

## 2. PROJECT:

Phase 2 Clinical Trial – A placebo-controlled safety and efficacy of ambroxol in individuals with MND

## PRINCIPAL INVESTIGATOR:

Associate Professor Bradley Turner  
The University of Melbourne, VIC

***“Ambroxol targets multiple key disease pathways implicated in MND, including disruption of connections between motor neurons and muscle which occurs very early in MND.” – Associate Professor Bradley Turner***

In this study researchers will assess the long-term safety and effectiveness of ambroxol in MND patients. Ambroxol is a prescription medication used as a cough suppressant. It is being re-purposed in the Fight against MND because it also restores lipid metabolism, which is dysfunctional in people living with MND. The phase 2 trial aims to enrol 50 Australian patients at 5 sites across Australia in Sydney, Adelaide, Perth and Launceston.

### KEY HIGHLIGHTS:

This trial is a long-term safety and efficacy study of an already-approved drug re-purposed for MND. Preclinical studies that progressed this drug to clinical testing in people with MND were supported by a FightMND Drug Development grant.

### AMOUNT INVESTED BY FIGHTMND IN THIS CLINICAL TRIAL:

\$2,000,000

### Q&A:

*Why is this important, and how could it benefit patients?*  
We tested ambroxol in multiple animal models of MND across different laboratories, showing a consistent benefit in motor function, muscle strength and motor neuron protection. This consistent effect across independent research teams provided convincing evidence that ambroxol may benefit MND. My team is excited to play a role in advancing a drug candidate from our lab to clinical studies in people living with MND.



Associate Professor Bradley Turner