An Increased Use of External Data to Inform Survival Extrapolations in NICE Technology Appraisals

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While there are several factors that impact final NICE recommendations, the rate of positive recommendations in oncology TAs using external data to inform survival extrapolations appeared similar to those that did not.

Background

Since the relaunch of the Cancer Drugs Fund in 2016, there has been an increase in the number of technology appraisals (TAs) in oncology, reaching an all-time high in 2021-2022 (50 TAs).[1] In 2020, the National Institute for Health and Care Excellence (NICE) released its technical support document (TSD) on the use of external data to inform the extrapolations of immature survival (TSD 21).[2] Until May 2021, 15.3% of TAs in oncology used external data.[3] It remains unclear whether the use of external data has increased since the release of TSD 21.

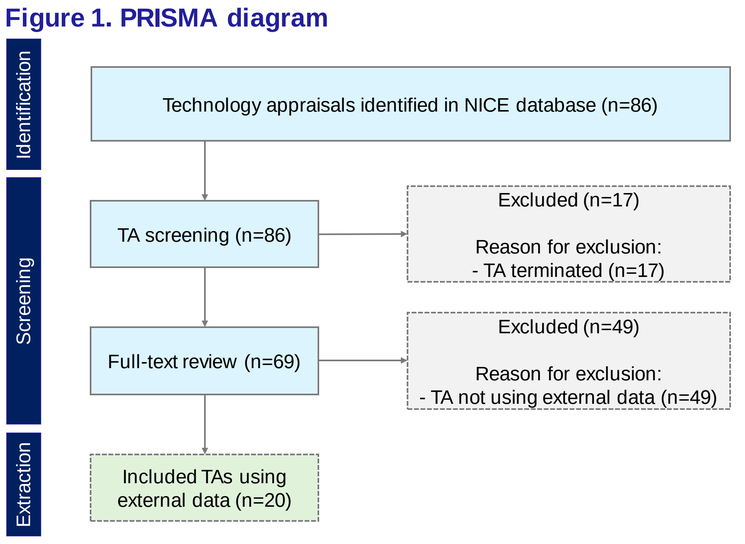
Objective

To investigate the frequency, methods, and acceptance of using external data to quantitatively inform survival extrapolation in oncology TAs published before and after May 2021. An existing dataset used in a previous analysis was updated to include TAs published after May 31st, 2021.[3]

Methods

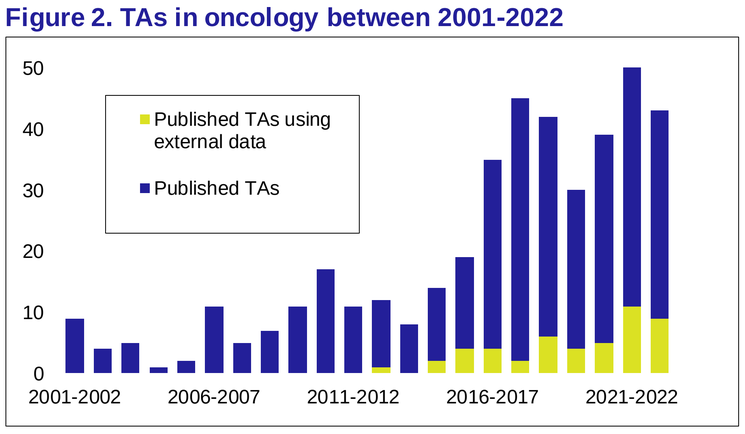
All oncology TAs from NICE published from June 1st, 2021, to May 2nd, 2023, were screened. Some TAs were excluded due to being terminated. Eligible TAs were considered for full-text review to explore when external data was used to quantitatively inform survival extrapolations. Full-text review included an assessment of the submission and evidence review group report, and final NICE recommendation (recommended, optimised, CDF or not recommended).

Figure 1. PRISMA diagram



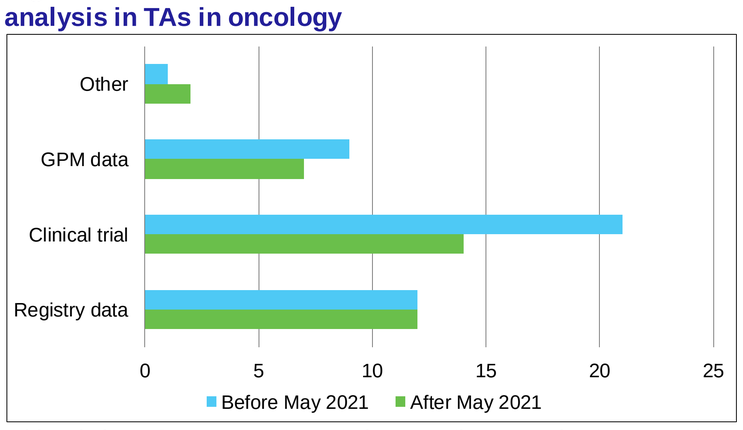
Abbreviations: NICE=National Institute for Health and Care Excellence, TA=technology appraisal

Figure 2. TAs in oncology between 2001-2022



Abbreviations: TA=technology appraisal

Figure 3. External data sources used to inform survival analysis in TAs in oncology



Abbreviations: GPM=general population mortality, TA=technology appraisal

Results

A total of 86 TAs in oncology were published since the previous analysis.[3] Due to termination, 17 TAs were excluded. Overall, 20/69 (29.0%) of TAs used external data, compared to 28/183 (15.3%) of TAs published until May 2021 (**Figure 1**). The use of external data to quantitatively inform survival increased in the past five years along with the increased number of TAs (**Figure 2**). The rate of positive recommendations among included TAs using external data (21/24 [87.5%]) was found to be similar to TAs not using external data (42/45 [93.3%]).

Registry data and historical clinical trial data were used most frequently (12 and 14 TAs; **Figure 3**), mostly to inform health-state transition probabilities or survival extrapolations for the external control-arm (**Table 1**). Critique from the ERG were mainly focused on:

- lack of transparency,
- data reliability and generalizability,
- data immaturity, and
- the introduction of bias.

Table 1. TAs using external data since TSD21

#	TA	Year	Technology	Indication	NICE categorization	Type of external data used
1	TA720	2021/22	Chlormethine gel	Lymphoma	Optimised	Registry data
2	TA724	2021/22	Nivolumab	Lung cancer	Not recommended	Clinical trial data, registry data
3	TA740	2021/22	Apalutamide	Prostate cancer	Recommended	Clinical trial data
4	TA741	2021/22	Apalutamide	Prostate cancer	Optimised	Clinical trial data
5	TA754	2021/22	Mogamulizumab	Mycosis fungoides	Optimised	Retrospective observational study
6	TA761	2021/22	Osimertinib	Lung cancer	Optimised (CDF)	Clinical trial data, registry data, general population mortality data
7	TA766	2021/22	Pembrolizumab	Melanoma	Recommended	General population mortality data, clinical trial data
8	TA772	2021/22	Pembrolizumab	Lymphoma	Recommended	Clinical trial data
9	TA795	2021/22	Ibrutinib	Lymphoma	Not recommended	Registry data
10	TA796	2021/22	Venetoclax	Leukaemia	Recommended	Clinical trial data, registry data
11	TA802	2021/22	Cemiplimab	Squamous cell carcinoma	Optimised	Registry data, retrospective observational data
12	TA810	2022/23	Abemaciclib	Breast cancer	Recommended	Retrospective observational data, previous TA submissions
13	TA813	2022/23	Asciminib	Leukaemia	Recommended	Clinical trial data
14	TA818	2022/23	Nivolumab	Pleural mesothelioma	Optimised	Clinical trial data
15	TA823	2022/23	Atezolizumab	Lung cancer	Recommended (CDF)	General population mortality data, clinical trial data, registry data
16	TA830	2022/23	Pembrolizumab	Renal cell carcinoma	Recommended	General population mortality data, registry data, clinical trial data
17	TA831	2022/23	Olaparib	Prostate cancer	Not recommended	Clinical trial data, general population mortality data
18	TA837	2022/23	Pembrolizumab	Melanoma	Recommended	Registry data, clinicial trial data
19	TA851	2022/23	Pembrolizumab	Breast cancer	Recommended	General population mortality data, clinical trial data, registry data
20	TA876	2022/23	Nivolumab	Lung cancer	Recommended	General population mortality data, KOL estimates

Abbreviations: CDF=Cancer Drug Fund, KOL=key opinion lead, NICE=National Institute for Health and Care Excellence, NR=not reported, TA=technology appraisal, TSD=technical support document

Conclusions

There has been an increase in the use of external data sources to quantitatively inform survival extrapolation since TSD 21. Although the final NICE recommendation remains a multifactorial decision, the rate of positive recommendations for TAs using external data appeared similar to those that did not. However, the direct impact of external data use on final NICE recommendations remains unclear.

References

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2. Rutherford, M. J., Lambert, P. C., Sweeting, M. J., Pennington, B., Crowther, M. J., Abrams, K. R., & Latimer, N. R. (2020). Flexible methods for survival analysis TSD. London: National Institute for Health and Care Excellence.
3. Reitsma, F., Pham, H. A., & Heeg, B. (2022). POSB269 The Use of External Data to Inform Survival Extrapolation in Oncology Technology Appraisals: A Comprehensive Review. Value in Health, 25(1), S179-S180.

Disclosures

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