



## IP12

How to Measure and Value Health Benefits to Facilitate Priority Setting for Pediatric Population? Development and Application Issues

17:00 – 18:00 September 10

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## Speakers

- |                   |                                                                                                              |
|-------------------|--------------------------------------------------------------------------------------------------------------|
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## Generic Multi-Attribute Utility (MAU) Instruments for Paediatric Populations

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## Conflict of Interests

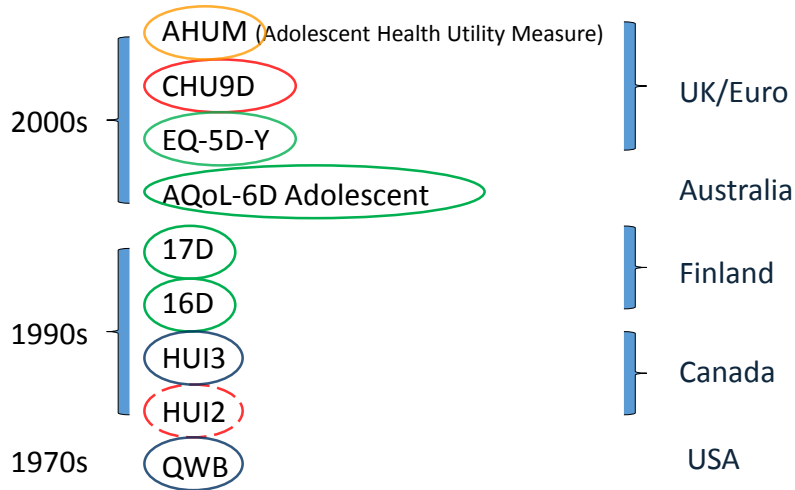
- The AQL instrument was developed by Prof. Richardson and the AQL team at CHE, Monash University; GC is the current contact person for the AQL instruments
- GC was involved in the development of Australian-specific & Chinese-specific CHU9D tariffs

1. What paediatric MAU instruments are available?
2. What techniques have been used for health state valuation among young people?
3. Are MAU instruments comparable?
4. Mapping: what's special for paediatric population?
5. MAU vs SWB: substitutes or complements?

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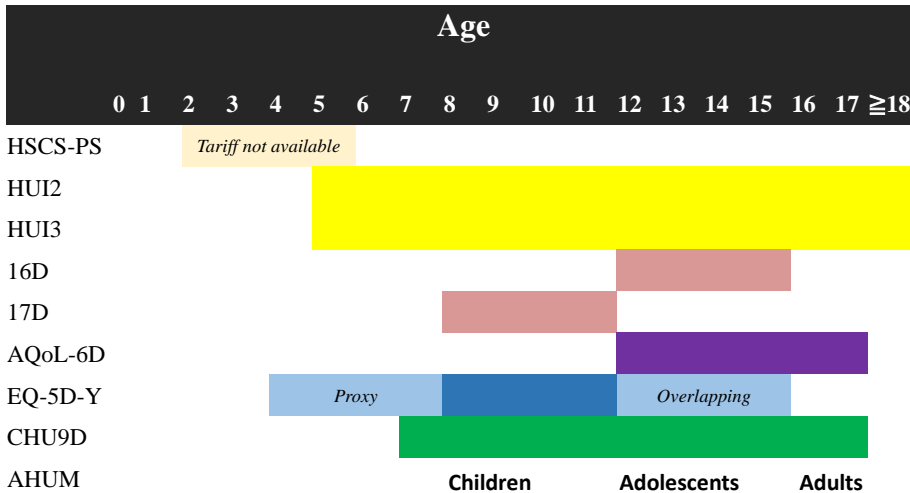
## I. What Paediatric MAU Instruments are available?

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Adaption				
	Item deleted	Item added	Response level added	Item reformulated to be age-appropriate
16D from 15D	sexual life	physical appearance; friends	None	usual activities
17D from 16D	distress	ability to concentrate, learning ability and memory, anxiety	None	vision, vitality, depression
AQoL-6D Adolescent from AQoL-6D	None	None	household tasks	household tasks, getting around, self-care, friendships, family, community, despair, agitation, control, coping, frequency of pain, degree of pain, seeing, communication
EQ-5D-Y from EQ-5D	None	None	None	mobility, self-care, usual activities, pain/discomfort, and worried, sad or unhappy



HSCS-PS, Health Status Classification System-Preschool

Summary of preference-based instruments for paediatric populations

Instrument	Dimension and domains descriptive system	No. of items (response levels)	Valuation method	No. of health states	Valuation study participants
HUI-2 [37]	7: sensation, mobility, emotion, cognitive, self-care, pain, fertility	15 (4-6)	VAS & SG	8900	Canada: (age groups 5-8, 12-16, 8-16) proxy report: General population
HUI-3 [38]	8: sensation, pain/discomfort, emotion, mobility, vision, hearing, speech, cognition	12 (5-6)	VAS & SG	972,000	Canada: (age groups 5-8, 12-16, 8-16) proxy report
AQoL-6D [62]	6: independent living, senses, mental health and relationships	20 (4-6)	TTO	7.6x 10 <sup>3</sup>	Australia: adult general population sample
SF-6D <sup>®</sup> [63]	6: physical functioning, role limitations, social functioning, pain, mental health, vitality	11 (3 and 6)	SG	18,000	UK: adult general population sample (N=611)
EQ-5D-5L-5D-Y [64]	5: mobility, self-care, usual activities, pain/discomfort, anxiety/depression	5 (3)	TTO	243	UK: children and adolescents
16DF [65]	16: mobility, vision, hearing, speech, breathing, sleeping, discomfort and symptoms, eating, excretion, school, hobbies, friends, depression, mental function, distress, vitality and physical appearance	16 (5)	RS	1.5x 10 <sup>5</sup>	Finland: adolescent-school children aged 12-15 years. (N=213)
17DF [66]	17: mobility, vision, hearing, speech, breathing, sleeping, discomfort and symptoms, eating, excretion, school, concentration, learning, memory, hobbies, friends, depression, anxiety, vitality and physical appearance	17 (5)	RS	7.6x 10 <sup>4</sup>	Finland: parent proxy report - School children aged 8-11 years. (N=115)
CHU9D [67]	9: worried, pain, tired, annoyed, sleep, schoolwork/homework, ability to join in activities and daily routine	9 (1-5)	SG	1,053,125	UK: adult general population sample mean age 48 years
AHUM [68]	6: self-care, pain, mobility, health perceptions, self-fragile and perceptions of strenuous activities	6 (3-7)	TTO	16,800	UK: adult general population ≥ 18 years
QWB-SA [69]	3: mobility, physical activity, social and self-care activity	76 (2-4)	VAS	945	US: adult population (aged 18-85 years.)

Source: Mpundu-Kaambwa C, Chen G, Huynh E, et al. Quality of Life Research (2018); Table 4)

- Chen G & Ratcliffe J. (2015). A Review of the Development and Application of Generic Multi-Attribute Utility Instruments for Paediatric Populations. *Pharmacoeconomics*, 33 (10): 1013-1028.
- Thorrington D & Eames K (2015). Measuring Health Utilities in Children and Adolescents: A Systematic Review of the Literature. *PLoS ONE*, 10 (8): e0135672.
- Wolstenholme JL, Bargo D, Wang K, et al. (2018). Preference-based measures to obtain health state utility values for use in economic evaluations with child-based populations: a review and UK-based focus group assessment of patient and parent choices. *Quality of Life Research*, 27 (7): 1769-1780.
- Kwon J, Kim SW, Ungar WJ, et al. (2018). A Systematic Review and Meta-analysis of Childhood Health Utilities. *Medical Decision Making*, 38 (3), 277-305.

## II. What Techniques Have Been used for Health State Valuation among Young People?

## Cardinal Technique Time-Trade Off (TTO)

Health State A		Health State B	
Physical ability	Excellent	Physical ability	Excellent
Social and family relationships	My close friendships make me generally unhappy. There are some group activities I am not involved in because of my health.	Social and family relationships	Excellent
Mental health	I usually feel sad I often feel worried I am sometimes calm and sometimes agitated	Mental health	Excellent
Coping	Excellent	Coping	Excellent
Pain	I suffer from severe pain. Pain often interferes with my usual activities.	Pain	Excellent
Vision, hearing & communication	Excellent	Vision, hearing & communication	Excellent

OR

Imagine that you are in Health State A and that you have 10 years left to live.

We are interested to know whether you would be prepared to live for less than 10 years if your health could be excellent as in B.

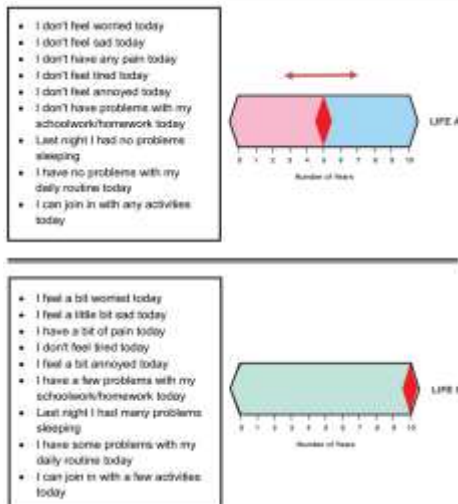
Please mark on the line the shortest time you would accept in B instead of 10 years in A.

This is the amount you have chosen to live in excellent health. You are giving up the rest of the 10 years.

Source: Moodie M, Richardson J, Rankin B, et al. Value in Health (2010; Fig. 3)

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## Cardinal Technique Time-Trade Off (TTO)



**(Better than dead)**

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Ordinal Technique  
Best Worst Scaling (BWS)

Health State X	Best	Worst	Second Best	Second Worst
I feel a little bit worried today	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
I feel a little bit sad today	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>
I have a little bit of pain today	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>
I feel a little bit tired today	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
I feel a little bit annoyed today	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
I have a few problems with my school work today	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
Last night I had a few problems sleeping	<input type="radio"/>	<input type="radio"/>	<input checked="" type="radio"/>	<input type="radio"/>
I have a few problems with my daily routine today	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>
I can join in with most activities today	<input checked="" type="radio"/>	<input type="radio"/>	<input type="radio"/>	<input type="radio"/>

Source: Ratcliffe J, Huynh E, Chen G, et al. Social Science & Medicine (2016; A.Fig. 1) 15

Ordinal Technique  
Discrete Choice Experiment (DCE)

Health description A	Health description B
You live for 10 years with the following then you die:	You live for 1 year with the following then you die:
You feel a little bit worried	You feel a little bit worried
You feel a bit sad	You feel very sad
You have a bit of pain	You don't have any pain
You feel quite tired	You feel quite tired
You feel quite annoyed	You don't feel annoyed
You can't do work/housework	You have many problems with your work/housework
You have a few problems sleeping	You can't sleep at all
You can't do your daily routine	You have a few problems with your daily routine
You can join in with any activities	You can join in with any activities

Which do you prefer?

Note: Adult Respondents

Source: Rowen D, Mulhern B, Stevens K & Vermaire JH. Value in Health (2018; Fig. 2)



- (BWS) DCE is rooted in random utility theory (Thurstone, 1927; McFadden, 1974)

$$U = V + \varepsilon$$

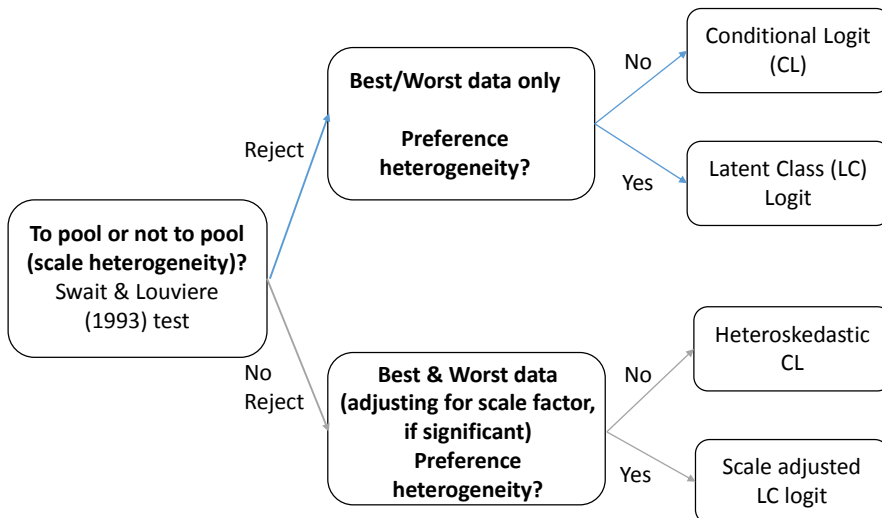
- With duration (see Rowen et al, Value in Health, 2018)

$$\mu_{ij} = \alpha_i + \beta_1 t_{ij} + \beta'_2 \mathbf{X}_{ij} t_{ij} + \varepsilon_{ij}$$

marginal rate of substitution

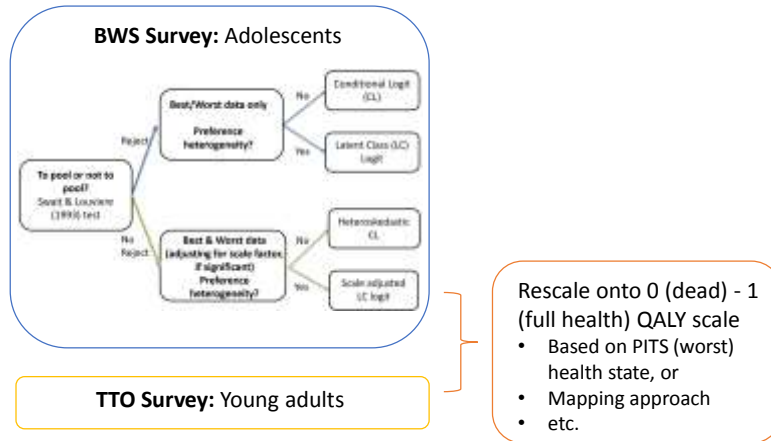
$$\frac{\beta_{2ij}}{\beta_1}$$

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Example from CHU9D Australian/Chinese valuation study

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Example from CHU9D Australian/Chinese valuation study

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### III. Are MAU Instruments Comparable?

*Health state classification system*  
+  
*value set*

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Australia

- **Class I** placed the most importance on the mental health dimensions of the CHU9D (e.g. Worried and Annoyed) and the least importance on daily activities (e.g. Activities, Daily routine, Sleep)
- **Class II** placed equal weights on all attributes

China

- **Class I** placed the most importance on the Activities dimension of the CHU9D and the least importance on the Annoyed dimension
- **Class II** placed the most importance on the Schoolwork dimension and the least importance on Pain

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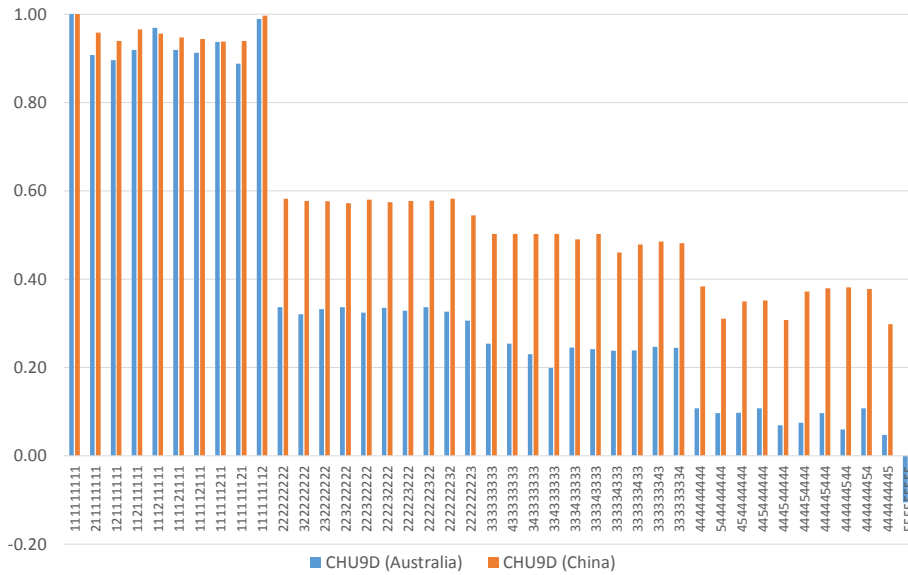
**The PITS (worst) health state**

UK (SG) Adults (16-87 years)	Australia (TTO) Young adults (18-29 years)	Mainland China (TTO) Young adults (17-20 years)	Neitherlands (DCE+Duration) Adults (rep adults)
0.34	-0.2118*	-0.0855*	-0.568

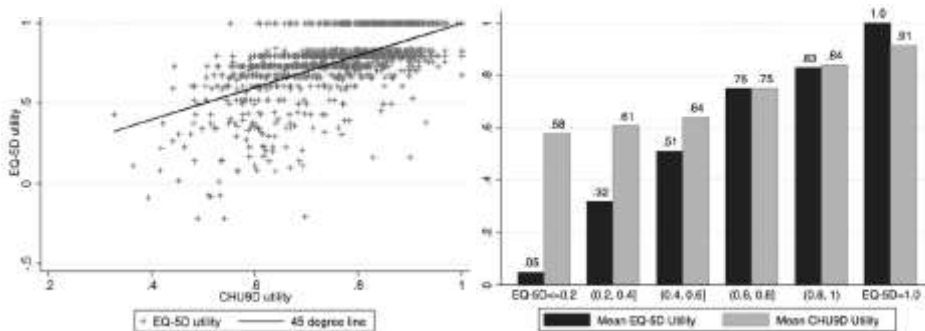
\*Value from the TTO part of the valuation task; not the final tariff

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## Country-specific tariff?



## Multi-Instrument Comparison



Note: EQ-5D-Y was scored using the EQ-5D tariff (developed from adults)

Source: Chen G, Flynn T, Stevens K, et al. Value in Health (2015; Fig. 1 & 2)

**Table 3** Mean, standard deviation, and responsiveness statistics among patients with 20% or greater improvement in CDRS-R between baseline and follow-up

	<i>N</i>	Baseline mean (SD)	Follow-up mean (SD)	Mean change (SD)	Effect size	Standardized response mean
<b>Multi-attribute utility instruments</b>						
HUI2	157	0.75 (0.18)	0.92 (0.10)	0.20 (0.20)	1.08	0.97
HUI3	159	0.56 (0.27)	0.88 (0.16)	0.32 (0.28)	1.19	1.17
EQ-5D-3L	174	0.81 (0.15)	0.89 (0.13)	0.08 (0.15)	0.53	0.51
QWB	178	0.60 (0.09)	0.71 (0.12)	0.11 (0.12)	1.17	0.86
SF-6D	171	0.67 (0.09)	0.79 (0.11)	0.12 (0.11)	1.25	1.02
<b>General health status measures</b>						
PEDS-QL total	177	64.3 (14.4)	78.4 (12.0)	-14.0 (12.6)	0.97	1.11
<b>RAND-36</b>						
Physical health composite score	169	0.71 (0.84)	0.66 (0.70)	0.05 (0.84)	-0.06	-0.06
Mental health composite score	169	-2.02 (1.34)	-0.32 (1.19)	1.70 (1.46)	1.26	1.16
<b>Disease-specific health status measures</b>						
QLDS	177	10.0 (7.7)	3.3 (4.4)	6.7 (6.8)	-0.87	-0.99

Source: Dickerson JF, Feeny DH, Clarke GN, et al. Quality of Life Research (2018)

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## IV. Mapping

- To predict the health utilities from non-preference based instrument for cost-utility analyses
- To facilitate the comparison on health utilities elicited from different instruments
  - E.g. Chen et al. (Medical Decision Making, 2016); Gamst-Klaussen et al. (Quality of Life Research, 2016).

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Mapping functions	Data sources	References
<b>PANEL A – GENERIC INSTRUMENTS</b>		
KIDSCREEN-10 → CHU9D	Online-panel (11-17 yrs)	Chen et al. (Health and Quality of Life Outcomes, 2014)
PedsQL GCS → EQ-5D-Y	Students (11-15 yrs); adult tariff	Khan et al. (Pharmacoeconomics, 2014)
PedsQL GCS → HUI3	Children with autism (4-17 yrs); proxy	Payakachat et al. (Autism Res. 2014)
PedsQL SF15 → CHU9D	Online-panel (15-17 yrs)	Mpundu-Kaambwa et al. (Pharmacoeconomics, 2017)
PedsQL GCS → CHU9D	Children with CSNS (5-13 yrs); proxy	Lambe et al. (Pharmacoeconomics, 2018)
<b>PANEL B – DISEASE-SPECIFIC INSTRUMENTS</b>		
SDQ → CHU9D	Mental health (5-17 yrs); proxy ← externally validated; proxy-self	Furber et al. (Quality of Life Research, 2014); Boyer et al. (Quality of Life Research, 2016)
CSNS, corticosteroid-sensitive nephrotic syndrome; SDQ, Strengths and Difficulties Questionnaire 27		

## V. MAU vs SWB: Substitutes or Complements?

- To facilitate resource allocation, the subjective well-being (SWB) (an alternative broader construct) has gained increasing attention in the policy debate.
- Evidence from adults:
  - ✓ **Complements**, e.g. Cubí-Mollá et al. (Value in Health, 2014, Parkinson's disease); Liu et al. (Quality of Life Research, 2018, psoriasis)
  - ✓ **Substitutes (strictly, it dependents)**, e.g. Chen et al. (Social Indicators Research, 2018); Engel et al. (Quality of Life Research, 2018, mental health)
- Evidence from children and adolescents:
  - ✓ **Complements**, e.g. Yang P (PhD Thesis, Xi'an Jiaotong University)

Thank you!

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