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POLICIES

The role of health technology assessment in coverage decisions on newborn screening

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Objectives: The role and impact of health technology assessment (HTA) in health policy has been widely discussed. Researchers have started to analyze how decisions on coverage of new technologies are made. Although the involvement of HTA may be an indicator of a well established decision process, this hypothesis requires validation. Also, it is not known whether HTA involvement is associated with other characteristics of decision making like participation or transparency. The primary objective of this study was to develop and test statements on the association between the publication of an HTA and coverage decision making for newborn screening tests in European Union countries.
Methods: Five statements were defined on the relative role of HTA during the steps of decision processes: trigger, participation, publication, assessment, and appraisal. For this purpose, data on twenty-two decision processes in the area of newborn screening across Europe were analyzed, defined as a coverage decision for a given disorder in a specific country. Decision processes were compared by whether the decision was accompanied by the publication of an HTA report. To test differences, nonparametric statistical tests were used.

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Results: The decision steps of trigger, participation and publication differed between the HTA and the non-HTA groups. No clear association between HTA and assessment methods in coverage decision making was identified.
Conclusions: It appeared that there is an association between HTA and coverage decision processes that are more explicit, inclusive, and transparent. It is unclear whether HTA is associated with formal evidence reviews and economic evaluations.

Keywords: Neonatal screening, Decision-making processes, Reimbursement, Health technology assessment, Fourth hurdle

Health technology assessment (HTA) should facilitate systematic and transparent assessment and appraisal of decision making for coverage of health technologies (28). A good decision process involves not only the best available scientific evidence but also a prudent choice of evaluation criteria and a deliberative decision-making procedure that is participative and transparent. Processes should focus on technologies that are likely to provide high value. Not all decision-making processes use formal HTAs. It is fair to ask whether the involvement of HTA in health policy making contributes to fair and legitimate decision processes and structured and transparent decision making. Do the steps of decision processes differ depending on whether an HTA exists when a coverage decision is made?

Research has previously tried to assess the role and impact of HTA and related programs on health policy processes (8;11). Other health policy research studies have assessed the criteria used for appraisal, the decision outcome and assessment methods (29). None of these studies explicitly examined the interdependence between HTA and coverage decision across countries.

The aim of this article was to explore the association between HTA and decision-making processes for newborn screening (NBS) in European countries and generate a set of hypotheses. NBS includes several promising technologies that are relevant to third party payers. In particular, tandem mass spectrometry (MS/MS) allows screening of multiple metabolic disorders in one step. The results of this exploratory study may shed light on how HTA contributes to coverage decisions.

METHODS

To generate hypotheses on the association between HTA and coverage decision making, we performed a literature search in the following databases: MEDLINE, EMBASE, and Web of Science. The purpose was to identify all studies that have explicitly addressed the role of HTA in coverage decision making, also by analyzing past coverage decisions. We searched titles and abstracts published after 1987 for references using a combination of the terms "health technology assessment", "coverage", "decision", and "influence" including synonyms which was last updated April 7, 2011. No systematic abstraction of data and appraisal of articles was performed as this step aimed to retrieve qualitative information to generate statements on the role of HTA.

For structuring of the steps of decision processes, several conceptual frameworks have been stated (5;14;23). Rogowski et al. describe the stylized elements of a decision process from the point where a technology enters a healthcare market to its diffusion into routine use (23). This structure captures both process and appraisal criteria. The following steps appeared relevant: *trigger* of a specific decision process; stakeholder *participation*; *publication* of decision and related documents during or after the decision process; *assessment* by scientific methods like systematic literature review; and *appraisal* of whether or not the technology should be funded in the light of the available evidence along with further ethical or political criteria. A structured scheme including indicators and ordinal rankings for each step of the framework has been validated with a small number of decisions and expert discussions (7).

From studies identified in the literature review, key principles of HTA (27), and the steps of the framework by Rogowski et al. (23), a group of statements was formulated that describe the association between HTA and steps of NBS decision making. As the publication of HTA and decision making mutually influence each other, no statement on the causality of associations was made. Table 1 provides an overview of the statements that were made for the case where an HTA was published.

A survey of recent NBS decisions in the European Union was conducted between August and December 2009. Experts were recruited through a scientific society (International Society of Neonatal Screening) and were either affiliated to NBS providers or third party payers of which forty-three completed the questionnaire (response rate 70 percent). Respondents were asked about decisions made since 2005 on screening for medium-chain acyl-CoA dehydrogenase deficiency (MCAD), cystic fibrosis, or congenital adrenal hyperplasia. If none of the conditions mentioned were identified, the survey also allowed for the analysis of other case studies. In this context, a case study was defined as screening of an inherited disorder (e.g., MCAD) using a specific test strategy (e.g., MS/MS). The survey was based on a web-based questionnaire where one question was stated for each indicator of the structured scheme which can be obtained from the authors upon request. A total of fifty-five decisions were reported from twenty-one countries.

Table 1. Steps of Decision Processes, Statements and Selected Operationalization

Step of decision process	Statement[a]	Operationalization	Indicator(s), according to structured scheme(11)
Trigger	There is a higher frequency that explicit criteria are stated at start of decision process to select technology and to define objective of decision.	Information whether decision process was started by definition of explicit criteria for selection of technology or, technology was selected *ad-hoc*.	*Start of decision process* - *Ad-hoc* selection - Explicit specification of criteria for trigger
Participation	The variety of involved stakeholders is higher. The level of involvement of participating stakeholders differs.	Number and types of different stakeholders being formally involved and their involvement.	*Types of stakeholders involved:* - Service provider(s) - Payer - Government - HTA group or agency - Patients/patient representatives - Industry – Academia - Other stakeholder(s) *Level of involvement of formally participating stakeholders:* - Information provision - Voting - Appeal
Publication	The transparency of the decision process is higher.	Number and types of different documents that have been published during or after the decision process.	*Types of documentation accessible to public during/after decision process* - Attendance at or minutes of appraisal meeting - Decision rationale - Decision outcome - Stakeholder comments - Rationale for assessment question from scoping - No information available - Other
Assessment	The scientific rigor of the methods used for assessment of effectiveness, costs / cost-effectiveness is higher.	Methods that were used for assessment of effectiveness and costs / cost-effectiveness.	*Assessment of effectiveness:* - No assessment of effectiveness - Expert opinion - (Systematic) literature review - Quantitative meta-analysis of studies *Assessment of cost-effectiveness:* - No assessment of cost-effectiveness - Cost estimate - Cost-effectiveness analyses
Appraisal	The relevance of aspects that are considered for the final decision differs.	Aspects that were considered relevant or strongly relevant for the decision outcome	*Aspects relevant for outcome of decision* - Effectiveness (health gain from testing) - Effectiveness (other benefit of knowledge from testing) - Expected costs - Cost-effectiveness - Budget impact - Effect on equitable access to health care - Severity of the disease - Quality of evidence - Availability of treatment for disease - Scientific interest in gathering further evidence - Lobbying activities by service provider(s) - Lobbying activities by industry - Lobbying activities by patients/ patient representatives - Lobbying by government - Third party payer's concern for cost containment - Other

[a]All statements are made for the case where an HTA has been published.

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Table 2. Analyzed Decision Options

Group of decisions	Country	Decision 1	Decision 2	Decision 3
HTA report	Denmark	MCAD	CAH	–
	The Netherlands	MCAD	Hb SS	–
	England	MCAD	Hb SS	CF
No HTA report	Belgium: Region of Flanders	MCAD	BIO	–
	Czech Republic	CAH	CF	–
	France	CF	–	–
	Germany	MCAD	CAH	–
	Hungary	MCAD	MSUD	GALT
	Romania	CH[a]	PKU[a]	–
	Slovenia	MCAD[b]	CAH[b]	–
	Switzerland	MCAD	–	–

[a] Expansion of number of newborns screened.
[b] Selective screening.
BIO, biotinidase deficiency; CAH, congenital adrenal hyperplasia; CF, cystic fibrosis; CH, congenital hypothyroidism; GALT, classical galactosemia (galactose-1-phosphate uridyltransferase deficiency); Hb SS, sickle cell disorders; MCAD, medium-chain acyl-CoA dehydrogenase deficiency; MSUD, maple syrup urine disease; PKU, phenylketonuria.

Results of decisions that have been evaluated by two respondents showed that the inter-rater reliability is low and answers need to be validated to obtain accurate results. To ensure validity of statistical tests, the data set was restricted to those twenty-two decisions that had been cross-validated by two experts from each country through application of a Delphi procedure. Conflicts remained in approximately 5 percent of answers. Then, statements from the expert that had the closest relation to the decision were considered, for example, statements from third party payers were preferred to service providers. Table 2 shows the countries and decisions included in the present analysis.

The indicator of HTA activity is the publication of an HTA report. The data set was split in two groups—decisions reporting the publication of an HTA or not. Related activity might have played a role but no other objective indicator could be drawn. Frequencies of categories for selected indicators describing the steps of decision processes were calculated. Due to the small sample size, a nonparametric method (two-sided Fisher's exact test) was applied to test for differences in distributions of frequencies of categorical variables between both groups. A two-sample Kolmogorov-Smirnov test was applied for comparison of the number of documents published and number of participating stakeholders, as no assumption about the distribution of variables could be made. This test assesses whether the distributions of variables between two samples differ statistically. For the step appraisal, answers from both respondents were considered. The sum of absolute deviations in the rating of relevance between both groups was additionally derived. To account for dependent observations, the test statistic of Fisher's exact test was corrected for the existence of two respondents. As the study objective was exploratory, no adjustment for multiple testing was made. Data evaluation was performed with SAS Version 9.2 (SAS Institute, Cary, NC).

RESULTS

The literature review retrieved fifty references excluding conference proceedings of which seventeen articles were identified that discussed the role of HTA in relation to coverage decision making. Two studies analyzed past decisions for single decision makers, but not in relation to the process of decision making (1;2). Other research discussed the role of HTA by conducting structured country comparisons, for example (17;18;20;24), or provided case studies for single countries, for example (4;15), displayed general categories on the role of HTA in the innovation process (3) or considered HTA as one of many factors to accelerate technology diffusion due to increased publicity (21). Other authors elicited decision-maker's preferences (16) or examined the role of early HTA to inform policy decisions (12). In addition to the published studies, an OECD survey was identified that gathered data on the role of HTA in decision processes for five technologies in twelve countries (19).

Among the twenty-two decisions on NBS technologies with two independent reports, an HTA report was available during or after the decision process for seven decisions (6;13;22;25;26;30). Table 3 displays an overview of the frequencies of the selected indicators grouped by existence of an HTA report. Table 4 shows the results of the Kolmogorov-Smirnov test.

Trigger

The specification of explicit criteria by stakeholders was found to be associated with the publication of an HTA. All processes with HTA reports were initiated after the specification of screening criteria. In contrast, 40 percent of processes which had no HTA publication were started *ad-hoc*, without explicit criteria. In Slovenia, for example, the screening

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Table 3. Overview of Results by Step of Coverage Decision Process and Existence of HTA Report for Decisions on NBS Technologies in Europe

Indicator	Category	Percent of selected categories: HTA report $n = 7$ decision processes[b]	Percent of selected categories: No HTA report $n = 15$ decision processes[b]	p value Fisher's exact test
Start of decision process	Ad-hoc selection	0%	40%	.1206
	Explicit specification of criteria for trigger	100%	60%	
Participation of stakeholder x[a]	Service provider(s)	100%	93%	1.0000
	Payer	71%	87%	.5646
	Government	100%	80%	.5227
	HTA group or agency	71%	0%	.000797
	Patients	100%	53%	.0513
	Industry	0%	13%	1.0000
	Academia	29%	20%	.2632
	Other	29%	20%	1.0000
Participation: degree of involvement	At least information provision	71%	13%	.0136
	At least voting	29%	87%	
Publication: Reporting of documents[a]	Attendance at or minutes of appraisal meeting	43%	20%	.3341
	Decision rationale	57%	20%	.1447
	Decision outcome	100%	87%	1.0000
	Stakeholder comments	29%	7%	.2273
	Rationale for assessment question from scoping	29%	0%	.0909
	No information available	0%	13%	1.0000
	Other	0%	0%	–
Assessment: effectiveness	At least expert opinion	0%	13%	.177
	At least (systematic) literature review	71%	80%	
	At least quantitative meta-analysis of studies	29%	0%	
	Missing	0%	7%	
Assessment: costs / cost-effectiveness	No assessment of costs	0%	13%	.3947
	Estimate of costs	100%	60%	
	Cost-effectiveness analyses	0%	27%	

Appraisal criteria[a]	HTA report: Not relevant	HTA report: Relevant	HTA report: Strongly relevant	No HTA report: Not relevant	No HTA report: Relevant	No HTA report: Strongly relevant	Sum of absolute deviations
Effectiveness (health gain)	0%	0%	100%	13%	27%	60%	80%
Severity of disease	0%	36%	64%	23%	30%	47%	46%
Expected costs	14%	43%	43%	23%	33%	43%	19%
Availability of treatment	0%	57%	43%	53%	27%	20%	106%
Quality of evidence	21%	50%	29%	27%	53%	20%	18%
Cost-effectiveness	43%	36%	21%	43%	43%	13%	15%
Lobbying: patients	36%	57%	7%	60%	30%	10%	54%
Effectiveness (benefit of knowledge)	79%	21%	0%	67%	27%	7%	25%
Budget impact	29%	71%	0%	57%	37%	7%	69%
Effect on equitable access to care	64%	36%	0%	47%	50%	3%	34%
Scientific interest for further evidence	79%	21%	0%	80%	20%	0%	2%
Lobbying: service provider(s)	79%	21%	0%	97%	3%	0%	36%
Lobbying: industry	100%	0%	0%	57%	43%	0%	86%
Lobbying: government	100%	0%	0%	100%	0%	0%	0%
Payer's concern for cost containment	100%	0%	0%	100%	0%	0%	0%

[a]More than one answer was possible.
[b]See Table 2 for a detailed overview of decisions.

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Table 4. Number of Participating Stakeholders and Published Documents

No. of mentioned categories per indicator	HTA report $n = 7$ decision processes[a] Mean	Median	SD	No HTA report $n = 15$ decision processes[a] Mean	Median	SD	p value Kolmogorov-Smirnov test
Participating stakeholders	4.9	5	1.35	3.4	4	1.45	.0679
Published documents							
– During & after decision	2.6	2	0.97	1.3	1	0.62	.0039
– During decision	1.4	2	0.98	0.6	0	0.99	.0573
– After decision	2.6	2	0.98	1.2	1	0.77	.0039

[a]See Table 2 for a detailed overview of decisions.

decision was said to be the result of the availability of appropriate screening equipment. Four countries reported explicit screening criteria. In the Netherlands, the Health Council developed its own criteria after reviewing the Wilson and Jungner criteria and other lists (13). In England, France, and Denmark, standing committees formally started the decision.

Participation

The mean number of involved stakeholders was larger where an HTA was conducted (4.9 versus 3.4 in the non-HTA group). Compared with the group with HTA, only the industry and third party payers were involved more frequently where no HTA was existent. Significant differences in the frequency of participation could only be identified for patients and HTA agencies toward a higher participation in the group with HTA report.

Differences in the opposite direction were found with regard to the degree of involvement of stakeholders. Where an HTA was provided, stakeholders were involved in information provision (71 percent). On the contrary, almost 90 percent of stakeholders in the group without HTA were involved in voting on the decision option and only 13 percent provided information. Decision processes with an HTA were thus more participative in terms of inclusion of stakeholders but simultaneously less participative in terms of the degree of participation.

Publication

For all decisions, some kind of information was reported, the only exception being in Slovenia. The number of published documents per decision significantly differs between the group with and without HTA. In the group of decisions with HTA the frequency of published documentation was higher for all types of documentation. In the Netherlands, stakeholder comments and the assessment question from scoping was accessible, whereas in England, minutes of the appraisal meeting were available. For decisions without HTA report, most frequently only the decision outcome was reported. Overall, no information was made publicly available during the decision process in 64 percent of decisions. In the group with HTA 29 percent of decisions reported that no information had been made available compared with 80 percent in the group without HTA involvement.

Assessment

The effectiveness of NBS was assessed in all decisions. In 13 percent of processes without HTA report the assessment was solely based on expert opinion. For one decision, no information on the type of assessment of effectiveness was provided. In decisions with HTA report, the assessment of effectiveness was based on different scientific methods. Expert opinion and a review of the literature were always considered. For decisions including an HTA, the conduct of a systematic review of the literature could be validated. For the other group, it was not possible to determine whether the literature was reviewed systematically. In Denmark, it was stated that a quantitative meta-analysis had been conducted by the American College of Medical Genetics (ACMG), with results that were adjusted to the Danish situation (26). However, the ACMG report was based on expert opinion and did not meet the criteria for a systematic review (10).

Costs or cost-effectiveness were reported to have been assessed in all decisions except for the decision in Slovenia. In one third of the decisions without HTA, the assessment was supposed to have included a cost-effectiveness analysis, but this was not documented. That term is often used loosely, and it was not possible to validate whether cost-effectiveness analyses were conducted according to best practice guidelines.

Appraisal

In Table 3, the aspects that appeared relevant or strongly relevant for the final decision outcome are displayed. For both groups the health gain from screening was considered to be most important criterion. It was considered strongly relevant in all decisions with HTA, compared with 60 percent of decisions without HTA. By comparing the sum of absolute deviations in the rating of relevance between both groups, the availability of treatment for the screened disease, budget impact, and health gain from testing were those aspects which were relatively more relevant in the group of decisions where an HTA was published. Although

lobbying activities were reported to play a minor role in both groups, respective activities by the industry were relatively more relevant in the group without HTA. On the contrary, involvement of patients was more important in decisions with HTA report. Only a few aspects significantly differed between the groups: expected costs and lobbying activities by service providers. For many appraisal aspects, test statistics could not be gathered as there were zero observations in at least one cell of the two-by-two table. For an overview of test statistics, see Supplementary Table 1, which can be viewed online at www.journals.cambridge.org/thc2011023. If results were adjusted for multiple testing, the significance level would drop below 0.0014. In this case, no difference between groups would be significant.

DISCUSSION

Some process steps for NBS decisions appear to have differed by whether an HTA report was prepared as part of the process. The existence of explicit criteria for selection of the technology in the trigger step was positively associated with the subsequent availability of an HTA report. Likewise, for an explicit rationale for the assessment question from the scoping process was significantly positively associated with the publication step. An implication is that if an HTA is conducted, the exact assessment question needs to be stated before the assessment.

Where an HTA was produced, service providers, government, HTA agencies, patients and academia were involved more often. To a great extent, this is likely to reflect stakeholder involvement in information provision. Involvement in the appraisal process did not differ depending on the conduction of an HTA. It seemingly guided stakeholders toward information provision during assessment instead of reducing the opportunity for single stakeholders to influence appraisal toward their interest. This may suggest that there is an association between the publication of an HTA and decision making toward higher transparency and other possibilities for stakeholder involvement. However, increased involvement has to be balanced against the apparently weaker mode of participation under an HTA regime (information provision versus voting).

In general, coverage decisions for newborn screening were based in part on an assessment of effectiveness, which should at least in part be based on a review of the literature. For decisions where an HTA was published, it could be validated that the literature review was systematic and based on a documented methodology. Although most decisions without HTA also reported the use of systematic reviews, it seems unlikely that systematic reviews were actually conducted in this group. All respondents reported that expert opinion was considered, which indicates that expert opinion was considered indispensible – independent from the existence of an HTA.

Contrary to prior expectation, no difference was found in the reported use of cost-effectiveness analysis relative to the presence of an HTA report. However, the quality of the assessments likely differed. More than half of the decision makers considered economic criteria of expected costs or cost-effectiveness as relevant criteria, regardless of the availability of HTA. Among them only one-eighth to one-fifth considered economic criteria to be strongly relevant.

If an HTA was conducted, decision makers appear to have considered tangible clinical criteria, for example, the health gain from screening, to be more strongly relevant, although this difference was not significant. The direction of distance is consistent with the expectation that HTA does not only suggest *better* quality decision processes in the sense of a greater degree of transparency and stakeholder participation (at least in terms of providing information), but also *better* criteria during assessment and appraisal in the sense of criteria which are typically targeted by scientific clinical studies.

Limitations

Although this is a larger sample of decision processes than has been considered previously, the results relied on a small data set. The number of decisions with HTA report was relatively higher in the selected than in the full data set (46 percent compared with 10 percent). Accordingly, the effect from HTA may be overestimated. The existence of an HTA report is a crude indicator for HTA activity. However, comparison of the results grouped by participation of an HTA agency showed no major difference. Furthermore, participation of an HTA agency in the decision process might overestimate effects as it does not suggest that the technology under consideration had been evaluated fully.

The issue of endogeneity should not be neglected. HTA informs decision makers and vice versa. Presumably, there are countries or regions with both an established participative and transparent decision processes and established HTA institutions while others lack both. This might potentially overestimate the influence from HTA activities. If a new HTA were set up in a country with a different tradition of health policy making, it might have little direct effect on policy processes.

This study assumed that decision making occurred at a single level for a given country. Decisions might be made at the national or regional government level and additionally, by healthcare providers and payers. Beyond this, coverage decisions always are subject to policy making so that decisions are influenced by healthcare system settings, available resources and stated priorities. For newborn screening, the prevalence of screened disorders further differs across populations. When describing single decision processes in detail, the particular context of decision making should be taken into account. However, this was not the subject here as we focused on the decision processes.

The findings on NBS decision processes are exploratory results that require confirmation, particularly for other technologies and in other countries. For exact estimation of the

interdependencies, statistical methods other than nonparametric tests are needed. Instruments such as regression analysis were not applicable to address the association between HTA and decision process due to the scarcity of data and missing parameter distributions. More thorough and precise analyses will also require larger data sets which are time consuming to collect.

Implications

The results offer implications for policy makers on the role of HTA in coverage decisions. Undertaking an HTA during a decision process not only may disclose relevant evidence in an objective manner but more transparent and participative decision processes may be promoted simultaneously. Vice versa, participative and transparent decision processes might ensure the proper use of HTA methodology.

Furthermore, the use of HTA appeared to be associated with an increased role of clinical effectiveness in decision making. At least to some extent, HTA may also guide decision makers toward more reasonable appraisal criteria. The finding that HTA was not associated with greater use of appraisal criteria such as cost-effectiveness, as has been noted earlier (28), indicates that HTA is not a determining factor in their consideration. If policy makers wish to be able to make use of cost-effectiveness information for appraisal, they should explicitly demand such information. The major limitation, though, for the use of cost-effectiveness information in NBS decision making seems to be lack of demand for this information. Even if it is reported in HTA, it may not be used by decision makers, as has been documented (9).

When an HTA was published, decision processes were always triggered by explicit criteria. This may indicate that the use of explicit criteria for triggering decisions may lead decision makers to commission an HTA because it can ensure that criteria relevant for triggering the process were appropriately assessed for the technology under investigation.

In addition to general implications, conclusions can be drawn for the use of HTA in NBS policy making. From eleven analyzed countries, only three had an HTA report provided in the context of the decision. In four countries without HTA reports, an HTA agency existed but did not produce a report because HTA was not a formal requirement in NBS decision processes or the decisions were made on regional levels (i.e., Flanders, Belgium). Decision makers may consider evidence from assessments produced in other countries as sufficient for their NBS decision making as was indicated by one respondent country, Denmark. Some of the qualitative results may be specific to NBS. Given that randomized clinical trials have not been conducted for NBS, with the exception of cystic fibrosis, the degree of scientific rigor is lower for NBS than for many technologies, such as pharmaceuticals.

Our findings of variability of perceptions among respondents of NBS policy process may have broader implications for research: equal processes can be perceived differently by different observers. Therefore, it is essential for future studies of the role of HTA to elicit input from multiple expert informants. Ideally, they should reflect different stakeholder groups to assure the validity of responses. This can shed light on the extent to a lack of agreement or transparency regarding policy-making processes.

CONCLUSION

This study examined the association between the publication of an HTA and the steps of decision processes in a structured and consistent manner for a distinct technological area. It explored associations between the steps of decision processes and publication of an HTA. The goal of HTA should be to ensure *good* decision processes for coverage of technologies. First evidence on possible associations between the HTA and process steps of decision making have been shown for selected steps and single indicators. Not only was HTA associated with the outcome of decision processes—as shown earlier—but results also suggest that decision processes themselves differ by the presence of an HTA in terms of stakeholder involvement and documentation of decisions.

Conducting an HTA may not necessarily be a guarantee of a deliberative decision process with higher transparency and improved possibilities of fair stakeholder participation, features that have been discussed earlier (19), but it appears to have positive associations with those attributes.

SUPPLEMENTARY MATERIAL

Supplementary Table 1
www.journals.cambridge.org/thc2011023

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CONFLICT OF INTEREST

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