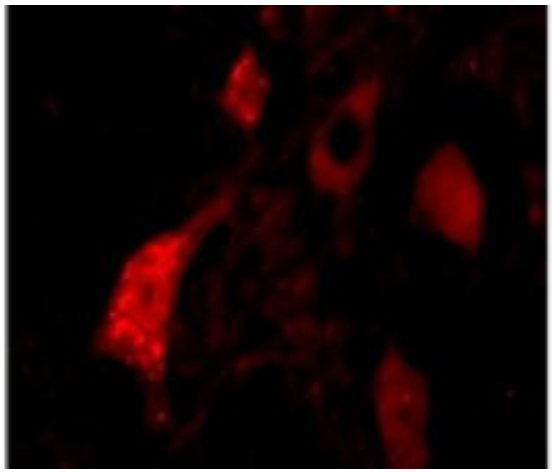
New therapy offers hope to spinal muscular atrophy patients

9 June 2010



This image shows the results of a new therapy technique for Spinal Muscular Atrophy patients developed by academics at the University of Sheffield. The study has led to the restoration of the SMN protein in motor neurons, as shown in this image. Credit: Professor Mimoun Azzouz, from the University of Sheffield's Department of Neuroscience

Children who suffer from the devastating disease Spinal Muscular Atrophy are set to benefit from a new breakthrough in therapy developments by researchers at the University of Sheffield.

The research, which was published in *Science Translational Medicine*, has shown that a novel gene transfer system has the potential to provide an effective therapeutic treatment for SMA patients.

SMA is a devastating motor neuron disease which affects children. It is caused by an abnormal survival motor neuron (SMN) gene, which leads to a reduction of SMN protein levels. The disease is currently incurable, and patients often require prolonged medical care as no effective treatments to alleviate the condition, currently exist.

The research team at Sheffield, led by Professor Mimoun Azzouz, tested whether a novel gene replacement therapy through a single injection was the most efficient way to treat the disease in a group of mice. They found that the injection, which expressed the SMN gene, successfully restored the SMN protein to normal levels and alleviated symptoms in the SMA model.

The new technique has the potential to develop a simple injection, without any requirement for risky and costly surgical interventions, and has achieved the highest therapeutic effects reported in the field to date. This has significant implications for the future treatment of SMA.

Professor Mimoun Azzouz, from the University's Department of Neuroscience, said: "I am delighted by the outcome of several years of efforts to tackle this devastating disease. These results bring us one step closer to a successful gene therapy treatment for patients with SMA."

More information: The paper, entitled ‘Systemic delivery of scAAV9 expressing SMN prolongs survival in a model of spinal muscular atrophy’ was published in Volume 2, Issue 34 of Science Translational Medicine: Chiara Valori, Ke Ning, Matthew Wyles, Richard J Mead, Andrew J Grierson, Pamela J Shaw, Mimoun Azzouz.

Provided by University of Sheffield