Systematic Literature Review on Economic Evaluations of Disease-Modifying Treatments in Multiple Sclerosis: Current Insights and Future Directions

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Introduction

- Multiple Sclerosis (MS) is a chronic, progressive neurological disease that affects the central nervous system, leading to a wide range of physical and cognitive impairments
- It encompasses various subtypes, including relapsing-remitting MS (RRMS), secondary progressive MS (SPMS), primary progressive MS (PPMS), and clinically isolated syndrome (CIS), each with distinct characteristics and progression patterns
- While there is no cure, treatment options primarily consist of disease-modifying treatments (DMTs), which aim to reduce the frequency of relapses, slow the progression of the disease, and improve patients' quality of life
- However, DMTs pose a challenge due to balancing high costs with clinical benefits, as well as selecting the best option from available treatments
- Economic evaluations, such as cost-effectiveness analyses, play a pivotal role in addressing these challenges

Objectives: We conducted a systematic literature review (SLR) to assess economic evaluations of DMTs for MS, focusing on temporal trends, presenting the latest models, and evaluating their alignment with clinical practice.

Methods

Methodology: An SLR was performed following the methodology recommended by the NICE guidelines and reported in accordance with the PRISMA statement.

Databases searched: PubMed, Embase, Cochrane CENTRAL, NHS Economic Evaluations Database, HTA Database, and EconLit, including all full-text English studies up to December 2022.

Study selection: Performed by two independent reviewers. Discrepancies were resolved through reviewer discussion.

Data extraction: Conducted by one reviewer and verified by a second independent reviewer.

Outcomes:
- Patient characteristics
- Model characteristics
- Treatment characteristics
- Model inputs (disease course, quality of life, costs)
- Model assumptions

Results

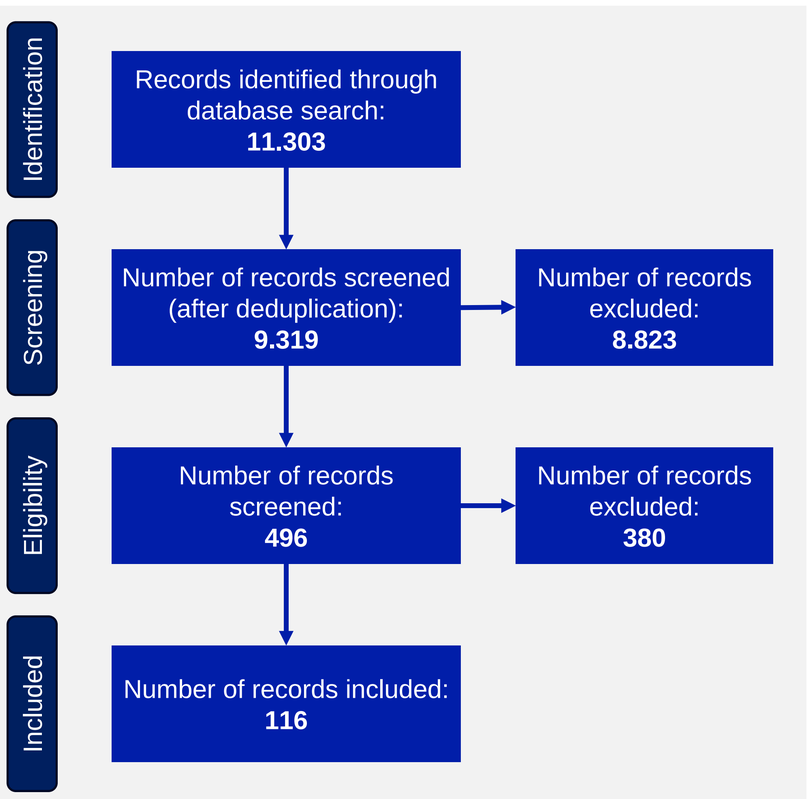

Figure 1. PRISMA flow diagram

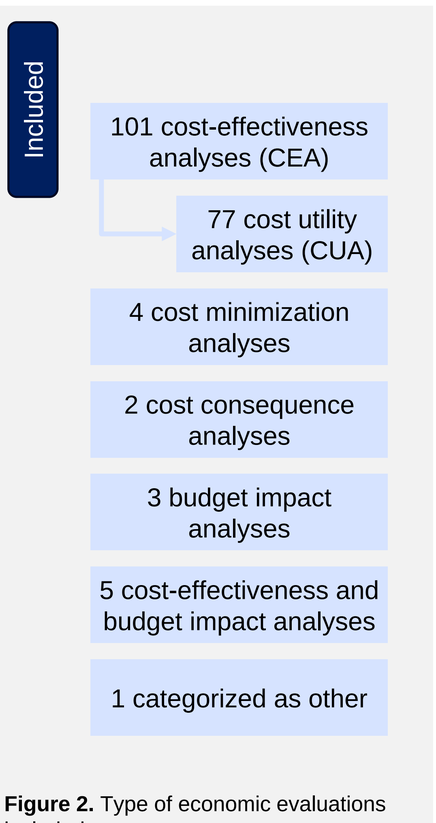

Figure 2. Type of economic evaluations included

Trends in economic modelling of MS populations:

- Most studies focus on modelling the RRMS population. Starting from 2003, economic models begin to include SPMS, either alone or in combination with RRMS. Highly active forms of MS began to be included around 2008, with a noticeable increase in economic studies focusing on these types through the 2010s and 2020s. CIS and clinically definite MS appear sporadically.

Model complexity and evolution:

- The majority (~70%) of CEAs report being based on the Expanded Disability Status Scale (EDSS)
- The trend indicates a transition from simpler models (mostly 7-state models) in the early 2000s to higher-granularity structures like 21-state models in recent years, capturing more detailed progression patterns within MS
- The discrete event simulation (DES) model provides an adaptable and innovative framework for modelling, enabling the simulation of personalized patient trajectories and treatment sequences

Discussion & Conclusions

Core insights:

- The reviewed economic evaluations share similarities in model structure and data sources, but also show differences, such as in the assumptions made on treatment adherence and switching

Evolving focus in economic models:

- Economic modelling has evolved from focusing predominantly on RRMS to including SPMS, highly active forms, and even PPMS, while also featuring more complex transition structures
- This increased model complexity reflects advancements in treatment, the need for detailed health state transitions, and better data collection on disease progression

Recent updates and innovation:

- Recent models include incorporating disutilities for caregivers and treatment-related symptoms, while emphasizing the need for additional clinically relevant outcomes, such as cognitive impairment
- In recent years, the use of DES models has emerged due to their ability to account for real-world complexities

Limitations:

- EDSS-based models primarily focus on physical disability, missing the full impact of the disease
- Data gaps also prevent models from accurately reflecting clinical practice, such as treatment switching

Future directions

Bridging the gaps between economic models and clinical practice is essential for enhancing model robustness, as it allows for a more accurate representation of **real-world complexities** and the actual **impact on patients**, which is crucial for optimizing **healthcare decision-making**

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