

## The Profile of a Contemporary Cohort of Participants with Primary Progressive Multiple Sclerosis in the NARCOMS Registry

**A Salter, N Thomas, G Cutter, RA Marrie, T Tyry**

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## Disclosures

**Amber Salter** has nothing to disclose.

**Nina Thomas** is an employee of Genentech, Inc.

**Ruth Ann Marrie** (last two years): Pharma-funded research: Sanofi-Aventis; Other sources of research funding: CIHR, PHAC, National Multiple Sclerosis Society, MS Society of Canada, MS Scientific Foundation, CMSC Foundation, Rx & D Health Research Foundation.

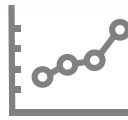
**Gary R. Cutter** serves on data safety monitoring committees for Antisense Therapeutics Limited, Sanofi-Aventis, Bayhill Pharmaceuticals, Bayer Pharmaceuticals, BioMS Pharmaceuticals, Daichi-Sankyo, Glaxo Smith Klein Pharmaceuticals, Genmab Biopharmaceuticals, Medivation, Peptimmune, PTC Therapeutics, Teva, Vivus, NHLBI, NINDS, National MS Society; and receives consulting or speaking fees from Alexion, Accentia, Barofold, CibaVision, Biogen-Idec, Novartis, Consortium of MS Centers, Klein-Buendel Incorporated, Enzo Pharmaceuticals, Somnus Pharmaceuticals, Teva, Biogen Idec, Advanced Health Media, EMD Serono, EDJ Associates, Aegis Creative Marketing, Eli Lilly, UT Southwestern University, Klein Buendel, University of Illinois Health Policy Center, Somnus Therapeutics.

**Tuula Tyry** has nothing to disclose.

## PPMS: Epidemiology and unmet needs



- More than 2.3 million people worldwide affected by MS<sup>1</sup>
  - ≈10% have PPMS
- No cure for MS<sup>2</sup>



- PPMS is characterized by a clinically detectable progressive course from disease onset<sup>3-5</sup>



- Currently there are no FDA-approved disease-modifying therapies to treat PPMS

- As we approach a new treatment era for PPMS, there is benefit in better understanding the needs of this patient cohort

FDA, Food and Drug Administration; MS, multiple sclerosis; PPMS, primary progressive MS.  
1. Atlas of MS 2013. <http://www.msif.org/wp-content/uploads/2014/09/Atlas-of-MS.pdf>. Accessed 27 May, 2016; 2. Markowitz CE, et al. *Am J Manag Care* 2010;16:S211–S218; 3. Andersson PB et al. *Arch Neurol* 1999;56:1138–1142; 4. Cottrell DA, et al. *Brain* 1999;122:625–639; 5. Confavreux C, et al. *N Engl J Med* 2000;343:1430–1438.

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## Learning objectives

- To use self-reported health information provided by participants in the North American Research Committee on Multiple Sclerosis (NARCOMS) Registry to assess the needs of the primary progressive multiple sclerosis (PPMS) patient population
- To better understand the demographic and clinical characteristics of patients with PPMS to help address the unmet need for effective treatments in this condition

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## Study objectives and survey distribution

### Study objectives

- To describe the demographic and clinical characteristics of NARCOMS participants who self-identified as having PPMS

### Survey distribution

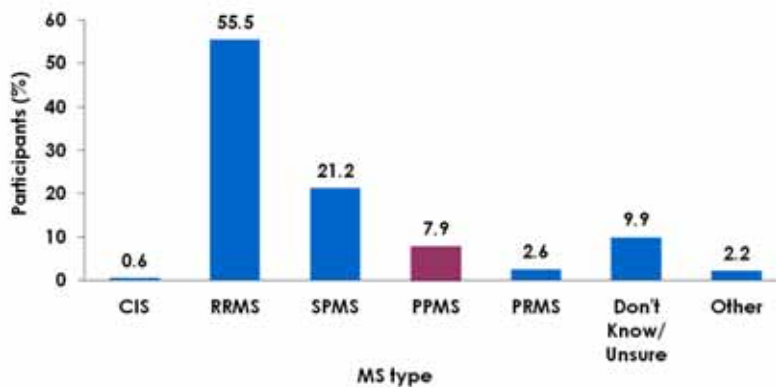
- The NARCOMS Registry collects a wide range of self-reported health-related information from participants via semiannual surveys
- The Spring 2015 Update survey had a total of **8,004** respondents

PPMS, primary progressive multiple sclerosis.

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## Study inclusion criteria

- Of the 8,004 survey respondents, **632 participants (7.9%)** who self-reported a PPMS diagnosis were selected for the study



CIS, clinically isolated syndrome; MS, multiple sclerosis; PPMS, primary progressive MS; PRMS, progressive-relapsing MS; RRMS, relapsing-remitting MS; SPMS, secondary progressive MS.

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## Demographics of the PPMS cohort

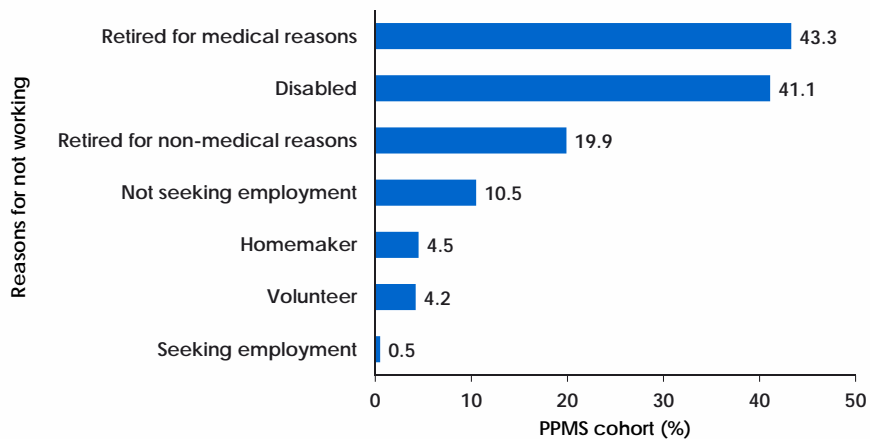
Characteristics	
Female, n (%)	396 (62.7)
Race, n (%)	
White	576 (93.8)
Black	17 (2.8)
Other	21 (3.4)
Age at survey completion, mean (SD), years	64.3 (8.9)
Residence, n (%)	
Private home/apartment/condo	522 (83.3)
Private home with home health aide	75 (12.0)
Assisted living	12 (1.9)
Nursing home	18 (2.9)
Has health insurance, n (%)	544 (86.1)

Numbers vary slightly across characteristics due to missing values.  
PPMS, primary progressive multiple sclerosis.

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## Employment status in the PPMS cohort

- A total of 552 participants (89%) from the PPMS cohort were not working for various reasons



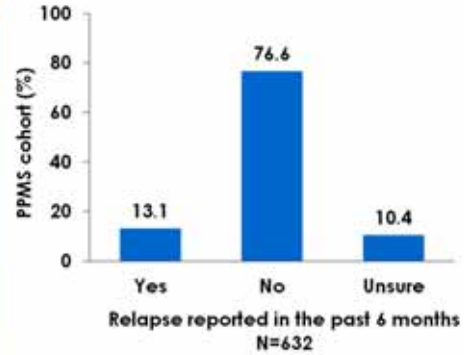
Participants could select all applicable answers

PPMS, primary progressive multiple sclerosis.

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## Clinical characteristics of the PPMS cohort

Characteristics of the PPMS cohort N=632	Mean (SD), years
Age at symptom onset	36.2 (11.0)
Age at diagnosis	44.5 (10.0)
Disease duration	19.7 (9.8)

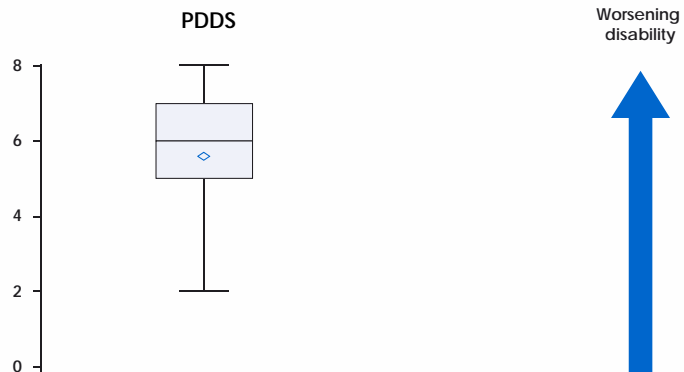


- A total of **201 participants (33%)** from the PPMS cohort reported off-label use of disease-modifying therapies (DMTs)

DMT, disease-modifying therapy; PPMS, primary progressive multiple sclerosis.

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## PPMS cohort: PDDS

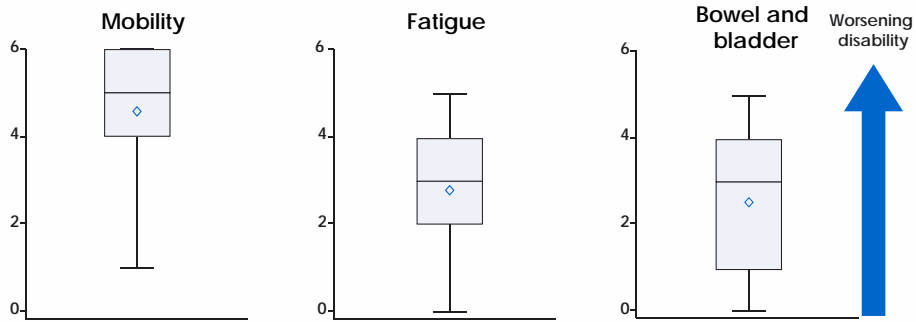


	Median	(25%, 75%)
PDDS	Bilateral Support	(Late Cane, Wheelchair/Scooter)

PDDS, Patient-Determined Disease Steps; PPMS, primary progressive multiple sclerosis.

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## PPMS cohort: Performance Scales

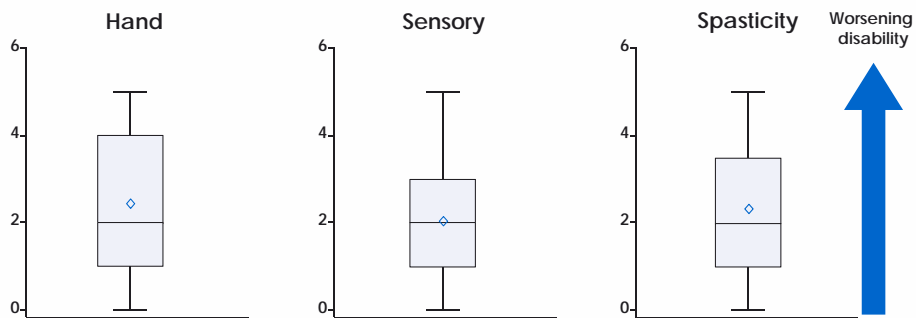


	Median	(25%, 75%)
Mobility PS <sup>1</sup>	Severe Gait Disability Bilateral Support	(Frequent Use of Cane, Total Gait Disability or Bedridden)
Fatigue PS <sup>1</sup>	Moderate	(Mild, Severe)
Bowel and bladder PS <sup>1</sup>	Moderate	(Minimal, Severe)

1. The original set of 8 Performance Scales have been copyrighted by Delta-Quest. Copyright Registration Number / Date: TXu000743629 / 1996-04-04; assigned to DeltaQuest Foundation, Inc., effective October 1, 2005. U.S. Copyright law governs terms of use.  
PPMS, primary progressive multiple sclerosis; PS, performance scale.

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## PPMS cohort: Performance Scales

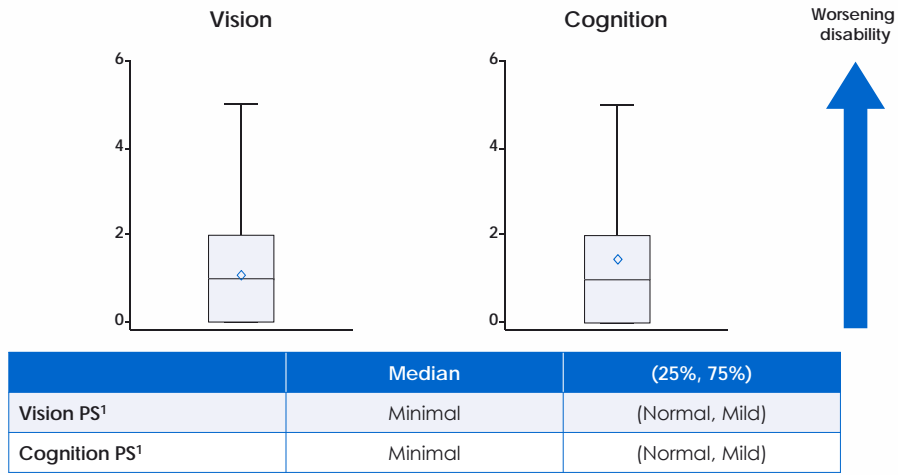


	Median	(25%, 75%)
Hand PS <sup>1</sup>	Mild	(Minimal, Severe)
Sensory PS <sup>1</sup>	Mild	(Minimal, Mild)
Spasticity PS <sup>1</sup>	Mild	(Minimal, Mild)

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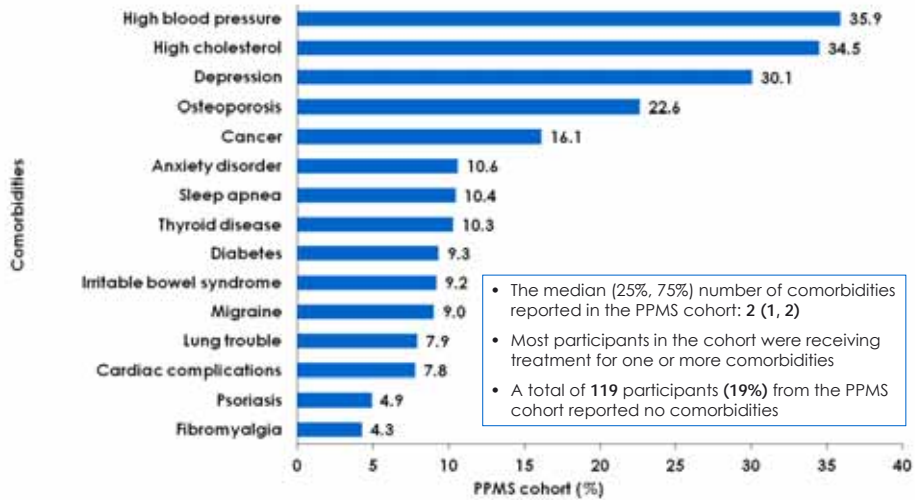
## PPMS cohort: Performance Scales



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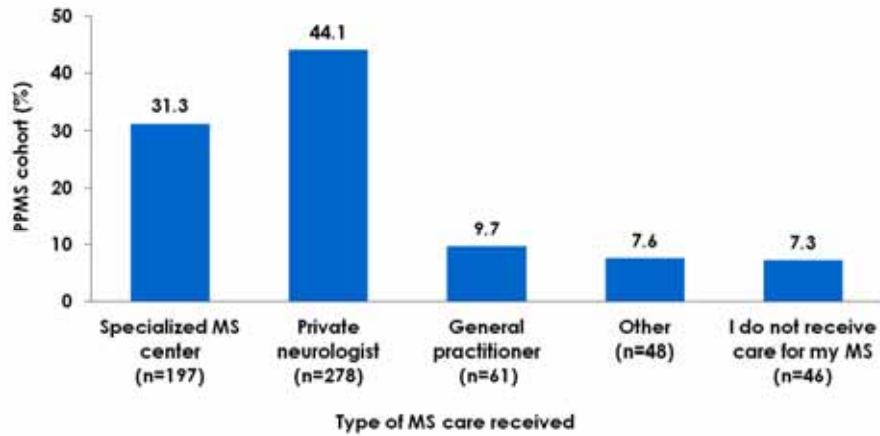
## Comorbidities in the PPMS cohort



Participants could select all applicable answers

## Type of MS care received by the PPMS cohort

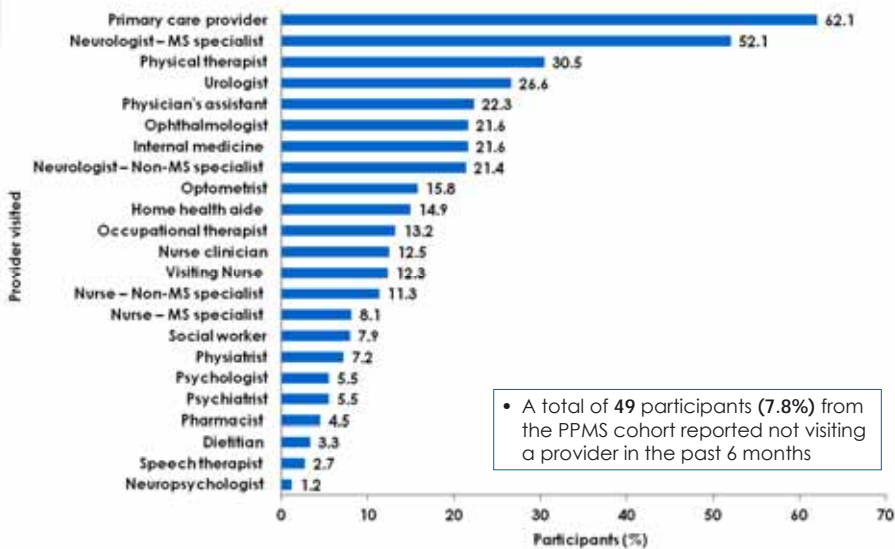
- Private neurologists were visited by a large proportion of the PPMS cohort (278 [44%])



MS, multiple sclerosis; PPMS, primary progressive MS.

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## Healthcare providers visited by the PPMS cohort in the past 6 months



- A total of 49 participants (7.8%) from the PPMS cohort reported not visiting a provider in the past 6 months

PPMS, primary progressive multiple sclerosis.

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## ER visits and hospitalization in the PPMS cohort

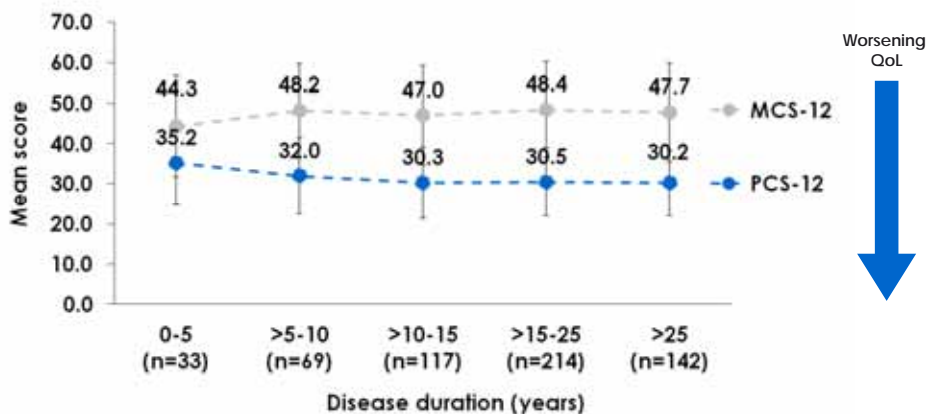
- A total of 20% (n=123) of participants in the PPMS cohort had been to the ER in the past 6 months
  - Of this subset, 29% had more than one encounter with the ER
- A total of 14% (n=86) of participants in the PPMS cohort had been admitted to the hospital in the past 6 months for the following reasons:
  - Non-MS related problem (43%)
  - UTI (26%)
  - Other MS-related problem (26%)
  - Rehabilitation (17%)
  - Exacerbation or worsening of MS (9%)

ER, emergency room; MS, multiple sclerosis; PPMS, primary progressive MS; UTI, urinary tract infection.

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## Quality of life by disease duration in the PPMS cohort

- The RAND-12 Health Survey, which yields the physical component summary (PCS-12) and mental component summary (MCS-12) scores, was used to assess quality of life in the PPMS cohort



The PCS-12 and MCS-12 of participants in the PPMS cohort stratified by disease duration is presented.

MCS, mental component summary; PCS, physical component summary; PPMS, primary progressive multiple sclerosis; QoL, quality of life.

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## Conclusions

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- The NARCOMS Registry includes detailed and current disease-related information on several hundred people with PPMS
- The prevalence of PPMS in the self-described MS cohort from the semi-annual Spring 2015 Update survey was almost 8%
- Participants with PPMS reported:
  - A high-level of disability
  - Varying degrees of physical and functional impairment
  - Visits to a range of healthcare providers
  - Multiple comorbidities
  - Off-label use of DMTs
- Further analysis of the available data, along with continued follow-up, will add to our understanding of this disease type and help address the unmet need in this less researched and less understood form of MS

DMT, disease-modifying therapy; MS, multiple sclerosis; PPMS, primary progressive MS.

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## Acknowledgments

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