ISPOR panel: Using HRQoL data from children and youth to strengthen HTA. What are the barriers? How can we improve current practice?

Novel Strategies for Assessing Preferences for Pediatric Health States

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Disclosures

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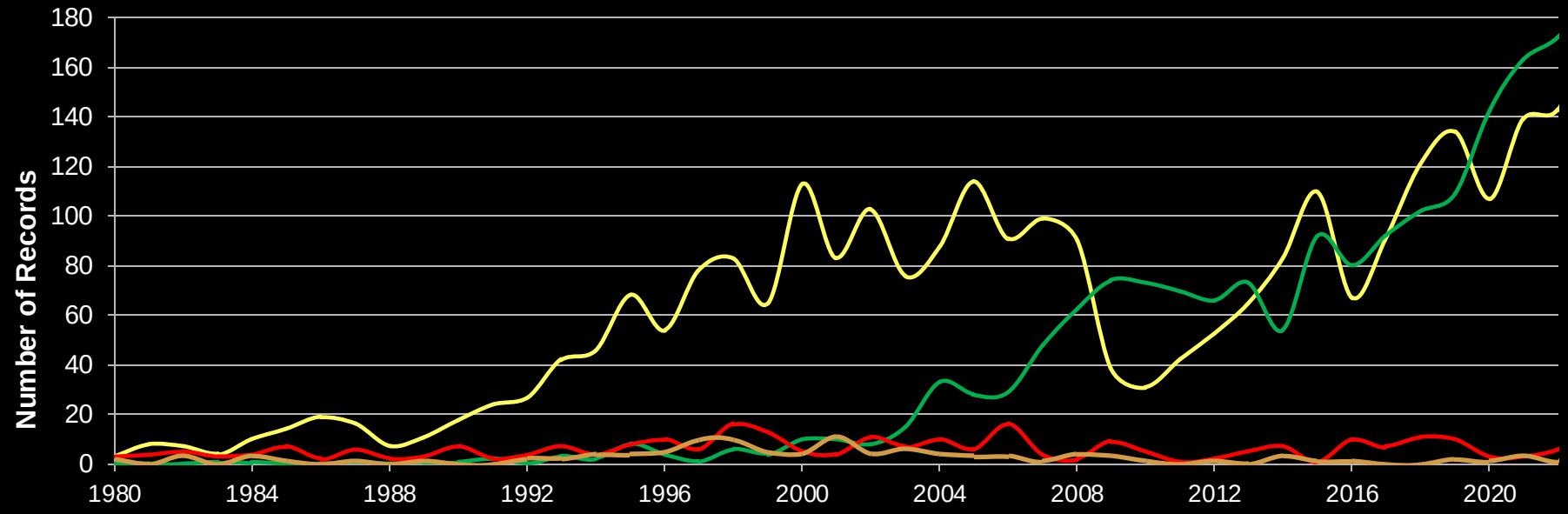
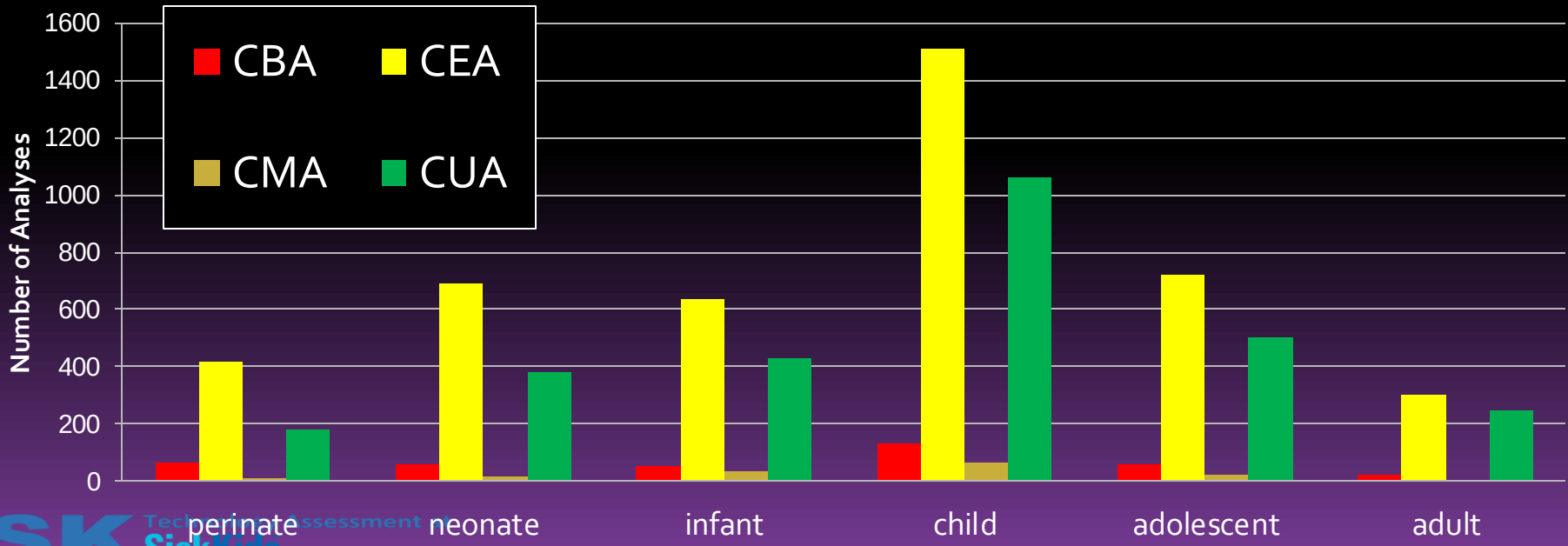
Pediatric Economic Evaluations 1980-2023 (n=5083)





TASK Technology Assessment at SickKids

Barriers to Pediatric Utility Estimation

- Lack of validated preference-weighted HRQoL instruments and value sets for very young children
- Utilities vary as a function of instrument and tariff set used
- Lack of consensus on the concept of HRQoL for children and how it varies by age group
- Need to capture HRQoL in parents/caregivers

Indirect Utility Elicitation Instruments for Young Children and Toddlers

Instrument	Age group (years)	Child-specific QoL attributes?	Whose preferences used to derive underlying weights?
17D	8-11	Yes	Parents
CHU-9D	7-11	Yes	Adolescents/students; General adult population
HUI2/3	5-8	No	Parents; General adult population
EQ-5D-Y	3-7	No	General adult population
HuPS	2-5	Yes	Parents
EQ-TIPS (TANDI)	0-3	Yes	N/A
IQI	0-1	Yes	Parents; General adult population

From: Kwon et al. *PharmacoEconomics* 2022. https://doi.org/10.1007/s40273-021-01128-0

Child-specific Instrument: CHU-9D

- Descriptive system
  - Developed in children
  - 9 dimensions: worried, sad, pain, tired, annoyed, schoolwork, sleep, daily routine, activities
- Preference weights
  - Sample of UK adult population: adult preferences using SG
  - Sample of Australian adolescent population valuing health states using BWS
- Self-completed by child
- Present state (no recall)

Stevens KJ. *Qual Health Res*. 2010

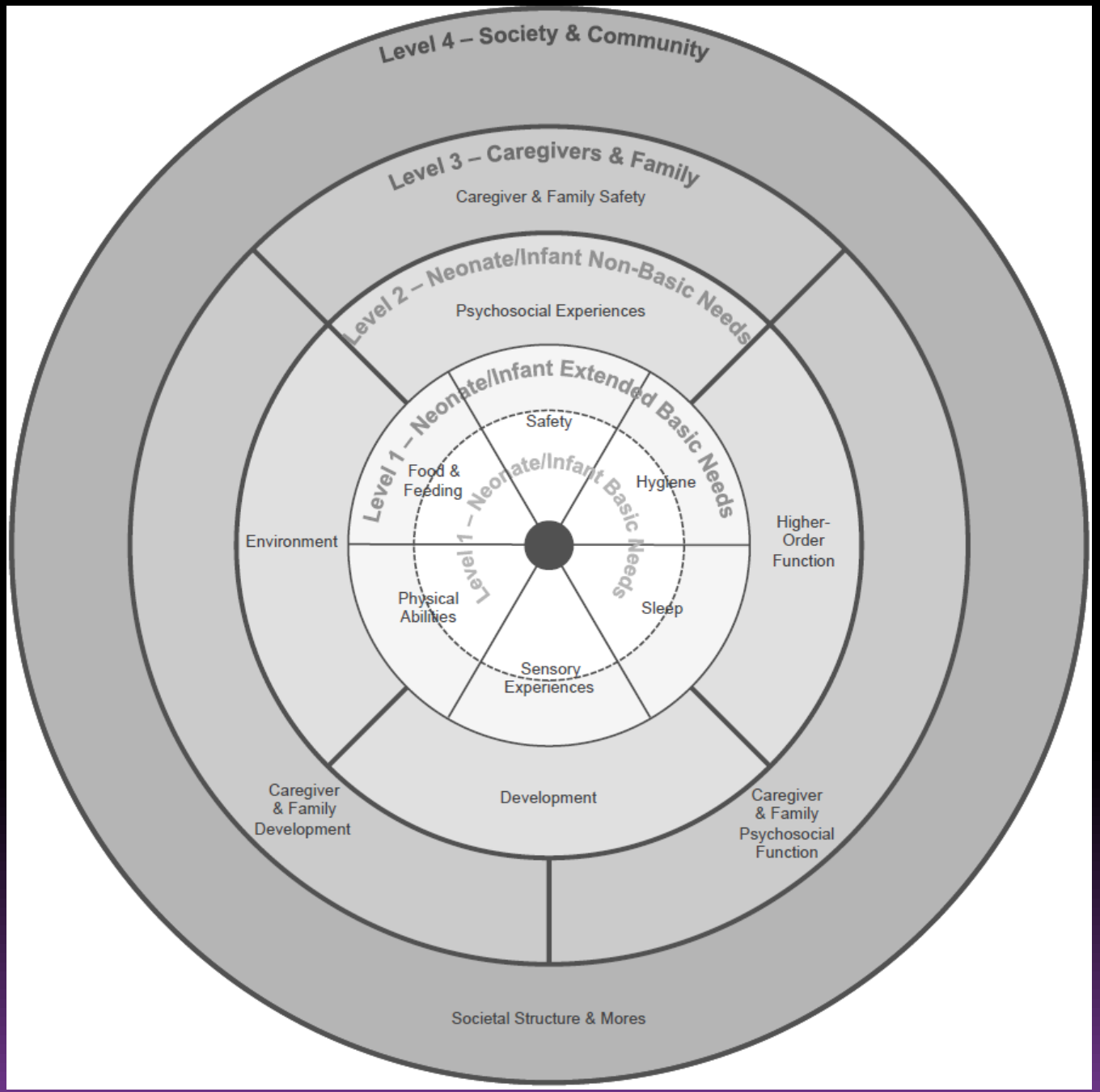
CHU9D and HUI Health Utilities in Pediatric Crohn's Disease (CD) and Ulcerative Colitis (UC)

			CHU9D Utility (Adult Tariffs)	CHU9D Utility (Youth Tariffs)	HUI2 Utility	HUI3 Utility
CD	Males (n = 127)	Mean (SD)	0.862 (0.122)	0.773 (0.203)	0.885 (0.152)	0.821 (0.220)
		Median (IQR)	0.880 (0.148)	0.810 (0.232)	0.926 (0.136)	0.879 (0.230)
		Range (minimum to maximum)	0.380–1.000	0.052–1.000	0.174–1.000	−0.160–1.000
	Females (n = 72)	Mean (SD)	0.838 (0.112)	0.721 (0.200)	0.855 (0.155)	0.791 (0.216)
		Median (IQR)	0.866 (0.163)	0.757 (0.274)	0.907 (0.177)	0.846 (0.224)
		Range (minimum to maximum)	0.558–1.000	0.167–1.000	0.266–1.000	0.081–1.000
UC	Males (n = 35)	Mean (SD)	0.869 * (0.115)	0.778 * (0.210)	0.854 (0.200)	0.799 (0.212)
		Median (IQR)	0.885 (0.154)	0.810 (0.311)	0.937 (0.189)	0.879 (0.264)
		Range (minimum to maximum)	0.567–1.000	0.230–1.000	0.212–1.000	0.232–1.000
	Females (n = 51)	Mean (SD)	0.806 * (0.129)	0.675 * (0.216)	0.791 (0.214)	0.706 (0.251)
		Median (IQR)	0.826 (0.174)	0.686 (0.296)	0.868 (0.222)	0.748 (0.307)
		Range (minimum to maximum)	0.480–1.000	0.174–1.000	0.257–1.000	0.011–1.000

* p = 0.02 (statistically significant difference between sexes), SD = standard deviation, IQR = interquartile range.

Bashir et al., *Children* 2021. https://doi.org/10.3390/children8050343

Health-related quality of life in neonates and infants: Theoretical construct



From: Oliveira et al. *Qual Life Res*. 2020
https://doi.org/10.1007/s11136-020-02432-6

To Improve Current Practice: Family Perspective

- Interdependency of HRQoL within a family
- Value of reporting HRQoL effects on caregivers and other family members
- Emphasis on individual preferences for health state valuation assumes the respondent is autonomous

Adapted from Basu A & Meltzer D. *J Health Econ*. 2005; 24:751-773

Moving the Guidelines Forward

PharmacoEconomics
https://doi.org/10.1007/s40273-023-01321-3

ORIGINAL RESEARCH ARTICLE

Check for updates

Recommendations for Emerging Good Practice and Future Research in Relation to Family and Caregiver Health Spillovers in Health Economic Evaluations: A Report of the SHEER Task Force

Edward Henry[1] · Hareth Al-Janabi[2] · Werner Brouwer[3] · John Cullinan[1] · Lidia Engel[4] · Susan Griffin[5] · Claire Hulme[6] · Pritaporn Kingkaew[7] · Andrew Lloyd[8] · Nalin Payakachat[9] · Becky Pennington[10] · Luz María Peña-Longobardo[11] · Lisa A. Prosser[12] · Koonal Shah[13] · Wendy J. Ungar[14] · Thomas Wilkinson[15] · Eve Wittenberg[16]

Accepted: 28 September 2023

Family Perspective

Lamsal *et al*.:

- Systematic reviews of methods used in 1) pediatric and 2) maternal-perinatal CUAs to include family health effects

1. Pediatric (*PharmacoEconomics* 2024; doi: 10.1007/s40273-023-01331-1)
   - 35 CUAs included quality-of life effects in family members
2. Maternal-perinatal (*PharmacoEconomics* 2024; doi: 10.1007/s40273-024-01397-5)
   - 62 CUAs considered QALYs or DALYS of mothers and/or neonates

Measuring Family HRQoL Effects

Pediatric CUAs (n=35):

- 24 studies (69%) incorporated effects on just one caregiver
- QALY loss of the caregiver/parent due to a child's illness or disutility of a child's illness on caregiver/parent most common approach
- 11 studies (31%) measured overall health utility of the caregiver or parent
- 13 studies (37%) studies calculated QALYs separately for children and parents/caregivers
- 17 studies (50%) studies summed child + parent QALYs and reported incremental family QALYs

Measuring Family HRQoL Effects

Maternal-Perinatal CUAs (n=54):

- 50 studies (93%) measured separate health utilities of pregnant woman and of child (neonate)
- 12 studies (22%) estimated a disutility or utility decrement of a maternal or fetal condition on pregnant woman and/or child
- 21 studies (39%) included neonate QALY losses due to fetal demise, stillbirth, or miscarriage
- 19 studies (35%) incorporated effects of neonatal demise on a mother's HRQoL
- 21 studies (39%) incorporated effects of a child's condition on a mother's HRQoL
- In 46 studies (85%) , QALYs of pregnant women and children were summed in each comparator group o determine family QALYs
- In 42 studies (78%), incremental family QALYs used in CUA

Approaches to Integrating HRQoL of Family Members

- Child's health state utility reduced by disutility of caregiving (NICE HST8, 2018)
- Child's health state utility reduced by disutility of parent/caregiver, with models including both child and caregiver health states (NICE, HST2 2015)
- QALYs calculated and reported separately for children and family members (Chatterton 2019)
- QALYs calculated and reported separately for children and family members and also summed (Tubeuf 2019)
- Family perspective via discrete choice experiments with attributes reflecting family effects

Next Steps to Further Improve Practice

- Validation studies for reworded, recalibrated and new instruments
- Comparative performance research
- Additive or multiplicative models for combining family members' utilities; statistical household functions
- Discrete choice methods to derive utilities for pediatric health state incorporating attributes relevant to family
- Health economic guidelines must be updated to reflect methodologic challenges and guide alternative approaches

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For more information:

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Canada Research Chairs / Chaires de recherche du Canada

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pathways in autism spectrum disorder

CHILD-BRIGHT Network

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