Effectiveness Is the Key to Cost-Effectiveness

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Health care spending has risen faster than the rest of the United States economy for the past several decades, and is now swamping federal and state government budgets. The rising cost of health care has important consequences: either spending on other programs (eg, education, public safety, and infrastructure) must be reduced, or taxes must rise, or the budget deficit must grow larger. In light of these facts, physicians, whose decisions determine the bulk of health expenditures, need to become better stewards of healthcare resources. Physicians are particularly able to determine which treatments are effective and to identify which patients benefit most from those treatments. With health care dollars scarce, physicians are in the best position to see that they are spent wisely.

Cost-effectiveness analysis is one tool physicians can use to assess the value provided by a treatment strategy. Despite its technical complexities, the basic principles of cost-effectiveness analysis are conceptually simple. One key principle is that cost-effectiveness is comparative, not absolute: the therapy of interest should be compared with the next best alternative therapy. One can assess the cost-effectiveness of coronary artery bypass graft (CABG) surgery compared with percutaneous coronary intervention (PCI), or the cost-effectiveness of CABG compared with medical therapy, but not of the cost-effectiveness of CABG in isolation. Another important principle is that treatments that cost more money may still be quite valuable if they are highly effective. Effectiveness is measured by the ultimate goals of treatment: patient centered outcomes, such as reduced mortality, improved symptoms, or better quality of life. Therapies that are highly effective by these measures are likely to be cost-effective, whereas therapies that increase costs but do not improve patient outcomes meaningfully do not provide value.

FREEDOM Trial

The Future Revascularization Evaluation in Patients with Diabetes Mellitus: Optimal Management of Multivessel Disease (FREEDOM) trial is a pivotal study in which patients with diabetes mellitus and multivessel coronary disease were randomized either to CABG or PCI. The trial reported a substantial improvement in clinical outcomes among the patients assigned to CABG. Over subsequent 5 years of follow-up, however, the gap in costs between CABG and PCI narrowed to just $3600. This pattern of cost savings in follow-up costs offsetting the initially higher procedure costs of CABG has been seen in previous trials, and is the consequence of the higher rates of repeat revascularization procedures after PCI, even with the use of drug eluting stents.

The key question is whether doing a CABG is worth spending an extra $3600 over 5 years. Patients assigned to CABG were clearly worse off early after the procedure, reflected by their longer hospital stays and lower quality of life scores at 1 month, but in the long run they had better outcomes than patients assigned to PCI. This pattern of early costs and late benefits poses a challenge to an economic analysis performed alongside a clinical trial: how to account fairly for treatment effects that last beyond the observed follow-up. The most conservative approach is to count only the life-years of survival added during trial follow-up, which is equivalent to the area between the survival curves. This so-called stop and drop estimate has the advantage of being entirely empirical, but it clearly underestimates the life-years saved by preventing deaths during the study, because the average life-expectancy of trial participants is closer to 15 years than 5. The stop and drop approach yields a biased estimate of cost-effectiveness when the full costs of treatment are captured, but the full benefits are not because they take years to accumulate. A fair accounting of clinical effectiveness in this situation requires a lifetime perspective, which involves using a model to calculate the life-years added by treatment after the end of trial follow-up. The key assumption is the durability of the observed treatment effect: does the observed risk reduction continue indefinitely, dissipate over a period of time, or stop completely at the end of trial follow-up? Intuitively, these assumptions can be visualized by the pattern of the survival curves after the end of trial follow-up: the curves might continue to separate, gradually become parallel, or immediately become parallel. The specific pattern is less important than projecting the survival curves further, because otherwise the number of life-years added by treatment would be substantially underestimated. The FREEDOM investigators used several
different models to calculate the life-years added CABG. The resulting cost-effectiveness ratios ranged between $8100 and $27,000 per quality-adjusted life-year added, all well below the oft-cited $50,000 willingness-to-pay benchmark. A variety of sophisticated sensitivity analyses confirmed the basic intuition about the effect of CABG for patients with diabetes mellitus and multivessel coronary artery disease: saving an additional 5 lives for 100 patients treated with an outlay of $3600 per patient is a pretty good value for the money spent.

Broader Implications
The FREEDOM trial confirms that CABG provides a substantial survival benefit compared with PCI for patients with diabetes mellitus and multivessel coronary disease, and the economic analysis confirms that this survival benefit is worth the higher cost. But it is important to emphasize that neither the clinical nor the economic results of FREEDOM can be extrapolated to all patients with coronary disease, or to all patients with diabetes mellitus. The pooled results of previous randomized trials of CABG and PCI demonstrated that patients with diabetes mellitus obtain a particular reduction in mortality from CABG: the hazard ratio for CABG compared with PCI was 0.70 among patients with diabetes mellitus, but this hazard ratio was 0.98 among the remaining patients (P=0.014 for the interaction between diabetes mellitus and treatment). Although earlier trials did not use drug-eluting stents, it is very likely that there is still substantial heterogeneity of treatment effects for contemporary coronary revascularization. So the cost-effectiveness of CABG among patients with diabetes mellitus documented in FREEDOM should not be extrapolated to all patients with multivessel coronary disease. Nor should they be extrapolated to all patients with diabetes mellitus, who might not meet the criteria for entry into FREEDOM, particularly asymptomatic patients.

The clinical effectiveness is not an inherent property of a specific treatment but varies depending on the clinical characteristics of the patient treated, particularly the indication for treatment. Most widely used therapies work well for some patients, but are often used more generally for broader indications in wider patient groups, in which the treatments are less effective or even ineffective. The key to practicing cost-effective medicine is to target treatments precisely to the patients and indications for which they are truly effective, because clinical effectiveness is the key determinant of cost-effectiveness.

Disclosures
None.

References

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