A Review of Oncology Submissions to NICE to See How Often Disease-Specific Quality of Life Data Have Been Accepted

Davies N, Dickinson O, Treharne C
Mtech Access, Bicester, UK

ISPOR poster acceptance code: HTA194
Abstract ID: 121231

Introduction

- The National Institute for Health and Care Excellence (NICE) makes recommendations at a national level for oncology drugs in England as part of their technology appraisals program
- To enable consistency across evaluations, NICE states a preference for the EQ-5D instrument, a generic quality of life (QoL) measure (1). When EQ-5D data are not available, the NICE manual recommends mapping other health-related QoL (HRQoL) measures (e.g. disease-specific measures) to EQ-5D (1)
- NICE acknowledges that, in some circumstances, the EQ-5D may not be the most appropriate measure. In such circumstances, other generic or disease-specific preference-based measures may be used, as long as sufficient evidence is provided regarding their appropriateness

Objectives

- To explore how often disease-specific QoL measures have been used in manufacturers' economic analyses in recent oncology submissions to NICE
- To understand approaches taken by manufacturers when using disease-specific QoL measures, and how these approaches have been received by NICE

Methodology

- All NICE single technology appraisals (STAs) for oncology products published between 1st April 2021 – 31st March 2022 were reviewed
- The following information was extracted from relevant committee papers and final appraisal documents:

1. Appraisal outcome
2. Source of QoL data
3. Mapping techniques applied to HRQoL

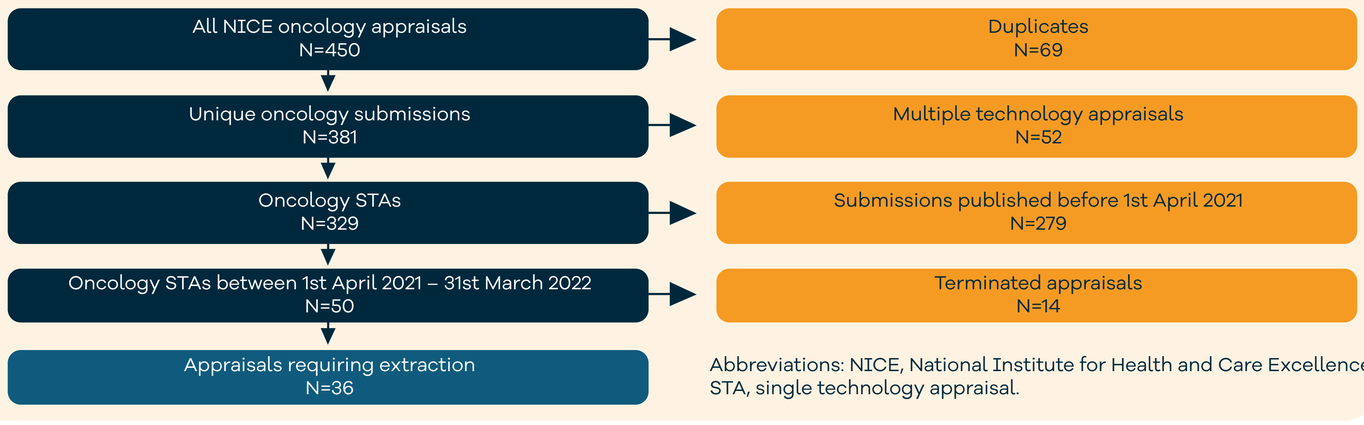
Conclusion

- In most recent oncology STAs, manufacturers have leveraged data collected using the generic EQ-5D instrument, in line with NICE preferences
- Where data collected via disease-specific instruments have been used, mapping to EQ-5D-3L via published algorithms has been accepted in the absence of collected EQ-5D data. However, manufacturers should carefully consider the clinical plausibility of resulting utility values. Where no mapping algorithms exist, or where mapped values lack plausibility, literature-based values may be acceptable alternatives
- Our research identified one appraisal where a disease-specific preference-based instrument was accepted, illustrating that NICE is willing to deviate from its preference for EQ-5D data where a case can be made for its unsuitability

Results

A total of 50 NICE STAs of oncology treatments were identified via a review of NICE's log of cancer appraisal recommendations (2); 36 appraisals met the inclusion criteria for data extraction.

Figure 1: PRISMA flow chart



Abbreviations: NICE, National Institute for Health and Care Excellence; STA, single technology appraisal.

Of the 36 appraisals meeting the inclusion criteria for the review:

30 used generic QoL measures: **29** EQ-5D (23 = EQ-5D-3L and 6 = EQ-5D-5L) and **one** SF-36 data mapped to EQ-5D-3L

Six leveraged disease-specific QoL data to inform their economic analyses

Appraisals using disease-specific QoL measures

- Two appraisals in multiple myeloma and cholangiocarcinoma (TA695 and TA722) used disease-specific QoL data from clinical trials mapped to EQ-5D-3L utility scores using published mapping algorithms (3,4), as suggested in the NICE methods (1). In both instances the **EORTC-QLQ-C30** instrument was mapped to EQ-5D-3L
- A third appraisal in non-small cell lung cancer (TA760) mapped **EORTC-QLQ-C30** values to EQ-5D-3L using a published algorithm by Khan et al (5). However, the manufacturer concluded that mapped values lacked clinical plausibility, and utility values were ultimately derived from the literature
- In an appraisal in thyroid cancer (TA742), the manufacturer searched for a mapping algorithm to map **EORTC-QLQ-C30** to EQ-5D-3L, but, in the absence of a suitable algorithm, leveraged utility values from past appraisals
- In an appraisal in chronic lymphocytic leukaemia (TA689), the manufacturer deemed that EQ-5D data collected for progressed disease were limited and lacked face validity. As a result, **EORTC-QLQ-C30** and **FACIT** utility values from literature, and used in previous appraisals, were used for progressed disease utility in the model's base case
- In appraisal TA756, NICE accepted the use of a disease-specific preference-based measure, the **MF-8D**. This was justified due to the inadequate psychometric properties of the EQ-5D in myelofibrosis (MF) and concerns that EQ-5D could not detect clinically meaningful changes in HRQoL in patients with MF

- Mukuria et al (2015) developed the MF-8D to overcome concerns related to using EQ-5D and EORTC QLQ-C30 in the MF population (6)
- MF-8D is derived by combining data from the MF-SAF and EORTC QLQ-C30 to generate utility scores
- Although the EORTC QLQ-C30 can capture functioning and generic symptoms associated with MF, it is less able to capture MF-specific symptoms and is not as responsive over time as the MF-SAF

The 8 dimensions of the MF-8D are:
1. Physical functioning (from EORTC QLQ-C30)
2. Emotional functioning (from EORTC QLQ-C30)
3. Fatigue (from EORTC QLQ-C30)
4. Itchiness (from MF-SAF)
5. Pain under ribs on the left side (from MF-SAF)
6. Abdominal discomfort (from MF-SAF)
7. Bone or muscle pain (from MF-SAF)
8. Night sweats (from MF-SAF)

Table 1: Overview of appraisals using disease-specific utility measures

TA	Drug	Indication	Disease-specific utility measure(s)	Mapping algorithm used to map to EQ-5D
TA689	Acalabrutinib	Chronic lymphocytic leukaemia	EORTC QLQ-C30, FACIT	Observed EQ-5D data from trial were limited; therefore, disease-specific measures from literature, and used in previous appraisals, were used with no additional mapping
TA695	Carfilzomib with dexamethasone and lenalidomide	Multiple myeloma	EORTC QLQ-C30	Proskorovsky et al (2014) (3) ordinary least squares mapping algorithm model
TA722	Pemigatinib	Relapsed or refractory advanced cholangiocarcinoma with FGFR2 alterations	EORTC-QLQ-C30	'Response mapping' technique by Longworth et al (2014) (4)
TA742	Selpercatinib	Advanced thyroid cancer with RET alterations	EORTC-QLQ-C30	No mapping conducted due to lack of published mapping algorithm. Attempts to map values resulted in implausible utility values. Base case utilities were sourced from literature
TA756	Fedratinib	Disease-related splenomegaly and symptoms in myelofibrosis	MF-8D	No mapping conducted due to concerns with EQ-5D's ability to detect changes in QoL. MF-8D accepted by the ERG
TA760	Selpercatinib	RET fusion-positive advanced non-small cell lung cancer	EORTC-QLQ-C30	Mapped values lacked clinical plausibility; therefore, utility values were sourced from the literature

Abbreviations: CDR, Cancer Drug Fund; ERG, Evidence Review Group; QoL, quality of life; RET, rearranged during transfection.

Recommended | Recommended (CDF) | Optimised recommendation

Four of the six appraisals using disease-specific QoL measures were ultimately recommended

The remaining two appraisals received an optimised recommendation meaning they were recommended for a smaller group of patients than the original scope of the appraisal

Mtech Access

ISPOR Europe 2022, 6–9 November 2022, Vienna, Austria

(Stand No. X2-304)

References

1. NICE. Guide to the methods of technology appraisal 2013. 2013. Available from: https://www.nice.org.uk/process/pmg9/resources/guide-to-the-methods-of-technology-appraisal-2013-pdf-2007975843781.
2. NICE. Technology appraisal data: cancer appraisal recommendations. 2022. Available from: https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/data/cancer-appraisal-recommendations
3. Proskorovsky I, Lewis P, Williams CD, et al. Mapping EORTC QLQ-C30 and QLQ-MY20 to EQ-5D in patients with multiple myeloma. Health Qual Life Outcomes 2014;12:35.
4. Longworth L, Yang Y, Young T, et al. Use of generic and condition-specific measures of health-related quality of life in NICE decision-making: a systematic review, statistical modelling and survey. Health Technol Assess. 2014;18(9):1-224.
5. Khan I, Morris S, Pashayan N, Matata B, Bashir Z, Maguirre J. Comparing the mapping between EQ-5D-5L, EQ-5D-3L and the EORTC-QLQ-C30 in non-small cell lung cancer patients. Health and Quality of Life Outcomes. 2016;14(1):60.
6. Mukuria C, Rowen D, Brazier JE, et al. Deriving a Preference-Based Measure for Myelofibrosis from the EORTC QLQ-C30 and the MF-SAF. Value Health. 2015; 18(6):846-55.

Abbreviations

CDF, Cancer Drug Fund; EA, Evidence assessment group; EORTC, European Organization for Research and Treatment of Cancer; EQ-5D, EuroQol-5-dimension; FACIT, Functional Assessment of Cancer Therapy; HRQoL, Health-related quality of life; MF, Myelofibrosis; MF-8D, Myelofibrosis-Symptom Assessment Form 8-dimension; NICE, National Institute for Health and Care Excellence; QoL, Quality of life; QLQ-C30, Quality of life questionnaire-C30; TA, Technology appraisal