


New study offers hope for Huntington's Disease patients

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UofL is study site for Phase 2 trial with novel treatment that may slow disease progression

Individuals in the early stages of Huntington's Disease (HD) or who are at risk of developing it may be able to play a part in efforts to conquer the disease. Patients are invited to apply for participation in SIGNAL, a Phase 2 research trial that will assess the safety, tolerability and effectiveness of VX15, a novel monoclonal antibody that may delay onset or slow the progression of HD.

Kathrin LaFaver, M.D., Raymond Lee Leiby Chair for Parkinson's Disease Research in the Department of Neurology at the University of Louisville, will lead the study in Louisville, one of 23 sites around the United States participating in SIGNAL. LaFaver also is the director of the Parkinson's and Movement Disorders Clinic at UofL Physicians.

Animal models have shown that monoclonal antibodies bind to and block a molecule that may cause inflammation in the brain of individuals who develop HD. In addition, VX15 may protect against the inflammation that has been shown to affect the thinking, movement and behaviors that affect HD patients.

Huntington's Disease is a genetic disorder that causes the progressive breakdown of nerve cells in the brain. It is characterized by personality changes, mood swings, depression, forgetfulness and impaired judgment. Patients experience unsteady gait and involuntary movements (chorea), slurred speech, difficulty in thinking and mood disturbances. HD affects approximately 30,000 Americans and more than 200,000 have the gene that causes the disease. HD is autosomal dominant, meaning that a parent with Huntington's Disease has a 50/50 chance of passing the gene trait that causes the disease on to his or her children.

The SIGNAL trial is the first time a monoclonal antibody will be investigated for potential treatment of HD. Participants in the trial will receive monthly intravenous infusion of the drug and be monitored with advanced brain scan techniques and analyses utilizing MRI and PET.

"This is a great opportunity for patients in early stages of Huntington's to be involved in a study that may slow the progression of the disease," LaFaver said. "The drug was already tested for safety in patients with multiple sclerosis and was well tolerated."

Trial participants should be individuals who:

- Are at risk for developing HD
- Have undergone genetic testing
- Are thought to be in the early stage of HD
- Are able to undergo brain scans (MRI and PET)
- Are at least 21 years of age

SIGNAL will enroll study participants through the second part of 2016. Participants in the study will receive monthly infusions for 12 months and follow up for an additional three months. Participants will receive study related medical care, tests and drugs used in the study, along with reimbursement for time spent

during in-person visits and reasonable travel and lodging costs.

For information on participating in SIGNAL, contact Annette Robinson, RN, BSN, CCRC at 502-540-3585, annette.robinson@louisville.edu.

Individuals also may contact the Huntington Study Group at 1-800-487-7671, email info@hsglimited.org or <http://www.huntington-study-group.org>.

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