www.hdreach.org
919-803-8128
info@hdreach.org

Ionis Study Provides HOPE for the Huntington's Disease Community, But There is Still Much Work to be Done!

Today, Ionis released the news that the Phase 1/2a Study of Ionis-HTTRx, the first therapy in clinical development designed to target the underlying cause of HD, has been completed.

For an easy to understand explanation of the Ionis study, check out HD Buzz: https://en.hdbuzz.net/249

Dr. Francis Walker MD, Professor of Neurology and Director of the Movement Disorder Clinic at Wake Forest University, weighed in on this exciting and hopeful news.

Exciting HD Scientific Research

The results of the Ionis clinical trial in Huntington's disease are quite exciting from a scientific standpoint. The drug, which is administered into the spinal fluid, does reduce spinal fluid levels of mutant huntingtin. This is a very strong sign that it is doing what the drug was planned to do, which is to lower the production of the mutant protein in brain cells. By doing this, this drug should help slow progression of the disease, and it may also help some sick neurons to clear existing accumulations of mutant protein.

The effect of the drug, however, on actual symptoms of HD and patient function, is not yet known. It appears safe and well tolerated which is a great start for a drug that is administered via a lumbar puncture (spinal tap). The study did not involve enough patients for us to know how effective it was for symptoms, and until the study is published, we will not know if it had effects on other signs of HD, such as slow gradual shrinkage of certain brain areas as seen on MRI. It sounds as if details such as this will not be presented for several months and not published until sometime late next year.

The news reports to do not include important questions that have yet to be answered:

- Does the drug penetrate and help all brain cells, or just those cells near the surface of the brain? Does the lowering of wild type huntingtin (the good, or non-mutated huntingtin protein) cause any unexpected problems?
- Does the lowering of mutant huntingtin in humans have the same beneficial effects in humans as it does in animals?

Further studies are needed and being planned with this drug to answer these important questions.

What else is good news?

There is a race on to find a great treatment for HD. A company called Wave Life Sciences is working on a similar form of treatment for HD that involves injections of medication into the spinal fluid, however,

www.hdreach.org
919-803-8128
info@hdreach.org

their compound should not lower wild-type (good) huntingtin. Studies with this compound should begin early next year and this will involve several sites in the USA, including Wake Forest. This compound will not work in everyone, and a blood test is needed to identify the 60-80% of patients for whom the drug should work. It is hoped we will also be a site for the Ionis drug when it gets to the next set of clinical studies (Phase 2). This may offer an opportunity to patients ineligible for the Wave Life Science drug. In addition, a company called Uniqure, is developing a drug that will be injected into the brain that may work in HD. The Signal trial also is showing some promise and will be re-opening enrollment in 2018. So stay tuned, we anticipate a steady stream of good news over the next few years.

What is next?

Right now, there is nothing new to do if you are a patient or a person who is gene positive, as it will be a while before the next set of studies comes along for the Ionis drug. In the meantime, if you are not part of ENROLL-HD, please sign up, as this will help get you into new studies as they become available, either with the Ionis drug or with other new potentially promising treatments for HD.

For additional information about participating in a clinical trials contact:

Christine O'Neill: coneill@wfubmc.edu or call 336-716-8611
Wake Forest Baptist Medical Center

Peggy Perry-Trice: peggy.perrytrice@duke.edu or call 919-684-0865
Duke Movement disorder Center, Durham, NC

Questions about HD or need help?

HD Reach provides access to healthcare, education and social support for any North Carolina family living with Huntington's disease. If you need help, contact HD Reach for additional help at info@hdreach.org or 919-803-8128.