#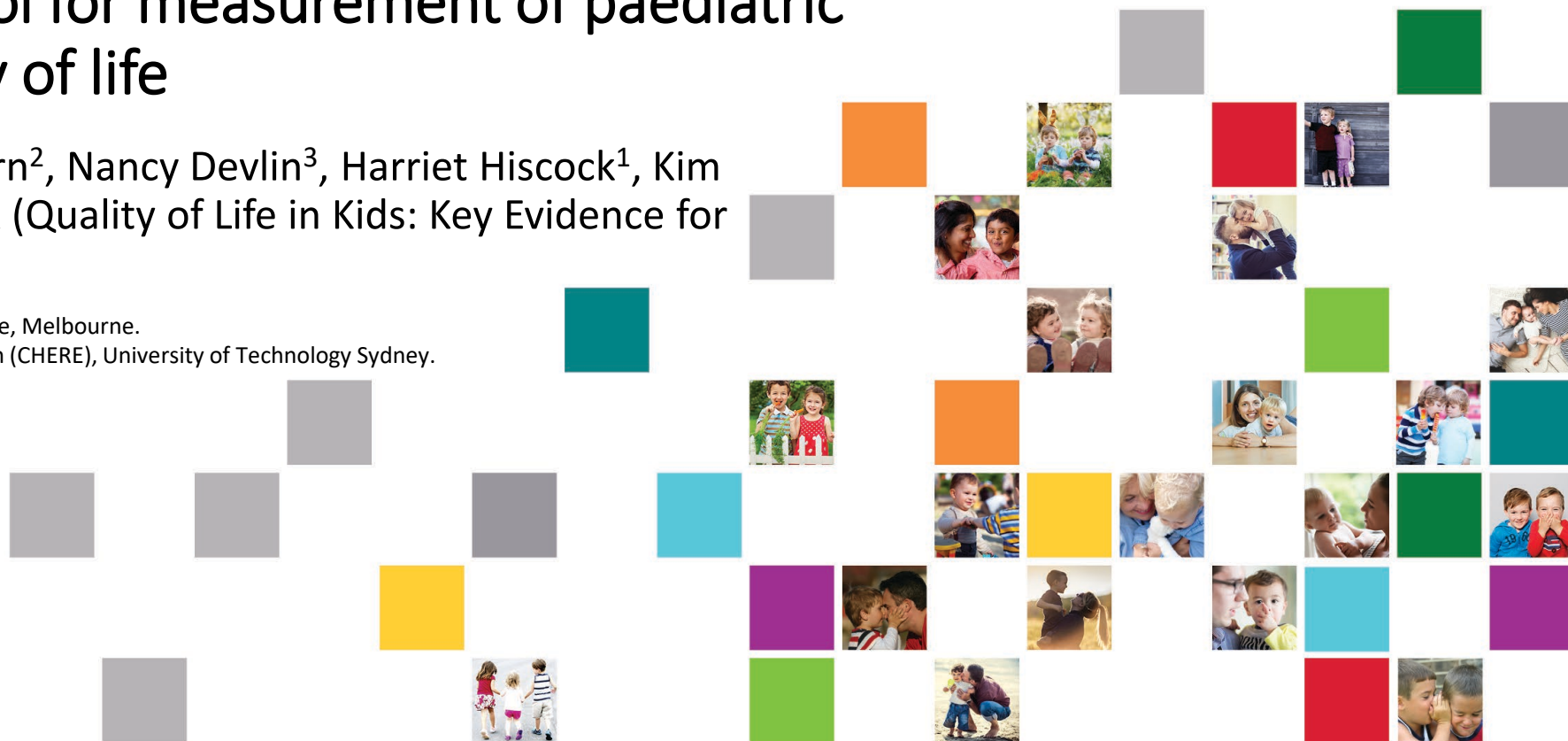 An Australian paediatric multi-instrument comparison (P-MIC) study protocol for measurement of paediatric health-related quality of life

Renee Jones[1], Brendan Mulhern[2], Nancy Devlin[3], Harriet Hiscock[1], Kim Dalziel[1,3] on behalf of QUOKKA (Quality of Life in Kids: Key Evidence for Decision Makers in Australia)

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Acknowledgements

Background

Health-related quality of life (HRQoL) informs:

Cost-effectiveness evidence, which is used to make decisions about public funding for pharmaceuticals, technologies and health services.(1)

These decisions have **large cost implications.** Over $37 billion was spent in Australia in 2019-20 on the Pharmaceutical Benefits Scheme (PBS) and Medicare Benefits Schedule (MBS).(2,3)

Patient outcome measurement, in clinical and research settings.(4)

1) Bulfone, L.; Younie, S.; Carter, R. Health Technology Assessment: Reflections from the Antipodes. *Value Health* **2009**, *12*, S28–S38., **2)** Services Australia, Commonwealth of Australia. Annual Report 2019–2020., **3)** Services Australia, Commonwealth of Australia. PBS Expenditure and Precriptions Report 1 July 2019 to 30 June 2020., **4)** Dawson, J.; Doll, H.; Fitzpatrick, R.; Jenkinson, C.; Carr, A.J. The routine use of patient reported outcome measures in healthcare settings. *BMJ* **2010**, *340*, c186.

Background

There are established methods for measuring HRQoL in adults, **considerable challenges arise in applying these to children (5)**:

- developmental range of children

- younger children unable to self-report

Health Technology Assessment (HTA) bodies in Australia and previous systematic reviews have noted a **lack of evidence on the psychometric performance of paediatric HRQoL instruments**, impacting decision making.(6,7)

5) Ungar, W. *Economic Evaluation in Child Health*; Oxford University Press: Oxford, UK, 2009., **6)** Rowen, D.; Keetharuth, A.D.; Poku, E.; Wong, R.; Pennington, B.; Wailoo, A. A Review of the Psychometric Performance of Selected Child and Adolescent Preference-Based Measures Used to Produce Utilities for Child and Adolescent Health. Value Health **2021**, 24, 443–460., **7)** Chen, G.; Ratcliffe, J. A Review of the Development and Application of Generic Multi-Attribute Utility Instruments for Paediatric Populations. *Pharmacoeconomics* **2015**, *33*, 1013–1028.

Aim

To **compare the performance of a range of paediatric HRQoL instruments** in terms of validity, reliability, responsiveness, acceptability, feasibility, and consistency **across age and disease groups**.

Methods: Study Design and Timeline

Study Design:

Paediatric multi-instrument comparison (P-MIC) study:

involving the prospective collection of multiple generic
and disease-specific paediatric HRQoL instruments
concurrently.

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Study Timeline:

May 2021	June 2021	Oct 2021	Jan 2022	Dec 2022	Ongoing analysis
Ethics approval	Hospital sample recruitment started	Start online panel recruitment	Recruitment completed	Main results completed	

Methods: Populations and Recruitment

Population:

6,100 Australian children aged 2-18 years.

1,000 The Royal Children's Hospital

1,500 Online panel population sample

3,600 Online panel disease groups sample (x9 disease groups)

Recruitment:

- Advertising on telehealth
- Approaching families in waiting rooms
- QR posters around the hospital
- Piggybacking on other studies
- Social media
- Advocacy groups
- RCH Childcare centre

Methods: Data Collection

Initial survey

2-8 weeks (n=5,900)
2 days (n=200)

Follow-up survey (indicates not at follow-up)

1,000 The Royal Children's Hospital

- Core

1,500 Online panel population sample

- Core

- Randomised to additional block

3,600 Online panel disease groups sample

- Core

- Randomised to additional block

- Disease specific measure

Core:

- Consent/demographics

- SDQ (Child mental health)

- EQ-HWB (Carer QoL)

- PedsQL

- TANDI

- EQ-5D-Y (3L and 5L)

- CHU9D

Additional blocks:

- AQoL

- HUI & EQ-5D-5L

- PROMIS-25

Methods: Data Collection

- **Children ≥7 years asked to self-report** HRQoL questions if able

- Allocated to **age-appropriate instrument version**

- **Order of core HRQoL instruments randomised** to minimise order effects (EQ-5D instruments separated)

- **Same order** for initial and follow-up

- 3 questions in follow-up survey to understand any **changes in child health** since initial survey

Methods: Statistical Analysis

Psychometric properties: analysed at the overall, domain, dimension, and item levels**.**
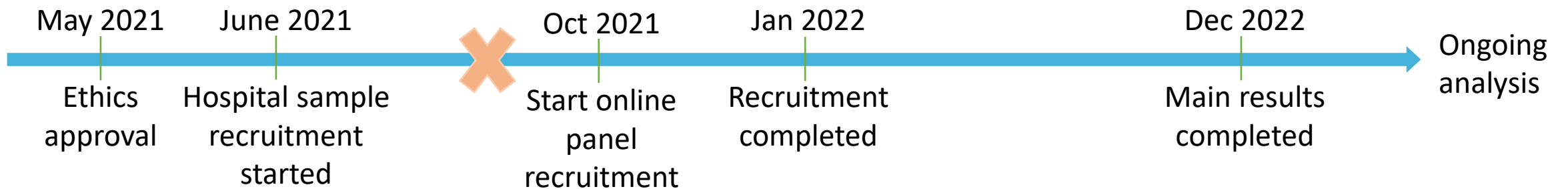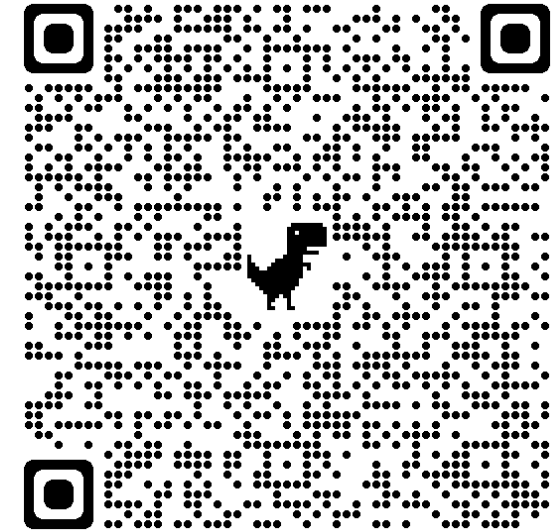
Outcomes: validity, reliability, responsiveness, acceptability and feasibility, and consistency.

Sub-group analysis: child age, gender, disease group (including acute versus chronic conditions), family socio-economic status (SES), and presence of anxiety/depression comorbidity.

Progress to date

- Ethics approval obtained

- Registered on ANZCTR

- Protocol paper published

- Signed up over 10+ hospital departments for recruitment

- Recruited n=423 of hospital sample, aiming for n=1,000

May 2021 — Ethics approval

June 2021 — Hospital sample recruitment started

Oct 2021 — Start online panel recruitment

Jan 2022 — Recruitment completed

Dec 2022 — Main results completed

Ongoing analysis

Implications

Evidence from this study will guide choice of paediatric HRQoL measures used in:

Clinical decision making

Clinical trials

Economic Evaluation

Research outcome measurement

Thank you

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Table 1. Summary of instruments by study sample.

Instrument	Sample 1, Sample Recruited via Hospital		Sample 2, Online Panel Population Sample		Sample 3, Online Disease Group Sample	
	Initial	Follow-Up	Initial	Follow-Up	Initial	Follow-Up
Demographic and non-HRQoL instruments						
Sociodemographic questions	X		X		X	
SDQ	X		X		X	
EQ-HWB	X		X		X	
Core HRQoL instruments						
PedsQL	X	X	X	X	X	X
TANDI (if <=3 years)	X	X	X	X	X	X
EQ-5D-Y 3L & 5L original (if >=5 years)	X	X	X	X	X	X
EQ-5D-Y 3L & 5L adapted (if <=4 years)	X	X				
EQ-5D-Y 3L original and adapted or EQ-5D-Y 5L original and adapted (if <=4 years)			X *	X *	X *	X *
CHU9D	X	X	X	X	X	X
Global Health Measure (single item)	X	X	X	X	X	X
Additional HRQoL instrument blocks						
HUI 2/3 and EQ-5D-5L (>11 years)			X *	X *	X *	X *
AQoL-6D (>4 years)			X *	X *	X *	X *
PROMIS-25 (>4 years)			X *	X *	X *	X *
Disease specific instruments						
Disease specific instruments (as per the recommended age range of the instrument)					X *	

X- indicates the instrument will be collected from the sample/time point. * Participant will only receive, if allocated, instrument based on disease group, and/or randomization to receive additional instrument, and/or randomization to receive EQ-5D-Y 3L original and adapted or EQ-5D-Y 5L original and adapted. Abbreviations: HRQoL health-related quality of life, SDQ Strengths and Difficulties Questionnaire, EQ-HWB EQ Health and Wellbeing Short Version, PedsQL Paediatric Quality of Life Inventory, TANDI Toddler and Infant Questionnaire, EQ-5D-Y EQ-5D Youth, CHU9D Child Health Utility, HUI2/3 Health Utilities Index Mark 2/3, EQ-5D-5L, AQoL-6D Assessment of Quality of Life, PROMIS-25 Patient-Reported Outcome Measurement Information System 25.

Category	__	__	__	__	__	__	__	__	__	__	__	__	__	__	__	__	__
Child Age	2	3	4	5	6	7	8	9	10	11	12	13	14	15	16	17	18
Demographic and non-HRQoL	Sociodemographic questions (parent/caregiver report) →																
	SDQ 2-4 (parent/caregiver proxy-report) [2–4]			SDQ 5-10 (parent/caregiver proxy-report) [5–10]						SDQ 11-18 (Child self-report if able, otherwise proxy-report) [11–18]							
	Carer QoL- EQ-HWB (parent self-complete) →																
	Parent/caregiver proxy-report [2–7]						*Child self-report if able, otherwise proxy-report* [8–18]										
Core HRQoL	PedsQL 2-4 [2–4]			PedsQL 5-7 [5–6]		PedsQL 5-7 if proxy or 8-12 if self [7]	PedsQL 8-12 [8–12]					PedsQL 13-18 [13–18]					
	TANDI [2–4]			n/a [5–18]													
	EQ-5D-Y 3L and 5L (adapted with guidance notes) [2–4]			EQ-5D-Y 3L and 5L [5–18]													
	CHU9D (with guidance notes) [2–4]			CHU9D [5–18]													
	Global Health Measure →																
Additional HRQoL*	HUI 2/3 →																
	n/a [2–11]										EQ-5D-5L [12–18]						
	n/a [2–4]			AQoL-6D [5–18]													
	n/a [2–4]			PROMIS-25 [5–18]													
Disease Specific	Disease-specific instruments (will be applied as per the recommended age range of the instrument)* →																