

# FIGHT MND.

Lead Investigator.  
**PROF STEVE VUCIC**  
Clinical Trial Name.  
**NANOCRYSTALLINE-GOLD  
TRIAL (RESCUE-ALS)**



Prof Steve Vucic

## Where do you work?

I work at the Westmead Hospital in Sydney.

## Can you summarise your clinical experience and research background?

I am a clinical academic at University of Sydney and senior staff specialist at Westmead Hospital. My area of research is in clinical neurophysiology, neurodegenerative diseases, ALS and related disorders as well as neuroimmunology. In addition, I also head the Westmead ALS/MS clinical trials service and have conducted a number of studies.

## Why did you begin searching for a treatment that prevents MND?

ALS is a devastating disease for which there is no effective therapy. I am passionate about finding a cure for this disease.

## Can you describe the work your team is currently pursuing?

The research conducted by our team is multifaceted and multidisciplinary. It incorporates assessment of brain function, molecular, genetic, biomarker and clinical trials research.

## How did you identify Nanocrystalline-Gold as a potential treatment for MND?

The potential of Nanocrystalline-Gold was identified via a relationship established through a Multiple Sclerosis clinical trial.

## How will this funding impact the potential for Nanocrystalline-Gold?

This funding from FightMND will enable the study to be conducted. Without this funding the trial would not be occurring in Australia.

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Lead Investigator.  
**PROF MATTHEW KIERNAN**  
Clinical Trial Name.  
**NANOCRYSTALLINE-GOLD  
TRIAL (RESCUE-ALS)**



Prof Matthew Kiernan

## Where do you work?

I work at the Brain and Mind Centre, University of Sydney; and Department of Neurology, Royal Prince Alfred Hospital, Sydney.

## Can you summarise your clinical and research experience?

As the Bushell Chair of Neurology, I am responsible for the discipline of neurology at the University of Sydney. From a clinical perspective, I am Professor of Neurology at the Royal Prince Alfred Hospital. I bring to these roles experience as President of the Brain Foundation and Editor-in-Chief of the Journal of Neurology, Neurosurgery & Psychiatry (BMJ Publishers, UK); Director, Discovery & Translation at the Brain & Mind Centre, University of Sydney and Past President of the Australian & New Zealand Association of Neurologists. At a clinical level, I run the Multidisciplinary Motor Neurone Disease Clinic at the Brain and Mind Centre and Royal Prince Alfred Hospital. I have also served as Chair of the Motor Neurone Disease Research Institute of Australia.

## What drew you to search for a cure for MND?

To me, MND is the most impenetrable and tragic human neurodegenerative disease. It is devastating to watch patients battle from being healthy and active, to becoming paralysed and locked in. Through my clinical and research experience, I want to help.

## Describe your thoughts when Nanocrystalline-Gold was identified as a potential treatment for MND?

Nanocrystalline gold is a really interesting compound that acts to support the intracellular biological reactions that are vital for the normal function of human motor neurones. Phase 1 studies have identified the potential of nanocrystalline gold to provide neuroprotection for human patients suffering from neurodegenerative disease.

Now we are ready to embark on novel clinical trial approaches for MND patients.

## What do you find exciting about Nanocrystalline-Gold?

This is a brand new territory - a really exciting opportunity to develop a new therapeutic approach to MND/ALS.

## How will this funding impact on your work and MND?

Our clinical trial would not have been possible without funding support from FightMND. On behalf of our extended team of clinicians, neuroscientists and industry partners, I would like to express gratitude to FightMND and all of its contributors and the FightMND army for helping to accelerate efforts to discover an effective treatment available to MND/ALS patients.

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## Clinical Trial Name. **NANOCRYSTALLINE-GOLD TRIAL (RESCUE-ALS)**

Nanocrystalline-gold (CNM-Au8) is a mixture of gold nanocrystals that can independently influence a cells energetic, metabolic and redox pathways. In a Phase 1 clinical trial CNM-Au8 was safe and well tolerated in 86 subjects who received CNM-Au8 for 21 consecutive days. Treatment with CNM-Au8 also lengthens the life-span of preclinical MND/ALS models and restores their functional behaviours, by protecting motor neurons from oxidative stress, toxicity and hyperactivity.

### **TRIAL OBJECTIVES:**

This Phase 2 clinical trial will test the safety and efficacy of CNM-Au8 in 42 MND patients at two clinical sites in Sydney: The Westmead Hospital and the Brain and Mind Centre.

### **EXPECTED OUTCOMES:**

- Demonstrate if CNM-Au8 slows progression of MND/ALS in patients;
- Verify the exact biomechanisms by which nanocrystalline-gold provides therapeutic benefits;
- Establish the long-term safety of CNM-Au8 for patients; and
- Inform the design of a wider-scale Phase 3 clinical trial for CNM-Au8.



Prof Vucic using a transcranial magnetic stimulation device to detect connections between the brain and hand.