Identifying Gaps in Patient Experience Data for Progressive Supranuclear Palsy

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BACKGROUND

Progressive Supranuclear Palsy (PSP) is a rare neurological disorder that impacts a patient's body movements, walking, balance, and eye coordination

PSP is estimated to affect as many as 5-17 in 100,000 people. Typical onset occurs between 45 and 74 years of age

The only treatment for PSP is symptomatic and supportive as there is no cure to date

Patient experience data (PED) have the potential to capture what is most bothersome to patients and caregivers who live with this complex and debilitating disease to improve quality of life

OBJECTIVE

To describe patient experience data in progressive supranuclear palsy and identify gaps in patient experience, unmet need, and opportunities for future research through a systematic literature review (SLR)

METHODS

An SLR was conducted in accordance with Preferred Reporting Items for Systematic Reviews and Meta-analyses (PRISMA)[1]

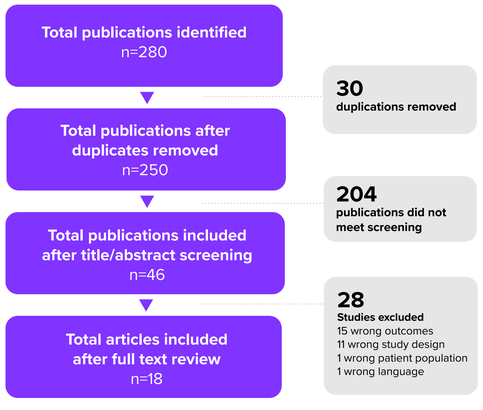
Studies available in English, with a sample size >1, and reporting on PED in patients with PSP were identified using PubMed and Embase (Table 1)

Two independent reviewers were used for title and abstract screening as well as during full-text review with third reviewer consensus. Data was extracted by a single reviewer (Figure 1)

Table 1: Screening Criteria

Study Characteristics	Screening Criteria
Patient Population	Patients with PSP
Outcome	Inclusion of PED as defined by the Federal Drug Administration which included any information "that captures patients' experiences, perspectives, needs, and priorities related to but not limited to"[2]: • Symptoms of their condition and its natural history • Impact of the conditions on their functioning and quality of life • Experience with treatments • Input on which outcomes are important to them • Patient preferences for outcomes and treatments • Relative importance of any issue as defined by patients
Study Type	• Database analyses • Cohort studies • Observational studies (retrospective and prospective) • Registry analyses • Descriptive studies • Non-randomized or non-controlled studies • RCTs • Surveys • **Excluded economic evaluations including models**
Time frame	January 1, 2018 to September 27, 2023
Geography	Global

Figure 1: PRISMA diagram

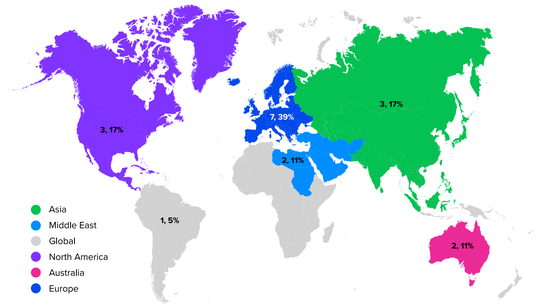


RESULTS

Study Characteristics

A majority of studies were conducted in Europe, followed by Asia (Figure 2)

Figure 2: Study Location



Clinical Outcome Assessments (COAs)

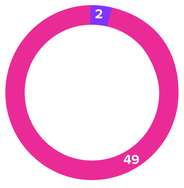
The PSP Rating Scale (PSPRS)

- Used in 5 studies
- Is a composite assessment that scores patients on daily activities, behavior, bulbar, ocular motor, limb motor and gait/ midline

Health-related Quality of Life Questionnaire for Patients with PSP (PSP-QoL)

- Used in 1 study
- Assesses the difficulty of mental and physical tasks within the previous four weeks and provides an overall score for life satisfaction

Figure 3: COAs Utilized



Across the **51** COAs used, only **2** COAs were specific to PSP (Figure 3)

20 **Unique patient-reported outcomes (PROs) were used across 13 studies**

The most common PROs included the PSP rating scale, the Parkinson's Disease Questionnaire-39 and the Unified Parkinson's Disease Rating Scale

7 **Unique observer-reported outcomes (ObsROs) were used across 10 studies**

The most common ObsROs used included the PSP Rating scale and the Neuropsychiatric Inventory

22 **Unique clinician-reported outcomes (ClinROs) were used across 13 studies**

The most common ClinROs used was the PSP Rating Scale, Mini Mental State Examination, and the Frontal Assessment Battery

9 **Unique performance outcomes (PerfOs) were used across 5 studies**

PerfOs used included Digit Span, Babcock Story Recall Test and the Verbal Fluency Test

2 studies reported information around the impact on caregivers using PROs

Zarit Burden Interview (ZBI)

To assess the level of subjective feelings of burden experienced by caregivers of older persons with dementia and other types of disability

Caregiver Strain Index (CSI)

To screen for caregiver strain after hospital discharge of an elderly family member

4 studies used surveys or interviews as primary data collection of PED

- One survey asked patients how well their treatment helps with their most significant or burdensome symptoms of PSP
- One survey asked patients were asked about their beliefs and preferences about exercise, difficulties coping with disease progression and how it impacts activities and the perceived barriers of PSP
- One survey asked patients about their ability to swallow and any challenges or modifications that the patient requires due to PSP
- One interview asked patients about their knowledge around PSP and if they valued involvement in advanced care-planning

Patient Characteristics

Mean age reported across 12 studies

67.4 – 80.0 years

Disease Duration and Onset

14 studies reported on disease duration

10 studies reported mean disease duration ranging from 1.2 years to 7.6 years

2 studies reported median disease duration of 4.0 to 4.7 years

4 studies reported range of disease duration of 1.0 to 12.8 years

2 studies reported age at onset of disease

1 study reported on both symptom onset (mean: 66) and age at diagnosis (mean: 67)

1 study reported median age of onset of 66 years (range: 49-80)

CONCLUSION & NEXT STEPS

COAs, specifically PROs, were the most prominent outcomes measured. Additional forms of PED, especially PSP-specific measures, would be useful to demonstrate impact of disease, patient journey, and caregiver burden

Gaps exist when it comes to understanding the patient journey of patients with PED. There are limited PED describing the timing and experience of PSP diagnosis

There is a need for modification or adaption of COA measures that currently exist to better characterize the PSP patient experience

REFERENCES

1. Page MJ, McKenzie JE, Bossuyt PM, et al. The PRISMA 2020 statement: an updated guideline for reporting systematic reviews. Bmj. Mar 292021;372:n71. doi:10.1136/bmj.n71
2. U.S. FDA. (2017). Plan for Issuance of Patient-Focused Drug Development Guidance Under 21st Century Cures Act Title III Section 3002. U.S. FDA. www.fda.gov

ABBREVIATIONS

COA: Clinical outcome assessment; ClinRO: Clinician-reported outcome; ObsRO: Observer-reported outcome; PED: Patient experience data; PerfOs: Performance outcome PRO: patient-reported outcome; PRISMA: Preferred Reporting Items for Systematic Reviews and Meta-analyses; PSP: Progressive Supranuclear Palsy; SLR: systematic literature review

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