A DISCRETE-CHOICE EXPERIMENT EVALUATING PREFERENCES FOR ON-DEMAND TREATMENTS FOR PATIENTS WITH PARKINSON'S DISEASE AND "OFF" EPISODES

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KEY FINDINGS

- In an online discrete-choice experiment (DCE) survey evaluating patient preferences for theoretical on-demand treatments of Parkinson's disease (PD)-related "OFF" episodes, participants placed the most importance on avoidance of high out-of-pocket costs and mode of administration
- A theoretical dissolvable sublingual film with no adverse events (AEs) was preferred over all other modes of administration and mode-specific AEs
  - The least preferred mode of administration was injection with possible injection-site reaction
- Participants were willing to pay considerably more for a theoretical
  - Dissolvable sublingual film with AEs vs an inhaled treatment with AEs ($24)
  - Dissolvable sublingual film with AEs vs an injectable with AEs ($52)
  - Treatment that decreased the time to FULL "ON" ($58)

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INTRODUCTION

- As PD progresses, nearly all patients receiving oral carbidopa/levodopa experience "OFF" episodes, defined as periods during the day when symptoms reappear or worsen[1]
- "OFF" episodes may have a significant negative impact on patient quality of life[2]
- Currently, there are 3 options for the on-demand treatment of "OFF" episodes that have been approved by the US Food and Drug Administration (**Table 1**)

Table 1. Approved On-Demand Treatments for "OFF" Episodes

Treatment	FDA Approval
APOKYN® (apomorphine hydrochloride injection), for subcutaneous use[3]	2004
INBRIJA® (levodopa inhalation powder), for oral inhalation use[4]	2018
KYNMOBI™ (apomorphine hydrochloride) sublingual film[5]	2020

FDA, US Food and Drug Administration.

OBJECTIVE

- To quantify patient preferences for theoretical on-demand treatments among patients with PD and "OFF" episodes

METHODS

Study Design

- Participants were recruited for an online DCE survey from September–October 2019
  - DCEs are based on the principle that products or services comprise multiple attributes and that the choice of a product or service is a function of the utility of each attribute
  - DCEs have been used to elicit patients' preferences for a wide range of health care topics[6,7]
- In each DCE question, participants selected between a pair of experimentally designed profiles for theoretical on-demand "OFF" episode treatments that varied by the attributes shown in **Table 2**
  - Attributes were selected based on qualitative interviews with 15 participants and were based on characteristics of existing on-demand treatments for "OFF" episodes; the survey instrument was then evaluated and revised based on pretest interviews with an additional 15 participants
- A full fractional design containing 72 DCE questions was used to create 8 blocks of 9 DCE questions each; participants were randomly assigned to 1 of these blocks

Table 2. DCE Attributes and Levels[a] of Theoretical Treatments

Mode of Administration; Possible AEs	Time to FULL "ON"	Duration of FULL "ON"	Out-of-pocket Cost Per 30 Doses
• Inhaled; no AEs • Inhaled; cough or mild respiratory infection • Injected; no AEs • Injected; injection-site reaction • Dissolvable sublingual film; no AEs • Dissolvable sublingual film; mouth or lip sores	• 15 minutes • 30 minutes • 60 minutes	• 1 hour • 1.5 hours • 2 hours	• $0 (no cost) • $10 • $30 • $90

[a]Values or categories used to characterize the theoretical treatment profiles in the DCE questions.
AE, adverse event; DCE, discrete-choice experiment.

Study Population

- Carbidopa/levodopa-treated adults (age 18–75 years) from the US with a self-reported diagnosis of PD for ≥5 years or <5 years but with "OFF" episode experience were recruited through a health care research recruiting firm (Global Perspectives, Norwich, England) using online research panels and other ad hoc recruiting sources (ie, recruiters' patient databases, physician referrals, online support groups, and targeted advertising on social media)

Statistical Analyses

- Data were analyzed using a random parameters logit model
  - The model related participants' choices to the differences in attribute levels across the alternative levels in each DCE question[8]
  - Variables for mode of administration, time to FULL "ON," and duration of FULL "ON" were effects-coded categorical variables
  - Cost was modeled as a continuous linear variable adjusted for the participant's income
  - The overall relative importance of each attribute was calculated as the utility difference between the most- and least-preferred levels and was conditional on the levels selected for the survey
- Results were used to calculate willingness to pay (WTP),[9] which was based on a median income of $87,500

RESULTS

- Among the 300 participants, 294 (98%) had experience with "OFF" episodes (**Table 3**)

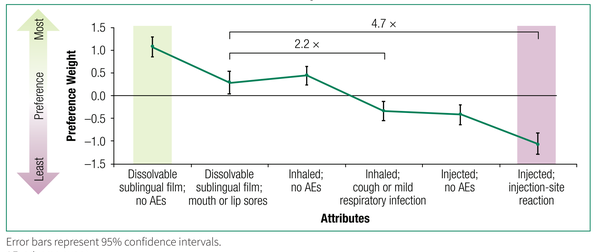
Table 3. Baseline Demographics and Clinical Characteristics

	Participants (N=300)
Age, y, mean	59.0
Male, n (%)	180 (60)
White, n (%)	248 (83)
Education, n (%)	
High school/GED	18 (6)
College degree/technical school or some college	185 (62)
Graduate/professional degree or some graduate school	95 (32)
Other/prefer not to answer	2 (<1)
Has caregiver,[a] n (%)	112 (37)
Time since PD diagnosis, n (%)	
In the past year	9 (3)
1–2 years ago	68 (23)
3–4 years ago	80 (27)
≥5 years ago	142 (47)
Don't know/not sure	1 (<1)
Experience with "OFF" episodes, n (%)	294 (98)
Frequency of "OFF" episodes,[b] n (%)	
Multiple times per day	74 (25)
Once a day	77 (26)
Every few days	91 (31)
About once a week	28 (10)
Every few weeks	17 (6)
About once a month or less	7 (2)

[a]Included full-time or part-time caregiver.
[b]Based on 294 participants who had experience with "OFF" episodes.
GED, General Educational Development; PD, Parkinson's disease.

- A dissolvable sublingual film with no AEs was preferred over all other modes of administration (**Figure 1**)

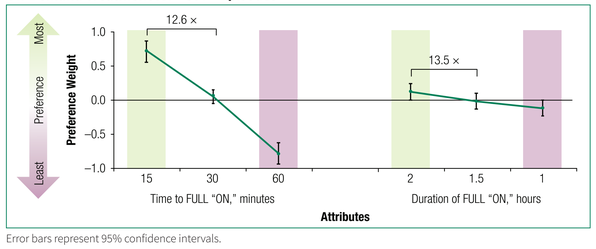
Figure 1. Participant Preferences for Mode of Administration With Possible AEs of a Theoretical On-Demand Treatment for "OFF" Episodes



Error bars represent 95% confidence intervals.
AE, adverse event.

- Shorter time to FULL "ON" and longer duration of FULL "ON" were preferred (**Figure 2**)

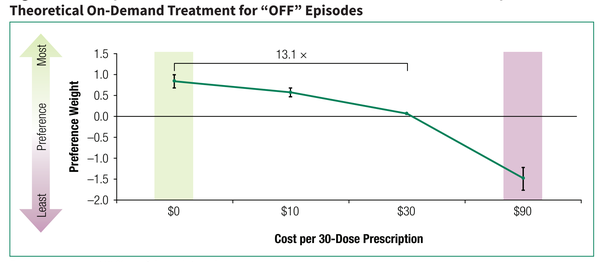
Figure 2. Participant Preferences for Time to and Duration of FULL "ON" of a Theoretical On-Demand Treatment for "OFF" Episodes



Error bars represent 95% confidence intervals.

- $0 out-of-pocket cost per 30-dose prescription was preferred over any greater cost (**Figure 3**)

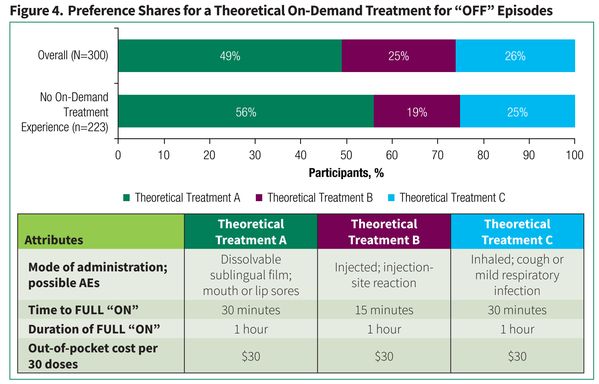
Figure 3. Participant Preferences for Out-of-Pocket Cost Per 30-Dose Prescription of a Theoretical On-Demand Treatment for "OFF" Episodes



Error bars represent 95% confidence intervals.

- Participant preference shares were greatest for a dissolvable sublingual film over other modes of administration (**Figure 4**)

Figure 4. Preference Shares for a Theoretical On-Demand Treatment for "OFF" Episodes

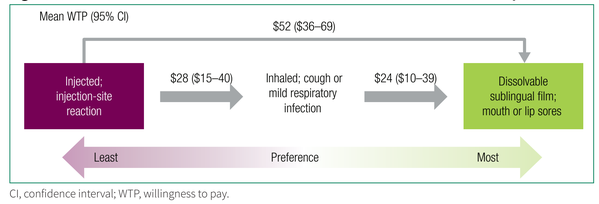


Attributes	Theoretical Treatment A	Theoretical Treatment B	Theoretical Treatment C
Mode of administration; possible AEs	Dissolvable sublingual film; mouth or lip sores	Injected; injection-site reaction	Inhaled; cough or mild respiratory infection
Time to FULL "ON"	30 minutes	15 minutes	30 minutes
Duration of FULL "ON"	1 hour	1 hour	1 hour
Out-of-pocket cost per 30 doses	$30	$30	$30

AE, adverse event.

- Participants were willing to pay more for preferred attributes of an on-demand treatment for "OFF" episodes (**Figure 5**)

Figure 5. WTP for Attributes of a Theoretical On-Demand Treatment for "OFF" Episodes



CI, confidence interval; WTP, willingness to pay.

- Mean WTP (95% confidence interval [CI]) to move from the least preferred mode of administration (injection with injection-site reaction) to the most preferred mode of administration (dissolvable sublingual film with no AEs) was $83 ($66–99)
- Mean WTP (95% CI) to decrease time to FULL "ON" from 60 to 15 minutes was $58 ($46–70)
- Mean WTP (95% CI) to increase duration of FULL "ON" from 1 to 2 hours was $9 ($1–17)

LIMITATIONS

- Study sample may be subject to selection bias based on the online nature of the survey and that it was not designed to be representative of the overall US population of patients with PD
- Participants self-reported demographic and disease information
- Data were based on theoretical choice profiles; therefore, differences can arise between stated and actual choices in the real world

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