Study: Drug significantly reduces chorea symptoms in patients with Huntington's disease

May 19 2023

Credit: Pixabay/CC0 Public Domain

The drug valbenazine statistically improves chorea, a movement disorder commonly associated with Huntington's disease, when compared to a placebo, according to a recent international study led by UTHealth Houston researcher Erin Furr Stimming, MD, who served as principal investigator on behalf of the KINECT-HD Huntington Study Group.

The study results—which were published in *The Lancet Neurology*—come one year after Furr Stimming, professor of neurology and Memorial Hermann Chair with McGovern Medical School at UTHealth Houston, presented an early abstract of the findings at the American Academy of Neurology 2022 Annual Meeting in Seattle.

"Positive study results remind us there is a reason for hope," said Furr Stimming, who is also director of the Huntington's Disease Society of America Center of Excellence at UTHealth Houston Neurosciences. "Chorea associated with Huntington's disease can negatively impact quality of life and functional independence; therefore, studying additional medications to address this hallmark motor symptom is imperative."

The Phase III, randomized, double-blind, placebo-controlled KINECT-HD study, which enrolled 128 participants, was designed to evaluate the efficacy of valbenazine as a once-daily treatment to reduce chorea associated with Huntington's disease, as well as evaluate the drug's safety and tolerability. Valbenazine is a selective vesicular monamine transporter 2 (VMAT 2) inhibitor that is not yet approved by the U.S. Food and Drug Administration. Chorea is an involuntary, irregular

movement, and the cardinal motor feature of Huntington's disease.

Compared with the placebo, valbenazine demonstrated a statistically significant reduction in chorea symptoms and improvement of overall chorea severity in patients with Huntington's disease. Improvement was seen as early as the second week of the study, as participants completed the lowest study dose (40 mg), with consistently greater improvement compared to the placebo in all subsequent visits, as the dose was adjusted in intervals. By the end of the 12-week trial, 82% of valbenazine-treated participants were taking 80 mg.

Notably, KINECT-HD marked the first time the Huntington's Disease Health Index (HD-HI) was implemented in a Phase III trial. HD-HI is a patient-reported outcome measure designed to evaluate clinically meaningful changes in Huntington's disease function in response to therapeutic interventions. Patients who received valbenazine reported improved mobility and hand/arm function, as well as decreased burden from abnormal movements, compared to patients who received the placebo.

"We are incredibly grateful to the participants and care partners for their dedication to this study," Furr Stimming said.

In December 2022, biopharmaceutical company Neurocrine Biosciences submitted a supplemental New Drug Application to the FDA for valbenazine as a treatment for chorea associated with Huntington's disease. The organization is expected to respond to the submission by August 20, 2023. The results from this study were included in the filing.

Huntington's disease is a rare, inherited disease that typically begins in a person's 30s or 40s, causing nerve cells in the brain to break down over time. About 40,000 people living in the U.S. have the fatal disease, while another 200,000 are at risk for inheriting the disease. No cure exists, but

medications and physical, speech, and occupational therapy can help manage symptoms.

More information: Erin Furr Stimming et al, Safety and efficacy of valbenazine for the treatment of chorea associated with Huntington's disease (KINECT-HD): a phase 3, randomised, double-blind, placebo-controlled trial, *The Lancet Neurology* (2023). DOI: 10.1016/S1474-4422(23)00127-8

Beatrice Heim et al, Valbenazine as treatment for Huntington's disease chorea, *The Lancet Neurology* (2023). DOI: 10.1016/S1474-4422(23)00163-1 , www.thelancet.com/journals/lan … (23)00163-1/fulltext

Provided by University of Texas Health Science Center at Houston