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Roche announces U.S., Canada sites for Phase 3 clinical trial of Huntington's disease drug

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At Risk for Huntington's Disease

HD is a genetically caused brain disorder that causes uncontrollable bodily movements and robs people's ability to walk, talk, eat, and think. The final result is a slow, ugly death. Children of parents with HD have a 50-50 chance of inheriting the disease. There is no cure or treatment.

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THURSDAY, DECEMBER 20, 2018

Roche announces U.S., Canada sites for Phase 3 clinical trial of Huntington's disease drug

Pharmaceutical firm Roche has announced 26 planned sites in the U.S. and Canada for its historic Phase 3 clinical trial of a gene-silencing drug to slow, halt, or perhaps even reverse the progression of Huntington's disease.

Called GENERATION HD1, the greatly anticipated trial will test the efficacy of the drug, RG6042. Roche expects to start enrolling volunteers in early 2019.

The announcement comes one year after the successful completion of the Phase 1/2a trial to measure the safety and tolerability of RG6042, developed by Ionis Pharmaceuticals, Inc.

RG6042 dramatically reduced the amount of mutant huntingtin protein in the cerebrospinal fluid (CSF) of trial participants. As a result, Roche took the unusual step of skipping a Phase 2 trial (testing efficacy for the first time) and going directly to a Phase 3 (confirming efficacy in hundreds of participants, or more).

In a statement released December 19 to the Huntington's Disease Society of America (HDSA) and other patient groups, Roche announced the sites listed below, grouped by province/state.

Canada

Alberta, Edmonton – University of Alberta
British Columbia, Vancouver – University of British Columbia
Ontario, Ottawa – Ottawa Hospital
Ontario, Toronto – Centre for Movement Disorders
Nova Scotia, Halifax – Queen Elizabeth II Health Sciences Centre
Quebec, Montreal – Centre Hospitalier de l'Université de Montréal

U.S.

Alabama, Birmingham – University of Alabama
Arizona, Phoenix – Barrow Neurological Clinic
California, Davis – University of California, Davis
California, Palo Alto – Stanford University
California, Pasadena – Arcadia Neurology Center
California, San Diego – University of California, San Diego
Colorado, Englewood – Rocky Mountain Movement Disorders Center
District of Columbia, Washington – Georgetown University
Florida, Tampa – University of South Florida
Illinois, Evanston – Northwestern University
Maryland, Baltimore – Johns Hopkins University
Massachusetts, Boston – Beth Israel Deaconess Medical Center
Missouri, St. Louis – Washington University in St. Louis

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New York, New York – Columbia University
Pennsylvania, Pittsburgh – University of Pittsburgh Medical Center
Tennessee, Nashville – Vanderbilt University Medical Center
Texas, Houston – University of Texas Health Science Center
Utah, Salt Lake City – University of Utah
Washington, Kirkland – Evergreen Health

Roche plans to announce sites in approximately 13 additional countries in the coming months. It hopes to enroll a global total of 660 volunteers with early HD symptoms at 80 to 90 sites. Each participant will receive the drug or placebo monthly over 25 months through a lumbar puncture (spinal tap), the way into the CSF.

The CSF bathes the brain. Because biopsies of the brain are currently not possible, measuring the effect of the drug in the CSF gives researchers a window onto the effects of the drug.

Moving as 'quickly as possible'

"It is important to note that these sites are not fully activated nor recruiting yet," the Roche announcement stated. "We hope to complete the final steps as quickly as possible."

According to the statement, Roche selected sites based on a variety of factors, including prior experience with HD studies, clinic infrastructure capacity, ability to run the study as quickly and completely as possible, patient population, and geographic location.

The news follows Roche's announcement last month of 16 sites for the HD Natural History Study, an arm of the RG6042 program to involve 100 observational study volunteers in Canada, Germany, the United Kingdom, and the United States.

The HD Natural History Study will seek to deepen understanding of the natural progression of HD, the role of the mutant huntingtin protein in the disorder, and the assessment of biomarkers (signs of the disease measured in patients) and their efficacy in predicting the effects of the drug. The volunteers will undergo four lumbar punctures to examine their CSF, but receive no drug.

Click here for the full text of Roche's December 19 statement.

In the U.S. and Canada, HD families can contact Roche/Genentech about the trial at 888-662-6728. Information about the trial is also available at ClinicalTrials.gov.

For additional background on GENERATION HD1, click here.

If effective, RG6042 would be the first treatment to affect the progression of Huntington's.

Stay tuned to this blog for future updates on GENERATION HD1.

Posted by Gene Veritas at 7:03 AM

Labels: biomarkers , brain , cerebral spinal fluid , clinical trial sites , GENERATION HD1 , HD Natural History Study , Huntington's disease , Ionis , lumbar puncture , mutant huntingtin , RG6042 , Roche , treatments

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