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EDITORIAL

VALUING HEALTH TECHNOLOGIES AT NICE: RECOMMENDATIONS FOR IMPROVED INCORPORATION OF TREATMENT VALUE IN HTA

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1. INTRODUCTION

Pricing, reimbursements and coverage decisions for medical innovations and services are complex. In many countries, valuing medical technologies is the responsibility of national health-care systems that strive for distributive efficiency under fiscal constraints on medical spending. In the UK, the National Institute for Health and Clinical Excellence (NICE) is charged with the difficult task of assessing new and existing medical technologies, and making recommendations that guide NHS coverage decisions. These are responsibilities that NICE carries out rigorously and with the objective of achieving efficiency in the allocation of NHS resources. Nevertheless, the current process of health technology appraisal (HTA) has been criticized for failure to systematically incorporate important sources of value from new innovations. In maintaining NICE's current approach to HTA, the UK risks suboptimal patient outcomes and social welfare relative to what could be achieved if limited NHS resources were targeted toward those technologies with greatest social value.

Perhaps recognizing these limitations, NICE commissioned Professor Sir Ian Kennedy in January, 2009, to carry out a study of valuing innovation aimed at addressing the approach that should be adopted by NICE to ensure that innovation is properly taken into account when establishing the value of new health technologies; whether particular forms of value be considered more important than others; how should innovation in health technologies be defined and understanding the relationship between innovation and value. As part of this process, we were asked to present our views on what such new approaches may be and how they could be implemented in practice within the NICE regulatory framework. This work was presented to the Kennedy Commission on May 19, 2009. We discuss these approaches below, and expand on ways in which important value dimensions can be integrated within the current framework. While framed in response to the Kennedy study, these views are broadly applicable to other health-care systems charged with valuing innovation and achieving distributive efficiency.

2. DISTRIBUTIVE EFFICIENCY AND CURRENT HTA

HTA, as currently practiced, focuses on the incremental cost-effectiveness ratio (ICER). That is the incremental cost per quality-adjusted life year (QALY) gained by recipients of treatment. The QALY

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can be a useful single measure that integrates life-expectancy, the risk of death and heath state utility. However, QALYs fail to capture important sources of value to patients and to society, leading NICE to inaccurately measure the value of innovations.

To economists, the notion of distributive efficiency in health-care systems involves maximizing utility – not simply patient health – subject to resource constraints. Further, what matters is not only *patient* utility, but the utility of society as a whole, now and in the discounted future. From this perspective, the current approach to HTA fails to capture three broad areas of value. First, while NHS strives to rigorously estimate clinical benefits of technologies, benefits to patient *well-being* or utility are either missed or not systematically incorporated into the ICER. Second, innovative technologies may generate large benefits to patients that accrue in the future through new indications or spillovers from the development of altogether new technologies. These dynamic benefits are not captured in the ICER. Finally, the perspective of NICE is explicitly stated as that of NHS alone, but many times this narrow perspective fails to reflect the values and preferences of the entire citizenry served by NHS.

There are several specific sources of value that are missed by the current approach to HTA. In order to frame the discussion, we discuss a subset that is well supported by empirical evidence. The existence of empirical evidence not only substantiates their inclusion in this discussion, but also provides an empirical basis for measuring value and systematically incorporating these dimensions into an appraisal protocol. Motivated by this evidence, we then suggest how integrating these measures can be done in practice within the regulatory framework of NICE. Incorporating these sources of value into HTA with rigor and consistency paves the way for increased social welfare within the health care sector.[1]

3. SOURCES OF VALUE NOT SYSTEMATICALLY CAPTURED IN HTA

3.1. Innovativeness of health technologies

Considered narrowly, innovativeness represents substantial therapeutic advances over the existing technologies. However, over and beyond immediate clinical effectiveness, innovative health technologies may also have large dynamic benefits to society. These dynamic benefits accrue through the discovery of new uses of the technology. In the case of pharmaceuticals, evidence suggests that a substantial share of utilization occurs outside the indication for which a new molecular entity is initially approved (Berndt *et al.*, 2006). In the UK, dynamic benefits of this type are reflected in part by the acknowledgement of the new PPRS that new indications or evidence might be rewarded with higher approved prices. This recognition of potential 'spillovers' of innovative technologies could also be built into HTA methodologies.

Novel innovations also have the potential to generate future innovations. Consistent with this finding, studies have estimated that innovators capture only a small fraction of the social surplus generated by a novel technology through reimbursement; the vast majority of the social value of a technology accrues to patients from improved health (Jena and Philipson, 2007). Part of the social surplus that is not captured by innovators comes from surplus generated by the introduction of spillover technologies or future indications of the new technology, particularly those that are even more effective than the initial novel technology.

[1]We remain agnostic on how society should weight specific dimensions of value discussed in text. Weights should depend on society's preferences over specific components of value. Evidence cited in this article is not intended to support weighting recommendations (although in principle they could be used for this purpose); rather, it is used as empirical evidence supporting the relevance of specific sources of value neglected by the current approach to HTA.

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3.2. Disease severity

The current approach to technology appraisal does not adequately or consistently capture the value placed on treatments that benefit the very sick or terminally ill. An existing literature, from economics theory to empirical survey studies (Dolan *et al*., 2005; Becker *et al*., 2007; Dolan and Kahneman, 2008), suggests that incremental health gains are valued differently depending on the severity of the disease. This argument is consistent with a fundamental principle of demand theory: that an incremental increase in the consumption of a good is valued more by someone who has lower levels of that good than by someone with more. In the health context, diminishing marginal utility suggests that an incremental improvement in health provides a greater increase in utility to sicker patients.

Societal preferences provide an additional source of value for treatments of severe diseases. Patients' utility and society's valuation of health outcomes are distinct concepts that in principle generate different assignments of value (Nord, 1993; Nord *et al*., 2009). Recent work suggests that the first half of individual value scales is worth about two-thirds of the social value scale (Dolan *et al*., 2008). In essence, the tax base supporting NHS seems to value helping people in the tax base in great need, e.g. those in life-threatening circumstances. This is consistent with the recent decision by NICE to place greater value on therapies for the terminally ill (NICE, 2008). While similar in concept to the institute's recent life-extending end-of-life treatment criteria, disease severity broadens the focus in a logically consistent framework to include patients with significant suffering even if they are not yet at the end of life.

3.3. Unmet need

Numerous studies document society's preference for equity in health care. Specifically, evidence suggests that society has willingness to trade off efficiency (as defined narrowly over patients' health and perhaps measured by QALYs) for equity in the *opportunity* of patients to benefit from medical treatment (Ubel and Loewenstein, 1996). Notably, when the treatment option in survey studies does not extend to *every* patient who could benefit, preferences for equity are significantly less strong (Ubel *et al*., 2000). Thus, society's willingness to trade-off efficiency for equity exhibits an 'all or nothing' quality.

These preferences are consistent with placing greater value on technologies that treat diseases for which no therapy is previously available. The appeal to equity of treatment opportunities is also consistent with placing value on orphan drugs that treat rare diseases. These diseases affect only a few individuals, a fact which, considered alone, does not justify placing additional social value on therapies (McCabe *et al*., 2005). However, as a result of their low prevalence, these diseases are often associated with few to no treatment options (Lichtenberg and Waldfogel, 2003; Yin, 2008). Indeed, preferences for equity (in terms of availability of treatments) are invoked as justification for laws that subsidize orphan drug R&D in the US, Singapore, Japan and the European Union (House of Representatives Subcommittee Report, 1982; Drummond *et al*., 2007).

The case of unmet need makes salient the difference between maximizing the health of individuals and maximizing social welfare of NHS participants. When distributive efficiency is defined over patient health, unmet need is only relevant insofar as the health technology in question delivers a sufficiently high health-based ICER. When defined over the utility functions of individuals in society, the presence of equity in the utility functions of individuals justifies placing additional value on technologies that benefit patients who otherwise have no therapeutic options.

3.4. Reduced caregiver burden

Certain illnesses are also associated with intensive financial, time and emotional costs of caretaking. For example, care related to mental illnesses or illnesses with major physical disability often demand large financial and non-pecuniary commitments of family. These costs have the potential to affect the health, well-being and even life-expectancy of spouses (Zivin and Christakis, 2007; Elwert and Christakis, 2008;

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Christakis and Allison, 2009). And the financial and emotional demands of care-giving can lead to a reallocation of resources toward the sick away from investments in the development of children (Perry, 2008). Reductions in these external costs resulting from health improvements potentially embody a significant source of unmeasured benefits from health technologies treating some illnesses. These spillovers are likely to occur on members served by NHS and therefore affect NHS spending indirectly or directly.

3.5. Patient compliance and related benefits

A variety of benefits can be achieved through enhancing patient compliance. First, improved compliance and adherence to prescribed regimens may lead to greater efficacy and improved health outcomes. Technologies that are associated with more convenient delivery systems, more convenient location of administration, reduction in the frequency of dosage or reduction in regimen complexity have been shown to achieve improved compliance in pharmacological treatments for cardiovascular diseases (Iskedjian *et al*., 2002; Dickson and Plauschinat, 2008), psychiatric illnesses (Pfeiffer *et al*., 2008), HIV (Boyle *et al*., 2008) and diabetes (Barnett, 2006), among many others. Further, reductions in treatment complexity and frequency, as well as more convenient modes and location of administration are associated with reductions in patient inconvenience and decreases in financial and time costs of caregivers, costs that are not systematically captured in the ICER framework. For example, in a study comparing oral to intravenous antimicrobial therapy, researchers found equivalence in efficacy, suggesting large savings in medical costs and patient well-being (Winfried *et al*., 1999). Improved compliance can in principle be captured by the QALY framework, but in practice is often not.

4. IMPLEMENTATION IN EXISTING HTA PROCESSES

4.1. Transparency and consistency

In the current approach to HTA, NICE formally incorporates QALY-based ICER calculations into recommendation decisions. However, there is a large set of recommendations that do not systematically incorporate measures of cost-effectiveness; this is evidenced by the set of adoption decisions that are not fully explained by cost-effectiveness data (Jena and Philipson, 2009). Likewise, other factors, including sources of value discussed above, are considered in some cases but not others. Every NICE recommendation produces winners and losers in a zero-sum game of allocating funding from a fixed budget. Justified or not, losers in the appraisal process (among them patients as well as innovators) are left with the impression that NICE has in the past invoked broader sources of value for only those technologies it intends to recommend. Indeed, empirically, considerable residual variability surrounds NICE recommendation decisions, even controlling for the estimated ICER (Delvin and Parkin, 2004; Jena and Philipson, 2009).

A revised approach to HTA should incorporate broader sources of value, and should do so in a transparent and consistent way.[2] Transparency and consistency appeal to a basic social preference for procedural justice (Dolan *et al*., 2007). To the losers of the zero-sum game, consolation comes only from a sense of fairness provided by a consistent and dispassionate evaluation process. This sense of fairness is a sentiment more easily achieved through transparency than through faith. Transparency and consistency of the appraisal process also has important financing benefits. When the criteria for positive recommendations are known, pharmaceutical, biotechnology and device manufacturers are able to

[2]In reports submitted to the Kennedy commission, patient groups and pharmaceutical firms commonly expressed a demand for greater transparency of NICE appraisals. Indeed, *Recommendation ♯1* of the Kennedy Report urges NICE to more actively develop strategies to improve transparency of the HTA process (Kennedy, 2009). The Kennedy Report and external submissions are available at: http://www.nice.org.uk/aboutnice/howwework/researchanddevelopment/kennedystudy.jsp.

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allocate capital across investment opportunities more efficiently. Clarity of the HTA process also allows innovators to obtain and to present the relevant evidence to NICE, which supports an accurate and time-efficient technology appraisal.

4.2. Alternative process to the ICER

We propose alternative approaches to technology appraisal, and demonstrate ways in which important value dimensions can be integrated within the current framework and more explicitly evaluate products on criteria that are neglected, or are considered but not in any systematic way.

4.3. Alternative process: an integrated ICER

Sources of value neglected by the current approach to HTA could be integrated directly into the ICER. Doing so would allow NICE to maintain the long-established threshold of £20 000. An integrated ICER could, for example, could incorporate diminishing marginal utility in health status. Social preference-based measures of quality (the 'Q' in QALY) can, if applied rigorously and consistently, can capture the greater value society places on treatments for severe diseases. Providing that adequate clinical and survey evidence can be obtained, the same ICER adjustment can capture both clinical effectiveness and social value judgments over any number of issues related to distributional and procedural justice (Dolan *et al*., 1999, 2003; Dolan and Tsuchiya, 2006). In this way, specific issues of disease severity, equity and unmet need, potential for innovative spillovers, or offsetting medical cost reductions and external benefits to caregivers can be incorporated in the existing ICER framework.

4.4. Alternative process: a two-part protocol

The demand for empirical evidence to support a rigorous and broader appraisal process, and the burden on manufacturers of providing this evidence, could also be balanced in a two-part protocol. In a two-part protocol, technologies are scored along key dimensions of value neglected by the current HTA. Scores would be given for dimensions of value that are publicly and transparently delineated by NICE (innovativeness, unmet need, disease severity, etc.). A composite and qualitative 'value score' would then be constructed. The composite value score would be considered jointly with the traditional ICER, which would be calculated in second and distinct step.

This two-part approach is particularly useful when the evidence on value outside of ICER is more qualitative initially; or when estimating value for inclusion in an integrated ICER, while feasible in principle, would require significant effort, time delays and in some cases, the development of new methodological tools. The adoption of new reimbursement policies is likely to stimulate the development of new measurement tools, just as it did in the case of measuring the ICER itself. The use of multiple scores to determine coverage and effective price levels has precedence elsewhere. For example, the French SMR and ASMR scoring system determines drug reimbursement and prices based in part on scores for both innovativeness and clinical effectiveness.

In the final appraisal, the ICER and a composite value score would be considered jointly and systematically. For example, the decision framework could explicitly assign higher ICER recommendation thresholds to technologies with higher value scores. This is in principle similar to the Multi-Criteria Decision Analysis (MCDA) system suggested by Jack Dowie.[3] This would ensure that patients will have access to technologies that generate extensive value to society not captured by the traditional ICER measure.

Alternative approaches to jointly considering a composite value score and ICER are also possible. For instance, *conditional* on an ICER score, more generous payment schemes could be assigned to

[3]http://knol.google.com/k/the-future-of-hta-is-mcda#.

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innovations with higher composite value scores so as to reflect the higher value of the technology to society. This has the advantage of a achieving a continuous level of coverage, avoiding coarse binary coverage recommendations inherent in the current framework, and in proposed MCDA-based approaches. 'Patient access' schemes, such as those established through the recent revision to the PPRS, provide a precedent for more general approaches to relating appraisal decisions, or reimbursement, to social value in a more continuous manner.

The advantages of two-part scoring system become clear when measuring, for example, the value of a technology's *innovativeness*. Exceptional effectiveness over and beyond existing technologies – one narrow but measurable definition of innovativeness – can be quantified by clinical effectiveness. Broader measures of innovativeness may not as easily be integrated into the ICER. For example, with regard to dynamic benefits of novel technologies, neither NICE nor manufacturers can identify at the time of appraisal which technology will ultimately generate spillover benefits of the type discussed above. Hence, while many sources of value can be integrated into the ICER, 'units of innovativeness' may not conform to the ICER, necessitating a separate innovation score to accompany an integrated ICER in the final appraisal. In some cases, measures of novelty – e.g. mechanistic novelty, etc. – can serve as an initial proxy of innovativeness. Ordinal scoring systems that account for innovativeness exist in the French system, and in the Priority Review given to drugs during the US Food and Drug Administration approval process (FDA, 2007). The precedent of the French and US systems suggests that an auxiliary review process for innovation is both feasible and sensible.

5. CONCLUSION

In summary, we believe that current ICER-based methods of valuing new health-care treatments do not adequately capture some dimensions of the full costs and benefits of those treatments to the tax base citizenry supporting the NHS. However, these additional benefits and costs of new technologies can be incorporated into the measurement procedures and existing regulatory framework of NICE.

Any framework that incorporates society's value for various health outcomes will require elicitation of social preferences over equity and distributional efficiency. Doing so will require that a number of technical survey issues need to be addressed before component weights can be implemented (Wailoo *et al*., 2009). Likewise, incorporating additional dimensions of value into HTA will require manufacturers to provide evidence supporting the benefits of their health technologies. Improving distributional efficiency in medical spending is worth this effort. And in the long run, incorporating these dimensions of value in a transparent framework will encourage manufacturers to obtain new data on wider social benefits; and more generally, to develop innovative technologies with the greatest value to society.

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