

social interactions and networks, behavior, cultural characteristics, and lifestyle. In addition, physical measurements, interviews, questionnaires, and images will be used to assess social behavior, neurodevelopment, physical growth, and family mental health and dynamics.

Although the Vanguard Study will determine the precise environmental assessments to be used, the current plan involves approaches including collection of biospecimens (e.g., blood, urine, saliva, skin swabs, cord blood and samples, placental tissue, and breast milk) and environmental samples (e.g., airborne particulate matter, dust, soil, and water), as well as community-based environmental data, to assess environmental exposures. The study will use observations of the interior and exterior of residences and neigh-

borhoods to identify sources of environmental contaminants and neighborhood characteristics. Questionnaires regarding household occupations, work commutes, lifestyle, hobbies, and daily routines will also be deployed.

The final design for the initial stages of the Main Study is anticipated within a few months after the release of an analysis of the proposed Main Study design, due next summer, from the Institute of Medicine and the National Research Council. Initial contracts should be awarded in the first half of 2015, and the study launched several months later. Because of the importance of the fetal and early-childhood periods, the NCS should provide complex new information within a year or two after launch. Of course, as data and samples accumulate, the study's value and impact should

grow commensurately. We expect its longitudinal collection of linked environmental, biologic, and phenotypic data and samples to provide important insights into health, growth, and development, not only of U.S. children but of people of all ages and countries.

Disclosure forms provided by the authors are available with the full text of this article at NEJM.org.

From the Eunice Kennedy Shriver National Institute of Child Health and Human Development (A.E.G., S.H.) and the Office of the Director, National Institutes of Health (F.S.C.) — both in Bethesda, MD.

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## Public Reporting, Consumerism, and Patient Empowerment

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Several forces in the United States — including the Affordable Care Act (ACA) of 2010 — have promoted greater public reporting of health care outcomes. By many accounts, this reporting is largely ignored by consumers (see graph),<sup>1</sup> perhaps because the information is hard to find or difficult to understand. We propose another potential explanation — namely, that the public spotlight is not aimed at information that most patients value.

Current public reports typically compare health care providers in terms of quality or cost to help consumers decide where or from whom to seek care. For example, patients in New York and Pennsylvania can view the cardiac-surgery outcomes for spe-

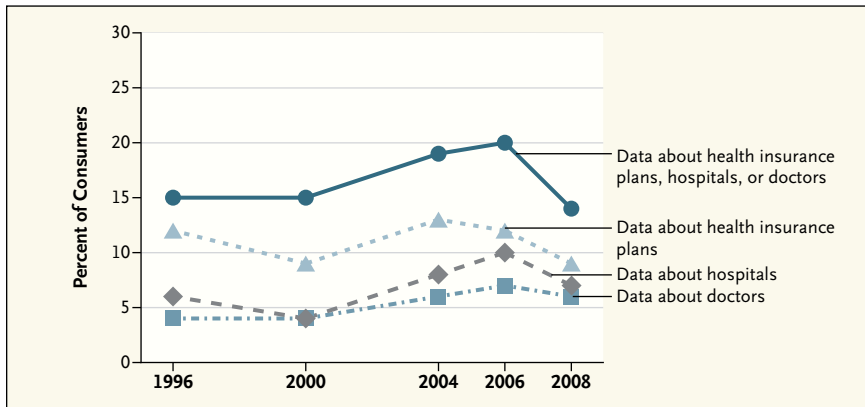
cific surgeons and hospitals. Such reporting assumes that patients have already decided to pursue cardiac surgery and are using this information simply to select the best provider. Unfortunately, this information does little to help patients decide whether they want or need surgery in the first place.

Current public reports also tend to assume that patients can accurately interpret quality metrics. For example, what is the difference between a hospital with a 1% complication rate and another with a 2% rate? One perspective is that the first facility is twice as good as the second. An alternative view is that the absolute risk of a complication is so low at both institutions that choosing between them should hinge

on other factors, such as convenience, cost, and reputation.

Patients may favor this latter interpretation more often than we imagine. Even some patients with education beyond high school have difficulty understanding basic statistics,<sup>2</sup> so it's not surprising that many of them view public reports as unhelpful. Rather than choosing between providers of a specific procedure, perhaps patients are seeking an answer to a more fundamental and personal question: "Is the proposed treatment or procedure the best option given my condition, my financial status, and my social or family situation?"

Presenting and explaining this information to patients — as the first step in what is termed shared



**Consumers' Access to and Use of Data Comparing Quality of Health Care, 1996–2008.**

Shown are the proportions of consumers who reported having access to information regarding insurance plans, hospitals, or doctors and who used that information in making health care decisions in the past year. Data are from the Kaiser Family Foundation (<http://kaiserfamilyfoundation.files.wordpress.com/2013/01/7819.pdf>).

decision making — has been associated with reductions in health care utilization. This new type of clinical practice, however, requires substantial investments in provider training, information systems, and process reengineering.<sup>2</sup> Most clinicians have neither the time nor the financial incentives to make such investments, especially in a fee-for-service environment. With physicians motivated to provide more care and patients lacking the information to question such care, there are few natural brakes on medical utilization. By focusing patients on choosing between providers of specific services, current public reporting thus does little to keep the proverbial horse of medical utilization inside the barn. Indeed, it provides a guided path for its escape.

This situation may be changing. The growth rate of real national health care spending fell to 0.9% for the years 2010 to 2012. Some of this slowdown can be attributed to the recession, but private-insurance redesign is also a major factor. Between 2006 and 2012, the prevalence of high-deductible health plans grew by

24%, exposing one third of privately insured employees to deductibles of at least \$1,000. Copayments have shown similar growth, with nearly half of covered employees paying \$25 or more for an office visit. These trends come at a time when most families have seen little increase in real wages or benefits.<sup>3</sup> Recent announcements by several large corporations speak to the growing trend of shifting more costs to employees and converting defined health benefits to defined contributions. In this environment, patients have become more discriminating consumers. Once shielded from health care costs, consumers are now seeing those bills eat further into their family budgets. That new awareness may explain why the number of physician visits among privately insured patients fell 17% nationally between 2009 and 2011.<sup>3</sup>

So are we seeing the dawn of a consumer-driven health care economy, in which patients undertake the same deliberations regarding medical purchases as they do when purchasing furniture or a new car? Many health care providers seem to think so. Both govern-

ment and industry are determined to cut health care expenditures. Providers are building infrastructure to prepare for the day when future payment is linked to reducing utilization and cost. Opportunities abound in areas where the health care sector has grown the most — diagnostic technology, high-tech procedures, expensive pharmaceuticals and devices, and post-acute care.

Patients may welcome this strategy, but they will want information that goes beyond current public reporting. To be sure, technical details of quality, safety, and process will remain important in health care, as they are in manufacturing, transportation, and hospitality. But consumers want clear and concise information that they can understand on factors such as out-of-pocket costs, the effectiveness of a procedure or treatment, and applicability to their personal condition and social situation.

The demand for this information coincides with the growth of personalized medicine, in which individuals' genetic profiles will increasingly be used in determining which drugs or cancer therapies to prescribe, as well as in predicting future disease. We now have a more urgent need for a similarly personalized approach to the health care decisions that patients encounter daily,<sup>4</sup> but there is more involved than just clinical decision making. The analogue of the genetic code in this case is a combination of the patient's clinical problem, the effectiveness and cost of the remedy, and the resulting social or economic consequences for a patient's family.

We are far from cracking this code. The economics of health care alone is a major barrier. Wide variation in hospital prices creates the impression that health

care defies conventional market logic.<sup>5</sup> Health care is entangled in complex pricing systems that even most health care professionals do not understand. Trained in diagnosis and treatment, physicians have little education in counseling patients on options and economic issues. With more of their own dollars at risk, however, patients will eventually insist that health care providers deliver the value they expect from other businesses.

This surge in consumerism has already stimulated the growth of retail delivery models. Companies including Wal-Mart, CVS Caremark, and Walgreens have entered the world of health care delivery, with capital, information technology, and national distribution systems. These firms offer convenient locations with standardized processes and are expert at managing cost and price. They are likely to be powerful change agents in this new era of health

care consumerism and may encourage other companies to enter this large segment of the U.S. economy.

As patients become more sophisticated purchasers of health care, they will push competition in health care delivery to look increasingly like that in consumer-goods industries. This competition could lead to product offerings that appeal to consumers with different needs. While some patients may seek greater odds of survival, others may seek a faster return to work or lower out-of-pocket costs. These options are at the core of “patient-centered” care.

To move health care in this direction, public reporting must shift from “one size somewhat fits all” to an approach that reports metrics reflecting the varied concerns and preferences of consumers. With better information, millions more patients can become smart shoppers and, in

the process, help bend the health care cost curve.

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## Expediting Drug Development — The FDA’s New “Breakthrough Therapy” Designation

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Many people with serious or life-threatening illnesses for which there are no satisfactory treatments are understandably eager to gain access to new therapies and are willing to trade off greater certainty about a drug’s performance for speed of access. Because the typical clinical drug-development program takes about 7 years, during which a substantial body of safety and efficacy data is generated, the Food and Drug Administration (FDA) has long-standing expedited pathways available for drugs being studied for such illnesses. However, many patients and their advocates con-

tinue to believe that clinical development is sometimes prolonged beyond what is necessary. During the congressional considerations leading up to passage of the FDA Safety and Innovation Act of 2012 (FDASIA), a variety of provisions related to this theme were put on the table. When the bill was enacted, two modifications of the Federal Food, Drug, and Cosmetic Act addressed the issue of drug development for serious illnesses: a new “breakthrough therapy” designation for investigational drugs and expansion of the statute regarding accelerated approval. The break-

through-therapy designation has since been introduced into the FDA portfolio of expedited programs for serious conditions.

The genesis of the new designation can be traced to several emerging trends in drug discovery and development. Most notable is the rise of molecularly targeted therapies, often paired with companion diagnostics, for treatment of cancer, genetic diseases, and increasingly, other serious illnesses. These therapies are directed at subgroups of patients (within the larger population with a given disease) who are predicted to benefit from