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Preferences for Parkinson's Disease Treatments: a Scoping Literature Review

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INTRODUCTION

- As Parkinson's Disease (PD) progresses, oral medications lose effectiveness, often requiring advanced treatments like deep brain stimulation, intestinal or subcutaneous infusions, and on-demand rescue therapies to control symptoms. Given the differences in efficacy, safety, and administration among these options, understanding patient treatment preferences is essential.
- To our knowledge, no study has provided a comprehensive synthesis of existing evidence for the treatment preferences of people with PD (PwP), which is essential for guiding clinical decision-making and ensuring that PD therapies align with patients' needs, goals, and values.

OBJECTIVE

- To identify and synthesize the available quantitative evidence on patient preferences for PD treatments to better understand decision-making between PwP and their physicians.

METHODS

- A scoping literature review was conducted to identify quantitative preference studies assessing PD treatments and surveying PwP, care partners, and/or physicians.

Search Strategy

- Searched in PubMed and EMBASE on November 11, 2024.
- Used the index terms "Parkinson's", "preferences", "discrete choice experiment", "best-worst scaling", "contingent valuation", and "threshold technique".

Inclusion/Exclusion Criteria

- **Study population:** PwP, their care partners, and healthcare professionals.
- **Type of studies:** Discrete choice experiment (DCE), best-worst scaling (BWS), contingent valuation (CA), and threshold techniques (TT) to elicit preferences.
- **Data sources:** Peer-reviewed articles, conference abstracts, and posters published in English since 1999, regardless of the study's country of origin. Reviews and opinion pieces were excluded from the analysis.

Study Selection and Data Extraction

- Titles and abstracts were imported into the online software system Rayyan (www.rayyan.ai/) for title/abstract screening, where duplicate publications were removed. After that, a full-text screening was conducted.
- Extracted data included:
  - **Study characteristics** (e.g., authorship, publication year, country, study design, sample size, interventions, population type, and analysed subgroups).
  - **Preference elicitation methods** (e.g., number of hypothetical treatment alternatives, attributes and levels, key preference results, trade-offs [maximum acceptable risks (MAR) and minimum acceptable benefits (MAB)], willingness to pay (WTP), and subgroup findings).

RESULTS

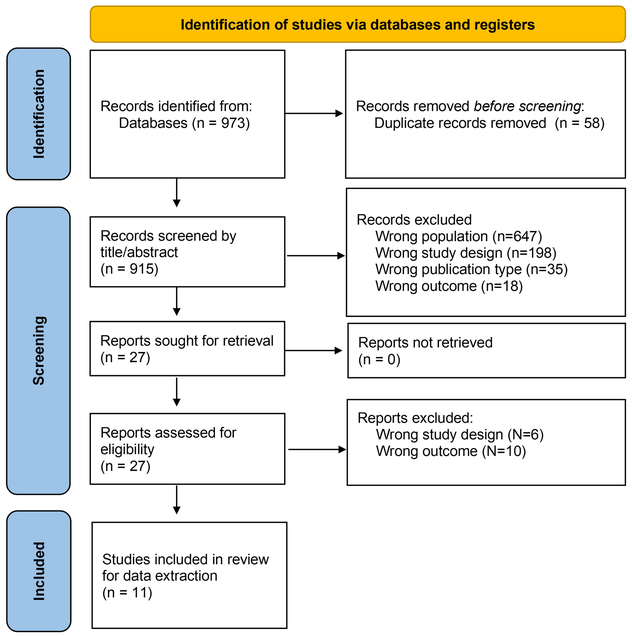
Study Attrition

- Out of the 973 articles identified through the database search, 915 abstracts were reviewed, and 27 were retrieved for full text assessment. Among these, 11 studies met the inclusion criteria and were included in the analysis **(Fig 1)**.

Characteristics of Included Studies

- The studies were conducted in US (n=5), UK (n=2), Spain (n=2), Netherlands (n=2), Sweden (n=1), France (n=1), Italy (n=1) and Japan (n=1).
- Participants included PwP (n=10) and neurologists (n=1). Sample sizes ranged from 40 to 2740 participants, with a mean age of PwP from 59 to 68 years. No study included caregivers as participants.
- Males were the majority in all studies, ranging from 53.3% to 67.2% of participants. On average, participants were diagnosed with PD more than 5 years prior to the survey administration.
- Studies employed DCE (n=8), BWS (n=3), and a TT (n=1), with one study using more than one method.
- Seven studies conducted subgroup analyses(e.g., based on previous experience with device-aided therapies (DATs), duration of diagnosis, daily hours of OFF time, age, etc)

Figure 1. Prisma Flow Diagram



Attributes and Levels

- The most common attributes were "ON time", reflecting efficacy on symptoms, and "route of administration" (ROA). Other common attributes included are indicated in **Table 1**
- "ON time" was frequently defined as "time without [troublesome] dyskinesia," and measured in terms of total duration per day, additional daily hours, or onset of treatment action.
- "ROA" included oral tablets, sublingual films, inhalation, injections, implanted device, portable pump, deep brain stimulation (DBS), and intraduodenal continuous infusion. (**Table 1**).

Relative Importance and Preferences

- Studies consistently identified "ON time" and "ROA" as important attributes of PD treatments with negative preferences for surgical requirements (brain or gastric).
- Frequency of device management, or maintenance, had an impact on preferences, with simpler device systems being favoured. Treatments requiring fewer doses of oral pills were generally preferred.

Trade-offs

- On average, respondents would be willing to take substantial safety risks for improvements in "ON time" or PD motor symptoms.
- Respondents accepted more frequent pill regimens in exchange for increased "ON time".
- However, these trade-offs were heterogeneous across studies and subgroups (e.g., people with previous experience with DBS were more willing to accept high risks for improvements in motor symptoms than people without DBS experience).

CONCLUSION

- "ON time" and "ROA" were frequently identified as important aspects.
- Current research primarily addresses established therapies, neglecting emerging ROA options such as subcutaneous infusions.
- Caregivers have an important role in supporting PwP in disease management and treatment decisions, but their perspectives are underrepresented in treatment preference research.
- Future research should address these gaps as new PD treatments emerge.
- Limitations: This study lacks systematic quality assessment, includes only 11 published studies, and may have reporting biases from unpublished or non-significant results.

Table 1. Characteristics of Preference Elicitation Methods and Attributes Included by study

	Fujioka et al. 2023	Hauber et al. 2020	Kerr et al. 2015	Marshall et al. 2017	Schölin et al. 2023	Serbin et al. 2023	Thach et al. 2021	Weernink et al. 2017	Lloyd et al. 2011
Design	2 treatment alternatives and an opt-out option with 12 choice tasks	2 treatment alternatives with 8 choice tasks	2 treatment alternatives with 8 choice tasks	DCE: 3 alternatives with 4 choice tasks BWS: 6 choice tasks	3 treatment alternatives with 9 choice tasks	2 treatment alternatives and an opt-out option with 9 choice tasks	2 treatment alternatives with 9 choice tasks	2 treatment alternatives with 9 choice tasks	NR
Statistical model	MNL	Interval regression model	RPL	DCE: RPL BWS: Cond. logit model	MNL, LC	RPL	RPL	RPL	Multi-level hierarchical logistic model
Outcomes	PW and RI	MAR, WTP	Odds ratios, MRS	DCE: PW	PW, RI	PW, RI, MAB, MAR	PW, RI, WTP	PW, RI	Odds ratios
Treatment efficacy	(1) Increase of daily "ON time" duration	(1) Daily hours of ON time; (2) Severity of movement symptoms; (3) Of pain; (4) Difficulty thinking clearly	(1) Daily hours of ON time; (2) How you feel during OFF time; (3) Predictability of 'OFF time'	(1) Effect on control of movement symptoms; (2) Ability to find the right words when speaking; (3) Ability to think clearly	(1) Aim of treatment; (2) Effect on symptoms	(1) Increase of daily "ON time" duration	(1) Time to full ON; (2) Duration of full ON	(1) Posture and balance problems; (2) Slowness of movement; (3) Tremor	(1) Impact on daily life; (2) Speech difficulties; (3) Movement control; (4) Dyskinesia; (5) OFF-periods
Safety	(2) Cognitive function related to treatment introduction; and (3) Symptoms of depression	(5) Risk of getting depression or anxiety; (6) Risk of having bleeding; (7) Risk of dying within 1 year	(4) Feelings of depression and anxiety		(3) Risk of severe side effects	(2) Additional minutes with troublesome dyskinesia; (3) Risk of diarrhea; (4) Risk of change in urine, sweat, or saliva color		(4) Dyskinesia; (5) Dizziness; (6) Drowsiness	
Route of administration	(4) Type of device			(4) Device type	(4) Type of treatment		(3) Mode of administration with possible AEs	(7) Treatment modality	
Convenience	(5) Number of pills after introduction of treatment; (6) Device management; (7) Surgery requirement	(8) Number of pills you need to take	(5) Dosing frequency	(5) Oral pill regimen		(5) Oral pill regimen			(6) Surgery type; (7) Medication need
Other					(5) Available knowledge treatment		(4) Out-of-pocket costs per 30 doses		

MNL: Multinomial logistic; RPL: Random parameter logit; Cond. Logit: Conditional logit; LC: Latent class; PW: Preference weights; RI: Relative importance; MAR: Maximum acceptable risk; WTP: Willingness to pay; MRS: Marginal rate of substitution; MAB: Minimum acceptable benefit, DCE: Discrete choice experiment; BWS: Best worst scaling; NR: No reported. If a study used more than one method, DCE attributes were reported. If an author conducted two different studies using the same attributes, only the more recent study is reported. Numbers between parentheses (n) indicate the attributes' numbering.

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DISCLOSURES
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