# Person-Centered Care and Trial Design for Progressive Supranuclear Palsy: A Collaborative Project of AFTD and CurePSP

Shana G. Dodge[1], Jessica Shurer[2], Farwa Ali[3], David Irwin[4], Sarah Kremen[5], Chi-Ying Lin[6], Julio Rojas-Martinez[7], Lawren VandeVrede[8], Catherine Wang[9], Anne-Marie Wills[8], Kristophe Diaz[2], Penny A. Dacks[1, 9]
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## Objective

To investigate the lived experiences of people with progressive supranuclear palsy (PSP) to inform strategies for early and accurate diagnosis, effective interventions, and person-centered clinical trial designs.

## Background

- Frontotemporal degeneration (FTD) is an umbrella term for a group of genetically and clinically related rare neurodegenerative conditions that result in impairments across multiple domains and shortened life expectancy.
- PSP is one such FTD disorder, primarily impacting movement, cognition, behaviors, language, and vision.
- Clinicians and researchers must identify potential participants for available clinical trials early in the disease and ensure experimental drugs or interventions address clinically meaningful domains.
- Clinical trial recruitment and retainment, however, are challenging given the rarity of the disease, barriers to accessing timely and accurate diagnoses, varied clinical presentations, and obstacles to finding people whose symptom burden does not preclude them from feasibly participating.

## Methods

In preparation for an Externally Led Patient-Focused Drug Development meeting, the Association for Frontotemporal Degeneration (AFTD) and the FTD Disorders Registry collaborated to develop the FTD Insights Survey, the largest community survey of people with FTD disorders. The anonymized online survey, deployed October 2020 through March 2021, queried aspects of the lived experiences of people with frontotemporal degeneration. AFTD and CurePSP worked together to extract data specific to survey respondents who reported living with a PSP diagnosis. Respondents included 27 persons with PSP (PwPSP) and 76 PSP care partners (CPs) (not matched dyads) in the US, Canada, and the UK.

| Demographic | PwPSP (n=27) | CPs (n=76) |
|---|---|---|
| **Age in years (M [SD])** | 70.5 [8.3] | 64.6 [8.6] |
| **Sex** | | |
| **Female** | 21 (81%) | 61 (81%) |
| **Male** | 6 (19%) | 14 (19%) |
| **Race or ethnicity** | | |
| **Caucasian** | 22 (81%) | 71 (93%) |
| **Hispanic/Latino** | 4 (15%) | 0 (0%) |
| **Black/African American** | 0 (0%) | 2 (3%) |
| **East Asian** | 0 (0%) | 1 (1%) |
| **Native Hawaiian/Pacific Islander** | 0 (0%) | 1 (1%) |
| **Prefer not to answer/Other** | 1 (4%) | 1 (1%) |
| **Education** | | |
| **Doctoral Degree** | 4 (15%) | 3 (4%) |
| **Master's Degree** | 4 (15%) | 15 (20%) |
| **College/Bachelor's Degree** | 10 (37%) | 33 (44%) |
| **Associate's Degree** | 3 (11%) | 13 (17%) |
| **High School Diploma** | 5 (19%) | 11 (15%) |
| **Elementary or Middle School** | 1 (4%) | 0 (0%) |
| **Country of residence** | | |
| **USA** | 23 (85%) | 70 (93%) |
| **Canada** | 4 (15%) | 4 (5%) |
| **UK** | 0 (0%) | 1 (1%) |

## Results

### DIAGNOSTIC ODYSSEY

60% of respondents noted that the person diagnosed had been initially given a different diagnosis. Of those given a previous diagnosis, the most endorsed were:

| | |
|---|---|
| Parkinson's disease | 74% |
| Mild Cognitive Impairment | 21% |
| Depression | 19% |
| Dementia with Lewy bodies | 8% |
| Alzheimer's disease | 8% |

Respondents experienced a lag between age when symptoms first emerged (ages 50-69) and age at PSP diagnosis (between ages 60-79).

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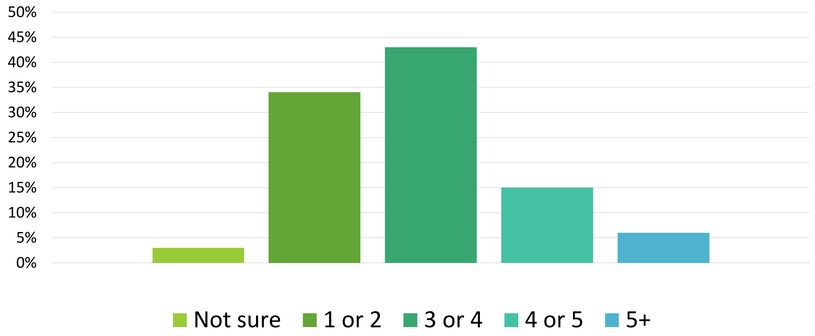
## Results (cont.)

### DIAGNOSTIC ODYSSEY (CONT.)

More than half reported seeing more than 3 doctors to obtain an accurate PSP diagnosis.

**Number of doctors consulted before a PSP diagnosis made:**




### SYMPTOM IMPACT

82% of current CPs reported that the person diagnosed is aware or mostly aware of his/her symptoms.

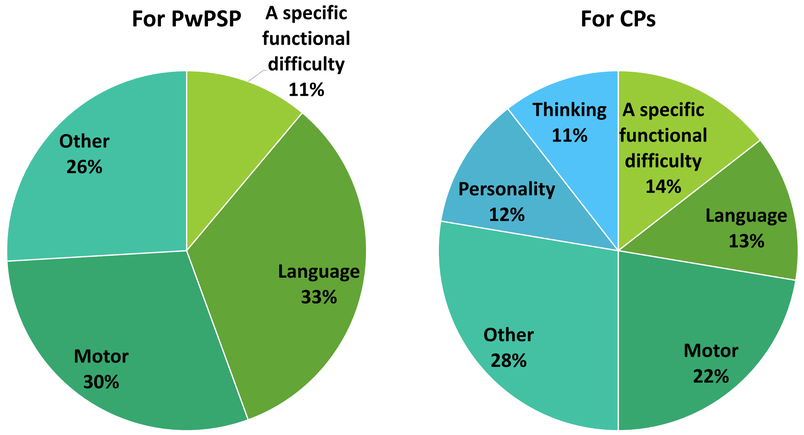
CPs and PwPSP cited language (e.g., speaking, finding words, understanding) and motor dysfunction (e.g., tremor, balance, performing movements) as being the most distressing symptoms.

CPs also reported distress associated with changes in their loved one's personality (e.g., acting differently or inappropriately in a social situation) and cognition (e.g., solving problems, making judgments, organizing).

### SYMPTOM IMPACT (CONT.)

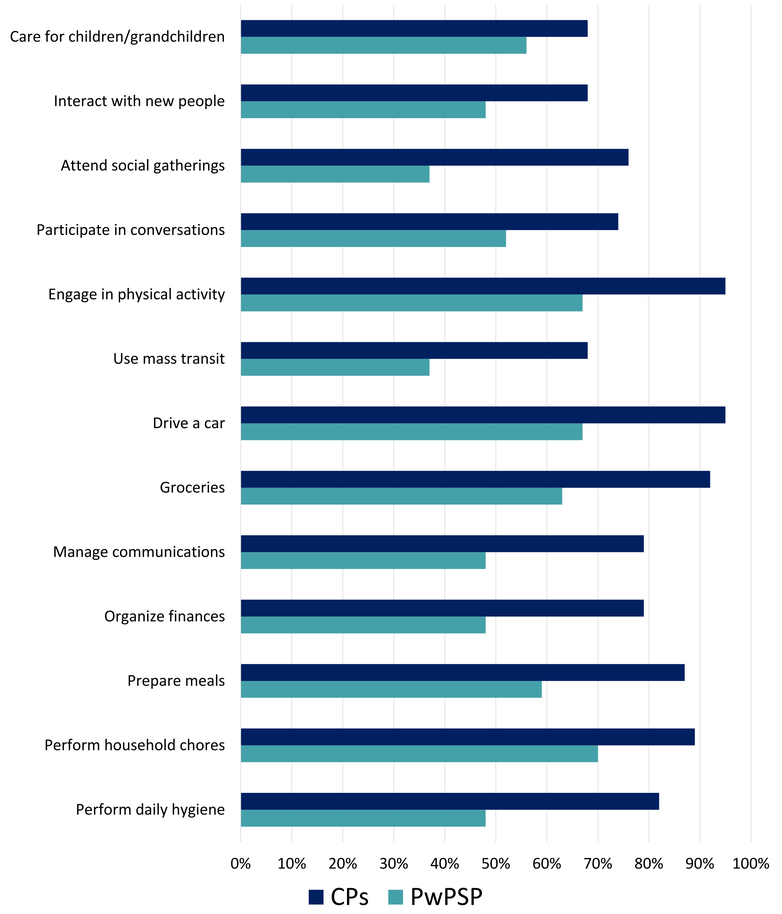
PwPSP and CPs also noted being distressed by other symptom domains, with fewer than 2% endorsing any one category, including behavior (repetitive or compulsive behavior, rigid routines); mood (anxious, not interested, depressed, irritable, emotional outbursts); relationships (getting along with others); sleep (not sleeping through the night, bad dreams, sleeping too much); spatial (judging distances, perceiving objects); delusions and hallucinations; eating/drinking; and memory.

**Most distressing symptoms**




Respondents noted a significant symptom impact on interpersonal relationships, activities of daily living within the home, and independence in the community

**Do symptoms make it difficult to do any of the following independently? (not matched dyads)**



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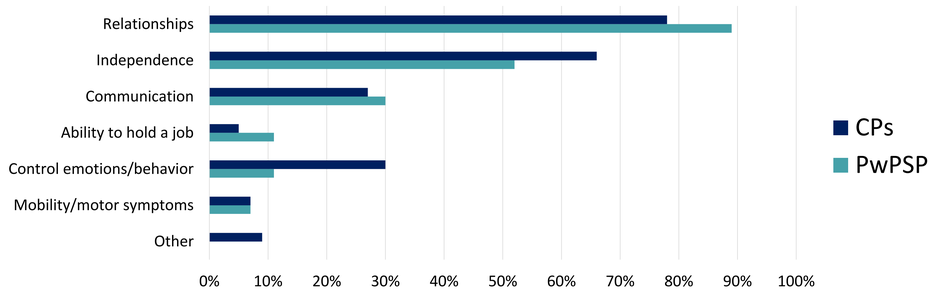
## Results (cont.)

### RESEARCH READINESS AND PRIORITIES

The majority of PwPSP (81%) and CPs (69%) expressed willingness to participate in a clinical trial for PSP. Both PwPSP and CPs reported a willingness for themselves or the person they care for to undergo a number of common clinical trial procedures, with PwPSP reporting higher rates of willingness than CPs. PwPSP and CPs reported relatively higher willingness rates for less invasive procedures such as blood sample collection (PwPSP: 92%, CPs, 72%), MRI (PwPSP: 92%, CPs: 60%), and genetic testing (PwPSP: 92%, CPs: 66%), than for lumber punctures (PwPSP: 63%, CPs: 28%).

Respondents indicated that they would most want a treatment that would improve independence, followed by one that would improve communication.

**I'd most want a treatment to improve (select all that apply)**




Of the very small group who reported to be unwilling (n=4) or very unwilling (n=0) to participate, they reported being more likely to participate if (n=1 each)

- I would get individual results for various tests completed during the study
- I would be reimbursed for all expenses
- A health professional could come to my home for some aspects of the study
- I could complete interviews and assessments from home using a computer or smartphone

## Conclusions

The FTD Insights Survey paints a picture of complex symptoms experienced with PSP, including impairment in multiple areas of cognitive functioning, regardless of initial diagnosis or first symptoms. The impact of symptoms on daily social and physical functioning is clear for both PwPSP and CPs alike.

Of note:

- Differences in reports between PwPSP and CPs could be a function of sampling bias (e.g., PwPSP who participated may be higher functioning that those who had a CP reporting on their behalf), true differences in their own experiences of and perspectives on PSP/FTD, or due to anosognosia.
- Group was overwhelmingly female, Caucasian, and highly-educated; more work is required to understand experiences with the diagnostic journey, disease impact, and research readiness in more diverse groups.
- Survey distributed by AFTD and the FTD Disorders Registry, which are FTD-focused organizations; respondents may have been more likely to present with cognitive and language dysfunction than if the survey had been disseminated through movement disorder channels.

The survey results reveal a complicated and lengthy diagnostic journey for individuals and families impacted by PSP. It is critical that healthcare providers caring for PwPSP and related disorders understand the implications of delayed diagnosis on the ability for people to participate in and benefit from research participation. Despite obstacles, respondents report high interest in participating in clinical research. PwPSP and CPs can be connected to trial participation opportunities through enrollment in the FTD Disorders Registry (ftdregistry.org), and through connection to CurePSP and AFTD.

Priority distressing symptoms and their impact on daily functioning and quality of life must be taken into consideration when tailoring plans of care for PwPSP. In addition, neurology providers should consider referrals for neuropsychological testing, rehabilitation therapies, clinical social work and other supports that can provide compensatory strategies and disease-specific education to help PwPSP and CPs adapt to changes to cognition, communication, and independence.

*Thank you to the survey participants for sharing their lived experience to help drive the science of FTD forward. Thanks also to Dr. Jonathan Rohrer and the Genetic Frontotemporal Dementia Initiative (GENFI), Dr. Adam Boxer, and Dr. David Knopman.*

**These and other data are available to researchers for analysis through the FTD Disorders Registry.**