

# FIGHT MND.

## FIGHTMND DRUG SCREENING PROGRAM

### WHAT IS THE FIGHTMND DRUG SCREENING PROGRAM?

The aim of the program is to find potential treatments for Motor Neurone Disease (MND).

The program uses new technology that allow motor neurones to be grown from patient's skin cells. These motor neurons are then used to screen thousands of drugs until one is found that can slow or prevent the progression of MND. With your participation, the program aims to identify and validate candidate drugs as quickly as possible so that they can be entered into clinical trials.

The program consists of a team of scientists, 2 robots, and a state-of-the-art facility dedicated solely to finding a treatment for MND. This program has been made possible by the generosity of an anonymous philanthropist from country Victoria, the Victorian State Government, FightMND and the Balcon Group Pty Ltd. The research will be conducted at the Florey Institute of Neuroscience and Mental Health in Melbourne in collaboration with Calvary Health Care Bethlehem, Barwon Health, Centre for Eye Research Australia, Murdoch University, Sporadic ALS Australia Systems Genomics Consortium and the Australian MND Registry.



Dr Chris Bye with one of the robots in the FightMND Drug Screening Platform

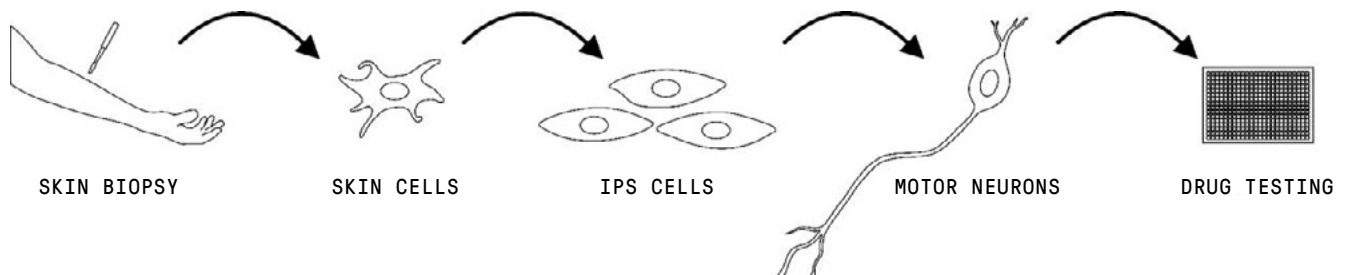
### WHY DO WE NEED PEOPLE WITH MND TO PARTICIPATE?

This program uses living cells from volunteers so that it can test drugs directly on cells derived from real MND patients. One of the main issues in identifying drugs that may help MND patients is finding a model to test the drugs on. It is unknown why most MND patients get sick, which means that it is difficult to replicate the disease process in the laboratory. The recent discovery that skin cells can be turned into iPS cells means that now a skin biopsy can be taken directly from a patient, turned into iPS cells, and used in the laboratory to test drugs.

### WHAT IS AN IPS CELL?

An iPS cell is a normal adult cell that has been returned to an "embryonic" state. This means that it can replicate indefinitely and can grow into any type of cell in the body. The program will grow the iPS cells into motor neurones for the drug screening. These motor neurones share the same DNA as the donating patient and give researchers the best possible chance of finding effective drugs for patients. A video describing the use of iPS cells in research from our collaborators at the Centre for Eye Research Australia is available here ([click here](#)).

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Work flow of how a skin biopsy becomes a motor neurone that can be used to screen drugs.

## WHAT IS INVOLVED?

The program required patient iPS cells to test the drugs on, and to verify their effectiveness when we find promising candidates. Participants in the program donated a blood sample, and a 3mm skin biopsy from the upper arm (with a local anaesthetic). Participants were also asked questions about their symptoms to match to their samples.

## HOW DOES THE DRUG SCREENING WORK?

There are millions of chemical compounds in the world. Compounds will be tested to find drugs that may help motor neurones recover from disease or slow their decline in health. First, drugs that have already been shown to be safe for use in humans will be tested. This allows any drugs that are shown to be effective to be tested in clinical trials sooner. Once all clinically approved drugs have been tested, novel compounds including natural compounds and known bioactive molecules will be tested until we find an effective therapy.

To test the drugs, a large robotics platform is used to grow motor neurons in high density culture plates. The state-of-the-art robotics platform allows researchers to treat micro samples of cells with different drugs to determine if they are effective in delaying disease symptoms.

To capture any delay in disease progression, the facility has installed an Australian first microscope that can record the health of living cells over a period of weeks. The microscope is equipped with a specialised automated loading system to maximise the numbers of drugs that can be screened every week.

The combination of robots and the microscope allows hundreds of potential drugs to be screened every week.

Researchers examining images of the motor neurones grown imaged by the microscope



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## CAN I PARTICIPATE?

The program will complete recruitment in October 2018.

For anyone who has questions or would like further information, please email [FightMND.DSP@florey.edu.au](mailto:FightMND.DSP@florey.edu.au) directly or call 03 8344 5272.



Dr Chris Bye preparing motor neurones in the dish for drug screening