Use of Patient Reported Outcomes (PROs) in Key Trials Supporting Marketing Authorisation: 5-Year Analysis of Company Evidence Submitted for National Institute for Health and Care Excellence (NICE) Single Technology Appraisal (STA)

PCR52

Agata K. Los[1], Grace G. Goldsmith[1], Rhian L.H. Kilty[1], Audrey E. Brown[1], Angaja Phalguni[1]

[1]Genesis Research Group, Newcastle, UK

Background

- Treatment and disease outcomes relating to all aspects of the patient experience, including quality of life (QoL) data, are increasingly being captured in clinical trials.[1]
- PROs can capture a variety of outcomes from the perspective of the patient ranging from general QoL, symptom burden, functional status, treatment satisfaction, and health-related behaviours.[2,3]
- With growing interest in capturing patient experience during clinical trials, the use of patient reported outcome measures (PROMs) has been recommended by regulatory authorities.

Objective

This study aimed to evaluate the use of PROMs in company evidence submitted for NICE STA in the last 5 years.

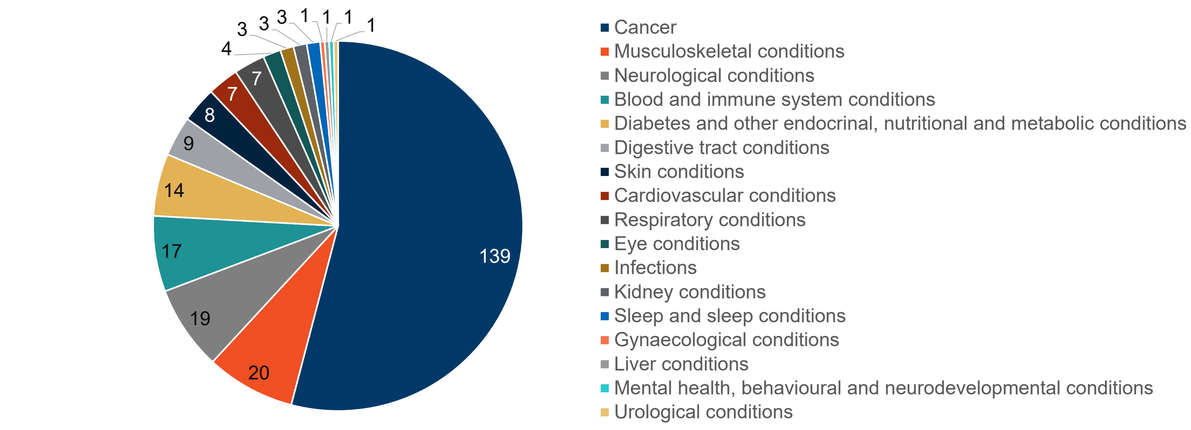
Methods

- A review of the technology appraisal evidence published on the NICE website between April 1st 2018 and April 1st 2023 was performed to identify recommended STAs.
- Technology appraisals which were terminated, replaced by newer guidance, or in development were excluded from the analysis.
- For each technology assessed in the STA, information on disease indication, key trials design, and types of PROMs used was collected. Key clinical trials were identified form the committee discussion section published on the NICE website. Further details including the use of PROMS were identified from the company submission documents. If the use of PROMs was unclear, handsearching of relevant clinical trial registries was conducted.

Results

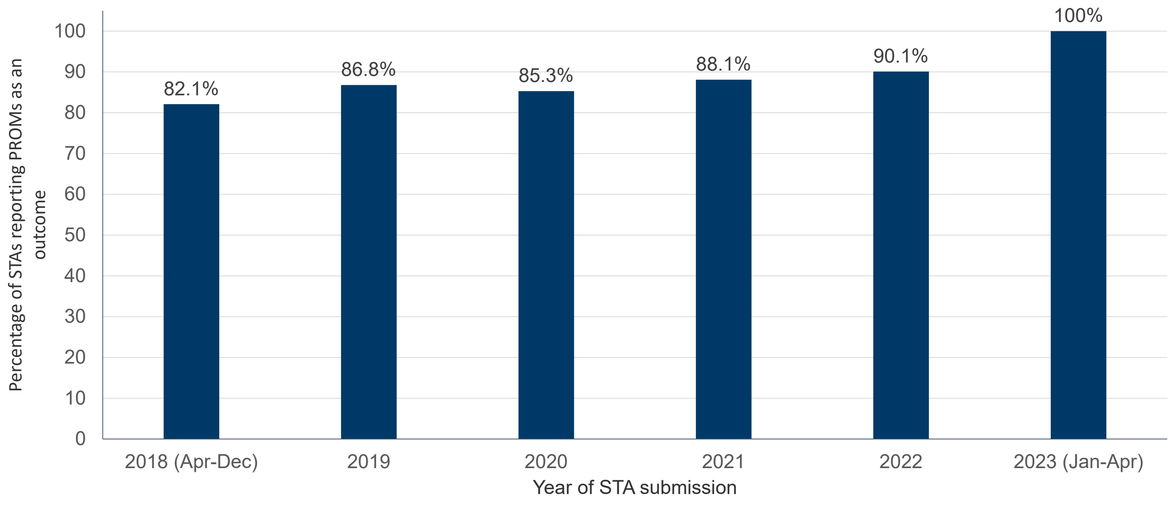
In total, 352 STAs were identified. After excluding appraisals which were terminated, replaced by newer guidance, or in development, 257 STAs were included. Of these 257 STAs, oncology was the most common indication (n=139; 54.1%), followed by musculoskeletal (n=20; 7.8%), and neurological (n=19; 7.4%) conditions.

Figure 1: Indication categories reported in STAs published between April 2018-April 2023.



From April 2018 to April 2023, 227 of 257 STAs included PROMs as an outcome in their key clinical trials. The number of submissions with PROMs listed as an outcome increased from 2019 to 2022. Among all STAs submitted during 2022, 90.1% of company submission documents reported PROMs as an outcome.

Figure 2: The number of STAs reporting PROMs as an outcome in company submission.



Results continued

131 different PROMs were identified (Table 1) and categorised into: (1) indication-specific QoL measures (n=76; 58.0%), (2) generic QoL measures for symptoms (e.g., pain), function (e.g., visual), or safety (e.g., adverse events) (n=30; 22.9%), and (3) generic QoL measures (n=25; 19.1%).

The EuroQol-5 Dimensions (EQ-5D) and the European Organization for Research and Treatment of Cancer Core Quality of Life Questionnaire (EORTC QLQ-C30) that assesses generic QoL, were the most commonly used PROMs (n=144; 63.4% and n=81; 35.7%, respectively).

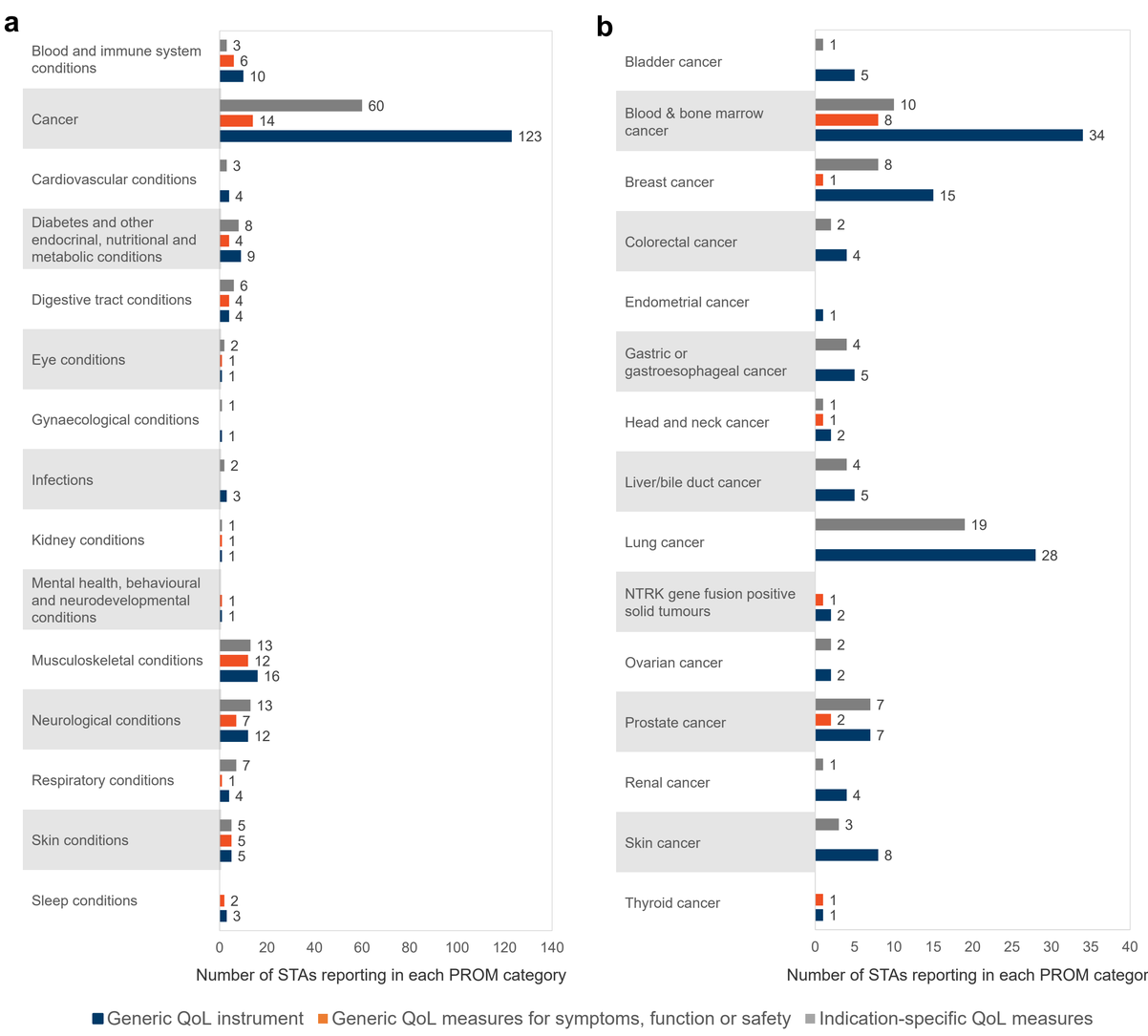
Table 1: List of PROMs used within STAs included in this study.

Generic QoL measures (n=25)
ACCEPT, BRIEF GEC, CDAI, CHAQ, CHQ, CTSQ-16, DCOA, EORTC QLQ-C30, EQ-5D, FACT-G, ITQOL, LASA, MDASI, PedsQL, PGA, PGIC, PGIS, PHQ-9, PRO-CTCAE, PROMIS SF-7a, SF-12, SF-36, SHS, WLQ, WPAI
Generic QoL measures for specific function, symptom or safety (n=30)
BFI, BPI-SF, DLQI, ESS, FACES, FACIT-bladder, FACIT-cognitive, FACIT-F, Fatigue Severity NRS, FOSQ-10, GAD-7, HADS, HIT-6, IIEF, INQOL, ISI, Itch NRS, Itchy-QoL, IWQOL-Lite-CT, KKCQ, NEI-VFQ-25, Neuro-QoL, PAC-QOL, PAC-SYM, QOLIE, Skindex-29, Sleep disruption score, TRIM-weight, TWP, VFQ-25
Indication specific QoL measures (n=76)
ACQ, ADHD-RD/ ASRS, AE-QoL, AQLQ, ASAS HI, ASQoL, BASDAI, DDS2, DTSQ, EoE-QoL-A, EORTC QLQ-BIL21, EORTC QLQ-BR23, EORTC QLQ-BR45, EORTC QLQ-CLL16, EORTC QLQ-CR29, EORTC QLQ-H&N35, EORTC QLQ-HCC 18, EORTC QLQ-LC13, EORTC QLQ-OES18, EORTC-QLQ-MY-20, EORTC-QLQ-PR25, FACT/GOG-NTX, FACT-B, FACT-BI, FACT-BMT, FACT-C, FACT-E, FACT-ES, FACT-Hep, FACT-IT, FACT-Kidney Symptom Index, FACT-Lym, FACT-M, FACT-O, FACT-P, FAIM, FAQLQ, FOSI, GGISIS, HADS-A, HADS-D, HAQ-DI, HAT-QoL, HCC, HIVSTQ, IBDQ, IGISIS, K-BILD, KDQOL-36, LCSS ASBI, MIBS-4, MIDAS, MSFC, MSIS, MSQoL, MSQoL-54, NCCN FBISI-18, Norfolk QoL-DN, NSCLC-SAQ, POEM, Pruritus Likert scale, PSSD, QIDS-SR16, QOLCE, QoLISSY, RA-WIS, RQLQ(S)+12, SAPS, SGRQ, SILC, SNOT-22, UFS-QOL, WPAI-AD, WPAI-GH, WPAI-MS, WPAI-RA

Among all 227 STAs which reported PROMs as an outcome within key clinical trials, the majority of STAs used generic QoL measures (n=197), followed by indication specific QoL measures (n=124), and generic QoL measures for specific symptoms, function, or safety (n=58) (Figure 3a).

We further analysed PROMs used in oncology STAs (Figure 3b). Across 139 STAs, 124 (89.2%) STAs included PROM assessments, the majority of which used generic QoL measures (n=123; 99.2%). Indication-specific QoL measures were used in 62 STAs (50.0%), and use of generic QoL measures for symptoms, function, or safety were reported in 14 STAs (11.3%). Most oncology STAs used a combination of generic and indication-specific PROMs (46.8%) or generic PROMs only (41.9%).

Figure 3: Number of STAs reporting PROM categories by: a) all indications; b) oncology indications.



CONCLUSIONS

- Within the last 5 years of STAs there has been an increase in the number of company submission documents including PROMs in a wide range of indications.
- Across all STAs reporting PROMs, there was a large variety of instruments used with the greatest number and diversity grouped within indication-specific instruments. The EQ-5D and the EORTC QLC-C30 were the most commonly used PROMs, capturing details about patient's general QoL.
- Among all indications, the most common type of instruments used captured information about patient's general QoL, followed by the use of indication-specific instruments.
- Most oncology STAs use a combination of generic and disease-specific PROMs to capture both QoL and general/disease-specific symptomatology.
- The increasing inclusion of PROMs in company submission documents demonstrates the importance of QoL data to support health technology assessment decision making.

REFERENCES

1. Maruszczyk K, Aiyegbusi OL, Cardoso VR, Gkoutos GV, Slater LT, Collis P, Keeley T, Calvert MJ. Implementation of patient-reported outcome measures in real-world evidence studies: Analysis of ClinicalTrials.gov records (1999-2021). Contemp Clin Trials. 2022 Sep;120:106882. doi: 10.1016/j.cct.2022.106882.
2. Weldring T, Smith SMS. Article Commentary: Patient-Reported Outcomes (PROs) and Patient-Reported Outcome Measures (PROMs). Health Services Insights. 2013;6. doi:10.4137/HSI.S11093
3. Cella D, Hahn EA, Jensen SE, et al. Patient-Reported Outcomes in Performance Measurement. Research Triangle Park (NC): RTI Press; 2015 Sep. Types of Patient-Reported Outcomes. Available from: https://www.ncbi.nlm.nih.gov/books/NBK424381/

CONTACT INFORMATION

Angaja Phalguni angaja.phalguni@genesisrg.com
Audrey Brown audrey.brown@genesisrg.com
www.genesisrg.com

Presented ISPOR Europe 2023 - 12-15 November 2023 Copenhagen, Denmark.