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A Fully Enrolled, Novel, Patient-Centred Real-World Evidence Study Designed to Better Understand Active and Nonactive Progressive Multiple Sclerosis Using Health Records in the United States

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BACKGROUND

- MS is a chronic autoimmune, inflammatory, demyelinating disorder of the central nervous system that causes neurological symptoms
- In the US, MS has only one diagnosis code according to the International Classification of Diseases-10 Clinical Modification despite different outcomes in PMS and RRMS and between active and nonactive disease, thus making real-world data difficult to analyse by sub-types and disease activity
- Previously we described the study design for a novel, patient-centred real-world evidence study that utilises health records in the US and reported preliminary data regarding demographic and healthcare resource utilisation for patients with PMS[1]

Medical records are needed to properly identify patients with PMS and to comprehensively understand their burden and unmet need

OBJECTIVE AND METHODS

Objective: To provide an update on the patient demographics and healthcare resource utilisation of patients with active/nonactive PMS in this novel, patient-centred, real-world evidence study using health records in the US

Study design

- As described previously,[1] this study is an analysis of available and de-identified information from medical records collected using the PicnicHealth digital record platform, a patient-centred, real-world health record aggregator, of patients with active and nonactive PMS receiving routine clinical care in the US
- Adult patients (aged ≥18 years) with confirmed MS enrolled in this study from July 2021 to September 2022; patients with documented PMS (PPMS or SPMS) were identified and included
  - Patients with PMS or PRMS were recategorised to the PPMS subtype if they had evidence for progression before evidence of a relapse, or to the SPMS subtype if they had evidence of a relapse before evidence for progression
    - Patients without information on the timing of progression or relapses were categorised as unknown
  - Patients were categorised as active if they had any relapses or Gd+ lesions reported in the previous 2 years; patients were categorised as nonactive if they did not meet these criteria
    - To determine if the number of previous years changed the percentage of patients with active/nonactive disease, a sensitivity analysis was conducted using only the prior year to determine activity status
    - Data were received up to the end of March 2023
- All available medical records from routine clinical care were obtained, and relevant clinical data were structured from narrative text into a de-identified dataset by the PicnicHealth platform
  - Clinical data, along with information about the provider and care site, were extracted from inpatient and outpatient records, as well as procedure and radiographic reports
- Descriptive statistics were reported

RESULTS – PATIENT CHARACTERISTICS

- A total of 500 patients with PMS (187 PPMS; 309 SPMS; 1 PRMS; 3 unknown) were included in the study; characteristics for these patients are shown in **Table 1**
  - Most patients had nonactive PMS (75% of patients with PPMS; 66% of patients with SPMS; 70% overall)
  - The median (IQR) age in the overall patient population was 54 (46–61) years; the majority of patients were female (65–67% of patients with PPMS; 75–78% of patients with SPMS; 72% overall)
  - The most common race was White/Caucasian (74–83% of patients with PPMS; 75–81% of patients with SPMS; 80% overall), followed by Black/African American (9–10% of patients with PPMS; 12–17% of patients with SPMS; 12% overall); for ethnicity, 4–9% of patients with PPMS, 4–7% of patients with SPMS and 6% overall identified as Hispanic/Latino
- Sensitivity analysis showed higher percentages of patients with nonactive PMS (83% of patients with PPMS, 81% of patients with SPMS, 82% overall) when using only the prior year rather than the previous 2 years to categorise disease activity

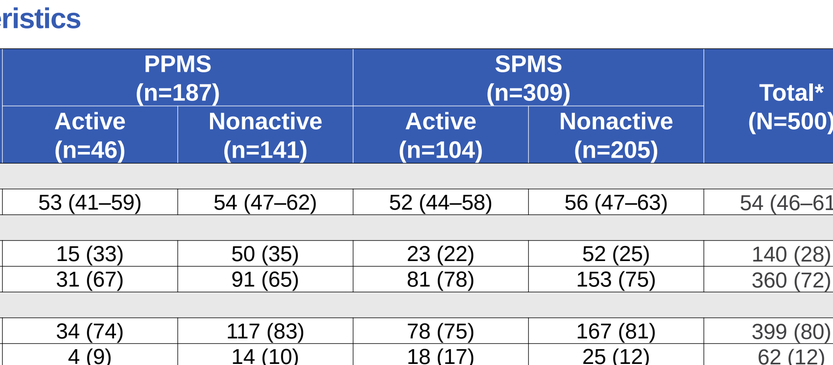
Table 1. Patient Characteristics

Characteristic	PPMS (n=187)		SPMS (n=309)		Total* (N=500)
	Active (n=46)	Nonactive (n=141)	Active (n=104)	Nonactive (n=205)	
Age, years					
Median (IQR)	53 (41–59)	54 (47–62)	52 (44–58)	56 (47–63)	54 (46–61)
Sex, n (%)					
Male	15 (33)	50 (35)	23 (22)	52 (25)	140 (28)
Female	31 (67)	91 (65)	81 (78)	153 (75)	360 (72)
Race, n (%)					
White/Caucasian	34 (74)	117 (83)	78 (75)	167 (81)	399 (80)
Black/African American	4 (9)	14 (10)	18 (17)	25 (12)	62 (12)
Other	4 (9)	6 (4)	2 (2)	5 (2)	17 (3)
Unknown	3 (7)	4 (3)	3 (3)	7 (3)	17 (3)
Prefer not to say	1 (2)	0 (0)	3 (3)	1 (0)	5 (1)
Ethnicity, n (%)					
Hispanic/Latino	2 (4)	12 (9)	7 (7)	8 (4)	29 (6)
Not Hispanic/Latino	42 (91)	122 (87)	92 (88)	188 (92)	448 (90)
Unknown	0 (0)	0 (0)	0 (0)	2 (1)	2 (0)
Prefer not to say	2 (4)	7 (5)	5 (5)	7 (3)	21 (4)

*Of the 500 patients in the overall patient population, three had an unknown diagnosis and one was diagnosed with PRMS

- Patients were geographically represented across the US regions (**Figure 1**)

Figure 1. Patient Numbers (%) Across the US*



*The sum of the percentages for the overall patient population and individual subgroups across regions may not add up to 100% due to rounding. †Of the 115 patients in the west, the overall population includes one patient with an unknown diagnosis. ‡Of the 166 patients in the south, one had a diagnosis of PRMS and two had an unknown diagnosis

HEALTHCARE RESOURCE UTILISATION

- Healthcare resource utilisation for patients with PMS is shown in **Table 2**
  - In the overall population, patients had a median (IQR) of 10 (7–14) years of visits available from 6 (3–8) care sites across 14 (7–28) total providers and 3 (2–5) neurology providers
    - For patients with active or nonactive PPMS, there was a median of 8.3 (5.9–11.2) and 8.2 (5.3–12.1) years of visits available from 6 (3–8) and 5 (3–7) care sites, across 14 (5–19) and 12 (6–24) total providers and 3 (2–4) and 3 (2–4) neurology providers, respectively
    - For patients with active or nonactive SPMS, there was a median of 10.2 (6.7–13.3) and 10.9 (8.1–15.5) years of visits available from 6 (4–9) and 6 (3–9) care sites, across 19 (10–44) and 13 (6–29) total providers and 4 (2–6) and 4 (2–5) neurology providers, respectively
  - In the overall population, median (IQR) number of hospitalisations was 1 (0–4) with a median length of stay of 1 (0–7) day; 60% (301/500) of the overall population had at least one hospitalisation, for whom the median (IQR) length of stay was 5 (2–15) days
    - For patients with active PPMS or nonactive PPMS who required hospitalisation, the median length of stay was 6 (3–16) and 4 (2–11) days, respectively
    - For patients with active SPMS or nonactive SPMS who required hospitalisation, the median length of stay was 7 (3–19) and 5 (2–13) days, respectively

Table 2. Healthcare Resource Utilisation of Patients with PMS

Characteristic	PPMS (n=187)		SPMS (n=309)		Total* (N=500)
	Active (n=46)	Nonactive (n=141)	Active (n=104)	Nonactive (n=205)	
Median years of visits (IQR)	8 (6–11)	8 (5–12)	10 (7–13)	11 (8–16)	10 (7–14)
Median number of care sites (IQR)	6 (3–8)	5 (3–7)	6 (4–9)	6 (3–9)	6 (3–8)
Median number of providers (IQR)	14 (5–19)	12 (6–24)	19 (10–44)	13 (6–29)	14 (7–28)
Median number of neurology providers (IQR)	3 (2–4)	3 (2–4)	4 (2–6)	4 (2–5)	3 (2–5)
Median number of hospitalisations (IQR)	1 (0–4)	1 (0–3)	2 (1–6)	1 (0–3)	1 (0–4)
Median hospital stay, days (IQR)	2 (0–10)	1 (0–4)	4 (1–13)	1 (0–6)	1 (0–7)
Number of patients with ≥1 hospitalisations (%)	28 (61)	76 (54)	82 (79)	112 (55)	301 (60)
Median hospital stay, days (IQR)	6 (3–16)	4 (2–11)	7 (3–19)	5 (2–13)	5 (2–15)

*Of the 500 patients in the overall patient population, three had an unknown diagnosis and one was diagnosed with PRMS

LIMITATIONS

- These data are limited to the US and may not be representative of other countries, especially ones with different labelling and treatment patterns
- These are descriptive data without any adjusting for key factors, such as disease severity, and may not be comparable with other data sources
- Patients self-enrolled in this study, which may create a selection bias and may not be fully representative of the PMS population
- Patients who are misdiagnosed as RRMS or who have a delayed PMS diagnosis are not included in this study
- Patients with a general diagnosis of PMS or PRMS and who have missing data may be misclassified
- Patients who do not report their relapses to providers or do not undergo routine MRIs may have missing data and overrepresent the nonactive disease population
- Linked claims data were not used to confirm full utilisation or determine cost for these patients

CONCLUSIONS

Results from this study examining real-world evidence showed that patients with PMS had demographic features consistent with the overall MS population in the US[2]

- **Demographic data showed that the median age was 52–56 years; there were more females, especially in the SPMS population; and most patients were White/Caucasian (74–81%) followed by Black/African American (9–17%)**
- **Consistent with the initial data,[1] the majority of patients had nonactive PMS**
  - **The sensitivity analysis shows that shortening the time without disease activity to 1 year increases the percentage of patients with nonactive PMS**
- **Overall, patients in this study had a median of 10 years of data from health care visits available and were geographically represented across the US**
  - **A median of 3–4 neurology providers per patient contributed data, representing the robustness of this data set**
- **These patients continue to have a high unmet need as they required substantial healthcare resource utilisation with the majority having at least one hospitalisation and the median length of these hospital stays was 4–7 days**

This novel, patient-centred study has robust real-world data with more complete medical records that can be used to better understand unmet need and generate meaningful future insights

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1. Watson C *et al.* Presented at ECTRIMS, October 11–13, 2022, Milan, Italy. 2. Wallin MT, *et al. Neurology* 2019;92:e1029–e1040.

ABBREVIATIONS

Gd+ = Gadolinium enhancing; IQR = interquartile range; MS = multiple sclerosis; PMS = progressive multiple sclerosis; PPMS = primary progressive multiple sclerosis; PRMS = progressive relapsing multiple sclerosis; RRMS = relapsing-remitting multiple sclerosis; SPMS = secondary progressive multiple sclerosis

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