Evaluating New Diagnostic Technologies: Perspectives in the UK and US

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In March 2008, Lord Darzi, a Parliamentary Under Secretary of State for Health in the UK, announced formation of a Health Innovation Council “to act as an overarching guardian for innovation from discovery through to adoption” for new diagnostic technologies, while also “holding the Department of Health and the National Health Service to account for helping to overcome barriers and taking up innovation.”

In 2001 the Institute of Medicine in the US attributed shortcomings in the quality of health care to 4 main reasons: (a) the growing complexity of science and technology, (b) the increase in chronic conditions, (c) a poorly organized delivery system, and (d) constraints on exploitation of the revolution in information technology. The Institute concluded that science and technology have advanced more rapidly than the ability of health care systems to deliver them safely, effectively, and efficiently, and proposed a number of initiatives to address these issues. The emphasis on these themes in the UK and the US are indicative of the poor state of translational research in medicine, a situation that is probably reflected in many health systems across the world, and equally so in laboratory medicine. Concerns about the quality of health care point to the need for a greater focus on outcomes research—a call made by George Lundberg for laboratory medicine several years ago (1). This issue was recognized in the UK in a report published recently by the Royal College of Pathologists and the Foundation for Genomics and Population Health (2).

In the UK there is no formal system for evaluation and adoption of new diagnostic tests, although the Department of Health is sponsoring a health technology assessment program. Regulation is covered by a European Directive, CE marking (Conformité Européenne), which certifies that a product has met European Union health, safety, and environmental standards. Furthermore, there is no formal system of reimbursement.

However, the UK Department of Health is committed to the introduction of a tariff as well as to a process that uses commissioning as a means to separate the purchaser of services from the provider. The commissioner’s focus will be on care pathways, with improving outcomes and increasing value-for-money as key objectives. Undoubtedly these objectives are also applicable to the US health care system. Unfortunately, neither country has an obvious mechanism for linking the evaluation of new tests with an assessment of the value of the technology in the context of the care pathway.

Scenarios for overseeing diagnostic innovations involve a number of challenges. Both translational research and the evidence base for diagnostic techniques are inadequate, and little incentive exists for providing necessary evidence. A more systematic approach to the evaluation of new tests has been called for. This approach addresses 4 aspects of a diagnostic test: (a) analytical validity, whether the test measures what it is claimed to measure (and whether there is evidence to justify CE marking); (b) clinical validity, whether the test answers the clinical question being asked (e.g., screening, diagnosis, prognosis, monitoring); (c) clinical usefulness, whether the test leads to better outcomes; and (d) social context, including ethical, economic, and legal issues (3). Sackett and Haynes (4) pointed out that the key question is “Do patients undergoing the diagnostic test fare better than similar untreated patients?” In other words, does the use of the test lead to better outcomes? Most of the evidence available today deals with the analytical validity of a test, and to a lesser extent its clinical validity, with little evidence available regarding clinical usefulness, i.e., impact on patient outcomes. This lack of emphasis on clinical validity may be attributable to the low priority given to translational research on diagnostic tests and the lack of requirements for submission of evidence of impact on patient outcomes. Incentives for change are lacking, given that health care is managed on a “silo” basis, focusing on the cost of the test rather than potential benefits to the care pathway and the health economy (5).

Such incentives must be built on acknowledgement that the use of a diagnostic test can lead to improved outcomes (when existing evidence demonstrates this possibility). Furthermore, reimbursement must be based on recognition of improved patient outcomes. This philosophy is embodied in the concept of “pay for...
performance,” which is already applied to care pathways but not yet to laboratory medicine.

The UK report (2) made a number of key recommendations: (a) commissioners (purchasers) and health care professionals should be encouraged to use only tests for which sufficient evidence of utility exists, (b) a body of independent experts should be responsible for evaluating the evidence of test performance and making recommendations about clinical applications, (c) statutory regulators should be empowered to require that evidence (or lack of it) relating to test performance be placed in a database that is accessible to the public, and (d) this database should include the evidence, or lack of evidence, on the clinical validity and utility of each test.

In contrast to the system used in the UK, the US procedure for evaluation of new technologies is complicated, multifaceted, and inextricably linked to payment. The process involves the US Food and Drug Administration (FDA),3 the Centers for Medicare and Medicaid Services (CMS), and other payers as well as the American Medical Association; assignment of a Common Procedural Terminology (CPT) code; and most importantly, acceptance by practitioners. The process typically takes several years from submission of the technology for FDA clearance or approval to the endpoint, routine use of the new technology.

The US system focuses conceptually on evidence that new technology is clinically effective, i.e., the technology is the right test when it is performed on the right patient at the right time. The outcomes used to demonstrate clinical effectiveness naturally depend on the clinical question(s) as driven by the indication or claim for the technology. After the initial development and testing procedures, the process leading to the availability of virtually any new diagnostic technology (with the exception of “home brew” type assays) begins with a request for clearance or approval submitted to the FDA. This evidence-based process addresses accuracy and reliability; appropriate labeling that includes recommendations for use, and risks vs benefits. The first element requires evidence for accuracy and reliability demonstrated by use of investigations performed in the appropriate study population. These investigations must employ a valid reference method or comparator, with appropriate outcomes and performance assessments that reasonably assure that the technology is safe and effective for the intended use. For the second element, labeling with indications for use must be supported by appropriately designed studies and documented outcomes. The third element involves adequate evidence for analysis of the potential risks and benefits when the technology is used as intended. The stipulation “as intended” holds key importance because the FDA, like CMS and other payers, performs evidence-based evaluations only for claims submitted by the manufacturers. Thus this third element is necessarily associated with positive or negative outcomes for the intended patient population and with broader public health. The FDA rarely requires clinical outcome studies for in vitro diagnostic methods (6), unless such studies are justified by manufacturer claims. Although disagreement may arise regarding conclusions drawn by the FDA, an asset of the process is its transparency. Readers can examine the reviews of 510(k) and premarket approval submissions in the FDA database at http://www.fda.gov/cdrh/oivd (accessed June 25, 2008).

After FDA approval or clearance has been obtained, the technology can be considered for CMS payment or coverage. Decisions regarding CMS coverage also involve an evidence-based process. To be eligible for CMS coverage, the new technology must be demonstrated to be “reasonable and necessary” for the diagnosis and treatment of the target illness. Although CMS uses the standard principles of evidence-based medicine for this process, no standard definition exists for “reasonable and necessary,” so such analyses involve some degree of subjectivity. Individual studies and their relevance to the CMS population, and overreaching conclusions on the direction and magnitude of the risks and benefits are considered. Possible outcomes of the CMS evaluation are a decision for non-coverage, coverage with or without limitations, or coverage at the discretion of local CMS contractors.

CPT-code assignment determines the amount of coverage payment allotted for a new technology. Once a CPT code is assigned to the new technology, payment to providers is made available contingent upon favorable coverage policies from CMS and private payer plans. The actual amount of payment can be determined by “cross-walking” to a clinically similar existing CPT code or undergoing a “gap-fill” for the new CPT code when there is no existing clinically similar CPT for the technology. Another critical step, in addition to the FDA, CMS, CPT, and payment assignment processes, is provision of evidence that convinces practitioners of an advantage of the new technology in terms of better patient outcomes or cost savings. Such evidence is necessary for acceptance of the new technology in practice and inclusion of the technology in guidelines and care pathways. Establishing technologies as part of best practice will almost certainly become more important in the future. In the US system, prob-

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lematic issues with reimbursement for laboratory testing are widely recognized, and some options have been discussed in a report from the Institute of Medicine [7]. Pay for performance is a paradigm founded on the notion that evidence-based practice improves the quality of care and ultimately benefits patient outcomes [8]. In practice, institutions receive greater reimbursement per patient case if they are high performers as indicated by selected benchmarks and standards; those institutions viewed as underperforming will receive less reimbursement. Thus a realignment of incentives is emerging that is intended to encourage more efficient and effective provision of the right care at the right time for the right patient. Although only incomplete evidence links these programs to better care, the argument for linking compensation to effective care is compelling: programs seeking to do so have been implemented by health maintenance organizations in the US and for family practitioners in the UK. Could pay for performance be applied to laboratory medicine in the context of improving patient outcomes and, if so, how could it be applied?

Implementation of pay for performance as a way to allocate financial resources underscores the importance of high-quality evidence for the effectiveness of any new technology and the impact of evidence-based medicine on health outcomes. This selective allocation could profoundly impact the use of new diagnostic technologies and will profoundly impact health care management, because benefits will be seen in other parts of the health care organization. Under the influence of pay for performance or a similar paradigm, technologies with substantial potential might fall by the wayside unless appropriate evidence is available to demonstrate their positive impact. Thus it will be essential to obtain convincing evidence demonstrating a positive impact on patient outcomes at the time that a new technology becomes available.

Changes in the process of adopting new diagnostic technologies will have a major impact on laboratory medicine for practitioners and industry professionals as well as health care organizations, highlighting the need to involve health care organizations (purchasers and providers), academia, grant-funding organizations, and industry in issues surrounding funding and gathering of necessary evidence. These new approaches set the stage for an exciting translational research agenda for the future. They challenge innovators, developers, and manufacturers of new technology to identify the intended use(s) and produce the evidence of impact on health care outcomes. Meanwhile health care organizations (purchasers, providers, regulators, and payers) must acknowledge the paradigm shift so that the impact of laboratory medicine on outcomes is recognized in health care management practice.

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**References**