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## Affordability Challenges to Value-Based Pricing: Mass Diseases, Orphan Diseases, and Cures

Patricia M. Danzon, PhD\*

The Wharton School, University of Pennsylvania, Philadelphia, PA, USA

### ABSTRACT

**Objectives:** To analyze how value-based pricing (VBP), which grounds the price paid for pharmaceuticals in their value, can manage “affordability” challenges, defined as drugs that meet cost-effectiveness thresholds but are “unaffordable” within the short-run budget. **Methods:** Three specific contexts are examined, drawing on recent experience. First, an effective new treatment for a chronic, progressive disease, such as hepatitis C, creates a budget spike that is transitory because initial prevalence is high, relative to current incidence. Second, “cures” that potentially provide lifetime benefits may claim abnormally high VBP prices, with high immediate budget impact potentially/partially offset by deferred cost savings. Third, although orphan drugs in principle target rare diseases, in aggregate they pose affordability concerns because of the growing number of orphan indications and increasingly high prices. **Results:** For mass diseases, the transitory budget impact of treating the accumulated patient stock can be managed by stratified rollout that delays

treatment of stable patients and prioritizes patients at high risk of deterioration. Delay spreads the budget impact and permits potential savings from launch of competing treatments. For cures, installment payments contingent on outcomes could align payment flows and appropriately shift risk to producers. This approach, however, entails high administrative and incentive costs, especially if applied across multiple payers in the United States. For orphan drugs, the available evidence on research and development trends and returns argues against the need for a higher VBP threshold to incentivize research and development in orphan drugs, given existing statutory benefits under orphan drug legislation.

**Keywords:** affordability, cures, orphan drugs, pharmaceuticals, value-based pricing.

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### Introduction

Value-based pricing (VBP) seeks to ground the prices paid and coverage decisions for pharmaceuticals on their value, as measured primarily by health gain to the patient (incremental efficacy and safety) plus any net savings in medical costs. (Other societal costs and benefits, such as equity and prevention of contagion, could be included if the perspective is societal.) Drugs that are priced to be cost-effective at a specified threshold (cost per quality-adjusted life-year [QALY]) should be reimbursed. In a welfarist context, the threshold and health budget reflect consumers’ willingness to pay (WTP) for health-related versus non-health-related goods, given incomes, preferences, and technologies. In equilibrium, VBP can be designed to achieve the maximum health gain for a given budget (static efficiency) and create second best optimal dynamic incentives for research and development (R&D) investment in a global context [1].

In practice, changes in technology imply that application of VBP may sometimes conflict with affordability, at least in the short run. A new drug (class) is deemed “unaffordable” if paying for all eligible patients at the VBP price would force either an overrun of the

payer’s budget or displacement of other cost-effective treatments. In the long run, changes in health technologies can in principle be accommodated by increasing health budgets if consumers are willing to allocate more resources to health care. Thus, affordability is mainly a problem of disequilibrium. This article examines three prototypical affordability contexts and the possible approaches to deal with each. Because affordability of a treatment depends on price and disease prevalence, these three contexts correspond to different situations of high price and/or high prevalence.

First, a high-price/high-prevalence threat to affordability is likely to occur with new, highly effective treatments for chronic, progressive diseases, such as hepatitis C. For such diseases, the initial disease prevalence exceeds the annual incidence of new cases and the current treatment yields medical cost savings that accrue mainly in the future. The transitory budget impact of treating the initial patient stock can thus far exceed the steady-state annual cost of treating new cases. Stratification of treatment is potentially an effective approach to dealing with unaffordability in such high-prevalence, progressive disease contexts.

Second, affordability is a potential concern for “cures” such as gene therapies that might claim extremely high VBP prices on the

\* Address correspondence to: Patricia M. Danzon, The Wharton School, University of Pennsylvania, 3641 Locust Walk, Philadelphia, PA 19104.

E-mail: [danzon@wharton.upenn.edu](mailto:danzon@wharton.upenn.edu)

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basis of their potentially long-lived benefits (The emphasis on “cure” is to highlight the uncertainty as to actual long-term effects of such treatments.) Installment payments are evaluated as a solution. Contingent payments appropriately shift risk to producers and align payment with accrual of benefits, but also create insurance agency and transaction costs.

Third, orphan drugs raise concerns about longer term affordability because their increasingly high prices and growing numbers imply a growth in expenditures at more than twice the rate for nonorphan drugs [2]. This prospect, that current growth rates of orphan drug prices and volumes will require either abnormal budget increases or cuts in other programs, raises the issue addressed here, of whether orphan drugs should continue to be exempt from standard VBP thresholds, given the evidence that rare diseases are no longer neglected but now account for 30 to 40 of new medicines approved each year [3].

In this article, the first section summarizes the basic VBP framework. The second section examines the high-prevalence/high-price context of mass, progressive diseases and considers how the role of stratification depends on disease and drug characteristics. The third section discusses high-priced cures and evaluates proposals for installment payments. The fourth section discusses orphan drugs, with concluding remarks in the last section.

## Theoretical VBP Framework

VBP is grounded in a welfarist framework in which either consumers choose among competing private health plans that offer different coverage/premium choices or taxpayers choose an annual health budget through a political process. In either case, the private/public payer has a budget that is fixed in the short run and reflects consumers/taxpayers' expected marginal utility of spending on health care versus other consumption, given incomes and preferences. The payer—public or private—maximizes the expected health gain for enrollees by setting a threshold or marginal WTP for health (e.g., \$100,000/QALY) and reimburses for drugs/indications that meet this cost-effectiveness threshold. In a market context, insurers could offer a menu of plans, with higher WTP threshold plans offering greater technology coverage and higher premiums. A consumer's choice of plan thus implies a choice of premium (budget), threshold, and coverage generosity for the year. For public plans, a similar process operates: choice of the health budget implies a threshold, given the technology set. This approach is potentially consistent with efficient use and investment in pharmaceuticals (static and dynamic efficiency), if adopted unilaterally by payers in each country [1].

This approach implies a maximum VBP price that a manufacturer can charge for a new drug and still meet the cost-effectiveness threshold, which depends on the value created (subscripts “n” and “0” denote the new and comparator treatments, respectively).

$$VBP_n^{\max} = P_0 + [(C_0 - C_n)^1 + K(Q_n - Q_0)^1] + \sum^T [(C_0 - C_n)^t + K(Q_n - Q_0)^t]. \quad (1)$$

The maximum VBP price of the new drug is the sum of 1) the price of the comparator  $P_0$ , 2) a premium that reflects cost offsets plus incremental health gain in the current period  $[(C_0 - C_n)^1 + K(Q_n - Q_0)^1]$ , and 3) expected future cost offsets and health gain over the patient's life, appropriately discounted  $\sum^T [(C_0 - C_n)^t + K(Q_n - Q_0)^t]$ . The VBP price would grant all the consumer surplus to the producer, which in theory provides optimal incentives for investment in R&D at the margin. High expected returns may encourage multiple competitors of slightly differentiated products. Whether price competition then transfers

some surplus to consumers depends on payer bargaining strategies and consumer price sensitivity.

In this welfarist approach, in equilibrium the payer's health care budget (B), the WTP threshold (K), and services reimbursed are simultaneously determined, given available technologies, consumer incomes, preferences, and other factors. Over time, changes in incomes, technologies, or other factors could lead to revision of the budget, the threshold, and services reimbursed. In the long run, new cost-effective medical technologies can be accommodated by displacement of inferior technologies and by growth of the health budget, but such adjustments take time. Unanticipated expansion of the technology set can upset the budget balance for a given threshold, leading to short-run affordability problems.

## Affordability and High-Prevalence, Progressive Diseases

Equation 1 implies that a VBP-priced new drug is easily “affordable” within the existing budget if it creates value solely by reducing current medical costs, with no change in future costs or current or future QALYs. In this case, the VBP premium for the new drug is accommodated by current cost offsets, resulting in budget neutrality for the payer.

In contrast, a new technology potentially increases current year expenditures when its value derives primarily from reducing future medical costs and/or providing QALY gains, current or future, because future cost savings and all QALY gains justify VBP premiums that add to current year expenditures without any current savings. The larger the potential treatment population, the more likely such technologies appear unaffordable.

This concurrence of large treatment population with high VBP price reflecting future cost savings and QALY gains is most likely to occur for highly effective new treatments of progressive diseases that entail rising medical costs and deteriorating quality and duration of life as patients age, such as hepatitis C. A treatment that stops disease progression offers large future medical savings and future QALY benefits per patient, and hence justifies a high VBP price. Moreover, slow disease progression leads to an accumulated initial prevalence of patients potentially eligible for treatment that far exceeds the annual incidence of new cases. For payers, the short-run budget impact of treating the initial patient stock far exceeds the steady-state annual cost of treating new cases, because of the diminishing number of eligible patients once the initial stock has been treated and because realization of deferred savings offsets new outlays. This “unaffordable” short-run budget impact of treating the accumulated patient stock is most severe if the new drug requires only a short treatment, as for hepatitis C. A maintenance drug that is effective at preventing further progression of a chronic disease could not justify such a high VBP price per unit. Essentially, maintenance treatment spreads the cost over many years, whereas a cure that requires a single, highly effective course of treatment concentrates the cost in the price of that short treatment, and hence is more likely to pose a transitory affordability challenge, as for hepatitis C.

## Long-Run (Budget) Adjustment versus Short-Run (Stratification) Adjustment

Expansion in the set of available medical technologies may optimally require different adjustments in the long run versus the short run. In the long run, consumers may choose to increase the health budget relative to non-health-related consumption and possibly also raise or lower the WTP threshold K if the marginal utility of health care relative to non-health-related goods changes.

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