PHARMACY SYSTEMS OUTCOMES AND POLICY
COLLEGE OF PHARMACY

OFF Episode quality of Life Impact scAle (OFFELIA) in people with Parkinson's Disease: new evidence of construct validity

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INTRODUCTION

- OFF Episodes (OEs) in people with Parkinson's disease (PwP) are characterized by the emergence of motor and/or non-motor symptoms such as anxiety and depression, leading to functional disability and decreased quality of life (QOL).[1]
- Current patient-reported outcome measures (PROMs) for assessing OE severity primarily track "OFF" time hours using patient diaries but lack comprehensive data on the diverse and critical motor and non-motor symptoms associated with OEs.[2]
- Given the lack of available PROMs that measure the impact of OEs on QOL in PwP, the applicant team recently developed a new 18-item instrument Off-Episode Quality Life Impact Scale (OFFELIA).

OBJECTIVES

- The objective of this study was to investigate the convergent and discriminant validity of the OFF-Episode Quality of Life Impact ScAle (OFFELIA) in relation to other established health-related quality of life measures used in Parkinson's Disease research.[3]

METHODS

- Participants completed the cross-sectional health and disease questionnaire "Impact and Communication on OFF Periods" as part of a standard schedule of activities at Fox Insight, an online data platform for people with PD developed by the MJ Fox Foundation and EQ-5D-5L [4], Parkinson's Disease Questionnaire-8 (PDQ-8) [5], The Penn Parkinson's Daily Activities Questionnaire-15 (PDAQ-15) [6], Geriatric Depression Scale (GDS) [7], Unified Parkinson's Disease Rating Scale (UPDRS II) [8], Non-Motor Symptoms Questionnaire (NMSQ) [9].
- OFFELIA was scored as two subscales: functioning (12-items) and psychological well-being (5-items).
- Convergent validity was examined between the summary score of two OFFELIA subscales and six disease-specific and generic measures: EQ-5D-5L [4], PDQ-8 [5], PDAQ-15 [6], GDS [7], UPDRS-II [8], NMSQ scores [9], using Pearson correlations (r). The correlation coefficient (r) was considered strong if it is greater than or equal to 0.7, moderate if it is less than 0.7 but greater than or equal to 0.3, and weak if it is less than 0.3. [10]
- Discriminative validity was assessed by conducting known group comparisons (KGC) using Cohen's effect sizes (ES) and analysis of variance F-ratios based on unpredictability, duration, onset and frequency of OEs. The ES was calculated between each severity group and the higher severity group for each OE indicator and instrument, using Cohen's criteria (small ES = 0.2, medium ES = 0.5, and large ES = 0.8). [11]

RESULTS

Figure 1. Respondent demographic and summary characteristics

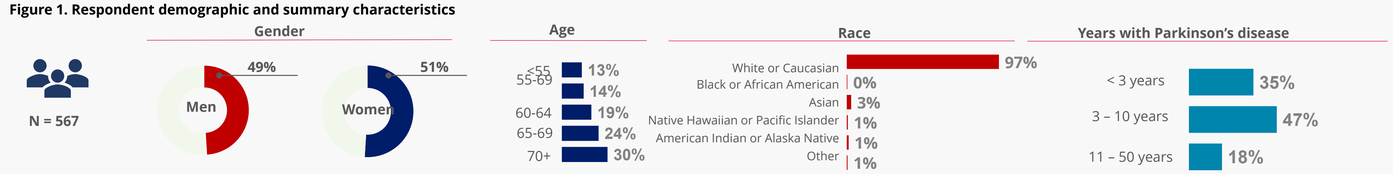

Table 1. Convergent validity results: OFFELIA with health measurement scales

Instruments	Functioning Factor[c]	Functioning Factor (without Employment item) [c]	Psychological Well-being Factor[c]
EQ5D Index	-0.55	-0.56	-0.38
EQ5D VAS	-0.40	-0.40	-0.30
PDQ-8	0.60	0.60	0.50
GDS	0.44	0.45	0.38
UPDRS-II	0.57	0.59	0.34
PDAQ-15	-0.41	-0.42	-0.30
NMSQ	0.47	0.47	0.36

All correlation coefficient (r) p-values were statistically significant at p<0.01; PDQ-8=Parkinson's Disease Questionnaire; EQ-5D-5L=EuroQol-5 Dimension; PASE=Physical Activity Scale for the Elderly; GDS=Geriatric Depression Scale; UPDRS=Unified Parkinson's Disease Rating Scale; NMSQ=Non-Motor Symptoms Questionnaire; PDAQ-15= The Penn Parkinson's Daily Activities Questionnaire-15.

Moderate associations (0.3 ≥ r > 0.7) were found between the OFFELIA subscales and the EQ-5D-5L index score, EQ VAS, PDQ-8, UPDRS-II, GDS and NMSQ

Table 2. Known group comparison: performance of EQ-5D-5L, PDQ-8, and OFFELIA subscales

	EQ-5D-5L					PDQ-8					OFFELIA - Functioning (F1)			OFFELIA- Psychological Well-Being (F2)		
Categories (n)	Mean	ES[a]	F[b]	F_1 / F_{EQ-5D}	F_2 / F_{EQ-5D}	Mean	ES[a]	F[b]	F_1 / F_{PDQ}	F_2 / F_{PDQ}	Mean	ES[a]	F[b]	Mean	ES[a]	F[b]
Unpredictability (Proportion of OFF episodes that are unpredictable)																
0 (93)	0.73		9.3	2.42	1.37	17.64		8.93	2.53	1.42	23		22.55	12.01		12.71
<25% (256)	0.68	0.27				22.06	0.35				27.79	0.47		13.34	0.26	
25-50% (106)	0.60	0.42				27.54	0.38				34.63	0.66		15.93	0.51	
>50% (73)	0.62	0.09				26.03	0.09				31.12	0.32		15.52	0.08	
Duration (Duration of each OFF period, on average)																
<15 min (48)	0.75		4.32	2.87	2.54	16.08		5.72	2.17	1.92	20.83		12.4	10.6		10.98
15-30 min (161)	0.70	0.26				20.75	0.37				26.72	0.59		12.79	0.43	
30-45 min (123)	0.65	0.26				23.86	0.22				29.5	0.27		14.72	0.37	
45-60 min (150)	0.63	0.1				25.69	0.12				31.84	0.21		15.13	0.08	
>120 min (39)	0.63	0				27.08	0.08				32	0.01		16.05	0.17	
Onset (Years since initial onset of OFF periods)																
<1 year (190)	0.69		2.55	7.55	1.78	20.25		6.64	2.90	0.68	25.19		19.24	13.17		4.53
1-5 years (278)	0.66	0.15				23.47	0.23				29.22	0.39		13.79	0.12	
6-10 years (52)	0.63	0.16				27.76	0.29				34.46	0.49		15.69	0.35	
>10 years (18)	0.59	0.19				32.64	0.26				39.94	0.48		16.56	0.16	
Frequency (Frequency of OFF periods per day in an average week)																
0 (30)	0.69		6.74	3.01	1.95	19.48		10.51	1.93	1.25	23.6		20.29	12.97		13.13
1 per day (187)	0.72	-0.16				18.5	-0.08				24.52	0.10		12.02	-0.20	
2 per day (161)	0.64	0.41				24.75	0.45				29.95	0.54		14.78	0.55	
3 per day (110)	0.61	0.14				27.27	0.16				33.71	0.35		15.83	0.19	
>4 per day (39)	0.62	-0.05				30.53	0.20				35.31	0.14		16.21	0.07	

OFFELIA can discriminate patients better than the EQ-5D-5L and PDQ-8 across different severity levels of unpredictability (F-ratio: 2.42 and 2.53 respectively), duration (F-ratio: 2.87 and 2.17 respectively), onset (F-ratio: 7.55 and 2.90 respectively) and frequency (F-ratio: 3.01 and 1.93 respectively).

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CONCLUSION

Initial evidence supports the construct validity of OFFELIA, a new instrument for evaluating the impact of OEs on HRQL and functioning in PD patients, and demonstrates its potential to better capture the impact of OEs and clinical benefits of new and existing therapeutic strategies.

"The Fox Insight Study (FI) is funded by The Michael J. Fox Foundation for Parkinson's Research. We would like to thank the Parkinson's community for participating in this study to make this research possible."
Data used in the preparation of this article were obtained from the Fox Insight database (https://foxinsight-info.michaeljfox.org/insight/explore/insight.jsp) on 28/03/2022 . For up-to-date information on the study, visit https://foxinsight-info.michaeljfox.org/insight/explore/insight.jsp."

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