

Assessing the Value of Medical Devices—Choosing the Best Path Forward: Where Do We Go From Here?

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KEY POINTS . . .

Coverage decisions will always remain local, but the need for high-level evidence review to manage the total cost of care are only increasing for both payers and providers.

Medical device manufacturers increasingly need to demonstrate product value to an array of stakeholders, creating challenges including shifting financial risk from payers to providers, limited incentives to consider long-term outcomes, and industry's responsibility to serve all socioeconomic populations.

The application of value-based approaches to medical devices may not be as straightforward as it is for drugs, but a framework that expands decision criteria based on key outcome measures that demonstrate value may facilitate better treatment decisions.



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The health care market landscape change from traditional fee-for-service towards a more value-based reimbursement model represents a paradigm shift for the device industry. Value-based assessments are increasingly applied to health care technology adoption decisions; however, routine implementation presents several challenges. For example, while randomized controlled trials (RCTs) are both the “gold standard” for demonstrating technology effectiveness and the primary form of evidence that many decision makers consider in their coverage and reimbursement, these may not be applicable for many devices (e.g., difficult to randomize, impractical or unethical to blind subjects or investigators, no or inappropriate comparator, operator variability). Blinding in a cardiovascular device trial may require a sham procedure (e.g., implantable stimulation device to treat heart failure versus sham stimulation), but ethical principles applied to research and the role of the investigator to maximize individual benefits as well as the design of a true sham device to maintain blinding may pose a challenge.

The lack of robust clinical data makes cost-effectiveness evaluations very difficult to conduct. Therefore, an understanding of the level of evidence needed to inform payer decisions for reimbursement or procurement will require medical device companies to create an evidence development strategy (e.g., the use of real-world data alongside a traditional RCT) that substantiates the total value of their device to a wide array of stakeholders.

A Manufacturer's Perspective

The US health policy reform debate is incomplete without consideration of both the costs and benefits of medical devices. Generally, we recognize the health benefits conferred by medical technology. A recent US study estimated the net health system benefit and positive gross domestic product (GDP) impact of 11 common medical technologies in heart disease, orthopedics, and cancer, and forecasted the annual

financial benefit through 2035 to be \$23.6 billion (2010 \$US) [1]. Further, despite common misconceptions, spending on durable medical equipment—representing half of the device sector—remained constant in real terms, at about 2% of national health expenditures over the 1990-2014 period, with device inflation under 1% [2].

Each medical technology is unique. Based on price, medical need, health benefit, and target population, many devices will be subject to some form of net value assessment. Dimensions of value include clinical, economic, care quality, and contribution to population health management.

Device companies face myriad stakeholders seeking information on the relative benefit and cost, or “value,” of technology, including the care processes in which devices are used. These audiences range from patients, clinicians, specialty societies, and health care facilities to payers, government authorities, and health technology assessment (HTA) organizations. Incentives of such stakeholders frequently vary, if not outright conflict. Manufacturers are challenged to meet these information needs by several factors, including the continuing shift of financial risk from payers to providers, few incentives to consider long-term outcomes, industry's responsibility to serve all socioeconomic populations, and global price pressure, which can divert limited research resources.

While value assessment of drugs is a well-established discipline, principles for evaluating the cost-benefit tradeoffs associated with devices are at an early stage. Characteristics of medical devices hinder direct application of many pharmaceutical-focused value frameworks. Evaluating medical devices depends on understanding: (i) how devices can substantially alter entire care processes; (ii) operator variation; (iii) user learning curves; (iv) impact of often short product lifecycles; (v) pricing policy and disclosure; and (vi) the level of >

confidence around parameter estimates used in economic evaluations.

Device makers frequently—but should routinely—seek early input from stakeholders, including payers, on such issues as:

- Product development vis-à-vis a technology's potential role in care;
- Research goals, design, and relevant endpoints;
- Criteria for obtaining appropriate codes and optimal coverage and payment;
- Collaboration opportunities, particularly as value-based care proliferates;
- Mechanisms for value assessment updates, as innovation, care standards, and new data emerge; and
- Consideration of benefits over time frame consistent with patient benefits.

Not every technology confers net cost savings, hence the notion of cost-effectiveness and willingness-to-pay thresholds, i.e., a purchaser must consider its own, and perhaps society's, desire to invest in a medical device that requires an incremental expenditure but confers an additional clinical benefit. If stakeholders attempt to view one another's perspectives, incentives, and relevant time horizons, they may be able to move toward value assessment that fairly considers costs and benefits appropriate to improve patient outcomes and care quality for acceptable levels of financial investment.

A Regulatory Perspective

The Food and Drug Administration (FDA), whose requirements for approval of drugs and devices are based on safety and efficacy data, and the Centers for Medicare and Medicaid Services (CMS), whose requirements for coverage determinations are based on items and services that are considered reasonable and necessary, operate under different statutory and regulatory requirements in determining availability and influencing uptake, respectively, of new medical technologies. Prior to the establishment of the FDA and CMS Parallel Review program, the FDA's evidentiary standards used in regulatory decision making and CMS' evidentiary standards used in coverage decisions created two separate evidentiary submission hurdles for many device manufacturers. Under current practice, the FDA-CMS collaborative effort under the Parallel Review program provides an

opportunity for FDA and CMS to review submitted clinical data simultaneously to support timely access to new medical devices. This public-public partnership may benefit patients through access to safe and effective medical devices, incentivize innovation for the device industry, and expedite diffusion of innovative medical devices into the health system. The partnership between FDA and CMS highlights a shared common interest between the two agencies in minimizing regulatory hurdles that may commonly arise in premarket approval (or clearance) transactions.

Post Regulatory Approval Hurdle

A similar strategic partnership model (e.g., public-private, private-private) that brings together experts from academia, the device industry, payers, hospitals, regulators, and patient groups will be valuable for driving the diffusion of high-quality, high-value medical devices into the health care system. In order to achieve this much broader objective we must understand the potential impact of innovative medical technology as a driver of rising health care costs against finite budgets and whether investment results in better value in health care.

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Payers' scrutiny of spiraling health care costs and efforts to control spending has created an increasingly complex fourth hurdle (also known as the “post regulatory hurdle”) for medical devices. Inefficiencies and rising costs have been common themes across many major industrialized countries [3,4] and striking an acceptable balance between safety, efficacy, and cost has proven to be difficult. In the United States, health spending as a proportion of GDP has outpaced that of the Netherlands (11.1%), Switzerland (11.1%), and Sweden (11.0%) with 16.4% of GDP in 2013, which is well above the Organization for Economic Co-operation and Development (OECD) average of 8.9% [5]. Countries facing

these rising health care costs have moved towards cost containment strategies that involve technology assessments and hospital payments based on diagnosis-related groups and the value of products or services [3]. While the Medicare health insurance program in the United States does not formally rely on cost-effectiveness analysis in their decisions about coverage and reimbursement [6], other public payers in developed countries have found success in using value-based approaches to negotiate prices or set reimbursement levels both for devices and pharmaceuticals [3].

There is increasing support for incorporating cost-containment strategies and demonstration of value into medical device reimbursement or procurement decisions [7,8]. However, the application of value-based approaches to medical devices may not be as straightforward as it is for drugs. This is partly because of the differences that exist between drugs and devices [9], but a framework that expands decision criteria used in purchasing and the procurement of medical devices may facilitate better treatment decisions in terms of access, health outcomes, cost savings, and efficiency. For example, a broader criterion for assessing the value of left ventricular assisting devices (LVADs) in patients with end-stage heart failure may consider outcome measures such as safety, clinical effectiveness (e.g., impact on 1-year survival), functional status, quality of life, budget impact, cost-effectiveness (e.g., cost per QALY), and ease of use. Collecting data on outcomes measures that communicate clinical, patient, and economic product value will support device claims of value and create a stronger evidence-based value proposition. This approach will be strategically important for devices that are considered to have a substantial budgetary impact or have some level of clinical uncertainty.

The proper regulatory infrastructure and support from the FDA is required to determine what constitutes sufficient, valid scientific evidence prior to marketing. The FDA has published guidance documents that articulate the agency's current thinking regarding the acceptability of valid clinical data throughout the clinical development program and trial design challenges for medical devices [10-12]. However, to move towards an acceptable value-assessment framework for reimbursement or medical device procurement decisions

will require stakeholders to coalesce around best practices that not only allow for assessments that are transparent and relevant to the decision maker, but encourage high standards in the collection, design, conduct, and reporting of results. These practice standards should be useful in answering clinical questions as well as supporting reimbursement decisions that currently influence which devices are prioritized for purchase, the level of financing, and the amount procured.

A Payer's Perspective

The health care marketplace contains a broad base of stakeholders, each with their own perspective and bias, often working in silos. Within this marketplace, payers work to develop coverage policies to manage the medical and pharmacy benefits of their beneficiaries. The coverage process is a complex and often local one, taking into account multiple factors including the available published evidence for health care technologies. Evidence reviews of health care technologies are being developed and deployed by payers with little coordination or collaboration, creating an environment where there is significant duplication of effort. In addition, current processes lack transparency and marketplace engagement of stakeholders. Payers, manufacturers, medical specialty societies, and health systems can benefit by working together, leveraging the strengths of each organization to deliver high-quality, best-evidence reviews that may be used across the market for local coverage decisions.

The assessment of value is an evolving science. At its core, the value of a health care technology relates to its ability to affect the overall net health outcomes for a particular patient population. Net health outcome is the measure by which all benefits and harms associated with a technology are considered. When added together, the net benefit or net harm is one essential element in the evaluation of value. By this definition it is easy to see how the value of a technology may vary based on the known improvement in the net health outcomes for one population over another. Assessment of the net health outcome is challenging, requiring definitional accuracy of the: (P)opulation; (I)nterventions; (C)omparators; (O)utcomes of interest for the technology under review. Once the scope of the review is established under this PICO construct, the work of evaluating the published evidence can begin.

Accurately defining the PICO and gathering the best set of evidence may best be done through a transparent process including stakeholder input. BCBSA has developed such a process called Evidence Street to foster marketplace transparency and collaboration. Engaged stakeholders include payers, manufacturers, and clinical experts. Over time, this transparent market-based process could align the timing and requirements for payer-evidence reviews used for coverage. Coverage decisions will always remain local but the needs for high-level evidence review to manage the total cost of care are only increasing for both payers and providers. Access to a common set of high-quality evidence reviews, developed through a transparent and efficient process, would create market-based efficiency and save costs across the health care system.

The definition of value will continue to evolve and become more refined within the health care marketplace. Already established is the need to evaluate health care technologies within the population of intended use. The more transparent and engaged payers are with stakeholders—including, but not limited to, regulators and industry—the more likely we are to identify technologies of value.

References

- [1] Chatterjee A, King J, Kubendra S, DeVol R. Healthy savings: medical technology and the economic burden of disease. Milken Institute. July 2014. [2] US Census Bureau and OECD, 2016. [3] Stabile M, Thomson S, Allin S, et al. Health care cost containment strategies used in four other high-income countries hold lessons for the United States. *Health Affairs* 2013;32(4):643-52. [4] Bentley TGK, Effros RM, Palar K, Keeler EB. Waste in the U.S. Health Care System: A Conceptual Framework. *Milbank Q* 2008;86(4):629-659. [5] Organization for Economic Cooperation and Development. Country note: How does health spending in the United States compare? Available from: <https://www.oecd.org/unitedstates/Country-Note-UNITED%20STATES-OECD-Health-Statistics-2015.pdf>. [Accessed September 6, 2016]. [6] Neumann PJ, Rosen AB, Weinstein MC. Medicare and cost-effectiveness analysis. *N Engl J Med* 2005;353(14):1516. [7] Sorenson C, Kanavos P. Medical technology procurement in Europe: A cross-country comparison of current practice and policy. *Health Policy* 2011;100(1):43-50. [8] Sorenson C, Drummond M, Khan BB. Medical technology as a key driver of rising health expenditure: disentangling the relationship. *ClinicoEconomics and Outcomes Research*:

- CEOR* 2013;5:223-234. [9] Drummond M, Griffin A, Tarricone R. Economic evaluation for devices and drugs—same or different? *Value Health* 2007;12(4):402-4. [10] Food and Drug Administration. (2016). “Adaptive Designs for Medical Device Clinical Studies” (issued July, 2016). Available at: <http://www.fda.gov/ucm/groups/fdagov-public/@fdagov-meddev-gen/documents/document/ucm446729.pdf>. [Accessed December 28, 2016]. [11] Food and Drug Administration. (2015). “Acceptance of Medical Device Clinical Data from Studies Conducted Outside the United States” (issued April, 2015). Available at: <http://www.fda.gov/ucm/groups/fdagov-public/@fdagov-meddev-gen/documents/document/ucm443133.pdf>. [Accessed December 28, 2016]. [12] Food and Drug Administration. (2013). “Design Considerations for Pivotal Clinical Investigations for Medical Devices” (issued November, 2013). Available at: <http://www.fda.gov/downloads/MedicalDevices/DeviceRegulationandGuidance/GuidanceDocuments/UCM373766.pdf>. [Accessed December 28, 2016]. ■

Additional information:

The preceding article is based on an issue panel presentation given at the ISPOR 21st Annual International Meeting.

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