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Commentary

Expanded HTA, Legitimacy and Independence

Comment on "Expanded HTA: Enhancing Fairness and Legitimacy"

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Abstract
This brief commentary seeks to develop the analysis of Daniels, Porteny and Urrutia of the implications of expansion of the scope of health technology assessment (HTA) beyond issues of safety, efficacy, and cost-effectiveness. Drawing in particular on experience in the United Kingdom, it suggests that such expansion can be understood not only as a response to the problem of insufficiency of evidence, but also to that of legitimacy. However, as expansion of HTA also renders it more visibly political in character, it is plausible that its legitimacy may be undermined, rather than enhanced by, independence from the policy process.
Keywords: Health Technology Assessment (HTA), Equity, Budget Impact, Threshold, Independence, Regulation, Legitimacy

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In their article 'Expanded HTA: Enhancing Fairness and Legitimacy',[1] Norman Daniels, Thalia Porteny, and Julian Urrutia consider the implications of the increasing tendency to broaden the scope of health technology assessment (HTA) to embrace questions of ethics (such as equity), budget impact and financial protection, alongside its longer-standing concerns with safety, efficacy, and cost-effectiveness. Such expansion enables HTA to furnish decision-makers with the additional evidence which is needed for them to make judgements on the distribution of scarce healthcare resources, given that all such judgements will encompass matters beyond the 'traditional' HTA remit. Nonetheless, the authors note that while such expansion thereby assures the relevance of HTA to allocative decision-making, it also brings with it a danger of 'overreach,' which might imperil the legitimacy of the recommendations which emerge from the HTA process. However, this risk can be minimised, in the view of the authors, if expanded HTA is embedded in a fair, deliberative process which meets the conditions of the 'accountability for reasonableness' model and which, to secure legitimacy, should be independent of vested interests.

The analysis offered by Daniels, Porteny, and Urrutia is extremely valuable in alerting us to the ongoing need to confront problems of legitimacy as institutions and processes for decision-making on allocation of healthcare resources continue to evolve, as seems certain to happen. Given the significant fiscal pressures on contemporary health systems across the globe, newer HTA agencies have been conferred with a regulatory function which their oldest counterparts did not possess: they operate as a key component of political strategies to reduce inefficiencies and waste in healthcare. However, the boundary between the 'rationalisation' of ineffective interventions and the 'rationing' of those which are effective is extremely fuzzy,[2] especially from the wider public's perspective.

The consequence is, that, as HTA expands to meet the charge of irrelevancy – that is, that its process of evaluation and the recommendations which result have tended previously to provide insufficient evidence upon all of the matters which decision-makers need to consider when making judgements on distribution of scarce healthcare resources – and thus to play a regulatory role, so it tends increasingly to generate the 'suspicion, distrust and even resistance' which characterises the 'legitimacy problem' in healthcare resource allocation.[3] Efforts to address this problem provide in themselves further rationales for extension of the scope of HTA.

Instructive in this regard is the early history of the best-known HTA agency in the world, the United Kingdom's National Institute for Health and Care Excellence (NICE). Although there was an awareness from the date of the establishment of the Institute in 1999 that its activities would necessitate making judgements on matters beyond those which could be inferred from the available scientific evidence base,[4] 'initially NICE found it difficult to address issues of equity with any degree of sophistication,'[5] and thus, its recommendations were based primarily on evaluation of data on clinical efficacy and cost-effectiveness. This 'technocratic fix' proved inadequate because, as leading figures in the Institute noted, scientific evidence is neither perfect nor all-embracing. 'Social value judgments' – that is, those concerning 'what is appropriate and acceptable for society in delivering healthcare'[6] – were also needed, but 'NICE and its advisory bodies... have no particular legitimacy to determine the social values of those served by the NHS [National Health Service].'[7] To address this dimension of the problem of legitimacy, NICE took two steps. First, it published a set of guidelines on social value judgements, with which all guidance produced by the Institute was expected to comply (and with an obligation to explain clearly any departure from it).[8] Secondly, it established a deliberative body, the Citizen's Council, as a means of

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ascertaining the views of the United Kingdom public on questions relating to social values and feeding into the Institute's work (including the development of the guidance on social value judgements). While these developments have not passed uncriticised,[9,10] they appear to correspond to the model of expanded HTA noted by Daniels, Porteny, and Urrutia and to their prescription of its embeddedness within a deliberative process, consistent with 'accountability for reasonableness.'

On Daniels, Porteny and Urrutia's analysis, such embeddedness should have sufficed, at least in part, to offset concerns as to the legitimacy of NICE recommendations which might have arisen as it expanded the scope of the criteria which are considered within the HTA process. Yet, the Institute continues to encounter challenges to its role in determining affordability of treatments and services, that is, the budget impact of HTA. A particular area of controversy is the 'threshold' applied to determine whether an intervention can be deemed cost-effective, and thus, recommended for coverage. Although NICE merely has a somewhat ill-defined mandate to consider 'the broad balance of benefits and costs of the provision of health services…in England,'[11] its application of such a threshold – calculated on the basis of cost per quality-adjusted life-year (QALY) gained – is effectively determinative of access to the treatments and services which it appraises. This is so because a legal obligation exists to fund interventions which the Institute recommends for use, while scarcity of resources renders it highly improbable that any non-recommended interventions would be routinely covered (although clinicians might intermittently succeed in securing access to such treatments or services for individual patients based upon proof of exceptional circumstances).[12] This key decisional tool has engendered considerable controversy. Since determination of the threshold is logically equivalent to determination of the available NHS budget – a matter which it is 'constitutionally improper' for NICE to decide – the Institute must act as a 'threshold searcher' rather than as a 'threshold setter.'[13] But it is unclear that the threshold which applies is set at the correct level: that is, NICE recommendations may displace more QALYs than are gained,[14] and thus, 'the approval of new drugs is doing more harm than good to NHS patients overall.'[15] Furthermore, dissatisfaction with the threshold has prompted activity in the political arena to dispense with it in certain circumstances: initially through pressure upon NICE to establish an exception for life-extending, end-of-life treatments; and latterly in the form of an election manifesto promise to create a specified fund for financing provision of cancer treatments, in essence circumventing the NICE HTA process altogether for such interventions.

It might be argued that any problems of legitimacy occur in this context precisely because of an absence of 'embeddedness.' That is, while *certain aspects* of NICE's expanded form of HTA might be seen as deriving legitimacy from a fair, deliberative process (albeit that, as noted above, even these are not immune to criticism), its engagement with questions of budget impact is not so regarded because no such process of deliberation on this matter has occurred. It is notable that, while the Citizen's Council has discussed the issue of whether departure from the threshold is ever permissible,[16] the *a priori* questions of whether a cost-effectiveness threshold should exist at all, and if so, at what level it should be fixed, have not been put to it. But it is also plausible that the difficulties encountered by NICE in this context are indicative that HTA, especially in its expanded form, is – as NICE has acknowledged – inextricably intertwined with priority-setting in healthcare, which is 'inescapably a political process.'[17] To put matters somewhat differently, and adopting the metaphorical terminology which is deployed in the literature, HTA can be regarded less as a 'bridge' between scientific research and policy-making,[18] with the implication that it connects the two but is somehow distinct from each; but rather as an activity which 'belongs to both communities'[19] – thus, the issues addressed within HTA cannot be disentangled from the concerns and interests which arise in the political arena. For example, NICE's concession to pressure to adjust the threshold in respect of end-of-life treatments can itself be seen as a political act.

It seems to this author that reading expanded HTA as a deeply politically-charged process, albeit one with scientific underpinnings, presents us with a somewhat different set of conclusions from those drawn by Daniels, Porteny, and Urrutia, especially in relation to the value of independence. Certainly, adherence to this characteristic assists in protecting against that 'skepticism about the legitimacy of coverage decisions…[which] derives from the belief that HTA is in the service of vested interests that control the health system.'[1] But it generates a further problem: here, "overreaching" becomes a question of democratic (il)legitimacy, of (in)competence to determine matters – or at least, to apply decisional criteria – which, constitutionally, should properly be decided by elected representatives. As seen in the case of the NICE threshold, it is the objectification, technification and depoliticisation of an inherently political question as the basis for an allocative decision which generates misgivings, criticism and opposition in relation to HTA.

This is not a problem peculiar to the health policy and management arena. The rise of HTA, and its continuing expansion in scope, can be seen as an illustration of a broader trend in modern governance: the rise of the 'regulatory state.' Theorists have identified that a central puzzle of this form of political arrangement surrounds the legitimacy of decisions taken by the various independent regulatory agencies which undertake many of the tasks previously assigned to elected and accountable politicians.[20] However, there is no easy resolution of this problem. Effective mechanisms of accountability are likely to prove of crucial importance, as Daniels, Porteny, and Urrutia recognise. But designing these is awkward because of an inherent tension with the independence which is the central characteristic of this form of governance. To take an example: giving politicians the 'last word' on decisions emerging from the HTA process provides some degree of accountability to elected representatives, and hence to the public. Yet it substantially undermines one of the primary political rationales for the evolution of the 'technocratic fix' of HTA in the first place: its capacity to 'defang political conflict' on matters of resource allocation in healthcare.[21] Even if a presumption exists that the recommendations of the HTA agency will be followed (as called for by Daniels, Porteny, and Urrutia), the requirement to 'sign off' the decisions at ministerial level creates a space for articulation of precisely the type of dissension and disagreement which politicians

sought to minimise through creation of independent bodies such as NICE.

There is little doubt that the expansion of HTA – both in terms of its scope, but also its geographical reach across health systems – will continue well into the foreseeable future. Daniels, Porteny, and Urrutia have provided a valuable service in alerting us to some of the implications of this development. It has been my intention in this brief comment to offer additional reflections which should serve to demonstrate that further critical analysis of expanded HTA is certainly warranted. Given both the significance of this key regulatory activity of the modern state and the controversy which it inevitably attracts, it is to be hoped that neither analysts nor makers of health policy will shrink from undertaking such a task.

Ethical issues

Not applicable.

Competing interests

Author declares that he has no competing interests.

Author's contribution

KS is the single author of the paper.

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