Chorea in Huntington Disease

Overview

Chorea in HD

Many people with HD experience chorea, a troublesome involuntary movement disorder.[1] HD is a hereditary progressive neurodegenerative disorder in which neurons within the brain break down. HD causes movement, cognitive, and psychiatric disorders with a wide range of signs and symptoms.[1-3]

~90%

of adults **with HD** experience **chorea**[4]

People with chorea develop abnormal, abrupt, or irregular movements that can involve various parts of the body.[1]

Chorea can interfere with speech, swallowing, posture, and gait.[1,2] Chorea can make it difficult to perform many activities of daily living, including, speaking, eating, or taking a shower.[2,5]

According to a survey of 115 participants, some people have reported facing a social stigma; both physical and psychological aspects of chorea symptoms may make them experience low confidence and restrict them from participating in social or leisure activities.[6]*

HD Fast Facts

There are **~41,000** HD symptomatic Americans.[7]

Over 200,000 people are at-risk for inheriting HD.[7]**

HD symptoms generally appear between **ages 30-50.**[7,8]

HD symptoms **typically worsen** over a **10- to 25-year** period.[7]

* A survey was provided to HD participants and/or their caregivers via PatientsLikeMe.com (9 February 2017-22 March 2017), comprising multiple-choice and open-ended questions designed to assess how chorea impacts HRQoL and overall functioning, and the importance of treating chorea. The HDQLIFE measurement system was used to evaluate patient-reported outcomes of chorea and compare Anxiety and Stigma scores in participants with high chorea versus those with low chorea [HDQLIFE Chorea scores ≥ 60 (n = 45) vs. < 60 (n = 38)]. A total of 115 participants (n = 35 caregivers; n = 80 individuals with HD) were included in this study.

**At-risk refers to a person whose mother or father has HD or has inherited the HD gene and who therefore has a 50-50 chance of inheriting the disorder.

Valbenazine

An Investigational Treatment for Chorea in Huntington Disease

Valbenazine is investigational and not approved for use in HD in any country

Status: Ongoing Phase 3 open-label study

At Neurocrine Biosciences, we are studying valbenazine as an investigational treatment for patients with chorea associated with HD.

Valbenazine is a selective vesicular monoamine transporter 2 (VMAT2) inhibitor being investigated for the potential treatment of chorea in HD. It is believed to lead to reversible reduction of dopamine release by selectively inhibiting VMAT2, a transporter that plays a key role in dopamine signaling. High levels of dopamine are thought to be associated with HD.

We have designed this novel compound to provide sustained plasma and brain concentrations of the active drug to allow for once-daily dosing.

Clinical Studies

KINECT-HD data analysis and preparation for supplemental new drug application (sNDA) is in progress.

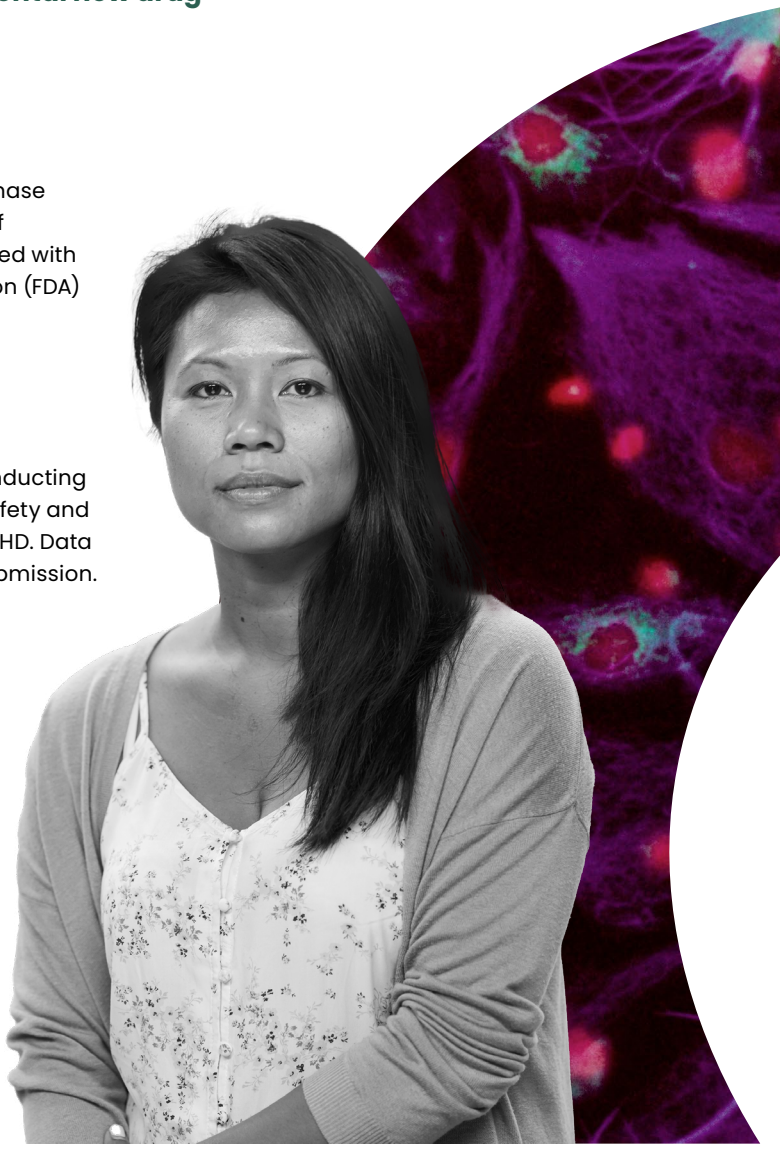
KINECT HD

In December 2021, we announced top-line data from our KINECT-HD Phase 3 registrational study evaluating the efficacy, safety, and tolerability of valbenazine as a once-daily treatment in adults with chorea associated with HD. We plan to submit an sNDA to the U.S. Food and Drug Administration (FDA) in 2022.

KINECT HD2

In collaboration with the Huntington Study Group, we are currently conducting KINECT-HD2, a Phase 3, open-label study to evaluate the long-term safety and tolerability of valbenazine for the treatment of chorea in patients with HD. Data from the KINECT-HD2 open-label study will be included in the sNDA submission.

For more information about the KINECT-HD2 Phase 3, open-label study, please visit **HuntingtonStudyGroup.org** or **ClinicalTrials.gov**.



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