Health Economics in Pediatric Orthopaedic Surgery

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Abstract: Healthcare expenditures in the United States continue to rise without corresponding improvements in outcomes. Because of this, there is increasing pressure on physicians to consider the economic impact of their medical decisions. Unfortunately, physicians in general are unfamiliar with interpreting and performing various health economic analyses. A basic understanding of health economics may help physicians understand and participate in key policy discussions which may shape the future of medicine and surgery.

In the field of pediatric orthopaedics specifically, the literature involving health economic evaluation is sparse. However, many interventions in this field are low cost with potential benefits that accrue over a child’s long lifespan. Economic evaluation can help objectively quantify the impact of these interventions, as well as bolster responsible medical decision making.

The purpose of this review is to introduce commonly utilized healthcare economic tools including cost-benefit, cost-effectiveness, and cost-utility analyses. We focus on several key concepts including value, quality, time, cost, and discounting. To help illustrate these concepts, health economic literature relevant to pediatric orthopaedics is discussed. Finally, we highlight limitations inherent to health economic evaluations in general and those applicable to pediatric orthopaedics specifically. This discussion may help lay the groundwork for future studies and for further involvement in policy making.

Key Concepts:
• Healthcare economic evaluations like cost-benefit analysis (CBA), cost-effectiveness analysis (CEA), and cost-utility analysis (CUA) utilize concepts like value, quality, time, cost, and discounting.
• There are unique challenges when performing healthcare economic analyses in the pediatric orthopaedic literature.
• An adequate understanding of these concepts may help pediatric orthopaedists to understand and participate in key policy discussions that may shape the future of healthcare.

“All models are wrong, but some are useful.” – George E. P. Box

Introduction
Healthcare expenditures in the United States continue to rise disproportionately to those in other developed countries and do so without corresponding improvements in measured outcomes.2-4 To curb this trend, approximately two-thirds of physician societies have begun to consider cost when developing clinical guidelines5 and many industrialized nations are now considering cost-effectiveness when making insurance coverage recommendations.6 Unfortunately, physicians as a whole have limited experience with the interpretation and analysis of health economic analyses.7,8 Without a baseline understanding of the health economic literature,
how analyses are performed, and their advantages and limitations, physicians are ill-equipped to play an active role and participate in cost-driven policy discussions.9

This is particularly relevant in orthopaedic surgery where high procedural costs and questions regarding meaningful clinical benefit have resulted in attempts to reduce the frequency of certain orthopaedic procedures.4,10 To increase economic evaluation and to engage policymakers on the value of orthopedic surgery, the American Academy of Orthopaedic Surgeons (AAOS) formed the AAOS Value Project Team in 2012.4 Despite these efforts, there continues to be a dearth of economic analyses in the orthopaedic literature.4,7,9

Only a handful of health economic studies have been published in the field of pediatric orthopaedics in the United States,11–15 and no detailed reports exist in our literature for executing the methodology to perform such studies. Here we seek to introduce commonly utilized healthcare economic tools including cost-benefit analysis (CBA), cost-effectiveness analysis (CEA), and cost-utility analysis (CUA), with a focus on several key concepts including value, quality, time, cost, and discounting. In addition to helping understand already published economic studies, this paper may help lay the groundwork for future studies that could guide policy making. At the minimum, these concepts allow one to understand and communicate effectively with policymakers who usually don’t work in the patient and provider relationship.

Value

The concept of value in healthcare is simple, but contains complex undertones.16 In his seminal paper, Porter defined value as “the health outcomes achieved per dollar spent.”16 Although seemingly simple, value takes into account an individual’s needs, wishes, and preferences, and changes depending on historical and societal contexts, and it varies from the perspective of the patient, provider, employer, and economist.17 With the advent of Evidence-Based Medicine, a foundation was laid for Value-Based Medicine (VBM), which factors in the quality of life perceived by a patient after an intervention.18 According to Brown et al. who first introduced the concept of VBM, value can be thought of as the quality obtained for the cost expended.18, 19

\[
Value = \frac{Quality}{Cost}
\]

Health Economic Analyses

When determining value, several types of health economic studies can be used: cost-benefit analysis (CBA), cost-effectiveness analysis (CEA), and cost-utility analysis (CUA). These studies differ based on how they quantify quality. In a CBA, both cost and quality are assigned monetary values.8 For example, Tjoumakaris et al. performed a CBA using revenue and overhead to determine the profitability of orthopaedic coverage of local high school football games. Ultimately they determined that team coverage is potentially profitable, resulting in an hourly wage of $116.24 in their study.12 While widely utilized in other fields, CBA is rare in healthcare economics because it quantifies costs and quality (and therefore value) in terms of money, which some have argued may be ethically objectionable.20

CEAs avoid this issue by using “natural units” to assess quality.8 Natural units can be thought of as disease-specific metrics like pain scores, infection rates, death rates, patient reported outcome measures, and any other measure of treatment effectiveness.7,8 Stated in terms of the value equation:

\[
Value_{CEA} = \frac{Quality_{CEA}}{Cost_{CEA}} = \frac{Disease - Specific\ Outcome}{Costs}
\]

Limitations of CEAs include a lack of generalizability across different disease states and procedures. For example, if the number of dislocations prevented after a total hip arthroplasty is a disease-specific measure used in a CEA to assess THAs, the results cannot be compared to an analysis of carpal tunnel release.
Disease-specific measures will be discussed in detail later in this article. The use of CEAs also rely on high-quality studies with good outcome reporting; given that only 3-6% of orthopedic studies are randomized controlled trials, this limitation is particularly important in the field of orthopedics.7,8

Cost utility analyses (CUA) differ from CEAs in that quality is expressed in terms of utility which, in general terms, is a preference-based assessment of various health states.7,8 Utility is a general measure of health which can be applied to patients of different disease states and after different procedures. Hence, this allows one to compare the results of an intervention in one field to another and thus CUAs are the most popular in medicine and will be our focus in this paper.

\[
\text{Value}_{\text{CUA}} = \frac{\text{Quality}}{\text{Costs}} = \frac{\text{Utility}}{\text{Costs}}
\]

**Quality and Utility**

Quality in CUAs is assessed using the metric of utility which is derived from economic and decision theory and is a measure of general health.21 General health is scored on a numeric scale from 0 to 1, with 0 defined as death and 1 defined as perfect health. Three methods are commonly used in healthcare literature to directly determine utility: the standard gamble method, the time trade-off method and the visual analog/feeling thermometer.22-24

**Standard Gamble Method**

With this method a patient must decide whether to 1) remain in the current state of health, or to gamble on an intervention with an assigned probability (p) for either perfect health (p) or death (1-p). For example, a standard gamble score of 0.7 signifies that a patient would accept a 30% chance of immediate death for a state of perfect health. Thus, the probability of death or perfect health are tipped on a scale from 1 to 0 until the patient becomes indifferent to the ultimate choice. The apathy) is defined as the utility weight of one’s medical condition or life as a whole.

**Time Trade-Off Method**

The time trade-off method values utility by quantifying the hypothetical amount of life a person is willing to sacrifice to obtain their ideal state of health. For example, a time trade-off score of 0.4 signifies that a patient would sacrifice 60% of their remaining life for a state of perfect health. These scores have been utilized within orthopedics in areas such as adult reconstruction, but as one may imagine, this method would be inappropriate for the pediatric patient.25

**Visual Analog Scale/Feeling Thermometer**

In young patients for whom the mental exercise of standard gamble and time tradeoff are too abstract, the visual analog scale/feeling thermometer is utilized.24,26-29 This involves providing a description of a given health state in plain language, and asking subjects to numerically rank their preference for each state from a scale of 0 to 100 (i.e., the feeling thermometer), with 100 being perfect health and 0 being equivalent to the worst health imaginable. Utilities obtained from the feeling thermometer are then converted to a score from 0 to 1 by dividing by 100 to convert utilities into the standardized format. While these are common ways to directly measure utility, it is more practical to indirectly ascertain utility from validated assessments of health quality (e.g., questionnaires).

**Outcome Measures and Process Measures**

In general, most measures of health quality are grouped into two main categories: outcome measures and process measures (Table I). Outcome measures can be further subcategorized into general or disease-specific measures. Of note, utility is an outcome measure which is general, whereas disease-specific measures are the type of outcome measures used in CEAs.

**Disease-specific outcome measures** focus on interventions related to a particular disease, area of the body, or injury pattern.30 These include the Muscular Dystrophy Spine Questionnaire for Duchene’s Muscular Dystrophy and the Caregiver’s Priorities and Child
Health Index of Life with Disabilities (CPCHILD) Questionnaire for cerebral palsy, which are valid and reliable for their respective diagnoses. Disease-specific outcome measures are advantageous in detecting health changes in specific conditions. However, they may not reflect the patient’s overall health status or quality of life, and thus may be less relevant to the holistic picture of the patient. They are difficult to generalize, cannot be used to make comparisons to other studies, and cannot be converted to utility for use in CUAs.

General outcome measures, on the other hand, are used to assess a patient’s overall health. Although some literature suggests that general outcome measures may not be as sensitive to changes in health for specific disease processes as disease specific measures, other literature suggests they capture quality measures in a similar fashion. General outcome measures are preferred over disease-specific measures as they allow for the calculation of utility for CUAs.

Assessing the quality of an outcome measure is a difficult task and often involves nuanced statistics and unfamiliar terminology. Veihweger et al. propose an eleven-step guide to evaluating the quality of an outcome, citing critical analysis of the target domain, sample size, and appropriate measurement tools, amongst others. These authors report that in various fields of pediatric orthopaedics, such as clubfoot and spine deformities; studies have not broadly evaluated pediatric outcome measures. However, there are some general outcome measures in pediatric orthopaedics, including the Child Health Questionnaire (CHQ) and the Pediatric Orthopaedic Data Collection Instrument (PODCI).

The other common measure of health quality, process measures, encompass objective variables including surgical complications, hospital length of stay, surgical site infections, and mortality associated with an episode of care. The strength of process measures is the concrete and understandable nature of the data. However, their role in assessment of quality remains unclear as they do not incorporate patient-centered metrics, which are preferred for evaluating outcomes in surgical patients.

In a systematic review, Kennedy et al. found that mortality and postoperative complications were the most commonly reported quality indicators after pediatric orthopedic surgery. While process measures may be employed in CEAs, there is no feasible way to convert them to QALY or utility, precluding their use in CUAs.

In summary, (1) general outcomes are preferred over disease specific outcomes measures and process measures. (2) General outcomes measures should convert to utility for use in the value equation for CUAs. As found by Kennedy et al., this may not currently be the case. A shift in the paradigm of value-based

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Table I: Health Quality Measures
research is essential to ensure pediatric orthopedic researchers are reporting the appropriate outcome measures in cost and value-related studies.

**Time**

Utility accrued over time is expressed as QALYs. In equation form:

\[ \text{Value}_{\text{CUA}} = \frac{\text{Value}}{\text{Cost}} = \frac{\text{Utility} \times \text{Time}}{\text{Cost}} = \frac{\text{QALY}}{\text{Cost}} \]

In graphical form, time lies on the X-axis and utility on the Y-axis, with QALYs being equal to the area under the curve.\(^{38}\) (Figure 1)

Therefore, one can imagine that if a certain utility is derived from an intervention, and if that utility remains constant over time, then the area under the curve (or QALY) would increase over time (Figure 1A). In a scenario in which utility remains constant, the intervention is presumably successful thus minimizing or negating the need for future interventions, and thus maintaining or minimizing the future cost. Plugging cost, utility, and time into the value equation, we see that value can increase over time. A similar outcome is seen when utility increases over time (Figure 1B). On the other hand, if the utility of an intervention decreases over time, the area under the aforementioned curve would increase at a slower rate, and the value would decrease over time (Figure 1C). This highlights the importance of follow-up duration reported in health economic literature, as the value in the short-term after an intervention might not be an accurate representation of the long-term value.

When considering economic evaluation of pediatric interventions, the importance of time cannot be overemphasized. Compared to adults, children have a

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**Figure 1.** Graphical representation of the relationship of time, utility and quality-adjusted life years (QALYs). The area under the curve is equal to QALYs. A) When utility remains constant over time, QALYs increase over time. B) When utility increases over time, QALYs increase over time. C) When utility decreases over time, QALYs decrease over time.
longer lifespan during which the benefits of an intervention can accrue.\(^4\) Furthermore, interventions like clubfoot casting, closed reduction and casting of fractures, and Pavlik harness use for hip dysplasia are low cost and high utility, and therefore may have a high value.\(^4\) While this makes theoretical sense, it has not been confirmed in existing orthopaedic literature. There remains a dearth of CUAs and other health economic studies examining pediatric orthopedic interventions, and pediatric interventions in general. Jain et al. examined the management of adolescent idiopathic scoliosis and concluded that operative treatment was economically favorable; however, the findings were based on many assumptions over their analytical model’s 69-year lifespan.\(^13\) They noted that estimating QALYs in children is fraught with difficulties, including separating the success of an intervention from the natural resolution of a condition with aging. They also noted issues with combining pediatric and adult QALYs for the aging child who will become an adult, given the lack of validation of adult quality measures in children.\(^13\)

**Costs & Discounting**

From an economic perspective, a cost is any use of a resource, whether it be a material or a service.\(^8\) All costs should be represented using the same units like currency. Costs can be considered direct (e.g., medications, devices, and surgical fees) or indirect (e.g., facility costs, lost productivity, and wages).\(^8\) Of note, direct costs are not the same as charges, which include markups and can deviate substantially from the true cost of a material or service.\(^7\) Hospital charges can be used to back-calculate costs using cost-to-charge ratios; however, this method is historically inaccurate as it relies on population-level data, rather than direct measurement of patient-level costs.\(^15\) Unfortunately, true costs are often not readily disclosed by hospitals and vendors. Even when they are, the cost of a product or service can vary substantially between institutions.\(^39\) Despite these difficulties, direct costs are often obtained from reimbursement data, published literature, and estimates. This of course is problematic; Okike et al. showed that orthopedists correctly approximated the cost of orthopaedic implants only 17-21% of the time.\(^40\) Methods to consolidate cost information have been proposed, like the time-driven activity-based costing (TDABC) method, which involves making process maps accounting for the cost of all resources used across a complete cycle of care for a certain condition over time. While accurate, this method is intensive and requires a lot of time and resources to perform.\(^41\)

Estimating direct costs is similar for adult and pediatric populations. However, that is not the case for indirect costs, which are often more difficult to estimate in pediatric populations. In adults, one common method for determining indirect costs is the Human Capital Approach, which essentially sums the amount of lost income while out of work.\(^42\) For obvious reasons, this is not applicable to a non-employed child. Instead, one must take into account the indirect losses stemming from the child’s caregiver; for example, a parent missing work while their child is out of school.\(^43\) This becomes more complicated for prolonged illnesses—when the child grows old enough to enter the workforce, but is unable to do so secondary to their illness. How does one accurately calculate indirect losses not knowing which profession the child will go into?

These issues highlight the complexity and limitations of cost evaluation in the pediatric population. One potential solution is to focus solely on one indirect cost over a short time period. For example, Carabin et al. determined the indirect cost of common daycare-acquired infections in toddlers; they focused solely on the cost of finding a caregiver for the child over a 6-month window.\(^44\) While this strategy simplifies the model mathematics, it may not be appropriate for illnesses and interventions with longer natural histories.

With longer follow-up times, costs and health effects will diminish over time.\(^8,18,45\) This is based on the idea that people value current costs and benefits more than
future costs and benefits. Another way to rationalize this principle is through a simple monetary example—$100 today can be worth $102 next year if invested in a riskless government bond with 2% interest accrual, whereas $100 next year is only worth $100.\textsuperscript{45} Similarly, health today is valued more than future health; the resultant productivity may increase income or other commodities over time.\textsuperscript{18} Health economic evaluations adjust for these value changes over time by \textit{discounting} both costs and health effects.\textsuperscript{8,18,45} Discounting usually falls between 0-5\% per annum based on national guidelines, with Canada discounting at 1.5\% annually, U.S., Italy and Germany at 3\%, and Australia at 5\%.\textsuperscript{45,46} Of note, Belgium, Poland, Russia, and the Netherlands discount costs and health effects at different rates, but this is less common and often fraught with methodological issues.\textsuperscript{14} In the UK, the National Institute of Health Care Excellence (NICE) set discount rates at 3.5\% in 2004.\textsuperscript{45} In 2011, NICE released an amendment allowing for differential discounting at 3.5\% for costs and 1.5\% for health effects in cases where substantial treatment effects would last over 30 years.\textsuperscript{14} In the case of mifamurtide, a drug used to treat pediatric osteosarcoma, the transition to differential discounting brought the incremental cost effectiveness ratio down from £57000/QALY to £36000/QALY, demonstrating the impact that discounting has on pediatric economic evaluations.\textsuperscript{14} Discounting remains a controversial topic, with analysts continuing to debate discount rates, equal versus differential discounting, and whether discount rates should decline or remain constant over time.\textsuperscript{45}

**Cost-Effectiveness**

Once the QALY/costs has been determined from the value equation in a CUA, the inverse can be taken to derive the final unit: costs/QALY.

\[
\text{Value}_{\text{CUA}} = \frac{\text{Value}}{\text{Costs}} = \frac{\text{Utility} \times \text{Time}}{\text{Cost}} = \frac{\text{QALY}}{\text{Cost}}
\]

\[
1 = \frac{\text{Costs}}{\text{QALY}} = \frac{\text{Cost}}{\text{QALY}}
\]

Taking the inverse makes the unit more intuitive in that one is now asking how much an intervention costs to gain a single QALY. The additional cost per extra unit of effect is termed the Incremental Cost Effectiveness Ratio (ICER).\textsuperscript{47} This is usually compared to a societal willingness-to-pay threshold which is a monetary value assigned to a person’s willingness to pay for a reduction in risk.\textsuperscript{7} In the United States, this number has historically been between $50,000-$100,000 per QALY, with most authors using $50,000/QALY as a threshold for cost effectiveness.\textsuperscript{48} For example, Jain et al. performed a CUA looking at the cost-utility of operative versus nonoperative treatment of Adolescent Idiopathic Scoliosis (AIS) and found an incremental cost utility ratio of $20,600/QALY below the societal willingness-to-pay threshold of $50,000/QALY.\textsuperscript{13}

Besides calculating a cost/QALY below the willingness-to-pay threshold, another way to utilize health economic data to make a cost-effective decision is through a decision-analysis model.\textsuperscript{7} This is a way to organize probabilities of events and their outcomes to assess potential impacts of a clinical decision.\textsuperscript{8} In general, this is done by estimating the probability of a change in the patient’s health following an intervention, with this process repeated for multiple possible outcomes. This can be done using a decision tree, which flows unidirectionally, beginning with the decision of whether to implement the intervention (Figure 2). From there, the tree branches to include multiple subsequent outcomes with various treatment strategies, all associated with probabilities that are estimated from clinical, epidemiological, patient-centered, and economic data.\textsuperscript{8} Because these trees can become overly complex, they are best used for shorter time frames without recurrent health events. For longer time frames, and to model a disease in which the patient flows bidirectionally between health states, a Markov model may be utilized.\textsuperscript{49} In this model, patients are considered to be in discrete health states, and events are represented as transitions between health states. Similar to decision trees, probabilities and economic data can be applied. Unlike
Figure 2. Hypothetical example of a decision tree used in clinical decision analysis (incomplete for simplicity). Square nodes represent decision points. Circular nodes represent outcomes dictated by chance. For example, if delayed surgery is chosen, one can follow the decision tree to determine the probability of Complication A and subsequent Re-Operation.

decision trees, Markov models are usually evaluated by matrix algebra, allowing for more complex calculations and decision modeling. Despite this advantage, simple decision trees are frequently favored for a limited number of health state possibilities. Although they model only a finite number of health state transitions, when those health state transitions represent the large majority of real-world clinical situations, the accuracy gained by Markov modeling is unlikely to change the conclusions on whether an intervention is cost-effective. Adding more granular and time-varying outcomes may detract from a functional and coherent decision tree model that is applicable to the majority of patients undergoing treatment for a given condition. Therefore, the small proportion of patients who might experience these rarer outcomes would be unlikely to shift the model results.

Conclusion

The orthopaedic community is working hard to understand and minimize surgical costs, yet there remains a paucity of health economic analyses in the realm of pediatric orthopedic surgery. In particular, we lack comprehensive data that demonstrates the personal and societal benefits that are provided via contemporary treatment of pediatric orthopaedic conditions. Patient-centered outcome measures are an essential metric for determining value and should be the focus of value-related pediatric orthopaedic surgery research, yet orthopedic studies in children commonly focus on process measures such as mortality. When compared to analysis of treatment in adults, economic analyses in children differ in significant ways. Children have longer life expectancies; thus, value may accrue more profoundly over time. Outcome measures in adults often are not validated in children. Cost measurements are more challenging and less tangible in the pediatric population who do not work yet have many years of future earning potential.

In summary, clinicians and researchers are in the nascent stages of understanding value in pediatric orthopedic surgery, and work is needed to overcome shortcomings in current economic analyses when studying the pediatric population. Increasing our understanding and participation in health economic evaluation may allow pediatric orthopedic surgeons to partake in policy discussions that will shape the field going forward. As Professor Box taught, although all models are wrong, some are useful. Knowing how to create a useful model allows us to take charge of the narrative around cost and value in pediatric orthopaedics.
Additional Links
https://www.aaos.org/aaosnow/2020/jan/research/research03/
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References


