

## 2. TREATMENTS TARGETING MULTIPLE CAUSES OF MND

### PROJECT:

Validation of the clinical stage drug candidate RRx-001 as a novel disease-modifying therapeutic for MND

This project will perform preclinical safety tests needed to advance a drug called RRx-001 towards a clinical trial for MND patients. RRx-001 is a drug currently being tested in a Phase 3 clinical trial for treating cancer. Investigators believe it may benefit MND because it also targets many mechanisms linked to the disease, including an overactive immune system, the build-up of harmful proteins, oxidative stress in motor neurons and their overactivity. This pre-clinical study examines if RRx-001 can delay the onset and progression of MND, prevent the loss of motor neurons, and improve the performance of body movements that deteriorate because of MND.

### KEY HIGHLIGHTS:

Because RRx-001's safety has already been demonstrated in people, successful project outcomes will allow quick transition of the drug to a Phase 2 clinical trial for MND. This international project is a collaboration between researchers at EpicentRx, Inc., in the USA, The University of Queensland and Royal Brisbane and Women's Hospital.

### AMOUNT INVESTED BY FIGHTMND IN THIS RESEARCH PROJECT:

\$719,951

### Q&A:

*What problem are you trying to solve with this project?*

MND is a huge problem, not only because of the devastation that it wreaks on the lives of patients and their families, but also because of the profound financial, economic, and social costs that it imposes on society. The goal—and the hope—for this project is that it will contribute, even in some small way, to a fuller mechanistic understanding of the key drivers behind MND. The cause(s) of MND remain elusive; however, further understanding of how RRx-001 reduces symptoms and improves outcomes may provide key insight into MND and further our fight for effective control and a cure for this devastating disease.

### PROJECT LEAD:

Dr Tony Reid  
EpicentRx, Inc., California, USA



Above: Dr Tony Reid | Below: Co-Investigator Dr Richard Gordon analysing the latest results from preclinical studies in MND models

***“Curiously, and unexpectedly, we found that one of the drugs we use to treat cancer, RRx-001, may have the potential to reduce the symptoms of MND and make a significant difference in the quality of life for patients with MND.” – Dr Tony Reid***