

# Cost-Effectiveness Analysis 2.0

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Cost-effectiveness analysis in U.S. health care seems poised for a second act of sorts. Although it has never actually gone away, efforts to apply it have encountered resistance, and the federal government and some health care organizations have sometimes prohibited its use or relegated it to a minor role. But several developments are helping to recharge the field. One is the embrace of its methods by prominent groups that are using it to measure and communicate the value of new drugs and other interventions. Another is the publication of new guidelines for such analysis from a national panel that is updating recommendations for the field from 1996.<sup>1-3</sup>

Cost-effectiveness analyses reveal the trade-offs involved in choosing among alternative interventions, with the goal of obtaining the most health possible for the available resources. Such analyses help define and illuminate the potential health benefits lost when the best alternative is not selected. Researchers have conducted cost-effectiveness analyses on a wide range of topics. Some organizations, such as the Advisory Committee for Immunization Practices, which establishes national immunization policy recommendations on behalf of the Centers for Disease Control and Prevention, have used these analyses in their deliberations.

The Medicare program, however, does not consider cost-effectiveness when deciding whether to cover and pay for new thera-

pies, though it has made exceptions for certain preventive services. The Affordable Care Act forbids the Patient Centered Outcomes Research Institute from considering ratios of cost per quality-adjusted life-year (QALY), the usual way of presenting the results of cost-effectiveness analysis, as thresholds in establishing which health care services are cost-effective or recommended. Reasons for the opposition to cost-effectiveness analysis are multifaceted and reflect mistrust of the underlying methods or the motives of the parties conducting the analyses, or a desire on the part of many Americans to deny or downplay the underlying problem of resource scarcity in health care.<sup>3</sup>

Developments over the past year may signal a shift, however. In 2014, the American College of Cardiology and the American Heart Association released new guidance for developers of clinical practice guidelines emphasizing the importance of “value” considerations in such guidelines and have chosen to highlight ranges of cost-per-QALY thresholds as complements to traditional grading methods based on the strength of the clinical evidence.<sup>4</sup> At the same time, the Institute for Clinical and Economic Review, a nonprofit organization that uses cost-effectiveness analysis as part of a process for assessing the value of drugs and other technologies, has received widespread attention for a series of reports on therapies for heart failure, multi-

ple myeloma, and other conditions.<sup>5</sup> Other organizations, such as the American Society for Clinical Oncology, Memorial Sloan Kettering Cancer Center, and the National Comprehensive Cancer Network, have released their own value frameworks that examine components such as clinical benefit, adverse events, and quality of life, though they do not aggregate these measures into formal cost-effectiveness analyses.

Then, in September 2016, the Second Panel on Cost-Effectiveness in Health and Medicine, which we cochaired, published a report revising guidance on various aspects of cost-effectiveness analysis reflecting advances in the field.<sup>2,3</sup> A key change pertains to the recommended perspective for analyses. The original panel on cost-effectiveness, convened in 1993 by the U.S. Public Health Service, recommended that to improve the quality and comparability of cost-effectiveness analyses, analysts should use a “reference case” incorporating a set of standard methodologic practices. It further advised that reference-case analyses should assume a “societal perspective,” reflecting the viewpoint of a decision maker considering the broad allocation of resources across the population. Under such a perspective, the analyst considers all parties affected by the intervention and counts all significant outcomes and costs that flow from it, regardless of who experiences them.

Our panel (which was convened by a leadership group that

grew out of the original panel) recommends that cost-effectiveness analyses include not one but two reference cases — one based on a societal perspective and one on a health care sector perspective. The former is recommended because of the importance of capturing the broad consequences of health interventions, including those outside the health care sector; the latter is a nod to pragmatism, because it is more closely tied to the resource implications considered by health sector decision makers. It is also a call for clarity, since differentiating the two perspectives should help con-

sector (e.g., effects on economic productivity, social services, legal or criminal justice, education, housing, or the environment), such an inventory allows analysts to clarify those consequences for decision makers.

Whether the medical community is ready to accept the use of cost-effectiveness analysis remains to be seen. Our panel's report devotes considerable attention to ethical issues, such as distributive concerns (who receives health benefits), that we believe are important. Confronting choices about how to spend society's limited resources on health care will

dollars currently being spent by choosing medical services more wisely.<sup>3</sup> Moreover, not using cost-effectiveness analysis is also a choice. If we don't explicitly consider the health benefits and costs of alternative health investments, we fail to draw on important information about whether those resources can be put to better uses than the ones under consideration.

Cost-effectiveness is only one of many elements involved in health care decisions. Others include patients' expectations; legal, ethical, cultural, and political concerns; and pragmatic issues of logistics, feasibility, and short-term budgetary pressures. Most health organizations involved in resource-allocation decisions in the United States and other countries have given the greatest weight and deepest consideration to the clinical evidence. That is appropriate, but we should not ignore the broad view we gain from using cost-effectiveness information. Recent work should advance the field and underscore that cost-effectiveness analysis can be an essential part of the debate.

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2. Sanders GD, Neumann PJ, Basu A, et al. Recommendations for conduct, methodological practices, and reporting of cost-effectiveness analyses: Second Panel on Cost Effectiveness in Health and Medicine. *JAMA* 2016;316:1093-103.
3. Neumann PJ, Sanders GD, Russell LB, Siegel JE, Ganiats TG, eds. *Cost-effective-*

### *Cost-effectiveness analyses reveal the trade-offs involved in choosing among alternative interventions.*

sumers of these analyses better understand the consequences of interventions that fall outside the health sector. Like the original panel, our panel notes that if they have specific decision makers in mind, analysts may wish to present results from the perspectives of those persons or entities in addition to the reference-case perspectives.

Our panel further recommends inclusion of an "impact inventory," a structured table listing the health and non-health-related effects of an intervention that should be considered in a societal reference-case analysis. When interventions have substantial effects beyond the formal health care

never be easy. Opponents of cost-effectiveness analysis may continue to frame any application of it as an unacceptable exercise in "rationing." Such a view implies that declining to consider cost-effectiveness obviates the need for making trade-offs.

Because it has refused to consider these trade-offs, the United States now spends far more of its national income on health care than other countries. In reality, although it is not possible to provide all potentially beneficial health care programs and interventions without sacrificing some other important goods, services, or programs, it is possible to obtain much more health from the

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## Psychocatalytic Benefits of the Unexpected

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Few doctors would say they deliberately try to catch their patients off guard, but doing so under the right circumstances can have salutary effects. Consider my first meeting with the young Ms. M. Having discovered her fiancé's infidelity, she had impulsively swallowed a bottle of aspirin and managed to get herself involuntarily committed to a locked psychiatric unit, where I met her the morning after her admission. She was feeling scared, angry, ashamed, and betrayed — and was in no mood to tell her embarrassing story to yet another intimidating stranger. I donned my friendliest persona but got nowhere: she refused to make eye contact, stonewalled my questions, and sneered at my evidently lame empathic comments. It was time for a new tack.

"Maybe you could tell me something about what you do for fun," I suggested.

"Nothing," she said.

"Favorite TV shows? Movies? Music?"

"I like music," she conceded irritably.

Here was a tiny handhold. Encouraged, I continued — cautiously: "Really? What kind?"

"All music."

"I'll bet you have a favorite artist."

"No."

"Taylor Swift? Beyoncé?"

Now her patience was expiring.

"I said, I like *all* music."

"Even country western?"

That did the trick. She appeared a bit startled, apparently wondering whether she'd heard me correctly, and then tried unsuccessfully to suppress a tiny smile, reluctantly peering sidelong at the annoying gray-haired psychiatrist I no doubt appeared. As she confided that indeed she couldn't listen to country western music (alas, a common aversion in a place like Massachusetts), it was clear to both of us that the hard part of the interview was over. She quickly became something of a chatterbox, which of course facilitated her capacity to engage with her treatment team and make the most out of what ended up being a brief hospitalization. What had happened?

My country western question was a test dose of the unexpected, an attempt to get Ms. M.'s attention in a new way that would allow a shift of her perspective about me as well as the entire treatment situation. By gently startling her, I wanted to telegraph something like the following: "I know your situation seems frightening and I seem ridiculous, but I'm actually a reasonably nor-

mal person who even has a sense of humor. Maybe we can see eye to eye on something unrelated to hospitals and overdoses." I knew that simply saying as much would have felt like performing a transfusion with a small-gauge needle. Given how anxious and guarded Ms. M. was, it seemed more effective to show rather than tell her that I was largely harmless, potentially trustable, even "relatable" — and not the patronizing authority figure she apparently expected. I slipped in a question that she might expect to hear from a fellow human being, if not necessarily from The Doctor.

Unexpected or surprising utterances can have what might be called "psychocatalytic" effects, triggering or crystallizing a change in perspective. This technique is useful in cases in which a patient would benefit from being nudged out of cognitive or emotional rigidity. A mildly amusing or ironic comment is often effective; where standard approaches based on evidence and logic fail, a touch of humor can sometimes succeed. My meeting with Ms. M. revealed a common indication for this approach: I had found myself wondering how I could get a patient to understand something that I couldn't explicitly tell her.

In another instance, I was try-