The power of one: examining the impact of single arm trials on oncology drug approvals by NICE and CADTH

MSR28

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BACKGROUND

Health Technology Assessment (HTA) systematically evaluates the clinical (i.e., comparative benefits) and economic value (i.e., value for money) of health interventions to inform decisions regarding their reimbursements [1,2].

However, in case of rare diseases or advanced/refractory cancers, single-arm trials (SATs) are commonly used to evaluate the treatments and inform decisions because randomized controlled trials are either not feasible or unethical.

Aim: To evaluate the impact of SATs on oncology treatment recommendations by the National Institute for Health and Care Excellence (NICE) in the UK and the Canadian Agency for Drugs and Technologies in Health (CADTH) in Canada.

METHODS

We reviewed the publicly available HTA reports and recommendations issued by the NICE and CADTH for oncology drugs (January 1, 2021 to December 31, 2022).

Keywords: Cancer, carcinoma, melanoma, multiple myeloma, leukemia, and lymphoma.

Eligibility: Submissions/studies that utilized an SAT design and did not make head-to-head comparisons to alternative treatments (i.e., placebo, best supportive care) except when assessing different dosing regimens within the trial.

Data extraction parameters:

- Product indication
- Clinical evidence base
- HTA critique around SAT
- HTA final recommendation

Data were extracted by one reviewer, and the quality was checked by another reviewer to ensure accuracy. All data were analyzed qualitatively.

RESULTS

Figure 1. HTAs reviewed

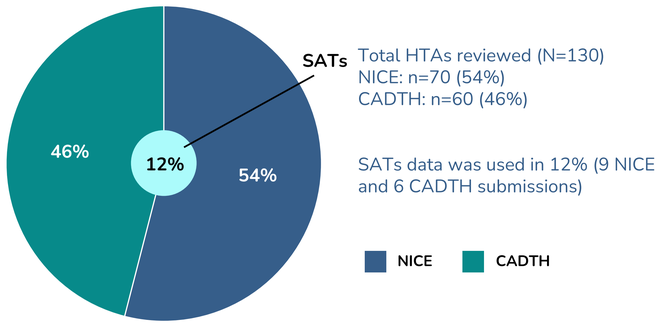
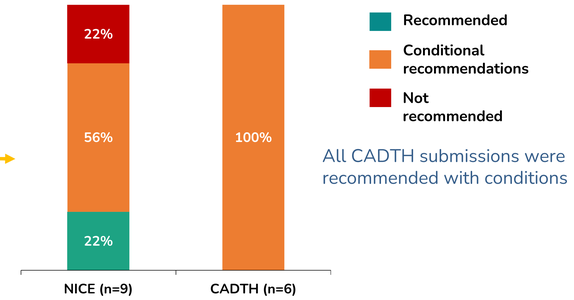
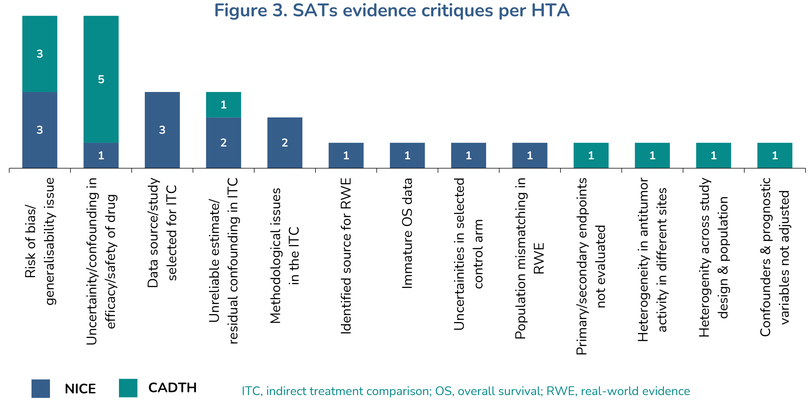


Figure 2. Decisions on technology appraisals using SATs per HTA



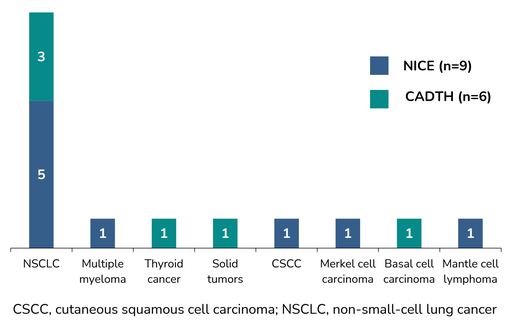
Among all SAT-based submissions, the most cited critiques by HTAs were concerns regarding population selection bias for external comparators, uncertainty regarding efficacy and safety, and methodological issues related to indirect treatment comparison (**Fig. 3**).

Figure 3. SATs evidence critiques per HTA



ITC, indirect treatment comparison; OS, overall survival; RWE, real-world evidence

Figure 4. Cancer types using SATs per HTA



CSCC, cutaneous squamous cell carcinoma; NSCLC, non-small-cell lung cancer

SATs data was predominantly used in non-small-cell lung cancer

Single arm trials **(SAT) are used in around 12% of oncology-related HTA submissions** to NICE and CADTH.

Common **critiques** of SAT-based submissions include **population selection bias**, **uncertainty about efficacy & safety**, **and methodological issues with indirect treatment comparison**.

While SAT-based submissions are becoming more accepted, **careful consideration must be given to defining the external comparator arm** to ensure robust and reliable HTA decision making.

References:

1. Griffiths EA, Macaulay R, Vadlamudi NK, et al. Value Health 2017; 20(10): 1245-1251.
2. Jaska A, Louder A, Maksymiuk C, et al. Value Health 2022; 25(12): 1967-1976.

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