Selecting preference-based measures (PBMs) for paediatric studies: A proposed decision-making framework

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Background

- HTA agencies evaluate new health technologies based on generic preference-based measures (PBMs*),[1] in an effort to inform decision-makers on policies supporting equitable and efficient health systems.[2] A lack of data concerning generic paediatric PBMs impedes the selection of PBMs in clinical studies of paediatric populations.
- This can lead to challenges in assessing benefits associated with paediatric treatments in economic evaluations, potentially limiting patient access to treatments.
  - It is estimated, for example, that only 29% of paediatric cost–utility analyses have used PBMs validated for use in children.[3]
- The Paediatric Utilities Working Group was initiated by field experts and Roche to develop a decision-making framework to select appropriate PBMs for paediatric disease populations early in the clinical development process.
  - Timely selection of appropriate PBMs in trials should prove beneficial to treatment consideration and clinical outcomes in the daily life of patients.

*PBMs are structured questionnaires evaluating a respondent's health state or health-related quality of life with a value that infers the preference for each health state.

Methods

- A recent systematic review of paediatric PBMs[4] was used as the base for this study.
- All available paediatric PBMs (for individuals <18 years of age) that use established preference-based methodology were catalogued in a database.
  - Properties of each PBM were collated in a database based on the original PBM validation papers.
- A series of workshops were held with PBM subject matter experts to identify the appropriate elements and a decision-making framework.
- The Paediatric Utilities Working Group collectively refined the elements and created a **Process map**, or decision-making framework, for PBM selection for paediatric clinical trials.

Results

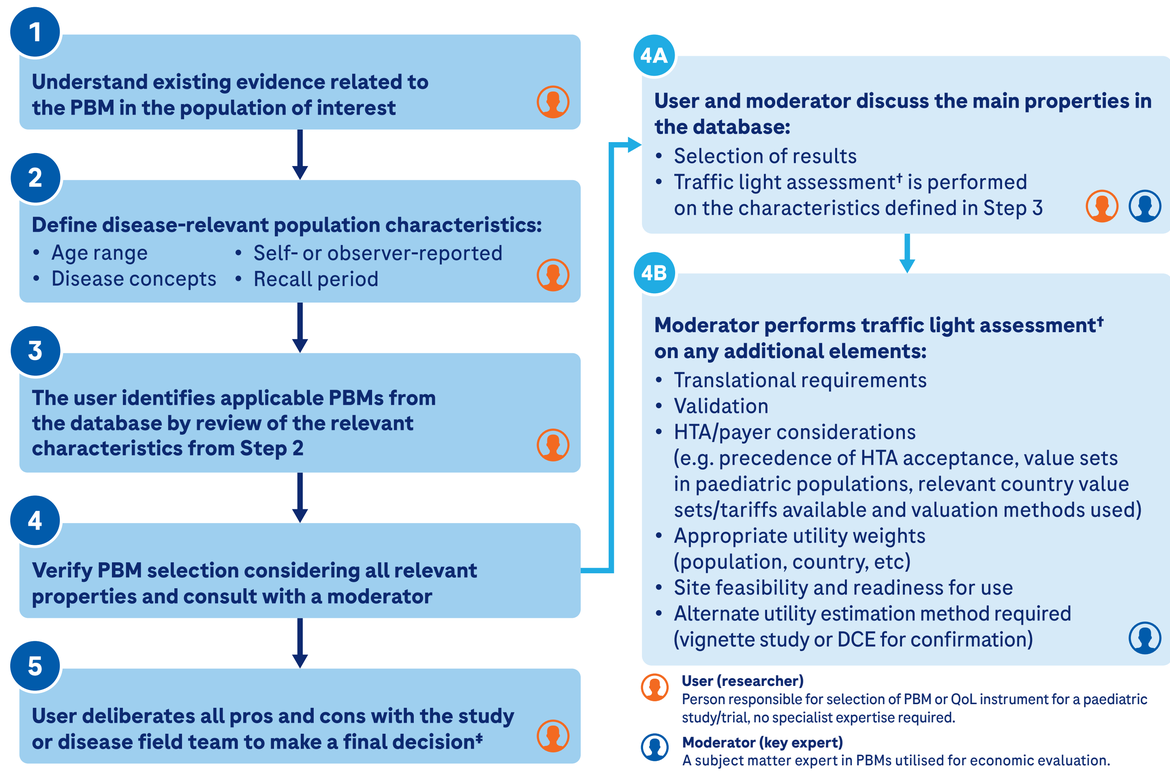
- A total of 19 generic multidimensional PBMs were identified in the systematic review,[4] from which 14 paediatric PBMs were included in the database.
  - PBMs were merged in the database if they were observer-reported versions and/or variants of the same PBM.*
- The database contains a catalogue of the final PBMs and over two dozen relevant properties (e.g. the target age group, concepts covered, country value sets available, and more) to consider during PBM selection.

- The database includes the following 14 PBMs identified for paediatric populations:
  - Infant health-related Quality of life Instrument (IQI)[5]
  - EuroQoL Toddler and Infant Populations (EQ-TIPS)[6]
  - Health Utilities-Preschool (HuPS)[7]
  - Health Utilities Index (HUI)[8]
  - Quality of Life Inventory-Disability (QI-Disability)[9]
  - Child Health 6-Dimensional (CH-6D)[10]
  - Child Health Utility 9D (CHU9D)[11]
  - 16-Dimensional Health-Related Measure (16-D)[12]
  - 17-Dimensional Health-Related Measure (17-D)[13]
  - EQ-5D[14]
  - EQ-5D-Y[15]
  - Adolescent Health Utility Measure (AHUM)[16]
  - Assessment of Quality of Life, 6-Dimensional, Adolescent (AQoL-6D Adolescent)[17]
  - Quality of Well-Being scale (QWB)[18]

*PBMs with different variations on the same measure or those that included self-reported and observer-reported versions were added to the database as a single PBM for ease of reference. For example, the HUI2, HUI3, as well as their respective observer-reported versions, were included as a single HUI PBM. EQ-5D-Y, EQ-5D-Y proxy and EQ-5D-Y-5L were included as a single EQ-5D-Y PBM.

Process map

- The study team identified choices to be made by "Users" and "Moderators" via the **Process map** in five discrete steps.



- In future, this decision-making framework may be mapped for use in specific diseases.

*Concepts can include various dimensions that may be disease specific or generic, for example activities of daily living, mobility behaviour/emotion and pain. †Traffic light assessment determines if the chosen PBM is valid for use (green), should be used only if no other valid PBM is available as some features are missing/invalid (orange), or the PBM is invalid for use in the target population (red). ‡In cases where no PBM is suitable, the moderator will deliberate with the disease team on the approaches needed to generate utilities.

Limitations

- Most PBMs included in the database were based on the review by Kwon J, et al. (2022).[4] More recent publications should be reviewed for possible inclusion of additional PBMs in the database.
- The focus of this decision-making **Process map** was based on generic PBMs. Disease-specific PBMs that have been validated for use in paediatric populations could be useful across a broader range of disease areas where there is a commonality in concepts, and could be considered in future versions of the database.
- This decision-making **Process map** has not been evaluated for other diseases and/or health settings, tested externally or validated with decision-makers.

Conclusions

- Initial assessment of the decision-making **Process map** suggests that it may enable a systematic and transparent method for optimising the selection of appropriate existing paediatric PBMs into studies and an accurate estimation of utilities for paediatric populations.
- More work is underway to assess the accuracy and usefulness of this **Process map**.

Database*

PBMs	IQI[5]	EQ-TIPS[6]	HuPS[7]	HUI[8]	QI-Disability[9]	CH-6D[10]	CHU9D[11]	16-D/17-D[12,13†]	EQ-5D/ EQ-5D-Y[14,15†]	AHUM[16]	AQoL-6D Adolescent[17]	QWB[18]
Age range	0–1 year	1–3 years	2–5 years	≥5 years	5–18 years	7–12 years	7–17 years	8–15 years	8–18 years	12–18 years	Adolescent‡	Not reported
Concepts	• Sleeping • Feeding • Breathing • Stooling • Mood • Skin • Interaction	• Movement • Play • Pain • Relationships • Communication • Eating • General health	• Vision • Hearing • Speech • Mobility • Dexterity • Emotion • Self-care • Learning • Thinking • Problem-solving • Pain • Behaviour • General health	• Vision • Hearing • Speech • Mobility • Dexterity • Emotion • Cognition • Pain	• Social Interaction • Physical health • Independence • Positive emotions • Leisure and outdoors • Negative emotions	• Studying • Exercise • Energy • Mood • Pain • Discomfort • Playing with friends	• Worried • Sad • Annoyed • Pain • School work • Daily routine • Tired • Joining activities • Sleep	• Mobility • Vision • Hearing • Breathing • Sleeping • Eating • Elimination • Speech • Mental function • Discomfort and symptoms • School and hobbies • Friends • Physical appearance • Anxiety • Vitality and hobbies • Ability to concentrate • Learning ability and memory	• Anxiety/ Depression • Mobility • Pain/ Discomfort • Self-care • Usual activities	• Self-care • Pain • Limitations walking around • Self-image • Perception of strenuous activities and health perceptions	• Communication • Energy • Hearing • Impact on social activities • Mental health • Mobility • Pain • Discomfort • Physical activity • Relationships • Social support • Self-care • Vision	• Chronic symptoms or problems • Physical symptoms • Mental health symptoms and behaviours • Mobility • Physical activity • Social activity
Self-reported vs. observer-reported	Observer	Observer	Observer	Both	Observer	Self	Both	Both	Both	Self	Self	Both
Recall period	Today	Today	Past week	General health	General health	Today[19]	Today or last night	Today	Today	Today	Past week	Past three days

*Refer to Process Map Step 4A. †For ease of reference the 16-D and 17-D, as well as the EQ-5D and EQ-5D-Y PBMs, are collated above. ‡Age range not specified.

Abbreviations

16-D, 16-Dimension Health-Related Measure;
17-D, 17-Dimension Health-Related Measure;
AHUM, Adolescent Health Utility Measure;
AQoL-6D, Assessment of Quality of Life, 6-Dimensinal, Adolescent;
CHU-9D, Child Health Utility 9D; DCE, discrete choice experiment;
EQ-TIPS, EuroQoL Toddler and Infant Populations;
HTA, Health Technology Assessment;
HUI, Health Utilities Index;
HuPS, Health Utilities-Preschool;
IQI, Infant health-related Quality of life Instrument;
PBM, preference-based measures;
QI-Disability, Quality of Life Inventory-Disability;
QoL, quality of life;
QWB, Quality of Well-Being scale;
SG, standard gamble;
TTO, time trade-off.

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