THE ORTHOPAEDIC FORUM

Cost-Effectiveness Analyses in Orthopaedic Surgery Raising the Bar

Prashant V. Rajan, BS, BA, Rameez A. Qudsi, MD, Lindsey L. Wolf, MD, and Elena Losina, PhD

Investigation performed at the Department of Orthopaedic Surgery and the Policy and Innovation eValuation in Orthopaedic Treatments Center, Brigham and Women's Hospital, Boston, Massachusetts

Rising health-care spending has renewed emphasis on value-based care for institutions and policymakers. As a result, economic evidence increasingly is being considered in the shaping of clinical guidelines and practices¹. Cost-effectiveness analysis using decision analytic models is a widely used methodology to establish the value of specific clinical interventions². Models are informed by data from multiple sources, and are therefore capable of projecting outcomes and costs over longer time frames than the durations of randomized controlled trials (RCTs). Additionally, model-based evaluations can compare multiple interventions in the same analysis.

In 1996, *The Journal of the American Medical Association* published the recommendations of the First Panel on Cost-Effectiveness in Health and Medicine, which summarized the state of the growing field of cost-effectiveness analysis and provided guidance on the design and conduct of cost-effectiveness analyses³. Over the more than 20 years since those recommendations, there has been a drastic increase in the number of cost-effectiveness studies published in all areas of medicine, including the field of orthopaedic surgery, albeit with variable methodologic rigor^{4,5}. In September 2016, the Second Panel on Cost-Effectiveness in Health and Medicine (the “Second Panel”) updated these recommendations to establish standard analytic and reporting practices, taking into consideration methodologic advancements over the last 2 decades⁶. The Second Panel’s

recommendations focus particularly on standards of model development and reporting, as well as addressing data uncertainty.

In applying the Second Panel’s recommendations, it is important to note relevant differences between interventions in orthopaedic surgery compared with other areas in medicine. Surgical procedures often occur as discrete episodes of care, associated with high up-front costs, a high likelihood of symptom relief, and low-to-moderate rates of complications. But symptom relief as a result of surgery may have time limits, and the sustainability of such relief may require additional surgeries over time. A good example of such a trajectory is total hip arthroplasty. This is typically a one-time procedure associated with long-term pain relief and functional benefit, a low rate of complications, and some risk of additional surgery, which may increase over time. In such scenarios, lifetime modeling, using knowledge or assumptions about the persistence of benefit over time and symptomatic trajectory among those not undergoing surgery, is critical.

Given the substantial variability in the quality of manuscripts reporting cost-effectiveness analyses in the field of orthopaedic surgery, we sought to help “raise the bar” of such studies by providing a focused reporting checklist for cost-effectiveness analyses in orthopaedic surgery that is consistent with the Second Panel’s recommendations (Table I). We

Disclosure: Research reported in this publication was supported by a T32 training grant (AR055885) from the National Institutes of Health. L.L.W. is supported by the American College of Surgeons Resident Research Scholarship. E.L. receives funding support from a National Institute of Arthritis and Musculoskeletal and Skin Diseases grant (K24AR057827). On the **Disclosure of Potential Conflicts of Interest** forms, which are provided with the online version of the article, one or more of the authors checked “yes” to indicate that the author had a relevant financial relationship in the biomedical arena outside the submitted work (<http://links.lww.com/JBJS/E268>).

TABLE I Checklist for Reporting Results of Cost-Effectiveness Analyses in Orthopaedic Surgery***Study design**

- Explicitly report model structure (e.g., Markov cohort, Monte Carlo simulation, decision tree)
- Provide proper justification for why the specific model was used to address the clinical question
- Project all outcomes (costs and effectiveness) to lifetime

Data elements

- Specify the methodology and the types of studies (e.g., RCTs, retrospective studies) used to generate study inputs
- Describe means of elucidating quality-of-life utilities (e.g., direct or indirect)
- Ensure that utilities account for age-related or morbidity-related changes over time
- Express effectiveness in QALYs
- Describe the methodology used to generate costs (ensure that analyses are based on costs and not charges)
- Report outcomes in incremental costs (U.S. dollars of current year) per QALY
- Discount all costs and QALYs at the same rate (e.g., 3%) and provide justification for the discount rate

Perspective

- Report outcomes from 2 reference case perspectives: health-care payer and societal

Data uncertainty

- Examine uncertainty using deterministic sensitivity analysis
- Examine uncertainty using probabilistic sensitivity analysis
- Specify the structure and logic of distributions used for probabilistic sensitivity analysis

Study interpretation

- Report whether the intervention(s) of interest offer good value for the dollars spent (set willingness-to-pay threshold)
- Account for uncertainty in the conclusions of the study
- Provide analysis from both health-care payer and societal perspectives
- Detail limitations of the analysis, including any assumptions made in the study design or data elements and how they influence the results and the conclusions

*RCTs = randomized controlled trials and QALYs = quality-adjusted life-years.

transition cohort analysis or Monte Carlo simulation should be used to simulate patient cohorts over time, where the risk of complications or revision varies over time, while decision tree models should be reserved for more simplistic scenarios. The time horizon utilized must be long enough to evaluate all relevant costs and benefits to the patient over his or her lifetime. Model-based evaluations should include the means of internal and external model validation.

In agreement with the Second Panel, all studies should report 2 separate reference case perspectives: a health-care payer perspective that includes formal medical costs reimbursed by a third-party payer or paid by patients out-of-pocket, and a societal perspective that incorporates all costs represented in the health-care payer perspective in addition to future medical costs, time costs of seeking care, time costs of unpaid caregivers, lost productivity, and future health-care consumption costs, when relevant⁶. Providing both perspectives can alter how we view the relative costs and benefits of a surgical intervention, especially in cases in which lost productivity or unpaid caregiving have a large impact on overall costs.

Valuation of health outcomes should be in terms of quality-adjusted life years, and the methodology used to elicit “quality-adjustments” or utility weights should be described and specified as direct (e.g., standard gamble^{7,8}, time trade-off⁸) or indirect (e.g., EuroQol-5D [EQ-5D]^{9,10}, Short Form-36 [SF-36]¹¹⁻¹⁵). Quality-of-life utility values should account for age-related and morbidity-related changes over time, and the summative health outcomes should be expressed as quality-adjusted life-years (QALYs). The recommendations from the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Task Force for Clinical Outcome Assessments highlights the need for clinical studies to be designed with these outcome measures in mind in order to maximize the meaningful information obtained¹⁶⁻²². Both reference case perspectives (health-care payer and societal) should report an incremental cost-effectiveness ratio (ICER) expressed as costs (\$) per QALY gained, evaluated against a set willingness-to-pay threshold²³.

All analyses should be transparent with regard to assumptions made and to the evaluation of uncertainty of results through sensitivity analyses. Given the paucity of efficacy data derived from RCTs relevant to many treatment decisions in orthopaedics, it is imperative that the data input parameters and the implications of certain data choices are accurately cited and clearly discussed. Sensitivity of results to uncertainty in data used to inform model parameters should be thoroughly examined using both deterministic and probabilistic sensitivity analyses. The ranges of data uncertainty for both types of sensitivity analyses should be justified and supported by references.

A proper cost-effectiveness analysis can be a powerful tool to generate useful economic and clinical projections. Following a “checklist” approach in the design and reporting of these studies will lead to more consistent reporting and higher-quality cost-effectiveness analyses in orthopaedics. Raising the bar in

emphasize that the modeling approach should fit the critical details of the clinical or policy question that is being examined. The model’s structure should be consistent with the nature of the clinical problem, and should capture all of the benefits and the adverse events of the treatments under consideration over both the short and long time horizons. A Markov state-

the collective cost-effectiveness literature within the field of orthopaedic surgery can help to pioneer new standards for the determination of value-based care. ■

Prashant V. Rajan, BS, BA^{1,2}
Rameez A. Qudsi, MD^{1,2}
Lindsey L. Wolf, MD³
Elena Losina, PhD^{1,2,4}

¹Department of Orthopaedic Surgery, Policy and Innovation eValuation in Orthopaedic Treatments Center, Brigham and Women's Hospital, Boston, Massachusetts

²Harvard Medical School, Boston, Massachusetts

³Center for Surgery and Public Health, Brigham and Women's Hospital, Harvard Medical School, Harvard T.H. Chan School of Public Health, Boston, Massachusetts

⁴Department of Biostatistics, Boston University School of Public Health, Boston, Massachusetts

E-mail address for P.V. Rajan: prajan@partners.org

E-mail address for R.A. Qudsi: rqudsi@partners.org

E-mail address for L.L. Wolf: llwolf@partners.org

E-mail address for E. Losina: elosina@partners.org

ORCID iD for E. Losina: [0000-0002-3424-0892](https://orcid.org/0000-0002-3424-0892)

References

- Schwartz JA, Pearson SD. Cost consideration in the clinical guidance documents of physician specialty societies in the United States. *JAMA Intern Med.* 2013 Jun 24;173(12):1091-7.
- Bambha K, Kim WR. Cost-effectiveness analysis and incremental cost-effectiveness ratios: uses and pitfalls. *Eur J Gastroenterol Hepatol.* 2004 Jun;16(6):519-26.
- Weinstein MC, Siegel JE, Gold MR, Kamlet MS, Russell LB. Recommendations of the Panel on Cost-Effectiveness in Health and Medicine. *JAMA.* 1996 Oct 16;276(15):1253-8.
- Brauer CA, Neumann PJ, Rosen AB. Trends in cost effectiveness analyses in orthopaedic surgery. *Clin Orthop Relat Res.* 2007 Apr;457:42-8.
- Daigle ME, Weinstein AM, Katz JN, Losina E. The cost-effectiveness of total joint arthroplasty: a systematic review of published literature. *Best Pract Res Clin Rheumatol.* 2012 Oct;26(5):649-58.
- Sanders GD, Neumann PJ, Basu A, Brock DW, Feeny D, Krahn M, Kuntz KM, Meltzer DO, Owens DK, Prosser LA, Salomon JA, Sculpher MJ, Trikalinos TA, Russell LB, Siegel JE, Ganiats TG. Recommendations for conduct, methodological practices, and reporting of cost-effectiveness analyses: Second Panel on Cost-Effectiveness in Health and Medicine. *JAMA.* 2016 Sep 13;316(10):1093-103.
- Gafni A. The standard gamble method: what is being measured and how it is interpreted. *Health Serv Res.* 1994 Jun;29(2):207-24.
- Morimoto T, Fukui T. Utilities measured by rating scale, time trade-off, and standard gamble: review and reference for health care professionals. *J Epidemiol.* 2002 Mar;12(2):160-78.
- EuroQol Group. EuroQol—a new facility for the measurement of health-related quality of life. *Health Policy.* 1990 Dec;16(3):199-208.
- Brooks R. EuroQol: the current state of play. *Health Policy.* 1996 Jul;37(1):53-72.
- Ware JE Jr, Sherbourne CD. The MOS 36-item short-form health survey (SF-36). I. Conceptual framework and item selection. *Med Care.* 1992 Jun;30(6):473-83.
- McHorney CA, Ware JE Jr, Raczek AE. The MOS 36-item short-form health survey (SF-36): II. Psychometric and clinical tests of validity in measuring physical and mental health constructs. *Med Care.* 1993 Mar;31(3):247-63.
- Patel AA, Donegan D, Albert T. The 36-item short form. *J Am Acad Orthop Surg.* 2007 Feb;15(2):126-34.
- Brazier J, Roberts J, Deverill M. The estimation of a preference-based measure of health from the SF-36. *J Health Econ.* 2002 Mar;21(2):271-92.
- Brazier J, Usherwood T, Harper R, Thomas K. Deriving a preference-based single index from the UK SF-36 Health Survey. *J Clin Epidemiol.* 1998 Nov;51(11):1115-28.
- Caro JJ, Briggs AH, Siebert U, Kuntz KM; ISPOR-SMDM Modeling Good Research Practices Task Force. Modeling good research practices—overview: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force-1. *Med Decis Making.* 2012 Sep-Oct;32(5):667-77.
- Roberts M, Russell LB, Paltiel AD, Chambers M, McEwan P, Krahn M; ISPOR-SMDM Modeling Good Research Practices Task Force. Conceptualizing a model: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force-2. *Med Decis Making.* 2012 Sep-Oct;32(5):678-89.
- Siebert U, Alagoz O, Bayoumi AM, Jahn B, Owens DK, Cohen DJ, Kuntz KM. State-transition modeling: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force-3. *Med Decis Making.* 2012 Sep-Oct;32(5):690-700.
- Karnon J, Stahl J, Brennan A, Caro JJ, Mar J, Möller J. Modeling using discrete event simulation: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force-4. *Med Decis Making.* 2012 Sep-Oct;32(5):701-11.
- Pitman R, Fisman D, Zaric GS, Postma M, Kretzschmar M, Edmunds J, Brisson M; ISPOR-SMDM Modeling Good Research Practices Task Force. Dynamic transmission modeling: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force Working Group-5. *Med Decis Making.* 2012 Sep-Oct;32(5):712-21.
- Briggs AH, Weinstein MC, Fenwick EA, Karnon J, Sculpher MJ, Paltiel AD; ISPOR-SMDM Modeling Good Research Practices Task Force. Model parameter estimation and uncertainty analysis: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force Working Group-6. *Med Decis Making.* 2012 Sep-Oct;32(5):722-32.
- Eddy DM, Hollingworth W, Caro JJ, Tsevat J, McDonald KM, Wong JB; ISPOR-SMDM Modeling Good Research Practices Task Force. Model transparency and validation: a report of the ISPOR-SMDM Modeling Good Research Practices Task Force-7. *Med Decis Making.* 2012 Sep-Oct;32(5):733-43.
- Neumann PJ, Cohen JT, Weinstein MC. Updating cost-effectiveness—the curious resilience of the \$50,000-per-QALY threshold. *N Engl J Med.* 2014 Aug 28;371(9):796-7.