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Real-world Evidence in Multiple Sclerosis: HTA Decision-Making Influencer or Not?

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INTRODUCTION

Multiple sclerosis (MS) is an autoimmune disease that affects the central nervous system (brain and spinal cord). There is no cure for MS at this time; however, disease modifying treatments exist that slow progression, control symptoms and help maintain a normal quality of life. Collecting data from clinical trials is costly and time intensive.[1-4] For health technology assessment (HTA) bodies that make reimbursement decisions, real-world evidence (RWE) is becoming more important in understanding lifelong diseases like MS, given that randomized controlled trials provide limited insights on clinical practice. Moreover, the high treatment costs of MS necessitate careful reimbursement decision-making. Our study aimed to understand how RWE is enabling HTA bodies to make reimbursement decisions.

OBJECTIVES

This review aims to assess the value of RWE in recent HTA appraisals for MS in four key reimbursement markets: England, Scotland, Canada and Australia.

METHODS

We evaluated published HTA appraisals in MS conducted between 2018 and 2022 across the four key HTA bodies: the National Institute for Health and Care Excellence (NICE)[5] in the UK, the Scottish Medicines Consortium (SMC)[6] in Scotland, the Canadian Agency for Drugs and Technologies in Health (CADTH)[7] in Canada and the Pharmaceutical Benefits Advisory Committee (PBAC)[8] in Australia. RWE and reimbursement data in the HTA appraisals published in the above listed HTA bodies were screened by two independent reviewers; discrepancies were checked by a third.

RESULTS

- Twenty-five HTA appraisals were relevant to the review criteria; RWE data were presented in 18 of these appraisals (72%). RWE data were taken from registries and observational studies. Of these 18 appraisals, 16 (88.8%) were accepted by HTA bodies, including seven appraisals from NICE, five from the SMC and four from CADTH **(Table 1)**
- The two HTA submissions that were not recommended (one each from NICE and CADTH) found the cost-effectiveness estimates of the drug molecule to be higher than the willingness to pay threshold

Table 1: RWE included in HTA submissions for MS

HTA body	Relevant hits in MS indication	RWE included in HTA submissions	Successful HTA submission
NICE	10	8	7
SMC	6	5	5
PBAC	2	0	0
CADTH	7	5	4
TOTAL	**25**	**18**	**16**

Key: CADTH, Canadian Agency for Drug and Technologies in Health; HTA, health technology assessment; MS, multiple sclerosis; NICE, National Institute for Health and Care Excellence; PBAC, Pharmaceutical Benefits Advisory Committee; RWE, real-world evidence; SMC, Scottish Medicines Consortium.

- Among the 16 accepted appraisals, data were reported for primary progressive, secondary progressive and relapsed/recurrent MS **(Table 2)**

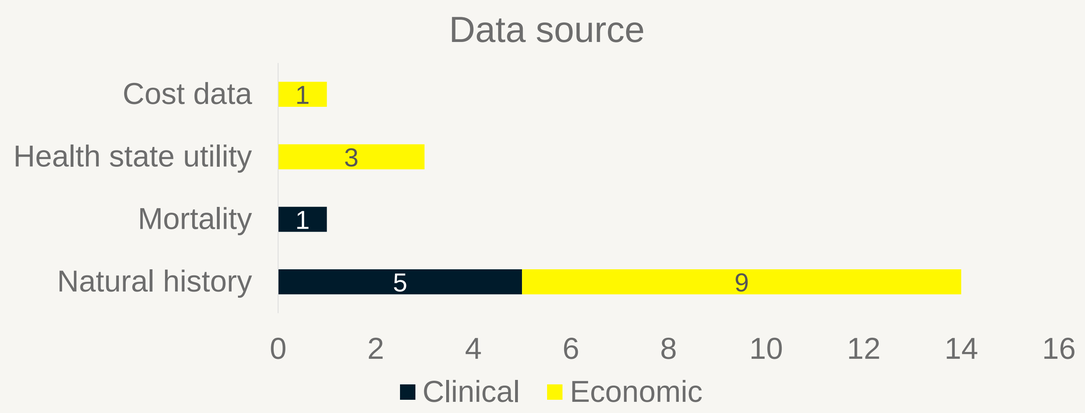
Table 2: Successful HTA submissions with RWE in MS by disease subtype

Type of MS	NICE	SMC	CADTH	PBAC	Total
Primary progressive MS	1	1	1	0	3
Secondary progressive MS	1	1	0	0	2
Relapsed/Recurrent MS	5	3	3	0	11

Key: CADTH, Canadian Agency for Drug and Technologies in Health; HTA, health technology assessment; MS, multiple sclerosis; NICE, National Institute for Health and Care Excellence; PBAC, Pharmaceutical Benefits Advisory Committee; SMC, Scottish Medicines Consortium.

- Among the 16 accepted appraisals, RWE was presented as economic evidence in 13 submissions, with nine submissions capturing the natural history of disease (including transition probabilities for health states). RWE was also presented as clinical evidence in six of 16 submissions, with five submissions reflecting on the natural history of MS **(Figure 1)**
- There were three HTA submissions where health state utilities were derived from RWE, among which two focused on disease severity and one on fatigue disutility score **(Figure 1)**
- In one of the submissions to the CADTH, the probability of mortality was derived from real-world data for the Canadian general population, by adjusting all-cause mortality. In another CADTH submission, costs for patient management were derived from RWE and adjusted to 2017 Canadian dollars **(Figure 1)**

Figure 1: RWE data included in HTA submissions



Key: HTA, health technology assessment; RWE, real-world evidence.

CONCLUSIONS

- RWE provides information on validity and insights from clinical practice beyond the conventional randomized controlled trials. It is increasingly playing a role in reimbursement decisions/market access, helping bridge various evidence gaps and strengthen the HTA review process
- For MS appraisals, HTA bodies have encouraged and accepted RWE as supportive data for both economic models and clinical evidence, thereby striving to better understanding the natural history and management of this chronic disease
- As managing MS is associated with high costs, using RWE data can help assess patients in clinical practice and can potentially accelerate their access to care

An electronic version of the poster can be viewed by scanning the QR code.

REFERENCES

1. https://medlineplus.gov/ency/article/000737.htm (accessed on 29 September 2022); **2.** https://www.mayoclinic.org/diseases-conditions/multiple-sclerosis/symptoms-causes/syc-20350269 (accessed on 29 September 2022); **3.** https://www.hopkinsmedicine.org/health/conditions-and-diseases/multiple-sclerosis-ms (accessed on 29 September 2022); **4.** https://www.massbio.org/news/member-news/using-real-world-data-for-patients-with-multiple-sclerosis/ (accessed on 29 September 2022); **5.** https://www.nice.org.uk/; **6.** https://www.scottishmedicines.org.uk/; **7.** https://www.cadth.ca/; **8.** https://www.pbs.gov.au/pbs/industry/listing/participants/pbac.

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