

Advances in measurement and valuation of healthrelated quality of life in young children

Professor Kim Dalziel

Chair of Health Economics, Centre for Health Policy, The University of Melbourne, Australia

Visiting Scholar, 2024, Susan B. Meister Child Health Evaluation and Research (CHEAR) Center, University of Michigan

kim.dalziel@unimelb.edu.au

of Melbourne, Australia Research (CHEAR)



Funding & conflicts

Funding

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Conflicts

I have received previous or current funding from the EuroQol Foundation who is the developer and copyright holder of some instruments included in this study. The EuroQol Foundation are providing some direct funding for this research.





Australian paediatric multi-instrument comparison (P-MIC) study



DATA COLLECTION

Initial survey Demographics, non HRQoL & HRQoL instruments.

> 4-weeks or small subset 2-days

Follow-up survey (42%)

Change in health qxs and HRQoL instruments.



Children ≥7 years asked to self-report HRQoL (57% self report)



INSTRUMENTS

Core HRQoL (N=6,787)

Received by all (EQ-5D-Y-3L, EQ-5D-Y-5L, CHU9D, PedsQL) EQHWB carers

Additional HRQoL

Only online panel randomised to receive additional: N=1,523 Completed AQoL-6D N=1,730 Completed PROMIS-25 *N=2,262* Completed HUI2/3

Condition specific

Only online panel condition groups receive corresponding instrument



TOTAL P-MIC POPULATION

Australian children and adolescents aged 2-18 years. *N=6,787*



N=1,067 Tertiary paediatric hospital, Melbourne, Australia



N=1,894 Online panel general population sample



N=3,826 Online panel condition group sample (~400 per condition)

YOUNG CHILDREN 2-4 years

N=842 3(1)

N=313 (37%)

N=279 (33%) need



- Mean and SD of child age
- Child has **chronic** condition or disability
- Child has **special healthcare**





Psychometric validity: young children

Research Question



• What is the psychometric comparative performance HRQOL instruments (adapted EQ-5D-Y, EQ-TIPS, CHU9D, HUI) for young children <5 years?



Methods

- Ceiling/ floor effect
- Test-retest reliability
- Known group validity
- Responsiveness







Included HRQoL instruments

Instrument	EQ-5D-Y (adapted 2-4 years)	CHU9D (under 5 years)	EQ-TIPS	HUI3	PedsQL
Domains	Movement	Worried	Movement	Vision	Physical
	Self-care	Sad	Play	Hearing	Emotional
	(helping with)	Pain	Pain	Speech	Social
	Usual activities	Tired	Social	Ambulation	Day care
	Pain	Annoyed	Interaction	Dexterity	
	Sad/worried	Pre-school	Communication	Emotion	
		Sleep	Eating	Cognition	
		Routine		Pain	
		Activities			





EQ-5D-Y adapted for 2-4 years



Dalziel et al. Value in Health. 2023. A Qualitative Investigation to Develop an Adapted Version of the EQ-5D-Y-3L for Use in Children Aged 2-4 Years.

VanHeusden... Dalziel. 2024. PharmacoEconomics. Psychometric Performance Comparison of the Adapted versus Original Versions of the EQ-5D-Y-3L and -Y-5L in Proxy Respondents for 2- to 4-Year-Olds.









CHU9D: response distribution by SHCN



Xiong... Dalziel. 2024. PharmacoEconomics. Psychometric Properties of Child Health Utility 9D (CHU9D) Proxy Version Administered to Parents and Caregivers of Children Aged 2–4 Years Compared with Pediatric Quality of Life Inventory[™] (PedsQL)







EQ-TIPS response distribution by health status



Young children- 2-4 years

Known group validity

Known Group	PedsQL	Adapted EQ- 5D-Y-5L	CHU9D (utility)	HUI (LSS)	EQ-TIPS (2/3 years)		
	Cohen D, *p<0.	Cohen D, *p<0.05,**p<0.01, ***p<0.001					
SHCN No SHCN	0.96***	1.01***	0.75***	1.12***	0.94***		
Poor/fair Good/v good/excellent	0.88***	1.02***	1.3***	0.69***	1.19***		
EQ-VAS <80 EQ-VAS>81	1.01***	1.07***	-	0.74***	1.11***		

Cohen D effect size thresholds 0.2 to <0.5, 0.5 to <0.8, and 0.8 or more denote small (red), medium (yellow), and large (green) effect sizes, respectively. CHU9D utility is calculated based on Australian adolescent preferences.

Young children- 2-4 years

Responsiveness

LSS general health change	Improved SRM	Same SRM	Worsened SRM			
PedsQL	0.41	-0.12	-0.15			
Adapted EQ-5D-Y-5L	0.27**	-0.05	-0.41			
CHU9D (utility)	0.25	-0.1	-0.44			
HUI3	-0.15	0.05	-0.64			
EQ-TIPS	0.18	0.04	-0.2			
*p<0.05, **p<0.01, ***p<0.001, Standardised response mean (SRM) thresholds of <0.2, 0.2-<0.5, 0.5-<0.8, and 0.8						

or more denote trivial, small, moderate, and large effect sizes. CHU9D utility is calculated based on Australian adolescent preferences.

Summary Results for 2-4 years

Generic P-PROM	Number questions?	Free?	Mean time to complete?	Well distributed? ^a	Reliable when no change? ^b	D
PedsQL	23	Х	86 secs	\checkmark	\checkmark	
EQ-5D-Y-5L adapted CHU9D	5 9	√* √*	27 secs 67 secs	\checkmark	\checkmark	
EQ-TIPS	6	√*	26 secs	\checkmark	?	
HUI 3	15	X	112 secs	\checkmark	? (sample size <50)	

*Free for non-commercial use.

✓ Evidence of significant performance; X No evidence of significant performance; ? Inconclusive evidence. ^a \checkmark Less than 15% of participants with a special healthcare need reported the lowest severity or frequency level (i.e., 'no problems') across all items ^b \checkmark ICC (moderate, good or excellent) ≥0.5

Jones R et al. Comparative Psychometric Performance of Common Generic Paediatric Health-Related Quality of Life Instrument Descriptive Systems: Results from the Australian Paediatric Multi-Instrument Comparison Study. PharmacoEconomics. 2023. Jones R et al. Collecting Paediatric Health-Related Quality of Life Data: Assessing the Feasibility and Acceptability of the Australian Paediatric Multi-Instrument Comparison (P-MIC) Study. Children. Published 2023.

Key take aways

- A number of validated instruments exist suitable for measuring HRQoL of children aged 2-4 years e.g. CHU9D for under 5 years, EQ-5D-Y for 2-4 years
- These are ready to be used for:
 - Clinical applications as PROMs
 - Registries and longitudinal studies- natural history
- Still need to be scored to produce utilities lacksquare
 - For use in economic evaluation

Valuation issues for utility scoring

- Do preferences differ? young child vs older child, parent vs general population
- Anchoring challenges: population won't trade life years for children leading to higher utility scores, adolescents will trade producing lower utility
- Consistency of values across childhood
- Appropriateness of methods: time horizon may cross child age
- Options being developed such as PedsUtil: short form PedsQL suitable across childhood
- Potential for mapping to other instruments or ages

ISPOR Taskforce: Valuing HRQoL of Children & Adolescents in Economic Evaluation (Pediatric Utilities): Tuesday 7th May, 11.45am SESSION 218

Points at which HRQoL measurement can differ- unrelated to health

Professor Kim Dalziel University of Melbourne www.quokkaresearchprogram.org

kim.dalziel@unimelb.edu.au

dalzielk@umich.edu

<u>@kmdalziel</u>

Kim Dalziel

Comparison of EQ-HWB-S scores between parents of a child with a health condition and the general population sample

Bailey, Dalziel...2024. PharmacoEconomics. The Validity of the EuroQol Health and Wellbeing Short Version (EQ-HWB-S) Instrument in Parents of Children With and Without Health Conditions.

■ General population Child health condition

CHU9D <5 years valuation example

- Research questions:
 - Do people's preferences differ for younger children aged 2-4 years old compared to older children, when valuing CHU9D?
 - What are the general population's preferences for CHU9D for children aged 2-4 years old?
 - Do parents differ from the general adults in preferences?

Valuation task design

Online survey programmed using Qualtrics Recruit participants by survey company (Cint)

Survey	D	Design				
	Randomly allocated Arm 1	Randomly a				
Source population	General adults (su	b-population				
Instrument	CHU9D 2-4 version	CHU9D 5-17				
Main tasks	12 DCE tasks (+1 dominant task)	12 DCE task				
Anchoring	VAS	VAS				
Perspective	Think of a 2–4-year-old child	Think of a 1				
Sample size	1200	1200				

allocated Arm 2

parents)

version

- ks (+1 dominant task)
- .0-year-old child

DCE task

- Partial DCE design: 5 varying lacksquareattributes, 4 overlapped (grey)
- With pop out guidance notes on ullethealth dimensions for <5 wording
- Analyse responses using \bullet conditional logit model

Considering your views about a 2-4 year old child: which health state do you prefer?

	Health State A	Health State B
	Very worried today	Quite worried today
	A little bit sad today	A bit sad today
	A lot of pain today	A lot of pain today
	Not tired today	Not tired today
	Very annoyed today	A bit annoyed today
1	A few problems with their schoolwork/homework today	A few problems with their schoolwork/homework today
	Some problems sleeping last night	A few problems sleeping last night
- -	Can't do their daily routine today	A few problems with their daily routine today
	Join in with no activities today	Join in with no activities today

Health State A

Health State B

Relative Attribute Importance Scores by study arm and RAI differences with 95% Confidence Intervals

CHU9D dimension	2–4-year-old		10-year-old		RAI difference (95% CI)	P-value
	RAI	SE	RAI	SE		
Worried	1.72	0.21	1.77	0.21	-0.05(-0.63,0.53)	0.873
Sad	2.06	0.23	1.84	0.22	0.22(-0.41,0.85)	0.506
Pain	3.31	0.36	2.80	0.30	0.51(-0.41,1.43)	0.282
Tired	1.01	0.14	1.00	0.13	0.01(-0.37,0.38)	0.971
Annoyed	1.00		1.00			
Schoolwork	1.41	0.17	1.50	0.18	-0.10(-0.59,0.39)	0.707
Sleep	2.08	0.24	1.97	0.22	0.10(-0.53,0.74)	0.761
Daily routine	1.86	0.21	2.10	0.23	-0.24(-0.84,0.37)	0.453
Join in activities	1.28	0.18	1.23	0.17	0.05(-0.44,0.53)	0.860

No evidence of a difference in the populations preferences when asked to think about a 2-4 year old versus a 10 year old Means the scoring for the CHU9D can be applied consistently across child age 2-18 years