2. GENE THERAPIES

PROJECT:
Targeted degradation of misfolded TDP-43 as a therapy for MND

PROJECT LEAD:
Dr Luke McAlary
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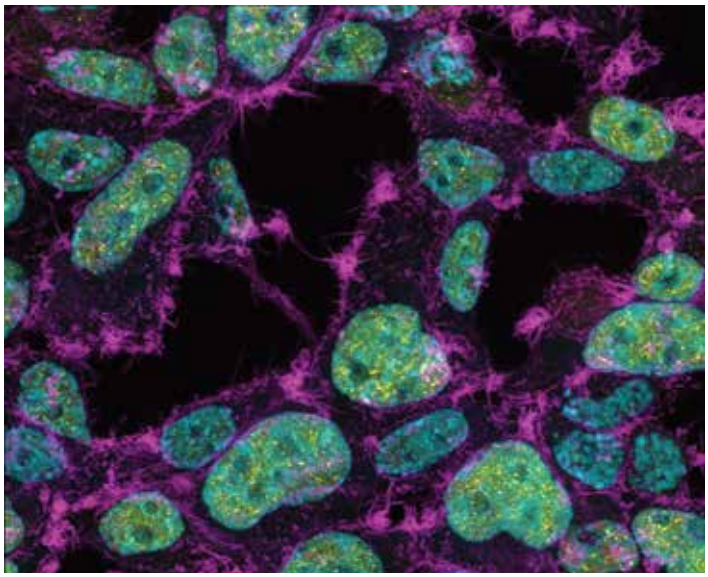
***"If these antibodies work to remove only toxic TDP-43 from cells, we have a potentially viable therapeutic method that may work in the future for those who suffer from MND."* – Dr Luke McAlary**

TDP-43 is a protein that normally keeps motor neurons healthy. However in MND, TDP-43 misbehaves, changes its structure, and becomes harmful to motor neurons. In this project, investigators are using exciting new drugs designed to recognise misbehaving TDP-43 protein. They will test if the drugs can identify and selectively remove the harmful TDP-43 from motor neurons, without affecting normal TDP-43 protein needed for them to function well.

KEY HIGHLIGHTS:
Dr McAlary is a first-time recipient of research support from FightMND. This project aims to target a pathology, misbehaving TDP-43 protein, present in almost all cases of MND.

AMOUNT INVESTED BY FIGHTMND IN THIS RESEARCH PROJECT:
$237,275

Q&A:
Why is this important and how will it benefit patients?
TDP-43 is a very obvious target in MND. One of the major problems associated with this protein is that it is extremely important to cell function, which means we cannot simply remove this protein altogether. If we are successful in only removing toxic TDP-43, there is potential this could become a future therapy.



Above: Dr Luke McAlary | Below: Human cells expressing TDP-43 (yellow) in the cell nucleus (cyan) with an actin counterstain (magenta)