

Promoting Biomedical Innovation and Economic Value: New Models for Reimbursement and Evidence Development

DISCUSSION GUIDE April 2011

The Outlook for Biomedical Innovation

Over the last several decades, the United States has experienced significant gains in overall life expectancy and health status. Biomedical innovation has played a crucial role in enabling these gains, by advancing our ability to prevent, diagnose, and treat disease. Much of this improvement has occurred in life-threatening illness. For example, from 1996 to 2006, the U.S. mortality rate from cardiovascular disease decreased by 29.2 percent, and from 1990 to 2006, the U.S. mortality rate for all cancer types decreased by 21.0 percent for men and 12.3 percent for women.^{1,2} In turn, improvements in health have tremendous economic value. For example, one study found that in 2002 alone, more than \$16 billion in direct medical costs was averted due to strokes and myocardial infarctions avoided through better hypertension treatment.³ Similarly, gains in cancer survival have created an estimated \$1.9 trillion in social value.⁴ The life sciences industry is also a critical part of the 21st-century U.S. economy. In 2008, the biopharmaceutical and medical technology industries employed 1.1 million Americans.⁵

The emerging fields of genomics, proteomics, and metabolomics, as well as advances in diagnostics and device technology, offer the promise of safer, more effective, and more individualized health care. These disciplines are enhancing our understanding of biology at a molecular level, explaining potentially important sources of variation among individuals, and even among cells in the same individual. To the extent that these variations explain the mechanisms of disease, and/or explain differences in risk of disease, rates of disease progression, and responses to treatment, they will improve our ability to prevent, detect, and treat diseases on a more personalized basis. Medical products that have grown out of these fields include molecular diagnostics to guide treatment selection, sophisticated biotechnology treatments, vaccines, and advanced medical devices to prevent, diagnose and manage cardiovascular disease, neurologic disorders, cancers, disabling orthopedic conditions, and many other major diseases. On the horizon are more potentially important technologies, such as gene therapy, regenerative medicine, and nanotechnology.

But the impact of these innovations on medical practice have been limited so far, and productivity in biomedical innovation has declined in recent years.^{6,7,8} Investors and other stakeholders have expressed concerns about regulation and payment for innovative technologies. Regulatory concerns include lack of transparency, unpredictable evidence requirements, high costs and time to conduct required studies, and long review timelines. With respect to reimbursement, innovators are concerned about the process of securing market access and reimbursement for new drugs, diagnostics, and other medical technologies, for which unclear and inconsistent evidence requirements increase uncertainty. Further, even when a product is covered, efforts to limit utilization through coverage limits, cost-sharing, prior authorization, and other means can make it difficult to forecast a new product's return on investment. Finally, questions have been raised about the consequences for innovation of payment reforms like accountable care organizations, which aim to make doctors and hospitals more cost-conscious.⁹

In response to concerns about the time, cost, and uncertainty of biomedical innovation, a number of new initiatives have been launched to promote biomedical innovation in the United States. The Cures Acceleration Network, an independent agency formed under the Affordable Care Act, will award grants to entities focused on developing new cures and treatments of diseases.¹⁰ Late in 2010 the National Institutes of Health announced that it is considering the creation of a new National Center for

Advancing Translational Sciences, which would focus on accelerating the development of new therapeutics based on promising scientific discoveries.¹¹ To address the need for regulatory reforms, the U.S. Food and Drug Administration (FDA) has taken steps to increase the transparency and predictability of its regulatory processes. In October 2010, the Agency launched its Advancing Regulatory Science for Public Health initiative, which is focused on furthering the field of regulatory science to support more effective regulatory review. In its first year, this initiative is exploring ways of transforming product development (e.g., through modernized toxicology, biomarkers for personalized medicine) and developing the expertise to regulate emerging medical technologies (e.g., nanotechnology), among other topics.^{12,13} This new initiative will build upon the work of FDA's Critical Path Initiative, which was launched in 2004 as a strategy to promote innovation by identifying and solving scientific challenges involved in medical product development. The Critical Path Institute (C-Path), a public-private partnership created in 2005 to support the initiative, has facilitated collaborative research on biomarkers for predicting safety during drug development and identification of new tools and models to better understand and develop therapies for neurodegenerative diseases like Alzheimer's Disease and Parkinson's Disease. In the area of medical devices, FDA's Center for Devices and Radiological Health Innovation Initiative has been established to facilitate safe and rapid approval of new technologies.¹⁴ Finally, private sector efforts, such as the work of the Council for American Medical Innovation, also seek to promote national policy that supports biomedical innovation.

Biomedical Innovation and the Affordability of Health Care

Although recent health care reforms provided health insurance coverage for many previously uninsured individuals, thereby enabling greater access to more routine and preventive care, health care costs continue to rise at an unsustainable rate. Recent surveys show an increasing percentage of Americans are going without recommended drugs and other health care due to cost.^{15,16} Health care spending was approximately 17 percent of gross domestic product (GDP) in 2010, and is projected to reach 26 percent by 2035. While the aging of the population and growing prevalence of chronic diseases, along with rising prices for health care products and services, are contributing to spending growth, the diffusion of advanced medical technologies has also been an important contributor.¹⁷ A 2008 Congressional Budget Office report estimated that over the last several decades, "expanded capabilities of medicine brought about by technological advances" were responsible for roughly half of the increase in health care spending. This is partly because a majority of biomedical innovations reflect advances in the diagnosis and management of chronic conditions – or breakthroughs that convert previously untreated and even fatal conditions into more manageable chronic conditions – rather than curative therapies associated with a one-time expenditure.¹⁸ There is also hope that many technologies will result in avoiding the costs associated with serious illnesses. For example, increased spending on certain types of biomedical innovation (e.g., prescription drugs) may be associated with a reduction in overall health care costs.¹⁹ However, much "preventive" biomedical innovation may not lower overall costs, because patients live longer and develop other, sometimes more costly, health problems.²⁰

The traditional response to rising health care costs has been to reduce payment rates broadly for health care providers and medical products, or to limit coverage and shift more costs to patients. To date, these "blunt instrument" policies have failed to bring cost growth down to sustainable levels. They have also been criticized for potential unintended consequences of limiting access to and quality of care, and hindering biomedical innovation that is moving in the direction of more targeted, high-value treatments. A narrow focus on price may result in short-term cost savings, but the long-term effects may be to drive up costs and reduce the quality of care provided.

Can We Promote Biomedical Innovation and Economic Value at the Same Time?

Is it possible to have a health care system that both promotes timely and valuable medical breakthroughs enabled by modern science, and one that achieves such innovation at the lowest possible cost? The goals of rapid innovation and financial sustainability are often described as being in tension

with each other, if not mutually exclusive. While some argue that biomedical innovation contributes disproportionately to cost growth, others argue that high prices and fee-for-service reimbursement are needed to sustain innovation for advanced medical products and technologies. **The goal of this meeting is to identify feasible steps to promoting rapid innovation and greater economic value in health care. Three expert panels will discuss the potential of new models for reimbursement and evidence development to achieve these goals.**

Paying for What We Want: Innovative Medical Products that Improve Health Outcomes and Avoid Unnecessary Costs

Drugs, diagnostics, and medical devices are currently reimbursed through a combination of approaches, including:

- Reimbursement based on quantity and unit costs (e.g., drugs dispensed by pharmacies and outpatient diagnostic tests);
- Fee-based payment to providers (e.g., physician-administered drugs, and outpatient diagnostic and therapeutic procedures);
- Bundled payments to providers (e.g., a bundled payment for an entire hospital stay that includes payment for drugs and devices used).

These payment systems – and particularly the first two – tie payments more directly to volume than value, and none tie payment directly to the evidence of a medical product’s performance. Rather, if such ties occur, it happens through coverage decisions, which may be difficult or costly to make accurately in individual cases. Recently, however, new models for performance-based reimbursement have been implemented and evaluated for some medical products. Nearly 60 such agreements were recently documented, mostly in Europe and Australia, with an increasing number in Canada and the United States.²¹ A key component of these models is a form of risk-sharing, in which payers and manufacturers each assume a level of financial responsibility for the achievement of mutually agreed-upon goals. Examples include:

- In 2007, Johnson & Johnson agreed to reimburse Britain’s National Health Service (NHS) for patients who did not respond to four cycles of treatment with the biologic therapy for multiple myeloma, bortezomib (Velcade®).²² The deal came after the National Institute for Clinical Excellence (NICE) refused to support coverage for the treatment because its cost-effectiveness analysis found the cost too high to justify the average benefits for the population. The concept of value-based pricing for all drugs has been proposed by the NHS for implementation in 2014.
- In 2008, UnitedHealthcare agreed to reimburse the Oncotype DX molecular diagnostic test at list price (\$3,460 per test) for 18 months while it and the test’s manufacturer, Genomic Health, track the results to determine if the genetic test is having the anticipated effect on actual clinical practice. If the number of women receiving chemotherapy exceeds an agreed-upon threshold, even if the test suggests the patients would not benefit from therapy, the insurer will receive a pre-negotiated lower price.
In 2009, Health Alliance Medical Plans agreed to a contract with the two manufacturers of risedronate sodium (Actonel®), an osteoporosis drug, in which the manufacturers would reimburse Health Alliance Medical Plans the costs of treating certain non-spinal fractures seen in patients who had correctly taken the medication.

Whether these so-called “value-based agreements” effectively improve outcomes or reduce costs remains to be determined. However, these models do have some intuitive advantages. For manufacturers, these agreements provide a mechanism to offer incentives to payers and increase market access while simultaneously protecting the list price of their products. This is particularly important for groundbreaking products that have required extensive research and development. From a

societal perspective, value-based payment models may offer greater assurance that patients receiving the treatments really do benefit, and they tend to promote the development of greater evidence regarding the real-world effectiveness and safety of medical products in particular kinds of patients. For payers, these agreements offer the intuitive appeal of tying payment to proven results in the population.

Despite the apparent promise of value-based payment agreements, there has been limited uptake, particularly in the United States. A major barrier is the setup cost, at least in the short term, involved in designing the contract. Without a large body of precedents, groundbreaking innovative agreements require the time and expertise of legal, financial, and scientific experts.²³ Monitoring the degree to which stakeholders are continuing to abide by the terms of the agreement, and the collection of utilization and outcomes data, require a sophisticated infrastructure, which may not already be in place. This may be particularly challenging to scale-up in the United States, where there are multiple third-party payers, each of which maintains its own data and payment infrastructure – often designed around fee-for-service payments. More work is also needed to effectively integrate performance-based medical product reimbursement with evolving reforms to benefit designs and provider payment systems. Further, attribution of health outcomes to medical product performance – versus physician or patient factors that could affect performance – can be difficult. Finally, accurate and timely measures of performance that are reliable enough for use may not be available.

Ideally, reforms to how we pay for medical products would be implemented and supported by reforms to provider payment and appropriate incentives for patients. Some newer models for the organization, delivery and financing of care – including patient-centered medical homes, payment bundling, and accountable care organizations – are designed to promote better quality at a lower cost by paying health care providers based on both costs and outcomes achieved in their patients. As these reforms are implemented, it will be important to monitor the uptake of new technology and the degree to which patterns of care reflect scientific evidence. Value-based insurance design (VBID) has been described as a way of designing benefits and cost sharing to encourage patient behaviors that improve health outcomes and lower long-term costs.²⁴

Issues in Measuring Value in Innovation

The technical challenges in measuring the performance of medical technologies are a substantial barrier to reforming payment systems. In particular, improved data systems and methods are needed to enable better measurement of utilization, outcomes, and potential modifiers and confounders of those outcomes at the individual level – over the life cycle of the technology.²⁵ Ideally, the development of such evidence would be less costly and more timely in both the pre-market and post-market settings. In the pre-market setting, clear and consistent evidentiary requirements for regulatory approval and reimbursement are key to reducing uncertainty and planning development programs. In the post-market setting, making better use of data generated through routine delivery of health care services (i.e., administrative claims and electronic medical records) and developing registries for new technologies hold promise. For example, the FDA's Sentinel Initiative for active post-market safety surveillance remotely queries the databases of collaborating health plans and integrated delivery systems, providing secure access to data on more than 70 million individuals.²⁶ The FDA's Center for Devices and Radiological Health (CDRH) has also recently launched the Medical Device Epidemiology Network (MDEpiNet) initiative, which will create a network of academic institutions for the purposes of collecting data regarding medical device performance and advancing methods for analysis of these data.²⁷ Other important infrastructure investments are being made to enable comparative effectiveness research, including a multi-payer claims database and distributed research networks.²⁸ The work of the newly established, Patient-Centered Outcomes Research Institute – and its methodology committee in particular – is intended to help develop and implement consensus-based methods for evaluating innovative new technologies, and identifying the subgroups of patients in whom they work best. Enhanced data and methods hold the potential for better evidence on the outcomes of treatment

strategies and technologies, as well as more robust measurement of the impacts of policies that influence the adoption and use of medical products.

Even with valid measures, payers and other stakeholders may disagree on how they should be used. For example, U.S. payers include public systems (e.g., Medicare, Medicaid, Department of Veterans Affairs, Department of Defense) and an array of private health plans, each of whom may define value and innovation differently – or place a higher value on different innovations. For a medical product manufacturer interested in developing a performance-based agreement for a new technology, negotiating a consistent set of terms is a daunting challenge. Without consensus-based measures, it may be difficult to provide stronger support for value.

The Path Forward

The Engelberg Center for Health Care Reform at Brookings and the Leonard D. Schaeffer Center for Health Policy and Economics at the University of Southern California (USC) are pleased to convene this forum to discuss practical steps that can promote biomedical innovation and economic value, with emphasis on new approaches to reimbursement and developing better evidence. The central questions for discussion are:

- How do current payment systems and benefit designs impact biomedical innovation, and what reforms would promote innovation and economic value?
- Does linking medical product reimbursement to clinical outcomes promote innovation and avoid unnecessary costs?
- How can we improve our capacity to measure innovation and value in the health care system, and for individual medical products?

Promoting biomedical innovation and economic value is imperative to preserving our nation's status as the global leader in biomedical innovation – and ensuring that individuals who can benefit from advanced technologies receive them as quickly and widely as possible. This conference and the ensuing activities at Brookings and USC will focus on identifying feasible steps toward developing and evaluating policies that support these objectives.

References

- ¹ Cardiovascular disease statistics. American Heart Association, 2011. Accessed at: <<http://www.americanheart.org/presenter.jhtml?identifier=4478>>.
- ² Jemal A, Siegel R, Xu J, Ward E. Cancer statistics, 2010. *CA Cancer J Clin* 2010;60(5):277-300.
- ³ Cutler DM et al. The value of antihypertensive drugs: a perspective on medical innovation. *Health Aff.* 2007 Jan-Feb;26(1):97-110.
- ⁴ Lakdawalla DN, Sun EC, Jena AB, et al. An economic evaluation of the war on cancer. *J Health Econ* 2010;29(3):333-46.
- ⁵ Gollaher DL and Goodall S. Competitiveness and Regulation: The FDA and the Future of America's Biomedical Industry. La Jolla, CA: California Healthcare Institute: 2011. Accessed at: <<http://www.bcg.com/documents/file72060.pdf>>.
- ⁶ Ibid.
- ⁷ Boat T. Insights from trends in biomedical research funding. *JAMA* 2010;303(2):170-1.
- ⁸ Gone tomorrow? A call to promote medical innovation, create jobs, and find cures in America. Columbus, OH: The Battelle Technology Partnership Practice, 2010. Accessed at: http://www.americanmedicalinnovation.org/sites/default/files/Gone_Tomorrow.pdf.
- ⁹ Gottlieb S. Accountable care organizations: the end of innovation in medicine? American Enterprise Institute for Public Policy Research Health Policy Outlook 2011:3:1-8.
- ¹⁰ Patient Protection and Affordable Care Act 2009 HR 3590 – 860.
- ¹¹ Proposed National Center for Advancing Translational Sciences. Bethesda, MD: National Institutes of Health, 2011. Accessed at: <<http://feedback.nih.gov/index.php/faq-ncats/#one>>.
- ¹² Advancing regulatory science for public health. Silver Spring, MD: US Food and Drug Administration, 2010. Accessed at: <<http://www.fda.gov/downloads/ScienceResearch/SpecialTopics/RegulatoryScience/UCM228444.pdf>>.
- ¹³ Hamburg M. Advancing Regulatory Science. *Science* 2011;331:987
- ¹⁴ CDRH Innovation Initiative. Center for Devices and Radiological Health. US Food and Drug Administration, 2011. Accessed at: <<http://www.fda.gov/downloads/AboutFDA/CentersOffices/CDRH/CDRHInnovation/UCM242528.pdf>>.
- ¹⁵ Doty MM, Collins SR, Nicholson JL, Rustgi SD. Failure to protect: why the individual insurance market is not a viable option for most U.S. families. New York, NY: The Commonwealth Fund, 2009. Accessed at: <<http://www.commonwealthfund.org/Content/Publications/Issue-Briefs/2009/Jul/Failure-to-Protect.aspx>>.
- ¹⁶ Low-wage workers and health care. Kaiser Public Opinion Survey Brief. Menlo Park, CA: The Henry J. Kaiser Family Foundation, 2008. Accessed at: <<http://www.kff.org/kaiserpolls/upload/7804.pdf>>.
- ¹⁷ Technological change and the growth of health care spending. Washington, DC: Congressional Budget Office, 2008.
- ¹⁸ Ibid.
- ¹⁹ Shang B, Goldman DP. Prescription drug coverage and elderly Medicare spending. Working paper 13358. Cambridge, MA: National Bureau of Economic Research, 2007. Accessed at: <http://www.nber.org/papers/w13358.pdf>>.
- ²⁰ Russell LB. Preventing chronic disease: an important investment, but don't count on cost savings. *Health Aff.* 2009 Jan-Feb;28(1):42-5.
- ²¹ Carlson JJ, Sullivan SD, Garrison LP, Neumann PJ, Veenstra DL. Linking payment to health outcomes: a taxonomy and examination of performance-based reimbursement schemes between healthcare payers and manufacturers. *Health Policy* 2010;96(3):179-90.
- ²² Garber AM, McClellan M. Satisfaction guaranteed—"payment by results" for biologic agents. *New England J of Med.* 2007;357:1575-7.
- ²³ Towse A, Garrison LP. Can't get no satisfaction? Will pay for performance help?: Toward an economic framework for understanding performance-based risk-sharing agreements for innovative medical products. *Pharmacoconomics* 2010;28(2):93-102.
- ²⁴ Chernew ME, Rosen AB, Fendrick AB. Value-based insurance design. *Health Affairs* 2007;26(2):w195-203.
- ²⁵ Tunis SR, Benner J, McClellan M. Comparative effectiveness research: Methods development, policy context and research infrastructure. *Stat Med.* 2010;29(19):1963-76.
- ²⁶ Behrman RE, Benner JS, Brown JS, McClellan M, Woodcock J, Platt R. Developing the Sentinel System - A national resource for evidence development. *N Engl J Med.* 2011; 364:498-9.
- ²⁷ FDA's MDEpiNet - improving medical device safety and effectiveness. Silver Spring, MD: US Food and Drug Administration, 2011. Accessed at: <http://www.fda.gov/AboutFDA/Transparency/track/ucm222320.htm#CDRH_Video>.
- ²⁸ Benner JS, Morrison MR, Karnes EK, Kocot SL, McClellan M. An evaluation of recent federal spending on comparative effectiveness research: priorities, gaps, and next steps. *Health Aff.* 2010 Oct;29(10):1768-76.