Affordability, or rather a lack of it, has become a new buzzword in health policy. It is raised by payers as a “heat shield” as they seek to protect themselves from the impact of expensive new drugs and medical technologies. Manufacturers, in contrast, argue that if something is a good value, compared with currently available treatments, then money should be found to pay for it. Mainstream economists see it as “Economics 101” that affordability cannot be separated from the willingness to pay for and/or the opportunity cost of providing a new intervention. Whether we can “afford” something depends not only on the cost, but on our income, wealth, borrowing capacity, and the alternative goods and services we want to spend our money on today and over time. If we really want something, we will try to find a way to make it “affordable” by adjusting spending priorities.

Discussion about the affordability of a new health intervention usually means that there is a substantial associated budget impact on a health system, requiring either additional funding or forfeiture of other health care services that are currently considered “good value for money.” We present three articles and two commentaries in this themed section that examine the issue of affordability from different perspectives. The three articles [1–3] suggest alternative methods to deal with affordability. One of the two commentaries [4] concludes that payers, although concerned about affordability, are not interested in alternative payment schemes for high-value interventions. They would rather rely on price negotiations, high copayments (where permissible), and restricting access to patient subgroups. Their concern is that increasing the financial capacity of the health system to accommodate new treatments will lead to higher prices and more long-term financial pressures, rather than to more patients accessing innovative treatments. Payers believe that it is better to find ways to manage within current budgets. The second commentary [5] agrees that increasing health system debt is to be avoided. Pressure needs to be applied to prices and efficiency because 1) payers have great difficulty negotiating lower prices; 2) there seems to be a presumption that even if prices reflect value at the margin, prices for products of higher value do not decrease over time, unlike for other products or services such as electronics; and 3) health system inefficiency needs to be addressed to release resources to fund new interventions.

We return to these points, but note that “affordability” has another key element. Danzon points out that sofosbuvir (Sovaldi®, Gilead Sciences Inc., Foster City, CA) as a treatment for the hepatitis C virus (HCV) was cost effective by most payers’ standards but considered unaffordable by many because of the large number of patients “warehoused” (i.e. diagnosed and waiting for treatment or carrying the HCV but undiagnosed) [3]. Savings from treatment are realized mainly in the future. Pearson puts it bluntly, “Apparently Sovaldi was cost effective, but unaffordable” [2]. A treatment can be cost effective and unaffordable because the conventional use of cost-effectiveness analysis implicitly assumes displacement of other health care services when a new service is introduced and compared with a marginal cost-effectiveness threshold (CET), the value of which is assumed to equal society’s willingness to pay for an additional unit of health [1].

Some new treatments, however, have the potential to have a budget impact that is nonmarginal. Full adoption requires an alteration in the CET if the threshold is interpreted as representing the opportunity cost (in terms of health lost) of introducing the new treatment [3] and if budgets are fixed. Some economists [6] have long argued that CETs ignore the “lumpiness” of investments and assume frictionless displacement or interchangeability, or fungibility, between different interventions or uses of money. Pekarsky notes that the displaced treatment might not be the least cost-effective treatment [7]. She argues that the assumptions by Weinstein et al [8] that enable them to conclude that the shadow price of health is equal to the least cost-effective intervention currently used and that if the cost effectiveness of the new treatment is less than this threshold, it should be adopted, may not hold. However, for most technology adoption decisions, we can behave as if the Weinstein and Zeckhauser (1973) assumptions are correct. We are at the margin. Budget impact is relevant only for planning purposes, for example, to decide 1) what will be postponed or discontinued to make room in the budget, or 2) how to obtain additional funding for the new treatment.

Three Views on Managing Affordability and Cost Effectiveness

The three articles in this section all propose pragmatic approaches to managing tension between affordability and cost effectiveness but from different perspectives. Danzon details how payers dealt with the HCV drugs challenge. Even though some payers obtained large discounts in exchange for accelerating access and pursuing HCV eradication, most payers pushed hard for discounts but waited for competitive treatments to be launched before extending access to all HCV patients. Priority was given to patients facing the highest risk for decompensated liver disease or liver transplantation. Those in earlier stages of disease waited for treatment. Slow disease progression in HCV made this feasible. We note that it may not always be possible to delay treatment on the expectation that no patient harm will result (e.g., a drug for Alzheimer’s disease that
needed to be used early to prevent irreversible brain damage and progression to dementia). Danzon points out that in addition to managing entry of a new cost-effective treatment into the health system, some way has to be found to absorb any additional cost for the new intervention by doing one or more of the following: 1) displacing another activity, 2) improving efficiency, 3) reducing/increasing a budget surplus/loss, or 4) raising premiums or taxes. It seems to us that she is suggesting a short-run impact requiring phasing in of new treatments and a longer-term need to align budgets and the treatments provided based on underlying citizen willingness to pay for health gain.

Danzon also considers orphan drugs, which typically have high prices per unit of health gain. Small patient numbers do not necessarily mean a manageable budget impact. US payers note that an estimated 10% of the US population has rare disease conditions. They contend that the cumulative effect of high prices for rare diseases could bankrupt the US health care system. The proposed price for the first Food and Drug Administration–approved gene therapy is $850,000 [10]. Danzon argues, convincingly, that most orphan drugs do not need to be priced at a level that results in incremental cost-effectiveness ratios greater than accepted US CETs. Evidence indicates they have lower research and development (R&D) costs, compensating for smaller patient numbers and multiple indications. Danzon recognizes that a greater CET may be needed for “a subset of orphan indications that incur disproportionate R&D cost per patient and limited opportunity for indication expansion” [1].

Lomas et al argue that affordability should and can be dealt with by adjusting the CET using a nonmarginal budget impact formula based on an empirical econometric calculation of opportunity cost and of the health care system's production function [3]. Such an approach faces two main challenges. First, estimating CETs and health production functions to estimate adjustments for nonmarginal budget impacts requires good data and assumptions. Lomas et al describe a method that requires the use of many assumptions and draws on a threshold estimation exercise, which in turn is constrained by the availability of health system data, choice of method, and the assumptions made. Nevertheless, as they note, “despite the limited number of observations available...the analysis demonstrates that an empirical examination of this relationship is possible” [3].

Second, there are timing issues. The cross-sectional approach used to estimate output responses may not reflect the ease (or lack of it) with which payers can switch resources from providing one treatment or service to another. Disinvestment takes time and is difficult to do unless you are substituting one treatment for another in the same population [11]. The authors note that their approach shows a clear incentive for manufacturers to look for ways of working with payers to “smooth” the budget impact to minimize displacement effects. Thus, we can see the potential for negotiation between manufacturers when budget impact is nonmarginal (as suggested by Lomas et al) and payers are looking to phase in uptake over time (as per Danzon). In addition, analysis may identify different cost effectiveness for different subgroups that could ease negotiation.

Pearson is very clear that global budget impact is a relevant criterion for the US health care system in pricing and use decisions [2]. He realized this when Sovaldi was “voted ‘low value’” by his advisory committee members because the budget impact was “so significant that it over-rode what was acknowledged to be excellent cost effectiveness.” He details the journey that the Institute for Clinical Economic Review (ICER) took in moving away from the use of a single global budget formula to adjust the price of a new treatment to reflect budget impact over and above the assessment of cost effectiveness. ICER revised the framework to report budget impact at different CET thresholds and rates of uptake to provide a basis for the payers and manufacturers to negotiate price and use. The move occurred for two reasons. First, the global budget impact estimates in ICER’s initial framework were inevitably arbitrary and hence much criticized. Attempts to assess “payer ability to pay” failed, leading him to believe “that basing a budget impact threshold on private payer’s self-reported ability to pay would be unlikely to receive an adequate level of trust in the United States” [2]. Second, estimating uptake based on clinical need was controversial and hard to make objective; therefore, ICER moved to an indicative presentation of budget impact based on different uptake rates. This recognizes that the adjustment consequences of budget impacts will be system specific, and different US payers might have different CETs, but still provides an “affordability and access alert” in addition to cost-effectiveness estimates.

One issue that arises is that if affordability is considered alongside cost effectiveness, as proposed by Pearson [3], it might be more attractive for manufacturers to invest in chronic treatments (in which payment is spread over many years) rather than “cures” (in which payment is typically “upfront”). One way around this is linking payment to outcomes in outcome-based contracts. Danzon rightly points out that transaction costs can be high, and in the US system with patient churn and a default to Medicare at the age of 65, the correct incentives may not be present. Karlsberg Schaffer et al note a proposal by Montazerhodjat et al [12] for the equivalent of the Affordable Care Act requirement for insurers not to discriminate against pre-existing conditions to be extended to require taking on a contractual payment profile for a “cure.” However, the payment profile would need to reflect the timing of benefits and costs if the churn and Medicare default issues identified by Danzon were to be addressed.

Thus, to summarize, the three articles provide different approaches to dealing with affordability, all of which include cost-effectiveness analysis. Danzon argues that cost effectiveness and budgets are linked but that over time the budget can adjust if people want it to. The key driver is the underlying willingness to pay for health gains over the patient’s lifetime. In contrast, Lomas et al argue that the appropriate way to deal with affordability issues is to lower the CET so that it represents more accurately the opportunity costs of the new intervention. With an adjusted CET, the budget impact of a new treatment should be affordable if all current treatments are required to meet that threshold. However, both approaches require assumptions to estimate either an appropriate value for willingness to pay or the CET. The alternative proposed by Pearson is to show how cost effectiveness and budget impact might vary at different prices and different rates of uptake. Payers can then use this information for their financial planning and for negotiations with manufacturers.

### Underlying Concerns

It is clear to us that, in addition to understanding how health systems using health technology assessment and value-for-money thresholds tackle treatments that are cost effective but not affordable, there are three fundamental concerns about the dynamics of health care that are also pertinent to the discussion.

The first is concern about the current dynamics of drug pricing. Danzon argues that value-based pricing produces optimal incentives for R&D. In the United States, however, health technology assessment (HTA)–informed value-based pricing is largely absent (ICER is one body seeking to introduce it). Launch prices often are hard to understand, and branded drug prices increase at rates greater than the consumer price index. In some countries, HTA-based pricing or reimbursement is effective. In others, manufacturers argue that they face monopolistic pricing pressure with little payer recognition of the need to reward
innovation. Of course, prices can go below the value-based price when there is competition and effective purchasing, which, as we have noted, was the case for HCV therapies. However, effective purchasing often is absent. Related to this is the extent to which post-patent expiry competition occurs and drives down prices for both conventional and biologic drugs in different countries [13]. This is a particular concern with biologic drugs and orphan conditions. Biosimilar price competition is starting to become more aggressive in Europe, in part as a result of payers tracking outcomes to reassure clinicians and patients, but has yet to emerge in the United States [14]. For orphan indications with small patient sizes, there may not be generic entrants to create a competitive market, and some form of post-patent expiry price regulation may be efficient. On the other hand, we have noted that intervention may be needed to ensure a “level playing field” of financial incentives for developing “cures” as compared to new treatments for chronic conditions.

The second fundamental concern is that health expenditure is growing at unsustainable rates. It is important to understand that, if people value health and if new technologies and longer life expectancy mean that they can buy health gain at a price they are willing to pay (albeit via public or private third-party payers), then the share of gross domestic product spent on health care is not a problem. Baumol wrote extensively that differential productivity and relative price effects would push up health care spending and yet, he argued, health care would still be affordable [15]. The problem is that most observers do not think that health systems are efficient and are concerned that high prices and low-value treatments, together with a failure to use information technology and electronic health records to improve efficiency, are driving up expenditures rather than citizen preferences for health. In reality, the second concern is a combination of our first concern about pricing dynamics, particularly (but not only) for drugs, and the third fundamental concern we identified in the commentaries—that health systems need to improve their efficiency and therefore generate more “headroom” to spend on good-value new treatments. Of course, many efficiency improvements are not cost reducing, but this might explain the resistance of payers to increasing the financial capacity of the system to make “cures” affordable [4,5]. They may also want to put on pressure to release resources within the system.

Conclusions

We conclude that:

Affordability matters. Cost-effectiveness analyses need to indicate whether there is any reason for expecting the budget impact of the interventions under review to be nonmarginal. Pragmatic payer approaches are needed, and the most relevant approach will depend on the health care setting.

More research is needed to understand what happens in practice when nonmarginal budget impact occurs and to estimate 1) the short-term opportunity cost of displacement from new interventions and 2) the underlying preferences of citizens for all health and health-related gains compared with their preferences for consuming other public and private goods and services.

Payer reluctance to adopt value-for-money treatments with high budget impact is understandable but risks distorting resource allocation. We need to avoid creating a bias in pricing, reimbursement, and HTA systems that make it easier to get a return on investment from introducing new chronic treatments rather than “cures.”

Overall efficiency in health care needs to be improved. Concern about the sustainability of health expenditure cannot be separated from affordability. Efficiency of drug purchasing could be improved by 1) increasing HTA-informed value-based pricing, 2) using competition and more effective procurement to improve value-for-money from the purchase of on-patent and off-patent treatments, and 3) encouraging best practice in the purchase of biosimilars by combining use of tendering to increase discounts with data collection to reassure clinicians and patients that outcomes are similar.

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