

The ACR16 trial is enrolling participants

A Phase IIB clinical trial of the dopamine stabilizer ACR16 is now being conducted by the Huntington Study Group under the direction of lead investigator Karl Kieburtz, M.D. Approximately twenty sites are now accepting volunteer participants with more sites expected to come on board shortly.

“I am excited about the study because we are testing a drug which could improve motor function in Huntington’s Disease by a mechanism we’ve never studied before. All the other drugs used in the past for movement symptoms have blocked dopamine and this is about improving how dopamine works,” Dr. Kieburtz said.

ACR 16 is owned by the NeuroSearch pharmaceutical company. There were good results in a small Phase II trial in Europe; 28 days of treatment with ACR16 resulted in a statistically significant improvement in the patients’ voluntary movements including parkinsonism and gait function. A Phase III European trial started enrolling patients in April 2008 and has been proceeding smoothly with about half the 420 participants enrolled so far. The good news is that the researchers report no safety issues. American and European researchers will be sharing safety data.

The European trial is a Phase III trial. The trial in the U.S. and Canada is called a Phase IIB trial. The North American study is a smaller (220 participants) and shorter study (13 weeks) with three dosages compared to the European one which is studying two dosages for six months.

The study still has good power so it’s possible that if the drug is effective, the results will be statistically significant and the two studies could be used together to apply for FDA approval. If on the other hand, there are indications of effectiveness at only one or two dose levels but the results do not reach statistical significance, a larger Phase III trial can be conducted with the most promising dose.

The primary measure of effectiveness for both the American and European trials is the effect of ACR16 on Huntington patients’ voluntary motor functions (such as gait/balance, hand functionality and parkinsonism) as measured by the modified Motor Score, mMS - a subscale of the Unified Huntington’s Disease Rating Scale (UHDRS). In addition, researchers will also look at all of the measures in the UHDRS including cognitive function and the severity of neuropsychiatric symptoms such as depression and anxiety as well as recording their overall clinical impression of the patients.

Dr. Kieburtz reports that enrollment has gotten off to a slow start because of the recent holiday season but he hopes to see more people volunteering soon, not only because this drug might be a treatment for HD symptoms but because of the growing number of clinical trials being conducted and proposed.

“I hope that we as a community can show that we can evaluate more than one potential treatment at a time. I like that we have a menu for participation in research from

observational trials to shorter and longer clinical trials. There's a range of possibilities to choose from but we need more volunteers to get the job done."

A list of participating sites can be found here: <http://www.huntington-study-group.org/Portals/0/HARTSiteList.pdf>

- *Marsha L. Miller, Ph.D., February 3, 2009*