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Drug reimbursement recommendations by the National Institute for Health and Clinical Excellence: Have they impacted the National Health Service budget?

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ABSTRACT

Objective: Determine whether reimbursement restrictions recommended by the National Institute for Health and Clinical Excellence (NICE) have impacted the United Kingdom (UK) National Health Service (NHS) budget.
Methods: Data were abstracted from NICE guidance documents and costing statements through March 2011. Estimated maximum and adjusted potential budget impact (PBI) on the NHS was derived using estimates of the UK marketing-approved population and the annual cost for the new drug. Descriptive and logistic analyses were used to estimate the correlation between the degree of restrictions on reimbursement recommended by NICE for each new drug indication and the PBI controlling for clinical effectiveness and cost-effectiveness.
Results: PBI was significantly correlated with the degree of reimbursement restrictions. In descriptive analysis, the adjusted PBI for drugs that were recommended without restrictions was £20.3 million (SD = 22.2) compared with £49.8 million (SD = 90.8) for those recommended with restrictions and £71.1 million (SE = 99.9) for those not recommended. In logistic analysis, the odds ratio for less restrictive reimbursement was 0.848 (95% CI, 0.762–0.945) for each £20 million increase in the adjusted PBI. Results were similar using the maximum PBI.
Conclusions: After controlling for clinical effectiveness and cost-effectiveness, the degree of reimbursement restriction recommended by NICE remains significantly correlated with the PBI, despite that fact that the NICE decision process does not consider budget impact. This correlation might be due to NICE consideration of effectiveness and cost-effectiveness for subgroups of the approved population.

1. Introduction

Since 1999, the National Institute for Health and Clinical Excellence (NICE) in the United Kingdom (UK) has made recommendations about access to selected new drugs that have been approved for marketing within their jurisdiction. Drugs are selected for review based on the burden of the disease, actual or potential resource impact to the National Health Service (NHS) or public sector, or

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inappropriate variation in use of a drug within the country [1]. The review process used by NICE includes a thorough review and discussion of the clinical effectiveness of the drug and its cost-effectiveness in different positions in the treatment pathway and/or in different subgroups covered by the UK marketing indication.

Manufacturer's submissions for the drugs selected by NICE for review are required to include estimates of the clinical and cost-effectiveness of the new drug. To ensure that these submissions provide appropriate information, NICE has provided guidelines on how these estimates are to be made, including the estimation framework, data sources for input parameter values, and outcomes presented [2,3]. Independent submissions or reviews of the manufacturers' submissions are funded by NICE and used to supplement the evidence provided by the manufacturer.

Only after its recommendation has been finalized does NICE produce a "costing statement" for the population that it has recommended for reimbursement. This "costing statement" is developed as a guide to implementation of the recommendation. Nevertheless, NICE guidelines for submissions from the manufacturers include a request for the manufacturers' estimates of the size of the population for which marketing approval has been granted, as well as the annual costs (or cost per course) of the new drug regimen including costs for administration and adverse events, estimated market share, and estimates of offsetting costs savings for the subsequent evaluation of the budget impact of the new drug [3]. Values from the manufacturers' submissions are frequently used to develop the budget impact estimates in the NICE "costing statement."

Three previous studies have presented quantitative analyses of reimbursement recommendations by NICE. A descriptive analysis comparing reimbursement recommendations by NICE, the Common Drug Review (CDR) in Canada, and the Pharmaceutical Benefits Advisory Committee (PBAC) in Australia showed, for all three HTA agencies, a negative relationship between the cost per QALY ratio and the probability of a positive recommendation [4]. A binary choice analysis of the recommendation to accept or reject a technology was performed by Devlin and Parkin [5] to explore the question as to whether NICE had a cost-effectiveness threshold and what other factors influenced its decisions using data from NICE appraisals through May 2002. They concluded that cost-effectiveness, uncertainty of the cost-effectiveness evidence, and the number of people affected by the disease were key correlates with the NICE decision. Although impact on the NHS budget was considered in this article, it was not included in the final estimates. A second article by the same team [6], using data from appraisals through December 2003, presented the results of a multinomial logit analysis for three recommendation categories: unrestrictive, restrictive, and not recommended. A potential budget impact (PBI) variable, defined as the UK marketing population multiplied by the annual cost or cost per course, was included in multivariate analyses but did not have a statistically significant effect on the recommendations. However, the authors found that interventions for which restrictions were recommended had a higher PBI than those that were recommended without restrictions.

The current study extends the analyses conducted by Dakin et al. [6] for NICE to include more recent data, through March 2011, and to use a hypothetical adjusted measure of PBI in addition to the maximum PBI. The correlation of the two measures of PBI with the recommended level of restrictions on reimbursement for new drug indications was estimated using descriptive and multivariate logistic analyses. The multivariate logistic analyses included as independent variables estimates of the clinical effectiveness, cost-effectiveness ratio, and the PBI, as well as other variables that have previously been shown to be associated with the probability of reimbursement in the UK [5,6].

2. Methods

2.1. Description of NICE database

A data file of recommendations by NICE was created by abstracting data from guidance documents available on the NICE Web site sorted by publication date (http://guidance.nice.org.uk//date). We reviewed all the technology appraisals on the NICE Web site between January 2001 and March 2011 that were for drug treatment interventions and not listed as terminated. We did not review technology appraisals that were for other types of health care interventions. There were 137 appraisals for drug treatment interventions. Of these, data from 40 technology appraisal documents were not abstracted for the following reasons: multiple products and indications were assessed but drug-specific information was not provided for all the abstracted variables ($n = 15$), information was not presented on the UK marketing-approved population size ($n = 4$), technology appraisal guidance was replaced with a more recent version ($n = 20$), and product was no longer available ($n = 1$). Those technology appraisals that had been replaced were not included, because the goal for the analysis was to represent the most recent NICE recommendations for any product.

In the data file, a unique identification number was created for each product and its indication. If a product had multiple submissions that included different indications, a new unique identification number was created for each combination of product and indication. If the guidance for a product and indication was later reviewed and replaced, only the latest guidance was abstracted, to more closely reflect NICE's current decision making. If multiple products were reviewed in the same technology appraisal and product-specific information was not available, the guidance was not included. The abstracted variables included those that have been shown to be associated with NICE's or PBAC's reimbursement decisions in previous studies [4–9] (see Appendix Table 1 for a detailed listing of all abstracted and derived variables). To ensure consistency in data abstraction, all data abstraction was performed by one researcher. The abstracted data were then subjected to quality checking by three other researchers to ensure accuracy and assess the credibility and consistency of judgments made in the initial abstraction.

The NICE recommendation was the outcome variable in this study. Three categories of outcomes were