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VICTORIA

Hope for MND sufferers as new drugs show promise in slowing the damage the disease does

Researchers at Melbourne's Walter and Eliza Hall Institute are developing a new class of treatments to dramatically slow the damage caused by motor neurone disease.

Grant McArthur, Herald Sun

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Melbourne researchers have made a major breakthrough in the fight against motor neurone disease, with the development of a new class of treatments able to block the killer process.

The latest discovery has raised scientists' hopes of eventually providing an extra 10 years of life for people diagnosed.

After discovering the way MND triggers inflammation and strikes its victims, a team led by the Walter and Eliza Hall Institute is leading the development of a new class of drugs showing promise in dramatically slowing the damage caused by MND.

The breakthrough may also help those with Parkinson's disease or some of the most common forms of dementia, if results from Melbourne laboratories can be transferred to patients during clinical trials in the coming years.

While development of a potential treatment is still in its early stages, lead researchers Assoc Prof Seth Masters and Dr Alan Yu said uncovering the pathways triggering the damage could eventually add a decade or more to the lives of people diagnosed with neurodegenerative disorders.

"Although it is being done in a laboratory it is a strong indication that this will translate to people later on," Assoc Prof Masters said.

Melbourne researchers discovered the way MND triggers inflammation and strikes its victims. Picture: AAP

"We are optimistic that there might be some good ways to target this and have beneficial effects on patients in the longer run.

“It is not going to stop people getting sick, but we think it is going to afford them a lot longer and healthier lives once the disease has started.”

About one in 10,000 Australians will be struck by MND, with the average life expectancy just two years from diagnosis.

A misfiring immune response causes inflammation which destroys motor neurons, the nerve cells controlling the muscles needed to move, speak, swallow and breathe.

But, after using samples from MND patients to derive stem cells, the Melbourne team were able to grow motor neurons perfectly matching the patients’ own so they could track the progress of the disease and identify the pathways driving the damage.

In partnership with colleagues from the University of Melbourne and Hudson Institute, the WEHI scientists identified an immune sensor called STING was triggering the inflammation.

In results published on Wednesday in the leading journal Cell, the researchers were then able designed specific compounds to attach to block STING, preventing it switching on inflammation and keeping motor neurons alive for far longer.

Animal studies have indicated the drugs could add up to 30-40 per cent to life expectancy, but would only have an effect after diagnosis, which could mean more than a decade in MND patients.

Because other neurodegenerative disorders such Frontotemporal Dementia and Parkinson’s disease are also fuelled by inflammation, development of the new class of treatments could have far wider benefits.

However, Assoc Prof Masters warned there were huge hurdles to jump before it would be known if the drugs could be used as an effective treatment in humans.

The most crucial may be overcoming the blood-brain barrier, a protective border of cells that prevents foreign toxins entering the brain where MND and similar degenerative conditions strike.

The researchers are now working with pharmaceutical companies to further develop potential treatments in the hope of human trials in the coming years.

“We really need to work out how to get these drugs some access into the brain,” Assoc Prof Masters said.

“These things we are testing in the lab, they look really good and so there is a lot of hope that those clinical trials should commence in the next year or two. Then we will find out more.”

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