Amyotrophic lateral sclerosis (ALS; also known as Lou Gehrig’s disease) is an incurable adult neurodegenerative disorder that progresses to paralysis and death. Genetic mutations are the cause of disease in 5% of patients with ALS.

Of immense interest, Hongxia Zhou, Xu-Gang Xia, and colleagues, at Thomas Jefferson University, Philadelphia, now show that progressive neuron degeneration can be halted in a rat model of familial ALS linked to mutations in the gene that carries the instructions for making the protein TDP-43.

Progressive motor neuron degeneration was stopped when expression of the ALS-associated mutant human TDP-43 was switched off. If expression of the mutant protein was switched off before many motor neurons had degenerated, the rats recovered function.

Conversely, if expression was switched off after most motor neurons had degenerated, functional recovery was minimal. These data indicate that mutant TDP-43 in motor neurons is sufficient to promote the onset and progression of ALS and that progression of motor neuron degeneration (and thereby progression of disease) is partially reversible in the rat model.

More information: Mutant TDP-43 in motor neurons promotes the onset and progression of ALS in rats J Clin Invest. doi:10.1172/JCI59130

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