Drug could slow motor neuron disease

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Dr Woodruff ... the drug could be trialled in patients in 2019. Credit: University of Queensland

A drug with the potential to delay the progression of motor neuron disease (MND) could be in human trials within three years.

University of Queensland researchers have shown the anti-inflammatory drug PMX205 is effective in animals with the disease, delaying the progression of symptoms and extending survival.

Associate Professor Trent Woodruff said a private company would undertake formal pre-clinical safety trials with the drug.

"As long as the results from the safety studies are positive, the drug could be ready to be trialled in patients in 2019," Dr Woodruff said.

In the meantime, his laboratory at UQ's School of Biomedical Sciences will test the drug in a range of motor neuron disease models.

"To date we have only tested the drug in one model based on the inherited form of MND, but we believe the same inflammatory pathway is likely to be active in all forms of MND," Dr Woodruff said.

"Our next project will focus on sporadic MND, which accounts for 90 per cent of patients."

There is no known cure for MND, a terminal disease with an average life expectancy of two and a half years.

People with MND progressively lose the use of their limbs and ability to speak, swallow and breathe.

UQ's Dr John Lee, a Motor Neuron Disease Research Institute postdoctoral fellow who conducted the research, said PMX205 could help manage patient symptoms to improve quality and length of life.

"At the moment, the only drug available for patients prolongs survival by two to three months at most," Dr Lee said.

"In animal models, PMX205 made a visible difference to tremors, muscle strength and mobility, and if this is reflected in people, it could make a real difference to patients."

The drug has obtained "orphan drug" approval from United States and European licencing authorities, which allows for accelerated progression to human trials.

More information: John D Lee et al. Pharmacological inhibition of complement C5a-C5areceptor signalling ameliorates disease pathology in the hSOD1mouse model of amyotrophic lateral sclerosis, *British Journal of Pharmacology* (2017). DOI: 10.1111/bph.13730