



# Are existing measures of paediatric health-related quality of life fit for purpose for use in Health Technology Assessment?

## Academic Perspective

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Investigator Quality Of Life in Kids: Key evidence to strengthen decisions in Australia (QUOKKA)





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# Key research opportunities

- As investigator on academic-led paediatric clinical trials (>20) since 2014
  - ongoing issue to recommend HRQoL measure that cross child age and have scoring available for economic evaluation
- Understand how paediatric HRQoL measures were/were not being used to inform HTA funding decisions, suspicion that adult measure were used
- Lack of evidence about psychometric performance of paediatric PROMs
- Lack of measures for young children
- Lack of value sets to score paediatric PROMs
- Important normative questions specific to children
  - Perspective, framing, use of proxy, age and development



# QUOKKA Objectives

QUality OF Life in Kids: Key evidence to strengthen decisions in Australia (QUOKKA)

- draws on international research to produce improved approaches to measuring and valuing child health outcomes



## P-MIC Objectives

- Compare psychometric performance of widely used generic paediatric HRQoL instruments (EQ-5D-Y-3L, EQ-5D-Y-5L, EQ-5D for 12 plus, EQ-5D-Y age 2-4, EQ-TIPs, CHU9D, CHU9D under 5, PedsQL/PedsUtil, HUI2/3, AQuoL-6D, PROMIS-25)
- Compare performance by child age (2-18 years), report type (self-vs proxy), and by child health status/condition



*Protocol paper*

# Methods

## Australian Paediatric Multi-Instrument Comparison (P-MIC) study overview

### POPULATION

**Australian children and adolescents aged 2–18 years.**



Tertiary paediatric hospital,  
Melbourne, Australia



Online panel general  
population sample

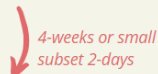


Online panel condition group  
sample (x9 condition groups)

### DATA COLLECTION

#### **Initial survey**

Demographics, non HRQoL & HRQoL  
instruments.



#### **Follow-up survey**

Change in health qxs and HRQoL  
instruments.



Children  $\geq 7$  years  
asked to self-report  
HRQoL

### INSTRUMENTS

#### **Core HRQoL**

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

#### **Additional HRQoL**

Only online panel randomised to  
receive one (AQoL-6D, HUI 2/3,  
PROMIS-25)

#### **Condition specific**

Only online panel condition groups  
receive corresponding instrument

## THIS ANALYSIS

- 10th August 2022
- Children aged 5–18 years
- EQ-5D-Y-3L, EQ-5D-Y-5L, CHU9D, PedsQL, HUI, and AQoL-6D

*Technical  
methods paper*



# Results – participant characteristics

Participant Characteristic	Total sample included in analysis, n(%) or mean (sd)
<b>Sample Characteristics</b>	
Completed initial survey, n(%)	5,945 (100)
Completed follow-up survey, n(%)	2,346 (39.5)
Recruited via hospital (sample 1), n(%)	759 (12.8)
Online panel general population (sample 2), n(%)	1,531 (25.8)
Online panel condition groups (sample 3), n(%)	3,655 (61.5)
Completed core HRQoL instruments (CHU9D, PedsQL, EQ-5D-Y-3L & 5L), n(%)	5,945 (100)
Completed AQoL-6D, n(%)	1,523 (25.6)
Completed HUI 2/3, n(%)	1,728 (29.1)
Proxy completed HRQoL instruments	2,083 (35.0)
<b>Child Characteristics</b>	
Child age, mean (sd)	10.9 (3.9)
Child gender- Female, n(%)	2,737 (46.0)
Child has a special healthcare need, n(%)	2,583 (43.4)
<b>Caregiver Characteristics</b>	
Caregiver age, mean (sd)	40.8 (8.5)
Caregiver highest education level- bachelor degree or above, n(%)	2,161 (36.4)



# Results – distribution of responses

Table summarising number and percentage of participants with a special healthcare need reporting the lowest severity/frequency level across all items

Instrument	Children with special healthcare need N(%)
PedsQL (23-items)	11 (0.4)
EQ-5D-Y-3L (5-items)	459 (17.8)
EQ-5D-Y-5L (5-items)	383 (14.8)
CHU9D (9-items)	103 (4.0)
AQoL-6D (20-items)	6 (1.1)
HUI 3 (8-domains)	49 (7.6)

More than 15% of participants reported the lowest severity/frequency level across all items, denoted by red text.

\*EQ-5D-Y has least items and greatest chance of clustering at lowest severity



# Results – known group validity

Table summarising mean total or sum score for children with and without a chronic health condition for each instrument

Instrument total or sum score	Chronic health condition (condition lasting at least 6 months)		
	Mean - No	Mean - Yes	Effect Size (Cohen's D)
PedsQL total score (↑better)	75.3	58.7	1.0
EQ-5D-Y-3L sum score (↓better)	6.0	7.4	-0.9
EQ-5D-Y-5L sum score (↓better)	6.4	8.9	-0.9
CHU9D sum score (↓better)	13.9	18.8	-0.8
AQoL-6D sum score (↓better)	32.7	42.9	-1.0
HUI 3 sum score (↓better)	10.4	13.5	-0.9

*Green cells indicates large effect size ( $\geq 0.8$ ).*

All instruments also demonstrated known group validity with large effect sizes across other known groups: special healthcare needs, VAS score= $\leq 80$ , PedsQL = $\leq 74.2$ , PedsQL = $\leq 69.7$

\*PedsQL and AQoL-6D have largest effect size



# Results – convergent validity

Table summarising convergent validity between all instruments

Instrument correlation combination	Total number of item combinations	Item combinations with moderate or strong correlation, n(%) item combinations
PedsQL and EQ-5D-Y-3L	115	63 (54.8)
PedsQL and EQ-5D-Y-5L	115	72 (62.6)
PedsQL and CHU9D	207	134 (64.7)
PedsQL and AQoL-6D	460	304 (66.1)
PedsQL and HUI 3	184	54 (29.3)
EQ-5D-Y-3L and EQ-5D-Y-5L	25	19 (76.0)
EQ-5D-Y-3L and CHU9D	45	28 (62.2)
EQ-5D-Y-3L and AQoL-6D	100	57 (57.0)
EQ-5D-Y-3L and HUI 3	40	16 (40.0)
EQ-5D-Y-5L and CHU9D	45	30 (66.7)
EQ-5D-Y-5L and AQoL-6D	100	56 (56.0)
EQ-5D-Y-5L and HUI 3	40	17 (42.5)
CHU9D and AQoL-6D	180	122 (67.8)
CHU9D and HUI 3	72	24 (33.3)

Across all instruments, items pre-specified to have at least moderate correlation demonstrated the case

\*HUI least correlated with other instruments

# Results- test/retest

Table summarising test-retest at 2 days

Instrument total or sum score	Total sample		
	N	ICC	95% CI
PedsQL total score	114	0.80	0.73, 0.86
EQ-5D-Y-3L sum score	114	0.83	0.76, 0.88
EQ-5D-Y-5L sum score	113	0.78	0.69, 0.84
CHU9D sum score	114	0.64	0.52, 0.74
AQoL-6D sum score	34	0.86	0.74, 0.93
HUI 3 sum score	35	0.82	0.67, 0.90

Green cells indicates good agreement (  $\geq 0.8$  ).

Yellow indicates acceptable agreement (0.7-0.79)

\*All except CHU9D ok for retest reliability

# Results- responsiveness

Table summarising responsiveness where there has been a change in child's general health (much better at 4 weeks)

Instrument total or sum score	N	Initial survey mean (sd)	Follow-up survey mean (sd)	Mean difference (sd)	p value	Standardised response mean (SRM)
PedsQL total score	213	73.42 (19.14)	74.96 (19.62)	-1.55 (15.54)	0.0740	-0.10
EQ-5D-Y-3L sum score	213	6.40 (1.68)	6.10 (1.68)	0.30 (1.65)	<b>0.0047</b>	0.18
EQ-5D-Y-5L sum score	209	7.25 (3.04)	6.67 (2.83)	0.59 (2.28)	<b>0.0001</b>	0.26
CHU9D sum score	213	14.99 (6.62)	13.06 (5.56)	1.93 (5.40)	<b>&lt;0.0001</b>	0.36
AQoL-6D sum score	39	35.33 (11.26)	35.85 (12.32)	-0.51 (8.22)	0.6667	-0.06
HUI 3 sum score	39	11.31 (3.33)	10.82 (4.65)	0.49 (3.62)	0.2029	0.14

\*EQ-5D-Y and CHU9D strongest and also able to significantly detect somewhat or much worse health

# Conclusions- best instruments by each criteria

	Distribution of responses- low severity	Known group validity	Convergent validity	Test re-test reliability 2-days	Responsiveness 4 weeks	Number of strong criteria (/5)
PedsQL	✓	✓	✓	✓		4/5
EQ-5D-Y-3L		✓	✓	✓	✓	4/5
EQ-5D-Y-5L	✓	✓	✓	✓	✓	5/5
CHU9D	✓	✓	✓		✓	4/5
AQoL-6D	✓	✓	✓	✓		4/5
HUI3	✓	✓		✓		3/5

## Next steps

- Focus analyses on different child ages, conditions (including condition specific HRQoL measures) and proxy/self-report
- Assessment of how value-sets perform on P-MIC data
- IRT and dimensionality
- How MID established on PedsQL (4.5 points) translates to changes on other instruments
- Understanding transition across instruments with age, boundaries of instruments
- Younger children (<5 years)
  - Reduced form PedsQL for valuation (PedsUtil)
  - EQ-5D-Y adapted for 2-4 year old
  - EQ-TIPS (formerly TANDI)
- International P-MIC
- Valuation questions naturally flow from psychometric work





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Thank you  
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