PARKINSON'S DISEASE EXPERIENCE FROM A PATIENT'S PERSPECTIVE: SYSTEMATIC REVIEW ON PATIENT REPORTED OUTCOMES

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INTRODUCTION

- **Parkinson's disease**, a neurologic disorder characterized by shaking, stiffness, and difficulty with walking, balance, and coordination[1] affects millions of people in the United States (U.S.) and worldwide.
- In 2012, a relatively new initiative about **Patient Focused Drug Development (PFDD)** was introduced by the Center for Drug Evaluation and Research – Food and Drug Administration (CDER FDA) to incorporate patient experiences and perspectives into the drug development process. [2]
- Patient Reported Outcomes (PROs) provides important data in understanding the experience of the patient with the disease. However, due to lack of clarity about whether well-developed PROs exist or which PRO instrument fits better for Parkinson's disease, understanding the nature and extent of patient experiences is hampered.
- This systematic literature review was performed to recognize and recapitulate PRO measures that have been validated and/or used in published studies of Parkinson's disease, which would better inform the selection of **patient reported outcome measures (PROMs)** that could potentially be used in forthcoming clinical trials. Additionally, this review identifies the common experiences of the patient with the disease.

OBJECTIVES

- Identify which patient reported outcome measures are being used in Parkinson disease patients.
- Identifying the most common measurement domains of the patient reported outcome measures used in the review

METHODS

- A systematic search of computerized databases **PubMed** and **PsycINFO** was conducted and the review was limited to Humans, English language publications between 2016 and 2020 with full text access.
- The search keywords include 'patient reported outcomes', '**PRO**', 'self-report', 'survey', 'index', 'Parkinson disease' and 'United States'.
- The studies were included based on the specific criteria, as follows:
  - (1) Adults with Parkinson's disease ≥18 years of age;
  - (2) Articles including "patient reported" outcome measures and experiences.
  - (3) Studies having sample size >50.
  - (4) Only US based studies.
  - (5) Studies published from 2016 to 2020.
  - (6) Studies published in English language
  - (7) Study designs such as randomized controlled trial, observational study, evaluation study, cross-sectional study, prospective/retrospective cohort or secondary analysis study
  - (8) Studies which were not primarily PRO focused but included the patient reported outcome measures were considered and included in the review.

RESULTS

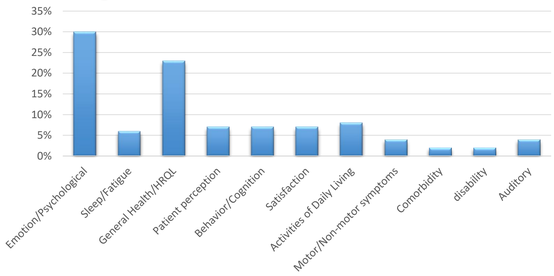
- In total, 650 articles were identified, and **36** articles met the criteria.
- **53** patient reported outcome measures were identified from the included articles.
- The common PRO measures were recognized and categorized into three groups: Condition-related, Health quality & Parkinson's disease-specific.
- The example of PRO table is as follows:

First Author, Year	Patient reported outcomes/measures	Measurement/ Assessment	Measurement Domain
1.Brown et al,2019[48] 2.Hinkle et al,2018[49] 3.Jones et al,2016[50] 4. Shamaskin-Garroway et al,2016 [9] 5.Salazar et al,2017[51] 6. Folmer et al,2017[52] 7.Kay et al,2018 [11] 8. Pontone et al,2017[53]	Beck Depression Inventory 2nd ed. (BDI-II)/ self-rated Beck Depression Inventory	Sleep changes and depression severity depression, suicidality	Emotion/ Psychological

- Overall, in this review of 36 studies, 16 of the 53 patient reported outcome measures utilized belonged to the **emotion/psychological** measurement domain as shown in Figure 1. Twelve measures fit in the health-related quality of life domain.
- Among the emotion/psychological measurement domain, **six** different patient reported outcome measures (**Hospital Anxiety and Depression Scale (HADS), Beck Depression Inventory, Geriatric Depression Scale (GDS), The Center for Epidemiological Studies Depression Scale (CES-D), Patient Healthcare Questionnaire (PHQ-2 and PHQ-9**) were used to assess depression in 16 different studies.

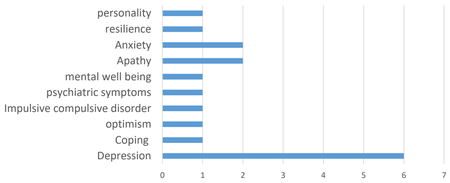
RESULTS (continued)

Figure 1. PROM Measurement Domains



- As summarized in Figure 4, in addition to depression, the emotion/psychological measurement domain included Anxiety, Apathy, Coping, personality, resilience, optimism, mental well-being, psychiatric symptoms and Impulsive compulsive disorder. Two patient reported outcome measures were used to assess Anxiety and Apathy each and rest all other patient experiences in the emotional domain were assessed by using one measure.

Figure 2. The number of patient-reported outcome measures in the emotion/psychological domain.



LIMITATIONS

- Only patient reported outcome measures from the published literature were included in this review. Hence there could be a possibility of missing other relevant measures.
- Studies outside United States were excluded which might have overlooked important patient reported outcome studies.

CONCLUSION

- This review provides a recent overview of the Parkinson's disease patient reported outcome measures used in studies in the United States published from 2016-2020.
- 36 studies were identified for this review based on specific criteria; a wide range of PROMs are used due to the enormous burden of Parkinson's disease.
- It was observed that the largest number of PROMs utilized belonged to the emotion/psychological domain indicating the need to understand the **mental and emotional health** of PD patients.
- The cardinal physical signs in Parkinson's disease such as rigidity, bradykinesia, and resting tremor are easily acknowledged and treated by healthcare providers on a regular basis. However, attention is required on the non-motor features as well.[3] Hence studies focusing on the mental and emotional health aspect of Parkinson's disease and strategies for addressing and ameliorating this debilitating aspect of the disease are warranted.
- Parkinson's Disease is complex with limited therapeutic options; hence **patient's perspectives** are important to consider in making decisions.
- Continuing to understand the experience of patients, to address their needs and speeding up research to develop effective treatments is essential. These type of studies in PD and other chronic diseases could be expected to increase in the coming years.

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FUNDING DISCLOSURE

This project was not funded

Presented at the International Society for Pharmacoeconomics and Outcomes Research 25th Annual Meeting, May 18, 2020, Poster No. PND87.

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