National Institute for Health and Clinical Excellence (NICE):

HOW DOES IT WORK AND WHAT ARE THE IMPLICATIONS FOR THE U.S.?

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Executive Summary

Overview of NICE

The growing emphasis on evidence-based decision-making in health care, especially regarding health technologies, has generated notable debate and discussion in the U.S. around establishing a more formalized process or system for collecting comparative effectiveness information. The National Institute for Health and Clinical Excellence (NICE) in the U.K. is the most discussed and debated system currently in operation. NICE represents the most sophisticated national attempt to systematically review the value, or relative costs and benefits, of various treatments. While NICE has been commended for various aspects of its review and appraisal processes, from the inclusion of stakeholders to rigorous use of evidence, it has not operated without controversy. Concerns abound regarding the methods employed during the appraisal and review process, the role and utility of cost-effectiveness evidence in decision-making, and the resulting impact on outcomes and access to health care.

In broad terms, NICE serves as an “arms-length” organization that provides national guidance on the promotion of good health and the prevention and treatment of various health conditions. Specifically, NICE’s remit is to consider both clinical and cost-effectiveness in developing its guidance. When it was established and during the majority of its existence, NICE has produced three types of guidance, including technology appraisals, clinical guidelines, and interventional procedures. In mid-2005, NICE also assumed the responsibilities of the Health Development Agency (HDA), which provided the Institute with authority to develop guidance on public health interventions or programmes.

Regarding its role in health care decision-making, NICE guidance essentially serves a quasi-law function in the National Health Service (NHS) and broader health care system. Beginning in January 2005, technology appraisals are supported by mandate, in that the NHS in England and Wales are each now legally obligated to provide funding for medicines and treatments recommended by NICE. Specifically, if NICE guidance supports that a particular technology be made available by the NHS to a certain patient group(s), then associated health care organizations are obligated to implement such recommendations. Moreover, NHS organizations are required to do so within three months of the date the guidance is issued.

Topic Selection and Prioritization

To prioritize assessments and initiate the guideline development process, NICE receives suggested topics from a number of sources. In general, the Department of Health (DH) commissions NICE to develop clinical guidelines, guidance on public health, and technology appraisals, while topics for the interventional procedures program are submitted directly to NICE, usually by clinicians. However, topics
for potential NICE guidance are also derived from health professionals; patients; the general public; manufacturers; and, within NICE itself. NICE compiles and maintains a list of all submitted topics, which aids in ensuring transparency.

**Guidance Development**

Guidance development entails a number of key processes, from topic selection to systematic review of evidence and consultation on draft guidance. Several aspects inherent to NICE’s procedures lend themselves to an effective deliberative process, many of which are focused on ensuring the highest degree of transparency and the participation of a wide range of stakeholders. NICE’s engagement of a broad representation of stakeholders, from multiple sectors and disciplines, serves to introduce a variety of perspectives into the appraisal and decision-making process. This is particularly helpful when reaching consensus on conflicting evidence or recommendations; making such judgments typically requires knowledge of the scientific literature, realities of clinical practice, and underlying social values. Moreover, as there is a paucity of evidence about patient treatment preferences and viewpoints on issues such as equity and fairness in health care, it is important to involve a variety of stakeholders in the process to elicit such perspectives. At the implementation stage, a high level of stakeholder involvement increases public and professional ownership in the guidance, which enhances the likelihood that it will effectively guide decision-making and clinical practice.

**Guidance Dissemination and Implementation**

NICE employs different mechanisms to disseminate its guidance, from publication on its website to distribution to local decision-makers. Adequate and timely communication and implementation is crucial, as guidance is only effective if it is indeed used by decision-makers to make health policy and/or provision decisions. This is especially true in the case of NICE, whereby its guidance carries significant weight, in terms of NHS funding of and access to health technologies. However, existing evidence suggests that the uptake of NICE guidance is often slow, patchy, and without adequate incentives for implementation. There are several factors that are considered to impact whether or not NICE guidance is fully implemented, including local political drivers; lack of provider support; deficient knowledge and understanding of the assessment process, media and patient group pressure; limited mechanisms for accountability; and poor financial planning of local decision bodies.

**Relevance to the U.S.**

In the U.K., NICE is constituted as a Special Health Authority within the NHS. Therefore, it has an ‘arms-length’ relationship with government, although its funding comes from the DH. Experience from the U.K. and elsewhere suggests that an appearance of independence is important for HTA agencies or entities, as the findings of HTA reports are often controversial. A survey of general practitioners in the U.K. showed that NICE was perceived as being independent from industry,
but not independent from government. Also, when NICE guidance is considered by the media, the Institute is normally referred to as ‘the government’s health watchdog’ or, occasionally, as ‘the NHS’s rationing body’.

In the U.S., the primary decision affecting the governance, funding, and organization of any HTA entity would be where it is located. That is, should it be a new Federal agency, part of an existing agency, or outside of government? Regardless of the governance arrangement(s), any public HTA entity will likely end up informing decisions of a variety of payers, even if the entity is only charged with informing the decisions of the Federal Government. Indeed, currently the HTAs generated for the Medicare Coverage Advisory Committee (MCAC) decisions are posted on the Centers for Medicare and Medicaid Services (CMS) website and are available for consultation by private health plans.

**Remit for HTA Agency**

On the international level, NICE is probably in a minority among HTA entities in having such a clear remit to consider cost-effectiveness. The recent debate in the U.S. has been conducted using the term ‘comparative effectiveness’. For many commentators, the study of comparative effectiveness would involve consideration of clinical outcomes only, usually through the conduct of clinical trials, comparing relevant technologies in a real life (i.e., routine practice) setting. On the other hand, some commentators acknowledge that an assessment of comparative effectiveness could also consider costs.

**Scope of Analyses**

As the debate about comparative effectiveness progresses in the U.S., the breadth, or restriction, in the scope of the required analyses will be a critical issue. At one end of the spectrum, the HTA effort could focus solely on the funding and conduct of clinical trials to compare alternative technologies. Unlike most of the trials currently funded by industry (e.g., Phase III for drugs), these trials are likely to compare two or more widely-used therapies, enroll large numbers of patients, and have long-term follow-up. They are also likely to be quite costly, so their number will be limited, even with the budgets currently being proposed for the comparative effectiveness initiative, which at present range up to $4-6 billion a year. The current funding level for NICE is more modest (around $50 million), but crucially the Institute does not commission primary research, such as comparative clinical trials. Rather, it relies on systematic reviews of the existing literature, in addition to economic modeling. The main reason for this is the emphasis on the timeliness of the assessment. That is, since a decision has to be made (on the appropriate use of a health technology), the principal need is to develop the best possible guidance given currently available data.

If, in the U.S., the emphasis in ‘comparative effectiveness’ assessment were to be on large, long-term, controlled trials, this would have to be developed under a scheme similar to ‘coverage with evidence development’, since the technologies would have to be approved for funding in order to allow such trials to take place.
Under ‘coverage with evidence development’, funding for the technology to be studied is contingent upon participation in the clinical trials. Therefore, more discussion of the design of such schemes, including study requirements, funding and risk-sharing arrangements (if any) is urgently required.

**SETTING PRIORITIES**
Priorities for assessment by NICE are set by the government in the U.K., according to published criteria. If any HTA entity in the U.S. were also servicing the decision-making needs of the Federal Government, a similar process could apply. For example, the topics could relate to those technologies for which coverage decisions are required. If an HTA entity in the U.S. were seeking to be relevant to a wider range of health care decision-makers, the nature of the process for setting priorities is less clear, although many private health plans also cover Medicare enrollees.

**ASSESSMENTS VS. APPRAISALS**
NICE makes a clear distinction between assessments, where only the technical analysis is undertaken, and appraisals, where the evidence is evaluated and the decisions made. The Institute relies heavily on expert committees in its decision-making processes (e.g., Appraisal Committee, Guideline Review Panels) and this adds somewhat to the appearance of independence. The experts are a mixture of academics (across several disciplines including medicine, statistics, and economics), NHS decision-makers, and patient representatives. There is no reason why a similar approach should not work for an HTA entity in the U.S., although it may be more of a challenge to secure adequate representation from the various decision-making groups.

A more fundamental issue in the U.S. context is whether an HTA entity would have a decision-making role at all, given the diverse nature of the health care system. It is possible, indeed more likely, that the responsibilities of any entity in the U.S. would cease at the assessment stage. Assessments could then be made publicly available, for the various payers to use (or not use) as they see fit.

**Summary and Conclusions**
Although the increased use of HTA in the U.S. may lead to a more cost-effective use of health care resources, it will also increase the burden on industry to produce data. In addition, to the extent that HTA is linked to decisions about the pricing and reimbursement of medicines, it will lead to greater controls on the prescribing and use of drugs. These wider considerations are beyond the scope of this project. Although there are considerable differences between the health care systems in the U.K. and the U.S., several important lessons can be drawn from the U.K. experience with the NICE model.

**1. INDEPENDENCE OF HTA ENTITY**
The governance and organization of any HTA entity is critically dependent on whether its role is to serve the decision-making needs of one major payer or
the needs of many decision-makers in a diverse system. However, whatever its governance and organization, it is important that any HTA entity is as independent as possible.

2. **Consider All Health Technologies**

In order to make the broadest impact, an HTA entity should consider all health technologies (not just drugs). Priorities for topics need to be set in an explicit manner and assessments should be rigorous and transparent, conducted in accordance with a clear set of methods guidelines.

3. **Involve All Major Stakeholders**

Any HTA entity should make strenuous efforts to involve major stakeholders in the development of methods guidance, the scoping of individual assessments and in commenting on the results of studies. Early and consistent involvement of technology manufacturers is particularly important, as they play a major role in conducting the studies on which the assessments are based. Ideally, assessments should be carried out by independent research groups, under the general direction of the HTA entity, and should be as transparent as is possible.

4. **Broaden Types of Evidence and Improve Methods Synthesis**

There needs to be a debate in the U.S. about the pros and cons of focusing the HTA effort on the conduct of additional large, long-term randomized controlled trials, versus investing more effort in improving the methods of evidence synthesis using available data. There also needs to be discussion of how the production of more evidence on health technologies is linked to decision-making, perhaps through a system of ‘conditional reimbursement’.

5. **Avoid Narrow Focus on Clinical Outcomes**

More discussion is required about the ways of incorporating economic factors in assessments, in order to provide relevant information for decision-makers in the U.S. context. A focus on clinical outcomes alone is inappropriate as it may exclude important advantages and disadvantages of health technologies, such as impacts on quality of life and savings in other healthcare resources. On the other hand, calculation of a single (incremental) cost-effectiveness ratio may not be very helpful, given the great diversity across the U.S. in health care budgets, practice patterns, and cost levels.

6. **Provide Evidence Interpretation, Not Guidance**

Finally, the most appropriate focus for an HTA entity in the U.S. is on undertaking high quality assessments (i.e., interpretation of the evidence), rather than appraisals (i.e., the production of guidance for decision-makers). The objective should be to produce high quality assessments that will enable various decision-makers to undertake an appraisal from their own perspective.