Reach2HD, a Phase II study in Huntington's disease, launched

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The Huntington Study Group (HSG), under the leadership of Ray Dorsey, M.D. with Johns Hopkins Medical and Diana Rosas, M.D. with Massachusetts General Hospital, is conducting a clinical trial in Huntington's disease (HD) throughout the United States and Australia, "A randomized, double-blind, placebo-controlled, study to assess the safety and tolerability, and efficacy of PBT2 in patients with early to mid-stage Huntington's disease" comparing a 100 mg dose or 250 mg dose versus placebo. The HSG is a not-for-profit group of physicians and other clinical researchers who are experienced in the care of HD patients and dedicated to clinical research of the disease. This trial is sponsored by Prana Biotechnology Limited (Melbourne, Australia) and is being managed by the University of Rochester Medical Center.

Huntington's disease is an inherited neurodegenerative disease which affects over 30,000 people in both the United States and Australia. HD is characterized by brain cell death that usually begins between the ages of 30 to 50, and results in motor, cognitive and behavioral signs and symptoms. While there are medications to help relieve some of the disease symptoms, there is no known treatment to address the cognitive impairment associated with HD.

Research has shown that normally occurring metals in the brain play a significant role in diseases such as Alzheimer's disease and more recently, HD. Researchers at Prana Biotechnology are identifying drugs designed to interrupt interactions between these biological metals and target proteins in the brain, to prevent deterioration of brain cells. One

of the chemical compounds, called PBT2, has shown in animal models, and as well as in a small group of patients with Alzheimer's disease, that it may improve cognition. There is some indication in animal models of HD, that the drug may improve motor function and control, increase life span and reduce the amount of brain cell degeneration. Based on these results, Prana is investigating whether the drug will have similar effects with HD patients.

Reach2HD will evaluate how safe and well tolerated PBT2 is at a dose of 100 mg or 250 mg a day compared to a placebo over six months. The trial will also measure whether there is an effect on cognitive abilities as well as other HD symptoms including motor and overall functioning of individuals with HD.

"We are excited to work with Prana to investigate the safety and tolerability of an interesting and innovative experimental treatment for Huntington's disease, PBT2," said Dorsey. "We have few treatment options for Huntington disease, and none for cognition. We hope this is a step to addressing this large unmet need for patients and their families."

Provided by University of Rochester Medical Center