

The London Ataxia Centre is recruiting participants for the Vico Therapeutics trial of the therapy VO659 in SCA1 and SCA3

In April 2023, Vico Therapeutics - a company based in the Netherlands which is developing therapies for severe neurological conditions - announced that they dosed the first person in their phase 1/2a clinical trial evaluating a drug they developed called VO659 for the treatment of SCA1, SCA3 and Huntington's Disease (HD) from one of their sites in Europe. VO659 is a type of therapy called an antisense oligonucleotide (ASO). ASOs are small pieces of genetic material that are being developed to treat a number of genetic conditions. They act by targeting mutated genes and influencing their ability to produce proteins.

In this case the ASO targets the mutated gene causing SCA1, SCA3 or HD, to prevent it from producing the protein which ultimately causes the condition. These three conditions are all caused by the increase in the length of a specific sequence called 'CAG repeats', which results in the production of toxic proteins that damage cells. VO659 targets these additional CAG repeats and stops them from resulting in the production of toxic protein aggregates.

Vico Therapeutics are now recruiting a small number of participants through the London Ataxia Centre for the phase 1/2a trial, which aims to investigate the safety and tolerability of four doses of VO659 in individuals with mild to moderate SCA1 and SCA3. There are a number of criteria in order to be able to participate in the trial, such as being 25 – 60 years old and having a genetically-confirmed diagnosis of SCA1 or SCA3 which is mild to moderate.

The trial is set to last for up to 42 weeks, with a 6-week screening period, then the study drug will be administered into the fluid surrounding the brain and spinal cord (cerebrospinal fluid) four times within a 13-week dosing period, followed by a 23-week post-dosing period. During the dosing period, cerebrospinal fluid and blood samples will be taken to assess safety, how the body interacts with the drug and the effects of the drug on the body at specific time points.

If you or someone you know is diagnosed with SCA1 or SCA3 and is interested in taking part in this trial, contact the London Ataxia Centre by emailing uclh.ataxia@nhs.net or calling 02034 483100.

To read more information about the trial, click [here](#) and [here](#).