

Australian Parkinson's Mission

Image: Dopaminergic neuron



What is Parkinson's disease?

Parkinson's disease currently affects around 100,000 Australians, with an additional 14,000 being diagnosed with the disease every year. Parkinson's costs the economy \$3 billion annually and the healthcare system \$600 million annually. Without a medical breakthrough, the number of Australians living with Parkinson's will double approximately every 15 years.

The exact cause of Parkinson's is unknown in around 90% of patients, with certain gene variations predictive of the development of the disease in only about 10% of the population. Sporadic cases, which make up the vast majority of cases, are thought to be caused by a complex interaction of both genetic and environmental factors. There is no known cure for this debilitating disease and treatments address only symptoms, not disease progression. No biomarkers exist either for diagnosis or measuring progression.



What is the Australian Parkinson's Mission?

The Australian Parkinson's Mission (the APM) is an Australian-led international collaboration between the Garvan Institute of Medical Research, Shake It Up Australia Foundation, The Cure Parkinson's Trust (UK), Michael J Fox Foundation (USA), Parkinson's Australia and the University of Sydney. The APM supports an innovative program combining clinical trials with genomics research, biomarker analysis and induced pluripotent stem cell (iPS Cells) testing for people with Parkinson's disease. The program has received \$30 million in Federal Government funding, the most significant investment in Australian Parkinson's research to date. The APM aims to identify and fast track effective treatments for people with Parkinson's, increase access to repurposed and new drugs and identify potential diagnostic tools for Parkinson's to enable early disease detection and intervention.

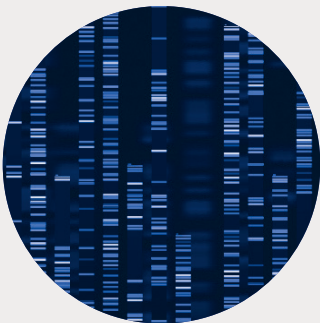


What does the program involve?

The APM will conduct innovative clinical trials integrated with genomic and biomarker approaches to revolutionise our understanding of Parkinson's, identify genetic targets for repurposed drugs and drug discovery in Australia, and provide proof-of-principle for a genomic-based precision medicine framework.

The four branches of the program are:

- **Clinical Trials:** The APM will conduct a number of clinical trials with the aim of identifying drugs that may slow and/or stop Parkinson's.
- **Genomics:** APM-funded research will focus on whether Parkinson's is a collection of genetically defined, distinct subtypes, and whether drugs found to be effective display specificity for any identified subtypes. Each person enrolled in an APM clinical trial will undergo whole genome sequencing to identify whether they have genomic variations contributing to their disease.
- **Biomarkers:** APM-funded research will look for blood-derived biomarkers (including from the samples taken from clinical trial participants) to investigate ways to more accurately diagnose Parkinson's, monitor disease progression and drug response in patients. If genetic subtypes can be established, potential subtype specific biomarkers will also be investigated.
- **iPS Cells:** The APM will use iPS Cell models, differentiated into neurons, to verify that Parkinson's patients can be subtyped through a shared phenotype identified by genomic analysis. The use of iPS Cell models will also enable the identification and confirmation of subtype-specific therapeutic targets that have the potential for repurposed drugs.



Research Collaborators and Neurologists of the APM

Chief Research Collaborators of the APM

- Associate Professor Antony Cooper, Garvan Institute of Medical Research
- Professor Glenda Halliday, University of Sydney
- Professor Daniel MacArthur, Garvan Institute of Medical Research
- Associate Professor Joseph Powell, Garvan Institute of Medical Research
- Associate Professor Sarah Kummerfeld, Garvan Institute of Medical Research
- Associate Professor Justin O'Sullivan, Liggins Institute Auckland
- Professor Deniz Kirik, University of Sydney
- Dr Nicolas Dzamko, University of Sydney
- Assistant Professor Vikram Khurana, Harvard Medical School
- Assistant Professor Jian Peng, University of Illinois

APM Clinical Trial One

The first clinical trial is a 3-1 trial of repurposed drugs that have demonstrated neuroprotective effects in preclinical experiments to assess their potential to change the way Parkinson's progresses. For the clinical trial 240 patients are currently being recruited across 8 sites nationally. These participants will continue on their usual Parkinson's medication. For further information on site locations, please visit our website.

Principal Investigators for APM Clinical Trial One

- Professor Simon Lewis – Brain and Mind Centre, NSW*
- Professor Dominic Rowe – Macquarie University Hospital, NSW
- Dr Stephen Tisch – St Vincent's Hospital, NSW
- Associate Professor Raymond Schwartz - Southern Neurology, NSW
- Dr Kelly Bertram – Alfred Hospital, VIC
- Dr Sam Heshmat – Gold Coast University Hospital, QLD
- Dr Julian Rodrigues – Hollywood Hospital, WA
- Dr John Tillet – Royal Adelaide Hospital, SA

*Professor Lewis will act as the Clinical Lead for the APM



*"Without the support of the community, many whose lives have been touched by Parkinson's, a project this ambitious would never have been possible."
- A/Prof Antony Cooper*

For more information

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