THE AUSTRALIAN

Top of mind: ASX companies tackling neurological disorders

By NADINE MCGRATH

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- There are several ASX biotech companies developing drugs to treat complex neurological conditions
- Global neurological disorder drugs market size set to reach US$149.17 billion by 2032
- Petra Capital's Tanushree Jain says neurological disorder treatments are crucial but a challenging drug development area

Drug discovery for neurological disorders is undergoing a renaissance, with several ASX healthcare companies leading the charge in finding new treatment for complex conditions affecting the brain.

The World Health Organization (WHO) lists neurological conditions as now the leading cause of ill health and disability worldwide.

The overall amount of disability, illness and premature death – known as disability-adjusted life years (DALYs) – caused by neurological conditions is up 18% since 1990.

"Neurological conditions cause great suffering to the individuals and families they affect, and rob communities and economies of human capital," said WHO director-general Dr Tedros Adhanom Ghebreyesus.

"It is more important than ever to ensure brain health is better understood, valued and protected, from early childhood to later life."

Challenges of treating neurological disorders

Petra Capital senior healthcare analyst Tanushree Jain told Stockhead that developing treatments for neurological disorders was an important area of drug development.

"There is huge unmet need attractive commercial opportunity, with very little competition in most cases," she said.

However, Jain said neurological disorders were also complex to understand and treat, presenting great challenges in R&D to pharmaceutical companies.

"You need to cross the blood-brain barrier, which is the first challenge for developers to overcome," she said.

"The second challenge is to design clinical trials for diseases which are heterogeneous.

"It's a hard task to develop a drug which works across all different forms of a disease."

Greater regulator support in growing market

The global neurological disorder drugs market size was valued at US$94.30 billion in 2024. It is projected to reach US$149.17bn by 2032, with a CAGR of 5.9% during the forecast period of 2025 to 2032.

Jain said over the years regulators had become more supportive given the huge unmet need for many neurological conditions.

"For a lot of the disease areas you hardly have any approved treatments," she noted.

"Regulators are getting more flexible around providing accelerated approvals pathways, allowing the use of biomarkers for efficacy and less onerous endpoints, which is attracting investment back into neuroscience drug development."

Jain explained that developers could now design trials in a way that gives them a better chance of success with narrower sub-populations and better defined targets.

"The bar of success is also low as you do not need to be a cure to be successful," she said.

Jain said majority of players were more attracted to the niche areas or acute indications within neurological disorders, while big pharma with deeper pockets tended to do registrational trials for larger indications like Alzheimer's.

She said **Argenica Therapeutics (ASX:AGN)** for example was targeting stroke as its lead indication, which is not rare but is an acute indication.

"You have a short follow-up in stroke, so can know pretty quickly if the therapy works or not and that has implications for the cost and time involved with running trials," she said.

First approved treatments for Alzheimer's

Success in getting the first approved treatments for Alzheimer's approved by major global regulators has also spurred on interest in neurological R&D.

In 2023, the US Food and Drug Administration gave its first full approval for a drug designed to slow progression of Alzheimer's.

Lecanemab, jointly developed by Eisai and Biogen and marketed under the trade name Leqembi, was granted accelerated approval in January 2023 before gaining traditional approval in the July.

Another treatment, Eli Lily's donanemab, marketed as Kisunla, received full FDA approval in July 2024.

"The bottom line is the interest has really picked up, particularly with some of the recent success around Alzheimer's treatments, a field which before this was considered as a graveyard for drug development," Jain said.

Time is brain in stroke detection and treatment

In the treatment of stroke, doctors say 'time is brain', meaning rapid triage and treatment is critical.

EMVision Medical Devices (ASX:EMV) is looking to developing its Emu and First Responder to deliver neurodiagnostic scans that not only detect stroke, but most importantly determine the type.

And on the treatment side Argenica is looking to improve treatment for stroke with its neuroprotective drug ARG-007.

"It is a bit like the holy grail for stroke to have a neuroprotection agent," Jain said.

"Their pre-clinical animal studies are very compelling and they're almost close to finishing a phase II trial, for which we are expecting top-line results in August this year.

"If those results are positive, it will be a key inflection point for the stock which will justify it moving into phase III registrational trials but could potentially also attract partner interest."

Argenica is also doing work on traumatic brain injury (TBI) and Hypoxic Ischaemic Encephalopathy (HIE), which occurs in newborn term infants when the brain does not receive enough oxygen or blood flow for a period.

It may occur at any time prior to labour, during labour and delivery, or immediately following delivery.

"All the indications have high unmet need and large markets," Jain said.

Neurizon looks to improve ALS treatment

Neurizon Therapeutics (ASX:NUZ) is targeting amyotrophic lateral sclerosis (ALS), the most common form of motor neurone disease (MND), and potentially other neurodegenerative diseases with its lead drug NUZ-001.

"Neurizon's early clinical trial data, including from an open label extension, is very compelling when compared to the couple of drugs currently on the market for ALS which don't extend patient lives for more than six months," Jain said.

Neurizon has been selected to join the Healey platform, which is trialling multiple ALS treatment candidates and this could potentially serve as a registrational trial for its drug.

"Recruitment into the trial is streamlined and the trial design was recently optimised, which we believe increases the chance of getting a positive outcome," Jain said.

The company will start two animal pharmacokinetic (PK) studies to lift a clinical hold on its Investigational New Drug (IND) application with the US FDA.

"It's straightforward and doesn't cost too much but they basically have to do it so it may delay start of the trial from original estimates by around six months," Jain said.

"We are now expecting the IND hold gets removed in August this year and then Neurizon should be in the position to start the Healey trial in October.

"With ALS there is a well-trodden accelerated approval pathway and we see a lot of regulatory flexibility with therapies even being approved on biomarker data."

Neuren's first approved treatment for Rett Syndrome

In March 2023 **Neuren Pharmaceuticals (ASX:NEU)** and its US partner Acadia were granted the first US FDA approval for a drug to treat Rett syndrome, a rare neurological disorder, which mostly affects girls and emerges in infancy.

"They are generating a lot of revenues from trofinetide, marketed as Daybue, in terms of milestone revenue and recurring royalties, which has given them significant financial firepower to develop their second asset," Jain said.

Neuren's second drug candidate NNZ-2591 is targeting rare neurological paediatric conditions including Phelan-McDermid syndrome, Angelman syndrome and Pitt Hopkins syndrome.

The company plans to start a single phase III registration trial for NNZ-2591 in Phelan-McDermid syndrome, with the same dose, population and duration as the phase II trial by mid-2025.

"They are waiting on an FDA meeting to finalise endpoints which is expected to take place in early April," Jain said.

"For each of these diseases there is very little competition and there is room for more than one product with attractive commercial opportunity."

Neurotech is also targeting Rett Syndrome

Neurotech International (ASX:NTI) is following in the footsteps of Neuren and targeting Rett Syndrome among other paediatric neurological disorders with its oral cannabinoid drug therapy NTI164.

The company has completed a phase 1/2 trial into Rett Syndrome. Neurotech has also successfully completed a phase 2/3 randomised, double-blind, placebo-controlled

clinical trial in Autism Spectrum Disorder (ASD).

The company reported significant and clinically meaningful results in a phase 1/2 trial for Paediatric Autoimmune Neuropsychiatric Disorders Associated with Streptococcal Infections (PANDAS) and Paediatric Acute-onset Neuropsychiatric Syndrome (PANS), collectively PANDAS/PANS.

The company has also received human ethics committee clearance for a Phase I/II clinical trial in spastic cerebral palsy.

"We have successfully demonstrated across four clinical trials in three separate paediatric neurological disorders that NTI164 is both a safe and effective intervention for use in children," CEO and managing director Dr Anthony Filippis said.

"Moreover, the clinical benefits observed are durable with a number of our patients now having passed over two years of daily NTI164 use, which underpins our strategy to accelerate the development of this important treatment in challenging neurological disorders where new therapies are urgently needed."

Exploring new Alzheimer's and depression treatments

Actinogen Medical (ASX:ACW) is developing novel therapies for neurological and neuropsychiatric diseases linked to dysregulated brain cortisol levels, targeting conditions like Alzheimer's disease and depression.

Actinogen is currently undertaking the XanaMIA phase 2b/3 Alzheimer's disease trial across local and US sites to test the efficacy of its novel small molecule compound Xanamem.

The company has started recruiting up to 220 patients, with interim analysis from the first 100 after six months of treatment expected in the December quarter this year. Final results for all 220 patients are expected in the second half of 2026.

Last August, the company released results of a phase 2a trial of Xanamem for depression, which showed "significant clinical benefit".

Xanamem works by inhibiting tissue production of cortisol in the brain, a suspected factor in Alzheimer's.

The two other treatments approved in the US for Alzheimer's inhibit the formation of amyloid protein plaques, linked to development of the disease.

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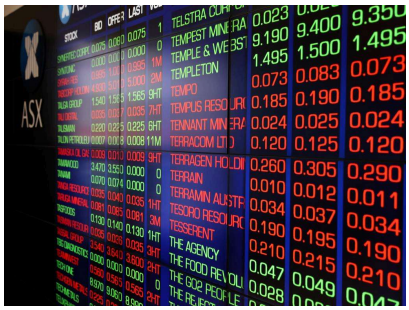

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