Neuroblastoma, Well-Designed Evaluations, and the Optimality of Research Funding: Ask Not What Your Country Can Do for You …

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Health care technology assessment (HTA) has garnered the attention of policy makers over the past 20 years because the introduction of new technologies is widely believed to have been a major driver of increased health care expenditures in developed countries (1). Careful evaluation of health care technology prior to widespread introduction and diffusion has become a priority for policy makers who are committed to evidence-based decision making, and those involved with HTA have argued the merits of including economic evaluations alongside clinical effectiveness evaluations. It has been recognized that rigorous economic evaluations of new technologies may not always be feasible, and even if feasible the evaluation itself may not be cost-effective (2). In this issue of the Journal, Soderstrom et al. (3) show that HTA-type research itself can be, as in their assessment of the economics of neuroblastoma screening evaluation, a remarkably cost-effective health care strategy from a societal perspective. Although extremely limited, the literature appears to support this conclusion, and Soderstrom leaves us with the tantalizing question of whether the current funding for well-designed HTA evaluations is sufficient. If not, he contends, the adverse health effects and wasteful health spending caused by the introduction of new but ineffective health services could be substantial.

But how much assessment is enough? What should society spend on medical research as a whole? On health services research? On HTA?

In the United States, the medical community and others have advocated strongly for increased federal funding of medical research. The American Medical Association, for example, has called for this funding to be “ample,” and to address the spectrum of basic biomedical research, translational research, clinical research and clinical trials, health services research, outcomes research, and prevention research (4). These types of efforts have been successful for at least some categories in the research spectrum. Although the U.S. federal government expenditure in the National Institutes of Health is just 5% of its expenditures in the Centers for Medicare and Medicaid Services (5), the NIH budget request for 2006 approaches $28.6 billion—an increase of more than 60% in just 5 years (6). And private industry funds perhaps twice as much basic and preclinical biomedical research as the NIH (7).

Formal, publicly funded HTA funding has not fared as well. The Agency for Healthcare Research and Quality (AHRQ) aims to improve the effectiveness of health care for Americans through health services research and HTA. Its budget request for 2006 is $319 million (5), about 1% of the NIH level of funding. And while some have suggested that federal support of health economics research has paralleled the growth in medical research in general, concerns have been expressed about gaps or duplications in some disease and intervention areas and lack of coordination of funding (8). The state of HTA in the United States has been severely criticized, as “decentralized, fragmented, and duplicative,” with an “erratic commitment” by government to its funding (9).

Does the federal government spend the appropriate amount on the spectrum of health research? One view is that because of the escalating costs of scientific investigation the recent growth in funding, where it has occurred, has barely kept up with inflation (10). Yet, in a global context the priorities and funding levels for research in the United States are arguably extravagant. Less than 10% of the world’s medical research funds are used to address problems that are responsible for 90% of the world’s burden of disease (11,12). Furthermore, it has been argued that medical research has become increasingly inefficient and ineffective and that the current level of funding in developed countries is inflated and will “correct” downwards in the future, following a trajectory of a funding contraction in the wake of overexpansion (13).

More difficult than seeking to address Soderstrom’s question about the appropriateness of research funding levels is grappling with the observation that it sometimes doesn’t seem to matter anyway. Though the Quebec Neuroblastoma Screening Project (QNSP) was never funded as an HTA study (even if that is a context in which it can justifiably be acclaimed), it was an extraordinarily good societal investment for the U.S. taxpayer. The project prevented widespread implementation of an ineffective, harmful, and costly intervention in North America, and it contributed substantially to the discontinuation of a long-established neuroblastoma screening program in Japan. But HTA studies are seldom effective in discouraging access to undesirable technologies. In the real world, policy-making decisions on the allocation of health care resources rest on social, political, and ethical dimensions that often cannot wait for well-designed scientific evaluations to deliver their results (2,14,15). As for changing clinical attitudes, the congruent results of the QNSP and a similar well-designed and conducted German study (16) have still been rejected by some physicians (17). This phenomenon has been seen elsewhere, notably in the ongoing debates and controversies around the interpretation of breast cancer screening studies.

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But the outcome of the QNSP did have the desired result. Soderstrom demonstrates that the study averted unnecessary morbidity for literally thousands of children—and did so while returning a commendable yield on financial investment, even at seemingly very conservative sensitivity analyses. A disturbing question flows from this case study, though: What if screening had been an effective technology? If so, this study could, in retrospect, have been deemed an economic disaster. Conceivably, it might then have cost society many millions of dollars by delaying the prevention of morbidity and premature mortality through a useful and beneficial intervention. In that situation the justification for having done the study would have been purely philosophical. Neither lives nor dollars would have been saved by the research. But future policy, for both health systems and clinical interventions, would have been based on rigorous, unbiased information rather than speculation and anecdote. This is what makes medicine different from quackery.

What then can we do in our community or academic practices? Advocate for more research funding—yes. But we have other obligations, too. The World Medical Association’s Declaration of Helsinki states that “medical progress is based on research … the primary purpose [of which] is to improve prophylactic, diagnostic, and therapeutic procedures and the understanding of the etiology and pathogenesis of disease. Even the best proven prophylactic, diagnostic, and therapeutic methods must continuously be challenged through research for their effectiveness, efficiency, accessibility and quality” (18).

The medical community must embrace research. In the last 5 years the National Cancer Institute’s spending has increased by 45%. During that same period the proportion of eligible adult cancer patients entering clinical research studies has remained static at less than 3%! In June 2005 the NCI announced plans to revamp its clinical trial system (19), continuing to aid all physicians in encouraging participation in clinical trials. It behooves us to do our part—to actively participate in clinical and health services research and to take up results in our practice.

REFERENCES

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NOTE

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