Defining Value-Based Pricing of Drugs

To the Editor—In a Viewpoint, Ms Kaltenboeck and Dr Bach defined a value-based price as one that relies on estimates from cost-effectiveness analyses generated by the Institute for Clinical and Economic Review (ICER). We agree that tying price to value could help efficiently allocate resources and that cost-effectiveness analysis is an informative tool, but using the proposed narrow definition of value-based pricing may limit clinician and patient access to valuable therapies.

The authors cited an article by Bach and Pearson for the definition of value-based pricing; however, the National Institute for Health and Care Excellence in the United Kingdom has used value assessments to inform or negotiate prices since 2000, and cost-effectiveness analyses have been conducted for decades.

Cost-effectiveness analysis is an attractive framework, but many important attributes of therapy are not typically incorporated. Reducing health disparities, providing hope for patients, and increasing patients’ choices are characteristics not captured in cost-effectiveness analyses. Ethical objections to quality-adjusted life-years (QALYs) make sole reliance on them worrisome, and downstream consequences are not considered (eg, improving the health of the head of a household to allow them to remain working and provide for their family). A true value-based price would account for these factors.

The complexity of analyses, including inputs and assumptions required, results in uncertainty, such that relying on a single value-based price may lead at times to invalid outcomes (and identifying a range of value-based prices could lead to challenges in price setting). As an example of varying methods, the authors’ assessment of evolocumab (Repatha) relied on clinical trial data as opposed to evidence from clinical practice settings, which underestimates disease burden and deflates the value-based price. Furthermore, their analysis considered the list price (which they incorrectly described as net price), despite evidence showing that the price in the community is far lower when accounting for discounts and rebates.

The article failed to consider the potential unintended consequences of their definition of value-based pricing. Manufacturers could be incentivized to steer their development programs away from therapies that provide, for example, incremental gains in life for terminal cancer patients—fearful that a value-based price would be inadequate to support reinvestment in research and development or might even be lower than production costs.

There are unanswered questions related to how value should be measured. Defining value solely using a single cost-effectiveness analysis from a single entity without sufficiently accounting for uncertainty may deny patients lifesaving therapies.

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Letters

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Conflict of Interest Disclosures: The authors have completed and submitted the ICMJE Form for Disclosure of Potential Conflicts of Interest. Dr Broder and Mr Ortendahl report receiving research support and support for this letter from Amgen to the Partnership for Health Analytic Research (PHAR) LLC, an organization that conducts research into health care–related costs and value, and being employees of PHAR. PHAR has conducted studies for multiple pharmaceutical, biotech, and life sciences companies including over the past 3 years for Abbvie, Alcea, AstraZeneca, Bristol-Myers Squibb, Boston Scientific, C. R. Bard, Celgene, Eisai, Eli Lilly, Ethicon, Genentech, Genomic Health, Helsinn, Ionis, Ipsen, Janssen, Jazz, Kite, Novartis, Onconova, Otsuka, Pathnostics, Prothena, Roche, Sage, and Shinogi. 1. Kaltenboeck A, Bach PB. Value-based pricing for drugs: theme and variations. JAMA. 2018;319(21):2165-2166. doi:10.1001/jama.2018.4871

In Reply—Dr Broder and Mr Ortendahl critique our definition of value-based pricing as relying on cost-effectiveness analyses from ICER. That is not correct; we only mentioned the work of ICER as an example of a value-based price. They also suggest that we argued for the use of cost-effectiveness analyses and QALYs as the only measure of benefit. We did not say that, nor, to our knowledge, does ICER. Moreover, our work on tools such as the DrugAbacus explicitly acknowledges the multidimensional nature of value assessment, considering, for example, rarity of disease, unmet need, and prognosis.

What we did say was that the definition of a value-based price is one anchored to evidence of a drug’s benefits and harms, and that it is “transparent and replicable, open...
to public input, is set at market entry, and allows for price adjustments based on postapproval evidence. These characteristics specifically address the concerns articulated in the letter. For example, gathering public input in an open and transparent forum is critical for understanding aspects of a treatment that patients value but that are not fully captured by clinical evidence. It also allows patients to air their concerns about access and ability to benefit from the treatment, which can be hampered by high prices. It is precisely this transparency and public input that are needed to give manufacturers an explicit signal about which research and development and pricing strategies will sustain the high levels of innovation needed to guarantee continued access to lifesaving treatments.

We appreciate the suggestion to clarify the term “net price.”

We believe that our use of it—referring to the reduction in the price of evolocumab after rebates from an outcomes-based contract—is appropriate. Assuming this contract also included the 26.3% in concessions for cardiovascular drugs suggested by the authors, the net price would be $9596 per year, still 4 to 5 times the benchmark we provided—ICER’s estimate of $1725 to $2242. To call this a value-based price, several things would need to change. The price net of rebate would be known, rather than guessed at. It would be anchored to evidence of evolocumab’s value from a replicable analysis of its benefits and harms reflecting input from all stakeholders, including patients, rather than the manufacturer and payer alone. Also, it would have been established at the time the drug became available, and changed with new evidence of its efficacy.

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CORRECTION

Incomplete Information in Flow Diagram: In the Original Investigation entitled “Five-Year Follow-up of Antibiotic Therapy for Uncomplicated Acute Appendicitis in the APPAC Randomized Clinical Trial,” published in the September 25, 2018, issue of JAMA,1 the box in Figure 1 describing 5-year follow-up for patients who were randomized to receive antibiotic therapy should have shown that all of the 30 patients who discontinued intervention underwent appendectomy. This article was corrected online.


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