drug worth it? Navar et al. (2017) find that 35% of patients given a prescription of a PCSK9 inhibitor and approved by their insurance did not fill the prescription. Paying for such an expensive drug may represent a significant pressure on the patient's finances. Meanwhile, undergoing drug treatment may provide the benefit of preventing a heart attack or a stroke that would have occurred under standard statins treatment. However, there is no guarantee that the drug will achieve this goal. For each patient, the decision of whether or not to buy the drug is extremely complex and is based on a large variety of idiosyncratic factors. Yet, it is reasonable to expect overall that the cumulative demand for the drug decreases with the price. Under uniform pricing, the patient must pay to obtain the drug even if she eventually suffers a heart attack or stroke, hence, the patient's risk attitude plays a role. Under the full-rebate outcome-based refund contract, patients do not bear any financial risk. Obtaining a specific form for the demand function requires a decision-making model for value-maximizing risk-sensitive patients.

Next, consider the decision that the payer is facing. The payer's role is to ensure the health of its patient population while keeping costs under control. After the launch of a new, expensive drug, the payer must decide whether to cover it. Denying coverage avoids incurring costs, but hurts the patients as fewer will afford purchasing a potentially helpful drug. Approving coverage might incur high costs due to the payer's cost share for each filled prescription. However, if the drug works and reduces the rate of heart attacks and strokes, the payer's benefit is two-fold: first, it benefits indirectly from the patients' better health status, and second, it will face lower future health care costs. Consider now the decision that the pharmaceutical firm, Amgen, is facing. When setting a price for the drug, the firm must balance its own profit margin with the effect that the price has on the payer's coverage decision and on demand from price- and risk-sensitive patients. Under a uniform pricing scheme, where the firm receives payment regardless of treatment outcomes, the firm does not bear any risk. Under full-rebate outcome-based pricing, the firm receives payment only for patients who do not suffer a heart attack or stroke, which could yield a higher price.

The effect of implementing outcome-based pricing on the patients, the firm and the payer is intuitively unclear. The patient demand may increase because, without financial risk, more patients may choose to purchase the drug if it is covered. The patient demand may also decrease if the price is higher or because of a lack of coverage due to too high costs for the payer. Even if the demand

is improved, the patient welfare may not be if the price is too high. A higher price improves the profit margin for the firm, but the requirement to refund patients who suffer a heart attack or stroke could lower the firm's profit. For the payer, with coverage, a higher price raises expenses for each successful treatment, but the refund for unsuccessful treatment could contribute to lowering the total cost. To determine quantitatively the effect of outcome-based pricing, we introduce in Section 2 a stylized model that captures some of the key aspects of the problem to derive insights.

Outcome-based pricing is viable only if it benefits both contractual parties. If one of the parties fares worse under outcome-based pricing than under uniform pricing, they can be incentivized to enter the contract if the other party shares some of its gain. We examine in Section 3 whether outcome-based pricing can advantage both the firm and the payer simultaneously, or if a win-win arrangement can be designed to improve its performance.

### 1.3. Contributions and Literature Review

Our goal is to analyze the effect of outcome-based pricing for new pharmaceuticals on patients, payer and pharmaceutical firm. We propose to answer the following research questions: (1) Are the firm, the payer, and patients better or worse off under outcome-based pricing? Are there any drug characteristics that make outcome-based pricing more beneficial to each stakeholder? (2) Can outcome-based pricing be modified to improve its performance? The answers to these questions have implications for both health-policy makers and pharmaceutical industry leaders. From a health-policy design perspective, outcome-based pricing contracts are practical only if they improve upon the traditional pricing system for *both* parties involved. For payers, it is critical that a new pricing system help control expenditures and benefit patients. For pharmaceutical firms, an indication of the type of drugs for which outcome-based contracts have the potential to improve profits would help drive contractual negotiations and make long-term innovation investment decisions.

In this paper, motivated by the case of Repatha, we introduce a stylized analytical model to determine the effect of outcome-based pricing for new brand-name pharmaceuticals. Our model captures the interaction between heterogeneous price- and risk-sensitive patients who decide whether to purchase the drug; a payer deciding on whether to provide coverage for the drug despite its limited success rate; and a price-setting pharmaceutical firm seeking to maximize expected profits. We consider both a traditional uniform pricing system, where payment is required to obtain the drug, as well as a full-rebate outcome-based pricing system, where the firm receives no payment when the treatment does not achieve a pre-specified result (we study the case of outcome-based pricing with partial rebate in Appendix C). We investigate how the drug pricing scheme affects the patients' access and welfare, the payer's coverage decision, spending and overall benefit, and

the pharmaceutical firm's profit. Our aim is to understand under what conditions outcome-based pricing could benefit the different stakeholders as compared with a uniform pricing system.

The health policy literature discusses qualitatively the role of outcome-based pricing for pharmaceuticals, identifying barriers to adoption and possible solutions (Garber and McClellan 2007, Neumann et al. 2011). Outcome-based pricing for pharmaceuticals has been used since the mid 1990s in several countries (Carlson et al. 2010) but the literature on ex-post evaluation is limited (Garrison Jr. et al. 2013). The sparse evidence indicates that the impact on containing costs was mixed at best, and in some cases negligible (Navarria et al. 2015). In contrast, we adopt a model-based analytical approach to evaluate outcome-based pricing. An increasing volume of healthcare operations literature evaluates quantitatively the role that payment systems play in realigning incentives among payer, providers and patients (e.g., Jiang et al. 2012, Andritsos and Tang 2018).

The health economics literature has studied some aspects of risk sharing agreements. Lilico (2003) analyzes the patient/payer welfare under uniform pricing and under risk-sharing, taking risk aversion into account, when the price is set so the manufacturer earns zero profit. The author finds that the patient prefers risk-sharing. Barros (2011) studies risk-sharing between a drug manufacturer and a payer who decides which patients get the treatment when the price is the same across pricing systems. He finds that payer and drug manufacturer are better off under outcome-based pricing as long as the manufacturer does not anticipate the agreement. Antonanzas et al. (2011) consider a similar setting but with a price set using Nash bargaining. They obtain that whether the health authority prefers risk-sharing depends on the trade-off between monitoring costs, production cost and utility from treatment. Mahjoub et al. (2018) extend Barros (2011) by allowing the manufacturer to adjust prices when risk-sharing is used and the payer to set the rate of rebate. They find that the manufacturer earns no profit under the risk-sharing agreement regardless of the price. In these papers, the payer decides which patients obtain the drug to maximize a combination of payer and patient benefit. In a recent healthcare operations working paper, Xu et al. (2019) study the insurer's formulary design. They find that outcome-based rebates have no effect when the insurer is risk-neutral. When the insurer is risk-averse, the manufacturer earns a higher profit and the insurer's spending increases with outcome-based rebates. Olsder et al. (2019) study a variety of mechanisms to improve access to rare disease treatments, including outcome-based pricing, in the presence of government subsidies. Consistent to our work, they numerically find that outcome-based pricing can result in higher prices. Yapar et al. (2019) consider risk-sharing agreements where the price depends on post-marketing data. Our paper contributes to this literature in three main ways. First, our model captures patient choice with regards to obtaining treatment or not, based on the price, co-insurance rate, risk attitude, and heterogenous benefit

from treatment. Hence, we investigate whether each category of agent (firm, payer, patients) benefits from outcome-based pricing, where each agent makes its own optimal decision (respectively, price, coverage, drug purchase). Second, we identify the key role played by the chance of treatment success on the performance of risk-sharing mechanisms as compared with uniform pricing for the firm, payer and patients – an insight not revealed in the literature to date. Third, we study how to modify outcome-based pricing (e.g., via transfer payments) to improve its performance.

Our work is also related to the marketing literature on money-back guarantees. Money-back guarantees can be used as a way to give a signal on product quality when consumers cannot directly assess quality before purchase (Moorthy and Srinivasan 1995). In this literature, a money-back guarantee means that the buyer can return the product for any reason and get her money back. In retail, money-back guarantees offered by retailers can help enhance the store image, and their cost is alleviated by suppliers taking back returned merchandise for a full or partial refund. In some cases, money-back guarantees may increase the retailer's profits by encouraging consumers to try new products, hence, increasing sales volume (Davis et al. 1995). Furthermore, they may allow the retailer to charge higher prices because the reduced risk for the consumer increases her willingness to pay. However, consumers may try to free-ride by extracting some utility and returning the product after usage. Outcome-based pricing for pharmaceuticals presents some similarities with money-back guarantees in the retail industry. There are also some key distinctions: the patient does not *choose* to return the drug, as treatment success is outside the patient's control; the patient cannot take advantage of free-riding; there is no salvage value in case of treatment failure; and the total surplus is influenced by the cost incurred by the payer, who bears a portion of the cost.

Our results shed light on whether outcome-based pricing holds promise as a pricing system for drugs. We find analytically that when the payer's direct benefit from treatment success is high, the firm and payer cannot simultaneously benefit from outcome-based pricing, and who will benefit is determined by the probability of treatment success. If this probability is low (i.e., the drug is "high-risk"), the firm benefits; otherwise the payer does. Therefore, outcome-based pricing is unlikely to provide the solution to the issue of high payer expenditures. However, we show that for a high-risk drug, an outcome-based contract enhanced with a transfer payment can simultaneously benefit the firm, payer, and sometimes also the patients. Nevertheless, the firm and the payer's interests may be aligned when the payer's direct benefit from treatment success is very high. We assess numerically the robustness of these results when the assumption of high payer's direct benefit from treatment success is relaxed. We generate a wide range of scenarios by varying all input parameters. We find that, as long as the payer offers coverage under both pricing systems (i.e., the payer's direct benefit is not too low and/or the cost not too large), our results on price, demand, firm, payer and transfer

payment remain valid in 80-100% of the scenarios considered. Interestingly, outcome-based pricing may reduce the payer’s incentives to provide coverage due to a sharp demand expansion and price increase. Outcome-based pricing may also reduce the patient welfare despite a higher demand, because of the high price and possible lack of coverage. Furthermore, we observe that patient risk sensitivity and loss aversion, when high, act as a barrier to drug access.

## 2. Model

We consider the interaction between a pharmaceutical firm (e.g., Amgen) producing a new patented brand-name drug (e.g., Repatha), a payer, and a population of  $n$  patients who were prescribed the drug and are covered by the payer. The firm incurs a variable cost  $c$  for producing the drug (the research and development fixed costs are considered to be sunk costs and thus do not influence the firm’s decision-making in this stage). The firm selects the price  $p$  for the drug. Following the price announcement, the payer decides whether or not to cover the drug. If the payer covers the drug, patients who purchase the drug must pay a fraction  $\beta \leq 50\%$  (co-insurance rate) of the price, while the payer pays the rest. (KFF (2016)[Exhibit 9.4] shows that in practice, co-insurance rates are less than 50%.) If the payer does not cover the drug, a patient who chooses to purchase it anyway is responsible for the entire price. We denote  $\bar{\beta}$  the patient cost share, i.e.,  $\bar{\beta} = \beta$  when the payer chooses to provide coverage for the drug, and  $\bar{\beta} = 1$  otherwise (for ease of exposition, we omit to explicitly include the price argument for  $\bar{\beta}$ ). The drug effectiveness is limited and a given patient (and her physician) cannot accurately predict whether the drug will work on her before undergoing treatment. For each patient that the drug is prescribed to, there is a chance  $q \in (0, 1)$  that the drug achieves a pre-defined goal (i.e., the treatment “succeeds”). For Repatha, treatment success is defined as not suffering a heart attack or stroke. If the treatment succeeds, the patient surplus is determined as the additional value gained over the value of the standard treatment (e.g., statin therapy). To capture patient heterogeneity, we model the value gained by each patient as a random variable,  $V$ , uniformly distributed on  $[0, \bar{v}]$ . The realization of  $V$  may depend on the treatment options available to the patient, illness severity, opportunity cost of being ill, other medications the patient is currently taking, past treatments and outcomes, tolerance to side-effects, demographics, comorbidities, etc. This information is available to the patient; thus, in our model each patient observes her own realization  $v$  of value  $V$  before deciding whether to purchase the drug. If the treatment fails or if the patient does not purchase the drug, the patient surplus is zero as she resorts to the standard treatment. Likewise, if the treatment succeeds, the payer receives a fixed surplus  $v'$  over the standard treatment case. (Appendix F considers the case of  $v'$  heterogeneous across patients and perfectly correlated with  $v$ . We consider the case of a constant  $v'$  in the main body to be consistent with the literature (e.g., Mahjoub et al. 2018) and to capture the fact that the



payer does not have information on patient idiosyncracies – potential loss of income, presence of dependents, personal tolerance of side effects – that affect a patient’s value from treatment success, and thus would instead use the value from a “generic” patient.) The value  $v'$  may include direct cost savings for the payer due to a reduction in future healthcare needs, indirect cost savings for society due to avoiding a loss of productivity, as well as a mission-driven benefit to the payer from a beneficiary’s good health status. Introducing this key parameter captures the trade-off for the payer between the high cost of a drug and its potential for providing a benefit to the payer and society. Table 4 in Appendix A summarizes the notation.

Several comments are in order. First, the premise of outcome-based pricing is that the outcome of the drug treatment can be objectively observed, and the payer and the firm have agreed in advance on what clinical endpoints define a “success”. For Repatha, according to the contract with Harvard Pilgrim, the treatment is deemed successful if the patient does not suffer a heart attack or stroke while taking the drug. Using examples from other existing outcome-based contracts, for a diabetes drug such as Januvia, Janumet (Merck & Co.) or Trulicity (Eli Lilly), success means bringing blood-sugar levels below a pre-specified target (Neumann et al. 2011). For a cancer drug such as Velcade (Johnson & Johnson), success is measured by a reduction of at least 50 percent in serum M protein, a biomarker for disease progression, within the first 4 months of treatment (Carlson et al. 2010). Outcome-based pricing would arguably be problematic to implement on endpoints that are more ambiguous to measure, such as fatigue or mental decline.

Second, in reality, the patients’ decision-making regarding the treatment route involves a complex balancing of many criteria. We summarize the treatment success benefit into a value  $v$  that patients estimate. This value represents the patient’s surplus gain over the standard treatment upon achieving the clinical endpoint defined as “success”. This approach recalls that of cost-effectiveness studies that use the concept of QALYs (quality-adjusted life-years) to assess quantitatively the benefits of medical interventions. Fonarow et al. (2017)[Table 3] evaluate the average lifetime *incremental* QALY gained from the Repatha treatment over only statins at 0.39. Using the common valuation of \$150,000 per QALY (Anderson et al. 2014), the drug provides an average incremental value of \$58,500. In our model where patients’ individual surplus from success (over the standard treatment) are uniformly distributed on  $[0, \bar{v}]$ , the average gain from taking the drug is  $q\bar{v}/2$ . Estimating  $q = 26\%$  (Fonarow et al. 2017)[eTable1] implies that for Repatha,  $\bar{v} = \$450,000$  and so the surplus gain over the standard treatment is uniformly distributed on  $[0, \$450,000]$ .

Third, we measure both the patient and the payer’s surplus in case of treatment success with respect to the status quo, i.e., the standard treatment (e.g., statin therapy), and we assign the same zero surplus in case of treatment failure as for the status quo. This assumption is in line with the

literature, e.g., Barros (2011). It amounts to assuming that, from a medical perspective (exclusive of financial considerations), the drug treatment is no worse than the standard treatment. For the case of PCSK9 inhibitors, Husten (2018) cites prominent cardiologists supporting this assertion, by stating that “if the drug was very cheap then we would use it in everybody”; “PCSK9 inhibitors are safe and effective”. It is important to observe that the model fits not only drugs that provide better prevention than the standard treatment, but also some curative drugs, for which success is defined as a positive reaction (e.g., tumor size decreasing for cancer, blood sugar brought below a target for diabetes), and failure as the absence of a reaction within a certain time frame. If the treatment fails, the patient can revert to the standard treatment with no other loss – beside the monetary expense – than the (usually relatively short) time spent attempting the new treatment. For example, the drug Kymriah (tisagenlecleucel) by Novartis is used in patients whose cancer has not gotten better with other treatment or has relapsed two or more times (NCI 2019). In 2017, Novartis entered an agreement with CMS so that the company will only be paid for the drug if patients respond to it by the end of the first month following the one-time treatment (Loftus 2017). Focusing our work on this category of drugs allows us to better disentangle the trade-off faced by the patient: without any medical downside of the drug compared to the standard treatment, they must decide whether the high price is worth the potential clinical benefit.

Fourth, we assume that the chance of success,  $q$ , is known. In practice, it is difficult to estimate it precisely. The results of closely monitored medical trials administered in a carefully selected patient population are not always reproduced in a less controlled environment and in a general population. We examine the consequences of jointly mis-estimating the chance of success in Appendix D.

Fifth, the model also assumes that the co-insurance rate,  $\beta$ , is fixed and the payer solely decides whether to cover the drug or not. When covered, new brand-name drugs are usually included in the last tier in the formulary, for which the co-insurance rate is already set and applies to all drugs in this tier. Torrey (2018) states that the last tier, which corresponds to specialty drugs, is usually for drugs that “are newly approved pharmaceutical drugs that your payer wants to discourage because of their expense”. For Repatha, we found that “Medicare plans typically list Repatha in Tier 5 of their formulary. Tier 5 drugs are usually non-preferred brand-name drugs” (GoodRx 2019).

Sixth, we consider that the firm is a monopolist (Appendix E considers a model of symmetric duopoly competition). We justify this assumption in the context of a new drug due to three factors. (i) *Patent protection*. During the phase from the FDA approval until the patent protection expires (which lasts generally about 8-10 years), the drug often has little to no competition. Repatha holds nearly 70% of the PCSK9 market (Pagliarulo 2019). (ii) *Uniqueness of biologic drugs*. Biologic drugs (e.g., Repatha) cannot be replicated (Cancer Treatment Centers of America 2018). Biosimilars are

comparable but not chemically identical to their name-brand counterparts. IQVIA (2018) shows that in 2016, \$102.3 billion was spent on biologic drugs, of which only 3% was for drugs subject to biosimilar competition – and for those drugs, biosimilars achieved only 10% of the sales. Indeed, the FDA has only approved 12 biosimilars to date, only three of which being actively marketed (HRI 2018). This indicates that biologic drugs are often not subject to intense competition. (iii) *Lack of competition in some pharmaceutical markets.* Even synthetic drugs that are off-patent often encounter little competition from generics. According to Fox (2017), “drug companies are thwarting competition through a number of tactics, and the result is high prices, little to no competition, and drug quality problems”. AAM (2018) states that almost 80% of the 100 best-selling drugs extended their monopoly protection at least once. Therefore, we consider a monopoly setting in our model.

We acknowledge, however, that in some cases, competitive forces may exert a role in the pricing decision. For example, there exists a rival drug to Amgen’s Repatha, named Praluent, made by Sanofi/Regeneron. The two drug makers have been in a (still unresolved) patent infringement legal battle since 2014. Both have recently reduced their price after disappointing initial sales to improve access to their drug. However, after the pharmacy benefit manager Express Script and Sanofi/Regeneron agreed to a lower price on Praluent, Amgen estimated that “the Express Scripts formulary decision will impact 2,000, or 6%, of Repatha patients” (Gatlin 2018), indicating that the competition with Praluent has a moderate intensity. We consider a monopoly setting to focus on the effect of outcome-based pricing in the absence of competitive forces. However, we emphasize the lack of a comprehensive treatment of competitive effects as a limitation of the generality of our findings. The study of how competition affects the performance of outcome-based pricing (beyond the simple model presented in Appendix E) represents an interesting direction of future research.

We compare two pricing systems. In a traditional uniform pricing system, the pharmaceutical firm charges based on volume: any patient buying the drug pays the fraction  $\bar{\beta}$  of the drug price, and the payer is responsible for the remainder, regardless of treatment outcomes. In an outcome-based pricing system with full rebate (we study the case of outcome-based pricing with partial rebate in Appendix C), the firm charges based on performance: it receives payment only when the treatment is successful. If the treatment is successful, the price is split among the patient and the payer as under uniform pricing, according to the rate  $\bar{\beta}$ . The timeline of events is as follows. First, the firm announces the price. Next, the payer decides whether to cover the drug. The patient then estimates her own potential value  $v$  from treatment, and decides whether to purchase the drug. If she purchases the drug, it may succeed, or fail. Under uniform pricing, for each patient purchasing the drug, the firm receives  $p$  and incurs cost  $c$ . The payer cost is  $(1 - \bar{\beta})p$ , and the payer gains a surplus  $v'$  over the standard treatment in case of treatment success. The patient cost is  $\bar{\beta}p$ , and

**Figure 1** Patient decision tree and payoffs

		Payoffs					
		Uniform pricing			Outcome-based pricing		
		Patient	Insurer	Firm	Patient	Insurer	Firm
	Drug purchase	$v - \bar{\beta}p$	$v' - (1 - \bar{\beta})p$	$p - c$	$v - \bar{\beta}p$	$v' - (1 - \bar{\beta})p$	$p - c$
	Failure	$-\bar{\beta}p$	$-(1 - \bar{\beta})p$	$p - c$	0	0	$-c$
	No drug purchase	0	0	0	0	0	0

Note:  $\bar{\beta} = \beta$  if the payer chooses to provide coverage, and  $\bar{\beta} = 1$  otherwise.

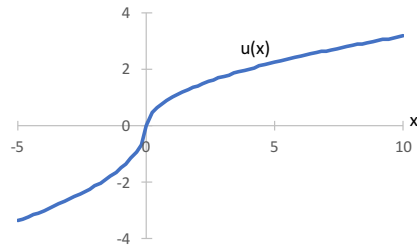
the patient gains surplus  $v$  in case of treatment success. Under outcome-based pricing, the firm receives  $p$  in case of success, and 0 in case of failure, in addition to incurring cost  $c$ ; the payer gains  $v' - (1 - \bar{\beta})p$  in case of success, and 0 in case of failure, while the patient surplus is  $v - \bar{\beta}p$  in case of success, and 0 in case of failure. The payoffs are illustrated in Figure 1.

To capture the effect of different payment mechanisms on risk allocation, consistent with prospect theory, we model the patient value function as perceived with respect to a reference point (the status quo, i.e., the standard treatment), with a concave shape for gains and a convex shape for losses, and with a steeper slope for losses than for gains (Kahneman and Tversky 1979). A patient generally makes decisions regarding expensive drugs on a relatively small number of occasions over a lifetime, and is likely sensitive to the risk of paying a large amount of money and possibly incurring losses for a drug that does not work for her. Prospect theory frames behavior as risk averse for gains, and loss averse, but risk-seeking, for losses (Kahneman and Tversky 1979). Prospect theory has been applied to medical decision-making, using the patient's current health status as the reference point (Treadwell and Lenert 1999, Lenert et al. 1999, Rasiel et al. 2005). Accordingly, we use a power function to model the patient value when receiving payoff  $x$ :

$$u(x) = \begin{cases} x^\alpha & \text{if } x \geq 0 \\ -\lambda(-x)^\alpha & \text{if } x < 0, \end{cases}$$

where  $0 < \alpha < 1$  and  $\lambda \geq 1$ . This S-shaped value function captures risk aversion over positive payoffs and risk-seekingness together with loss aversion over negative payoffs (Tversky and Kahneman 1992). The value function is illustrated in Figure 2. The limit case of risk-neutral patients can be obtained when  $\alpha$  approaches 1, while a larger intensity of risk aversion for gains (and of risk-seekingness for losses) is represented by a smaller  $\alpha$ . Hence, we refer to the intensity of patient risk sensitivity as  $1 - \alpha$  ( $> 0$ ), which we assume to be common to all patients. Furthermore,  $\lambda$  characterizes the intensity of the patients' loss aversion, also assumed to be common to all patients. A value of  $\lambda$  above 1 captures the fact that the patient response to a loss tends to be more extreme than the response to a gain of the same amplitude (Tversky and Kahneman 1986).

**Figure 2** Patient value function with  $\alpha = 0.5$  and  $\lambda = 1.5$



In contrast, we model the firm and the payer as risk neutral. The firm selects the price to maximize expected profits. The payer decides whether to provide coverage for the drug or not. Both the payer and the firm serve a large population of patients. Therefore, they benefit from risk-pooling effects that make them somewhat immune to large variations and justify a risk-neutral approach. This assumption is consistent with the health economics and the healthcare operations literatures (Barros 2011, Jiang et al. 2012, Lee and Zenios 2012, Adida et al. 2017).

The payer's role is two-fold: it must keep its financial costs down while maintaining the health and welfare of the patient population. To capture this dual role, we introduce an objective function for the payer that comprises both (i) the financial cost and possible payer benefit of covering the drug, and (ii) the cumulative expected patient payoff. Hence, in our model the payer internalizes both its own costs and benefits, as well as those of the patients. This modeling choice for the payer's objective is consistent with the literature (Barros 2011, Andritsos and Tang 2018, Mahjoub et al. 2018, Guo et al. 2019). We model the payer's objective using the patient expected payoff rather than the cumulative patient welfare for three reasons. (i) From a practical perspective, due to the patient's risk-sensitivity, the patient welfare has an arbitrary scale and a different unit than the payer's payoff, and so adding these quantities does not make physical sense. To combine them, we would need to include a multiplying factor that would be hard to estimate. (ii) It may be seen as reasonable to assume that, in its goal to optimize the net social benefits, the payer evaluates the objective, risk-neutral benefit to society by evaluating the expected patient payoff. (iii) Consider the system comprising the payer and the patients. As explained above, we model the objective of the payer as that of maximizing some measure of the welfare of this system. In this system, the payer is risk-neutral and the patients are sensitive to risk. In the context of healthcare payments, Adida et al. (2017) discuss the challenges of defining the objective of a social planner when some agents in the system are sensitive to risk. They refer to the literature on group decision theory and supply chain management, and they use the concept of Pareto optimality as a criterion. They show that when at least one agent of the system is risk-neutral, finding the Pareto-optimal outcome is equivalent to maximizing the system's expected payoff, regardless of the patients' utility functions. We follow a similar approach by assuming that the payer maximizes this system's expected payoff.

We provide in the next section a precise mathematical expression for the total expected patient welfare, the objective of the firm and the objective of the payer under the two pricing systems that we consider in the paper. We make the following assumption.

ASSUMPTION 1. Let  $z \equiv 1 + \left(\lambda \frac{1-q}{q}\right)^{1/\alpha}$  ( $> 1$ ). We assume that  $\beta c/\bar{v} < \min\{q, 1/z\}$ .

While  $z$  depends on  $q$ , for ease of exposition we omit the argument. Consider a drug that costs 1 to the patient and has a probability  $q$  of success. Quantity  $z$  satisfies the equation  $q \cdot u(z-1) + (1-q) \cdot u(-1) = 0$ . Hence,  $z$  is the minimum value gain in case of success that is required for the patient to choose to purchase the drug under uniform pricing (see Figure 7 in Appendix B). For the indifferent patient, the risk premium is the expected gain, i.e.,  $q(z-1) - (1-q) = qz - 1$ . Let

$$q_0 \equiv \frac{1}{1 + \lambda^{-\frac{1}{1-\alpha}}}.$$

Note that  $q_0 = 50\%$  when  $\lambda = 1$ , and  $q_0 > 50\%$  when patients are loss-averse (i.e.,  $\lambda > 1$ ). Lemma 1 in Appendix G shows that the risk premium  $qz - 1$  is positive if and only if  $q < q_0$ .

DEFINITION 1. We refer to a drug as “high-risk” when  $q < q_0$ , and as “low-risk” when  $q > q_0$ .

Assumption 1 ensures that, when the payer provides coverage for the drug, the drug can be profitable under either uniform pricing or outcome-based pricing. If the condition of Assumption 1 does not hold, then the price required to generate a positive demand (when the drug is covered by the payer) would have to be so low that the firm would make a loss from selling the drug, and would thus exit the market. In this paper, we focus on drugs that the firm may offer under either uniform pricing or outcome-based pricing without incurring a loss when the payer covers it.

### 3. Pricing Systems

We analyze the firm’s price decision and resulting payer’s coverage decision, as well as the patient demand, firm profit, payer payoff and objective and patient welfare under two different pricing systems. For each pricing system, we proceed by backward induction. We start with each patient’s decision to purchase the drug or not and the resulting demand, then we analyze the payer’s decision to cover the drug or not, and finally we determine the firm’s pricing decision.

#### 3.1. Uniform Pricing

**3.1.1. Patients’ Decision: Demand** The expected patient welfare under uniform pricing for a patient with treatment success value  $v$  who buys the drug is:

$$\begin{cases} q(v - \bar{\beta}p)^\alpha - (1-q)\lambda(\bar{\beta}p)^\alpha & \text{if } v - \bar{\beta}p \geq 0 \\ -q\lambda(-v + \bar{\beta}p)^\alpha - (1-q)\lambda(\bar{\beta}p)^\alpha & \text{else.} \end{cases}$$

Therefore, the patient buys the drug if and only if  $v \geq \bar{\beta}pz$ . Hence, the drug gains a market segment if  $\bar{\beta}pz \leq \bar{v}$ , and then the expected demand is  $\frac{n}{\bar{v}}(\bar{v} - \bar{\beta}pz)$ . The total expected patient welfare is then given by the sum of each patient’s welfare, that is, when  $\bar{\beta}pz \leq \bar{v}$ ,

$$\begin{aligned} W_{\text{patient}}^U &= n \int_{\bar{\beta}pz}^{\bar{v}} [q(v - \bar{\beta}p)^\alpha - (1 - q)\lambda(\bar{\beta}p)^\alpha] \frac{1}{v} dv \\ &= \frac{n}{\bar{v}} \left[ \frac{q}{\alpha + 1} ((\bar{v} - \bar{\beta}p)^{\alpha+1} - (\bar{\beta}p)^{\alpha+1}(z - 1)^{\alpha+1}) - (1 - q)\lambda(\bar{\beta}p)^\alpha(\bar{v} - \bar{\beta}pz) \right]. \end{aligned}$$

The total expected patient payoff is, when  $\bar{\beta}pz \leq \bar{v}$ ,

$$\Pi_{\text{patient}}^U = n \int_{\bar{\beta}pz}^{\bar{v}} [q(v - \bar{\beta}p) - (1 - q)\bar{\beta}p] \frac{1}{v} dv = \frac{n}{\bar{v}}(\bar{v} - \bar{\beta}pz) \left[ \frac{q}{2}(\bar{v} + \bar{\beta}pz) - \bar{\beta}p \right].$$

**3.1.2. Payer's Decision: Drug Coverage** For each patient buying the drug, the payer incurs cost  $(1 - \bar{\beta})p$  and, with probability  $q$ , earns value  $v'$ ; thus, the payer's expected payoff is

$$\Pi_{\text{payer}}^U = \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}pz)^+ (qv' - (1 - \bar{\beta})p).$$

Since the payer's goal is to maximize the combination of its own payoff and the total expected patient payoff, as a measure of net social benefits (as discussed in Section 2), the payer decides whether or not to provide coverage so as to maximize its objective defined by

$$W_{\text{payer}}^U = \Pi_{\text{payer}}^U + \Pi_{\text{patient}}^U = \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}pz)^+ \left[ qv' - p + \frac{q}{2}(\bar{v} + \bar{\beta}pz) \right].$$

The following result determines when the payer chooses to provide coverage.

**PROPOSITION 1.** *Under uniform pricing, (a) if  $p \geq \bar{v}/(\beta z)$ , the payer is indifferent with regards to its coverage decision; (b) if  $p < \bar{v}/(\beta z)$ , then*

1. *if  $q \in (0, q_1]$ , the payer offers drug coverage for any drug price;*
2. *if  $q \in (q_1, q_2]$ , the payer offers drug coverage for any drug price  $p \in [0, \bar{v}/z]$ ; for drug prices  $p \in (\bar{v}/z, \bar{v}/(\beta z))$  the payer offers drug coverage iff  $p \leq p_1 \equiv q(v' + \bar{v}/2)/(1 - qz\beta/2)$ ;*
3. *if  $q \in (q_2, 1)$ , for drug prices  $p \in [0, \bar{v}/z]$ , the payer offers coverage iff  $p \leq p_2 \equiv qv'/(1 - qz(1 + \beta)/2)$ ; for drug prices  $p \in (\bar{v}/z, \bar{v}/(\beta z))$  the payer offers drug coverage iff  $p \leq p_1$ ,*

*where  $q_1 \in (0, q_2)$  is the unique solution to  $zq = 2/\beta$  on  $[0, 1]$  and  $q_2 \in (0, q_0)$  is the unique solution to  $zq = 2/(1 + \beta)$  on  $[0, 1]$ .*

In its decision to cover the drug or not, the payer balances the cost due to coverage with the dual benefit of providing coverage – benefit for itself through  $v'$ , and benefit for the patients who purchase the drug and have a successful outcome. Proposition 1 states that: (a) when the price is so high that demand is zero, the payer is indifferent; (b) otherwise, when the chance of treatment success is low, the payer offers coverage regardless of the price. Indeed, when a drug is very risky, even with coverage, only the patients who stand to benefit greatly from the treatment (if successful) will choose to purchase the drug despite the risk of failure and financial losses. A lack of coverage would prevent some of these patients from accessing the drug, thereby hurting the patient payoff

and hence, the payer's objective. However, when the chance of treatment is larger, the payer offers coverage only when the price is below a threshold. When the drug is less risky, more patients choose to purchase it, including patients who have less value to gain out of the treatment. Thus, the payer elects to provide coverage only when the price is reasonable to control costs. In particular,  $p_1$  is the maximum price so the payer provides coverage when a lack of coverage means no patient purchases the drug (i.e.,  $\bar{v}/z < p < \bar{v}/(\beta z)$ ), and  $p_2$  is the maximum price so the payer provides coverage when there is a market for the drug even without coverage (i.e.,  $p \leq \bar{v}/z$ ).

**3.1.3. Firm Decision: Price** In the next step of the backward induction, the firm decides what to charge for the drug, anticipating the coverage decision by the payer and the resulting patient demand. The firm selects a price  $p$  to maximize its overall expected profit, given by

$$\Pi_{\text{firm}}^U(p; \bar{\beta}) = (p - c) \frac{n}{\bar{v}} (\bar{v} - \bar{\beta} p z)^+.$$

The next result determines the firm's optimal pricing decision. We use the following notations:

$$p^* = \frac{c}{2} + \frac{\bar{v}}{2\beta z}, \quad \bar{p}^* = \frac{c}{2} + \frac{\bar{v}}{2z}.$$

**THEOREM 1.** *Under uniform pricing, one of the following cases holds:<sup>1</sup>*

1.  $q \in (0, \bar{q}_1]$ , or  $(q \in (\bar{q}_1, 1]$  and  $v' \geq p^*(1/q - z\beta/2) - \bar{v}/2$ ), then the firm prices at  $p^*$  and the payer provides coverage;
2.  $q \in (\bar{q}_1, 1]$  and  $\max\{c, \bar{v}/z\} \cdot (1/q - z\beta/2) - \bar{v}/2 < v' < p^*(1/q - z\beta/2) - \bar{v}/2$ , then the firm prices at  $p_1$  and the payer provides coverage;
3.  $q \in (q_2, 1)$ ,  $c \leq \bar{v}/z$  and  $p_3(1/q - z(\beta+1)/2) \leq v' \leq (\bar{v}/z)(1/q - z(\beta+1)/2)$ , then the firm prices at  $p_2$  and the payer provides coverage;
4.  $q \in (q_2, 1)$ ,  $c \leq \bar{v}/z$  and  $v' < p_3(1/q - z(\beta+1)/2)$ , then the firm prices at  $\bar{p}^*$  and the payer does not provide coverage;
5.  $q \in (\bar{q}_1, 1]$  and  $c > \bar{v}/z$  and  $v' \leq (c/q)(1 - qz\beta/2) - \bar{v}/2$ , then the firm makes no profit regardless of its price decision and the payer does not provide coverage,

where  $\bar{q}_1 \in (q_1, q_2)$  is the unique solution of the equation  $zq = 1/\beta$  on  $[0, 1]$ ,  $p_3$  is the unique solution on  $[c, \bar{p}^*]$  of the equation  $\Pi_{\text{firm}}^U(\bar{p}^*; 1) = \Pi_{\text{firm}}^U(p_3; \beta)$ , and  $q_1, q_2, p_1, p_2$  are defined in Proposition 1.

Theorem 1 obtains the optimal pricing decision by the firm and coverage decision by the payer. There are four possible prices in equilibrium. When the chance of success is low enough to incite the payer to provide coverage regardless of the price (i.e.,  $q \leq q_1$ ), or when either  $v'$  is high or  $q$  is low enough (i.e.,  $q \leq \bar{q}_1$ ), the firm selects the unconstrained optimal price under coverage,  $p^*$ . The payer's self-interest motivates it to provide coverage and the firm can price freely to maximize its

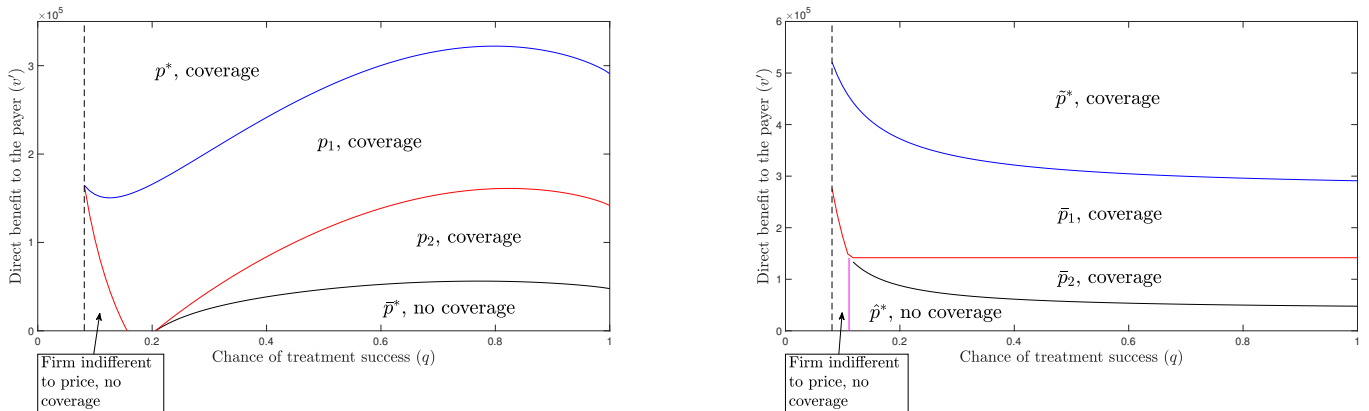
<sup>1</sup>The five cases are exhaustive, as can be seen in the proof.



profit (i.e., the payer coverage decision is “non-binding” – that is, the payer would offer coverage even at a slightly higher price – and we have an interior solution). When the chance of success is above  $\bar{q}_1$  and value  $v'$  is moderately high, the firm prices at  $p_1$ : the payer has more limited incentives to offer coverage, so the firm prices as high as it can to ensure coverage, at a price high enough that there would be no market without coverage (boundary solution). When the chance of success is above  $q_2$ , the cost is low (i.e.,  $c \leq \bar{v}/z$ ) and value  $v'$  is moderately low, the firm prices at  $p_2$ : the payer has little direct incentive to cover the drug, and the firm prices as high as it can to ensure coverage, at a price low enough that there would be some demand without coverage (boundary solution). Pricing at that relatively low point is made possible by the low cost. When the chance of success is above  $q_2$ , the cost is low and value  $v'$  is low, the firm prices at  $\bar{p}^*$ : the firm cannot induce the payer to cover the drug; however, because the cost is low, it can make a profit from the no-coverage demand and it prices at its profit-maximizing price in the absence of coverage (interior solution). If the cost is high (i.e.,  $c > \bar{v}/z$ ), the firm cannot achieve a profit without coverage because the price required would be lower than the cost. If the payer’s value from treatment success is too low to incentivize coverage, the firm cannot make any profit regardless of its price decision. Figure 3 (left panel) illustrates the different scenarios of pricing decisions when the chance of success and value  $v'$  vary.

Once the price is obtained, the total expected patient welfare and payoff, the payer’s expected payoff and objective, and the firm profit can be obtained by direct substitution.

**Figure 3** Firm’s pricing decision and payer’s coverage decision under uniform pricing (left panel) and outcome-based pricing (right panel)



Note:  $\bar{v} = 450,000$ ,  $\beta = 0.37$ ,  $\alpha = 0.8$ ,  $\lambda = 1.1$ ,  $c = 50,000$ . The dashed line indicates the minimum value of  $q$  that validates Assumption 1. We have  $q_1 = 0.2\%$ ,  $\bar{q}_1 = 2.7\%$ ,  $q_2 = 19\%$ ,  $q_0 = 62\%$ .

### 3.2. Outcome-Based Pricing

**3.2.1. Patients' Decision: Demand** The expected patient welfare under outcome-based pricing for a patient with treatment success value  $v$  who buys the drug is:

$$\begin{cases} q(v - \bar{\beta}p)^\alpha & \text{if } v - \bar{\beta}p \geq 0 \\ -q\lambda(-v + \bar{\beta}p)^\alpha & \text{else.} \end{cases}$$

Therefore, the patient buys the drug if and only if  $v \geq \bar{\beta}p$ . In particular, the patient's risk sensitivity and loss aversion play no role here as the patient bears no risk under outcome-based pricing. The drug gains a market segment if  $\bar{\beta}p \leq \bar{v}$ , and then the expected demand is  $\frac{n}{\bar{v}}(\bar{v} - \bar{\beta}p)$ . The total expected patient welfare is then given by the sum of each patient's welfare, that is, when  $\bar{\beta}p \leq \bar{v}$ ,

$$W_{\text{patient}}^O = n \int_{\bar{\beta}p}^{\bar{v}} [q(v - \bar{\beta}p)^\alpha] \frac{1}{\bar{v}} dv = \frac{qn}{\bar{v}(\alpha + 1)} (\bar{v} - \bar{\beta}p)^{\alpha+1}.$$

The total expected patient payoff is, when  $\bar{\beta}p \leq \bar{v}$ ,

$$\Pi_{\text{patient}}^O = n \int_{\bar{\beta}p}^{\bar{v}} [q(v - \bar{\beta}p)] \frac{1}{\bar{v}} dv = \frac{nq}{2\bar{v}} (\bar{v} - \bar{\beta}p)^2.$$

**3.2.2. Payer's Decision: Drug Coverage** Similarly to the uniform pricing setting, the payer's expected payoff under outcome-based pricing is

$$\Pi_{\text{payer}}^O = \frac{nq}{\bar{v}} (\bar{v} - \bar{\beta}p)^+ (v' - (1 - \bar{\beta})p),$$

and the payer's objective is

$$W_{\text{payer}}^O = \Pi_{\text{payer}}^O + \Pi_{\text{patient}}^O = \frac{nq}{\bar{v}} (\bar{v} - \bar{\beta}p)^+ \left( v' - p + \frac{\bar{v} + \bar{\beta}p}{2} \right).$$

The following result determines when the payer chooses to provide coverage.

**PROPOSITION 2.** *Under outcome-based pricing, (a) if  $p \geq \bar{v}/\beta$ , the payer is indifferent with regards to its coverage decision; (b) if  $p < \bar{v}/\beta$ , then*

1. *for drug prices  $p \in [0, \bar{v}]$ , the payer offers coverage iff  $p \leq \bar{p}_2 \equiv 2v'/(1 - \beta)$ ;*
2. *for drug prices  $p \in (\bar{v}, \bar{v}/\beta)$  the payer offers drug coverage iff  $p \leq \bar{p}_1 \equiv (v' + \bar{v}/2)/(1 - \beta/2)$ .*

Similar to Proposition 1, when the price is so high that demand is zero, the payer is indifferent. Different from the uniform pricing case, we note that, because the payer and the patients incur no cost in case of failure under outcome-based pricing, the payer's decision is independent of the chance of success. To balance costs and benefits, the payer selects to offer coverage when the price is sufficiently low. In particular,  $\bar{p}_1$  is the maximum price that the firm can charge to ensure the payer provides coverage when a lack of coverage means no patient purchases the drug (i.e.,  $\bar{v} < p < \bar{v}/\beta$ ), and  $\bar{p}_2$  is the maximum price that the firm can charge to ensure the payer provides coverage when there is a market for the drug even without coverage (i.e.,  $p \leq \bar{v}$ ).

**3.2.3. Firm Decision: Price** In the next step of the backward induction, the firm decides what to charge for the drug, anticipating the coverage decision by the payer and the resulting patient demand. The firm selects a price  $p$  to maximize its overall expected profit, given by

$$\Pi_{\text{firm}}^O(p; \bar{\beta}) = (qp - c) \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}p)^+.$$

The next result determines the firm's optimal pricing decision. We use the following notations:

$$\tilde{p}^* = \frac{c}{2q} + \frac{\bar{v}}{2\beta}, \quad \hat{p}^* = \frac{c}{2q} + \frac{\bar{v}}{2}.$$

**THEOREM 2.** *Under outcome-based pricing,*

1. *if  $v' \geq \tilde{p}^*(1 - \beta/2) - \bar{v}/2$ , the firm prices at  $\tilde{p}^*$  and the payer provides coverage;*
2. *if  $\max\{c/q, \bar{v}\} \cdot (1 - \beta/2) - \bar{v}/2 < v' < \tilde{p}^*(1 - \beta/2) - \bar{v}/2$ , the firm prices at  $\bar{p}_1$  and the payer provides coverage;*
3. *if  $c/q \leq \bar{v}$  and  $\bar{p}_3(1 - \beta)/2 \leq v' \leq \bar{v}(1 - \beta)/2$ , the firm prices at  $\bar{p}_2$  and the payer provides coverage;*
4. *if  $c/q \leq \bar{v}$  and  $v' < \bar{p}_3(1 - \beta)/2$ , the firm prices at  $\hat{p}^*$  and the payer does not provide coverage;*
5. *else (i.e., if  $c/q > \bar{v}$  and  $v' \leq (c/q)(1 - \beta/2) - \bar{v}/2$ ), the firm makes no profit regardless of its price decision and the payer does not provide coverage,*

where  $\bar{p}_3$  is the unique solution on  $[c/q, \hat{p}^*]$  of the equation  $\Pi_{\text{firm}}^O(\hat{p}^*; 1) = \Pi_{\text{firm}}^O(\bar{p}_3; \beta)$  and  $\bar{p}_1$  and  $\bar{p}_2$  are defined in Proposition 2.

The intuition behind Theorem 2 is similar to that of Theorem 1. Depending on the payer's direct benefit from treatment success,  $v'$ , the firm prices at one of four price points. It may price at the unconstrained optimum with coverage,  $\tilde{p}^*$ . It may price just high enough to ensure coverage at a price ( $\bar{p}_1$ ) where there is no market without coverage. If the cost is low enough, it may price just high enough to ensure coverage at a price ( $\bar{p}_2$ ) where there is a market without coverage. It may price at the unconstrained optimum without coverage,  $\hat{p}^*$ . If the cost is too high and the payer's incentive too low, the firm cannot induce coverage nor achieve any profit. We observe that the patients' risk sensitivity and loss aversion play no role in the price, firm profit, payer objective, and expected patient payoff; it only impacts the total expected patient welfare. Figure 3 (right panel) illustrates the different scenarios of pricing decisions when the chance of success and value  $v'$  vary.

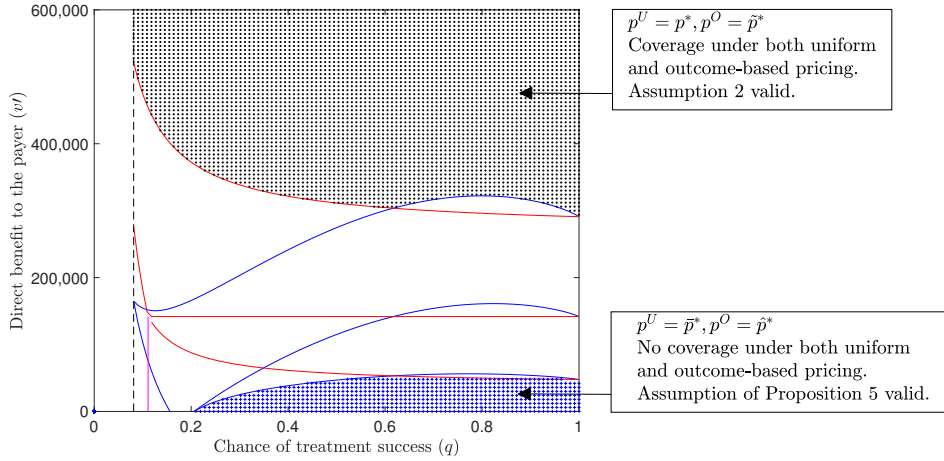
Once the price is obtained, the total expected patient welfare and payoff, the payer's expected payoff and objective, and the firm profit can be obtained by direct substitution.

## 4. Discussion

We now compare the price, demand, firm profit, payer payoff and objective, and patient payoff under the two pricing schemes. As illustrated in Figure 4, many different scenarios may occur –

for this example, we find a total of 15 different regions; in theory, there may be up to 25 possible regions. For the sake of brevity, in Sections 4.1 and 4.2 we study analytically *two* of these regions (highlighted in Figure 4), where the firm selects the unconstrained optimal prices respectively with and without coverage under both pricing systems. We relax these restrictions in the Repatha numerical example of Section 5.2 and in Section 5.3, where we investigate numerically all regions.

**Figure 4** Combined regions of the firm's pricing decision under uniform and outcome-based pricing



Note:  $\bar{v} = 450,000$ ,  $\beta = 0.37$ ,  $\alpha = 0.8$ ,  $\lambda = 1.1$ ,  $c = 50,000$ . In the top highlighted area, the payer chooses to cover the drug under both uniform and outcome-based pricing, and the firm selects respectively prices  $p^*$  and  $\tilde{p}^*$ . This region corresponds to Assumption 2 and to the analytical results of Section 4.1. In the bottom highlighted area, the payer chooses not to cover the drug under both uniform and outcome-based pricing, and the firm selects respectively prices  $\tilde{p}^*$  and  $\hat{p}^*$ . This region corresponds to Proposition 5 and Section 4.2.

#### 4.1. Case of Large Benefit to the Payer

**ASSUMPTION 2.** In Section 4.1 only, we assume  $v' \geq \tilde{p}^*(1 - \beta/2) - \bar{v}/2$  and either  $q \leq q_1$  or  $v' \geq p^*(1/q - \beta/2) - \bar{v}/2$ .

Assumption 2 ensures that under uniform pricing, the firm prices at  $p^U = p^*$  while under outcome-based pricing, the firm prices at  $p^O = \tilde{p}^*$ . Moreover, the payer provides coverage for the drug. Under Assumption 2, the payer's direct benefit from treatment success is high enough so the firm needs not incentivize the payer to offer coverage via a lower price. Section 5.1 explains how  $v'$  can be estimated and describes cases in which Assumption 2 holds in the Repatha example.

**PROPOSITION 3.** Suppose Assumption 2 holds. Then

1. The outcome-based price is larger than the uniform price.
2. The expected demand under outcome-based pricing is higher than under uniform pricing iff the drug is high-risk (i.e.,  $q < q_0$ ).

3. *The firm's expected profit under outcome-based pricing is higher than under uniform pricing iff the drug is high-risk (i.e.,  $q < q_0$ ).*
4. *The total expected patient payoff under outcome-based pricing is higher than under uniform pricing iff either the drug is low-risk (i.e.,  $q > q_0$ ) or  $\beta c < \bar{v}q_0$  and the drug is very high-risk (i.e.,  $q < q_3$ , where, for  $\beta c < \bar{v}q_0$ ,  $q_3$  is the unique solution on  $(0, q_0)$  of the equation  $\beta^2 c^2 (z^2 - z/q)/(2\bar{v}) + z\beta c - \bar{v} = 0$ ).*
5. *The expected payer's payoff under outcome-based pricing is higher than under uniform pricing iff either (i) the drug is high-risk (i.e.,  $q < q_0$ ) and the direct benefit to the payer is very high (i.e.,  $v' > M$ , where  $M = (1 - \beta)(\bar{v}^2/(z\beta c) + \beta c/q)/(2\beta)$ ), or (ii) the drug is low-risk (i.e.,  $q > q_0$ ) and the direct benefit to the payer is moderate (i.e.,  $v' < M$ ).*
6. *The payer's objective under outcome-based pricing is higher than under uniform pricing iff either (i) the drug is high-risk (i.e.,  $q < q_0$ ) and the direct benefit to the payer is very high (i.e.,  $v' > M'$ , where  $M' = \bar{v}(\bar{v}/(z\beta^2 c) - 1)/2 + \beta c(2/\beta - 1 - zq)/(4q)$ ), or (ii) the drug is low-risk (i.e.,  $q > q_0$ ) and the direct benefit to the payer is moderate (i.e.,  $v' < M'$ ).*

Our intuitive reasoning in Section 1.2 described that prices may rise under outcome-based pricing, and that the effect of the price increase on the agents *may or may not* be compensated by the risk reallocation. Proposition 3 confirms that the price is higher, and whether the drug is high-risk or low-risk is the main driver for whether each agent benefits under outcome-based pricing.

We make several comments on the above result. First, Proposition 3 states that outcome-based pricing leads to a higher price than uniform pricing to counteract the firm's lost income from patients whose treatment fails. This finding is consistent with qualitative observations on outcome-based pricing: “the deals don't stop drug companies from charging high starting prices for new drugs” and “any rebates or discounts in outcomes-based contracts are off an already inflated number” (Loftus 2017). Second, the result states that when the drug is high-risk (i.e.,  $q < q_0$ ), outcome-based pricing increases patient demand for the drug despite the price increase. Under uniform pricing, risk-sensitive patients might balk at the risk of spending a large sum to obtain a drug that is unlikely to succeed – an issue made irrelevant under outcome-based pricing. Third, we find that when the drug is high-risk (i.e.,  $q < q_0$ ), outcome-based pricing increases the firm's profit. The combination of a larger demand and higher prices counterbalances the firm's lost income from failed treatments. Fourth, the patient total payoff benefits from outcome-based pricing when the drug is either low-risk or very high-risk (i.e.,  $q > q_0$  or  $q < q_3$ ). In the former case, limiting the demand to the patients who benefit most from the drug overall improves the patient payoff by reducing the total out-of-pocket cost. In the latter case, for a very risky drug, the absence of risk for patients outweighs the negative impact of the higher price. Fifth, the payer's payoff comparison

**Table 1** Summary of comparison of outcome-based pricing vs. uniform pricing under Assumption 2 (from Proposition 3)

	High-risk drug ( $q < q_0$ )	Low-risk drug ( $q > q_0$ )
Price higher under:	Outcome-based pricing	Outcome-based pricing
Demand higher under:	Outcome-based pricing	Uniform pricing
Firm profit higher under:	Outcome-based pricing	Uniform pricing
Patient payoff higher under:	Outcome-based pricing if $q < q_3$ Uniform pricing if $q > q_3$	Outcome-based pricing
Payer payoff higher under:	Uniform pricing if $v' < M$ Outcome-based pricing if $v' > M$	Outcome-based pricing if $v' < M$ Uniform pricing if $v' > M$
Payer objective higher under:	Uniform pricing if $v' < M'$ Outcome-based pricing if $v' > M'$	Outcome-based pricing if $v' < M'$ Uniform pricing if $v' > M'$

is, as expected, sensitive to the value of the payer's direct benefit from a successful treatment,  $v'$ . If  $v'$  is extremely large (i.e.,  $v' > M$ ), the payer prefers whichever pricing system maximizes the demand, to yield as many successful treatments as possible.<sup>2</sup> If  $v'$  does not exceed this extreme threshold, the payer's payoff improves under outcome-based pricing when the drug is low-risk (i.e.,  $q > q_0$ ) as the demand is lower, because only the patients with highest value from treatment success choose to purchase the drug. The combination of a smaller demand and lack of financial liability for failed treatments compensates for higher prices and overall leaves the payer better off than under uniform pricing. Sixth, the payer's objective comparison is also sensitive to the value of  $v'$ , since the payer's objective is influenced by the payer payoff. When  $v'$  is extremely high (i.e., above  $M'$ ), the payer's objective is aligned with the demand. Otherwise, similarly to the patients' and payer's payoffs, the payer's objective under outcome-based pricing is higher when the drug is low-risk.

We observe that the firm's interest is aligned with the patient access (i.e., demand), that is, the firm benefits when the drug is high-risk. However, as long as  $v'$  is not excessive, the firm's and the payer's interests are at odds: they do not simultaneously benefit from outcome-based pricing (see Table 1). Conversely, when  $v'$  is extremely large, the firm's and the payer's interests are aligned and both prefer outcome-based pricing when this pricing system increases demand, that is, when the drug is high-risk.

These results highlight some possible unintended consequences of outcome-based pricing from a health policy design perspective. The pharmaceutical firm would rationally choose to implement outcome-based pricing only when it generates more profit than uniform pricing. This type of arrangement would not be considered if "a drug company did not consider it a win for them" (Thomas and Ornstein 2017). Under Assumption 2, we find that the firm has incentives to use outcome-based pricing only for high-risk drugs. For this type of drugs, outcome-based pricing

<sup>2</sup>It is important to note that the threshold  $M$  on  $v'$  for this situation to occur is very large. In our numerical examples,  $M$  often reaches a value far above  $\bar{v}$ , especially for moderate costs.

expands the demand but worsens the payer's payoff and objective, unless the direct value to the payer from a successful treatment is extremely high. Therefore, giving firms the option to enter outcome-based pricing schemes could hurt the payer unless  $v'$  is extremely large.

To resolve this issue, we design a modified outcome-based pricing contract that both payer and firm prefer over uniform pricing for high-risk drugs. The basic idea is to have the firm share some of its incremental gains to create a win-win situation for payer and firm that incentivizes participation.

**PROPOSITION 4.** *Suppose Assumption 2 holds. If  $v' > M'$ , the firm's and payer's interests are aligned. If  $v' < M'$ , there exists a transfer payment between firm and payer that makes outcome-based pricing better than uniform pricing for both parties if and only if the drug is high-risk (i.e.,  $q < q_0$ ). The transfer is from the firm to the payer and can be of any amount from  $W_{payer}^U - W_{payer}^O > 0$  up to  $\Pi_{firm}^O(p^O; \beta) - \Pi_{firm}^U(p^U; \beta) > 0$ .*

This result establishes conditions for a transfer payment to exist between the firm and the payer that makes outcome-based pricing advantageous to both of them relative to uniform pricing when their interests are otherwise misaligned. When  $v' < M'$  and the drug is high-risk, the firm gains from implementing outcome-based pricing but the payer does not. We find that the firm's gain is sufficient to cover the payer's loss due to outcome-based pricing, and hence, there exists a transfer payment from the firm to the payer such that outcome-based pricing benefits both after the transfer. When  $v' < M'$  and the drug is low-risk, the payer gains from implementing outcome-based pricing and the firm does not. However, we find that there is no transfer payment such that outcome-based pricing can benefit both parties, because the payer's gain is outweighed by the firm's profit loss. In summary, outcome-based pricing cannot simultaneously benefit both the payer and the firm when  $v' < M'$ . Yet, Proposition 4 thus shows that outcome-based pricing together with a simple transfer payment, has the potential to perform better than uniform pricing for both the firm and the payer – but for high-risk drugs only. Note that it is unnecessary to modify the outcome-based pricing system when  $v' > M'$  because the firm's and the payer's interests are then aligned in preferring outcome-based pricing for high-risk drugs, and in preferring uniform pricing for low-risk drugs.

Proposition 4 also states that, when it is possible to design a transfer payment, this payment is not unique. There is a continuum of transfer payments that achieve the goal of making both parties better off. Under each of these arrangements, the firm's and the payer's shares of the total payoff vary. In practice, the negotiating power of the payer with respect to the firm can help determine the share to be received by each party and thus the precise amount to be transferred. This result recalls the way revenue-sharing contracts coordinate supply chain decisions while allocating a varying share of profits to the retailer and the supplier (Cachon and Lariviere 2005).

Another implication of Proposition 4 is that the patients' loss aversion and risk sensitivity improve the range of drugs for which the entire system may benefit from a modified outcome-based

pricing contract. Either a stronger loss aversion or risk sensitivity (larger  $\lambda$  or  $1 - \alpha$ ) leads to a higher threshold  $q_0$ . Hence, as the loss aversion or risk sensitivity increases, fewer drugs are low-risk.<sup>3</sup> Proposition 4 shows that for low-risk drugs, there is no transfer payment that can make outcome-based pricing outperform uniform pricing. By shrinking the set of low-risk drugs, a stronger loss aversion or risk sensitivity thus widens the type of drugs for which outcome-based pricing, possibly modified with a transfer payment, can be used to benefit payer and firm simultaneously.

#### 4.2. Case of Low Benefit to the Payer

Section 4.1 considered the case of a high direct benefit to the payer from treatment success. As a first step to understand how generalized those results are, we now consider the opposite extreme – a low benefit to the payer. We focus on the case when the benefit to the payer is too low to incentivize coverage under either pricing system (see Figure 4), yet the cost is low enough for the firm to earn a market share from patients who choose to pay the entire price out-of-pocket.

**PROPOSITION 5.** *Suppose  $q \in (q_2, 1)$ ,  $c \leq \min\{\bar{v}/z, \bar{v}q\}$  and  $0 \leq v' < \min\{p_3(1/q - z(\beta + 1)/2), \bar{p}_3(1 - \beta)/2, M''\}$  where  $M'' \equiv \bar{v}(\bar{v}/(zc) - 1)/2 + c(1 - zq)/(4q)$ . Then the firm prices at  $\bar{p}^*$  under uniform pricing and at  $\hat{p}^*$  under outcome-based pricing, and the payer does not offer coverage under either pricing system. Moreover,*

1. *Proposition 3 parts 1 through 3 are valid.*
2. *The total expected patient payoff under outcome-based pricing is higher than under uniform pricing iff the drug is low-risk (i.e.,  $q > q_0$ ).*
3. *The expected payer's payoff under outcome-based pricing is higher than under uniform pricing iff the drug is high-risk (i.e.,  $q < q_0$ ).*
4. *The payer's objective under outcome-based pricing is higher than under uniform pricing iff the drug is low-risk (i.e.,  $q > q_0$ ).*
5. *There exists a transfer payment between firm and payer<sup>4</sup> that makes outcome-based pricing better than uniform pricing for both parties if and only if (i) the drug is high-risk (i.e.,  $q < q_0$ ) or (ii) the drug is low-risk (i.e.,  $q > q_0$ ) and<sup>5</sup>  $2v' + \bar{v} \geq (c/2)(3/q - z)$ .*

Table 5 in Appendix B summarizes the results. We find that, similar to the case of a high  $v'$ , when  $v'$  is so low that there is no coverage under either pricing mechanism, the price continues to be higher under outcome-based pricing. The demand and the firm profit remain higher under outcome-based

<sup>3</sup>For  $\lambda = 2$ ,  $q_0$  takes the value 90.97% when the risk sensitivity  $1 - \alpha$  equals 0.7, and  $q_0$  takes the value 72.91% when the risk sensitivity  $1 - \alpha$  equals 0.3. The effectiveness of new drugs is rarely very high, hence, in practice, due to loss aversion most new drugs are likely to be considered high-risk.

<sup>4</sup>We provide this result only as a means to establish the extent of the robustness of Proposition 4 when Assumption 2 is relaxed. In practice, it is unlikely to see a transfer contract agreement when the payer does not cover the drug.

<sup>5</sup>The proof illustrates that this condition on  $v'$  can be met even when  $v'$  is low enough for Proposition 5 to hold.



pricing as long as the drug is high-risk, for the same reasons as discussed after Proposition 3. The result on the patient payoff is similar to Proposition 3 except that outcome-based pricing is worse for patients for *all* high-risk drugs.

The main difference with Proposition 3 is that the payer's payoff benefits from outcome-based pricing for high-risk drugs, and thus the payer's payoff is aligned with the firm profit (and demand), and at odds with the patients' payoff. The payer does not cover the drug so its payoff stems solely from benefit  $v'$  gained for each treatment success, and thus the payer's payoff is higher when demand is higher. The absence of coverage thus sharply changes how the payer's payoff is affected by the pricing systems. However, in the payer's objective, the patients' payoff outweighs the payer's payoff because  $v'$  is very low, so the payer's objective is aligned with the patients' payoff and at odds with the payer's payoff. Hence, consistent with Proposition 3 in the case when  $v'$  is not excessive, the payer's objective remains at odds with the firm profit. This proposition illustrates in particular that when there is no coverage under both pricing systems, parts of Propositions 3 and 4 are no longer valid because a lack of coverage fundamentally alters the payer's financial interests under each of the pricing systems.

### 4.3. Extensions

We test the robustness of our findings by considering several extensions of our model. Appendix C investigates the case of a fixed partial rebate in case of failure. We find that the partial reimbursement case is a hybrid of the uniform pricing and the outcome-based pricing with full rebate cases studied in the main body of the paper. Hence, the results for both the payer's coverage decision and the firm's pricing decision are intermediate between the two extremes represented by the uniform pricing and outcome-based pricing with full rebate. In particular, partial-rebate contracts give rise to smaller gaps with uniform pricing than full-rebate contracts, but do not improve the possibility of benefiting simultaneously the payer and the firm. We examine the consequences of jointly mis-estimating the chance of success in Appendix D. Poor estimation of the chance of success affects the actual payoffs, i.e., the firm profit, payer objective and patient welfare. However, the main insights of our paper remain qualitatively valid when all agents mis-estimate of the chance of success in the same way. Appendix E considers a model of symmetric duopoly competition. Because of symmetry, the patient selects the cheaper drug, if any. Hence, the firm with the higher price captures zero demand. It follows that in equilibrium, under both pricing systems, the firms achieve no profit. This finding is consistent with the well-known result that Bertrand competition leads to firms pricing at the marginal cost and earning zero profit. Hence, the firms are indifferent between the pricing systems. We show that the patient payoff is higher under outcome-based pricing, and the payer's payoff comparison depends on  $v'$  and on whether the drug is low-risk or

high-risk: when  $v'$  is reasonably high, the payer's payoff benefits from outcome-based pricing if and only if the drug is high-risk. Appendix F analyzes the case of  $v'$  heterogeneous across patients and perfectly correlated with  $v$ , i.e.,  $v' = kv$  for a fixed given  $k$ . Our results mirror to a large extent the case of a constant benefit to the payer studied in the main body of the paper. Essentially, the coverage decision and the optimal price depend on how much the payer values treatment success: how large the scaling factor  $k$  is in the correlated case, how large  $v'$  is in the constant benefit case. Therefore, the results obtained under a constant benefit to the payer qualitatively continue to hold when the payer's benefit is perfectly correlated to the patient's, where the amplitude of  $k$  plays a role analogous to the amplitude of  $v'$ . The main differences are that there are two price points with coverage under both pricing systems (instead of three with a constant  $v'$ ) and that under uniform pricing, the payer prices at  $p^*$  if and only if  $k$  is high enough, while for a constant  $v'$ , the same holds either when  $v'$  is high enough or when the drug is very high-risk.

## 5. Numerical Analysis

In this section, we analyze numerically the performance of outcome-based pricing relative to uniform pricing. Section 5.1 discusses how to calibrate the parameters of the model both (i) to focus on the specific example of the Repatha drug as a base case (Section 5.2), and (ii) to more generally establish the robustness of the results obtained in Section 4 when Assumption 2 is relaxed (Section 5.3).

### 5.1. Parameter Calibration

We calibrate the model parameters using the Repatha example as a base case. To study the robustness of our analytical results, we also design a large set of parameters that aims to capture many other realistic scenarios. The selected parameter values are summarized in Table 2. Fonarow et al. (2017)[eTable1] estimate that patients on the drug have a 74% lifetime chance of experiencing a myocardial infarction (heart attack), ischemic stroke, or cardiovascular death; hence, we set  $q = 26\%$  in the Repatha example. In particular, since  $q < 50\% < q_0$ , Repatha is a “high-risk” drug. To capture a wide variety of drugs in the robustness study, we consider  $q$  taking values from 10% to 70%. A new branded drug such as Repatha, when covered, is generally considered as a specialty drug for pharmacy benefits, placed in the last tier in the formulary with a co-insurance rate higher than generics or preferred drugs. The co-insurance rate for this tier averaged 37% in 2016 (KFF 2016). Hence, we set  $\beta = 37\%$  in the base case, and we consider  $\beta$  varying from 27% to 47% in the robustness study. Previous research that estimates the intensity of the loss-aversion effect finds values around 2 (Tversky and Kahneman 1992, Hardie et al. 1993). Similar values were obtained in the healthcare area of application. O'Brien et al. (2002) report that in the context of new

**Table 2** Parameter values in the Repatha example (base case) and in the robustness study.

		Base case	Robustness study
Chance of treatment success	$q$	26%	{10%; 25%; 40%; 55%; 70%}
Patient co-insurance rate	$\beta$	37%	{27%; 32%; 37%; 42%; 47%}
Patient loss-aversion	$\lambda$	2	{1.2; 1.6; 2; 2.4; 2.8}
Patient risk sensitivity	$1 - \alpha$	0.3 (low risk sensitivity) 0.7 (high risk sensitivity)	{0.1; 0.3; 0.5; 0.7; 0.9}
Variable production cost	$c$	[\$0; \$93,594] (low risk sensitivity) [\$0; \$3682] (high risk sensitivity)	{0.05; 0.2; 0.35; 0.5; 0.65; 0.8; 0.95} $\times c_{\max}$
Payer's direct benefit from treatment success	$v'$	{ \$25,000; \$100,000; \$300,000; \$420,000 }	{0.1; 0.3; 0.5; 0.7; 0.9; 1.1; 1.3} $\times \bar{v}$
Maximum patient gain	$\bar{v}$	\$450,000	\$450,000

$c_{\max} \equiv \bar{v} \min\{q/\beta, 1/(\beta z), 1\}$ . The number of patients  $n$ , a scaling factor, is normalized to  $\bar{v}$ .

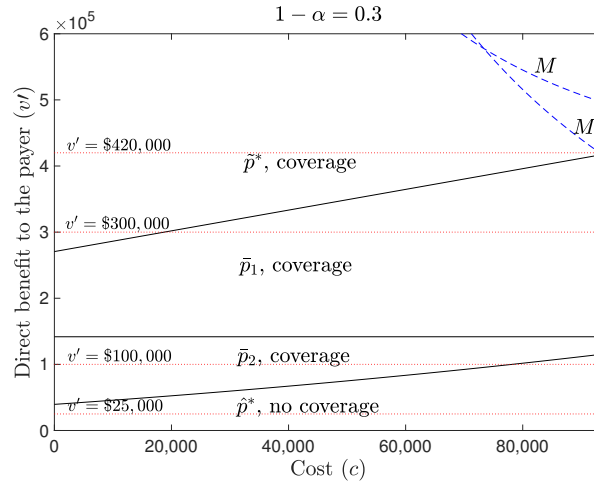
pharmaceuticals, the loss aversion coefficient was estimated at 1.9. Rasiel et al. (2005) use a loss aversion coefficient of 2 in the context of treatment options for terminally ill patients. Accordingly, we set  $\lambda = 2$  as a base-case value, and we let  $\lambda$  vary from 1.2 to 2.8 in the robustness study. To capture the effect of the patients' risk sensitivity, we consider two scenarios in the base case, where the risk sensitivity equals  $1 - \alpha = 0.3$  (low risk sensitivity) and  $1 - \alpha = 0.7$  (high risk sensitivity). In the robustness study, we let  $\alpha$  span the entire range by selecting values from 0.1 to 0.9. Due to the difficulty of evaluating the variable production cost of the drug, we show the results over the entire range of costs such that (i) Assumption 1, which guarantees the profitability of the drug under both pricing systems, is valid, and (ii) the variable production cost does not exceed the maximum gain to patient from treatment success. We note that the range of acceptable costs may depend on the risk sensitivity. Highly risk-sensitive patients demand a lower uniform price, which makes certain high-cost drugs unprofitable, whereas these drugs could be profitable under a lower risk sensitivity. Since Repatha is high-risk, the production cost ranges from 0 to  $\bar{v}/(\beta z)$  in the base case. In the Repatha example, the value of  $v'$  may be based on the payer's direct cost savings due to avoiding healthcare costs associated with treating a patient who suffers a major adverse cardiovascular event, the indirect cost saving for society (e.g., avoiding loss of productivity), and a mission-driven benefit from maintaining a beneficiary's good health status. Fonarow et al. (2017)[eTable1] estimate that the incremental direct cost savings from using Repatha amount to \$39,837. AHA (2017) estimates that the indirect costs of a heart attack or a stroke are 75% of the direct costs, bringing the total (direct and indirect) cost to \$69,715. The mission-driven benefit to the payer from maintaining a beneficiary in good health may vary according to the type of payer (e.g., Medicare might value beneficiaries' health more than a private insurer), and is harder to estimate. Supposing that, in addition to cost savings, the payer values the beneficiary's treatment success at the average patient benefit,  $\bar{v}/2$ , then  $v'$  would take a value near \$295,000. Assumption 2 would then be valid for the entire range of costs in case of high risk sensitivity and, in case

of low risk sensitivity, for marginal production costs up to around \$16,000. This said, to capture the role of value  $v'$ , we consider four scenarios:  $v' \in \{\$25,000; \$100,000; \$300,000; \$420,000\}$  in the base case. In the robustness study, to generate instances of the problem that span as many of the outcome regions as possible, we set  $v'$  as varying from  $0.1\bar{v}$  to  $1.3\bar{v}$ . As explained in Section 2, we set  $\bar{v} = \$450,000$  in the Repatha example. Since both the cost  $c$  and value  $v'$  are set relative to  $\bar{v}$ , it is unnecessary to vary  $\bar{v}$  in the robustness study. Table 6 in Appendix B shows how the scenarios are distributed among all the possible regions of price and coverage outcomes.

## 5.2. Repatha Example Numerical Results

For these input parameters, we find  $q < \bar{q}_1$ . Thus, under uniform pricing, the firm prices at  $p^*$  regardless of the value of  $v'$ , and the payer offers coverage. Figure 5 illustrates the optimal pricing strategies under outcome-based pricing for low risk sensitivity (all high-risk-sensitivity figures can be found in Appendix B). Assumption 2 is thus valid iff  $v' \geq \tilde{p}^*(1 - \beta/2) - \bar{v}/2$ , that is, if the scenario is in the region labelled “ $\tilde{p}^*$ , coverage” in Figure 5. We observe that this is the case for all costs and both levels of risk sensitivity only in the base-case scenario  $v' = \$420,000$ . We also observe that the thresholds  $M$  and  $M'$  defined in Proposition 3 take very high values. Therefore, unless  $c$  is very high, most reasonable values of  $v'$  fall below the thresholds  $M$  and  $M'$ .

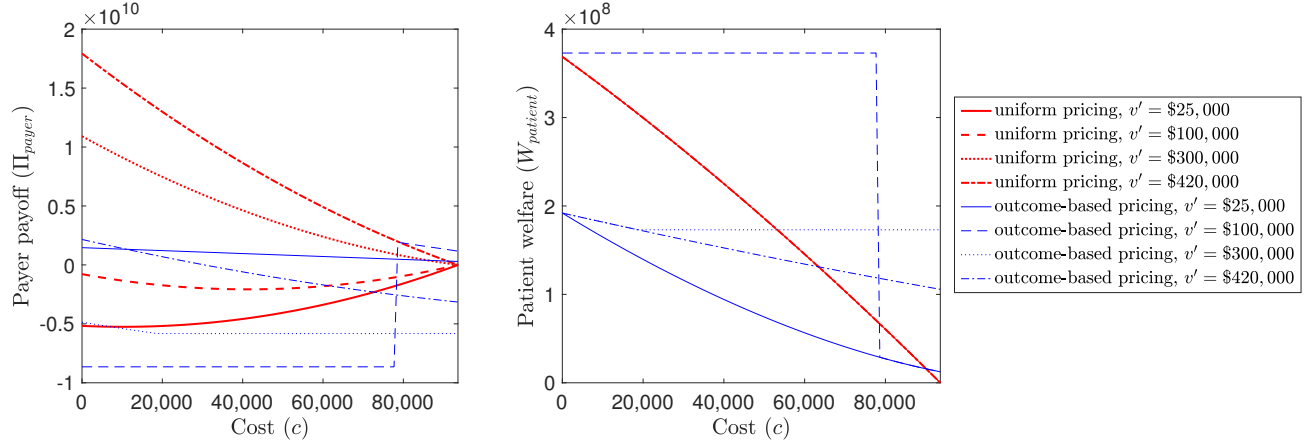
**Figure 5** Outcome-based pricing optimal price and payer coverage decision under low risk sensitivity



Note: the solid black lines are the limits of the price regions, the dotted red lines are the scenarios of values of  $v'$ .

Figure 6 illustrates the payer payoff and patient welfare under the two pricing schemes over a range of variable costs for low risk sensitivity. We make several observations. First, we observe that for a high-risk drug such as Repatha, outcome-based pricing fails to achieve the goals stated by its proponents. (i) A possible unintended consequence of outcome-based pricing is that it may

**Figure 6** Payer payoff and cumulative patient welfare (low risk sensitivity)



reduce the payer's incentives to provide coverage for the drug.<sup>6</sup> As noted above, in this numerical example the payer offers coverage under uniform pricing regardless of  $v'$ . However, as can be seen in Figure 5, under outcome-based pricing, the payer chooses not to provide coverage when  $v'$  is low and/or  $c$  is high (e.g.,  $v' = \$25,000$ ;  $v' = \$100,000$  and  $c > \$78,000$ ). Furthermore, the payer has less incentives to offer coverage when the patient is less risk-sensitive. For example, when  $v' = \$100,000$ , there is coverage under high risk sensitivity, but under low risk sensitivity coverage is not offered when the cost is too high. By removing all risk exposure for the patient, full-rebate outcome-based pricing expands demand dramatically for a high-risk drug, to a point that the payer may find it too expensive to offer coverage, in scenarios where it would provide coverage under uniform pricing. (ii) Furthermore, not only does outcome-based pricing not help keep prices under control (as proved in Sections 4.1 and 4.2 in special cases and verified numerically in Section 5.3<sup>7</sup>), while it benefits the firm, it fails to improve the payer's payoff due to high price and high demand, unless either of the following occurs. (a)  $v'$  is so low that the payer chooses not to offer coverage under outcome-based pricing (Figure 6, left panel with  $v' = \$25,000$ ). This case is in line with Section 5.3 observations. (b) Both  $v'$  and the cost are very high (this does not occur in the low-sensitivity example as  $v' < M$  in all cases, but does occur in Figure 9 in Appendix B, left panel with  $v' = \$420,000$  and  $c > \$3400$ ). Consistent with Proposition 3 part 5, the payer's payoff is higher under outcome-based pricing when  $q < q_0$  and  $v' > M$ . The payer values successes so much that it sides with the pricing mechanism that favors the highest demand, i.e., outcome-based pricing.

<sup>6</sup>Table 6 in Appendix B shows that out of 30,625 scenarios in our robustness study, 2220 scenarios yield coverage under uniform pricing and no coverage under outcome-based pricing, while only 107 yield the reverse.

<sup>7</sup>Consistently with our analytical results, we found that the price, patient demand and firm's profit are higher under outcome-based pricing for all values of  $v'$  considered in this example, even if there is no coverage under outcome-based pricing (results not shown here due to space constraints). Moreover, a transfer payment exists in all considered scenarios.

Second, our numerical results help us evaluate the benefits of outcome-based pricing for the patient welfare in case of a high-risk drug (due to lack of tractability, we did not obtain analytically the effect of the pricing systems on patient welfare). We find that the patient welfare is improved under outcome-based pricing as compared to uniform pricing in two types of scenarios (Figure 6, right panel): (i)  $v'$  is low enough so the price is under control (but not so low that there is no coverage; e.g.,  $v' = \$100,000$  when  $c < \$78,000$  – at higher costs the payer stops offering coverage); (ii) the cost is high, so under uniform pricing demand is low and the price is high which hurts risk-sensitive patients (e.g.,  $v' = \$420,000$  and  $c > \$63,000$  or  $v' = \$100,000$  and  $c > \$90,000$ ). Outcome-based pricing benefits the patient welfare even more strongly when the risk sensitivity is high (Appendix B) since the patient bears no risk under this pricing scheme. Hence, for a high-risk drug, if the firm accepts to share part of its profit gains with the payer, outcome-based pricing does have the potential to benefit not only both the firm and the payer but also the patients. However, it is important to note that outcome-based pricing may also hurt the patient welfare despite a higher demand because better access (i.e., higher demand) does not sufficiently compensate for the high price compared to uniform pricing (e.g.,  $v' = \$420,000$  and  $c < \$63,000$ ) or because of a lack of coverage under outcome-based pricing (e.g.,  $v' = \$25,000$  and  $c < \$90,000$ ).

Finally, we find that the patient's risk sensitivity and loss aversion, when high, have a detrimental effect on access for a high-risk drug such as Repatha. High risk sensitivity and high loss aversion act as a barrier to access because they restrict the type of drugs deemed profitable by the firm. The drug is profitable under uniform pricing when the cost is below  $\bar{v}/(\beta z)$ . When patients are more sensitive to risk or more loss averse,  $z$  is larger, and thus the range of eligible costs is narrower. Indeed, under uniform pricing, more risk-sensitive or loss-averse patients are more sensitive to the price charged, and thus only low-cost drugs (which can be priced low) can be profitable.

### 5.3. General Case: Robustness

In this section, we seek to understand from a general standpoint whether the analytical results of Section 4.1 continue to hold when Assumption 2 is invalid.<sup>8</sup> To assess robustness comprehensively, we conduct a numerical study testing the findings of Proposition 3 and 4 over a wide variety of input parameters (see Table 2). Table 3 reports the results.

We observe from this analysis that when Assumption 2 is relaxed, the results of Propositions 3 and 4 remain valid in the large majority of cases. Without Assumption 2, Proposition 3 predicts correctly in only 68-72% of cases the pricing system under which the payer payoff or objective

<sup>8</sup>In Figure 10 in Appendix B, we visualize whether the main results of Propositions 3 and 4 hold over a range of values of the chance of success ( $q$ ) and direct benefit to the payer ( $v'$ ) in an example calibrated on Repatha.

**Table 3** Results of general numerical study

Scenarios:	All $N = 30,625$	Assumption 2 valid $N = 12,344$	Assumptions of Proposition 5 valid $N = 269$	Assumption 2 not valid $N = 18,281$	Assumption 2 not valid, coverage under both systems $N = 13,852$
Prop. 3 part 1 (price)	100%	100%	100%	99%	100%
Prop. 3 part 2 (demand)	90%	100%	100%	84%	100%
Prop. 3 part 3 (firm)	88%	100%	100%	79%	93%
Prop. 3 part 4 (patient)	75%	100%	100%	58%	60%
Prop. 3 part 5 (payer payoff)	84%	100%	0%	72%	96%
Prop. 3 part 6 (payer objective)	81%	100%	100%	68%	80%
Prop. 4 (transfer payment)	89%	100%	100%	81%	96%
Payer and firm payoffs at odds iff $v' < M$	88%	100%	0%	80%	96%
Payer objective and firm payoff at odds iff $v' < M'$	85%	100%	100%	74%	84%

is higher. Yet, in a large majority (74% and 81%, respectively) of cases, (i) the payer objective and firm payoffs remain at odds whenever  $v'$  is not excessive, and (ii) a transfer payment exists only for high-risk drugs – two key insights of our analytical findings of Section 4.1. We also obtain (not shown in the table) that when firm profit and payer objective are either both improved under outcome-based pricing, or can be via a transfer payment, then the patient payoff is improved in only 40% of cases overall (19% of cases where Assumption 2 is valid, 58% of cases where it is not). This indicates that patients are likely to be hurt by outcome-based pricing.

To understand the circumstances causing the results not to hold in a number of scenarios, based on observations from Section 4.2, we also analyze the 13,852 scenarios where Assumption 2 is not valid but there is coverage under both pricing systems. In those scenarios, the payer objective and firm payoff are very often at odds whenever  $v'$  is not excessive (84% of cases) and the result on transfer payment is valid in 96% of cases. Hence, lack of coverage (due for example to a too low  $v'$  or too high cost) appears a main driver for our results to be violated, which is consistent with the observations of Section 4.2. To summarize, the findings obtained under Assumption 2 are very robust as long as both pricing systems lead the payer to provide coverage.

## 6. Limitations

Our work has several limitations. Our model does not apply to drugs that can potentially harm the patient compared to the standard treatment. Our stylized model captures patient heterogeneity solely through the value gained from treatment success. There may be several other sources of heterogeneity in reality. The quantity of the drug necessary (and thus the effective price) for a course of treatment may vary due to demographics, indication, or treatment duration. In some cases, patients can be clustered in subgroups with a different chance of success across groups. These sources of heterogeneity can affect each other as well (e.g., a drug with two indications could have different chances of success and cost different prices for a course of treatment based on the

indication). Similarly, our model assumes complete information. In practice, some parameters of the model may be difficult to estimate, e.g., production cost (for the payer), patients' risk sensitivity and loss aversion. The firm or the payer may have more information than the patient on the drug effectiveness. The firm may not have a good estimate of the payer's benefit due to treatment success. It may be interesting to study how information asymmetry could affect the performance of outcome-based pricing. Furthermore, our model assumes a monopoly setting while in reality, the drug may be subject to competition. The lack of a comprehensive treatment of competitive effects represents a limitation of the generality of our findings. Moreover, we model the payer's benefit from treatment success as homogenous across patients. We extend some of our analytical results when that benefit is perfectly correlated with the patient's in appendix, but the absence of a complete analysis of the (fully and partially) correlated case is another limitation of our work.

## 7. Concluding Remarks

This paper analyzes the effect of outcome-based pricing for a new brand-name drug in comparison with the traditional uniform pricing system in the presence of risk-sensitive and loss-averse patients. Full-rebate outcome-based pricing eliminates the risk that payer and patient pay for a drug that does not work. We use a stylized model and a numerical study to show that the firm's profit and the payer's objective are often at odds, especially if there is coverage under both pricing systems. Who will benefit from outcome-based pricing is determined by the probability of treatment success. Hence, outcome-based pricing might not resolve the issue of high payer expenditures. The payer's payoff and the firm's profit may be aligned, however, when the payer's direct benefit from a successful treatment,  $v'$ , is extremely high or when it is so low that there is no coverage. In addition, outcome-based pricing may provide less incentives for the payer to offer coverage. Outcome-based pricing shields patients from risk exposure and thus leads to a high demand under coverage, so the cost of covering the drug can become prohibitive for the payer. Nevertheless, the demand remains higher under outcome-based pricing even without coverage. While outcome-based pricing might not benefit the payer for a high-risk drug, adding a transfer payment from the firm to the payer can make outcome-based pricing beneficial to both payer and firm. Moreover, we find that outcome-based pricing can improve patient welfare when  $v'$  is low but there is coverage, or when the cost is high. Therefore, if accompanied by a transfer payment, outcome-based pricing for high-risk drugs has the potential to improve the system performance and each agent's welfare, despite yielding high prices. However, if  $v'$  is very low, or if the cost is low, outcome-based pricing may reduce the patient welfare due to a lack of coverage or due to high prices compared to uniform pricing. Finally, the patients' risk sensitivity and loss aversion affect access to the drug. If the risk sensitivity or



the loss aversion is high, pharmaceutical firms have no incentive to bring to market high-risk drugs that do not have a low production cost, which limits the type of drugs available to patients.

Implementation of outcome-based pricing would have to overcome a number of important practical hurdles. Some legal issues must be addressed (e.g., the Medicaid “best price” rule mandating that the pharmaceutical firm offer state Medicaid programs the best price given to any other purchaser). Payer and firm must agree on how to define and measure treatment “success”, and must have in place (and decide how to pay for) an infrastructure for collecting and sharing data on each patient taking the drug, even as patients might change doctor and health insurance in the course of their treatment. They must also agree on how to tackle issues of patient adherence with the treatment protocol. The best candidates for outcome-based pricing appear to be drugs that have a low chance of success and for which it is easy to measure the effect of the drug. In the long run, a more widespread use of variants of outcome-based pricing systems could affect pharmaceutical firms’ investment decisions for the type of medications that are worth attempting to develop.

Our paper captures contracts where the performance with regards to patient health outcomes affects the price. There are other types of performance-based contracts that are used in practice. In particular, a good performance can be linked to a better formulary placement (Seeley and Kesselheim 2017, Xu et al. 2019). In other cases, the discount and/or formulary placement may be tied to the patient adherence level (Carlson et al. 2009). These types of contracts constitute an interesting area of future research.

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## Material for Online Supplement

## Appendix A: Notation

Table 4 Summary of notation used in the paper

$n$	Number of patients
$\bar{v}$	Maximum patient gain due to treatment success
$V$	Random patient gain (uniformly distributed) due to treatment success
$v$	Individual patient gain due to treatment success realization
$v'$	Payer gain due to treatment success
$q$	Chance of treatment success
$\beta$	Patient co-insurance rate
$\bar{\beta}$	Effective co-insurance rate: $\bar{\beta} = \beta$ if the payer covers the drug, $\bar{\beta} = 1$ otherwise
$u(\cdot)$	Patient value function
$1 - \alpha$	Patient risk sensitivity
$\lambda$	Patient loss aversion
$c$	Variable production cost
$z$	$= 1 + (\lambda(1/q - 1))^{1/\alpha}$
$q_0, q_1, \bar{q}_1$	Thresholds probabilities; $q_0 = (1 + \lambda^{-\frac{1}{1-\alpha}})^{-1}$ solves $zq = 1$ , $q_1$ solves $zq = 2/\beta$ , $\bar{q}_1$ solves $zq = 1/\beta$ ,
$q_2, q_3$	$q_2$ solves $zq = 2/(1 + \beta)$ , $q_3$ solves $(\beta^2 c^2 / (2\bar{v}))(z^2 - z/q) + z\beta c - \bar{v} = 0$ ,
$p_1, p_2, p_3$	Threshold prices; $p_1 = q(v' + \bar{v}/2)/(1 - qz\beta/2)$ , $p_2 = qv'/(1 - qz(1 + \beta)/2)$ , $\Pi_{\text{firm}}^U(\bar{p}^*; 1) = \Pi_{\text{firm}}^U(p_3; \beta)$ ,
$\bar{p}_1, \bar{p}_2, \bar{p}_3$	$\bar{p}_1 = (v' + \bar{v}/2)/(1 - \beta/2)$ , $\bar{p}_2 = 2v'/(1 - \beta)$ , $\Pi_{\text{firm}}^O(\bar{p}^*; 1) = \Pi_{\text{firm}}^O(\bar{p}_3; \beta)$
$M, M', M''$	Threshold direct benefit to the payer; $M = (1 - \beta)(\bar{v}^2/(z\beta c) + \beta c/q)/(2\beta)$ , $M' = \bar{v}(\bar{v}/(z\beta^2 c) - 1)/2 + \beta c(2/\beta - 1 - zq)/(4q)$ , $M'' = \bar{v}(\bar{v}/(zc) - 1)/2 + c(1 - zq)/(4q)$
$p^*, \bar{p}^*, \tilde{p}^*, \hat{p}^*$	Optimal interior prices with and without coverage under uniform pricing and outcome-based pricing; $p^* = c/2 + \bar{v}/(2\beta z)$ , $\bar{p}^* = c/2 + \bar{v}/(2z)$ , $\tilde{p}^* = c/(2q) + \bar{v}/(2\beta)$ , $\hat{p}^* = c/(2q) + \bar{v}/2$
$U$	Upperscript for uniform pricing system
$O$	Upperscript for outcome-based pricing system
$p^j$	Price under system $j$ , $j \in \{U, O\}$
$N^j$	Demand under system $j$ , $j \in \{U, O\}$
$\Pi_k^j$	Total payoff for agent $k$ under system $j$ , where $k \in \{\text{patient, firm, payer}\}$ and $j \in \{U, O\}$
$W_k^j$	Total welfare for agent $k$ under system $j$ , where $k \in \{\text{patient, payer}\}$ and $j \in \{U, O\}$
$\Delta^j$	Difference in payer objective between offering and not offering coverage under system $j$ , $j \in \{U, O\}$

## Appendix B: Supplemental Figures and Tables

Figure 7 illustrates the interpretation of quantity  $z$  for a given  $q$ .

Table 5 summarizes the results of Proposition 5.

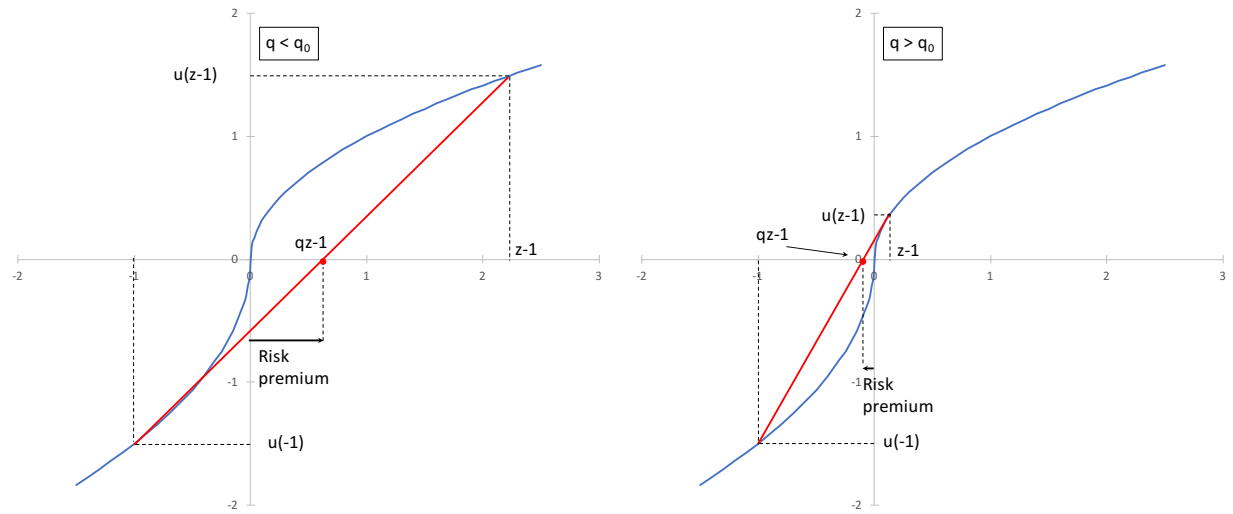
Table 6 shows how the scenarios in the numerical robustness study of Section 5.3 are distributed among all the possible regions of price and coverage outcomes defined by Theorems 1 and 2..

Figure 8 illustrates the optimal pricing strategies under outcome-based pricing for high risk sensitivity ( $1 - \alpha = 0.7$ ) in the Repatha example of Section 5.

Figure 9 shows the payer payoff and patient welfare for high risk sensitivity ( $1 - \alpha = 0.7$ ) in the Repatha example of Section 5.

Figure 10 illustrates on an example the regions where, respectively, the firm profit and the payer payoff are improved under outcome-based pricing relative to uniform pricing (left panel). It also shows the regions

**Figure 7** Illustration of parameter  $z$



Note:  $\alpha = 0.5$ ,  $\lambda = 1.5$  (i.e.,  $q_0 = 0.69$ ), and  $q = 0.5$  (left panel) and  $q = 0.8$  (right panel)

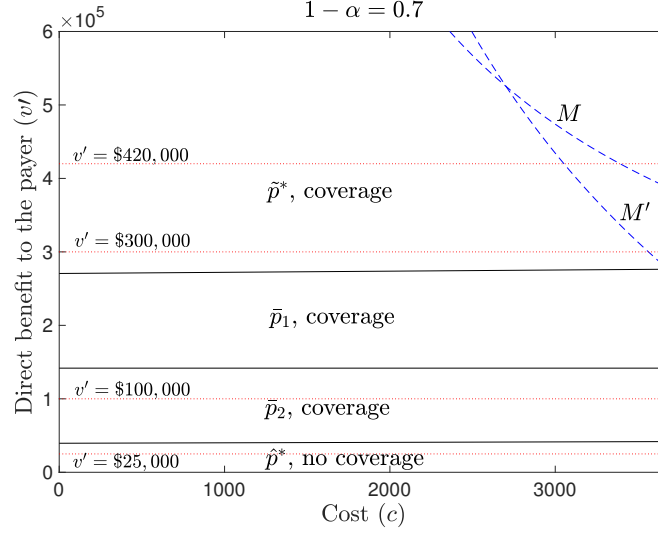
**Table 5** Summary of comparison of outcome-based pricing vs. uniform pricing under the assumptions of Proposition 5

	High-risk drug ( $q < q_0$ )	Low-risk drug ( $q > q_0$ )
Price higher under:	Outcome-based pricing	Outcome-based pricing
Demand higher under:	Outcome-based pricing	Uniform pricing
Firm profit higher under:	Outcome-based pricing	Uniform pricing
Patient payoff higher under:	Uniform pricing	Outcome-based pricing
Payer payoff higher under:	Outcome-based pricing	Uniform pricing
Payer objective higher under:	Uniform pricing	Outcome-based pricing

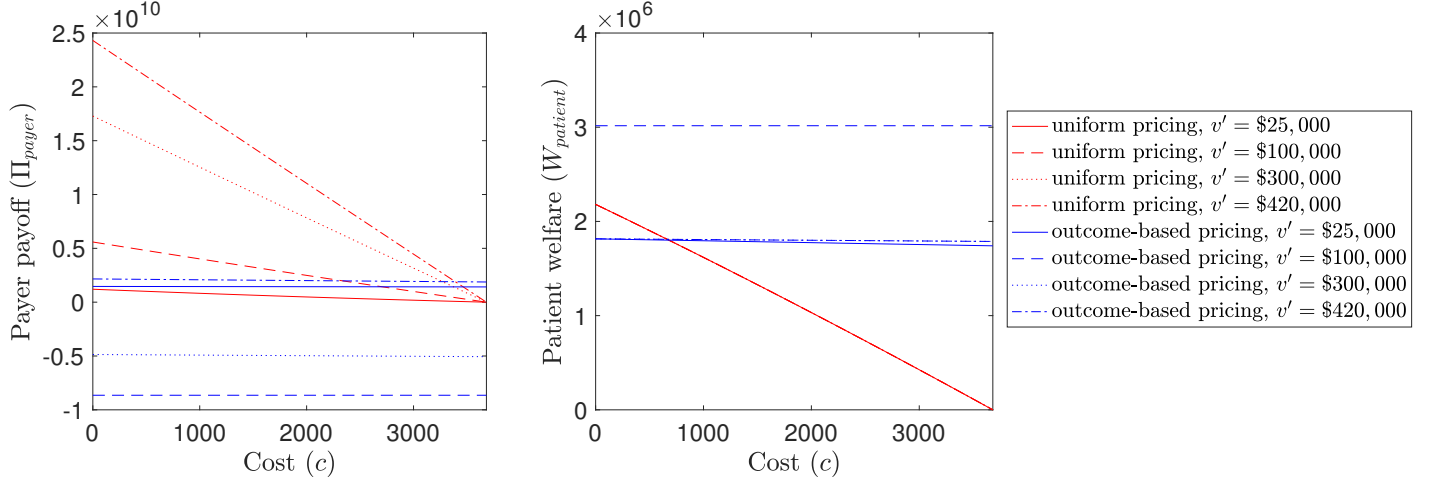
**Table 6** Distribution of the scenarios in the numerical robustness study of Section 5.3 among all the possible regions of price and coverage outcomes

		Theorem 2 case (outcome-based pricing)					Total
		1	2	3	4	5	
Theorem 1 case (uniform pricing)	1	12,344	8138	3016	1307	173	24,978
	2	245	1815	354	500	159	3073
	3	14	152	118	81	0	365
	4	0	50	53	338	70	511
	5	0	4	0	120	1574	1698
Total		12,603	10,159	3541	2346	1976	30,625

where there exists a transfer payment between firm and payer that makes outcome-based pricing better than uniform pricing for both parties (right panel). In the example used in this illustration, there are 13 different regions (delimited by the red and blue lines). The top-most region, that corresponds to the region above both the highest blue line and the highest red line, is where Assumption 2 is valid (high  $v'$ ). In the 12 remaining regions, the assumption is violated. In particular, the region in the bottom right that lies below both the lowest blue line and the lowest red line is where the assumptions of Proposition 5 are valid.

**Figure 8 Outcome-based pricing optimal price and payer coverage decision under high risk sensitivity**

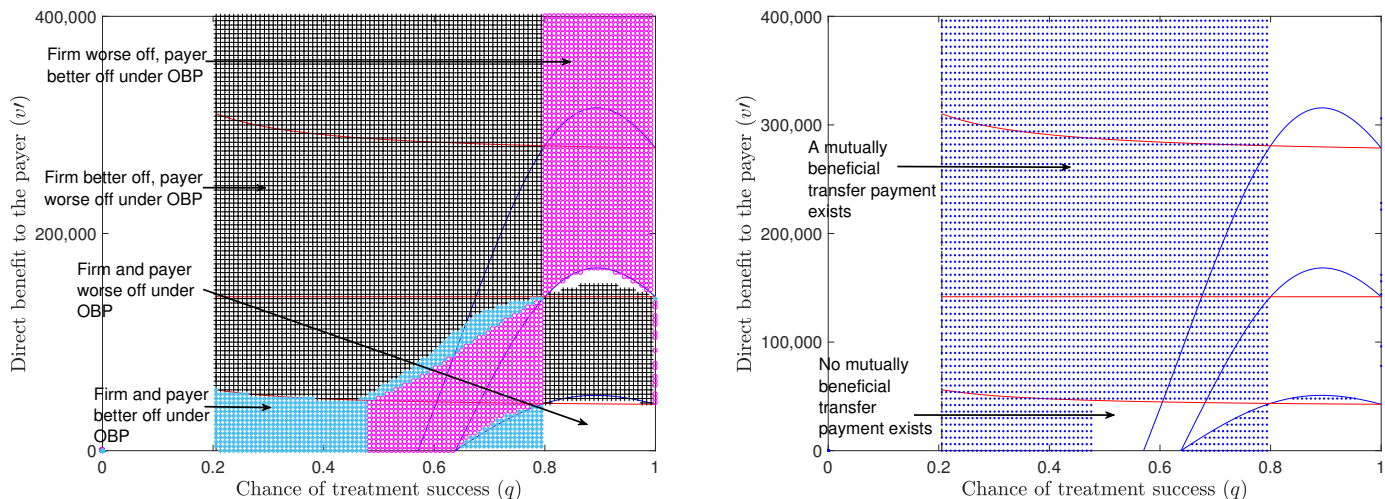
Note: the solid black lines are the limits of the price regions, the dotted red lines are the scenarios of values of  $v'$ .

**Figure 9 Payer payoff and cumulative patient welfare (low risk sensitivity)**

We observe from the left panel of the figure that, even when Assumption 2 does not hold, in most cases the firm's and the payer's interests remain at odds (black and magenta zones). However, some exceptions exist – in the areas in blue or white. The main one applies when the benefit to the payer is very low (i.e., region below the lowest red line, so there is no coverage under outcome-based pricing), and either the chance of treatment success is very low (i.e., below about 48%) or it is high enough so there is also no coverage under uniform pricing (i.e., region below the lowest blue line): then the firm and the payer's interests are aligned. There is also a sizeable region (black region to the right of  $q_0$  and magenta region to the left of  $q_0$ ) where, while the interest of the firm and payer are at odds, outcome-based pricing benefits the firm for low-risk drugs and the payer for high-risk drugs, rather than the other way around as stated in Proposition 3 under Assumption 2.



**Figure 10** Regions where (a) only the firm benefits (black-marker zones), only the payer benefits (magenta-marker zones), both benefit (blue-marker zones), and none benefits (no marker) from outcome-based pricing; (b) there exists a transfer payment between firm and payer that makes outcome-based pricing better than uniform pricing for both parties (blue-marker zones)



Note:  $\bar{v} = 450,000$ ,  $\beta = 37\%$ ,  $\alpha = 0.5$ ,  $\lambda = 2$ ,  $c = 20,000$ ,  $q_0 = 0.8$ . Red lines are the outcome-based pricing price and coverage regions; blue lines are the uniform pricing price and coverage regions.

We observe from the right panel of the figure that, even when Assumption 2 does not hold, Proposition 4 remains valid in the large majority of cases: there exists a transfer payment between firm and payer primarily whenever the drug is high-risk. Two exceptions exist: (i) there is no transfer payment when the drug is moderately high-risk, there is coverage under uniform pricing but not under outcome-based pricing – in the region in white to the left of  $q_0$ , (ii) there is a transfer payment when the drug is low-risk, there is coverage under outcome-based pricing but not under uniform pricing – in the narrow region with blue markers to the right of  $q_0$ .

In summary, it can be concluded from this analysis that in a large majority of cases (in particular those when there is coverage under both pricing systems), our analytical results regarding when the firm and payer benefit from outcome-based pricing and regarding the existence of a transfer payment appear to continue to hold even when Assumption 2 is invalid. However, there are non-negligible exceptions when there is no coverage under at least one of the two pricing systems. These findings are in line with those of our comprehensive numerical study of Section 5.3.

## Appendix C: Extension: Outcome-Based Pricing with Partial Rebates

In practice, a wide variety of outcome-based contracts have been used, and not all of them require a 100% reimbursement in case of treatment failure (Keohane and Petrie 2017). Some contracts require the firm to pay partial rebates if the drug does not succeed (e.g., UnitedHealthcare's agreement with Genomic Health for Oncotype DX in 2007; Merck & Co and Eli Lilly & Co. agreed to some rebates if their diabetes drugs fail to reduce patients' blood-sugar levels to pre-specified targets (Carlson et al. 2009)). Equivalently, others

require the firm to pay the cost of treating the patient further in case the drug does not achieve its goal (e.g. Health Alliance Medical Plans's agreement with Procter & Gamble and Sanofi-Aventis for Actonel in 2008) (Carlson et al. 2010). To understand the effect of a broader class of outcome-based pricing contracts, we now consider the case of outcome-based pricing with partial rebate. Namely, in case of treatment failure, the firm refunds the patients and payer fraction  $1 - \theta \in [0, 1]$  of their expenses. Note that uniform pricing is a special case of partial rebate with  $\theta = 1$  and outcome-based pricing with full rebate is a special case of partial rebate with  $\theta = 0$ . If a patient with value  $v$  purchases the drug, the agents' payoffs are thus as shown in the following table:

	Patient	Payer	Firm
treatment success	$v - \bar{\beta}p$	$v' - (1 - \bar{\beta})p$	$p - c$
treatment failure	$-\theta\bar{\beta}p$	$-\theta(1 - \bar{\beta})p$	$\theta p - c$

In particular, each agent's payoff can be seen as a linear combination of the payoff under uniform pricing and under outcome-based pricing, assigning a weight of  $\theta$  to uniform pricing and  $1 - \theta$  to outcome-based pricing.

The expected patient welfare under outcome-based pricing with partial rebate for a patient with treatment success value  $v$  who buys the drug is:

$$\begin{cases} q(v - \bar{\beta}p)^\alpha - (1 - q)\lambda(\theta\bar{\beta}p)^\alpha & \text{if } v - \bar{\beta}p \geq 0 \\ -q\lambda(-v + \bar{\beta}p)^\alpha - (1 - q)\lambda(\theta\bar{\beta}p)^\alpha & \text{else.} \end{cases}$$

Therefore, the patient buys the drug if and only if  $v \geq \bar{\beta}p\bar{z}$ , where

$$\bar{z} \equiv 1 + \theta \left( \lambda \frac{1 - q}{q} \right)^{1/\alpha} = 1 - \theta + \theta z.$$

We assume that

$$\frac{\beta c}{\bar{v}} < \frac{\theta + q(1 - \theta)}{\bar{z}}.$$

Similar to Assumption 1, the above assumption ensures that the drug can be profitable under coverage.

The drug gains a market segment if  $\bar{\beta}p\bar{z} \leq \bar{v}$ , and then the expected demand is  $N^P = \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}p\bar{z})$ . Note that  $N^P = \theta N^U + (1 - \theta)N^O$ . The total expected patient welfare is then given by the sum of each patient's welfare, that is, when  $\bar{\beta}p\bar{z} \leq \bar{v}$ ,

$$\begin{aligned} W_{\text{patient}}^P &= n \int_{\bar{\beta}p\bar{z}}^{\bar{v}} [q(v - \bar{\beta}p)^\alpha - (1 - q)\lambda(\theta\bar{\beta}p)^\alpha] \frac{1}{\bar{v}} dv \\ &= \frac{n}{\bar{v}} \left[ \frac{q}{\alpha + 1} ((\bar{v} - \bar{\beta}p)^{\alpha+1} - (\bar{\beta}p)^{\alpha+1}(\bar{z} - 1)^{\alpha+1}) - (1 - q)\lambda(\theta\bar{\beta}p)^\alpha (\bar{v} - \bar{\beta}p\bar{z}) \right]. \end{aligned}$$

The total expected patient payoff is, when  $v \geq \bar{\beta}p\bar{z}$ ,

$$\Pi_{\text{patient}}^P = n \int_{\bar{\beta}p\bar{z}}^{\bar{v}} [q(v - \bar{\beta}p) - (1 - q)\theta\bar{\beta}p] \frac{1}{\bar{v}} dv = \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}p\bar{z}) \left[ \frac{q}{2} (\bar{v} + \bar{\beta}p\bar{z}) - \bar{\beta}p(q(1 - \theta) + \theta) \right].$$

For each patient buying the drug, the payer incurs cost  $\theta(1 - \bar{\beta})p$  in case of failure and earns value  $v' - (1 - \bar{\beta})p$  in case of success; therefore, the payer's expected payoff is

$$\Pi_{\text{payer}}^P = \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}p\bar{z})^+ (qv' - q(1 - \bar{\beta})p - (1 - q)\theta(1 - \bar{\beta})p).$$

The payer decides whether or not to provide coverage so as to maximize its objective defined by

$$W_{\text{payer}}^P = \Pi_{\text{payer}}^P + \Pi_{\text{patient}}^P.$$

Let  $\Delta^P = W_{\text{payer}}^P|_{\bar{\beta}=\beta} - W_{\text{payer}}^P|_{\bar{\beta}=1}$  the difference in payer objective between offering and not offering coverage. It follows that the payer provides coverage iff  $\Delta^P \geq 0$ . The following result determines when the payer chooses to provide coverage. (The proof is similar to that of Proposition 1 and is thus omitted for brevity.)

**PROPOSITION 6.** *Under outcome-based pricing with partial rebate, (a) if  $p \geq \bar{v}/(\beta\bar{z})$ , the payer is indifferent with regards to its coverage decision; (b) if  $p < \bar{v}/(\beta\bar{z})$ , then*

1. *if  $\theta + q(1 - \theta) \leq \beta q\bar{z}/2$ , the payer offers drug coverage for any drug price;*
2. *if  $\beta q\bar{z}/2 < \theta + q(1 - \theta) \leq (1 + \beta)q\bar{z}/2$ , the payer offers drug coverage for any drug price  $p \in [0, \bar{v}/\bar{z}]$ ; for drug prices  $p \in (\bar{v}/\bar{z}, \bar{v}/(\beta\bar{z}))$  the payer offers drug coverage iff  $p \leq q(v' + \bar{v}/2)/(\theta + q(1 - \theta) - q\bar{z}\beta/2)$ ;*
3. *if  $\theta + q(1 - \theta) > (1 + \beta)q\bar{z}/2$ , for drug prices  $p \in [0, \bar{v}/\bar{z}]$ , the payer offers coverage iff  $p \leq qv' / (\theta + q(1 - \theta) - q\bar{z}(1 + \beta)/2)$ ; for drug prices  $p \in (\bar{v}/\bar{z}, \bar{v}/(\beta\bar{z}))$  the payer offers drug coverage iff  $p \leq q(v' + \bar{v}/2)/(\theta + q(1 - \theta) - q\bar{z}\beta/2)$ .*

Note that when  $\theta = 0$  (outcome-based pricing with full rebate case as studied in the main body of the paper), since  $1 + \beta < 2$ , the first two cases may not occur. When  $\theta = 1$  (uniform pricing), case 1 is equivalent to  $q \leq q_1$  and case 2 is equivalent to  $q_1 < q \leq q_2$ .

In the next step of the backward induction, the firm decides what to charge for the drug, anticipating the coverage decision by the payer and the resulting patient demand. The firm selects a price  $p$  to maximize its overall expected profit, given by

$$\Pi_{\text{firm}}^P(p; \bar{\beta}) = [p(\theta + q(1 - \theta)) - c] \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}p\bar{z})^+.$$

The next result determines the firm's optimal pricing decision in different scenarios. (The proof is similar to that of Theorem 1 and is omitted for brevity.) We use the following notations:

$$p^{P*} = \frac{c}{2(\theta + q(1 - \theta))} + \frac{\bar{v}}{2\beta\bar{z}}, \quad \bar{p}^{P*} = \frac{c}{2(\theta + q(1 - \theta))} + \frac{\bar{v}}{2\bar{z}}.$$

**THEOREM 3.** *Under outcome-based pricing with partial rebate,*

1. *if  $\theta + q(1 - \theta) \leq \beta q\bar{z}/2$  or if  $v' \geq p^{P*}[\theta + q(1 - \theta) - q\bar{z}\beta/2]/q - \bar{v}/2$ , then the firm prices at  $p^{P*}$  and the payer provides coverage;*
2. *if  $\theta + q(1 - \theta) > \beta q\bar{z}/2$  and  $\max\{c/(\theta + q(1 - \theta)), \bar{v}/\bar{z}\} \cdot [\theta + q(1 - \theta) - q\bar{z}\beta/2]/q - \bar{v}/2 < v' < p^{P*}[\theta + q(1 - \theta) - q\bar{z}\beta/2]/q - \bar{v}/2$ , then the firm prices at  $q(v' + \bar{v}/2)/(\theta + q(1 - \theta) - q\bar{z}\beta/2)$  and the payer provides coverage;*

3. if  $c/(\theta + q(1 - \theta)) \leq \bar{v}/\bar{z}$ ,  $\theta + q(1 - \theta) > (1 + \beta)q\bar{z}/2$  and  $p_3^P[\theta + q(1 - \theta) - q\bar{z}(1 + \beta)/2]/q \leq v' \leq (\bar{v}/\bar{z})[\theta + q(1 - \theta) - q\bar{z}\beta/2]/q - \bar{v}/2$ , then the firm prices at  $qv'/(\theta + q(1 - \theta) - q\bar{z}(1 + \beta)/2)$  and the payer provides coverage;
4. if  $c/(\theta + q(1 - \theta)) \leq \bar{v}/\bar{z}$ ,  $\theta + q(1 - \theta) > (1 + \beta)q\bar{z}/2$  and  $v' < p_3^P[\theta + q(1 - \theta) - q\bar{z}(1 + \beta)/2]/q$ , then the firm prices at  $\bar{p}^{P*}$  and the payer does not provide coverage;
5. if  $c/(\theta + q(1 - \theta)) > \bar{v}/\bar{z}$ ,  $\beta q\bar{z}/2 < \theta + q(1 - \theta)$  and  $v' \leq (c/q)[\theta + q(1 - \theta) - q\bar{z}\beta/2]/[\theta + q(1 - \theta)] - \bar{v}/2$ , then the firm makes no profit regardless of its price decision and there is no coverage;

where  $p_3^P$  is the unique solution on  $[c/(\theta + q(1 - \theta)), \bar{p}^{P*}]$  of the equation  $\Pi_{firm}^P(\bar{p}^{P*}; 1) = \Pi_{firm}^P(p_3^P; \beta)$ .

Outcome-based pricing with partial rebates is thus a hybrid of uniform pricing and outcome-based pricing with full rebate. It yields results that are intermediate between the results of the two payment schemes analyzed in the main body of the paper. The proximity to either uniform pricing or outcome-based pricing with full rebate is controlled by the parameter  $\theta$ . Hence, results that are true under outcome-based pricing with full rebates (e.g., the sum of payer payoff, patient payoff and firm profit is higher than under uniform pricing for a high-risk drug) remain valid with partial rebate but with a lesser intensity. In particular, the comparison of outcome-based pricing with partial rebate with uniform pricing is similar to the comparison of outcome-based pricing with full rebate with uniform pricing. Hence, partial-rebate contracts give rise to smaller gaps with uniform pricing than full-rebate contracts, but do not improve the possibility of benefiting simultaneously the payer and the firm.

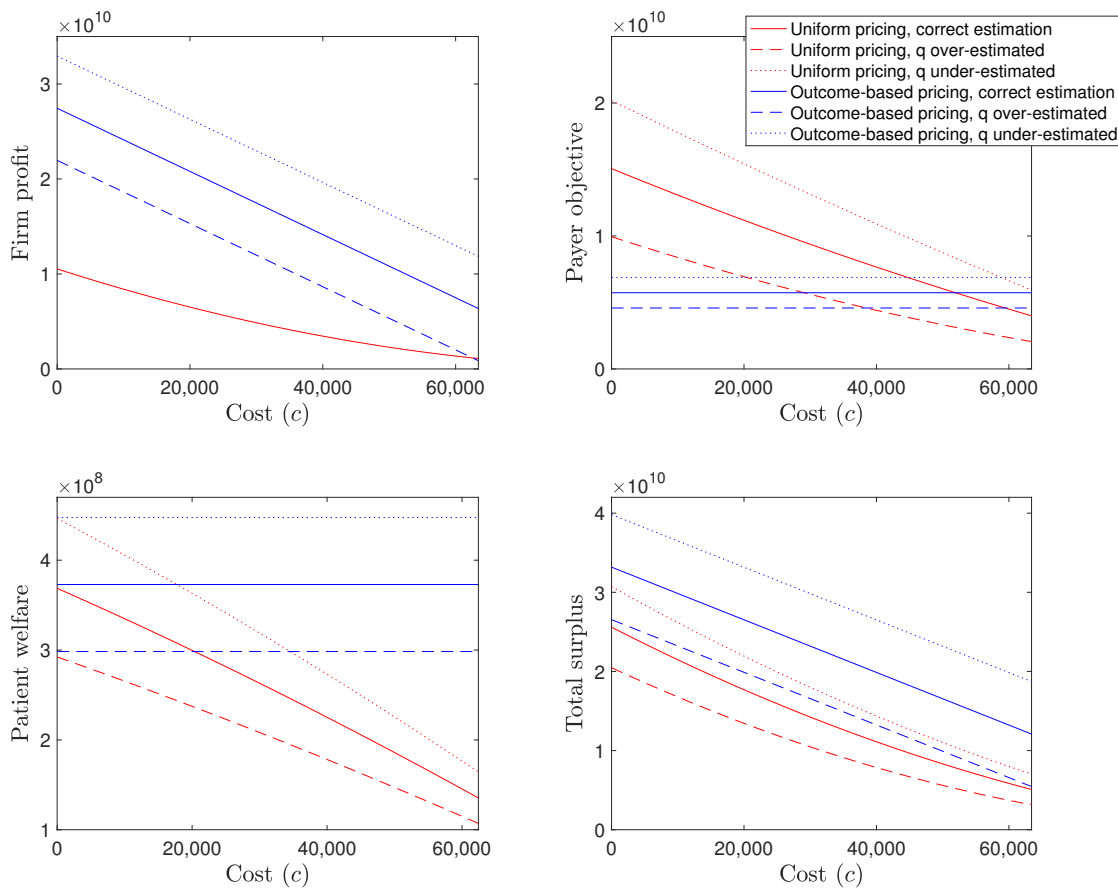
Should the rebate fraction be decided first (within  $[0, 1]$ , before the price and coverage decisions, it follows from our analysis that whoever benefits from outcome-based pricing relative to uniform pricing would prefer to set the rebate rate as high as possible, and whoever does not benefit from outcome-based pricing would prefer to set the rebate rate as low as possible. Hence, in the case of high enough  $v'$ , (a) for a high-risk drug, the firm prefers a full rebate, while the payer prefers a full rebate if  $v'$  is very high, and the payer prefers zero rebate if  $v'$  is intermediate (b) for a low-risk drug, the firm prefers zero rebate while the payer prefers zero rebate if  $v'$  is very high, and the payer prefers a full rebate if  $v'$  is intermediate.

## Appendix D: Extension: Mis-estimation of the Success Probability

In practice, estimating the chance of success for a new drug is a challenging task. The results of closely monitored medical trials administered in a carefully selected patient population are not always reproduced in a less controlled environment and in a more general population. Hence, the rate of success obtained in the course of prior medical trials may not accurately describe the chance that a general patient would reach a successful treatment outcome, and it can be unclear how to estimate the true chance of success. In this section, we examine the effect of an outcome-based pricing system when the probability of success turns out to have been mis-estimated. Let  $\tilde{q}$  be the estimate upon which patients, the payer and the firm base their decisions, and  $q$  the actual chance of success (which is found out after the decisions are made and determines the payoffs). Let  $\tilde{z} = 1 + (\lambda(1/\tilde{q} - 1))^{1/\alpha}$ . Similar to Assumption 1, we assume that  $\beta c/\bar{v} < \min\{\tilde{q}, 1/\tilde{z}, q, 1/z\}$ . The demand is based upon the *estimated* chance of success, which thus determines the price and demand.

Under uniform pricing, the firm profit is not affected by the mis-estimation because the firm does not bear any risk. The results of Proposition 1 and Theorem 1 remain valid, after substituting  $\tilde{q}$  and  $\tilde{z}$  for  $q$  and  $z$ . The actual success rate influences the realized patient welfare and realized patient payoff, and the payer's realized payoff and objective. Under outcome-based pricing, while the firm's actual profit results from the actual success rate, the firm sets its price according to its anticipated profit, which depends on the estimated probability  $\tilde{q}$ . The results of Proposition 2 and Theorem 2 remain valid, after substituting  $\tilde{q}$  and  $\tilde{z}$  for  $q$  and  $z$ .

**Figure 11** Firm profit, payer objective, patient welfare and total surplus when  $q$  is estimated correctly, over-estimated, or under-estimated



We evaluate numerically the effect of mis-estimating the chance of success. Using the same parameters as in Section 5 in the case  $v' = \$100,000$ , we use  $\tilde{q} = 26\%$  and we consider two scenarios of mis-estimation: over-estimation (i.e.,  $q = 0.8\tilde{q}$ ) and under-estimation (i.e.,  $q = 1.2\tilde{q}$ ). Figure 11 illustrates the firm profit, payer objective, patient welfare and total surplus (sum of firm profit, patient payoff and payer payoff) with low patient risk sensitivity (the case of high risk sensitivity leads to similar results and is omitted for the sake of brevity).

Under uniform pricing, mis-estimating the chance of success impacts the patients and payer. Over-estimating  $q$  leads to a lower than anticipated rate of success, and hence a lower patient welfare and payer objective. Likewise, under-estimating  $q$  leads to a higher than anticipated rate of success, which benefits the patient and the payer. The firm profit is not affected because the demand is based on the mis-estimated chance of success and the firm profit is independent of the actual outcomes.

Under outcome-based pricing, mis-estimating the chance of success impacts all agents. The firm misjudges the fraction of treatments it will receive payment for, and thus its net profit. Over-estimating  $q$  hurts the firm, as the chance of success turns out to be lower than anticipated. Even though the more frequent failures do not directly generate more expenses for the payer and the patients under outcome-based pricing, patient welfare and payer objective suffer due to lower benefits from treatment successes. Likewise, under-estimating  $q$  leads to a higher profit for the firm, higher patient welfare and higher payer objective.

One of the main findings of our paper is that the payer and the firm's interest are often at odds, except when  $v'$  is very small or the cost is high. This results remains valid under mis-estimation, as the firm remains better off under outcome-based pricing regardless of the mis-estimation scenario, and the payer prefer uniform pricing except when the cost is high. Another one of our main results is that supplementing outcome-based pricing with a transfer payment between firm and payer could yield a contract that outperforms uniform pricing for payer and firm. Examining the total surplus, we find that this result, obtained under perfect estimation of the chance of success, continues to be valid under imperfect estimation: the total surplus under outcome-based pricing remains higher than under uniform pricing both when the chance of success is under-estimated and when it is over-estimated.

## Appendix E: Extension: Symmetric Duopoly Firm Competition

In this section, we consider the case of two symmetric firms simultaneously competing in the drug market. Each firm offers a drug with the same characteristics, i.e., the same production cost, same co-insurance rate, same chance of success, and same patient and payer benefit upon success. As a result, given the option to select one of the two drugs (or not to purchase at all), the patient would select the cheaper drug, if any. Hence, the firm with the higher price captures zero demand. Regarding the firms' simultaneous price decision, each firm has an incentive to price one increment below the other to capture all the demand while maintaining profits as high as possible. It follows that in equilibrium, under uniform pricing both firms end up pricing at the marginal cost,  $c$ , and achieve no profit. (Either both receive coverage, if the cost is low enough, or none does if the cost is too high.) By the same reasoning, in equilibrium under outcome-based pricing, both firms price at the minimum price (adjusted marginal cost),  $c/q$ , and achieve no profit. This finding is consistent with the well-known result that Bertrand competition leads to firms pricing at the marginal cost and earning zero profit. Hence, the firms are indifferent between the pricing systems. It remains to determine how the patients and the payer fare under this setting.

We focus on the case when the production cost is low enough compared to potential gains by the patient and/or the payer so that the payer chooses to offer coverage when the firms price respectively at the marginal cost under uniform pricing, and at the adjusted marginal cost  $c/q$  under outcome-based pricing. Otherwise,

the drug is so expensive to produce that the payer never chooses to cover it, even when the firm prices at the lowest possible price point. A sufficient condition to ensure this is  $c < \bar{v}/z$ , which states that under uniform pricing, if the patient cost share is equal to  $c$ , then the drug attracts a non-zero demand. We also assume that the marginal production cost is low enough compared to the payer's potential benefit, i.e.,  $(1 - \beta)c < qv'$ . This assumption states that under uniform pricing, the payer's direct expected surplus from the drug treatment exceeds its cost share when the price is set to the marginal cost. These assumptions are not very restrictive as the marginal production costs of drugs tends to be low (despite high fixed cost of development) compared to their potential benefits.

Using the expressions of Sections 3.1 and 3.2, the patients' and payer's payoffs under uniform and outcome-based pricing are respectively given by:

$$\begin{aligned}\Pi_{\text{patient}}^U &= \frac{n}{\bar{v}}(\bar{v} - \bar{\beta}cz) \left[ \frac{q}{2}(\bar{v} + \bar{\beta}pz) - \bar{\beta}p \right] \\ \Pi_{\text{payer}}^U &= \frac{n}{\bar{v}}(\bar{v} - \bar{\beta}cz)^+ (qv' - (1 - \bar{\beta})c) \\ \Pi_{\text{patient}}^O &= \frac{nq}{2\bar{v}} \left( \bar{v} - \bar{\beta}\frac{c}{q} \right)^2 \\ \Pi_{\text{payer}}^O &= \frac{nq}{\bar{v}} \left( \bar{v} - \bar{\beta}\frac{c}{q} \right)^+ \left( v' - (1 - \bar{\beta})\frac{c}{q} \right).\end{aligned}$$

After simplifications, we obtain

$$\begin{aligned}\Pi_{\text{patient}}^U - \Pi_{\text{patient}}^O &= -\frac{n\beta^2 c^2 q}{2\bar{v}} \left( z - \frac{1}{q} \right)^2 \leq 0 \\ \Pi_{\text{payer}}^U - \Pi_{\text{payer}}^O &= \beta c \left( z - \frac{1}{q} \right) ((1 - \beta)c - qv').\end{aligned}$$

Therefore, the patient payoff is better off under outcome-based pricing regardless of the chance of treatment success. However, the payer's payoff benefits from outcome-based pricing if and only if the drug is low-risk.

## Appendix F: Extension: Perfectly Correlated Patient and Payer Benefits

The main body of the paper models the payer's benefit upon treatment success as a homogenous constant  $v'$ , while the patient's benefit is a variable  $V$  whose realizations  $v$  are uniformly distributed over  $[0, \bar{v}]$ . In this section, we consider the case when the payer's benefit is also heterogenous across patients, and perfectly correlated with the patient's benefit, i.e.,  $v' = kv$  where  $k > 0$  is a known constant. Under this modified model, (i) we analyze the patient, payer and firm decisions under uniform pricing and outcome-based pricing and (ii) we compare the price, demand, total expected patient payoff, expected payer payoff, and payer objective under the two pricing systems.

### F.1. Uniform pricing

The patient purchase decisions are not affected by the payer's benefit from treatment success. Hence, as shown in Section 3.1.1, the expected demand is  $\frac{n}{\bar{v}}(\bar{v} - \bar{\beta}pz)^+$  and the total expected patient payoff is

$$\Pi_{\text{patient}}^U = \frac{n}{\bar{v}}(\bar{v} - \bar{\beta}pz)^+ \left[ \frac{q}{2}(\bar{v} + \bar{\beta}pz) - \bar{\beta}p \right].$$

The payer's expected payoff is given by:

$$\begin{aligned}\Pi_{\text{payer}}^U &= \frac{n}{\bar{v}} \int_{\bar{\beta}pz}^{\bar{v}} [q(kv - (1 - \bar{\beta})p) - (1 - q)((1 - \bar{\beta})p)] dv \\ &= \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}pz)^+ \left[ \frac{qk}{2} (\bar{v} + \bar{\beta}pz) - (1 - \bar{\beta})p \right].\end{aligned}$$

Hence,

$$W_{\text{payer}}^U = \Pi_{\text{patient}}^U + \Pi_{\text{payer}}^U = \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}pz)^+ \left[ \frac{q}{2} (k + 1) (\bar{v} + \bar{\beta}pz) - p \right].$$

The payer's optimal coverage decision is as described in the following proposition.

**PROPOSITION 7.** *Under uniform pricing, (a) if  $p \geq \bar{v}/(\beta z)$ , the payer is indifferent with regards to its coverage decision; (b) if  $p < \bar{v}/(\beta z)$ , then*

1. *if  $k + 1 \geq 1/(qz\beta)$ , the payer provides drug coverage;*
2. *if  $2/(qz(\beta + 1)) \leq k + 1 < 1/(qz\beta)$ , the payer offers drug coverage for any drug price  $p \in [0, \bar{v}/z]$ ; for drug prices  $p \in (\bar{v}/z, \bar{v}/(\beta z))$ , the payer offers drug coverage iff  $p \leq \hat{p}_1 \equiv \bar{v}q(k + 1)/(2 - \beta zq(k + 1))$ ;*
3. *if  $k + 1 < 2/(qz(\beta + 1))$ , the payer does not offer drug coverage.*

The result above states that when the price is so high that there is no demand for the drug with or without coverage, the payer is indifferent. When the price is moderately high, so that there is demand for the drug with coverage, the payer unconditionally provides coverage when  $k$  is high so the payer stands to benefit a lot in case of success. Similarly, the payer does not offer coverage when  $k$  is low, i.e., when the payer does not benefit enough from treatment success. For intermediate values of  $k$ , there is coverage only when the price is sufficiently low.

We next consider the firm's pricing decision, where the firm anticipates both the payer's coverage decision and the patients' ensuing purchase decisions. The firm seeks to maximize its profit  $(n/\bar{v})(p - c)(\bar{v} - \bar{\beta}pz)$ . We thus obtain the following result for the firm's optimal pricing decision under uniform pricing.

**THEOREM 4.** *Under uniform pricing,*

1. *if  $k + 1 \geq A$ , then the firm prices at  $p^*$  and the payer provides coverage;*
2. *if  $B \leq k + 1 < A$ , then the firm prices at  $\hat{p}_1$  and the payer provides coverage;*
3. *if  $k + 1 < B$  and  $c \leq \bar{v}/z$ , then the firm prices at  $\bar{p}^*$  and the payer does not provide coverage;*
4. *if  $k + 1 < B$  and  $c > \bar{v}/z$ , then the firm makes no profit regardless of its price decision and the payer does not provide coverage,*

where

$$A \equiv \frac{2}{\beta q z} \frac{\bar{v} + c\beta z}{3\bar{v} + c\beta z}, \quad B \equiv \frac{2}{q \left( \beta z + \frac{\bar{v}}{\max\{c, \bar{v}/z\}} \right)}.$$

The result above states that when the payer benefits enough from treatment success, the firm can price at the unconstrained optimal price under coverage,  $p^*$ . For intermediate amplitude of the payer's benefit, the firm prices just high enough to ensure coverage ( $\hat{p}_1$ ). For lower values of  $k$ , the payer does not cover the drug,



and so the firm prices at the profit-maximizing price without coverage when the cost is low enough so that a profit can be made without coverage. For too high costs, the firm may not make a profit. These results mirror to a large extent those of Section 3.1 obtained when the payer's benefit is a constant.

## F.2. Outcome-based pricing

The patient purchase decisions are not affected by the payer's benefit from treatment success. Hence, as shown in Section 3.2.1, the expected demand is  $\frac{n}{\bar{v}}(\bar{v} - \bar{\beta}pz)^+$  and the total expected patient payoff is 0 if  $\bar{v} < \bar{\beta}p$ , and otherwise is given by:

$$\Pi_{\text{patient}}^O = \frac{nq}{2\bar{v}}(\bar{v} - \bar{\beta}p)^2.$$

The payer's expected payoff is given by:

$$\begin{aligned}\Pi_{\text{payer}}^O &= \frac{n}{\bar{v}} \int_{\bar{\beta}p}^{\bar{v}} [q(kv - (1 - \bar{\beta})p)] dv \\ &= \frac{nq}{\bar{v}}(\bar{v} - \bar{\beta}p)^+ \left[ \frac{k}{2}(\bar{v} + \bar{\beta}p) - (1 - \bar{\beta})p \right].\end{aligned}$$

Hence,

$$W_{\text{payer}}^O = \Pi_{\text{patient}}^O + \Pi_{\text{payer}}^O = \frac{nq}{\bar{v}}(\bar{v} - \bar{\beta}p)^+ \left[ \frac{k+1}{2}(\bar{v} + \bar{\beta}p) - p \right].$$

The payer's optimal coverage decision is as described in the following proposition.

**PROPOSITION 8.** *Under outcome-based pricing, (a) if  $p \geq \bar{v}/\beta$ , the payer is indifferent with regards to its coverage decision; (b) if  $p < \bar{v}/\beta$ , then*

1. *if  $k+1 \geq 1/\beta$ , the payer provides drug coverage;*
2. *if  $2/(\beta+1) \leq k+1 < 1/\beta$ , the payer offers drug coverage for any drug price  $p \in [0, \bar{v}]$ ; for drug prices  $p \in (\bar{v}, \bar{v}/\beta)$ , the payer offers drug coverage iff  $p \leq \hat{p}_2 \equiv \bar{v}(k+1)/(2 - \beta(k+1))$ ;*
3. *if  $k+1 < 2/(\beta+1)$ , the payer does not offer drug coverage.*

We next consider the firm's pricing decision, where the firm anticipates both the payer's coverage decision and the patients' ensuing purchase decisions. The firm seeks to maximize its profit  $(n/\bar{v})(qp - c)(\bar{v} - \bar{\beta}p)$ . We thus obtain the following result for the firm's optimal pricing decision under outcome-based pricing.

**THEOREM 5.** *Under outcome-based pricing,*

1. *if  $k+1 \geq A'$ , then the firm prices at  $\tilde{p}^*$  and the payer provides coverage;*
2. *if  $B' \leq k+1 < A'$ , then the firm prices at  $\hat{p}_2$  and the payer provides coverage;*
3. *if  $k+1 < B'$  and  $c/q \leq \bar{v}$ , then the firm prices at  $\tilde{p}^*$  and the payer does not provide coverage;*
4. *if  $k+1 < B'$  and  $c/q > \bar{v}$ , then the firm makes no profit regardless of its price decision and the payer does not provide coverage,*

where

$$A' \equiv \frac{2}{\beta} \frac{\bar{v}q + \beta c}{3\bar{v}q + \beta c}, \quad B' \equiv \frac{2}{\beta + \frac{\bar{v}}{\max\{c/q, \bar{v}\}}}.$$

The intuition is similar to that detailed under uniform pricing. These results mirror to a large extent those of Section 3.2 obtained when the payer's benefit is a constant.

### F.3. Discussion

Similar to the case with a constant payer benefit, there may be many different regions defined by a combination of optimal price under uniform pricing and outcome-based pricing. To understand the impact of perfect correlation between the payer's benefit and the patient's benefit, we study analytically the same region as that studied in Section 4.1. Namely, similar to Assumption 2, we make the following assumption.

ASSUMPTION 3. *We assume  $k + 1 \geq \max\{A, A'\}$ .*

Assumption 3 ensures that under uniform pricing, the firm prices at  $p^*$  while under outcome-based pricing, the firm prices at  $\tilde{p}^*$ . Moreover, the payer provides coverage for the drug.

The comparisons of prices, expected patient demand, firm expected profit, and total expected patient payoff obtained in Proposition 3 under Assumption 2 remain valid for the case of perfectly correlated payer and patient benefit under Assumption 3. The next result determines how the payer payoff and the payer objective under uniform pricing compares to those under outcome-based pricing when the payer's benefit is perfectly correlated to the patient's.

PROPOSITION 9. *Under Assumption 3,*

1. *The expected payer's payoff under outcome-based pricing is higher than under uniform pricing iff either (i) the drug is high-risk (i.e.,  $q < q_0$ ) and the benefit to the payer is very high (i.e.,  $k > \bar{M}$ ), or (ii) the drug is low-risk (i.e.,  $q > q_0$ ) and the benefit to the payer is moderate (i.e.,  $k < \bar{M}$ ), where*

$$\bar{M} = (1 - \beta) \frac{c \left( 1 + \frac{\bar{v}^2 q}{\beta^2 c^2 z} \right)}{\bar{v} q \left( 1 + \frac{\beta c}{2 \bar{v} q} (qz + 1) \right)}.$$

2. *The payer's objective under outcome-based pricing is higher than under uniform pricing iff either (i) the drug is high-risk (i.e.,  $q < q_0$ ) and the benefit to the payer is very high (i.e.,  $k > \bar{M}'$ ), or (ii) the drug is low-risk (i.e.,  $q > q_0$ ) and the benefit to the payer is moderate (i.e.,  $k < \bar{M}'$ ), where*

$$\bar{M}' = \frac{c \left( 1 + \frac{\bar{v}^2 q}{\beta^2 c^2 z} \right)}{\bar{v} q \left( 1 + \frac{\beta c}{2 \bar{v} q} (qz + 1) \right)} - 1.$$

The result above strongly mirrors that of Proposition 3.

PROPOSITION 10. Under Assumption 3, there exists a transfer payment between firm and payer that makes outcome-based pricing better than uniform pricing for both parties if and only if the drug is high-risk (i.e.,  $q < q_0$ ).

Hence, our results appear overall robust to the assumption of a fixed payer benefit in case of treatment success.

## Appendix G: Proofs

### Proof of Proposition 1

Let  $\Delta^U = W_{\text{payer}}^U|_{\bar{\beta}=\beta} - W_{\text{payer}}^U|_{\bar{\beta}=1}$  the difference in payer objective between offering and not offering coverage. It follows that the payer provides coverage iff  $\Delta^U \geq 0$ .

We start by showing that there exists a unique  $q_2 \in [0, 1]$  such that  $zq = 2/(1 + \beta)$ . Moreover,  $q_2 \leq q_0$ . To do so, we show that  $zq$  is a function of  $q$  that is convex in  $q$ . We then prove that it is decreasing on  $[0, q_0]$  and that  $zq > 1$  implies  $q < q_0$ . Before proceeding with the proof, we show a preliminary result.

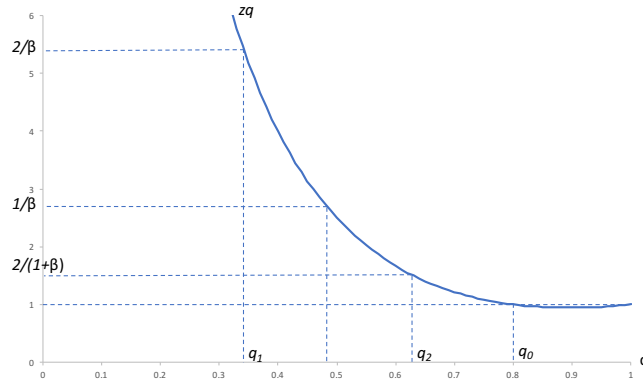
LEMMA 1. *If  $q = q_0$  or  $q = 1$ , then  $1/z = q$ . Else, we have  $1/z > q$  if and only if  $q > q_0$ .*

**Proof of Lemma 1**

$$qz - 1 = q - 1 + q \left( \lambda \frac{1-q}{q} \right)^{1/\alpha} = (1-q) \left[ \lambda^{1/\alpha} \left( \frac{1-q}{q} \right)^{1/\alpha-1} - 1 \right] = (1-q) \left[ \lambda^{1/\alpha} \left( \frac{1}{q} - 1 \right)^{\frac{1-\alpha}{\alpha}} - 1 \right].$$

Thus, since  $\alpha < 1$ , we have  $1/z = q$  iff  $q = 1$  or  $(1/q - 1)^{1-\alpha} = \lambda^{-1}$ , i.e.,  $q = q_0$ . Moreover, as long as  $q < 1$ , we have  $qz - 1 < 0$  whenever  $q > q_0$ .  $\square$

**Figure 12** Illustration of  $zq$  as a function of  $q$  for  $\beta = 0.37$ ,  $\lambda = 2$  and  $\alpha = 0.5$



We now proceed with the proof of Proposition 1. We derive

$$\frac{\partial z}{\partial q} = -\frac{z-1}{\alpha q(1-q)}.$$

It ensues that

$$\begin{aligned} \frac{\partial(zq)}{\partial q} &= 1 + (z-1) \left( 1 - \frac{1}{\alpha(1-q)} \right) \\ \frac{\partial^2(zq)}{\partial q^2} &= \frac{(z-1)(1-\alpha)}{\alpha^2 q(1-q)^2}, \end{aligned}$$

which is positive since  $\alpha < 1$  and  $z > 1$ . Therefore,  $zq$  is a convex function of  $q$ . By Lemma 1, the function  $zq$  takes value 1 at two points:  $q = q_0 < 1$  and  $q = 1$ . It follows that  $zq$  is unimodal on  $[0, 1]$ , reaching a minimum at a point located between  $q_0$  and 1. Moreover, since  $\alpha < 1$ , we have  $\lim_{q \rightarrow 0} zq = \infty$ . Thus,  $zq$  is monotonically decreasing from  $[0, q_0]$  onto  $(1, \infty)$ . Moreover, for any  $q \in [q_0, 1]$ ,  $zq \leq 1$ . Finally, observe that, since  $\beta < 1$ , we have  $2/(1 + \beta) > 1$ . It thus follows that there exists a unique  $q_2 \in [0, 1]$  such that  $zq = 2/(1 + \beta)$ , and moreover,  $q_2 \leq q_0$ . Hence, we observe that  $zq \geq 2/(1 + \beta)$  iff  $q \leq q_2$ .

We have

$$\Pi_{\text{patient}}^U = \frac{n}{\bar{v}} (\bar{v} - \bar{\beta}pz)^+ \left[ \frac{q}{2} (\bar{v} + \bar{\beta}pz) - \bar{\beta}p \right].$$

Thus, when  $\beta pz < pz \leq \bar{v}$  (i.e.,  $p \leq \bar{v}/z < \bar{v}/(\beta z)$ ), we have

$$\begin{aligned} W_{\text{payer}}^U &= \Pi_{\text{payer}}^U + \Pi_{\text{patient}}^U = \frac{n}{\bar{v}} (\bar{v} - \beta pz) (qv' - (1 - \beta)p) + \frac{n}{\bar{v}} (\bar{v} - \beta pz) \left[ \frac{q}{2} (\bar{v} + \beta pz) - \beta p \right] \\ &= \frac{n}{\bar{v}} (\bar{v} - \beta pz)^+ \left( qv' - p + \frac{q}{2} (\bar{v} + \beta pz) \right). \end{aligned}$$

Therefore,

$$\begin{aligned} \Delta^U &= \frac{n}{\bar{v}} (\bar{v} - \beta pz) \left( qv' - p + \frac{q}{2} (\bar{v} + \beta pz) \right) - \frac{n}{\bar{v}} (\bar{v} - pz) \left( qv' - p + \frac{q}{2} (\bar{v} + pz) \right) \\ &= \frac{n}{\bar{v}} pz(1 - \beta) \left[ qv' - p \left( 1 - \frac{qz}{2} (1 + \beta) \right) \right]. \end{aligned}$$

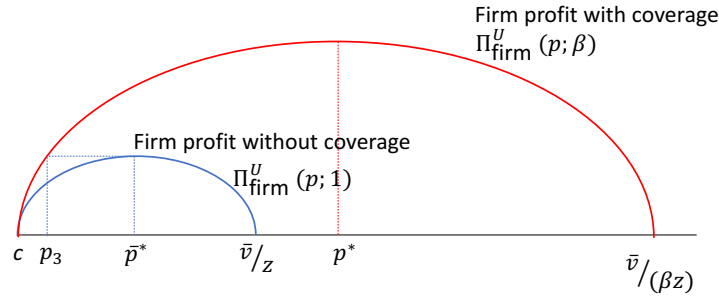
It follows that if  $1 - qz(1 + \beta)/2 \leq 0$  (i.e., if  $zq \geq 2/(1 + \beta)$ , or equivalently,  $q \leq q_2$ ), then  $\Delta^U \geq 0$  for any  $p$ . If  $1 - qz(1 + \beta)/2 > 0$ , that is, if  $q > q_2$ , then  $\Delta^U \geq 0$  iff  $p \leq p_2 \equiv qv'/(1 - qz(1 + \beta)/2)$ .

Now suppose  $\beta pz \leq \bar{v} < pz$  (i.e.,  $\bar{v}/z < p \leq \bar{v}/(\beta z)$ ), that is, there is a positive demand when the payer offers coverage, but there is no demand under no coverage by the payer. Then, when the payer offers no coverage its objective is zero (no patient payoff and no payer payoff). In that case,

$$\Delta^U = \frac{n}{\bar{v}} (\bar{v} - \beta pz) \left( qv' - p + \frac{q}{2} (\bar{v} + \beta pz) \right) = \frac{n}{\bar{v}} (\bar{v} - \beta pz) \left( q \left( v' + \frac{\bar{v}}{2} \right) - p \left( 1 - \frac{qz\beta}{2} \right) \right).$$

Similarly to the reasoning presented above, there exists a unique  $q_1 \in (0, q_2)$  such that  $zq \geq 2/\beta$  iff  $q \leq q_1$ . Hence, if  $q \leq q_1$ , then  $\Delta^U \geq 0$  for any  $p$ . If  $q > q_1$ , then  $\Delta^U \geq 0$  iff  $p \leq p_1 \equiv q(v' + \bar{v}/2)/(1 - qz\beta/2)$ .  $\square$

**Figure 13** Firm profit function with and without coverage for the proof of Theorem 1



### Proof of Theorem 1

Note that  $\beta \leq 50\%$  guarantees  $(\bar{v}/z)(1 - 1/(2\beta)) < c/2$ , and hence,  $p^* > \bar{v}/z$ .

First consider the case when  $c > \bar{v}/z$ . The relevant price domain leading to a nonzero profit for the firm is  $p \in [c, \bar{v}/(\beta z)] \subset (\bar{v}/z, \bar{v}/(\beta z)]$ . Thus, without payer coverage, there is no demand for the drug, and the firm does not achieve any profit. Furthermore, note that  $\Pi_{\text{firm}}^U(p; \beta)$  is concave in  $p$  and reaches a maximum at  $p^*$ . From Proposition 1, if  $q \leq q_1$ , the payer provides coverage regardless of the price, and if  $q > q_1$  the payer offers coverage iff  $p \leq p_1$ . Thus, if  $q > q_1$  and  $p_1 \leq c$ , for any price on the relevant domain, the payer does not offer coverage and the demand is zero. If  $q > q_1$  and  $c < p_1 < p^*$ , the firm's profit is monotonically increasing on  $[c, p_1]$ , and equals zero for prices above  $p_1$ , thus the optimal price is  $p_1$ . If  $q > q_1$  and  $p_1 > p^*$ , or if  $q \leq q_1$ , the payer offers coverage at  $p^*$  and thus the firm's profit reaches its maximum at  $p^*$ .

Now consider the case when  $c \leq \bar{v}/z$ . The relevant price domain leading to a nonzero profit for the firm is  $p \in [c, \bar{v}/(\beta z)] = [c, \bar{v}/z] \cup (\bar{v}/z, \bar{v}/(\beta z)]$ .

1. From Proposition 1, if  $q \leq q_1$ , the payer provides coverage regardless of the price. Noting that  $\Pi_{\text{firm}}^U(p; \beta) = \frac{n}{v}(p - c)(v - p\beta z)$  is concave in  $p$  on  $[c, \bar{v}/(\beta z)]$  it follows that the profit reaches a maximum at its stationary point  $p^*$ . Now suppose  $q \in (q_1, 1]$  and  $p_1 \geq p^*$ . On the domain  $(\bar{v}/z, \bar{v}/(\beta z)]$ , which contains  $p_1$ , there is coverage up to  $p_1$ . Since the payer offers coverage at  $p^*$ , the optimal price is  $p^*$ . For any given price  $p \in [c, \bar{v}/z]$ ,  $\Pi_{\text{firm}}^U(p^*; \beta) \geq \Pi_{\text{firm}}^U(p; \beta) \geq \Pi_{\text{firm}}^U(p; 1)$ , where the first inequality is due to  $\Pi_{\text{firm}}^U(p; \beta)$  being increasing in  $p$  on  $[c, p^*]$ , and the second inequality results from the observation that at a given price, a given sale brings the same revenue under coverage and under no coverage, but the demand is lower when there is no coverage. Hence, overall the optimal price is  $p^*$ . Finally, we observe that when  $q \in (q_1, \bar{q}_1]$ , then it follows that  $p_1 \geq p^*$ . The proof of this statement is as follows. By Assumption 1, we have  $c \leq \bar{v}/(\beta z)$ . Hence,  $p^* = c/2 + \bar{v}/(2\beta z) \leq \bar{v}/(\beta z)$ . Moreover, we have

$$p_1 = q \frac{v' + \frac{\bar{v}}{2}}{1 - \frac{q\beta z}{2}} \geq \frac{\bar{v}}{\frac{2}{q} - \beta z}.$$

Furthermore, when  $q \leq \bar{q}_1$ , then  $zq \geq 1/\beta$ , or equivalently,  $2z\beta \geq 2/q$ , i.e.,  $z\beta \geq 2/q - z\beta$ . It follows that  $p_1 \geq \bar{v}/(\beta z) \geq p^*$ .

2. We have  $\bar{v}/z < p_1 < p^*$ . On the domain  $(\bar{v}/z, \bar{v}/(\beta z)]$ , which contains  $p_1$ , there is coverage up to  $p_1$ . Beyond  $p_1$ , since the no-coverage demand equals zero for prices above  $\bar{v}/z$ , the profit is zero. Under coverage, the profit function is concave in  $p$  and reaches a maximum at  $p^*$ , thus it is monotonically increasing on  $(\bar{v}/z, p_1]$ , and the optimal price is  $p_1$ . Note that for any given price  $p \in [c, \bar{v}/z]$ ,  $\Pi_{\text{firm}}^U(p_1; \beta) \geq \Pi_{\text{firm}}^U(p; \beta) \geq \Pi_{\text{firm}}^U(p; 1)$ . Therefore, overall the optimal price is  $p_1$ .
3. We have  $p_1 \leq \bar{v}/z$  and  $p_2 \geq p_3$ . On the domain  $(\bar{v}/z, \bar{v}/(\beta z)]$ , the price is above  $p_1$ , and thus, from Proposition 1, there is no coverage, and hence, the firm's profit is zero regardless of the price it selects on this range. Therefore, the firm selects a price on the range  $[c, \bar{v}/z]$ . On this range, there is coverage for a price up to  $p_2$ . To the left of  $p_2$ , the profit function with coverage is monotonically increasing, and thus the firm would select price  $p_2$ . To the right of  $p_2 \geq p_3$ , the value of the profit function without coverage does not exceed  $\Pi_{\text{firm}}^U(\bar{p}^*; 1) = \Pi_{\text{firm}}^U(p_3; \beta) \leq \Pi_{\text{firm}}^U(p_2; \beta)$ . Therefore, the optimal price overall is  $p_2$ .
4. We have  $p_1 \leq \bar{v}/z$  and  $p_2 < p_3$ . Similar to the previous case, the firm selects a price on the range  $[c, \bar{v}/z]$ . First, suppose  $p_2 \leq c$ . On the domain  $[c, \bar{v}/z]$ , the price is above  $p_2$ , and thus, from Proposition 1, there is no coverage. Noting that  $\Pi_{\text{firm}}^U(p; 1) = \frac{n}{v}(p - c)(v - pz)$  is concave in  $p$  on  $[c, \bar{v}/z]$ , it follows that the profit reaches a maximum at its stationary point  $\bar{p}^*$ . Second, suppose  $c < p_2 < p_3$ . On the domain  $[c, \bar{v}/z]$ , which contains  $p_2$ , there is coverage for a price up to  $p_2$ . To the left of  $p_2 (< \bar{p}^*)$ , the profit function with coverage is monotonically increasing, and thus reaches its highest value at  $p_2$ . To the right of  $p_2$ , the profit function without coverage is concave and reaches a maximum at  $\bar{p}^*$ . Since  $\Pi_{\text{firm}}^U(\bar{p}^*; 1) = \Pi_{\text{firm}}^U(p_3; \beta) > \Pi_{\text{firm}}^U(p_2; \beta)$ , it follows that the optimal price is  $\bar{p}^*$ .
5. We have  $p_1 \leq \bar{v}/z < c$ . On the domain of prices  $[c, \bar{v}/(\beta z)]$ , the payer does not offer coverage regardless of the price, and the firm's profit without coverage is zero for all prices because the demand is zero.

It remains to prove that no other case may occur, namely, that if  $q \in (\bar{q}_1, q_2]$  then  $p_1 > \bar{v}/z$ . We proceed by contradiction. Suppose  $p_1 \leq \bar{v}/z$  and  $q \in (\bar{q}_1, q_2]$ . We have  $q > \bar{q}_1 > q_1$ . From the proof of Proposition 1,  $q > q_1$  means that  $qz < 2/\beta$ . Hence, the denominator of  $p_1$ , namely,  $1 - qz\beta/2$ , is positive. Hence,

$$p_1 \leq \frac{\bar{v}}{z} \Leftrightarrow q\left(v' + \frac{\bar{v}}{2}\right) \leq \frac{\bar{v}}{z} - \frac{q\beta\bar{v}}{2} \Leftrightarrow v' \leq \bar{v}\left(\frac{1}{zq} - \frac{1+\beta}{2}\right) < 0,$$

where the last inequality follows from observing, from the proof of Proposition 1, that  $q < q_2$  implies  $zq > 2/(1+\beta)$ . This contradicts  $v' \geq 0$ .  $\square$

### Proof of Proposition 2

When  $p \leq \bar{v}$ , we have

$$\Delta^O = \frac{nq}{\bar{v}}p(1-\beta)\left[v' - p\frac{1-\beta}{2}\right].$$

Hence, the payer offers coverage iff  $p \leq 2v'/(1-\beta) = \bar{p}_2$ .

When  $\bar{v} < p$  ( $< \bar{v}/\beta$ ), we have

$$\Delta^O = \frac{nq}{\bar{v}}(\bar{v} - \beta p)\left(v' - p + \frac{\bar{v} + \beta p}{2}\right).$$

Hence, the payer offers coverage iff  $p \leq (v' + \bar{v}/2)/(1 - \beta/2) = \bar{p}_1$ .  $\square$

### Proof of Theorem 2

Note that  $\beta \leq 50\%$  guarantees  $\bar{v}(1 - 1/(2\beta)) < c/(2q)$ , and hence,  $\tilde{p}^* > \bar{v}$ .

The rest of the proof is identical to the proof of Theorem 1 with  $q > q_2$  and is thus omitted for brevity.  $\square$

### Proof of Proposition 3

1. Price comparison: we have

$$p^O - p^U = \tilde{p}^* - p^* = \frac{c}{2q} + \frac{\bar{v}}{2\beta} - \frac{c}{2} - \frac{\bar{v}}{2\beta z} = \frac{c}{2}\left(\frac{1}{q} - 1\right) + \frac{\bar{v}}{2\beta}\left(1 - \frac{1}{z}\right) = \frac{c}{2}\frac{1-q}{q} + \frac{\bar{v}}{2\beta}\left(\frac{z-1}{z}\right) > 0.$$

2. Demand comparison: the demand under uniform pricing is  $N^U = (n/\bar{v})(\bar{v} - \beta zp^U)$ , while under outcome-based pricing it is equal to  $N^O = (n/\bar{v})(\bar{v} - \beta p^O)$ . We have

$$N^O - N^U = \frac{n\beta}{\bar{v}}(zp^U - p^O) = \frac{n\beta c}{2\bar{v}}\left(z - \frac{1}{q}\right) = \frac{n\beta c}{2\bar{v}q}(qz - 1).$$

The result on the demand then follows from Lemma 1.

3. Firm profit comparison: after substituting the optimal prices into the profit function expressions, we have

$$\begin{aligned} \Pi_{\text{firm}}^O(p^O; \beta) - \Pi_{\text{firm}}^U(p^U; \beta) &= n\frac{(q\bar{v} - \beta c)^2}{4\bar{v}\beta q} - n\frac{(\bar{v} - c\beta z)^2}{4\bar{v}\beta z} \\ &= \frac{n}{4\bar{v}\beta}\left(\frac{(q\bar{v} - \beta c)^2}{q} - \frac{(\bar{v} - c\beta z)^2}{z}\right) \\ &= \frac{n}{4\bar{v}\beta}\left(q\bar{v}^2 + \frac{c^2\beta^2}{q} - \frac{\bar{v}^2}{z} - \beta^2 c^2 z\right) \\ &= \frac{n}{4\bar{v}\beta}(qz - 1)\left(\frac{\bar{v}^2}{z} - \frac{c^2\beta^2}{q}\right). \end{aligned}$$

By Assumption 1, we have  $\beta c/\bar{v} < q, 1/z$ . It follows that  $(\beta c/\bar{v})^2 < q/z$ , and hence,  $\bar{v}^2/z - \beta^2 c^2/q > 0$ . The result on the firm profit then follows from Lemma 1.

4. Patient payoff comparison: we have

$$\begin{aligned}\Pi_{\text{patient}}^O - \Pi_{\text{patient}}^U &= \frac{nq}{2\bar{v}}(\bar{v} - \beta p^O)^2 - \frac{n}{\bar{v}}(\bar{v} - \beta p^U z) \left[ \frac{q}{2}(\bar{v} + \beta p^U z) - \beta p^U \right] \\ &= N^O \frac{q}{2}(\bar{v} - \beta p^O) - N^U \left[ \frac{q}{2}\bar{v} - \beta p^U \left(1 - \frac{qz}{2}\right) \right] \\ &= \frac{q}{2}\bar{v}(N^O - N^U) - \frac{\beta q}{2}N^O p^O + \beta \left(1 - \frac{qz}{2}\right) N^U p^U \\ &= \frac{q}{2}\bar{v} \frac{n\beta c}{2\bar{v}q}(qz - 1) - \frac{\beta q}{2} \frac{n}{4\beta\bar{v}} \left( \bar{v}^2 - \frac{c^2\beta^2}{q^2} \right) + \beta \left(1 - \frac{qz}{2}\right) \frac{n}{4\bar{v}\beta z}(\bar{v}^2 - c^2\beta^2 z^2) \\ &= \frac{n\beta c}{4}(qz - 1) - \frac{qn\bar{v}}{8} + \frac{qn}{8\bar{v}} \frac{c^2\beta^2}{q^2} + \frac{n\bar{v}}{4z} - \frac{nc^2\beta^2 z}{4\bar{v}} - \frac{qn\bar{v}}{8} + \frac{nqc^2\beta^2 z^2}{8\bar{v}} \\ &= \frac{n\beta c}{4}(qz - 1) - \frac{n\bar{v}}{4z}(qz - 1) + \frac{nc^2\beta^2}{8q\bar{v}}(qz - 1)^2 \\ &= \frac{n\beta c}{4}(qz - 1) \left[ 1 - \frac{\bar{v}}{z\beta c} + \frac{\beta c}{2q\bar{v}}(qz - 1) \right].\end{aligned}$$

By Assumption 1, we have  $1 - \bar{v}/z\beta c < 0$ . Hence, if  $qz - 1 < 0$  (i.e.,  $q > q_0$ ), then  $\Pi_{\text{patient}}^O - \Pi_{\text{patient}}^U > 0$ . Now suppose  $qz - 1 > 0$  (i.e.,  $q < q_0$ ). It remains to determine the sign of

$$1 - \frac{\bar{v}}{z\beta c} + \frac{\beta c}{2q\bar{v}}(qz - 1)$$

for  $q < q_0$ . We have

$$\begin{aligned}1 - \frac{\bar{v}}{z\beta c} + \frac{\beta c}{2q\bar{v}}(qz - 1) &> 0 \Leftrightarrow \frac{\beta c}{2q\bar{v}}(qz - 1) > \frac{\bar{v}}{z\beta c} - 1 \\ &\Leftrightarrow \frac{\beta^2 c^2}{2\bar{v}} \cdot \frac{1}{q} \cdot z \cdot (qz - 1) + z\beta c - \bar{v} > 0.\end{aligned}$$

Let

$$\varphi(q) \equiv \frac{\beta^2 c^2}{2\bar{v}} \frac{1}{q} z(qz - 1) + z\beta c - \bar{v}.$$

Note that  $1/q$  is decreasing in  $q$  and, from the proof of Proposition 1,  $z$  is decreasing in  $q$  and, for  $q < q_0$ ,  $qz$  is decreasing in  $q$ . Hence  $\varphi(q)$  is decreasing in  $q$ . Furthermore,  $\lim_{q \rightarrow 0^+} \varphi(q) = \infty$  because  $\varphi(q) = \beta^2 c^2(z^2 - z/q)/(2\bar{v}) + z\beta c - \bar{v} = 0$  and as  $q$  approaches  $0^+$ , we have  $z^2 = O((1/q)^{2/\alpha})$ ,  $z/q = O((1/q)^{1/\alpha+1})$ , and  $z = O((1/q)^{1/\alpha})$ ; hence, the term in  $z^2$  dominates. Moreover,  $\lim_{q \rightarrow q_0^-} \varphi(q) = z_0\beta c - \bar{v} = \beta c/q_0 - \bar{v}$  where  $z_0$  is the value of  $z$  for  $q = q_0$ . (Note that Assumption 1 ensures that  $\beta c/q - \bar{v} < 0$ , but  $\beta c/q_0 - \bar{v}$  may in general be positive or negative.) Therefore, when  $\beta c/q_0 - \bar{v} < 0$ , by continuity of  $\varphi(q)$ , when there exists  $q_3 \in (0, q_0)$  such that  $\varphi(q_3) = 0$ . It follows that, for  $q < q_0$ ,  $\varphi(q) > 0$  iff  $q < q_3$ . Thus,  $\Pi_{\text{patient}}^O - \Pi_{\text{patient}}^U > 0$  iff  $q > q_0$  or  $q < q_3$ . When  $\beta c/q_0 - \bar{v} > 0$ , since  $\varphi(q)$  is decreasing in  $q$ , then  $\varphi(q) > 0$  on  $[0, q_0]$ .

5. Payer payoff comparison: we have

$$\begin{aligned}\Pi_{\text{payer}}^O - \Pi_{\text{payer}}^U &= \frac{n}{\bar{v}} [(\bar{v} - \beta p^O)q(v' - (1 - \beta)p^O) - (\bar{v} - \beta p^U z)(qv' - (1 - \beta)p^U)] \\ &= N^O q(v' - (1 - \beta)p^O) - N^U (qv' - (1 - \beta)p^U)\end{aligned}$$

$$\begin{aligned}
&= qv'(N^O - N^U) - (1 - \beta)(qN^O p^O - N^U p^U) \\
&= qv' \frac{n\beta c}{2\bar{v}q} (qz - 1) - (1 - \beta) \left[ q \frac{n}{4\beta\bar{v}} \left( \bar{v}^2 - \frac{c^2\beta^2}{q^2} \right) - \frac{n}{4\bar{v}\beta z} (\bar{v}^2 - c^2\beta^2 z^2) \right] \\
&= v' \frac{n\beta c}{2\bar{v}} (qz - 1) - (1 - \beta) \frac{n}{4\beta\bar{v}} (qz - 1) \left( \frac{\bar{v}^2}{z} + \frac{c^2\beta^2}{q} \right) \\
&= \frac{n\beta c}{2\bar{v}} (qz - 1) \left[ v' - \frac{1 - \beta}{2\beta} \left( \frac{\bar{v}^2}{z\beta c} + \frac{\beta c}{q} \right) \right] \\
&= \frac{n\beta c}{2\bar{v}} (qz - 1) (v' - M).
\end{aligned}$$

6. Payer objective comparison: we have

$$\begin{aligned}
W_{\text{payer}}^O - W_{\text{payer}}^U &= \Pi_{\text{payer}}^O - \Pi_{\text{payer}}^U + \Pi_{\text{patient}}^O - \Pi_{\text{patient}}^U \\
&= \frac{n\beta c}{2\bar{v}} (qz - 1) \left[ v' - \frac{1 - \beta}{2\beta} \left( \frac{\bar{v}^2}{z\beta c} + \frac{\beta c}{q} \right) \right] + \frac{n\beta c}{4} (qz - 1) \left[ 1 - \frac{\bar{v}}{z\beta c} + \frac{\beta c}{2q\bar{v}} (qz - 1) \right] \\
&= \frac{n\beta c}{4\bar{v}} (qz - 1) \left[ 2v' - \frac{1 - \beta}{\beta} \left( \frac{\bar{v}^2}{z\beta c} + \frac{\beta c}{q} \right) + \bar{v} - \frac{\bar{v}^2}{z\beta c} + \frac{\beta c}{2q} (qz - 1) \right] \\
&= \frac{n\beta c}{4\bar{v}} (qz - 1) \left[ 2v' - \frac{\bar{v}^2}{z\beta^2 c} - \frac{c}{q} + \frac{\bar{v}^2}{z\beta c} + \frac{\beta c}{q} + \bar{v} - \frac{\bar{v}^2}{z\beta c} + \frac{\beta cz}{2} - \frac{\beta c}{2q} \right] \\
&= \frac{n\beta c}{4\bar{v}} (qz - 1) \left[ 2v' - \frac{\bar{v}^2}{z\beta^2 c} - \frac{c}{q} + \frac{\beta c}{2q} + \bar{v} + \frac{\beta cz}{2} \right] \\
&= \frac{n\beta c}{2\bar{v}} (qz - 1) (v' - M').
\end{aligned}$$

□

#### Proof of Proposition 4

We have

$$\begin{aligned}
&\Pi_{\text{firm}}^O(p^O; \beta) + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U(p^U; \beta) + W_{\text{payer}}^U) \\
&= \frac{n}{4\bar{v}\beta} (qz - 1) \left( \frac{\bar{v}^2}{z} - \frac{c^2\beta^2}{q} \right) + \frac{n\beta c}{4\bar{v}} (qz - 1) \left[ 2v' - \frac{\bar{v}^2}{z\beta^2 c} - \frac{c}{q} + \frac{\beta c}{2q} + \bar{v} + \frac{\beta cz}{2} \right] \\
&= \frac{n\beta c}{4\bar{v}} (qz - 1) \left( 2v' + \bar{v} - \frac{2c}{q} + \frac{\beta c}{2q} + \frac{\beta cz}{2} \right).
\end{aligned}$$

First, consider the case  $zq > 1$  (i.e.,  $q < q_0$ ). By Assumption 2,

$$v' \geq \tilde{p}^* \left( 1 - \frac{\beta}{2} \right) - \frac{\bar{v}}{2} = \frac{c}{2q} - \frac{c\beta}{4q} + \frac{\bar{v}}{2\beta} - \frac{3\bar{v}}{4}.$$

It follows that

$$\begin{aligned}
\Pi_{\text{firm}}^O(p^O; \beta) + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U(p^U; \beta) + W_{\text{payer}}^U) &\geq \frac{n\beta c}{4\bar{v}} (qz - 1) \left( -\frac{c}{q} + \frac{\bar{v}}{\beta} - \frac{\bar{v}}{2} + \frac{\beta cz}{2} \right) \\
&> \frac{n\beta c}{4\bar{v}} (qz - 1) \left( -\frac{c}{q} + \bar{v} \left( \frac{1}{\beta} - \frac{1}{2} \right) + \frac{\beta c}{2q} \right),
\end{aligned}$$

where the last inequality is due to  $z > 1/q$ . By Assumption 1,  $\bar{v} > \beta c/q$ . Hence, since  $1/\beta > 1 > 1/2$ , we have

$$\Pi_{\text{firm}}^O(p^O; \beta) + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U(p^U; \beta) + W_{\text{payer}}^U) > \frac{n\beta c}{4\bar{v}} (qz - 1) \left( -\frac{c}{q} + \frac{\beta c}{q} \left( \frac{1}{\beta} - \frac{1}{2} \right) + \frac{\beta c}{2q} \right) = 0.$$



Next, consider the case when  $zq < 1$  (i.e.,  $q > q_0$ ). By Assumption 2,

$$v' \geq p^* \left( \frac{1}{q} - \frac{z\beta}{2} \right) - \frac{\bar{v}}{2} = \frac{c}{2q} - \frac{cz\beta}{4} + \frac{\bar{v}}{2\beta zq} - \frac{3\bar{v}}{4}.$$

It follows that

$$\begin{aligned} 2v' + \bar{v} - \frac{2c}{q} + \frac{\beta c}{2q} + \frac{\beta cz}{2} &\geq \frac{c}{q} - \frac{cz\beta}{2} + \frac{\bar{v}}{\beta zq} - \frac{3\bar{v}}{2} + \bar{v} - \frac{2c}{q} + \frac{\beta c}{2q} + \frac{\beta cz}{2} \\ &= -\frac{c}{q} + \bar{v} \left( \frac{1}{\beta zq} - \frac{1}{2} \right) + \frac{\beta c}{2q} \\ &> -\frac{c}{q} + \bar{v} \left( \frac{1}{\beta zq} - \frac{1}{2} \right) + \frac{\beta cz}{2}, \end{aligned}$$

where the last inequality is due to  $1/q > z$ . By Assumption 1,  $\bar{v} > \beta cz$ . Since  $1/(zq) > 1$  and  $1/\beta > 1 > 1/2$ , we have  $1/(\beta qz) - 1/2 > 0$ . Hence,

$$2v' + \bar{v} - \frac{2c}{q} + \frac{\beta c}{2q} + \frac{\beta cz}{2} > -\frac{c}{q} + \beta cz \left( \frac{1}{\beta zq} - \frac{1}{2} \right) + \frac{\beta cz}{2} = 0.$$

Therefore,  $\Pi_{\text{firm}}^O(p^O; \beta) + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U(p^U; \beta) + W_{\text{payer}}^U)$  has the same sign as  $qz - 1$ , that is, it is negative.  $\square$

### Proof of Proposition 5

Based on Theorem 1 part 4 and Theorem 2 part 4, in the case considered in the proposition, the firm prices at  $\bar{p}^*$  and the payer does not provide coverage under uniform pricing, and the firm prices at  $\hat{p}^*$  and the payer does not provide coverage under outcome-based pricing. Note that the prices are equal to those of Proposition 3 after substituting  $\beta = 1$ .

1. The results follow directly from the proof of Proposition 3 after substituting  $\beta = 1$ .
2. Using the proof of Proposition 3 after substituting  $\beta = 1$ , we obtain that the patients' payoff is higher under outcome-based pricing when  $q > q_0$ . When  $q < q_0$ ,  $\Pi_{\text{patient}}^O - \Pi_{\text{patient}}^U$  has the sign of  $1 - \bar{v}/(zc) + (qz - 1)c/(2q\bar{v})$ . Since we assumed  $M'' \geq 0$ , we have  $\bar{v}(\bar{v}/(zc) - 1)/2 \geq c(qz - 1)/(4q)$ . Hence,

$$1 - \frac{\bar{v}}{zc} + \frac{c}{2q\bar{v}}(qz - 1) \leq 1 - \frac{\bar{v}}{zc} + \frac{c}{2q\bar{v}} \frac{2q\bar{v}}{c} \left( \frac{\bar{v}}{zc} - 1 \right) = 0.$$

3. The results follow directly from the proof of Proposition 3 after substituting  $\beta = 1$  and noting that  $M = 0$  when  $\beta = 1$ .
4. The results follow directly from the proof of Proposition 3 after substituting  $\beta = 1$  and noting that  $M'$  takes the value  $M''$  when  $\beta = 1$ .
5. From the proof of Proposition 4, we have

$$\Pi_{\text{firm}}^O + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U + W_{\text{payer}}^U) = \frac{nc}{4\bar{v}}(qz - 1) \left( 2v' + \bar{v} - \frac{2c}{q} + \frac{c}{2q} + \frac{cz}{2} \right) = \frac{nc}{4\bar{v}}(qz - 1) \left( 2v' + \bar{v} - \frac{3c}{2q} + \frac{cz}{2} \right).$$

First, consider the case  $zq > 1$  (i.e.,  $q < q_0$ ). Since  $v' \geq 0$ , we have

$$\Pi_{\text{firm}}^O + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U + W_{\text{payer}}^U) \geq \frac{nc}{4\bar{v}}(qz - 1) \left( \bar{v} - \frac{c}{q} - \frac{c}{2q} + \frac{cz}{2} \right)$$

$$= \frac{nc}{4\bar{v}}(qz - 1) \left( \left( \bar{v} - \frac{c}{q} \right) + \frac{c}{2} \left( z - \frac{1}{q} \right) \right) \geq 0$$

where the last inequality follows from  $c \leq q\bar{v}$  and  $z > 1/q$ .

Next, consider the case  $zq < 1$  (i.e.,  $q > q_0$ ) and  $2v' + \bar{v} \geq (c/2)(3/q - z)$ . It follows that  $\Pi_{\text{firm}}^O + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U + W_{\text{payer}}^U) \leq 0$ . Finally, consider the case  $zq < 1$  (i.e.,  $q > q_0$ ) and  $2v' + \bar{v} < (c/2)(3/q - z)$ . It follows that  $\Pi_{\text{firm}}^O + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U + W_{\text{payer}}^U) > 0$ .

Note that even under the constraints imposed on  $\bar{v}$  in the proposition ( $\bar{v} > cz, c/q$  and  $M'' \geq 0$ ), it is possible to have either  $\bar{v} < (c/2)(3/q - z)$  or  $\bar{v} > (c/2)(3/q - z)$  when  $q > q_0$ . In the former case, the threshold imposed onto  $v'$  (i.e.,  $2v' \geq (c/2)(3/q - z) - \bar{v}$ ) is positive, and in the latter case, the threshold is negative, implying that  $v'$  automatically satisfies it. For instance, consider the example  $\alpha = 0.5, \lambda = 2, q = 0.82$ . Then  $q_0 = 0.8$ , so we have  $q > q_0$ . If  $\bar{v}/c = 1.23$ , then  $\bar{v}/c > 1/q > z$  and  $M'' > 0$ , but  $\bar{v}/c < (1/2)(3/q - z)$ . If  $\bar{v}/c = 1.25$ , then we still have  $\bar{v}/c > 1/q > z$  and  $M'' > 0$ , but  $\bar{v}/c > (1/2)(3/q - z)$ .  $\square$

### Proof of Proposition 6

When  $p \leq \bar{v}/\bar{z}$ , we obtain after simplifications

$$\Delta^P = \frac{n}{\bar{v}} p \bar{z} (1 - \beta) \left[ qv' + p(1 + \beta) \frac{q\bar{z}}{2} - p(\theta + q(1 - \theta)) \right].$$

Hence, if  $(1 + \beta)q\bar{z}/2 \geq \theta + q(1 - \theta)$ , then  $\Delta^P \geq 0$  for all prices. Otherwise, for  $(1 + \beta)q\bar{z}/2 < \theta + q(1 - \theta)$ , then  $\Delta^P \geq 0$  iff  $p \leq qv' / (\theta + q(1 - \theta) - q\bar{z}(1 + \beta)/2)$ .

When  $\bar{v}/\bar{z} < p \leq \bar{v}/(\beta\bar{z})$ , then

$$\Delta^P = \frac{n}{\bar{v}} (\bar{v} - \beta p \bar{z}) \left[ qv' + \frac{q}{2} (\bar{v} + \beta p \bar{z}) - p(q(1 - \theta) + \theta) \right].$$

Hence, if  $q\bar{z}/2 \geq \theta + q(1 - \theta)$ , then  $\Delta^P \geq 0$  for all prices. Otherwise, for  $q\bar{z}/2 < \theta + q(1 - \theta)$ , then  $\Delta^P \geq 0$  iff  $p \leq q(v' + \bar{v}/2) / (q(1 - \theta) + \theta - \beta q\bar{z}/2)$ .  $\square$

### Proof of Proposition 7

If  $p \geq \bar{v}/(\beta z)$ , the payer is indifferent between covering and not covering the drug as  $W_{\text{payer}}^U|_{\bar{\beta}=\beta} = W_{\text{payer}}^U|_{\bar{\beta}=1} = 0$ .

If  $\bar{v}/z \leq p < \bar{v}/(\beta z)$ , the payer offers coverage iff  $W_{\text{payer}}^U|_{\bar{\beta}=\beta} \geq 0$ , that is, iff  $(q/2)(k + 1)(\bar{v} + \beta p z) - p \geq 0$ . Hence, when  $qz > 2/(\beta(k + 1))$ , the payer offers coverage for any price, but otherwise it only offers coverage when  $p \leq \hat{p}_1$ . Observe that

$$\hat{p}_1 \geq \bar{v}/z \Leftrightarrow qz \geq 2/((k + 1)(\beta + 1)); \quad \hat{p}_1 < \bar{v}/(\beta z) \Leftrightarrow qz < 1/(\beta(k + 1)).$$

Moreover, since  $\beta < 1$ , we have

$$\frac{2}{(k + 1)(\beta + 1)} < \frac{1}{\beta(k + 1)} < \frac{2}{\beta(k + 1)}.$$

Therefore, when  $\bar{v}/z \leq p < \bar{v}/(\beta z)$ ,

- if  $qz \geq 1/(\beta(k+1))$ , the payer offers coverage for any price;
- if  $2/((k+1)(\beta+1)) < qz < 1/(\beta(k+1))$ , the payer offers coverage iff  $p \leq \hat{p}_1$ ;
- if  $qz \leq 2/((k+1)(\beta+1))$ , the payer does not offer coverage regardless of the price.

If  $p < \bar{v}/z$ , the difference in payer objective between offering and not offering coverage is

$$\Delta^U = W_{\text{payer}}^U|_{\bar{\beta}=\beta} - W_{\text{payer}}^U|_{\bar{\beta}=1} = \frac{n}{\bar{v}}(1-\beta)zp^2 \left[ (1+\beta)(1+k)\frac{zq}{2} - 1 \right].$$

The payer offers coverage iff  $\Delta^U \geq 0$ , that is, iff  $qz \geq 2/((k+1)(\beta+1))$ .  $\square$

#### Proof of Theorem 4

The condition  $(\bar{v}/z)(1 - 1/(2\beta)) < c/2$  ensures that  $p^* > \bar{v}/z$ . The proof is similar to the proof of Theorem 1. Suppose  $c > \bar{v}/z$ . The relevant price domain is  $[c, \bar{v}/(\beta z)]$ . Without payer coverage, there is no demand for the drug, and the firm does not achieve any profit. With coverage, the firm profit is concave and reaches its maximum at  $p^*$ . Hence, from Proposition 7, if  $k+1 \geq 1/(qz\beta)$ , the firm prices at  $p^*$ ; if  $k+1 < 2/(qz(\beta+1))$ , the firm is indifferent to the price decision; and otherwise, the firm prices at  $p^*$  when  $p^* \leq \hat{p}_1$ , at  $\hat{p}_1$  when  $p^* > \hat{p}_1 \geq c$ , and is indifferent to the price decision when  $\hat{p}_1 < c$ . After simplifications, we find that  $\hat{p}_1 < c$  iff  $k+1 < B$  and  $p^* > \hat{p}_1$  iff  $k+1 < A$ .

Now suppose  $c \leq \bar{v}/z$ . With coverage, the firm profit is concave and reaches its maximum at  $p^*$ ; without coverage the firm profit is concave and reaches its maximum at  $\bar{p}^*$ . Using the reasoning detailed in the proof of Theorem 1, at a given price, coverage leads to higher firm profit than no coverage. Hence, from Proposition 7, if  $k+1 \geq 1/(qz\beta)$ , the firm prices at  $p^*$ ; if  $k+1 < 2/(qz(\beta+1)) = B$ , the firm prices at  $\bar{p}^*$ ; and otherwise, the firm prices at  $p^*$  when  $p^* \leq \hat{p}_1$ , at  $\hat{p}_1$  when  $p^* > \hat{p}_1$ .  $\square$

#### Proof of Proposition 8

If  $p \geq \bar{v}/\beta$ , the payer is indifferent between covering and not covering the drug as  $W_{\text{payer}}^O|_{\bar{\beta}=\beta} = W_{\text{payer}}^O|_{\bar{\beta}=1} = 0$ .

If  $\bar{v} \leq p < \bar{v}/\beta$ , the payer offers coverage iff  $W_{\text{payer}}^O|_{\bar{\beta}=\beta} \geq 0$ , that is, iff  $(k+1)(\bar{v} + \beta pz)/2 - p \geq 0$ . Hence, when  $(k+1)\beta/2 - 1 \geq 0$ , the payer offers coverage for any price, but otherwise it only offers coverage when  $p \leq \hat{p}_2$ . Observe that

$$\hat{p}_2 \geq \bar{v} \Leftrightarrow k+1 \geq 2/(\beta+1) \quad \hat{p}_2 < \bar{v}/\beta \Leftrightarrow k+1 < 1/\beta.$$

Moreover, since  $\beta < 1$ , we have  $2/(\beta+1) < 1/\beta < 2/\beta$ . Therefore, when  $\bar{v} \leq p < \bar{v}/\beta$ ,

- if  $k+1 \geq 1/\beta$ , the payer offers coverage for any price;
- if  $2/(\beta+1) \leq k+1 < 1/\beta$ , the payer offers coverage iff  $p \leq \hat{p}_2$ ;
- if  $k+1 < 2/(\beta+1)$ , the payer does not offer coverage regardless of the price.

If  $p < \bar{v}/z$ , the difference in payer objective between offering and not offering coverage is

$$\Delta^O = W_{\text{payer}}^O|_{\bar{\beta}=\beta} - W_{\text{payer}}^O|_{\bar{\beta}=1} = \frac{nq}{\bar{v}}(1-\beta)p^2 \left[ \frac{(1+\beta)(1+k)}{2} - 1 \right].$$

The payer offers coverage iff  $\Delta^O \geq 0$ , that is, iff  $k+1 \geq 2/(\beta+1)$ .  $\square$

**Proof of Theorem 5**

The condition  $\bar{v}(1 - 1/(2\beta)) < c/(2q)$  ensures that  $\hat{p}^* > \bar{v}$ . The proof is similar to the proof of Theorem 4 and is omitted for brevity.  $\square$

**Proof of Proposition 9**

1. Payer payoff comparison: we have

$$\begin{aligned}
\Pi_{\text{payer}}^O - \Pi_{\text{payer}}^U &= \frac{n}{\bar{v}} \left[ (\bar{v} - \beta p^O) q \left( \frac{k}{2} (\bar{v} + \beta p^O) - (1 - \beta) p^O \right) - (\bar{v} - \beta p^U z) \left( q \frac{k}{2} (\bar{v} + \beta p^U z) - (1 - \beta) p^U \right) \right] \\
&= N^O q \left( \frac{k}{2} (\bar{v} + \beta p^O) - (1 - \beta) p^O \right) - N^U \left( q \frac{k}{2} (\bar{v} + \beta p^U z) - (1 - \beta) p^U \right) \\
&= q \frac{k}{2} \bar{v} (N^O - N^U) + \left( \frac{k\beta}{2} - (1 - \beta) \right) q N^O p^O - \left( \frac{qk\beta z}{2} - (1 - \beta) \right) N^U p^U \\
&= \frac{kn\beta c}{4} (qz - 1) + q \frac{n}{4\beta\bar{v}} \left( \frac{k\beta}{2} - (1 - \beta) \right) \left( \bar{v}^2 - \frac{c^2\beta^2}{q^2} \right) - \frac{n}{4\bar{v}\beta z} \left( \frac{qk\beta z}{2} - (1 - \beta) \right) (\bar{v}^2 - c^2\beta^2 z^2) \\
&= \frac{kn\beta c}{4} (qz - 1) - (1 - \beta) \frac{n\bar{v}}{4\beta z} (qz - 1) + \frac{nk c^2 \beta^2}{8\bar{v}q} (q^2 z^2 - 1) - (1 - \beta) \frac{n\beta c^2}{4\bar{v}q} (qz - 1) \\
&= \frac{n}{4} (qz - 1) \left[ k\beta c - (1 - \beta) \left( \frac{\bar{v}}{\beta z} + \frac{\beta c^2}{\bar{v}q} \right) + (qz + 1) \frac{kc^2\beta^2}{2\bar{v}q} \right] \\
&= \frac{n}{4} (qz - 1) \left[ k\beta c \left( 1 + \frac{\beta c}{2\bar{v}q} (qz + 1) \right) - (1 - \beta) \frac{\beta c^2}{\bar{v}q} \left( 1 + \frac{\bar{v}^2 q}{\beta^2 c^2 z} \right) \right].
\end{aligned}$$

2. Payer objective comparison: we have

$$\begin{aligned}
W_{\text{payer}}^O - W_{\text{payer}}^U &= \Pi_{\text{payer}}^O - \Pi_{\text{payer}}^U + \Pi_{\text{patient}}^O - \Pi_{\text{patient}}^U \\
&= \frac{n}{4} (qz - 1) \left[ k\beta c \left( 1 + \frac{\beta c}{2\bar{v}q} (qz + 1) \right) - (1 - \beta) \left( \frac{\bar{v}}{\beta z} + \frac{\beta c^2}{\bar{v}q} \right) + \beta c \left( 1 - \frac{\bar{v}}{z\beta c} + \frac{\beta c}{2q\bar{v}} (qz - 1) \right) \right] \\
&= \frac{n}{4} (qz - 1) \left[ (k + 1)\beta c \left( 1 + \frac{\beta c}{2\bar{v}q} (qz + 1) \right) - \frac{\beta c^2}{\bar{v}q} \left( 1 + \frac{\bar{v}^2 q}{\beta^2 c^2 z} \right) \right].
\end{aligned}$$

$\square$

**Proof of Proposition 10**

We have

$$\begin{aligned}
&\Pi_{\text{firm}}^O(p^O; \beta) + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U(p^U; \beta) + W_{\text{payer}}^U) \\
&= \frac{n}{4\bar{v}\beta} (qz - 1) \left( \frac{\bar{v}^2}{z} - \frac{c^2\beta^2}{q} \right) + \frac{n}{4} (qz - 1) \left[ (k + 1)\beta c \left( 1 + \frac{\beta c}{2\bar{v}q} (qz + 1) \right) - \frac{\beta c^2}{\bar{v}q} \left( 1 + \frac{\bar{v}^2 q}{\beta^2 c^2 z} \right) \right] \\
&= \frac{n\beta c}{4\bar{v}} (qz - 1) \left( \bar{v}(k + 1) \left( 1 + \frac{\beta c}{2\bar{v}q} (qz + 1) \right) - \frac{2c}{q} \right).
\end{aligned}$$

First, consider the case  $zq > 1$  (i.e.,  $q < q_0$ ). By Assumption 3,

$$k + 1 \geq A' = \frac{2}{\beta} \frac{\bar{v}q + \beta c}{3\bar{v}q + \beta c}.$$

It follows that

$$\Pi_{\text{firm}}^O(p^O; \beta) + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U(p^U; \beta) + W_{\text{payer}}^U) \geq \frac{n\beta c}{4\bar{v}} (qz - 1) \left( \frac{2\bar{v}}{\beta} \frac{\bar{v}q + \beta c}{3\bar{v}q + \beta c} \left( 1 + \frac{\beta c}{2\bar{v}q} (qz + 1) \right) - \frac{2c}{q} \right)$$

$$\begin{aligned}
 &> \frac{n\beta c}{4\bar{v}}(qz-1) \left( \frac{2\bar{v}}{\beta} \frac{\bar{v}q + \beta c}{3\bar{v}q + \beta c} \left( 1 + \frac{\beta c}{\bar{v}q} \right) - \frac{2c}{q} \right) \\
 &= \frac{n\beta c}{4\bar{v}}(qz-1) \left( \frac{2}{\beta q} \frac{(\bar{v}q + \beta c)^2}{3\bar{v}q + \beta c} - \frac{2c}{q} \right) \\
 &= \frac{n\beta c}{4\bar{v}}(qz-1) \left( \frac{2c}{q} \frac{(x+1)^2}{3x+1} - \frac{2c}{q} \right).
 \end{aligned}$$

where the last inequality is due to  $z > 1/q$  and where  $x \equiv \bar{v}q/(\beta c)$ . By Assumption 1,  $\bar{v} > \beta c/q$  (i.e.,  $x > 1$ ). Moreover, we obtain

$$\begin{aligned}
 \frac{\partial}{\partial x} \frac{(x+1)^2}{3x+1} &= \frac{(x+1)(3x-1)}{(3x+1)^2} > 0 \quad \text{when } x > 1; \\
 \left. \frac{(x+1)^2}{3x+1} \right|_{x=1} &= 1.
 \end{aligned}$$

Hence, we have

$$\Pi_{\text{firm}}^O(p^O; \beta) + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U(p^U; \beta) + W_{\text{payer}}^U) > \frac{n\beta c}{4\bar{v}}(qz-1) \left( \frac{2c}{q} - \frac{2c}{q} \right) = 0.$$

Next, consider the case when  $zq < 1$  (i.e.,  $q > q_0$ ). By Assumption 3,

$$k+1 \geq A = \frac{2}{\beta q z} \frac{\bar{v} + c\beta z}{3\bar{v} + c\beta z}.$$

It follows that

$$\begin{aligned}
 \bar{v}(k+1) \left( 1 + \frac{\beta c}{2\bar{v}q}(qz+1) \right) - \frac{2c}{q} &\geq \frac{2\bar{v}}{\beta q z} \frac{\bar{v} + c\beta z}{3\bar{v} + c\beta z} \left( 1 + \frac{\beta c}{2\bar{v}q}(qz+1) \right) - \frac{2c}{q} \\
 &= \frac{2\bar{v}}{\beta q z} \frac{\bar{v} + c\beta z}{3\bar{v} + c\beta z} \left( 1 + \frac{\beta cz}{2\bar{v}} + \frac{\beta c}{2\bar{v}q} \right) - \frac{2c}{q} \\
 &> \frac{2\bar{v}}{\beta q z} \frac{\bar{v} + c\beta z}{3\bar{v} + c\beta z} \left( 1 + \frac{\beta cz}{\bar{v}} \right) - \frac{2c}{q} \\
 &= \frac{2}{\beta q z} \frac{(\bar{v} + c\beta z)^2}{3\bar{v} + c\beta z} - \frac{2c}{q} \\
 &= \frac{2c}{q} \frac{(y+1)^2}{3y+1} - \frac{2c}{q}
 \end{aligned}$$

where the last inequality is due to  $1/q > z$  and where  $y \equiv \bar{v}/(\beta cz)$ . By Assumption 1,  $\bar{v} > \beta cz$  (i.e.,  $y > 1$ ). Hence,

$$\bar{v}(k+1) \left( 1 + \frac{\beta c}{2\bar{v}q}(qz+1) \right) - \frac{2c}{q} > \frac{2c}{q} - \frac{2c}{q} = 0.$$

Therefore,  $\Pi_{\text{firm}}^O(p^O; \beta) + W_{\text{payer}}^O - (\Pi_{\text{firm}}^U(p^U; \beta) + W_{\text{payer}}^U)$  has the same sign as  $qz-1$ , that is, it is negative.  $\square$