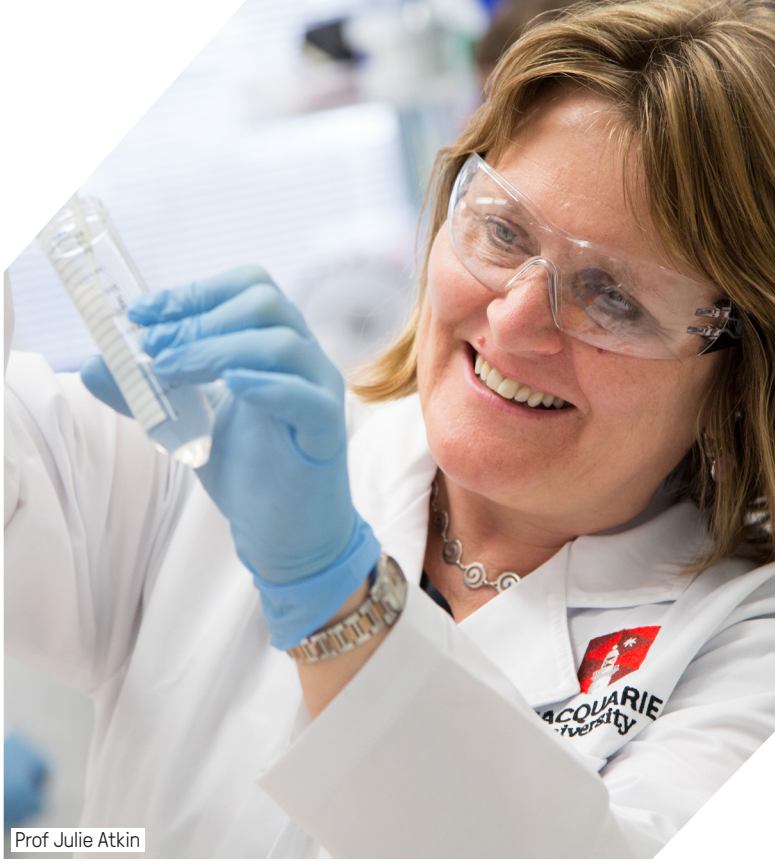
FIGHT MND.


Prof Julie Atkin

Researcher.
PROF JULIE ATKIN

Drug Development Project.
MODULATING ACTIN DYNAMICS IN MND AS A NOVEL THERAPEUTIC APPROACH

Where do you work?

At the Macquarie University Centre for Motor Neuron Disease Research, Faculty of Medicine and Health Sciences, Macquarie University, Sydney. We are one of Australia's largest facilities for MND research.

Can you describe your research experience and background?

I am a cell biologist/neuroscientist and I have worked exclusively on MND for the last 17 years. My research involves investigating the basic processes that trigger neurodegeneration in motor neuron cells in MND, and from this, designing new therapeutic approaches. Prior to working in MND, I worked in antibody research and also in the biotechnology industry for several years.

Why did you become an MND researcher?

I really wanted to work on something that had tangible benefits for human health. Neuroscience really fascinated me and MND is a disease for which there is no effective treatment. Hence there is a real need to find new therapeutic strategies for this terrible illness.

Tell us about your most surprising or exciting research finding?

In 2014 we discovered the normal function of C9orf72, the protein that is mutated in the greatest proportion of cases of familial MND (and related disorder frontotemporal dementia). We found that it is normally involved in mediating transport within cells.

What is actin?

Actin is a long, thin protein that is made up of lots of individuals subunits joined end-to-end. It allows a cell to hold its shape and move, and it is part of the motor neuron 'cytoskeleton'. Actin also has important roles in how motor neurons communicate with each other.

How did you recognise that repairing actin may be a way to treat MND?

We have found that surprisingly, actin is abnormal in MND patients and in disease models in the laboratory. Luckily however, there are several already approved drugs available that can restore actin to its normal form again. We will trial these drugs in this project.

What will this funding enable you to achieve for people living with MND?

This funding will make an enormous difference to our work. Trialling new drugs is very expensive and therefore impossible to do without appropriate support. This funding will allow us to determine whether these actin drugs are protective in MND, and if so, if they can be used as a new treatment. Research into drug discovery is particularly difficult to find funding for. Hence, we are very excited about this next stage of our work and grateful to FightMND for this opportunity.

FIGHT MND.

Prof Atkin and team in the laboratory at the Macquarie University Centre for Motor Neuron Disease Research in Sydney.

The Drug Development Project

Cells, including motor neurons in the body have their own type of skeleton, known as a 'cytoskeleton'. A key protein/molecule that makes up the cytoskeleton is called 'actin'. In healthy cells, actin forms long branch-like cellular structures that continuously form and break. By looking at human MND spinal cord and MND models, this research team identified that actin does not function properly in MND, leading to the formation of too many long branches that accumulate in cells and disrupt normal cellular functions.

The team have identified a new class of drugs that can break apart these long branches and restore the balance of actin in cells. In this study, they will test these novel drugs in multiple MND preclinical models and develop new drugs as promising candidates to prevent actin abnormalities. They will determine if these drugs are able to effectively enter the brain and which drug is the best to develop as a potential candidate for treating MND.

OBJECTIVES

- To identify the most effective actin modulating compound and test its ability to alter disease phenotypes in a wide range of preclinical models of MND.

OUTCOMES

- To have a lead 'actin-repair' drug ready to test in a future clinical trial for MND patients in Australia.