Grant 2 Research UCL Project May 2019

ADDOR is pleased to announce their second grant in support of the continued fight against Huntington's disease (HD), by donating to a research project being undertaken at University College London (UCL).

Donation to University of London (UCL)

In December 2017 researchers at the UCL and IONIS pharmaceuticals, partnered with Roche, released the top level results on the exciting breakthrough in the fight against Huntington's disease (HD). More detailed information from their announcements can be found on the ADDOR website at addor.co.uk

Summary

For the first time. Using an experimental drug, injected into spinal fluid, the levels of toxic proteins in the brain were safely lowered.

The research team, at UCL said "there is now hope the deadly disease can be stopped".

DNA is the key to all life, with genetic information stored in regions known as genes. Everyone has two copies of each gene. In Huntington's disease an error, known as mutation, effects one of the Huntington's genes. This gene produces a protein called Huntingtin, which plays a key role in maintaining neuronal and brain cells healthy. When the protein is faulty it destroys the cells and leads to devastating mental and physical symptoms with sufferers requiring 24 hour care and often passing away before 50. The IONIS-HTTRx treatment is through a process called 'Gene silencing' where the drug can specifically target the faulty Huntington's gene, stopping the production of the faulty protein and correcting the disease.

Prof Sarah Tabrizi, the lead researcher and director of the Huntington's Disease Centre at UCL, said "For the first time we have the potential, we have the hope, of a therapy that one day may slow or prevent Huntington's disease. This is of ground breaking importance for patients and families."

Doctors are not calling this a cure. They still need vital long-term data to show whether lowering levels of huntingtin will change the course of the disease.

In April 2019 the full details of the trial were presented to scientists and the exciting results of HDF-Supported Research were published in the New England Journal of Medicine.

The President of the Hereditary Disease Foundation, Nancy Wexler exclaimed "we are ecstatic by this very encouraging news!"

The first human trial, which uses a novel approach to lower the level of the devastating huntingtin protein in the nervous system, is a success. The drug targeting the cause of Huntington's was safe and well tolerated.

Nancy Wexler said “we are excited that we will now be moving forward to a global Phase 3 trial sponsored by F.Hoffman-LaRoche, which licensed the medicine. HDF has supported this promising therapy for the past decade”.

Full information regarding the published articles are available on the following links and through the ADDOR website.

Read the article online or click here for the PDF.

Read the editorial online or click here for the PDF.

View supplementary appendix online or click here for the PDF

ADDOR is supporting this research effort by providing a donation of £500 and plan to make further donations.

If you would like to help in the fight to defeat HD, fund research and help HD sufferers.

Please donate at addor.co.uk