

Commentary: Steven D. Pearson**A US approach to value-based drug assessment**

The United States is the only industrialised nation without an organised national health technology assessment programme to guide coverage decisions or pricing for new drugs. In part this is because the federal government is prohibited from negotiating the price it pays for drugs in its insurance programmes for the elderly and the poor. It is also a reflection of a political culture that has long been hostile to considerations of cost-effectiveness in funding and insurance coverage decisions.

But this landscape is shifting rapidly. After several halcyon years of stability in overall drug spending, Americans faced newly resurgent double-digit price increases in 2014 that were partly associated with substantial spending on new drugs to treat hepatitis C. This introduction and other forces have awakened not only American insurers but also clinicians, policymakers, and the public to the problem of rising drug costs and to the underlying question of whether the US is receiving a reasonable value for the prices it pays for drugs.

There are now several efforts underway in the US to include assessments of 'value' as part of an evaluation of new drugs. The American Society of Clinical Oncology has recently published a draft value framework intended to inform individual discussions among patients and clinicians. Memorial-Sloan Kettering's Abacus project has created an online tool to explore different ways to weigh various components of value and compare the resulting price with actual market prices.

And the Institute for Clinical and Economic Review (ICER), a non-profit research organisation that I founded eight years ago, produces health technology assessment reports on drugs, devices and other medical services. ICER distributes all of its reports as public goods, uses conflict of interest provisions and convenes hearings at which independent committees of experts debate the merits of our reports and vote on the evidence.

We have received significant notice for our reports and meetings on the new drugs for hepatitis C infection, and have recently announced a grant that will allow us to grow so that reports can be produced for all of the most significant new medicines nearing approval by the US Food and Drug Administration.

One of the areas that I would like to highlight is our implementation of a new value assessment framework developed with significant stakeholder input. This framework is a way of reviewing the importance of new drugs by taking into consideration their clinical effectiveness, long-term cost-effectiveness and the potential benefits to patients or others that fall outside traditional clinical outcomes measurements. Our value framework has gained attention in part because it explicitly uses formal cost-effectiveness thresholds of \$100,000-\$150,000 per QALY. But the greatest controversy has arisen over its inclusion of potential budget impact analyses using list prices and five-year uptake assumptions as one component of 'provisional

health system value.' Combining long-term perspectives on value through cost-effectiveness analyses with short-term affordability considerations meets the needs of insurers and other policymakers but it has triggered concerns from manufacturers who believe that budget impact analyses have an inherent bias against innovative products for large populations.

Linked to the conceptual design of our value assessment framework is our approach to calculating 'value-based price benchmarks.' We use our work to answer a key question: what is a 'reasonable' price for a new drug? We calculate one price range related to the price at which a new drug would meet our cost-effectiveness thresholds. And then we apply our analysis of potential budget impact to determine whether the drug would add healthcare cost growth at a rate exceeding the anticipated rate of growth in the overall US economy. If the estimated potential budget impact falls above this threshold, we identify the price needed to remain within the threshold and identify this as an 'alarm bell' price beyond which extra measures to manage affordability may be needed. Details of the assumptions and calculations that go into our value-based price benchmarks are available on our website www.icer-review.org.

In reports using this approach we have found that some new drug prices do align well with our definition of value, while others do not. ICER's review of the Novartis drug Entresto for heart failure found that the company's list price in the US of \$4,560 was well within the range for long-term cost-effectiveness, requiring only a 9% discount to put it below the threshold for potential budget impact. In contrast, our analyses of the two new PCSK-9 inhibitor drugs for high cholesterol suggest that a reasonable value-based price range based on optimistic assumptions about the long-term clinical benefits would be 45%-62% lower than the \$14,350 average annual list price in the US, and because the drug is for a large possible patient population, a price reduction of 85% would be needed for it not to cause an unsustainable increase in healthcare budgets.

Major insurers have described their growing use of ICER reports in considerations of coverage, and more recently they have also started using ICER value-based price benchmarks in negotiations with drug makers. Manufacturers have stated their concerns with ICER's methodology but have also expressed delight when a specific drug is found to represent high value.

The growth of interest in the US in new approaches to drug pricing feels unlikely to subside. We hope that our approach will serve as an anchor for discussions in the future.

This commentary was written by Dr Steven D. Pearson, president, and Mitchell Stein, policy and communications director, at the Institute for Clinical and Economic Review in Boston, Massachusetts, US.