

Experimental Drug Fights Huntington's Disease

The first human trial of an experimental drug that was designed to fight Huntington's has delivered success beyond what researchers had realistically hoped for.

Results from the clinical trial which involved 46 patients with early Huntington's showed that an injectable drug known as IONIS-HTTRx successfully decreased levels of the harmful Huntingtin protein in the participants' nervous system.

Sarah Tabrizi, a neurologist from University College London's Huntington's Disease Centre says that "the results of this trial are of ground-breaking importance for Huntington's disease patients and families. For the first time a drug has lowered the level of the toxic disease-causing protein in the nervous system, and the drug was safe and well-tolerated."

he drug, which is injected into the spinal fluid in order to reach the brain, has been in development for over a decade, but until now researchers weren't exactly sure how it would impact the toxic Huntingtin protein in humans.

Huntington's disease is a progressive brain disorder that causes uncontrolled movements, cognitive impairment, emotional problems, and an early death in most people who have the condition. It is caused by a mutation in the Huntingtin gene, which produces the Huntingtin protein - but the genetic variant ends up producing abnormally long versions of this protein, which divide into toxic fragments that can bind to and impede neurons.

Over the course of the trial, the participants who received increasing doses of IONIS-HTTRx exhibited lower levels of Huntingtin protein concentration in their spinal fluid. This is the first time that the toxic protein has been successfully decreased in patients' nervous systems, signaling what could be the start of the first working treatment for Huntington's.

However, as promising as these results are, it's still early days yet. The researchers intend to present and publish their findings next year, but the bigger question remains whether IONIS-HTTRx can also mitigate the symptoms of Huntington's disease, which hasn't been assessed yet. There's a lot on the line here. If future clinical trials deliver signs as promising as these early results, the IONIS-HTTRx injections might one day be able to prevent people with Huntington's from getting ill or showing symptoms of the disease.

Beyond Huntington's, researchers believe that this early success of IONIS-HTTRx shows just how powerful gene-silencing medicine has become, meaning we're closer than ever to potential treatments for other genetic conditions, such as Parkinson's or Alzheimer's. Philippa Brice, a molecular biologist who wasn't involved in the study, says that "this is a potential game-changer, not only for Huntington's disease patients but also for genomic medicine in general. Though more work needs to be done, if gene silencing lives up to this promise we could be on the brink of some of the personalized treatments that patients with severe genetic diseases need so badly."

Source: sciencealert

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