Potential 'holy grail' drug gets 100th patient for trial

Dr Steve Gourlay, Actinogen CEO

Alzheimer's Australia Victoria's Associate Professor Michael Woodward

Jim Northey, who has been taking part in a trial of a new Alzheimers wonder drug, pictured at home in Caloundra with wife, Bev. Picture: Lachie Millard

Pill could halt Alzheimer's

Adrienne Tam

A once-a-day pill that could completely revolutionise the treatment of Alzheimer's disease has reached a significant milestone after the Australian biotech firm behind the potential wonder drug acquired its 100th patient in its trial.

The trial of the "holy grail" drug is being conducted by Australian company Actinogen Medical, which has spent the last decade developing the pill originally discovered by researchers at the University of Edinburgh.

The World Health Organisation (WHO) recently granted Actinogen Medical the non-proprietary name 'emestedastat' for its new drug Xanamem, and gave it a first-in-class, which Actinogen said was a rare global endorsement that placed Actinogen as one of the most promising players in neuroscience worldwide.

The drug is being trialled across 35 sites in Australia and the US.

"There are more Australians than US patients in the trial at this stage, so Australia is leading the charge," Actinogen CEO Dr Steve Gourlay said.

"It's probably fair to say there's roughly equal numbers from New South Wales and Victoria, and a number from other states, including Queensland and West Australia, who have contributed patients as well, because they have smaller populations."

Participants include 81-year-old Caloundra man Jim Northey whose wife Bev said she had noticed significant improvement in her husband during the treatment, to the extent she believed he must have been given Xanamem rather than the placebo which some trialists receive.

"Initially, I didn't know if it was going to work but as we got going along, we could see some definite improvements in Jim," she said.

"He was much more engaging, he was confident in himself, his memory in the tests they gave him ... I could see some real improvements in him. I felt very confident he was either not getting any worse or certainly getting better."

In a major development for dementia care, three of Melbourne's leading hospitals — Austin Health, Royal Melbourne Hospital, and The Alfred — are playing a pivotal role in testing the drug, as well as the Hammondcare Clinic in Malvern.

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>
> **Dr Steve Gourlay**
> **Actinogen CEO**

Unlike current Alzheimer's treatments that target amyloid plaques (the sticky protein long blamed for the disease), Actinogen is taking a radical new approach: targeting cortisol, the brain's key stress hormone.

The trial is being led by Austin Hospital's Associate Professor Michael Woodward OAM, one of the nation's most respected dementia experts.

"We've spent decades chasing amyloid with only marginal benefit," Prof Woodward said.

"If cortisol is the real driver of Alzheimer's symptoms, we could have a safer, simpler, and far more effective treatment on our hands — one that's home-grown, no less."

Alzheimer's disease, which results in worsening symptoms over a number of years, affected 411,100 Australians in 2023, according to Australian Institute of Health and Welfare.

Existing medication and antibody therapies for treating Alzheimer's disease include Amyloid drugs like Donanemab, which require monthly infusions and expensive PET scans, with the cost of treatment estimated to be between $40,000 and $80,000 per year.

"So a once-a-day pill with much greater ease of use, convenience and no complicated monitoring, would be a game-changer," Dr Gourlay said.

"We've now started to talk about potentially just regular specialist doctors, and even in the future, potentially GPs prescribing the medication because of its safety. And particularly, as we anticipate, we're basically developing [the drug] to be at least twice as good as these antibody therapies that were recently approved."

Most of the investment for the drug has come from Australia, with Dr Gourlay saying that more than $100m has been invested in the drug.

"It's also the weight of evidence pointing to cortisol was enormous. We've already showed that it works in depression now, so hopefully next year, we show clearly that it has a big effect in helping slow Alzheimer's," he said,.

An open-label extension trial, in which all participants receive the active drug, will begin in early 2026.