



Zorginstituut Nederland

Using HRQoL data from children and youth to strengthen HTA.

Experiences from the National Health Care Institute

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Disclaimer

Advisor working at National Health Care Institute with experience in pharmaceutical assessment and methods development with focus on use for policy/decision making

I am not a member of EuroQol group or received any funding from the EuroQol Group

Experienced problems regarding Quality of Life data

Quality of life (QoL) very relevant outcome measure for decision making

Often no QoL data available from clinical trials

- Not collected
- Selection of patients e.g. patients without progress
- Not validated or very specific disease related QoL instrument

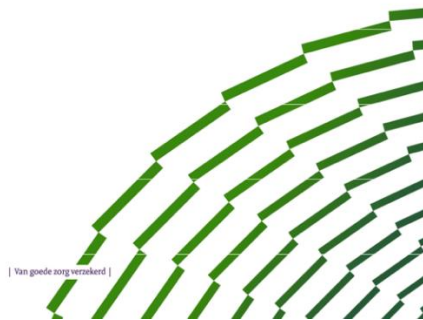
Additional issues QoL from children and youth

- Expert opinion from caretakers and health professionals
- Not always comparable instrument for adult population



Kwaliteit van leven bij pakketbeoordelingen

Definitief | 23 september 2024





Guideline for economic evaluations in healthcare

2024 version

New health economic guideline

Update of Dutch health economic guideline published in January 2024

October 2024 onwards: guideline mandatory for reimbursement dossiers from pharmaceutical industry

Two types of changes:

- **Methodological changes**, e.g. changes or new elements in reference case
- More clarification or guidance, e.g. extrapolation or expert opinion/elicitation

Guidance on quality of life

Since 2016 EQ-5D-5L mandatory

- Second choice EQ-5D-3L

Use of Dutch value sets

Children 8 to 12 years: EQ-5D-Y

Scenario analyses

- Quality of life informal caregiver (EQ-5D-5L)
- Other sources of quality of life data

Changes compared to the 2016 guideline

The most important changes in the current version compared to the previous version of the guideline are as follows:

- The discount rate for costs has been adapted to 3%;
- Medical costs in life years gained are included in the base case analysis;

- The results of both the base case analysis and scenario analyses must be based on the probabilistic analysis;
- Value of information (VOI) analysis, in the form of the expected value of perfect information (EVPI) and expected value of partial perfect information (EVPPi), is an obligatory element in the case of model-based economic evaluations;
- The quality of life of informal caregivers must be included in a scenario analysis when relevant;
- The EQ-5D-Y must be used to measure the quality of life of children aged 8-12.

The guideline also contains greater clarification regarding:

- empirical economic evaluations;
- the extrapolation of time-to-event data;
- subgroup analyses;
- uncertainty analyses;
- validation;
- expert opinion and expert elicitation.

Collecting HRQoL data by children and youth

Adult version too complicated for children

Measuring quality of life by person experiencing health problem

Table 1 – Recommendations for use of youth and adult EQ-5D versions in different age ranges of children and adolescents.	
Age range	Recommendation
0-7 years	No self-reported EQ-5D-Y for youngest children available at present <i>For children aged 4-7 years one of the proxy versions can be used.</i>
8-11 years	Use EQ-5D-Y <i>The EQ-5D-Y is more understandable for children than the Adult EQ-5D.</i>
12-15 years	Both Youth and Adult EQ-5D versions can be used (overlapping area) <i>Generally, EQ-5D-Y is recommended. Nevertheless, depending on study design, the usage of the EQ-5D adult version might be possible.</i>
16 years and older	Use one of the Adult versions (EQ-5D-3L or EQ-5D-5L) <i>Possible exception: a study with only children up to 18 years—in this case EQ-5D-Y for older children would be recommended in order to have only one EQ-5D version in the study. The switchover to the adult version could bring discontinuity because the adult and child versions are two different instruments.</i>
Reference: EuroQol Group. ¹⁷	

Advice given by EuroQol group:

- < 8 years: proxy versions
- 8 to 12 years: use EQ-5D-Y
- 12 to 15 years old: both adult and youth version can be used
- > 16 years: use adult version

Why these recommendations?

Consistency

Available value sets in the Netherlands

Preferably no expert opinion

Generic instruments



Remaining practical issues

When using which version?

- Studies having children/youth and adults
 - All participants same version or different versions
- Studies with longer follow-up starting with children/youth

Comparability value sets

- EQ-5D-3L, EQ-5D-5L and EQ-5D-Y all have own value sets
- Similar utilities for same health states?
 - if yes, no big problems
 - if not, how to deal with this, what is the 'real' utility



Some methodological issues

Important to capture realistic QoL measurements of patients

- Capturing deterioration and fluctuations in health and quality of life

Fluctuating health states

- Not uncommon in diseases affecting children/youth
- Unpredictable by episodic disorders, e.g. epilepsy and migraine
- Sometimes predictable, e.g. adverse events/side effects

Several challenges

- Timing of measurement (fixed or not)
- Recall period (1 day or longer back)
- Worst days often missing



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Thank you for your attention