Three ASX biotechs working to solve the neurodegenerative disease puzzle

Health & Biotech | 10 hours ago | **Nadine McGrath**

Neurodegenerative diseases remain one of medicine's toughest puzzles. These ASX biotechs are searching for answers. Pic: Getty Images

- **Several ASX healthcare companies are tackling neurodegenerative diseases where effective treatments remain limited**
- **Alterity is preparing for a phase III trial of its lead drug candidate ATH434-201 in Multiple System Atrophy**
- **Neurizon is working to develop a treatment for amyotrophic lateral sclerosis, the most common form of Motor Neurone Disease**

When 2025 Australian of the Year Neale Daniher died aged 65 in May he had become the Australian face of motor neurone disease (MND), after living with the condition for 13 years.

The former VFL/AFL legend referred to MND as "The Beast" and inspired a national wave of awareness and research funding for the condition through the FightMND foundation he co-founded.

Neurodegenerative diseases, including Alzheimer's, Parkinson's, MND, frontotemporal dementia and other related conditions, affect more than 57 million people worldwide, a figure that is expected to double every 20 years.

Morgans healthcare analyst Iain Wilkie told Stockhead despite the rising burden of neurodegenerative disease there are still no cures, and treatment options remain limited.

"It is definitely harder to think of a healthcare area that has been been tougher than the neurodegenerative or central nervous system space," he said.

"You can't biopsy the brain so patients often don't show up until the damage is well advanced and trials take a long time and cost a fortune."

However, he said research was building including from trial failures, while developments in modern science such as biomarkers and medical imagery could also lead to greater advancements.

"For any company that can crack the biology of neurodegenerative diseases and survive the regulatory gauntlet then the upside is massive," he said.

"Even a slowing of disease progression is enough and you don't need a cure at this point for a company to be successful."

Against this backdrop, here are three ASX-listed biotech companies working to intervene in and treat neurodegenerative diseases.

Alterity targets damaging iron build-up

Alterity Therapeutics (ASX:ATH) is preparing to initiate a phase III trial this year of its lead drug candidate ATH434-201 in patients with Multiple System Atrophy (MSA), a rare and aggressive Parkinsonian disorder.

Alterity recently announced a successful end-of-phase II meeting with the US Food and Drug Administration (FDA), in which the regulator agreed on key elements of its proposed phase III MSA trial design including dosing and endpoint.

The randomised, double-blind, placebo-controlled trial is set to enrol around 200 patients with "clinical and biomarker evidence" of MSA.

Participants will be randomly assigned to receive either ATH434, twice daily, or a matching placebo for 12 months.

The regulator agreed the primary endpoint will be the UMSARS Part I, an 11-item rating scale that measures the activities of daily living affected by MSA, such as walking, swallowing, speech, and dressing.

It is the same measure and dose as the phase II in which ATH434 delivered "clinically and statistically significant efficacy".

Alterity's chief medical advisor and neurologist Dr Daniel Claassen believes ATH434 has potential to treat MSA and other Parkinsonian disorders.

Claassen is Professor of Neurology at the Vanderbilt University Medical Center in the US and was lead investigator of Alterity's phase II trial.

"Neurodegeneration is still treated largely symptomatically, but we are at an inflection point where disease modification is possible," Claassen told Stockhead.

"MSA progresses quickly, and neuronal damage is well advanced by the time patients present, so the therapeutic window is narrow and hard to identify, since the neurodiagnostic tools needed to define an early disease state are limited.

"Together, these factors have made it difficult to show whether a therapy is genuinely slowing progression."

He said what sets ATH434 apart is how it works and that as we age, iron can build up in the brain in a reactive form that damages nerve cells and in MSA that process runs uncontrolled.

“Some therapies try to strip iron out altogether, but that approach is problematic because a certain amount of iron is also necessary for normal function,” he said.

“ATH434 works more like a chaperone and takes the harmful excess iron and guides it safely into the brain’s normal storage system, where it can no longer do damage.”

In Alterity’s Phase II trial, the 50mg dose produced a statistically significant 48% slowing of clinical progression on the modified UMSARS Part I, while MRI showed stabilisation of iron in the MSA-affected brain regions.

“Those results give us real confidence that we can change the course of MSA, and potentially other Parkinson’s-related disorders,” Claassen said.

The FDA has granted ATH434 fast track designation, while the FDA and the European Commission have granted orphan drug designation for the treatment of MSA.

Neurizon advancing ALS registration trial

Neurizon Therapeutics (ASX:NUZ) is working to develop a treatment for amyotrophic lateral sclerosis (ALS), the most common form of MND, which ultimately claimed Daniher’s life.

FightMND grant funding enabled Neurizon’s phase I study MEND of lead drug candidate NUZ-001 to be completed in Australia.

The active pharmaceutical ingredient in NUZ-001 is monepantel with Neurizon holding the human-use rights through a deal with NYSE-listed Elanco Animal Health.

Neurizon this week announced an initial five-year supply deal with Elanco securing long-term access to GMP-manufactured monepantel.

Taken as an oral tablet, NUZ-001 is designed to target biological pathways implicated in ALS pathology.

It is intended to boost autophagy and proteasomal activity, the cell's natural "clean-up" processes, and prevent toxic protein buildup, including TDP-43 aggregates, which are found in most ALS cases.

NUZ-001 has now progressed to Regimen I of the HEALEY ALS Platform Trial, representing a phase II/III registrational trial.

It has been designed to generate the clinical data required to support potential regulatory submissions, including to the US Food and Drug Administration (FDA).

The trial is part of the HEALEY ALS Platform Trial, a master protocol run by the Sean M. Healey & AMG Center for ALS at Mass General Brigham in partnership with the Network of Excellence for ALS (NEALS).

Entry into the program is highly competitive, with candidates selected by expert panels based on scientific merit and potential benefit in ALS.

Neurizon recently announced more than half of the planned 240 participants in the trial evaluating the drug had now been dosed with recruitment tracking ahead of schedule.

As of June 9, 167 had been assigned to Regimen I, with 129 receiving treatment across 78 active clinical trial sites in the US.

Neurizon said based on current recruitment momentum it remained well positioned for last participant dosing in Q2 CY27 and release of topline results in early Q3 CY27.

"Neurizon has made significant progress over the past two years, transforming into a late-stage clinical neurodegenerative disease company with a clearly defined development and regulatory pathway for NUZ-001," Interim executive chairman Sergio Duchini told Stockhead.

"Our participation in the Phase 2/3 HEALEY ALS Platform Trial, combined with strong recruitment momentum and the recent execution of a long-term supply agreement with Elanco, continues to strengthen the foundations supporting the program.

"We remain focused on disciplined execution across clinical development, regulatory strategy, manufacturing and commercial readiness as we work to maximise the opportunity for NUZ-001, subject to successful clinical outcomes and regulatory approval."

Actinogen takes different approach to Alzheimer's

Actinogen Medical (ASX:ACW) is tackling Alzheimer's, **which is the most prevalent neurodegenerative disorder**, with its lead drug candidate Xanamem.

Drug developers have traditionally focused on amyloid, an isolated protein first associated with Alzheimer's by German neurologist Dr Alois Alzheimer in the early 1900s (hence the name).

However, despite billions of dollars invested, many amyloid-targeting therapies have failed to deliver meaningful cognitive benefit, even when they successfully reduce amyloid plaque burden in the brain.

Actinogen is taking a different approach to treating Alzheimer's with Xanamem, which targets excess brain cortisol levels – the stress hormone.

Research suggests elevated brain cortisol may also play an important role in neurodegeneration.

Xanamem targets 11β-HSD1, an enzyme involved in the production of cortisol within the brain and expressed in regions governing memory and mood.

By selectively reducing cortisol production in key brain regions, Xanamem is designed to slow cognitive decline in Alzheimer's while maintaining normal systemic cortisol production.

Actinogen has described Xanamem as the "first and only potent oral inhibitor of 11β-HSD1".

Xanamem has also been shown to cross the blood–brain barrier, a hurdle that has proved challenging for other cortisol-targeting candidates.

A phase IIb/III XanaMIA trial of Xanamem in Alzheimer's is underway, with the company today announcing it had received its third positive Data Monitoring Committee (DMC) recommendation.

In the latest review the DMC examined available safety data from all 247 participants, including more than 100 with the full 36 weeks of treatment, and concluded that the XanMIA trial should continue without amendment. Topline results from the trial remain on track for November.

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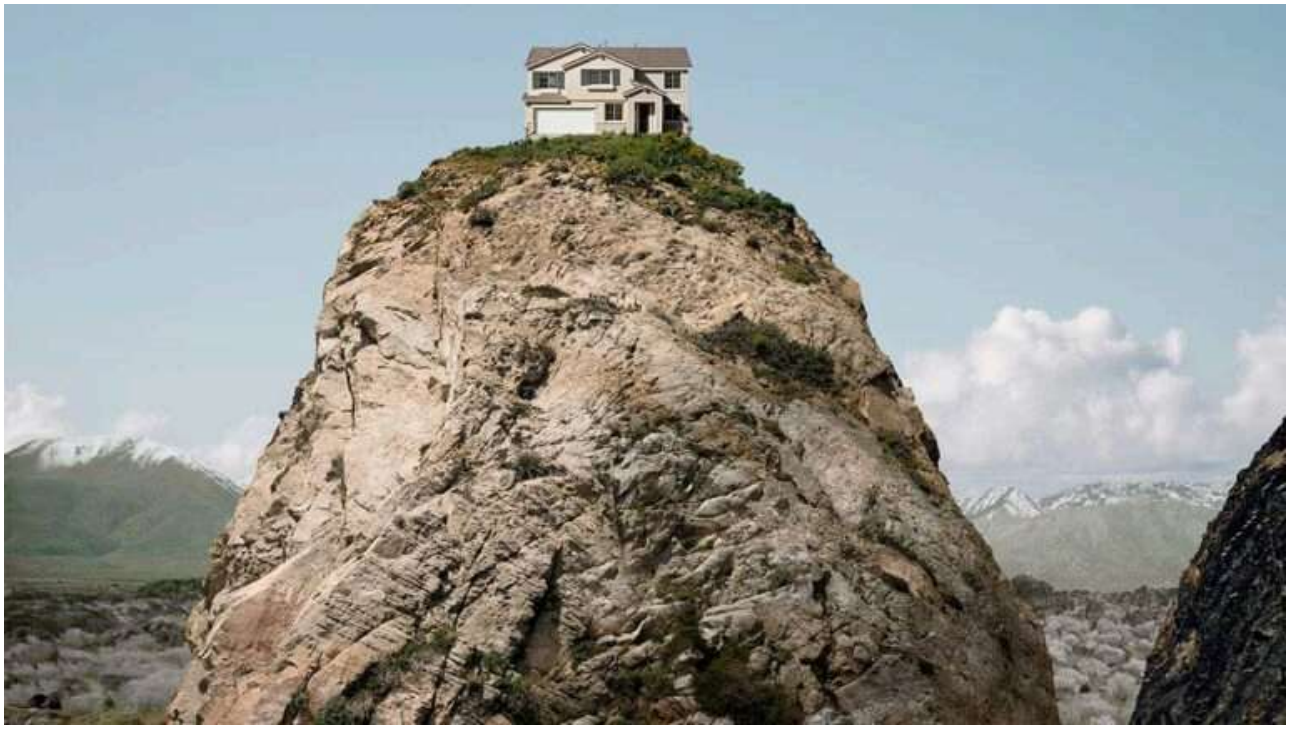

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