1. GENE THERAPY/DRUG DELIVERY

PROJECT:
Enhanced neuronal delivery, gene targeting and neuroprotection: development of a multimodal drug against MND

PROJECT LEAD:
Dr Loren Flynn
Murdoch University, WA

Above: Dr Loren Flynn | Below: Primary Investigator Dr Loren Flynn and Postdoctoral Fellow Dr Adam Edwards in the Perron Institute lab

A major obstacle for treating MND is the blood-brain barrier, a protective lining between the blood and brain that prevents entry of most drugs into the brain. Investigators in this project are developing a way to overcome this barrier so that a new, exciting, genetic drug targeting the SOD1 hereditary cause of MND can effectively reach motor neurons in the brain. Their pioneering approach will be to attach the genetic drug to a molecule that allows its transfer through the blood-brain barrier and promotes the health of motor neurons.

KEY HIGHLIGHTS:
Dr Flynn is a first-time recipient of FightMND funding as a lead Investigator. This project is the first step towards developing a "low-risk" way to deliver genetic drugs into the brain, which will substantially benefit the quality of life of people living with MND.

AMOUNT INVESTED BY FIGHTMND IN THIS RESEARCH PROJECT:
$249,974

Q&A:
What are you hoping to uncover from this project?
What's unique about our approach is that the carrier peptide our team has developed, on its own, protects neurons from early death. We hope to discover that, by joining the protective carrier peptide to our MND gene-targeting drugs, we can address the underlying cause of disease while protecting the neuron from further damage.

"I'm excited that this project has the potential to treat MND from multiple angles, giving us greater opportunity to solve and treat this insidious disease."
– Dr Loren Flynn