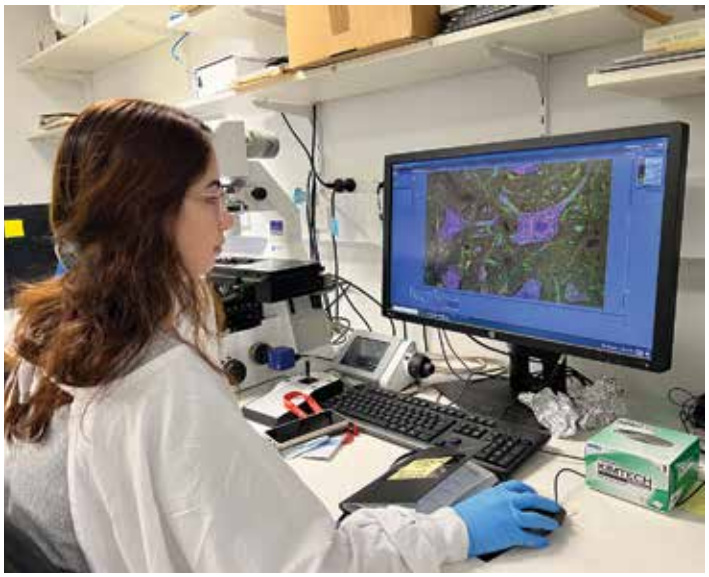
5. GENE THERAPIES

PROJECT:
Therapeutic targeting of TDP-43 through selective reduction of ataxin-2 expression with peptide-conjugated antisense oligonucleotides

PROJECT LEAD:
Dr Fazel Shabanpoor
The University of Melbourne, VIC



Above: Dr Fazel Shabanpoor | Below: Dr Azin Amin is analysing the effect of antisense oligonucleotides treatment on the formation of stress granules and TDP-43 inclusions inside motor neurons

"The exciting aspect of this project is the merger of two proven technologies - antisense and brain-penetrating peptides - to develop a novel and safe brain-penetrating peptide therapy."
– Dr Fazel Shabanpoor

TDP-43 is a protein that is vital to the health of motor neurons. However, in 97% of MND cases, TDP-43 misbehaves and contributes to their death. This study is developing and testing a gene-therapy approach to overcome two problems with TDP-43 in MND: the accumulation of TDP-43 into clumps that are harmful to motor neurons; and the abnormal placement of TDP-43 in motor neurons.

KEY HIGHLIGHTS:
Dr Shabanpoor is a FightMND Mid-Career Fellow. Supporting this project enables Dr Shabanpoor to continue his research into developing novel gene therapy strategies for treating MND.

AMOUNT INVESTED BY FIGHTMND IN THIS PROJECT:
$249,731

Q&A:
Why is this important and how will it benefit patients?
The proposed antisense therapy will target a pathology associated with TDP-43 aggregates inside motor neurons. TDP-43 aggregates are toxic to motor neurons and are present in 97% of ALS patients. Therefore, preventing the formation and accumulation of TDP-43 aggregates inside motor neurons holds significant therapeutic potential and it will be applicable for treating both sporadic and familial forms of MND.