FIGHT MND.


Dr Ning Shan

Researcher.
DR NING SHAN

Drug Development Project.
DEVELOPING M102 TO TREAT MND

Where do you work?

I work in Phoenix, Arizona (USA) and frequently travel to our corporate office at Radnor, Pennsylvania (USA), where the majority of the Aclipse team works.

What is your research experience and background?

I was trained as a physical chemist and received my PhD degree from the University of Cambridge, UK. Currently, I am a pharmaceutical executive with over 17 years of experience in preformulation, formulation, CMC (Chemistry, Manufacturing and Controls), Drug Metabolism and Pharmacokinetics, toxicology, regulatory filings, and protection of intellectual properties. I have a good understanding in pharmaceutical science, as well as the current industry best practice and regulatory requirements. In addition, I am a subject matter expert in organic solid-state chemistry, with a special focus on pharmaceutical cocrystals.

Can you tell us a little about Aclipse One Inc.?

Aclipse One Inc. is a subsidiary of Aclipse Therapeutics, which develops novel and highly differentiated drugs to treat orphan diseases with significant unmet medical needs. Our vision is to combine the understanding of novel biological pathways in orphan diseases and development of innovative drug candidates coupled with precision medicine approaches. Our approach allows us to reduce clinical and scientific risks in a stepwise fashion. Aclipse's Management have managed 12 orphan drug development programs and are extremely well versed with nonclinical and clinical development, regulatory filings, IP protection, business development, and product marketing.

Why did you decide to pursue MND research?

We are passionate to develop therapeutics to help people suffering from MND across the world. MND is 100% fatal, with patient average life expectancy for this devastating disease being 2-5 years post diagnosis. There is a significant unmet need for better approaches and more effective treatments in fighting MND, as currently approved drugs only marginally influence survival or disease progression. We want to make a difference.

Can you describe the current focus of your research teams?

The current focus of our research team is to complete the IND-enabling studies and initiate the first-in-human studies for M102. Based on its novel pathomechanisms and existing pharmacology data, we believe that our drug candidate M102 could impact, slow down, and potentially reverse the progression of MND.

Our collaborators at the Sheffield Institute for Translational Neuroscience (The University of Sheffield, UK) led by Professor Dame Pamela Shaw, a world-leading MND researcher, are dedicated to the development of therapeutic biomarkers to support the clinical development of M102. Aclipse has an exclusive, world-wide license from the University of Sheffield for all intellectual property related to M102.

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What excites you most about M102?

M102 is the only molecule in the competitive landscape that is an NRF2 and HSF1 dual activator that produces multiple effects on multiple neurodegenerative disease cellular mechanisms. M102 has a very comprehensive MND preclinical package showing significant efficacy in multiple cellular and animal MND models.

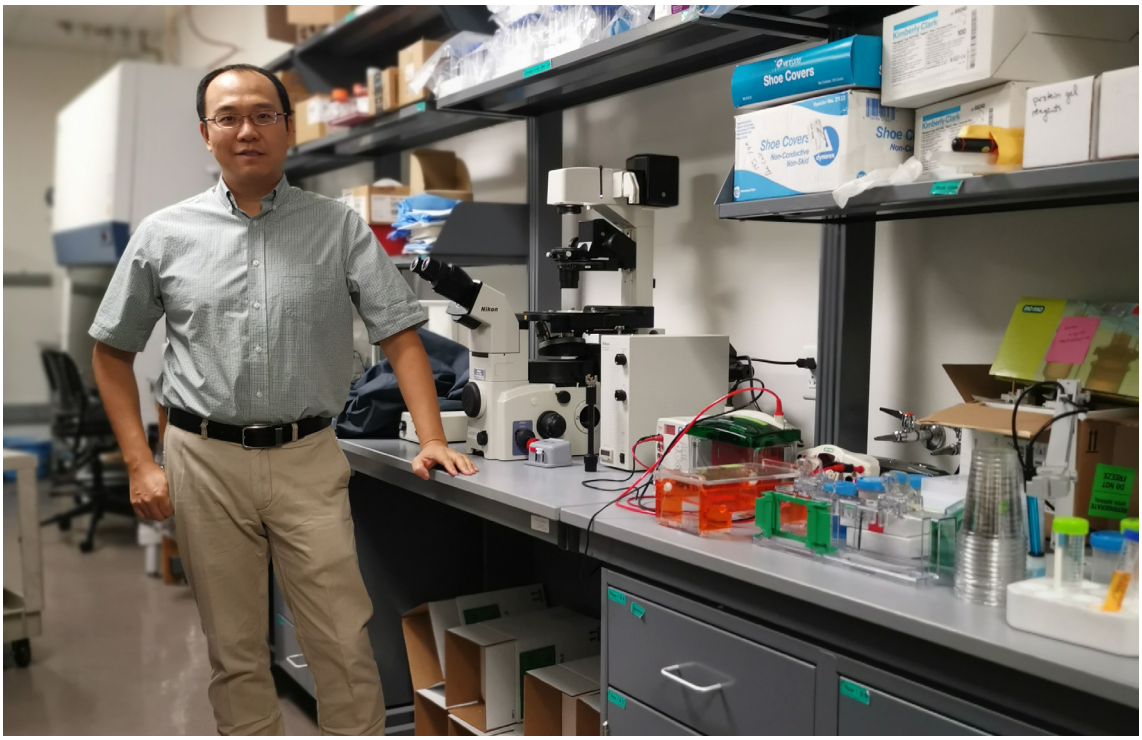
This project is a multi-national collaborative effort. What will this funding enable your team to achieve?

This funding will support an important set of IND-enabling studies for M102 and accelerate the initiation of its clinical development. In addition, this funding will also support the further development of therapeutic biomarkers that can be applied in the future clinical studies of M102 and potentially other therapeutics. We thank the FightMND army and FightMND for helping accelerate M102's development towards a clinical trial for people living with MND.

The Drug Development Project

While the cause of MND is still unclear, it is widely accepted that a number of different cellular pathways contribute to the death of motor neurons. Drugs that target one of these pathways have the potential to slow down the loss of motor neurons and disease progression. M102, a novel small-molecule, acts on multiple pathways and therefore can target multiple disease mechanisms, offering enhanced opportunities for intervention. Investigators have identified that the antioxidant and anti-inflammatory properties of M102, together with its ability to improve a motor neuron's energy levels and its communication with other cells and muscles, may be beneficial for MND.

This project is focused on supporting the development of M102 through the final stages of testing so that it is ready to use in MND clinical trials within the next 3 years. These studies will include upscaling the manufacturing of M102, toxicology studies and biomarker studies that will develop a test to determine if M102 is reaching its target and having the desired effect. These studies are all critical final steps in the advancement of M102 towards a clinical trial in people with MND.



Dr Shan in the Aclipse One Inc. laboratory in Pheonix, Arizona, USA.

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OBJECTIVES

- To complete the preclinical development for M102 and initiate its Australian Therapeutic Goods Administration clinical trial notification application.
- To develop biomarkers confirming that M102 is acting on intended targets when administered to MND patients during clinical trials.
- To develop biomarkers that can identify MND patients that will respond to treatment with M102.

OUTCOMES

- Regulatory approval of M102 for its clinical use to treat MND.
- The team hope to initiate a Phase 1 clinical study at the completion of this study at the end of 2022.