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Quality Assessment Of Economic Analyses in Pediatric Urology

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Abstract

Objective—To describe and evaluate economic analyses or economic evaluations in pediatric urological literature including study types such as cost-effectiveness analysis, are increasingly common in the medical literature.

Methods—We performed a systematic literature review of the MEDLINE, EMBASE, and Cochrane databases (1990–2011) to identify economic analyses of pediatric urologic topics. Studies were evaluated using published quality metrics. We examined the analysis type, data sources, perspective, methodology, sensitivity analyses, and the reporting of methods, results, limitations, and conclusions.

Results—We identified 2,945 non-duplicated studies, 60 of which met inclusion criteria. Economic analyses of pediatric urologic topics increased in number during the study period, from 1 study (2%) in 1990 to 7 (12%) in 2010 ($p < 0.0001$ for trend). The most common types of analyses were cost-effectiveness and cost-minimization (22 each, 37%), typically performed from the payer perspective (26, 43%). Although 44 (73%) correctly identified the analysis type, only 21 (35%) correctly identified the study perspective. Optimal data sources were used in 7 studies (11%). Appropriate inflationary discounting was used in 32% (17/53). Sensitivity analyses were not reported in 58% (31/53). The descriptions of study methods were adequate in 43 (72%); assumptions were adequately reported in 42 studies (70%). Most (37, 62%) adequately discussed limitations.

Conclusions—Although economic analyses are increasing in the pediatric urologic literature, there is a need for standardization in methods and reporting. Future investigations should attempt to follow standardized reporting guidelines and should pay particular attention to reporting of methods and results, including a comprehensive discussion of limitations.

Keywords

Cost Effectiveness; Cost Analysis; Pediatrics; Urology; Systematic Review

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INTRODUCTION

The concept of an economic analysis is an intuitive one; in health care as in most other areas, it seems only logical to maximize effectiveness while preserving value for patients. Unfortunately, the practical, “nuts and bolts” aspects of economic analyses are less intuitive, and these studies can be very challenging to execute in a methodologically sound manner. There is no single analytical or data-gathering technique that is perfectly suited for all questions and situations. Despite differences, however, guidelines do exist and serve as the basic standard by which economic analyses should be conducted and reported.

Economic analyses can be classified based on what they do (and do not) consider. When classifying these studies, Drummond *et al.*(7) recommended asking two questions (Table 1): first, is there a comparison of 2 or more alternative treatments? Second, are both costs and consequences (outcomes or effectiveness) of the alternatives examined? If the answer to both of these questions is “Yes”, then the analysis is considered to be a Full Economic Evaluation; these include cost-effectiveness (CEA), cost-utility analysis, and cost-benefit analysis. These three full evaluation types differ primarily in their measure of effectiveness (specifically, whether effectiveness is measured in effectiveness outcomes, quality-adjusted life, or in monetary form respectively). By contrast, if the answer to either of the above two questions is “No”, then the analysis is only a Partial Economic Evaluation. These include: cost-description, -identification, -outcome or -consequence if there is only one treatment studied and cost-analysis, -minimization or -comparison if only the costs (not the outcomes) of two or more competing treatments are compared.

In 1996, the United States Preventative Services Task Force convened the Panel on Cost-Effectiveness in Health and Medicine in order to develop consensus guidelines for economic analyses in medicine.(1) The Panel’s recommendations are widely considered to be the gold standard for how to perform economic analyses in healthcare. In brief, the Panel’s intent was to improve the quality of these analyses and facilitate comparison between studies. Despite these guidelines, however, the quality of economic analyses in urology is variable. While many studies are well-designed and executed, others use methods and reporting which do not conform to Panel guidelines. In particular, many manuscripts that are advertised as ‘cost-effectiveness’ studies do not in fact meet the working definition of a CEA.

The purpose of this report is thus to describe and evaluate the quality of contemporary economic analyses in the pediatric urologic literature.

METHODS

Search strategy

We performed a systematic review of studies in accordance with published recommendations.(2, 3) We identified studies published in English between January 1990 and June 2011 to identify economic analyses in pediatric urology. We searched the MEDLINE, EMBASE, and Cochrane electronic databases using the MeSH search terms “pediatric” or “child” and “cost,” “cost-effectiveness” or “economic” and then limited the results to the MeSH search terms “urology,” “urogenital system,” “male urogenital disease,” “female urogenital disease,” “urogenital abnormalities,” “urogenital neoplasms,” and “urogenital surgical procedures.” The resulting studies were screened to determine whether each dealt with a pediatric urologic topic and could conceivably meet the definition of an economic analysis. Reference lists of included studies were screened for missed studies. We excluded studies that primarily considered non-urologic topics such as sexually transmitted infections, medical-renal conditions, or studies where healthcare costs were an aside rather

than an integral component of the study. In cases of ambiguity, or where study reporting made evaluation difficult, we attempted to err on the side of inclusiveness.

Data Abstraction and Quality Assessment

Two authors (PJK and JCR) independently evaluated each study using a predetermined and pilot tested (on 4 unrelated economic evaluations) instrument derived from two previously published reports of quality assessment metrics for economic analyses (Appendix 1).^(4, 5) These quality metrics primarily focus on study methodology (*e.g.*, Was discounting used? If so, was it used appropriately?). Differences were resolved by consensus discussion, as recommended by previous publications.⁽⁶⁾ We additionally abstracted the publication year, country of origin, journal, research sponsor, corresponding author specialty and affiliation, analysis type, perspective, sources of both cost and effectiveness data, and source of utility values, if appropriate. Key points of our evaluation included accuracy of the study title and/or stated analysis type, stated perspective, and study methodology. All quality assessments were ultimately based on published national guidelines.⁽¹⁾ An overall assessment was made by counting the proportion of included quality criterion. Descriptive statistical analyses were performed using SPSS version 18 (Chicago, IL).

RESULTS

Description of Included Studies

We identified 2,945 non-duplicated studies, 72 of which were reviewed in full (Figure 1). Of these, 60 economic analyses met our inclusion criteria and were included in the systematic review (Appendix 2). Economic analyses of pediatric urologic topics increased in number during the study period, from 1 study (2%) in 1990 to 7 (12%) in 2010 ($p < 0.0001$ for trend, Figure 2). The majority of studies (45, 75%) originated from the United States, and the most common journal of publication (25, 42%) was the *Journal of Urology*. Most studies (43, 71%) did not indicate any financial sponsorship or support. Most corresponding authors (54, 90%) reported an academic affiliation; half (30, 50%) were urologists and the remaining were pediatric surgeons ($n=5$), nephrologists ($n=4$), pediatricians ($n=4$) and other/unknown ($n=17$). The most frequent topic was vesicoureteral reflux (22%), followed by urinary tract infection and circumcision (Table 2).

Quality of Included Studies

The most frequent actual types of economic analyses were CEA ($n=22$, 37%) and cost-minimization (also known as “cost-analyses”) studies ($n=22$, 37%). The remainders were cost-description studies (15, 25%), with the exception of one study whose stated methods were insufficiently complete to allow us to determine the study type. There were, 24 studies described by authors as CEA, however only 19 (79%) of these were truly CEA. The other studies described by authors as CEA were actually cost-analysis (4) or cost-description (1) studies. Conversely, there were three studies that were actually CEA even though they were described as cost-minimization ($n=2$) or cost-benefit ($n=1$) by authors. 15 of 19 (78%) studies referred to by the authors as “cost-analyses” actually met the definition of cost-analysis studies. Overall, study authors accurately described the study type in 44 of 60 (73%) studies.

Analyses were most frequently performed from an apparent payer perspective (26, 43%), with societal (10, 17%) and hospital (10, 17%) perspectives being the next most common. The analytic perspective was not stated in the majority of studies (36, 60%). Among the 24 studies that did identify an analytic perspective, 3 (13%) did not select cost sources consistent with their perspective. In two cases, study authors stated they used a hospital perspective, but their methods were that of a payer perspective. In an additional case, the

authors stated they used a societal perspective but included mixed cost sources that were incompatible with any single analytic perspective (hospital, societal, payer, or otherwise).

Among studies involving measures of effectiveness (n=49), the most common source of effectiveness data was local experience (21, 43%). The gold standard of effectiveness data, a systematic review of the literature, was used by only 5 (10%) studies. Randomized trial outcomes data were used by 2 studies (4%). An additional 19 studies (39%) used a more limited, non-systematic literature review to derive effectiveness data. The 2 remaining studies (4%) used an unclear source.

Most studies (21, 35%) used a combination of multiple cost data sources. The most common single source of “cost” data (12, 20%) was in fact charge (not cost) data; these data were typically obtained from billing records. Governmental reimbursement data was used in 8 studies (13%), payments for payer in 3 (5%), and RCC adjusted charges in 2 (3%), while only 4 (7%) used micro-costing. Ten studies (17%) did not provide a source of cost data. Similarly, of the 53 studies that spanned more than one year, 31 (58%) did not adjust costs on an annual basis. In total, 17 studies (32%) adjusted costs annually using an appropriate rate for the stated (or actual) analytic perspective.

Among the 10 cost-utility analyses, the most common source of utility data was author estimate (4, 40%), while 2 studies (20%) based their utility values on published values for similar health states and 1 (2%) with expert proxy. The gold standards for deriving utility values, direct or indirect elicitation methods, were used by 3 studies (30%). In total, only 18 studies (30%) used the best available sources for all parameter estimates, including cost, effectiveness, and utility values.

Model validation is a critical aspect of economic analyses for studies that are based on Markov or other modeling techniques. Of the 30 studies (50%) that used Markov models or decision trees to generate their economic comparisons, only 3 (10%) included an adequate description of the validation of their model.

Sensitivity analyses or other statistical methods to control for uncertainty were infrequently performed. Not including the 10 cost description studies – which do not require sensitivity analyses since no comparison of competing choices is made – 29/50 (58%) studies did not report any significant sensitivity analyses of cost and/or effectiveness data. Many did not explore the influence of both costs and effectiveness inputs (only 14/22 or 64% of CEA’s included both) and the more comprehensive type of sensitivity analysis, a probabilistic sensitivity analysis, was performed in only 5 studies (10%).

Most studies followed acceptable reporting standards. The title accurately reflected the study type in 44 studies (73%), and the objectives were clearly presented in 56 studies (93%). A reference case was appropriately presented in 46 studies (77%), and a competing choice analysis was appropriately presented in 44 (73%) studies. The descriptions of analytic methods were overall appropriate in 43 studies (72%), and the study assumptions were adequately described in 42 (70%) studies. More than half of studies (37, 62%) adequately discussed the limitations of their analysis. The conclusions of 49 studies (82%) were justified based on the study findings, and 36 (80%) studies noted and discussed their results in a broader policy context.

To summarize study quality, each study was assessed for inclusion of certain key criteria (with no weight given to the relative importance any particular criteria). 38 studies (63%) incorporated 50% or more of the assessed criteria, 17 studies (28%) incorporated 75%, and only 6 studies (10%) included 90%.

Comment

The term ‘cost-effective’ is increasingly used within the pediatric urologic literature. Given the complexity of economic analytic methods and nomenclature, it is unsurprising that these studies are challenging to perform and to review without training and experience in these methodologies.(8) Furthermore, it is difficult to provide cursory reporting guidelines that can adequately cover all economic analyses types. As previously reported in other healthcare fields,(9–13) many economic analyses in pediatric urology do not meet accepted reporting standards. Perhaps most concerning is that 58% of studies did not report any sensitivity analyses. Sensitivity analyses are a critically important component of economic analyses, and their absence makes critical evaluation and policy implications difficult to impossible.

Framework for an Economic Analysis

Any discussion of healthcare costs necessarily begins with consideration of who bears those costs. From a societal perspective, the term “costs” implies the resources consumed by medical conditions or treatments thereof, including resources such as patient and parental time and transportation. From a payer perspective, by contrast, these particular costs are irrelevant. Whether a parent misses one hour or one month of work caring for their child is meaningless from the point of view of their insurance company or of the treating hospital. While it may be in the interest of an individual payer or institution to know the direct medical costs of a particular treatment, when considering medical care from a policy standpoint a societal perspective (which includes both direct and indirect medical costs) is preferred. This is principally because the resource use from a societal perspective is the only way to examine the broad social costs and benefits of health care services. Furthermore, differences in included costs can make it difficult to compare studies conducted from different perspectives. For these reasons the 1996 United States Panel on Cost-Effectiveness recommends a “reference case” analysis from a societal perspective in addition to any other analytic perspective.(1)

Information on costs and effectiveness should be used to create an economic model. In general, estimates of clinical effectiveness for use in models should be based on a systematic review of the literature. In some cases, these estimates may also be based on clinical trial outcomes, or if no published data is available, derived from expert opinion. Similarly, “cost” data should represent true medical costs, ideally from direct costing of all consumed input resources (*e.g.*, microcosting); alternatively, in some cases accurate estimates of medical costs may be obtained using cost-to-charge conversion factors or other well-established sources such as governmental payer data. Billing (or charge) data does not reflect true medical costs and should be avoided.

Quality-of-life adjustments are often difficult to perform since utility values are frequently unavailable. When possible, these values should be obtained from formal econometric methods. When author estimates are used, wide sensitivity analyses should be included and we would recommend performing the analysis with and without quality adjustment.

When the costs and effects of medical interventions extend beyond one year, these inputs should be annually discounted to reflect the present value. The recommended discounting rate is 3–5% for a societal perspective.

The value and source of model inputs should be presented clearly so that readers might critically assess the quality of the inputs. More importantly, sensitivity analyses on all model inputs should be performed. These will allow a description of which inputs hold particular influence over the outcome of the analysis, especially when these values are derived from “expert opinion” rather than rigorous scientific investigation. Manually adjusting a single

input value over a broad range of values (one-way sensitivity analysis) will allow for determination of threshold values where the model conclusions would change. Two-way, three-way, and probabilistic type sensitivity analyses can further evaluate the stability of model conclusions.

Limitations

Our study should be considered in light of its limitations. As with any systematic review, we were limited by the available data from the included studies. We may have over- or underestimated the methodological quality of the included analyses, particularly in instances where methodological details were not provided. However, we would note that reporting problems are indeed one of the focal points of the study, as one of our goals was to identify areas for future improvement.

Additionally, judging the quality of economic analyses can be subjective.⁽⁶⁾ We accounted for this by performing all quality assessments in duplicate and resolving differences by consensus after performing a “training run” on 4 unrelated economic evaluations. Although this is the recommended method for judging quality data,⁽⁶⁾ it is possible that our judgments reflect a shared viewpoint rather than true methodological “quality.” Similarly, our assessment of overall quality was not based on any particular universal standard since the methods chosen should reflect the scope and intent of the analysis.

CONCLUSIONS

Although the use of economic analyses is increasing in the pediatric urologic literature, there is a need for standardization in methods and reporting. Future investigations should attempt to follow standardized reporting guidelines and pay particular attention to explanation of methods/results and a comprehensive discussion of limitations.

Supplementary Material

Refer to Web version on PubMed Central for supplementary material.

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FIGURE 1.

Flow diagram for search and selection process for included studies. Title screening and abstract review allowed exclusion of non-urologic topics and studies that were not exclusively pediatric.

FIGURE 2.
Graph showing the increasing number of economic analyses in the pediatric urology literature from 1990 to early 2010.

TABLE 1

Classification of Economic Analyses. When only a single intervention or only cost differences between two or more treatment options are considered, the study is classified as a Partial Economic Evaluation. Alternatively, when differences in both costs and outcomes between two or more options are considered, the study is one of the types of Full Economic Evaluations. (Adapted from Drummond et al. (7))

	Effectiveness Not Considered	Effectiveness Is Considered
Single Treatment or Intervention Considered	Partial Evaluation: Cost-description Cost-identification	Partial Evaluation: Cost-consequence Cost-outcome Description
Two or More Treatment or Intervention Options Compared	Partial Evaluation: Cost-comparison Cost-minimization Cost-analysis	Full Evaluation: Cost-effectiveness Cost-utility Cost-benefit

TABLE 2

Subjects of Included Economic Analyses

Subject	N (%)
Vesicoureteral Reflux	13 [^] (22%)
Urinary Tract Infection	10 (17%)
Circumcision	9 (15%)
Ureteropelvic Junction Obstruction	7 (12%)
Inguinal Hernia	5 (8%)
Cryptorchidism	4 [*] (7%)
Hypospadias	3 [*] (5%)
Congenital Adrenal Hyperplasia	2 (3%)
Varicocele	2 (3%)
Wilms' Tumor	2 (3%)
Bladder Exstrophy	1 (2%)
Multicystic Dysplastic Kidney	1 (2%)
Nocturnal Enuresis	1 (2%)
Ureterocele	1 [^] (2%)
Vaginal Agenesis	1 (2%)

^{*} Includes a shared article on cryptorchidism and hypospadias

[^] Includes a shared article on vesicoureteral reflux and ureterocele