

DDSIG New and Noteworthy

APTA Neurology Section

6/1/2017

Volume 2 Edition 4

New

Journal Club

Survey of the Huntington's Disease Patient and Caregiver Community Reveals Most Impactful Symptoms and Treatment Needs [Article Link](#)

Simpson JA, et. al. J Huntingtons Dis. 2016 Dec 15;5(4):395-403.

INTRODUCTION: The Huntington's Disease Society of America (HDSA) created and distributed 2 surveys on the symptom experience and treatment approaches for Huntington's disease. The objective of these surveys was to identify the specific symptoms that most impact the daily lives of individuals with Huntington's disease/Juvenile Huntington's disease (HD/JHD) and their caregivers and to solicit input on the types of treatments desired by HD affected families. The data were shared with the FDA to offer background and insight in preparation for the patient-focused meeting, as well as to ensure representation by the community in a manner that

would complement those who attended in person.

METHODS: Surveys were created using SurveyMonkey to capture patient and caregiver perspectives on HD symptoms and current treatments.

RESULTS: More than 3,600 responses to the two surveys were received. The data showed that both caregivers and individuals with HD were severely impacted by the cognitive and behavioral symptoms of HD with HD patients reporting problems with executive functioning and cognitive decline as most impactful to them. However, 30 percent of caregivers reported that chorea was the most impactful symptom compared to 17 percent of people with HD. Across all the symptom categories, patients reported a lower occurrence of symptoms than were reported by their caregivers.

CONCLUSIONS and CLINICAL IMPLICATIONS: With only one drug approved for treatment of a symptom of Huntington's disease and no disease modifying treatments available, there is a critical need for new medicines to treat the cognitive, psychiatric and motor symptoms associated with HD. While the surveys did not capture risk/benefit data, the data collected do provide new insights around the different perspectives of patients and caregivers. We believe that industry development of treatments would be well-informed by incorporating the patient community, which is more knowledgeable and engaged than given credit, in consideration of treatment regimens, risk-benefit and priorities for therapeutic development.

Noteworthy

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This article will be reviewed in the DDSIG Case-Based Journal Club on June 8th from 5-6:30pm MST / 7-8:30pm EST. We will email call-in information soon. You can also email us at neuroddsig@gmail.com to get the information.

DDSIG Welcomes Rebecca Martin to the Nominating Committee and congratulates Nora Fritz on her move to the ANPT nominating committee. A VERY sad Good-Bye to Tara McIsaac our out-going Chair.

Volunteer with the DDSIG!
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