Evaluating the Rational Use of Disease-Modifying Therapies for Relapsing-Remitting Multiple Sclerosis Using Claims Data

RWD: 290

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Introduction

Rational use of medicines and efficient allocation of health resources are essential to achieving optimal health outcomes and sustaining healthcare systems. This requires that medications are prescribed according to clinical need, in appropriate doses and durations, and at the lowest cost to patients and the health system [1].

In Australia, patient access to a growing range of disease-modifying therapies (DMTs) for multiple sclerosis (MS) has been enabled through the Pharmaceutical Benefits Scheme (PBS). The rising prevalence of MS, together with the increasing number of available DMTs, has transformed the treatment landscape for relapsing–remitting MS (RRMS). These developments have also driven substantial growth in PBS expenditure on DMTs, raising important questions about the rationality of use and efficiency of health investment in this therapeutic area.

This study uses national claims data to address gaps in post-market monitoring of rational medicine use. Specifically, it examines drug utilization relative to defined daily doses (DDD), trends in prevalence and treatment patterns, therapy persistence, and the relationship between usage and healthcare costs from a payer's perspective.

Methods

This study drew on three key data sources. Expenditure on DMTs was obtained from the PBS Item Reports and compared with overall PBS expenditure from the PBS Expenditure and Prescriptions Report. Patient-level utilization data were extracted from the 10% PBS dispensing sample, with DMTs identified using PBS item codes for multiple sclerosis.

DMTs were grouped by efficacy into low-efficacy (LE) therapies (glatiramer acetate, interferon-beta, teriflunomide, and dimethyl fumarate) and high-efficacy (HE) therapies (natalizumab, fingolimod, ozanimod, siponimod, alemtuzumab, cladribine, ocrelizumab, and ofatumumab) [2,3]. This classification reflects differences in clinical effectiveness and associated costs, both of which influence overall cost-effectiveness.

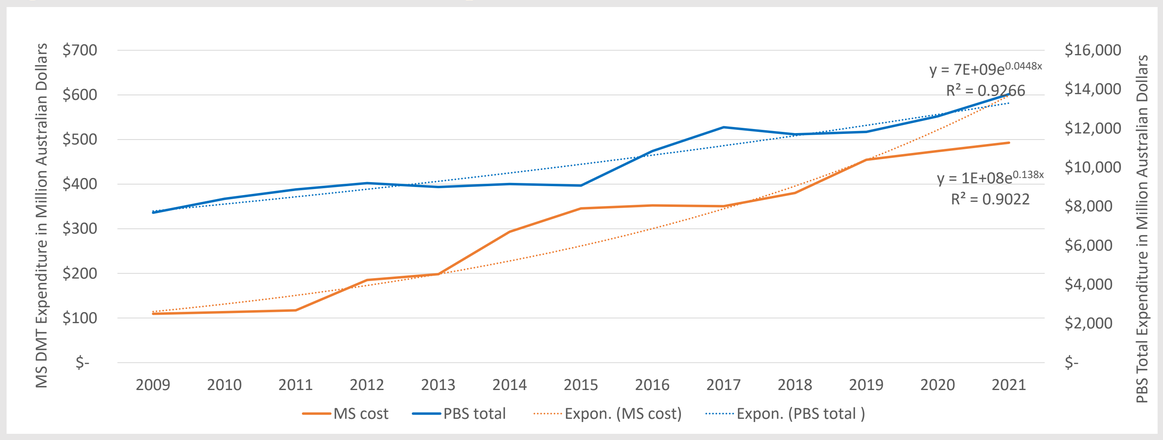
Descriptive analyses were performed in Microsoft Excel 2016, and treatment persistence was evaluated using Kaplan–Meier survival analysis in the Python *lifelines* package. Discontinuation was defined as either switching to another DMT or ceasing treatment, including due to death or other reasons. Patients who had not discontinued at the end of follow-up were right-censored.

Results

The usage and expenditure trends of DMTs

The expenditure of DMTs increased 4.5 folders from AUD $109.4M to AUD $492.9M between 2010 and 2021 (Fig. 1 orange line and left y-axis). During the same period, the compound annual growth rate for DMT expenditure was 13.6% (Fig. 1 dotted orange line), which was three times of the 4.43% annual growth of total PBS expenditures (Fig. 1 dotted blue line). This significant increase highlighted the growing financial impact of DMTs on the PBS.

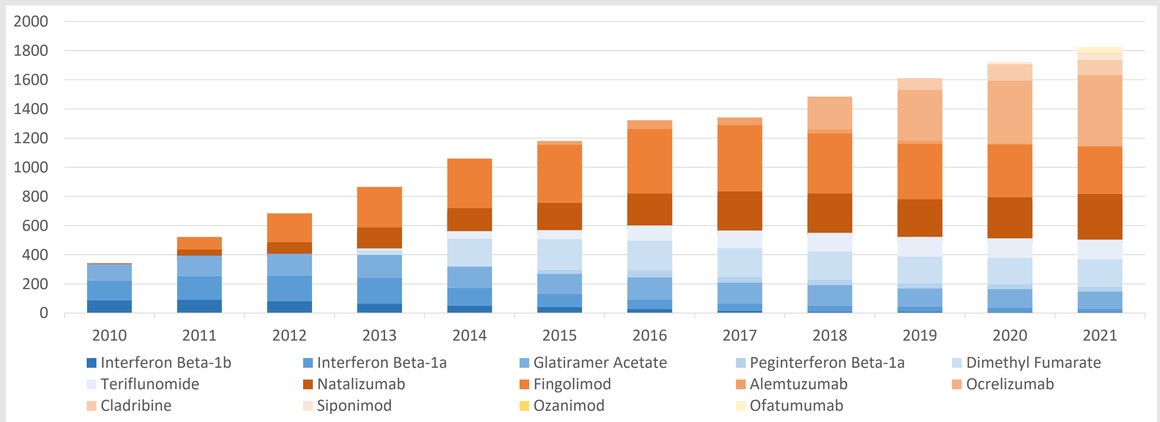
Fig. 1 PBS annual DMTs and Total Expenditures Between 2009 and 2021



The usage pattern of DMTs

During the study period, HE DMTs have progressively replaced LE treatments. Since 2016, patients on HE DMTs have outnumbered those on LE DMTs, and the number of patients on LE DMTs continued to decline thereafter (Fig. 2).

Fig. 3 Annual Trends in Patients Receiving DMTs



Dispensed dosages of DMTs

Generally, the DDD per patient per year exhibited consistent pattern for each DMTs over the years, with variations observed among HE and LE drugs (Fig. 3 and Fig. 4). For new treatments listed in the PBS, it typically took about one year after initial listing for a drug to reach a quiescent period of DDD per patient usage. Among all DMTs, Interferon Beta-1a was notably characterized by exceptionally high doses and varieties in usage over time.

Fig. 3 Annual Dispensed DDD per Patient for LE DMTs

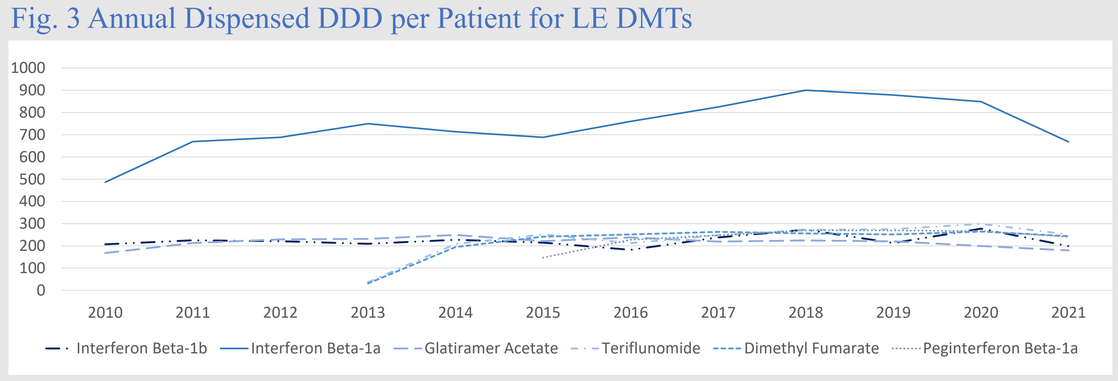
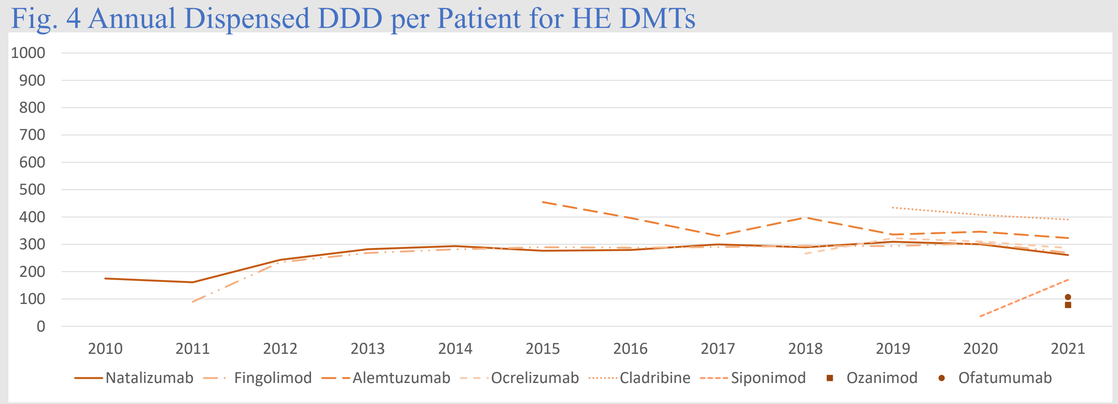


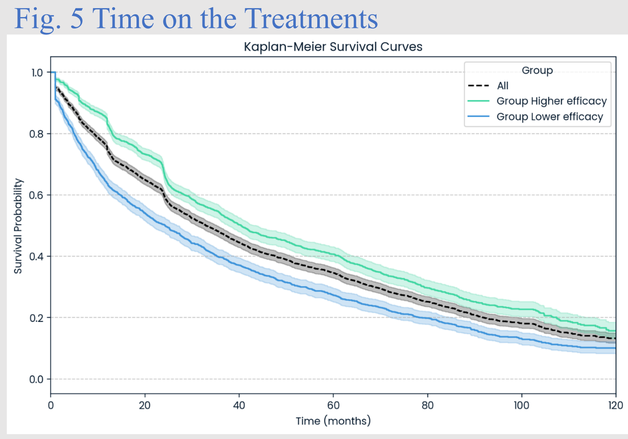
Fig. 4 Annual Dispensed DDD per Patient for HE DMTs



Patient persistence on the DMTs

The Kaplan-Meier analysis of the time on treatment (Fig. 5) suggested that patients on HE DMTs exhibited a greater persistence compared to those received LE treatments (36.66 vs 33.81 months).

Fig. 5 Time on the Treatments



Discussion

In this study, we examined key drivers of rising expenditure on DMTs: the number of patients receiving treatment, the usage pattern across different DMTs, including uptake and switch, dosages, and time on treatment. The number of MS patients on DMTs increased fivefold over the study period, driven by both growth in new treatment initiations and high persistence among existing patients, particularly those on high-efficacy therapies. By contrast, the average annual per-patient dosage, measured as DDD dispensed per year, remained stable for most DMTs. This indicates that increasing patient numbers and sustained persistence were the primary contributors to rising utilization and costs.

A notable exception was Interferon Beta-1a (non-pegylated), for which Australian patients exhibited unusually high use in terms of the dosages per patient. Concurrently, the PBS delisted two Interferon Beta-1a products: Rebif® in December 2022 and Avonex® in April 2023 [4].

Discussion (cont.)

This study harnessed the strength of the robust real-world administrative data with detailed drug dispense information at individual patient level to conduct a comprehensive view of DMT usage patterns in Australia. Our findings align with these delisting decisions of the PBS and demonstrated the value of real-world data in identifying patterns of irrational use.

The methodology used in this study offers high scalability and generalizability for supporting broader post-market drug monitoring, detecting of irrational use of drugs, enhancing budget impact analysis and control.

References

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