

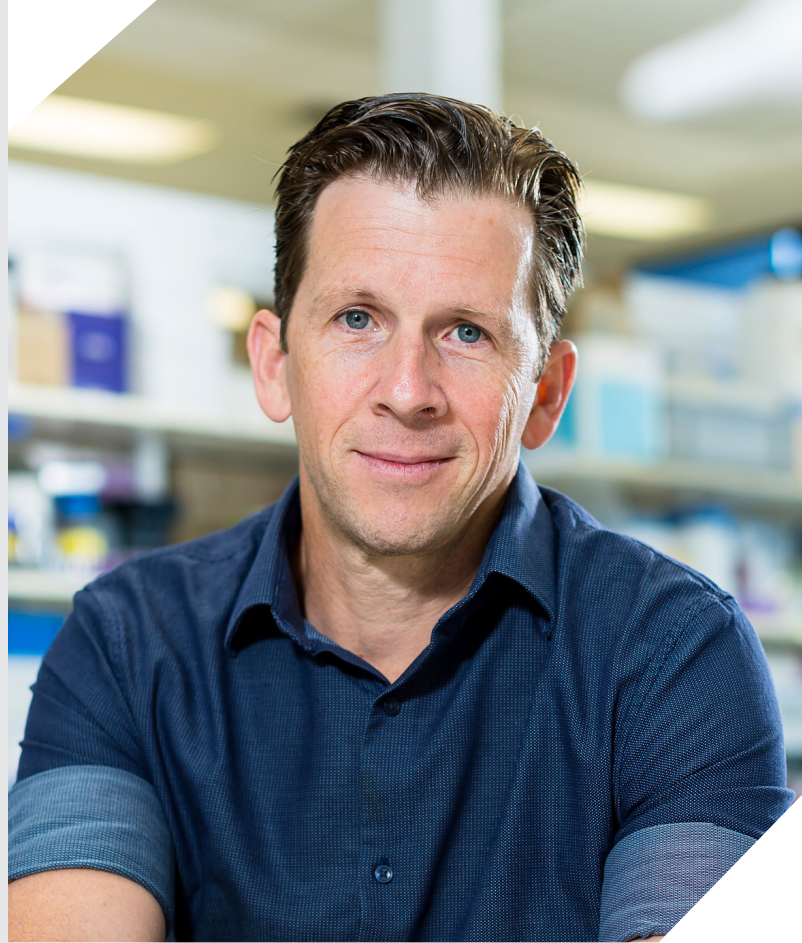
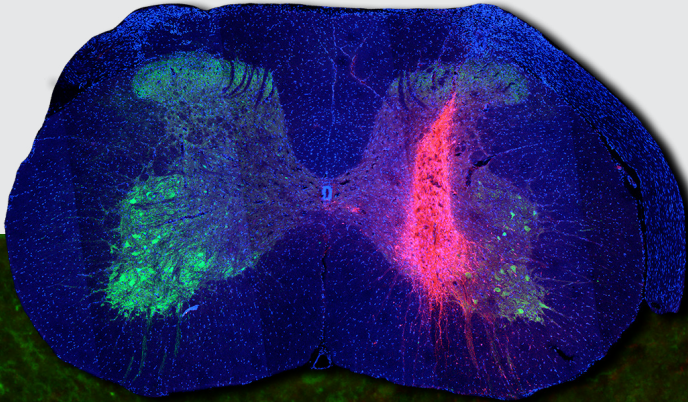
# FIGHT MND.

## Project

### Regenerative medicine

#### Subpial spinal cord delivery as a stem cell-based treatment for MND

Recent clinical trials showed that transplantation of stem cell-derived products into the spinal cord can partially delay disease progression in MND patients. This project will explore a promising new approach to improve stem cell-based therapy for MND. If successful, it will deliver a platform for rapid translation of outcomes to clinical application in patients with MND.



## Project Lead

### A/Prof Lachlan Thompson

The Florey Institute of Neuroscience and Mental Health, The University of Melbourne, VIC

With a career mostly dedicated to developing cell-based therapies for Parkinson's disease (PD), A/Prof Lachlan Thompson has established a "strong foundation of understanding around how neural circuitry can be re-established in the damaged central nervous system (CNS) through transplantation of the correct cell type."

"Stem cells are an exciting resource in the context of CNS repair but we are yet to see their full potential. I'm really optimistic that pre-clinical research aimed at harnessing that potential, by understanding the best cell type and how to deliver them, will lead to promising new clinical trials in patients," A/Prof Thompson says.

## Stem cell research

The focus of A/Prof Thompson's research team is pre-clinical development of stem cell-based therapies, to develop treatments for various neurological conditions, including Parkinson's disease, stroke and MND. The goal of the work at this pre-clinical level *"is to try and identify therapeutic efficacy as a platform to launch clinical trials in patients,"* says A/Prof Thompson.

The project A/Prof Thompson and his team are currently investigating is stem cell therapy for MND.

*"Stem cells are a special cell type in that they are essentially the 'building blocks' for biology. They are responsible not only for the early stages of life in embryonic development, but also for tissue repair later throughout life,"* says A/Prof Thompson.

*"In the laboratory, we can grow these cells in large numbers in order to generate other specific cell types that may be therapeutic in certain settings,"* he adds.

Stem cells and some of the immature 'progenitor' cells they can generate can release molecules that can be therapeutic.

A/Prof Thompson provides an example: *"in a damaged nervous system, transplantation of stem or progenitor cells can slow the loss of cells affected by damage or disease. There is some pre-clinical evidence that transplantation of these cells in models of MND can protect motor neurons from degenerating,"* he says.

## Deploying knowledge to slow or stop MND

This creates the exciting prospect of translating this knowledge to treatment in patients that would slow or even stop disease progression.

*"The challenge is to identify the best way to deploy this for the greatest chance of success. We are still at the early phase of this research and need to understand fundamental questions such as which exact cell type to use and how best to deliver it,"* says A/Prof Thompson.

## A tremendous impact

A/Prof Thompson says that the funding provided by FightMND *"will have a tremendous impact on the capacity to push forward our aspiration to establish a stem cell therapy for MND."*

*"There is a lot of pre-clinical work to do in this area and the rate-limiting factor is overwhelmingly the ability to support talented research scientists to undertake the work,"* he says.

*"While there is undoubtedly potential there, harnessing and understanding this so that a therapy can be established with predictable and effective outcomes remains the key challenge for this field. Certainly a surmountable one we think,"* says A/Prof Thompson.

*"This funding will allow a postdoctoral scientist that might have otherwise been drawn to another field to remain dedicated to pursuing the establishment of a stem cell therapy for MND. It will undoubtedly move us closer to realising this goal."*

**FightMND has invested \$250,000 in this research.**

## About A/Prof Lachlan Thompson

A/Prof Lachlan Thompson completed his undergraduate studies (BSc) at The University of Melbourne before completing his doctoral degree at Monash University in 2002. He then undertook 5 years of postdoctoral training at Lund University in Sweden between 2003 and 2007 where he developed expertise in the area of neural transplantation and cell-based therapies for repair of the central nervous system.

In 2008 A/Prof Thompson moved back to Melbourne to establish a laboratory in this field at The Florey. It is an important and steadily growing area on the Australian research landscape and A/Prof Thompson's team are aggressively exploring the capacity for stem cells to be utilised as a therapy for MND.