Huntington's Disease patients need better understanding of risks

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For patients with Huntington's disease (HD), clinical trials can offer hope when there are no treatments available despite unknowns about whether the therapy will work or is safe. A new study in the *Journal of Huntington's Disease* found that although the HD community appears highly optimistic about HD research, patients are at risk for therapeutic

misconception. In order to allay patients' misgivings, investigators recommend improvements to patient-doctor communication to better convey trial goals, risks and benefits.

New therapies to modify the course of HD are currently entering clinical trials. "This is a very exciting time for the HD community," explained lead investigator Kristina Cotter, Ph.D., CGC, Department of Genetics, Stanford University School of Medicine, Stanford, CA. "Currently, there are no therapies that slow down the progression of HD; existing treatments only help manage some of the disease symptoms. There are several therapies being studied in trials that could potentially slow down the disease. However, there is a risk inherent in clinical trial participation because the trial is studying whether or not these new therapies are safe. Potential participants should be able to discuss their hopes and fears associated with the clinical trial."

In order to assess clinical trial attitudes and expectations investigators administered a questionnaire that rated participant attitudes towards a hypothetical HD clinical trial and how well participants understood the clinical research process. They also asked participants whether the way a therapy is administered (such as a pill or injection) changes their willingness to participate in a trial. The questionnaire was distributed through HD-related organizations. There were 73 responses from individuals who self-reported as clinically diagnosed with HD (20 patients) or who have the HD gene mutation but who are not yet symptomatic (22 patients), and 31 primary caregivers.

The study found that patients and families affected by HD believe that individuals with HD should participate in research. They viewed clinical trials positively and generally safe. They were able to appraise risks and benefits of research and were usually optimistic a new therapy would work. Individuals with prior HD-related research experience were less likely to have negative expectations about trials than those without

research experience. Respondents also wanted as much information about a study as possible, with women exhibiting higher information needs than men. Investigators also learned that willingness to participate was highest when the route of administration was minimally invasive and that invasive therapies (such as those injected into the spinal column, brain, or eye) might decrease the likelihood that an individual would participate in research.

Interestingly, investigators found that patients with HD were less likely to recognize the difference between clinical trials and the typical care received in an HD clinic. Finally, they found that patients with HD are more likely to believe that research participation is for personal benefit and that care they receive in a clinical trial is similar to that received in an HD clinic.

"It is possible that this belief is linked to the cognitive decline that occurs in HD," noted Dr. Cotter. "This tells us that as we recruit for clinical trials, we should take extra care to share detailed information about trial goals, risks and benefits, how a new therapy is administered, and how the trial is different from clinical care."

Regardless of the disease, participating in clinical trials is a big decision for patients and their families. This study provides clinicians with information about how to discuss research with the HD community:

- Emphasize the distinction between research and clinical standard of care and consider a patient's past experiences with clinical research when discussing trial opportunities
- Evaluate patients and caregivers for misunderstandings around the clinical research process
- Anticipate questions and concerns surrounding more invasive routes of administration
- Provide patients and caregivers with both written and verbal

information about the trial, bearing in mind that women may require more details than men

"It is very exciting to be able to offer possible disease-modifying therapies to patients with HD in clinical trials," commented co-investigator Sharon J. Sha, MD, MS, Department of Neurology and Neurological Sciences, Stanford University, Stanford, CA. "However, we need to make sure everyone involved—patients and families—can ask questions and receive all of the information they need to make informed decisions. This research is important because it reminds us, as clinicians, to provide better informed consent and to be wary of both the investigators' and participants' biases towards research participation."

The investigators hope that the findings from this study will help guide conversations among clinicians, researchers, and patients to help patients make the best decision for themselves and their families. "The better clinicians and researchers understand HD patients' and families' beliefs and values, the better equipped we will be to help them decide whether participation in clinical trials is appropriate for them," added co-investigator Andrea Hanson-Kahn, MS, LCGC, Department of Genetics, Stanford University School of Medicine and Department of Pediatrics, and Division of Medical Genetics, Stanford University Medical Center, Stanford, CA.

HD is a fatal genetic neurodegenerative disease characterized by atrophy of certain regions of the brain. It causes the progressive breakdown of nerve cells in the brain. HD patients experience behavioral changes and uncontrolled movements. Symptoms include personality changes, mood swings and depression, forgetfulness and impaired judgment, and unsteady gait and involuntary movements (chorea). Every child of an HD parent has a 50% chance of inheriting the gene. Patients usually survive 10-20 years after diagnosis.

More information: Kristina Cotter et al, Positive Attitudes and Therapeutic Misconception Around Hypothetical Clinical Trial Participation in the Huntington's Disease Community, *Journal of Huntington's Disease* (2019). DOI: 10.3233/JHD-190382