Understanding the Need for Assistance When Completing Measures of Patient-Reported Outcomes in Huntington Disease

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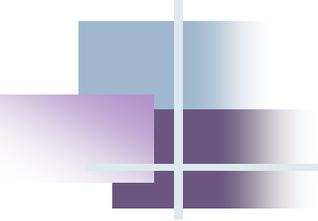
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Health-related quality of life is critically important for people with Huntington disease (HD)

- Motor, cognitive and psychiatric decline begins more than a decade before diagnosis (prodromal HD) and progressively worsens over the disease course (Paulsen, 2010).

- Unfortunately, the symptoms that are characteristic of HD may also impair the ability of affected individuals to complete patient-reported outcome (PRO) measures.

- One important methodological standard for a PRO instrument is to minimize respondent burden, i.e., the time, effort and other demands needed to complete the instrument (Scientific Advisory Committee of the Medical Outcomes Trust, 2002;US Food and Drug Administration, 2009;Groves, 2009).

Study Objectives

1) To describe the extent and type of assistance required by HD participants to complete PRO measures

2) To examine the impact of such assistance on PRO scores

➢ Hypothesis: participants with greater disease severity would require more assistance than those who were asymptomatic or had less disease severity

Study Methods: Huntington Disease Quality of Life (HDQLIFE)

- A sample of English-speaking adults (age 18+) across the prodromal and diagnosed disease severity spectrum completed PROs from Neuro-QoL and PROMIS identified as relevant to HD, and newly developed HDQLIFE item pools, through PROMIS Assessment Center.

- Participants were instructed that they could ask a caregiver, friend, family member or study staff member to assist them in using the computer, e.g., logging into the online study, reading questions aloud and/or clicking response options.

- Participants reported the amount and type of assistance they received.
  - Since these assistance questions were presented at the end of the online assessment, they were not presented to participants who stopped before completing all of the PRO surveys (*n*=56, 10.5%).

- Standard statistical tests were used to compare sociodemographic, clinical and PRO data across HD diagnostic groups and assistance groups, and to compare participants who were excluded from this report to those who were included.

- Multivariable linear regression models were created for PROs, and least-squares means were estimated to determine the effects of assistance on PROs after adjustment for other covariates.

Sociodemographic and Clinical Characteristics, by Huntington Disease (HD) Diagnosis

	Prodromal (*n*=191)	Early Stage HD (*n*=186)	Late Stage HD (*n*=99)	*p*-value
Sociodemographics				
Female Gender	122 (63.9%)	101 (54.3%)	55 (55.6%)	0.137
Age in Years	42.6 (12.2)	51.8 (12.4)	55.3 (11.5)	<0.001
Ethnicity, Race				
Hispanic, any race	3 (1.7%)	7 (3.9%)	1 (1.0%)	0.066
Non-Hispanic, White	173 (96.6%)	170 (94.4%)	90 (92.8%)	
Non-Hispanic, Other	3 (1.7%)	3 (1.7%)	6 (6.2%)	
Highest Education				
Less than HS	0 (0.0%)	4 (2.2%)	2 (2.0%)	0.001
HS/GED	20 (10.5%)	47 (25.4%)	21 (21.2%)	
More than HS	170 (89.5%)	134 (72.4%)	76 (76.8%)	
Clinician-Rated Disease Severity				
Independence Scale	97.9 (5.8)	85.2 (9.7)	61.2 (12.2)	<0.001
Total Motor Dysfunction Score	5.5 (6.1)	30.8 (14.7)	54.4 (21.3)	<0.001
Cognitive Performance Stroop Interference Test (# correct)	46.6 (15.6)	29.1 (10.1)	18.0 (9.2)	<0.001

Assistance in Completing PRO Surveys, by Huntington Disease (HD) Diagnosis

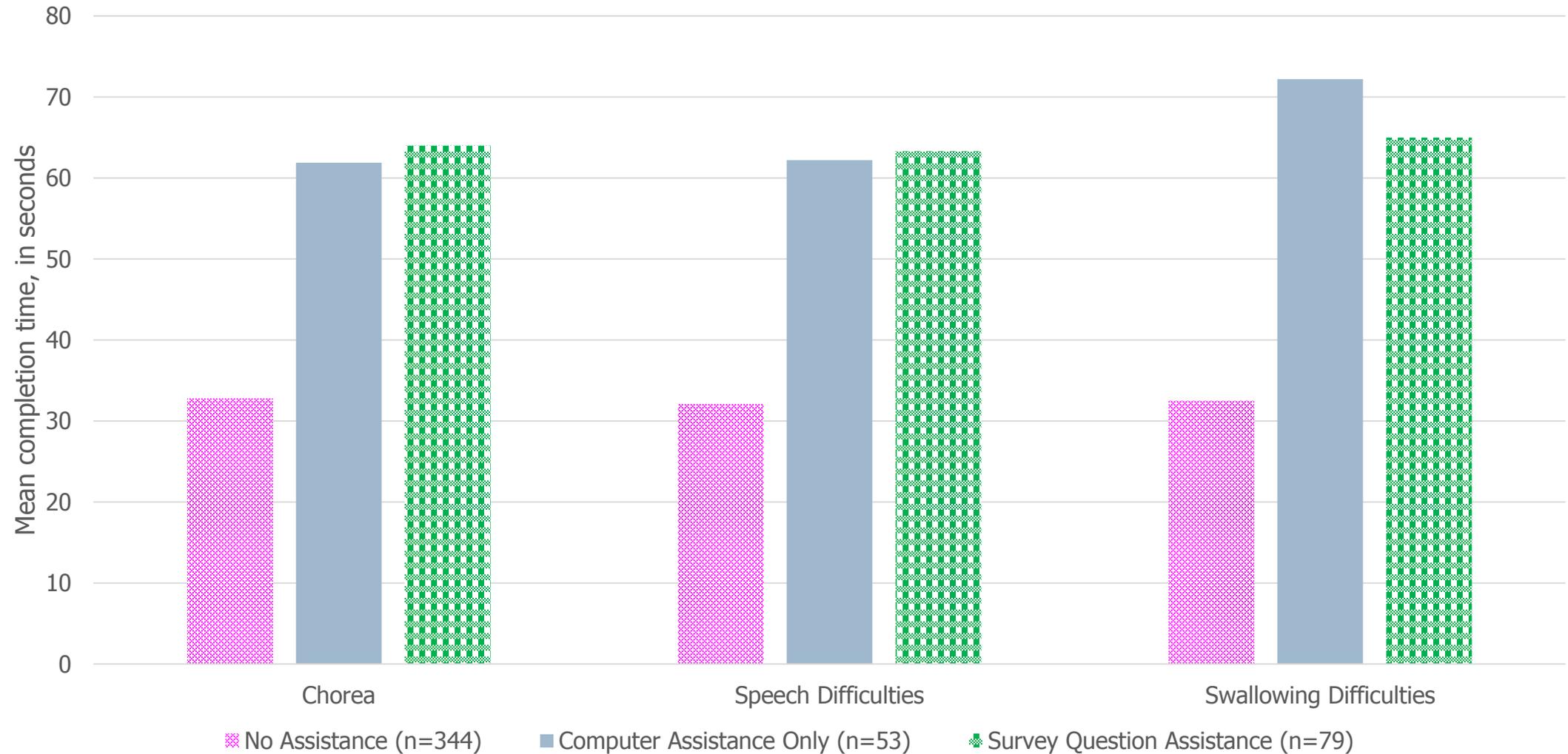|	Prodromal (*n*=191)	Early Stage HD (*n*=186)	Late Stage HD (*n*=99)	*p*-value
Assistance				
None	190 (99.5%)	132 (71.0%)	22 (22.2%)	<0.001
Some assistance provided	1 (0.5%)	54 (29.0%)	77 (77.8%)	
Type of Assistance				
None	190 (99.5%)	132 (71.0%)	22 (22.2%)	<0.001
Assistance with computer only	1 (0.5%)	28 (15.1%)	24 (24.2%)	
Assistance with survey questions (explain questions, remind me of important information, answer questions for me)	0	26 (14.0%)	53 (53.5%)	
How Often Assistance Needed				
Never	190 (99.5%)	132 (71.0%)	22 (22.2%)	0.010
Rarely (0-25% of the time)	0	20 (10.8%)	12 (12.1%)	
Sometimes (26-50% of the time)	1 (0.5%)	9 (4.8%)	10 (10.1%)	
Often (51-75% of the time)	0	9 (4.8%)	12 (12.1%)	
Always (76-100% of the time)	0	16 (8.6%)	43 (43.4%)	

Sociodemographic and Clinical Characteristics, by Type of Assistance Needed
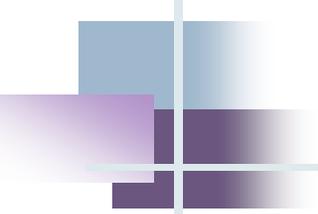
	No Assistance (*n*=344)	Assistance with Computer Only (*n*=53)	Assistance with Survey Questions (*n*=79)	*p*-value
Huntington Disease (HD) Diagnosis				
Prodromal	190 (55.2%)	1 (1.9%)	0 (0.0%)	
Early Stage HD	132 (38.4%)	28 (52.8%)	26 (32.9%)	<0.001
Late Stage HD	22 (6.4%)	24 (45.3%)	53 (67.1%)	
Sociodemographics				
Female Gender	210 (61.0%)	26 (49.1%)	42 (53.2%)	0.150
Age in Years	46.7 (12.5)	57.1 (11.3)	52.7 (14.3)	<0.001
Ethnicity, Race				
Hispanic, any race	8 (2.4%)	2 (3.8%)	1 (1.3%)	
Non-Hispanic, White	315 (99.0%)	48 (92.3%)	70 (92.1%)	0.079
Non-Hispanic, Other	5 (1.6%)	2 (3.8%)	5 (6.6%)	
Highest Education				
Less than HS	1 (0.3%)	2 (3.8%)	3 (3.8%)	
HS/GED	54 (15.8%)	14 (26.4%)	20 (25.3%)	0.002
More than HS	287 (83.9%)	37 (69.8%)	56 (70.9%)	
Clinician-Rated Disease Severity				
Independence Scale	91.8 (11.1)	70.8 (16.2)	66.6 (14.6)	<0.001
Total Motor Dysfunction Score	16.1 (15.9)	50.0 (22.6)	50.2 (19.9)	<0.001
Cognitive Performance				
Stroop Interference Test (# correct)	39.1 (15.8)	21.2 (8.0)	19.2 (10.2)	<0.001

Adjusted Health-Related Quality of Life Outcomes, by Type of Assistance Needed

	No Assistance (*n*=344)	Assistance with Computer Only (*n*=53)	Assistance with Survey Questions (*n*=79)	*p*-value
	Least-squares means (standard error), adjusted for diagnostic group and sociodemographics (gender, age, race/ethnicity)			
HDQLIFE Chorea	51.5 (1.1)	54.3 (1.4)	55.4 (1.3)	0.001
HDQLIFE Speech Difficulties	50.9 (1.1)	51.4 (1.4)	53.9 (1.3)	0.039
HDQLIFE Swallowing Difficulties	51.7 (1.1)	52.7 (1.4)	53.4 (1.3)	0.363
HDQLIFE Concern with Death and Dying	51.1 (1.5)	49.5 (1.9)	49.1 (1.8)	0.370
HDQLIFE Meaning and Purpose	49.5 (1.4)	50.0 (1.8)	50.5 (1.7)	0.805

Estimated Time to Complete 6-item Short Forms, by Type of Assistance Needed

Mean completion time, in seconds

Chorea   Speech Difficulties   Swallowing Difficulties

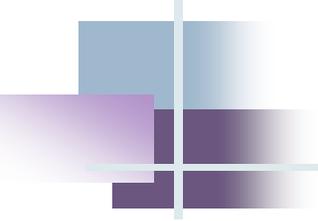No Assistance (n=344)   Computer Assistance Only (n=53)   Survey Question Assistance (n=79)

Conclusions and Implications

- To our knowledge, this is the first study to measure the extent to which HD research participants:
  - use assistance to complete online PRO surveys
  - and whether PRO scores differ between those who did and did not use assistance.

- Participants who received assistance with survey completion were older, more advanced in disease, and had greater clinician-rated cognitive and functional impairment than those who did not receive assistance.
  - This emphasizes the potential impact of HD on ability to complete PROs independently.
- Compared to those who did not receive assistance, participants who received assistance with survey completion had poorer scores on functional PROs (Chorea, Speech Difficulties). End of Life PROs (Concern with Death & Dying and Meaning & Purpose) did not differ.

Conclusions and Implications

- Individuals with more advanced HD are likely to require some assistance completing PRO surveys.
  - Clinicians and researchers should be aware of the necessity of assistance, and should plan the best strategies for assessing PROs in people with HD.
  - A recent meta-analysis of diverse adult samples reported overall equivalence in PRO scores between self-completion and assisted completion (Rutherford et al., 2016).
- Participants who received assistance were also less likely to have post-high school education than persons who did not require assistance.
  - Computerized PRO surveys may be more challenging for people with lower levels of education. Multimedia methods of survey administration have been developed to accommodate people with a range of literacy, language and computer skills in primary care and chronic disease populations (www.healthlitt.org). These methods might be useful for people with HD.

Acknowledgements

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