



**The Hospital for Sick Children  
Technology Assessment at SickKids (TASK)**

**WILL THE GROWTH SPURT CONTINUE? TRENDS IN  
CHILD HEALTH ECONOMIC EVALUATION:  
1980 TO 2013**

**Report No. 2016-01**

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**REPORT HIGHLIGHTS**

*The Report Highlights consists of a summary of the full report with the same name and should be evaluated in conjunction with the full report. Full documents are available for download at:*

**<http://lab.research.sickkids.ca/task/reports-theses/>**

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### **Conflicts of Interest**

The authors have no conflicts of interest to disclose.

## Introduction

The [Pediatric Economic Database Evaluation \(PEDE\) project](#) was developed to identify methodological gaps in order to improve the quality of pediatric economic evaluations. PEDE is a publicly available, searchable, comprehensive database of pediatric economic evaluations published since 1980.<sup>1,2</sup> Trends in pediatric economic evaluations highlight areas of recent growth in the field as well as identify areas for future methodological developments in pediatric health economics. Monitoring trends could help advance methodologies in child health economic evaluation and ultimately enhance pediatric healthcare decision-making.

## Objectives

The objectives of this study are to report and evaluate trends in pediatric health economic evaluation over the period of 1980 to 2013. The analysis compared study characteristics between two periods: 1980 to 1999 (early period) and 2000 to 2013 (late period).

## Key Messages

- A total of 2,630 published pediatric health economic evaluations were identified through PEDE between 1980 and 2013, indicating that the field of pediatric economic evaluation continues to grow.
- Substantially more CEAs and CUAs are being published compared with CBAs and CMAs (64.5% and 24.0% versus 7.7% and 3.7%, respectively) and this trend appears consistent regardless of the type of intervention, disease or age group being studied.
- Statistically significant changes in the distributions of analytic technique, journal type, intervention type and age groups were observed when comparing early (1980-1999) and late (2000-2013) periods.
- The main focus of pediatric health economic evaluations has consistently remained infectious diseases at 29.2%. This focus may not align with emerging child health priorities and target populations, such as adolescent health, injury, developmental disabilities, mental health, and the use of personalized medicine.

## Methods

The analysis was conducted using the [PEDE database](#). A publication is eligible for PEDE if one or more comparators exists and descriptions of both costs and health outcomes are present. Detailed inclusion and exclusion criteria for determining eligibility are provided elsewhere.<sup>1</sup> PEDE is updated annually using custom search strategies for retrieval of citations designed to achieve high sensitivity. Economic and medical literature citation databases routinely searched include MEDLINE, CINAHL, EMBASE, IPA, EconLit, the Cochrane Collection, NHS EED, DARE, HTA, and ERIC. Over 73 web sites of health technology assessment agencies and research groups are also searched for inclusion of eligible grey literature.

Trends in the pediatric economic evaluations were explored by performing one-way frequency distributions and two-way cross-tabulations on variables within the database. Descriptive statistics were used to describe the key characteristics of publications including publication year, disease category, intervention type, outcome measures, age group and target population. The trend analysis considered two main periods: 1980 to 1999 (early period) and 2000 to 2013 (late period). Study characteristics were compared between periods using a chi-squared statistic.

## Results

Between 1980 and 2013 a total of 2,630 pediatric economic evaluations were published, with numbers steadily increasing over time. The average annual increase in publication volume between 1980 and 2013 was 15.2%. Of the 2,630 economic evaluations included in PEDE, 64.5% are CEAs, 24.0% are CUAs, 7.7% are CBAs, and 3.7% are CMAs. The distribution of the types of analyses changed significantly over time ( $X^2 p < 0.0001$ ). Both CEAs and CUAs increased over time while the CMAs and CBAs have decreased (Figure 1). Pediatric economic evaluations were most often published in subspecialty medicine journals and pediatrics/perinatal medicine journals (35.3% and 26.2%, respectively). The most frequently studied age group was the child (between 1 and 12 years of age) at 40.6% of publications, which was consistent across both early (1980-1999) and late periods (2000-2013) (Table 1).

Economic evaluations were most frequently conducted for health prevention, health treatments and detection interventions accounting for 31.7%, 24.2% and 15.9% of the total, respectively.

The overall distribution of the type of intervention on which economic evaluations are conducted changed significantly over time ( $\chi^2$   $p < 0.0001$ ). Evaluations of health prevention interventions increased from 25.0% to 34.7% between the early and later periods, while small decreases ( $\leq 5\%$ ) were observed for other types of interventions (Table 2). CBAs were most frequently conducted for health prevention interventions (49.8%), CEAs were most frequently conducted for health treatment and health prevention interventions (25.5% and 24.2%, respectively), CUAs were most frequently conducted on health prevention interventions (50.5%) and CMAs were most frequently conducted for health treatment interventions (41.8%).

A wide variety of outcomes ( $n=1,280$ ) were measured across the 2,630 publications in PEDE. The most frequent types of primary outcomes were QALYs or similar units. Cases of non-infectious disease/condition/abnormality, cases of infectious disease/condition/abnormality and life years accounted for 22.0%, 18.6%, 13.6% and 13.2% of the total, respectively. When comparing the early (1980-1999) and late periods (2000-2013), the use of QALYs increased from 4.9% to 29.5%, paralleling the increased frequency of CUAs (Table 3).

Established disease classifications schema were applied to the publications in PEDE. Over the period covered by the database (1980-2013), both ICD-9-CM and ICD-10 schema<sup>3,4</sup> were used and were merged in this analysis. Overall, infective and parasitic diseases were most frequently studied in pediatric populations (29.2%), a trend which was consistent in both early and late periods. The next most commonly studied conditions were those of pregnancy, childbirth and the puerperium at 7.2%. The top ten target populations covered 42% of the studied populations and were: universal, defined as all individuals (10.1%), pregnant women (9.8%), children (5.6%), neonates (3.9%), vaccinated persons (2.9%), cancer patients or individuals at risk of cancer (2.5%), patients with asthma (2.4%), patients with or at risk of HIV (2.2%), patients with cardiac abnormalities (1.8%) and patients with or at risk of rotavirus (1.4%).

## Discussion

A substantial increase in the volume of publications was observed between 1980 and 2013. This growth paralleled the increasing acceptance and capacity for conducting health economic evaluations in adult health.<sup>5</sup> The rise in QALYs as a frequently reported outcome is consistent with the observed rise of CUAs. This suggests that there has been increased attention to

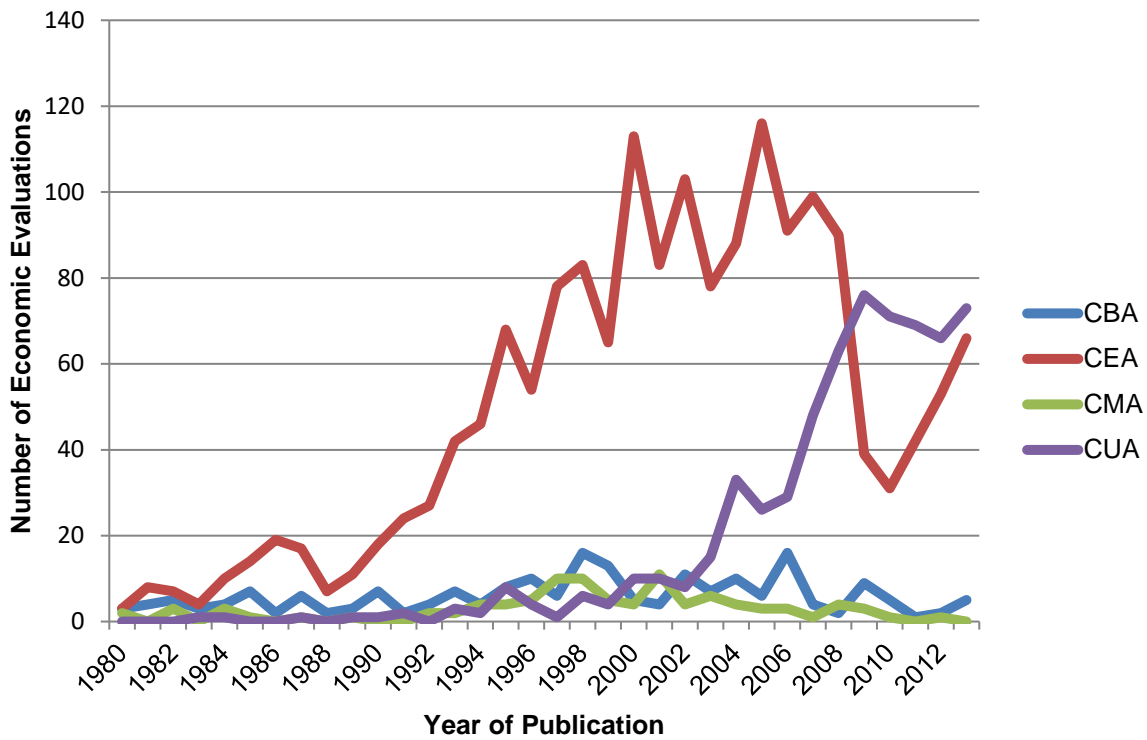
guidelines stipulating the use of CUA,<sup>6,7</sup> but measuring and reporting child health utilities continue to be a challenge.<sup>8-10</sup> In recent years there have been advancements in the development of pediatric-specific health state classification schemes and underlying preference weights which may help to improve the frequency and quality of pediatric CUAs.<sup>11-13</sup>

Some of the limitations associated with the development of the PEDE database were previously described<sup>1,14</sup> but include those related to development of search strategies, data extraction approaches and completeness of the database. Despite a small potential for missing citations, the multi-stage exhaustive search strategy employed in the building of the PEDE database, has resulted in a database that is more comprehensive and inclusive than existing ones with respect to pediatric economic evaluations.

Pediatric health economic evaluations are frequently conducted in the field of infectious diseases but expanding pediatric economic evaluations to focus on other disease areas and target populations to support emerging child health priorities such as non-infectious diseases, chronic conditions, mental health initiatives and advances in personalized medicine should be considered.<sup>15</sup> A recent analysis of data from the WHO's Global Health Observatory suggests that the contribution of infectious diseases to child mortality is likely to decline while congenital anomalies, non-communicable diseases and injuries are likely to increase.<sup>15</sup> The new Global Strategy for Women's, Children's and Adolescents' Health that is being developed in collaboration with the United Nations has identified adolescent health needs as an emerging priority.<sup>16</sup> The current innovations in the field of pharmacogenomics and personalized medicine also promise to impact future child health prevention strategies and treatments.<sup>17</sup> Economic evaluations of these initiatives will contribute to robust and evidence-informed policy decision-making in the field of child health.

The field of pediatric health economic evaluations continues to grow, supporting the need for the ongoing maintenance and updating of PEDE for pediatric researchers, health practitioners and those engaged in health technology assessment and systematic reviews. Ongoing analyses of the PEDE database will contribute to greater understanding of current approaches to pediatric health economic evaluation and an awareness of the need for more robust methodologies. This analysis of trends in pediatric health economic evaluations between 1980 and 2013 will further allow researchers and policymakers to identify areas of research needed to support informed decision-making in the field of child health.

**Figure 1. Distribution of economic evaluations in PEDE by year (1980-2013)**



**Table 1. Publications in early and late periods by age group (n=2630 records)**

Age Group	Early (1980-1999)			Late (2000-2013)			TOTAL	
	n	col%	row%	n	col%	row%	n	%
<b>Perinate</b>	162	13.1%	54.9%	133	5.1%	45.1%	295	7.6%
<b>Neonate</b>	171	13.8%	27.1%	460	17.5%	72.9%	631	16.3%
<b>Infant</b>	240	19.4%	34.6%	454	17.3%	65.4%	694	18.0%
<b>Child</b>	442	35.7%	28.2%	1126	42.9%	71.8%	1568	40.6%
<b>Adolescent</b>	190	15.3%	31.8%	408	15.5%	68.2%	598	15.5%
<b>Adult*</b>	34	2.7%	44.2%	43	1.6%	55.8%	77	2.0%
<b>TOTAL</b>	1239	100.0%	32.1%	2624	100.0%	67.9%	3863	100.0%

Each record may contain multiple age groups; a total of 3863 age groups were studied.

\*Adults consist of pregnant women or mothers administered an intervention aimed at improving health in the offspring, with outcomes measured in offspring.

**Table 2. Publications in early and late periods by intervention category**

Intervention Category	Early (1980-1999)			Late (2000-2013)			TOTAL	
	n	col%	row%	n	col%	row%	n	%
Dental	31	3.8%	45.6%	37	2.0%	54.4%	68	2.6%
Detection	150	18.5%	35.9%	268	14.7%	64.1%	418	15.9%
Diagnosis	25	3.1%	32.5%	52	2.9%	67.5%	77	2.9%
Educational	15	1.9%	19.0%	64	3.5%	81.0%	79	3.0%
Health care delivery	55	6.8%	32.4%	115	6.3%	67.6%	170	6.5%
Health program	90	11.1%	42.3%	123	6.8%	57.7%	213	8.1%
Health treatment	196	24.2%	30.8%	441	24.2%	69.2%	637	24.2%
Health prevention	202	25.0%	24.2%	631	34.7%	75.8%	833	31.7%
Surgical	45	5.6%	33.8%	88	4.8%	66.2%	133	5.1%
<b>TOTAL</b>	<b>809</b>	<b>100.0%</b>	<b>30.8%</b>	<b>1819</b>	<b>100.0%</b>	<b>69.2%</b>	<b>2630</b>	<b>100.0%</b>

**Table 3. Publications in early and late periods by outcome category**

Outcome Category	Early (1980-1999)			Late (2000-2013)			TOTAL	
	n	col%	row%	n	col%	row%	n	%
Cases of complications/ adverse events	66	8.2%	44.9%	81	4.4%	55.1%	147	5.6%
Cost	0	0.0%	0.0%	26	1.4%	100%	26	1.0%
Cases of cures/improvements/healing	90	11.1%	49.7%	91	5.0%	50.3%	181	6.9%
Cases of infectious disease/condition/abnormality	153	18.9%	42.9%	204	11.2%	57.1%	357	13.6%
Cases of injury	9	1.1%	45.0%	11	0.6%	55.0%	20	0.8%
Life years	110	13.6%	31.6%	238	13.1%	68.4%	348	13.2%
Cases of non-infectious disease/condition/abnormality	200	24.7%	40.8%	290	15.9%	59.2%	490	18.6%
Other	6	0.7%	24.0%	34	1.9%	85.0%	40	1.5%
Changes in physiologic measure	75	9.3%	37.1%	127	7.0%	62.9%	202	7.7%
Changes in behavioural/psychosocial	21	2.6%	25.9%	60	3.3%	74.1%	81	3.1%
QALYs, or similar unit	40	4.9%	6.9%	538	29.5%	93.1%	578	22.0%
Changes in quality of life	3	0.4%	14.3%	18	1.0%	85.7%	21	0.8%
Surrogate health outcomes	5	0.6%	17.9%	23	1.3%	82.1%	28	1.1%
Time outcomes*	18	2.2%	24.3%	56	3.1%	75.7%	74	2.8%
Unspecified	0	0.0%	0.0%	6	0.3%	100%	6	0.2%
Cases of vaccination	13	1.6%	41.9%	18	1.0%	58.1%	31	1.2%
<b>TOTAL</b>	<b>809</b>	<b>100%</b>	<b>30.8%</b>	<b>1806</b>	<b>100%</b>	<b>68.7%</b>	<b>2630</b>	<b>100%</b>

\*This category refers to days in a state or days absent from a state, time to achieve an outcome or to recover



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