Updated Recommendations for Cost-effectiveness Studies

To the Editor Cost-effectiveness is a relatively new discipline, for which guidelines continue to be developed and refined. The article by Dr Sanders and colleagues1 offered updated reference cases and perspectives as well as a checklist in an attempt to improve standards and minimize bias in published studies.

There is evidence that cost-effectiveness studies sponsored by pharmaceutical companies are more likely to report positive cost-effective results.2 As a means to address this and other potential biases, we agree with the authors’ recommendations to require a true societal health care perspective and include an impact inventory.

The authors included in the checklist that an analytic plan be described within the article. We propose that not only should the analytic plan be described in the methods section, but it should also be made public prior to the analysis. This would hold cost-effectiveness studies to the same standards as systematic reviews and randomized clinical trials, both of which have guidelines that require registration of studies that detail the methods and outcomes prior to execution.3,4 Registration of systematic reviews and randomized clinical trials have been incorporated into guidelines in an effort to address the problems of multiple comparisons and selective outcomes reporting.

Requiring cost-effectiveness investigators to commit to a prespecified analytic plan would promote transparency and differentiation between a priori and post hoc analyses. This differentiation would encourage investigators to develop robust analytic plans prior to performing their analysis and would limit the risk of manipulation of the model until the desired result is obtained, a problem similar to that found in multiple comparisons. The additional transparency would allow readers to understand more thoroughly how the investigators arrived at their results and see what modifications they made along the way.

Cost-effectiveness methodology has developed significantly during the last few decades and these new recommendations should help advance it even further. An added recommendation of trial registration would be another means of guarding against bias in a field just coming of age.

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To the Editor Dr Sanders and colleagues1 updated the guidelines for the reporting and conduct of cost-effectiveness studies. Their main recommendation for the inclusion of findings based on a societal perspective alongside the conventional health sector perspective will enable these types of studies to reflect the wider social benefits of improving health, particularly of interventions that primarily target health systems and populations rather than individuals.2

In operationalizing a societal perspective, Sanders and colleagues1 recommended the reporting of an impact inventory, which is an extensive list of outcomes attributed to the intervention including nonhealth as well as health indicators. Such lists are generally produced as part of a cost-consequence analysis and are not new.3 Although impact inventories are prone to the selective reporting of outcomes, the new guidelines guard against this by recommending prespecified economic evaluation protocols.

Nevertheless, the guidelines could be strengthened by also requiring the presentation of a predefined theoretical framework in each study that should indicate why these multiple outcomes are expected to be influenced by the intervention. This would act as a validity check on the composition of the impact inventories by making explicit proposed mechanisms of action and their link to the items listed in the inventories. The evaluation of a trial investigating a microfinance and health education intervention (primarily designed to prevent gender violence and spread of human immunodeficiency virus infection in rural communities in South Africa) provides an example of how such frameworks could be presented.4

In that study,4 outcomes were prespecified at individual, household, and community levels and held together by an overarching theoretical framework, indicating how the behaviors influenced by the intervention were linked to outcomes via multiple pathways. Prespecifying outcomes through this framework provided a comprehensive, multilevel account of how the intervention eventually effected change in the community and thus enabled a rigorous assessment of the social effect of the program.5

The update of the guidelines on the reporting of cost-effectiveness studies will help to generate the broad-ranging evidence needed to account for a societal perspective in the economic evaluation of health programs. The augmentation of these changes with the requirement to prespecify a theoretical framework justifying the outcomes assessed in each study will ultimately strengthen the credibility and utility of this evidence.

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In Reply We agree that both prespecification of cost-effectiveness analysis protocols and a common framework for summarizing the impact inventory would increase transparency, reduce the risk of bias, and promote the development and dissemination of high-quality cost-effectiveness analyses.

In the article and the book,1 the Second Panel on Cost-Effectiveness in Health and Medicine recommended that a written protocol be developed at the outset of an analysis that details key aspects of the study’s design and conduct (eg, the objective; the intervention, comparators, and populations under consideration; the time horizon; sources of data; key assumptions) and that this protocol be updated, and changes noted as the study progresses.

The panel discussed whether to recommend that the protocol be made publicly available at the outset of the study, similar to the requirements for systematic reviews and randomized clinical trials. The panel was supportive of such a requirement but viewed the question of how best to develop the needed infrastructure as a topic for future research.

A general framework describing the mechanisms of action of interventions, and their links to the items in the impact inventory, would also increase the comparability and the effect of cost-effectiveness analyses. Development of such a framework, which corresponds to the structure of the impact inventory and suits most analyses, is an important future research need.

In the meantime, the panel’s recommendation 3C advises analysts to present both summary and disaggregated measures of costs and health outcomes but stops short of recommending a single summary measure. Analysts can decide which items in the impact inventory to include in 1 or more summary measures and should clearly identify which items are included, describe how they are measured and valued, and provide a rationale for their inclusion.

We strongly support exploration of these topics as the field moves forward.

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CORRECTION

Omission of Funding/Support and Role of the Sponsor Acknowledgments and Disclaimer: In the Original Investigation entitled “Association Between End-of-Rotation Resident Transition in Care and Mortality Among Hospitalized Patients” published in the December 6, 2016, issue of JAMA,1 a funding/support acknowledgment and disclaimer were inadvertently omitted. A Funding/Support section should read: “This material is the result of work supported with resources and the use of facilities at the Veterans Affairs New York Harbor Healthcare System.” A Role of the Sponsor section should read: “The Veterans Affairs New York Harbor Healthcare System had no role in the design and conduct of the study; collection, management, analysis, and interpretation of the data; preparation, review, or approval of the manuscript; and decision to submit the manuscript for publication.” Also, a Disclaimer should read: “The views expressed in this article are those of the authors and do not necessarily reflect the position or policy of the Department of Veterans Affairs or the United States government.” This article was corrected online.


Missing Negative Symbol: In the Special Communication entitled “The Association Between Income and Life Expectancy in the United States, 2001-2014” published in the April 26, 2016, issue of JAMA,1 a negative symbol was missing from a 95% CI that appeared in Figure 8. In Figure 8, “Index for preventive care” row, the Pearson correlation coefficient (95% CI) data in column 2 should be “0.05 (~0.19 to 0.29).” In the online Supplement, an incomplete formula and 2 errors in eTable 18 have been corrected. This article was corrected online.


Language Change: The Viewpoint entitled “What to Believe and Do About Statin-Associated Adverse Effects” published in the November 15, 2016, issue of JAMA, included an inaccurate sentence regarding the findings of the STOMP trial. It was corrected to read: “This finding did not reach statistical significance, but patients taking statins developed symptoms at approximately twice the rate of patients taking placebo.” This article was corrected online.