

Comment on: “Can the EVIDEM Framework Tackle Issues Raised by Evaluating Treatments for Rare Diseases: Analysis of Issues and Policies, and Context-Specific Adaptation”

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The article by Wagner et al. [1] suggests the EVIDEM (Evidence and Value Impact on DEcisionMaking) framework to measure the value of treatments for rare diseases. EVIDEM assesses the value of treatments based on a set of predefined criteria. Using multi-criteria decision analysis (MCDA), each criterion obtains a specific weight that enables calculation of an aggregate rating score for each treatment. Criteria suggested by Wagner et al. [1] include disease severity, treatment health gain and population size. They are justified by major ethical theories such as utilitarianism, deontology and Rawls’s Theory of Justice [2–4]. Thus, “explicit trade-offs between competing ethical positions of fairness” [1] are made. Based on the additive multi-attribute utility function underlying MCDA in the hypothetical example, EVIDEM could be described, in my opinion, as calculating a ‘preference-weighted average of common ethical positions’ or, to put it even more simply, as voting for different ethical positions. In the case of EVIDEM, preferences/votes are obtained from experts.

Moreover, I see EVIDEM only as a preliminary compromise between a purely empirical and a purely theory-driven approach to value assessment. The former approach, which elicits so-called social value preferences, has been criticized for lack of a compelling ethical justification [5]. The latter approach, on the other hand, has been criticized for a lack of feasibility. As stated by Nord [6], the “debate among utilitarians and egalitarians has been unable to show that one position is more logically sound or more consistent

than [...] the other” (see, for example, Walker and Siegel [5] and Ubel [7]). EVIDEM thus can be seen as a pragmatic solution to what has been described as the problem of incommensurable ethical positions where rational agreement seems impossible.

Still, as an input for further development of the EVIDEM framework, I would like to make the following suggestions. First, there needs to be an independent ethical justification for MCDA, similar to what has been called for in the case of societal value preferences [5]. In fact, the additive form of the multi-attribute value function underlying MCDA in the hypothetical example has its strongest ethical justification in preference utilitarian theory [8]. This is not to say that a utilitarian foundation is necessarily wrong but to point to an implicit value judgment made by the authors. Relying on experts to provide the weights makes the underlying preference utilitarian approach, in fact, a paternalistic one. As seen in the discussion of measuring health states [9], it is also possible to use preferences of patients or the community to estimate the multi-attribute value function.

Hence, to avoid some of the implicit value judgments underlying MCDA, a stronger theoretical foundation is needed. As is well put by Walker and Siegel [5], “it is sometimes possible to construct policy around points of convergence between theories that oppose one another in their more comprehensive forms.” A prime example of such convergence is delineated by Daniels [10] when comparing his theory of justice for health with Sen’s capability approach: “Despite the difference in terminology—capabilities versus opportunity—the two views largely converge.” Further development in this area has subsumed not only Daniels’ theory of justice for health and Sen’s capability approach but also preference utilitarian

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theory under the umbrella of an autonomy-based conception of healthcare provision [11]. At least in the case of mental versus physical disability, the theory is able to provide some information on the priorities and thus the value of treatments. In my opinion, the EVIDEM framework should therefore only be considered as an intermediate and pragmatic solution to what otherwise requires further theoretical development. A theory-driven approach does not exclude the use of individual preferences per se but requires a theoretical justification for their use.

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