

REFINEMENT OF THE TREATMENT PREFERENCE QUESTIONNAIRE IN ADULTS WITH PARKINSON'S DISEASE AND OFF EPISODES

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BACKGROUND

- Little is known about factors influencing patients' preferences for on-demand or as-needed treatments for OFF episodes in Parkinson's disease (PD).
- OFF episodes are defined as the worsening or reemergence of symptoms (such as stiffness, tremors, and shaking) that commonly occur in relation to medication dosing but can be completely unpredictable in nature.
- There is a lack of fit-for-purpose, rigorously developed patient-reported outcome questionnaires suitable for evaluating patient preferences for PD OFF episode treatments.
- The **Treatment Preference Questionnaire (TPQ)** was developed to explore patients' experience with and preference for on-demand (i.e., taken as needed) OFF episode treatments.
 - The TPQ is currently being used in a phase 3, open-label, randomized, crossover trial to evaluate APL-130277, a sublingual formulation of apomorphine, compared with injectable (subcutaneous) apomorphine.
 - APL-130277 was shown in a pivotal trial (CTH-300; NCT02469090) to be effective and generally well tolerated in treating OFF episodes in patients with PD.
- The TPQ consists of 9 items evaluating preference for OFF episode treatment attributes that clinicians and patients identified as important in influencing treatment preference.¹
 - To facilitate ease and accuracy of response, each item utilizes the same 5-point response scale [Definitely prefer [Drug A], Somewhat prefer [Drug A], No preference, Somewhat prefer [Drug B], Definitely prefer [Drug B]].

OBJECTIVE

- To confirm the usability and content validity of the TPQ in patients with PD and current OFF episode experience.

METHODS

- Three rounds of qualitative interviews were conducted according to a semistructured interview guide with adults recruited via a qualitative research firm in North Carolina (United States).
 - The interview sample was selected to be similar to the APL-130277 clinical trial study population.
 - Key screening criteria were aged ≥ 18 years, self-reported physician diagnosis of PD and OFF episode experience, diagnosed ≥ 5 years ago, and currently taking levodopa/carbidopa.
 - Prior to the start of each interview, all participants provided informed consent.
- Interview Rounds 1 and 2 included concept elicitation and cognitive debriefing of the TPQ; Round 3 included debriefing only (cognitive debriefing activities were iterative).

Example concept-elicitation probes

In your own words, how would you describe an OFF episode? How, if at all, do these OFF episodes impact your daily life?

What treatment characteristics/attributes are important to you when you consider a treatment for your OFF episodes? Why?

- Ranking exercises were used to understand the relative importance of the treatment attributes.

Example cognitive debriefing probes

In your own words, what is this question asking you?

What do you think about the response choices, too many or too few?

When you think about what would influence your preference for an OFF episode treatment, is there anything missing from this questionnaire?

- Field notes and interview transcripts were used for thematic data analysis.

REFERENCE

- Ervin C, Thach A, Lee A, Navia B, Evans E, Doward L. Refinement of the treatment preference questionnaire in adults with Parkinson's Disease and off-episodes. Poster presented at the ISPOR 2019 European Conference. November 6, 2019. Copenhagen, Denmark. Value Health. 2019 Dec; 22(S3).

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RESULTS

27 individual interviews were conducted with 23 adults (4 adults participated in both Rounds 2 and 3) via phone or in person (Table 1).

Concept Elicitation

OFF Episode Experience

All participants consistently described OFF episodes as the reemergence or worsening of PD symptoms either between levodopa/carbidopa doses or at completely unpredictable times, as exemplified by the following quotes:

“...I just have to get up in the morning, and I have difficulty getting around. I'm very shaky, looking for something to hold onto. I typically get up and take my pills, then I go back and lay in bed for about half an hour until they kick in to my system.”

“Sometimes I can feel them coming on, and sometimes it's just, boom, and it's happening... I can be perfectly fine and I need to take a shower, it scares me because I don't want to fall in the shower.”



Participants most frequently reported the following treatment attributes as influencing their preference for an OFF episode treatment

Works (efficacy) quickly (n = 21)
(i.e., immediately to within 30 minutes to reduce or resolve symptoms)

How the medication is taken (n = 19)
(i.e., treatment mode, oral, inhaled, injected)

How long the medication lasts (n = 19)
(at least until their next regular dose of levodopa/carbidopa)

The ease in which the medication is taken (n = 17)
(e.g., administration is easy/does not require dexterity hindered by PD symptoms; can take while driving, outside the home, in public)

How easy the medication is to carry (n = 20)
(fits in a purse or pocket)

Is safe (n = 18)
(lacks unwanted side effects or long-term safety concerns)

To understand the relative importance of the treatment attributes, participants were asked to identify the top 3 attributes that most greatly influenced treatment preference.

The treatment attributes most frequently included among participants' top 3 important OFF episode treatment attributes were that a medication worked fast (n = 16), side effects (n = 10), and mode of administration/ease of administration (n = 10).

Participants also identified additional attributes as the single most important attribute influencing preference, including how long the medication lasted, frequency of administration, and cost.

TPQ Cognitive Debriefing

Participants across all three iterative rounds of cognitive debriefing interviews consistently indicated that the majority of TPQ items were clear and easy to understand and answer (bold text indicates interviewer probes).

Only one item (item 9) was revised between rounds; all Round 3 participants reported that the final item 9 (revised question and response scale) was easy to understand and answer, and no additional changes were recommended.

When probed, all debriefing exercise participants (n = 19; not all Round 1 participants could participate due to time constraints) reported that the TPQ assessed concepts **most relevant** to determining preference for an OFF episode medication.

Only treatment cost was noted as missing.

I think that the way this is set up is understandable... It [the TPQ] seems pretty straightforward.

Very easy. Was there any one question [1-8] that was hard to understand or that we could improve upon? I don't think so.

...those [response options] are good. Because it gives you a range of options...Definitely preferring to me means no contest. Yeah, this is a slam dunk. Somewhat is like, there's a little wiggle room in there.

...this [item 9] is easier because it's less involved. Yeah. It's more straightforward. And it follows the pattern of the other questions.

I can't think of anything else you'd want to put in there.

I'm thinking about feeling better, quicker, longer, being more normal.

Of course, I mean these [items] all make so much sense.

It's all important.

TPQ Items and Quotes of Participants' Perception of Items



1. The ease of using treatment...

Seems pretty straightforward... How simple is it... Well, I would suggest that it's convenient, it's accessible, it does not require a whole lot of forethought as to administering it.



2. Side effects...

...that's definitely...something to consider. I would imagine that the side effects create some level of distress, discomfort.



3. Use the treatment anytime, anywhere...

Yes. These are things that are pretty important...I'd like to be able to have the treatment in my purse, so I can go anywhere, and it's with me.

Well, it should be...extremely convenient, not a whole lot of preplanning or even consideration for where you are. Just get it done.



4. How quickly the medication worked...

...that would be a major factor to me...getting back 'ON'...it's important. ...that important because...if you're going to take something, you want it to be working right away.



5. How long the medication lasts...

I think what it's trying to say is how long did this drug...work on the 'OFF' symptoms.



6. Impact on daily activities...

[I]know what it's like to have my daily activities...put on hold because I'm waiting for something to...start working and get on... How long the treatment allowed me to do my usual daily activities. Well, that's really what's it all about.



7. Treatment satisfaction...

Well, a lot of things we've already been talking about, so overall satisfaction. How long does it take to get rid of my symptoms... Satisfaction would be that, and how effectively is it taking away the symptoms. How easy it was to use, the side effects, the ability to use it anywhere, how quickly it worked.



8. The overall effect of treatment

...overall effect in my treatment...How well did I get out of the ditch. How soon did I get out of the ditch? ...I'm looking at the big picture of how it, how it would have impacted me, how it would affect me, kind of everything all together.



9. Direct preference comparison between 2 treatments

Yeah, that's straightforward. It's clear. Okay. And in your own words, what is 9 asking you? Which one do I prefer. I'm thinking about the total experience...The convenience, the access, the satisfaction, you know, all those factors I would frame in the context of overall.

CONCLUSIONS

- Participants reported that all concepts important to influencing OFF episode treatment preference (with the exception of treatment cost) are assessed in the TPQ.
- All participants consistently interpreted, easily understood, and answered the majority of TPQ questions; changes made to item 9 to improve clarity and ease of response met with success.
- This qualitative evidence supports the content validity of the TPQ; a quantitative evaluation to provide additional evidence on the psychometric properties of the TPQ is planned.

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