

# 1. THERAPIES TARGETING MUSCLE

## PROJECT:

Intramuscular allosteric agonism of purinergic P2X7 receptor as a pharmacological approach to enhance skeletal muscle regeneration in MND

In MND, motor neurons in the brain and spinal cord die and the body's muscles waste away. While researchers know that protecting motor neurons is essential to overcoming MND, the role that muscles play in the progression of the disease is poorly understood. Investigators in this innovative study will test if a drug that helps rebuild muscle can overcome muscle loss that occurs with MND and slow down disease progression. The drug being tested is safe for use in people, so a successful outcome could see its quick progression into a clinical trial for MND, either alone or combined with drugs that directly target motor neurons in the brain.

## KEY HIGHLIGHTS:

Dr Giovanni Nardo is a first-time recipient of research support from FightMND. This international project is building a collaboration between researchers at the Mario Negri Institute in Italy and The University of Queensland.

## AMOUNT INVESTED BY FIGHTMND IN THIS PROJECT:

\$985,328

## Q&A:

*What excites you about your research project?*

The exciting aspect of our project is based on the opportunity of defining a therapeutic approach for ALS that will establish how the preservation of the muscular system is pivotal to protecting motor neurons. I am also excited by the possibility of developing this hypothesis with Prof Ngo and Dr Steyn at the University of Queensland with the aim of defining a stable scientific flow between Australia and Italy for the cure of ALS.

## PROJECT LEAD:

Dr Giovanni Nardo  
Mario Negri Institute for Pharmacological Research, Italy

***“The main strength of our proposal lies in the use of an easily accessible and low-cost candidate drug, for which biosafety has already been tested in humans.” – Dr Giovanni Nardo***



Dr Giovanni Nardo