

3. DISEASE MODELS

PROJECT:

Advanced modelling of upper motor neuron MND pathology using human pluripotent stem cells

PROJECT LEAD:

Professor Clare Parish
The University of Melbourne, VIC



Professor Clare Parish



Co-Investigator Dr Cameron Hunt

MND is complex and variable, making it difficult for researchers to develop an effective treatment for the disease. Investigators in this project aim to create a novel disease model using stem cells obtained from people living with MND. They will develop a stem cell-based model that recreates the exact types of motor neurons affected in MND and replicates the environment in the brain harmful to these motor neuron populations. Development of this superior model will lead to advanced preclinical drug screening capabilities and increase the likelihood of identifying promising disease-modifying therapies.

KEY HIGHLIGHTS:

Professor Parish is a first-time recipient of research support from FightMND. This project will use stem cells from people living with MND to establish an advanced disease model that recreates the specific types of motor neurons affected in MND.

AMOUNT INVESTED BY FIGHTMND IN THIS PROJECT:

\$249,956

Q&A:

Why is this important and how will it benefit patients?

Recognising the vast number of treatments that have failed to progress from preclinical animal studies into clinical translation, it is imperative that we develop new models of diseases that better recapitulate the human condition. Human stem cells, derived from patients, provide a novel means to model aspects of disease that are not achievable using non-human models. This is likely to lead to a greater understanding of the disease progression and the cell types involved, and will enable new and better targeted therapies.

“With a long-standing history in working with human stem cells, this is the first time our team has used patient lines to study disease mechanisms in MND.” – Professor Clare Parish