P49 Leveraging PROMIS and HealthMeasures to develop non-motor patient-reported outcome measures for early Parkinson's disease trials

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Objective: To develop fit-for-purpose patient-reported outcome (PRO) measures for early Parkinson's disease (PD) that assess non-motor symptoms using HealthMeasures item banks, primarily leveraging PROMIS, in alignment with FDA guidance for clinical trial endpoint assessment.

Methods: Northwestern University investigators employed a multi-phase approach using secondary analysis of symptom maps from the Wearable Assessments in The Clinic and at Home in PD (WATCH-PD) qualitative sub-study (N=40). Development steps included: (1) identifying candidate concepts of interest through literature reviews and stakeholder feedback; (2) refining concepts through WATCH-PD symptom map analysis; (3) mapping HealthMeasures items, including PROMIS; (4) drafting new items where necessary; and (5) removing items based on stakeholder input. Stakeholders (i.e., individuals with PD, clinicians, measurement experts, FDA representatives) provided input throughout. Next, to gather direct feedback on draft measures, a panel-style focus group method was implemented with a subset of individuals from the original WATCH-PD qualitative sub-study. Two independent participant groups (5–6 per group) attended four consecutive weekly focus groups (8 total sessions, N=11 participants) in February–March 2025. Discussions focused on item clarity, relevance, response options, and missing content.

Results: Twelve draft PRO measures (v1.1) were developed: cognitive function (9 items), depression (3 items), anxiety (5 items), fatigue (4 items), pain (4 items), daytime sleepiness (2 items), sleep disturbances (4 items), speech and voice changes (6 items), REM sleep behavior disorder (4 items), constipation (2 items), impaired swallowing (3 items), and urinary dysfunction (5 items). Focus group data are now being analyzed to refine these measures by incorporating participant perspectives from the original qualitative work and stakeholder input.

Conclusions: This research addresses a critical gap in early PD assessment by leveraging calibrated item banks to efficiently develop patient-centered PRO measures. Using existing HealthMeasures content accelerated development without compromising psychometric integrity. The refined measures will undergo cognitive interviews with persons diagnosed with early PD and care partners in the next phase. This collaborative process—integrating insights from regulators, clinicians, and people with PD—supports the creation of fit-for-purpose clinical trial endpoints sensitive to early disease changes in PD and suitable for evaluating disease-modifying treatments in future registration trials.