New hope for Huntington's sufferers

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A major breakthrough in the understanding and potential treatment of Huntington's disease has been made by scientists at the University of Leeds.

Researchers in the University's Faculty of Biological Sciences have discovered that one of the body's naturally occurring proteins is preventing 57 genes from operating normally in the brains of Huntington's sufferers. In addition, the destructive nature of this protein could potentially be halted using drugs that are already being used to help cancer patients.

"This is a really exciting breakthrough," says researcher Dr Lezanne Ooi. "It's early days, but we believe our research could lead to radical changes in treatment for Huntington's sufferers. The fact that these cancer drugs have already been through the clinical trials process should speed up the time it takes for this research to impact directly on patients."

Huntington's is an inherited degenerative neurological disease that affects between 6500 and 8000 people in the UK and up to 8 people out of every 100,000 in Western countries. Any person whose parent has Huntington's has a 50-50 chance of inheriting the faulty gene that causes it and everyone with the defective gene will, at some point, develop the disease.

It is characterised by a loss of neurons in certain regions of the brain and progressively affects a sufferer's cognition, personality and motor skills. In its later stages, sufferers almost certainly require continual nursing

care. Secondary diseases, such as pneumonia are the actual cause of death, rather than the disease itself.

Dr Ooi's research has identified the effects of one of the body's proteins on the neurons of Huntington's sufferers. Neurons are usually protected by the protein BDNF (brain derived neurotrophic factor), whose many functions also include encouraging the growth and differentiation of new neurons and synapses. However, in Huntington's sufferers, the repressor protein known as REST - which is usually found only in certain regions of the brain – enters the nucleus of the neuron and decreases the expression of BDNF.

She has also been studying some of the enzymes which assist the function of this protein. It is these enzymes that provide the mechanism for the protein to wreak havoc in the brains of Huntington's sufferers, and that are already being targeted in certain cancer drugs.

Currently, the symptoms of Huntington's can be managed through medication to help with loss of motor control and speech therapy but there is no definitive treatment. This research provides a first step in developing a treatment regime that may halt the onset of the disease.

"Huntington's is a devastating illness that affects whole families. Those who know they've inherited the faulty gene live in a shadow of uncertainty over how long their symptoms start to develop. It can also be particularly cruel since every child born to a parent that has the HD gene is at 50% risk of having inherited the gene," says Cath Stanley, Head of Care Services at the Huntington's Disease Association.

"As such, any developments in the understanding of this disease are welcome, but this breakthrough is particularly exciting as it opens up an avenue for researching a possible treatment using drugs that are already available, rather than starting from scratch."

Source: University of Leeds