

A Note on Objectives and Methods

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Chapter 2

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2.1 Objectives

The primary objective of the present report is to analyze the real-life performance and robustness of the process for technology appraisals and the methods for health technology assessments adopted by the National Institute for Health and Clinical Excellence (NICE). This report is focused on the application of these processes and methods, as put to practical use in a particularly challenging field of economic analysis, the evaluation of treatment strategies for attention-deficit/hyperactivity disorder (ADHD).

Thus the empirical part of this report will provide a critique of an application of economic evaluation methods on behalf of NICE, i.e., it will “appraise the appraisers” (Blades et al., 1987). This will lay the foundation for a broader discussion of implications for international health care policy-makers looking at NICE as a potential role model.

Occasionally, examples of the author’s own work in this field will be used to illustrate context and relevance (e.g., European data on the administrative prevalence of ADHD and budgetary impact projections; cf. Chapter 1, *Introduction*), as well as to present recent cost-effectiveness evaluations directly related to the NICE appraisal (e.g., European adaptation of an economic model developed on behalf of the Canadian Coordinating Office for Health Technology Assessments, CCOHTA, and European cost-effectiveness analyses based on the landmark NIMH MTA Study, cf. Chapter 6, *Discussion*), and the methods underlying these data will be briefly delineated in *Boxes* (see pages 11, 17, 127, 133, and 176).

2.2 Accountability for Reasonableness

The analysis of the NICE processes will be guided by a framework developed by Norman Daniels and James Sabin who have argued that the legitimacy of controversial limit-setting decisions in public health care systems hinges on a fair institutional decision process (Daniels and Sabin, 1997, 1998, 2002). In order to narrow the scope of controversy, they have proposed principles of “accountability for reasonableness”

(A4R), which “fair-minded people” should accept based on the idea that there exists a core set of reasons – that all center on fairness – on which there will be no disagreement.

A key element of fair process under A4R (Table 2.1) involves transparency about the decision making, including the grounds for decisions (the *publicity* condition, opening decisions and their rationales for scrutiny by all affected, not just the members of the decision-making group). Second, the *relevance* condition imposes an important constraint on arguments, because arguments are required to rest on scientific evidence – though not necessarily a specific kind of evidence – and to appeal to the notion of “fair equality of opportunity.” Although Daniels and Sabin acknowledge that stakeholder participation may improve deliberation about complicated matters, they believe it is neither a necessary nor a sufficient condition of A4R. However, they advocate an *appeals* component as an institutional mechanism to engage a broader segment of society in the process. This appeals process should provide those affected by a decision an opportunity to reopen deliberation, and offer decision-makers an option to revise funding decisions in light of further arguments. Fourth, *enforcement* entails voluntary or statutory regulation to make sure the first three conditions are met. It has been argued that proper enforcement of the decisions will also ensure that reasoning is decisive in priority-setting and not merely a theoretical exercise (Hasman and Holm, 2005).

Using A4R as a benchmark guiding the review of NICE processes turns out to be a timely endeavor: it was, to the knowledge of this author, not before August 2005 that Sir Michael Rawlins, Chairman of the Board of NICE, and Andrew

Table 2.1 Accountability for reasonableness framework

Conditions for fair priority-setting processes according to the “Accountability for Reasonableness” (A4R) framework developed by Daniels and Sabin (1997, 1998, 2002): Descriptors taken from Daniels (2001) and Mitton and Donaldson (2004)

Condition	Description
Publicity	Decisions regarding coverage of new technologies (and other limit-setting decisions) and their rationales must be publicly accessible.
Relevance	These rationales must rest on evidence, reasons and principles that fair-minded parties (managers, clinicians, patients and consumers in general) can agree are relevant to deciding how to meet the diverse needs of a covered population under reasonable resource constraints.
Revisions and appeal	There is a mechanism for challenge and dispute resolution regarding the limit-setting decisions, including the opportunity for revising decisions in light of further evidence or arguments.
Enforcement	There is either voluntary or public regulation of the process to ensure that the first three conditions are met.

Dillon, Chief Executive of NICE, explicitly committed NICE to submit itself to the principles of A4R: “NICE has adopted the principles of procedural justice – ‘accountability for reasonableness’ – as espoused by Daniels and Sabin (2002)” (Rawlins and Dillon, 2005b).

2.3 Methods

A qualitative study was done of NICE Technology Appraisal No. 98, “Methylphenidate, atomoxetine and dexamfetamine for attention deficit hyperactivity disorder (ADHD) in children and adolescents (Review of Technology Appraisal 13),” published in March 2006. The case analysis had descriptive, explorative, and explanatory elements. The analysis was primarily concerned with the real-life application of NICE processes and focused on the Technology Assessment Report (King et al., 2004b), since this document “is used as the basis of the appraisal” (NICE, 2004b). The resulting critique shall be presented and, hopefully, will be understood in a spirit of scientific inquiry.

First, the initial phase of the study consisted of defining a theoretical framework for the study. This included description of NICE technology appraisal processes, which fell within a period of substantial upgrade and definition of the so-called “reference case” analysis by NICE (see below, Chapter 3, *NICE Appraisal Process*). During this phase, a thematic framework was defined, comprising use of the A4R concept as a process benchmark, an in-depth critique of the technology Assessment Report underlying the appraisal, as well as a review of the clinical and economic literature on attention-deficit/hyperactivity disorder in order to incorporate the complex interrelated issues involved in this technology appraisal (cf. Chapter 1, *Introduction*).

The second phase of the study comprised data collection on a number of closely related strategies. (1) From May 2004 to publication of guidance in March 2006, the NICE website (www.nice.org.uk) was visited at intervals of less than one month each and checked for newly posted information and documents (including meeting minutes and announcements) on (a) the technology appraisal process and related methods, (b) clinical guideline development, (c) deliberations of the NICE Citizens’ Council, and (d) ADHD. (2) Scientific articles cited in these documents were obtained for analysis. (3) Independent literature searches (using the PubMed and EBSCO databases as well as Google Scholar) were conducted for articles on ADHD diagnosis, treatment, compliance, cost, and cost-effectiveness, and were (4) complemented by a search for relevant abstracts presented at international meetings in the fields of clinical psychiatry, child and adolescent psychiatry, pediatrics, health economics, and pharmacoeconomics. All searches for literature fully covered the technology assessment period (from June to December 2004; cf. Table 3.2, and Chapter 4, *NICE Appraisal of ADHD Treatments*). After May 2005, no more systematic searches for scientific literature were conducted, and new papers were added to the database in an opportunistic manner only. However, searches for full economic evaluations comparing at least two treatment options for ADHD were

updated in December 2006. Collected documents were indexed using categories including study type, product tested, and subject matter (e.g., treatment compliance) for further analysis and interpretation.

All key steps of the ADHD appraisal process were identified and compared with NICE process descriptions (NICE, 2004b,c). The Assessment Report (King et al., 2004b) was subjected to a critical appraisal by this author, which included an examination of design choices and justifications provided by the Assessment Group for internal and external consistency. Unless otherwise specified, citations in the following sections will refer to the Assessment Report (AR).

2.4 Limitations

The critique and discussion presented here should not be interpreted as an alternative health technology assessment of ADHD treatments. Any attempt to provide an independent systematic review would clearly exceed the limits of the present study, which is primarily interested in exposing strengths and weaknesses of the NICE process, with a view towards policy implications. On occasion, an alternative interpretation of data may be offered; however, this should be understood as a means to reveal the potential relevance of any pertinent gaps of the NICE assessment, and does not imply definite conclusions.

NICE was criticized by some observers for not paying enough attention to drug safety (Fletcher, 2000). The present analysis of the case of NICE Technology Appraisal No. 98 did confirm a strong emphasis on effectiveness and cost-effectiveness, but did not identify obvious shortcomings or substantial gaps with respect to drug safety, which would have had an impact on the economic evaluation. Correspondingly, the following critique will not provide a detailed review of safety considerations. Readers interested in this aspect of ADHD pharmacotherapy may wish to consult one of the recently published reviews of this subject, such as the papers by Wolraich et al. (2007), Pliszka (2007), Gibson et al. (2006), and Himpel et al. (2005).

As emphasized earlier in the *Introduction*, qualitative research cannot substitute for quantitative work; it is simply a complement allowing to “reach the parts other methods cannot reach” (Pope and Mays, 1995). On its own, empirical work based on a case study of one technology appraisal certainly cannot justify inductive inferences on more than 100 appraisals completed by NICE. It may, however, in a truly Popperian spirit, falsify certain unjustified assumptions and exaggerated expectations concerning the robustness of the NICE model. Then, any anomalies identified might generate hypotheses, which in turn could contribute to further improvement of technology appraisal processes.

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