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Stem cell discovery gives insight into motor neurone disease

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A discovery using stem cells from a patient with motor neurone disease could help research into treatments for the condition. The study used a patient's skin cells to create motor neurons - nerve cells that control muscle activity - and the cells that support them called astrocytes.

Researchers studied these two types of cells in the laboratory. They found that a protein expressed by abnormalities in a gene linked to motor neurone disease, which is called TDP-43, caused the astrocytes to die.

The study, led by the University of Edinburgh and funded by the Motor Neurone Disease Association, provides fresh insight into the mechanisms involved in the disease.

Although TDP-43 mutations are a rare cause of motor neurone disease (MND), scientists are especially interested in the gene because in the vast majority of MND patients, TDP-43 protein (made by the TDP-43 gene) forms pathological clumps inside motor neurons.

Motor neurons die in MND leading to paralysis and early death.

This study shows for the first time that abnormal TDP-43 protein causes death of astrocytes. The researchers, however, found that the damaged astrocytes were not directly toxic to motor neurons.

Better understanding the role of astrocytes could help to inform research

into treatments for MND.

Professor Siddharthan Chandran, of the University of Edinburgh, said: "Motor neurone disease is a devastating and ultimately fatal condition, for which there is no cure or effective treatment. It is not just a question of looking solely at motor neurons, but also the cells that surround them, to understand why motor neurons die. Our aim is to find ways to slow down progression of this devastating disease and ultimately develop a cure."

These findings, published in the journal Proceedings of the National Academy of Sciences., are significant as they show that different mechanisms are at work in different types of MND.

The research, led by the University of Edinburgh's Euan MacDonald Centre for Motor Neurone Research, was carried out in collaboration with King's College, London, Columbia University in New York, the University of California and the Gladstone Institutes in San Francisco.

Dr Brian Dickie, the MND Association's Director of Research Development, said: "From a therapeutic perspective this finding is important because it means that specific treatments targeted at astrocytes may only be relevant and effective, in specific subsets of patients who will have to be carefully selected for drug trials."

Dr Steve Finkbeiner, Associate Director of Neurological Research at the Gladstone Institutes, said: "We were delighted to be part of this international team, which brought together critical tools, innovative technologies and complementary expertise to do something that no single group could. We are hopeful that the creation of human models of ALS will deepen our understanding of the disease in ways that will help us ultimately find relevant therapies for patients."

Provided by University of Edinburgh