The Quality of Paediatric Cost-Utility Analysis Studies: 1997 to 2006

SK Kromm, J Bethell, F Kraglund, S Edwards, WJ Ungar, A Laporte, PC Coyte

SickKids

Health Policy, Management & Evaluation
UNIVERSITY OF TORONTO

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Cost-utility analysis (CUA) (I)

- One analytic technique of economic evaluation
- Health benefits quantified as single index of quality of life and years of life:
  - Quality-Adjusted Life-Years (QALYs), e.g.,
    - Preference weight: 0 to 1
    - 3 yrs in perfect health (‘preference weight’ 1.0) = 3.0 QALYs
    - 3 yrs with type 2 diabetes (‘preference weight’ 0.75)[1] = 2.25 QALYs

1) Dijkstra, R F, et al, *Diabet Med*,2006-Feb; 23(2):164-70

Cost-utility analysis (CUA) (II)

- Allows direct comparison, across diseases and populations using the incremental cost-utility ratio (ICUR), e.g.:
  - Intervention A vs Intervention B
    - (Cost A - Cost B) / (QALYs A - QALYs B)
    - = ($15,000 – $10,000)/(12 QALY – 7 QALY)
    - = ($5,000)/(5 QALY)
    - = $1,000 per additional QALY gained

Cost-utility analyses in children

- Challenging, because of problems measuring and valuing paediatric QALYs
  - Rapid developmental and functional changes
  - Unique patterns of health, disease and disability
  - Cognitive abilities
  - Dependence on parents

Descriptive & analytic

1. Describe published paediatric CUA studies:

1.1 Characteristics

1.2 Quality

2. Analyse factors associated with quality in published paediatric CUA studies

Study selection

Source: Paediatric Economic Database Evaluation (PEDE)

- http://pede.ccb.sickkids.ca/pede/
- Over 1,700 economic evaluation citations, from 1980 to 2006
- Evaluation of interventions directed at:
  - paediatric population [neonates → adolescents (<19 years)]
  - pregnant women or mothers (if outcomes measured in offspring)

Sample definition:

- Analytical technique = cost-utility analyses
- Publication date = 1997 to 2006 (10 years)
- n = 139 (<10% of titles in PEDE database)

Measures

Descriptive information:

- Study characteristics, e.g., assessment method, instruments used, use and type of proxy respondents, & preference source

Paediatric Quality Appraisal Questionnaire (PQAQ)

- Validated instrument (Ungar & Santos, 2003)
- 57 items divided into 14 domains
  - 11 items record descriptive information
  - 46 items rate quality (scale of 0 – 1)
  - Domain scores = unweighted mean of items within domain

Inter-rater Reliability

1) Inter-rater reliability:

- 10 papers reviewed by all four raters to quantify agreement

2) Analysis of reliability:

- Intraclass correlation coefficient (ICC) for domain scores
- Kendall’s coefficient of concordance for global rating

Reliability analysis

PQAQ Domain	Intraclass Correlation Coefficient (ICC)
1. Economic evaluation	0.52
2. Comparators	0.36
3. Target population	-0.18
4. Time horizon	0.35
5. Perspective	0.90
6. Costs & resource use	0.42
7. Outcomes	0
8. Quality of life (QoL)	n/a
9. Analysis	0.53
10. Discounting	0.80
11. Incremental analysis	0.29
12. Sensitivity analysis	0.79
13. Conflict of interest	0.67
14. Conclusions	0.64

Reliability analysis

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Data Extraction / Analysis

1) Data extraction:

- 139 papers divided among four raters (1 rater/paper)
- 21 articles excluded (e.g., systematic reviews, unavailable)
- **118 pediatric CUA studies reviewed**

2) Descriptive analysis:

- Univariate statistics (mean, sd, min, max) for domain scores
- Frequencies & percentages

3) Hypothesis testing:

- T-test and linear regression for domain score outcomes

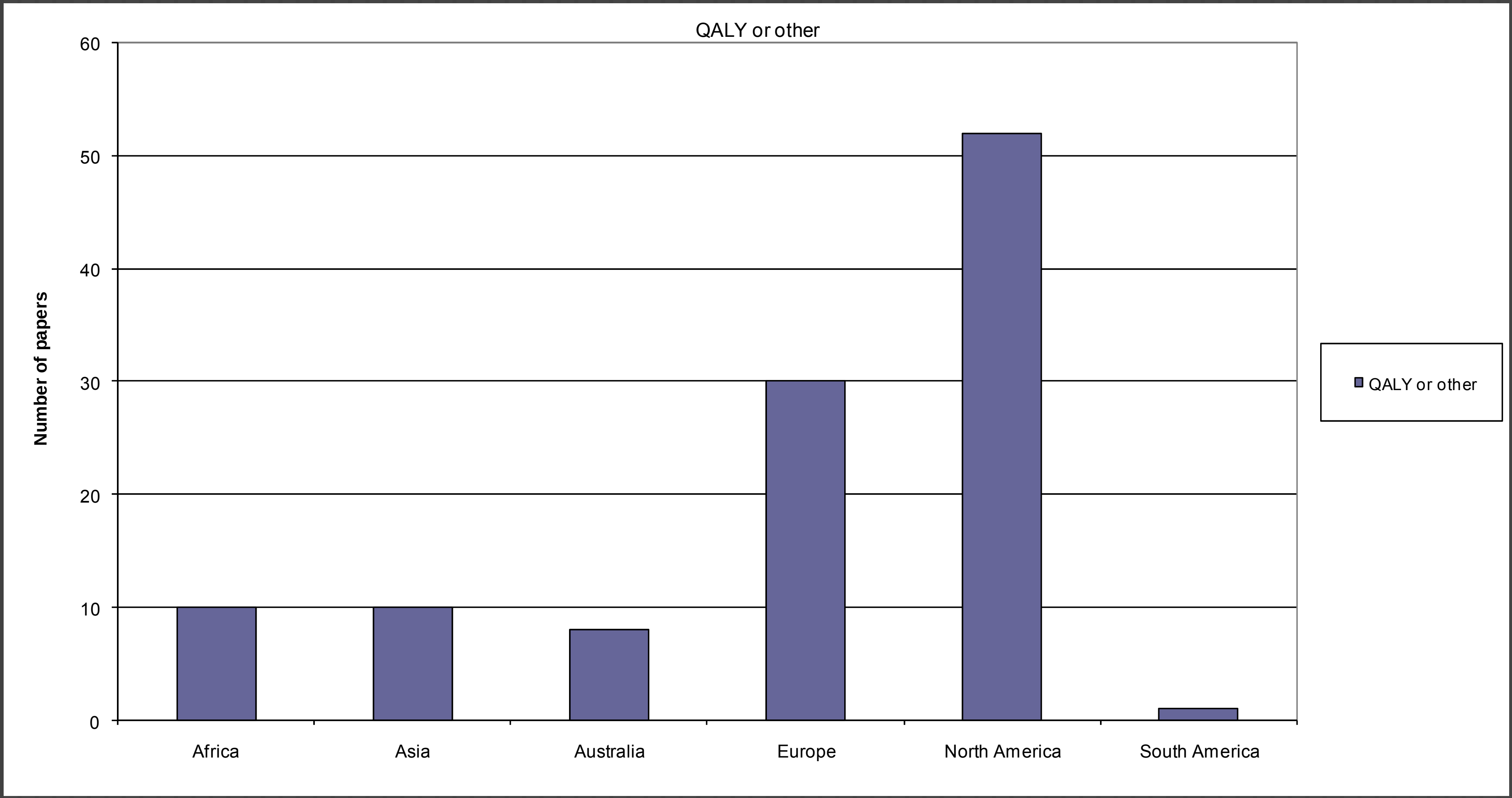
Descriptive: Study sample

- Type of study:
  - decision analysis (76%)
- Study perspective:
  - societal (55%)
- Intervention type:
  - immunization (34%)
  - pharmaceutical (20%)
- Quality of life considered:
  - child's alone (76%)
- Source of preference weights:
  - author/expert/lit. (42%), parent proxy (9%)
- Region of study population:
  - 44% US
- Health condition
  - Infectious disease (49%)

Descriptive: Source of preference

Author, year	Preference weight source	Preference weight, by impairment level (neonatal herpes)	
		Moderate	Severe
Little et al, 2005	Literature (clinicians, parents and adolescents, using standard gamble)	0.9	0.3
Thung et al, 2005	Literature (?)	0.5	0.1
Baker et al, 2004	Literature (clinicians, using EuroQoL)	0.52	0.16

Descriptive: Region of study population

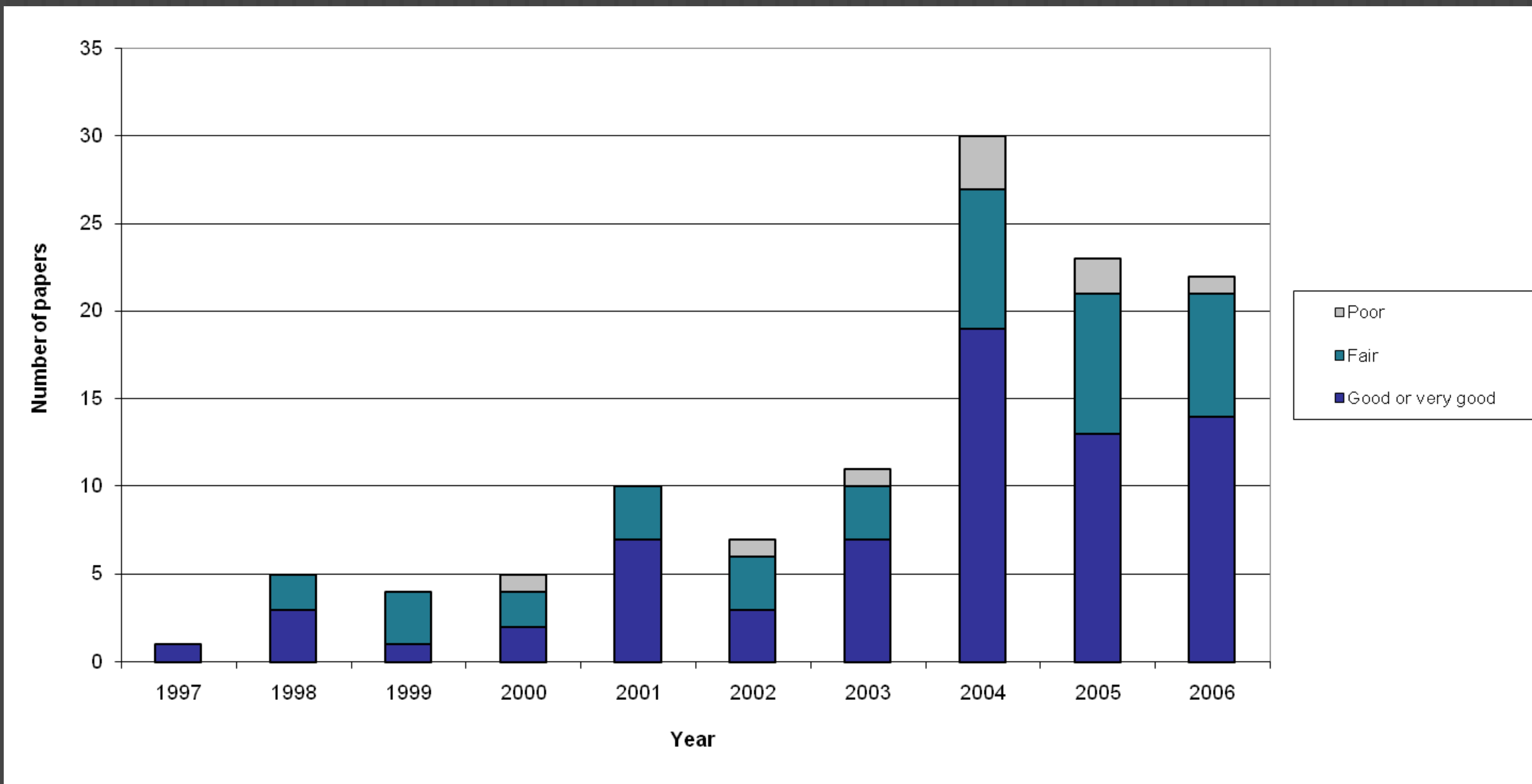


Descriptive: Quality of CUAs

PQAQ Domain	Domain score statistics			
	Mean	Standard Deviation	Minimum	Maximum
Economic evaluation	**0.83**	0.21	0.25	1.00
Perspective	**0.65**	0.36	0.00	1.00
Analysis	**0.51**	0.19	0.08	0.92
Discounting	**0.86**	0.34	0.00	1.00
Sensitivity analysis	**0.68**	0.32	0.00	1.00
Conflict of interest	**0.51**	0.43	0.00	1.00
Conclusions	**0.65**	0.22	0.17	1.00

Descriptive: Global Rating

Number of papers and global rating by year



Hypothesis Testing

Study quality a function of:

- Population age (≥5 years vs. 0-4 years)
- Journal type (methodological vs. clinical)
- Year of publication (increasing)

→ All showed some trends of being positively associated with study quality, but none were statistically significant (at $p = 0.05$)

Key Findings

- Source of pediatric preference weights rarely children
- Parental/family quality of life rarely included
- “Societal perspective” varied
  - US Panel on Cost-Effectiveness in Health and Medicine available since 1996

Limitations

- Reliability results
  - limited to 7 PQAQ domains
- Small numbers
  - in subgroups for hypothesis testing

Contribution of Study

- Demonstrates there is room for improvement in pediatric CUAs.
  - E.g., inconsistent reporting
- Shows that investigators often avoid the challenges of measuring and valuing pediatric QALYs.
  - Using preference weights derived from adults
  - Ignoring parental/familial quality of life

Further Research:

- Need to validate instruments in children or create new ones specifically for paediatric populations
- Need to reinforce guidelines for reporting (with researchers and reviewers)

Policy:

- Exercise judgement when using results from pediatric CUA's to make decisions.

Thank-you…. Questions?