August 12, 2024

PharmAust

A condensed clinical program to find next ALS drug

EMERGING COMPANY

SPECULATIVE BUY (initiation)

Stock code:	PAA AU
Price:	A$0.16
12-month target price:	A$0.42
Previous target price:	A$N/A
Up/downside to target price:	162.5%
Dividend yield:	0.0%
12-month TSR*:	162.5%
Market cap:	A$78m
Average daily turnover:	0.5m
Index inclusion:	N/A

** Total stock return – Up/downside to target price + 12-month forward dividend yield.*

Price performance

(%)	1M	3M	12M	3Y
Absolute	-22.0	-8.6	116.2	77.8
Rel ASX/S&P200	-20.1	-8.1	111.5	75.9

Source: IRESS

Iain Wilkie

+61 7 3334 4521

iain.wilkie@morgans.com.au

Scott Power

Analyst(s) own shares in the following stocks mentioned in this report:

– PharmAust

- PharmAust (PAA) is a clinical-stage biotechnology company that focuses on developing therapeutics for neurodegenerative diseases.
- We view PAA as a significant opportunity in the rare disease space and unlike many in the drug development space, offers a highly condensed clinical program which if successful may warrant an accelerated pathway to approval within the next 12 to 24 months.
- Following acceptance into the prestigious HEALEY platform for an adaptive Ph2/3 trial, investors can now look toward a catalyst-rich 24 months including: trial commencement late CY24, Ph2/3 readouts, and if results permit, US marketing authorisation and commercial launch.
- We initiate coverage on PAA with a target price of A$0.42 and Speculative Buy recommendation.

Event

- Initiation of coverage ahead of key clinical catalysts.

Analysis

- PAA's primary asset is a drug called monepantel which is set to progress into an adaptive Ph2/3 trial in the US as a treatment for the most common form of motor neuron disease (MND) called Amyotrophic lateral sclerosis (ALS). This trial is expected to commence recruitment in late CY24.
- Despite the lack of treatment options for the condition, the market potential for new treatments in this space is significant (>$2bn p.a.) attracting high pricing (>US$100k p.a.) for new treatments combined with a moderate prevalence rate with >20k patients living with the condition in the US, and ~5k new diagnoses p.a.

Forecast and valuation update

- We initiate on PAA with a risk-weighted NPV valuation of A$0.42 per share. This includes high clinical risk stage-gate assumptions based on historical success rates in central nervous system (CNS) disorders, which highlight the trial risks with a cumulative 14.1% likelihood of success (LOS) from Ph1 to approval.
- As a guide to what we think PAA could be worth in time as an approved on-market drug (fully derisked) and successful launch, we see a potential value of A$3.21 p/s which would imply a market capitalisation of A$1.5bn. Compared to other rare disease players in the space with on-market assets, we view this as fair. We map the pathway to this unrisked valuation in our model assumptions section.
- We view our initial MND/ALS market potential assumptions as modest, assuming the US as the primary target market (50% global TAM), narrower patient profile (higher ALSFR-S decline rate patients), and assume higher costs and more protracted timelines than current guidance. We expect to revise these factors as more information becomes available including Investigational New Drug (IND) acceptance, recruitment commencement, and initial recruitment rates.

Investment view

- We view PAA as strong proposition in the rare disease space with significant near-term catalysts in a condensed timeframe and strong precedent for an accelerated approval pathway. While considerable clinical risk remains, we view monepantel as a drug with a sound scientific basis in ALS, strong safety profile, and promising hint of potential efficacy above existing treatments. We have a Speculative Buy rating and risk-adjusted target price of $0.42 per share.

Price catalysts

- Ph2/3 IND acceptance (2Q25). Trial design will confirm the endpoints (implications: efficacy readthrough from Ph1), inclusion criteria (implications: recruitment rates), and patient population (implications: timeline / cost).
- FightMND grant funding decision (1Q25).
- Interim and full results from the open label extension study (ongoing to mid CY25).
- Ph2/3 recruitment commencement (end CY24).

Risks

- Clinical timelines, funding, failure of Ph2/3 program.

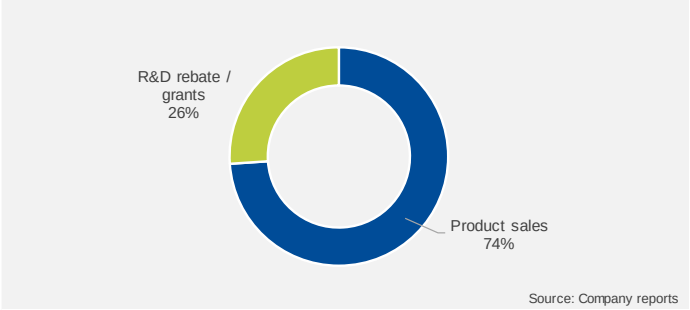
PharmAust

SPECULATIVE BUY — **as at August 12, 2024**

Price (A$):	0.16	12-month target price (A$):	0.42
Market cap (A$m):	78	Up/downside to target price (%):	162.5
Free float (%):	69	Dividend yield (%):	0.0
Index inclusion:	N/A	12-month TSR (%):	162.5

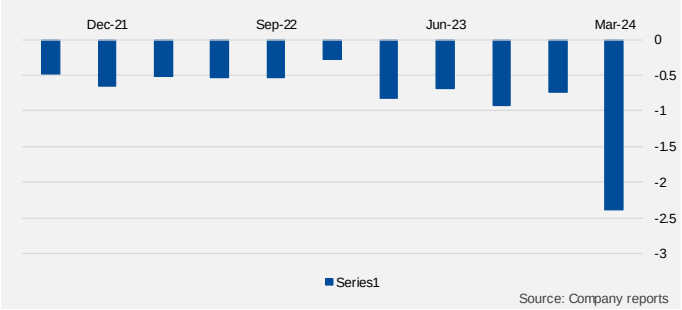
PharmAust is a clinical-stage biotechnology company developing therapeutics for neurodegenerative diseases. The company is focused on repurposing monepantel (MPL) for amyotrophic lateral sclerosis (ALS). ALS is the most common form of motor neurone disease (MND) and affects both upper and lower motor neurons.

Revenue by segment (A$m) - FY23



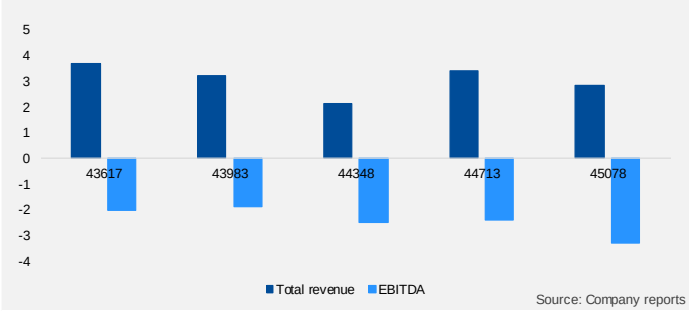
Source: Company reports

Operating cash outflow ex R&D rebates (A$m)



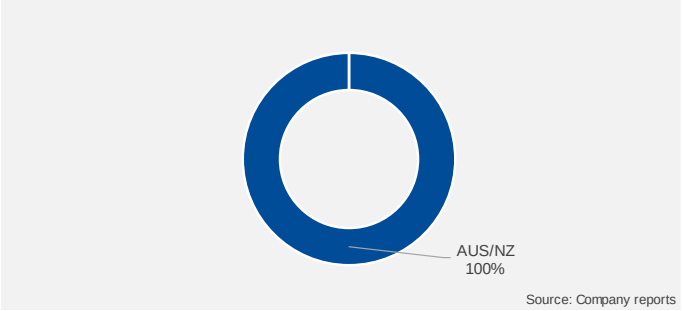
Source: Company reports

Revenue & EBITDA (A$m)



Source: Company reports

Revenue by geography (A$m) - FY23



Source: Company reports

Bull points

Focused on an underserved indication
There are currently only two drug treatments for MND/ALS. Both show no functional improvement over placebo and only minimal improvements in life expectancy.

Rare diseases - high investor interest
Share price performance of several ASX listed rare disease companies following clinical success has placed a spotlight on the sector. Investors are actively looking for these opportunities with reasonable grounds for clinical success.

Bear points

Pre-revenue
PAA is currently clinical stage and pre-revenue. R&D expenses are expected to increase significantly over the next 24 months as its Ph2/3 ALS trial commences.

Capital requirements
Running trials is highly capital intensive. While PAA is currently funded for trial startup and preparations, further capital may be required as the trial progresses.

Environmental, Social and Governance

Exposure
PAA does not own or control any buildings or vehicles, and therefore does not produce Scope 1 or 2 emmissions. While PAA has no specific set targets or actionable details around its environmental impact, the nature of its business being predominately clinical research - the footprint would be limited. PAA also makes a positive contribution to patient outcomes.

Management
PAA has adequate policies in place to ensure ethical conduct of its employees. While PAA have a Diversity Policy Charter, given the size and composition of the organisation it has not currently met its targets and looks to improve its ratios as scale of operations allow. Majority of the board is independent.

Source: Morgans

PharmAust Limited (PAA)

Overview

PAA is a clinical-stage biotechnology company focused on developing therapeutics for neurodegenerative diseases. Its major asset is a drug called monepantel which is an aminoacetonitrile derivative (AAD) drug currently approved as a sheep and cattle de-worming agent, however the core focus is human neurodegenerative conditions such as motor neurone disease (MND). Following a successful Phase 1 trial in MND, PAA is looking to advance its asset to a larger adaptive Ph2/3 trial.

Upcoming catalysts (FY basis)

Interim Open Label Extension (OLE) study data (ongoing to mid CY25)

IND acceptance (2Q25)

Ph2/3 ALS trial commencement (2Q25)

- Ph2/3 full recruitment (+6m following commencement: ~mid CY25)
  - Ph2 results (+6m following full recruitment: ~end CY25)
  - Ph3 results (+6m following Ph2 conclusion: mid-late CY26).

What we like about PAA

Significant market potential: The market potential for new treatments in the neurodegenerative disease space, particularly for ALS, is significant, with the potential market far exceeding US$2bn for new treatments.

Strong catalysts ahead: PAA is set to initiate a Phase 2/3 trial in late CY24, with several key milestones anticipated over the next 24 months, including trial commencement, readouts, and potential US marketing authorisation and commercial launch.

Positive initial trial results: The Phase 1 MEND study showed that monepantel met its primary safety and tolerability endpoints and indicated a 39% slowing in disease progression for ALS patients.

Manufacturing Capabilities: PAA has secured agreements with Syngene International and Catalent Pharma Solutions for the production of Good manufacturing practice (GMP) monepantel, ensuring readiness for commercial supply.

Risks

Clinical timelines, funding, and failure of the Ph2/3 program

Regulatory risk: The risk that a drug or treatment may not receive regulatory approval or may face delays in the approval process.

Competition: The risk that a competitor may develop a similar or more effective drug or treatment, reducing the market potential for the company's product.

Intellectual property: The risk that the company's patents may be challenged or invalidated, reducing the company's competitive advantage.

Market acceptance: The risk that the market may not accept the company's product, reducing its potential for commercial success.

Model assumptions

Due to the early-stage nature of the asset in ALS and the risks surrounding clinical development, we have valued the company on a risked-NPV. We have kept our modelling and assumptions fairly simple and high-level at this stage and major focus is on whether PAA has sufficient funds to achieve and progress its clinical objectives.

We have run a risk-adjusted model based including costs to self-manage all clinical studies and regulatory approvals to bring the product through to commercialisation, and assume all assets if approved to be 100% retained in-house.

MND / ALS:

Drug asset: rNPV valuation of A$200m / A$0.42 p/s.

Total valuation: A$200m / A$0.42 p/s

While we note MPL has a number of possible indications it could potentially address, our focus remains on ALS which we believe has the clearest near-term value and to which the market is tracking and prescribing value.

We focus only the US (~50% of global market) due to maturity of market, clearer regulatory pathways given the HEALEY platform's US trial exposure, and strong comps for pricing in this indication.

Within our model, our broad assumptions include:

ALS:

- US$3bn TAM
  - Based on low estimates of 20k of US population with the condition (up to 40k) multiplied by US$150k pricing expectations p.a. (comp to Relyvrio pricing).
    - See Market Potential section for upside to these assumptions.
    - We also assume a broad 5% p.a. TAM growth rate over the lifecycle of the drug.
- Peak market penetration of 20%. ~US$600m peak sales
  - We view this as a fair but low estimate due to the unmet needs in the indication, potential benefits both in quality of life, and patient longevity. We note Relyvrio had expectations to command 50% of its market (with peak sales of US$2.6bn – implying an addressable market of ~US$6bn) and achieved ~US$400m in sales within its first 12 months on market (post conditional approval) prior to removal from market. Market penetration will ultimately be determined by performance / pricing / coverage so happy to opt for an overly cautious assumption at this stage, noting a number of drugs in the space vying for application in the space.
- Assume a 5-year time to market peak with a peak hold of a further 5 years.
  - Again, upside remains to these assumptions given these rare-diseases which are awarded orphan drug designation are often underserved and patients have little choice in treatment options.
- Our model also assumes that PAA will manufacture and market the drug itself, including market standard marketing expenses. We ultimately view PAA bringing the drug to market by itself is an unlikely scenario given the size and skillset of the company leans more toward clinical discovery versus marketing. However, if successful, we would view this as a potential takeover target and

as such the valuation would remain relevant. Alternative exit scenarios could also include partnership/licensing (partial exit) arrangements although we could not find sufficient licensing arrangements (upfront / milestone / royalty rate) to peg this scenario to with any confidence.

- Lastly, we focus on the long-term potential of the drug rather than short-term financials. We view a short-term focus on P&L in this space is unwarranted and irrelevant outside of clear overspend, diversions, and timeline extensions. Cash at bank versus anticipated clinical development costs remaining is the main financial metric we will track. As long as the Company has sufficient capital to reach the major value inflection points.
    - Based on its 4Q cashflow report, PAA closed the period with A$10.7m in cash, plus A$7.8m raised through the SPP post period. We would also expect PAA to receive an R&D tax rebate of ~A$1.5m in December. This leaves PAA with around A$20m in cash versus an expected trial cost of ~A$25m plus working capital. For this reason, we assume further capital may be required and assume a further A$15m to be required over the next two financial years. At this stage, we assume this is to be raised through issue of shares at current prices.

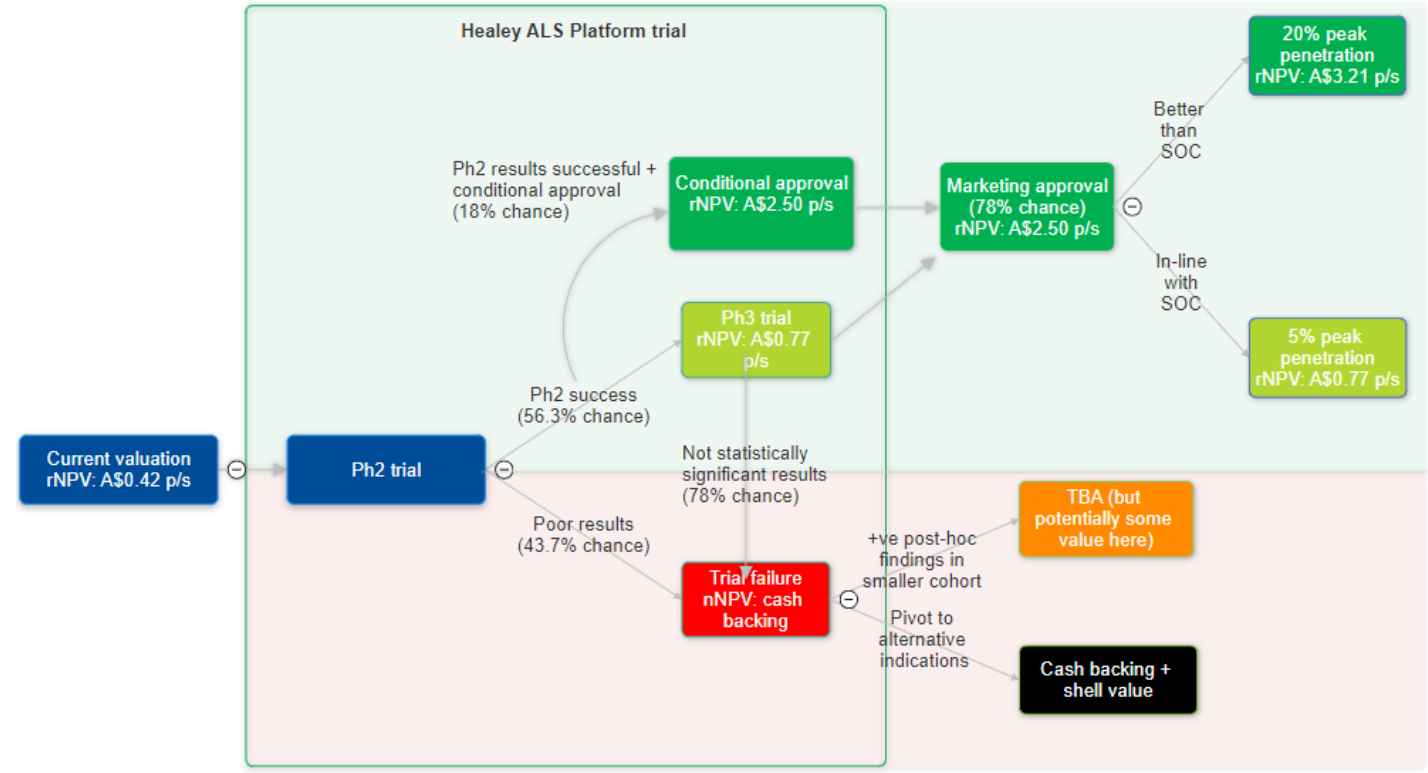
Valuation

Based on our assumptions, we value PAA on a risked-NPV basis alone in ALS of A$0.42 p/s. Further upside remains up to A$3.21 upon clinical derisking and commercialisation success.

Valuation scenarios

Below we illustrate the relative valuation scenarios based on maintaining our broad market assumptions to show the potential upside/downside based on clinical milestones being met. We note the clinical progression risk factors are based on average risks rates across the broad CNS disorders category and we may shift (higher or lower) from these rates as more data is released or insights gained. We also view it important to note the relatively high failure rate in the category of around 44%, but ultimately view the significant upside on progression along with the positive efficacy data (albeit small number of patients) produced from the Ph1 + OLE study as sufficient to warrant advocating for small initial positions.

Figure 1: Current scenario valuation tree



Source: Morgans estimates

About motor neuron disease (MND) / Amyotrophic lateral sclerosis (ALS)

ALS is the most common subset of a group of neurodegenerative diseases broadly referred to as motor neurone diseases (MNDs). MNDs are rare, but their rapid onset causes severe disability with a high rate of fatality (Disease Control and Prevention, 2020).

The condition affects the cells and nerves in the brain and spinal cord which control the muscles in our bodies. This results in rapid weakness and wasting of the muscles, often progressing to paralysis of the muscles in swallowing and breathing resulting in respiratory failure (National Institute of Neurological Disorders and Stroke, 2021).

The exact cause is not fully understood, with only 10% of cases being inherited (familial), while the remaining 90% having no clear genetic or environmental cause (idiopathic) (Mayo Clinic, 2022).

Disease statistics

- Incidence rate (# new cases p.a.) of 1.5 - 2 per 100,000 and a prevalence rate ~ 6 in 100,000 (existing cases) (Target ALS, 2022) .
- Deaths vary by geography, sex and age.
- One-third of patients die within 12 months of diagnosis, half dying within 15-20 months of diagnosis (Williams et al., 2023), and the average life expectancy from the time of diagnosis being ~ 2 to 3 years (Taylor et al., 2024).
- Average time from symptom onset to confirmation of diagnosis by a neurologist is 10 to 18 months.

Similar diseases

- Similar neurodegenerative diseases to MND include Alzheimer's disease (AD) and Parkinson's disease (PD). As with MND, these diseases are characterised by the progressive loss of neurons in the nervous system. There is currently no cure for AD or PD.

Treatment options for MND

- Currently there is no cure for MND.
- Treatment is multidisciplinary and largely symptomatic care; neurology, physical therapy, respiratory therapy, social work/care, and finally palliative care.
- Medications are used as supportive therapy, only delaying the progression of the disease.
- Two main drugs used to treat MND symptoms are Riluzole and Edaravone.
- **Riluzole** was the first drug approved by the U.S. FDA in 1995 to treat MND, however it cannot reverse the damage already done to motor neurons. May extend survival by 3 to 6 months.
  - Needs prescription and limited to patients with MND diagnoses less than 5 years (plus other criteria). Can be prescribed under PBS. PBS price $30, private prescription $180 for a one month supply.
  - Works as a neuroprotective and potentially extends survival and/or time to tracheotomy (assisted breathing). No evidence to suggest improvement in motor function, lung function, muscle twitching, muscle strength, or motor functions.
- **Edaravone** (Radicava®) was originally used to treat stroke patients in Japan and since been approved for use in ALS patients since 2017.
  - The drug works as an antioxidant by suppressing the hydroxyl radical (OH)-dependent and OH-independent lipid peroxidation pathways.
  - The mechanism of action is thought to protect nerve cells by mopping up damaging "free radicals" in the body.
  - Has shown to improve survival in ALS patients by 6 months, but has not shown not affect functional outcomes.
  - Cost is ~US$172k.
- US and Canada granted conditional approval to RELYVRIO® (sodium phenylbutyrate and taurursodiol), developed by Amylyx, for the treatment of adult MND based on data from a Ph2 trial only. September 2022 cost was ~US$158k p.a. Subsequent failure of Ph3 confirmatory trial in March 2024 and has since been pulled from the market.
- QALSODY® was granted accelerated approval in 2023 by FDA for adults with MND associated with SOD1 mutations (~2% of all MND cases). Via spinal injection. Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion recommending a marketing authorisation under exceptional circumstances for QALSODY®.
- Botulinum toxin (Botox) injections may be used to treat muscle stiffness by weakening overactive muscles. They may also be injected into the salivary glands to stop drooling. Excessive saliva can also be treated with medications such as amitriptyline, glycopyrrolate, and atropine.

Monepantel Mechanism of Action

- Monepantel is a veterinary drug that has been repurposed for use in treating MND/ALS. It is a potent inhibitor of the mTOR pathway, which is involved in cell growth, proliferation, and autophagy. Autophagy is a process cells use to recycle abnormal or defective components or molecules.
- In the context of MND/ALS, **misfolded proteins** play a significant role. These proteins, which can be produced by mutant genes, accumulate in motor neurons, inducing dysfunctions and leading to their death. Misfolded proteins can spread from neuron to neuron, enabling the disease to spread throughout the nervous system.
- Monepantel is believed to help clear these misfolded proteins that build over time in the motor neurons of MND/ALS patients, damaging and killing these cells. By activating the mTOR pathway, monepantel may enhance the clearance of these harmful proteins.
- Recent studies have shown promising results. A Phase 1 MEND study (NCT04894240) showed that monepantel met its primary safety and tolerability endpoints, with slowing in disease progression, in patients with MND/ALS. The trial featured 12 patients with MND/ALS who were given monepantel for a 24-hour single-dose pharmacokinetic study, followed by a 4-week repeated escalating dose study. The estimated rate of decline was –0.74 in ALS Functional Rating Scale-Revised (ALSFRS-R) points per month or a 39% slowing in ALSFRS-R decline when compared with the PRO-ACT database, an external historical control cohort. All patients completed the study, with no dose-limiting toxicities experienced.
- In conclusion, monepantel's action on the mTOR pathway and its potential to clear misfolded proteins could provide a new therapeutic approach for MND/ALS.

Monepantel in MND/ALS

Clinical studies

- **Phase 1 MEND Study**: This was a multicentre, open label study comprising a 24-hour escalating single-dose pharmacokinetic (PK) study and 4-week repeated escalating dose study to establish the safety, tolerability and PK parameters of monepantel administered orally to patients with MND/ALS. Twelve participants were enrolled in cohorts of 6 patients across 2 sites. Cohort 1 was administered 2 and 6 mg/kg/day dose levels, and Cohort 2 was administered 4 and 10 mg/kg/day dose levels. Upon completing the study, all participants continued receiving monepantel via a special access scheme and 10 patients were successfully enrolled on a 12-month open-label extension (OLE) study.
- An **Open Label Extension Study** of monepantel in Individuals With Motor Neurone Disease: This is a multi-centre, 12-month open label extension study, following Phase 1 MEND Study, with a single dose of monepantel once daily (QD) for the treatment of individuals with MND. A daily dose of 10 mg/kg monepantel (QD) will be studied in the OLE Study (MON-2023-001) to further evaluate long-term safety and efficacy in participants with MND/ALS that completed the Phase 1 Study (MON-2021-001).
    - Further interim data readouts are expected quarterly. Full cut of results anticipated through H2 CY25.
- PAA is currently finalising plans for an adaptive Phase 2/3 trial in the US through the HEALEY ALS Platform. Broad trial structure and value expectations as below:

Figure 2: HEALEY trial structure (proposed) – subject to IND approval

Pivotal Opportunity

- Independent validation of monepantel's potential as an ALS treatment
- Leverages a network of leading ALS Neurologists across the US
- Recognised by industry and the US FDA
- Fastest path to commercialization

Collaboration

- The trial is a large-scale collaboration across multiple clinical trial sites, industry partners, and researchers

Exposure

- Increases reach within the ALS research community, building on an existing partnership with Berry Consultants to create global and US awareness

Funding

- Additional opportunities for non-dilutive and strategic investments

Innovative Trial Structure

Design

- Shared master protocol
- >70 clinical sites across the US
- 3:1 active drug to placebo ratio
- 160-240 participants per regimen
- 5 regimens completed
- 2 regimens enrolment closed

Next Steps

- Approximately 3-month study design phase to create a regimen-specific protocol amendment
- File protocol amendment under MGH's IND
- Open US IND for monepantel
- PharmAust to supply monepantel
- Targeting enrolment Q4 CY 2024

Source: Company data

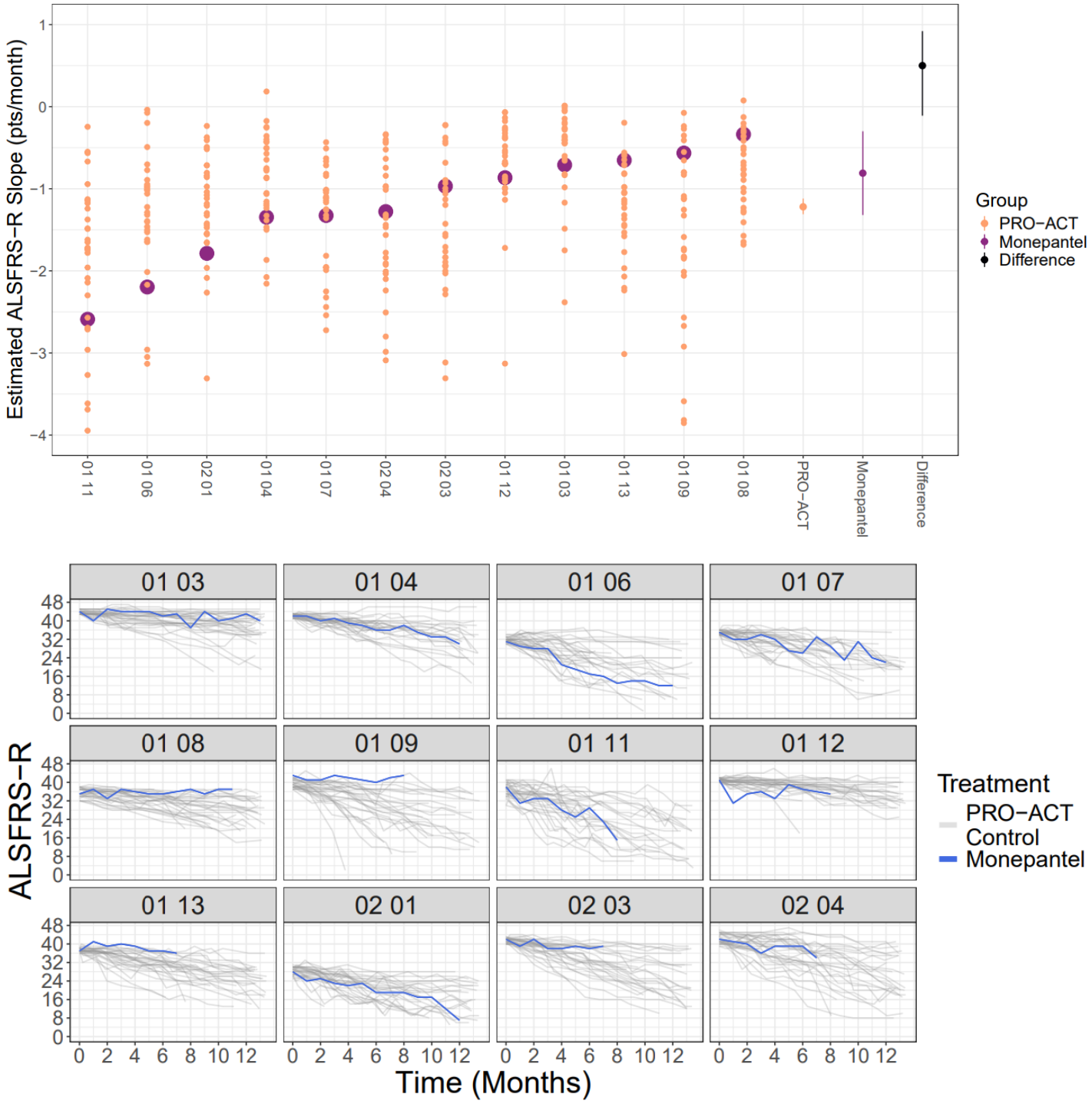
Clinical results

- **Safety and Tolerability Endpoints**: The safety profile of monepantel was favourable with no treatment-related deaths, no dose limiting toxicities, and all 12 patients successfully completing the study. A total of 56 treatment-emergent Adverse Events (AEs) were reported, with only 3 mild AEs considered possibly related to the study drug. Patients remained on daily treatment of monepantel for 8 to 12 months, and all participants continued receiving monepantel after the study.
- **Exploratory Efficacy Endpoints:** The study used several clinical efficacy markers including the ALS Functional Rating Scale-Revised (ALSFRS-R), ALSSQOL-R Quality of Life Questionnaire, Edinburgh Cognitive and Behavioural ALS Screen (ECAS), and Slow Vital Capacity (SVC). There were no significant differences in ALSFRS-R scores between pre-dose and end of treatment for all patients, suggesting that treatment with monepantel over 8 – 12 months slowed the rate of disease progression.
  - A comparative analysis of the rate of decline in ALSFRS-R scores against 30 matched controls from the PRO-ACT database highlighted the potential for monepantel to slow disease progression. For all 12 patients, the estimated rate of decline was -0.74 in ALSFRS-R points per month or a 39% slowing in ALSFRS-R decline. PAA's statistical consultants suggested that depending on the current severity of the disease (determined by the pre-treatment slope of decline), monepantel could provide patients with an additional 13.5- 56.5 months in median survival.
  - The results of the additional exploratory efficacy measures of ALSSQOL-R, ECAS, and SVC further supported slowing in disease progression. In addition, the analysis of biomarkers provided supplemental supporting evidence that monepantel slows disease progression with a large reduction in cerebrospinal fluid neurofilament light chain (NfL), a measure of neuronal damage.

Figure 3: ALSFRS-R Results for Pooled Treatment Group

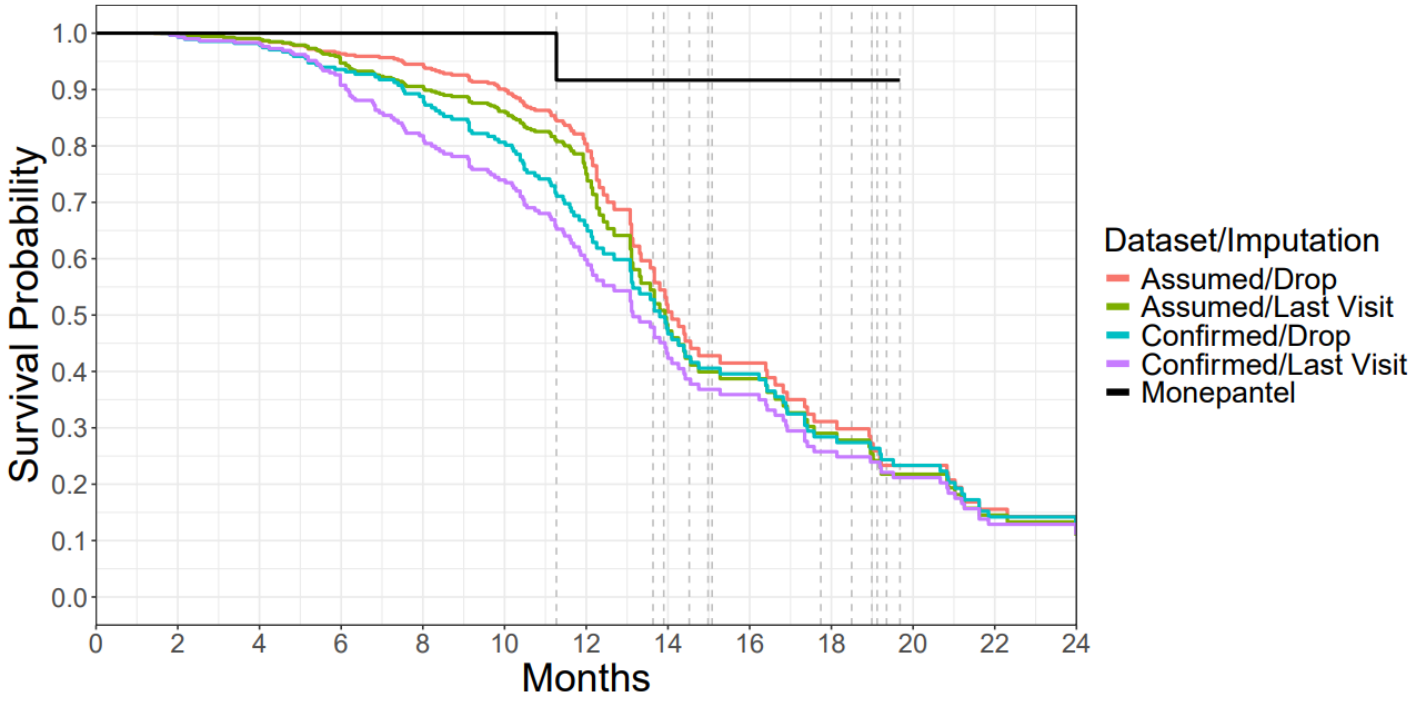
	Estimated Rate of Decline (points per month) *	Slowing in ALSFRS-R Decline *	Additional Life Expectancy **
Combined Cohort 1 + 2	- 0.74	39%	**13.5 – 56.5 months** [1]
Cohort 1 (Low Dose)	- 0.83	23%	
Cohort 2 (High Dose)	**- 0.60**	**58%**	
Relyvrio®	- 1.24	25%	**9 months**

*Berry Consultants analysis. ** Duration is dependent upon disease severity at baseline.*



Source: Company data

Figure 4: OLE survival results



Kaplan-Meier curves for each of the matched-control PRO-ACT datasets as well as the MPL treatment group. Vertical dashed lines represent the exposure time for all patients.

Source: Company data

- **Pharmacokinetics**: Concentrations of monepantel sulfone (MPLS), the active metabolite of monepantel, increased proportionally with the higher doses of monepantel. MPLS was found in the cerebrospinal fluid indicating that both monepantel and MPLS have the ability to cross the blood brain barrier.
- **Target Engagement (mTOR Pathway):** Target engagement of the mTOR pathway in the peripheral mononuclear blood cells was confirmed at all dose levels.

Market Potential

The market potential for MND/ALS is significant due to the lack of effective treatments. Given the different mechanism of action versus existing treatments, monepantel could be used as a monotherapy or in combination with other therapies if successful.

In order to put some crude market potential numbers around monepantel in the US alone, it would be fair to peg monepantel to Amylyx's Relyvrio® market expectations prior to its Ph3 failure.

- Relyvrio® had a list price of US$158k and based on an estimated 38k diagnosed MND patients in the US, this equates to ~US$6bn fully penetrated market for a new and promising treatment.
  - This married up with Relyvrio® peak sales expectations of US$2.6bn @ 50% market penetration prior to being withdrawn from the market.
  - Prior to being withdrawn from the market, Amylyx had generated US$381m in the first 12 months of its ALS drug being conditionally approved for sale.

Intellectual Property

PAA actively patents monepantel for cancer and other diseases reliant on the mTOR pathway, as well as analogues with potentially greater potency. Key patent going forward is the patent granted in the US (US 9,790,176) for “Compounds For The Treatment of mTOR Pathway Related Diseases”, providing the company with a broad patent protection over use of monepantel as well as analogues across all mTOR pathway-related diseases out to 2033 including neurodegenerative diseases.

In addition to its patent portfolio of which five patent families have been granted across most major jurisdictions, PAA also has the benefit of being granted orphan drug designation (ODD) for the treatment of ALS which grants 7 years of market exclusivity upon approval by the US FDA.

Manufacturing capabilities

- Syngene International and Catalent Pharma Solutions, both global leaders in the manufacture and commercial supply of pharmaceutical products, have entered into manufacturing process development agreements.
  - Syngene is tasked with producing 60 kgs of GMP monepantel, which includes an engineering batch of 15 kg and three process validation batches of 15 kg each. These batches are intended to validate the GMP manufacturing process, aid in product registration, and prepare the company for commercial supply.
  - Catalent Pharma Solutions will handle the GMP production of three registration batches, amounting to over 1m tablets. These batches are necessary for supporting product registration and facilitating commercial scale-up activities. The manufactured product will be utilised in the forthcoming adaptive Phase 2/3 trial.
- These manufacturing process development agreements are expected to be succeeded by a commercial supply agreement.
- PAA also recently (April 2024) appointed Dr. Herbert Brinkman as Head of Manufacturing. Dr. Brinkman brings over 30 years of experience in the pharmaceutical industry to the role. He has successfully launched nine products in the US and has prepared over 25 Chemistry Manufacturing and Control (CMC) sections for various regulatory filings. He has also filed 21 Abbreviated New Drug Applications (ANDAs) and is an inventor on 14 patents.

Other items

HEALEY ALS Platform Trial

The HEALEY ALS Platform Trial is an US initiative designed to accelerate the process of drug development and the path for effective treatment for ALS/MND. This is achieved by testing multiple drugs simultaneously and adaptively. This study design approach has been seen to be successful for cancer trials.

Compared to traditional drug development, the platform trial is estimated to find an effective therapy more quickly (average 3.4 vs. 8.5 years), with fewer total participants (average 880 vs. 1400), and fewer participants on placebo (average 220 vs. 700). Participants will have an equal chance to be randomised to all regimens that are active at the time of screening. Once randomised to a regimen, participants will be randomised in a 3:1 ratio to either study drug or placebo. (MND Association)

New regimens will be continuously added as new investigational products become available. The HEALEY ALS Platform Trial will enrol additional participants as each new regimen is available. This trial is recruiting in the US only.

The HEALEY ALS Platform Trial, like similar platform studies in cancer drug development, is testing multiple drugs at the same time, using specialised statistical tools. There is only one placebo group, however, meaning that ~75% of patients receive a treatment rather than a placebo.

This is a multi-centre, double-blind, placebo-controlled, perpetual, adaptive platform trial testing multiple investigational products in parallel to find safe and effective treatments for people living with ALS.

Potential grant funding

- FightMND is an Australian charity organisation founded in 2014 by former AFL player Neale Daniher. The organisation is dedicated to fighting MND and has invested more than A$55m into MND research since 2014. FightMND supports projects focused on overcoming key barriers preventing the advancement of potential treatments through to clinical trial.
  - PharmAust and FightMND have collaborated previously. In June of 2022, FightMND pledged $900k to PharmAust to conduct research into MND. Following the first $200k grant, PharmAust proceeded to a Phase 1 clinical trial.
- We view it likely that PAA wins a portion of the grant funds available, up to ~$2m. While not overly material in the sense of total funding requirements to run the Ph2/3 adaptive trial, we view the market would respond positively if awarded. Timing of grant awards is unknown at this stage however given this is an annual fundraising effort and award, it would be fair to expect the recipients to be awarded these funds by the end of CY24.

Financials

Figure 5: Historical financial statements

Historical data	FY19	FY20	FY21	FY22	FY23
Income Statement (A$m)					
Total revenue	3.7	3.2	2.1	3.4	2.8
Growth (%)	*27.8*	*-12.9*	*-33.0*	*58.0*	*-16.5*
Gross Income	n.a.	n.a.	n.a.	n.a.	n.a.
Growth (%)		n.a.	n.a.	n.a.	n.a.
Gross Margin (%)	n.a.	n.a.	n.a.	n.a.	n.a.
EBIT	-2.2	-2.2	-2.8	-2.7	-3.6
Growth (%)	24.7		-28.5	1.5	-31.8
EBIT Margin (%)	-59.9	-67.9	-130.3	-81.3	-128.3
EBITDA	-2.0	-1.9	-2.5	-2.4	-3.3
Growth (%)	27.7	6.4	-31.4	2.3	-35.4
EBITDA Margin (%)	-55.2	-59.3	-116.4	-72.0	-116.7
Net Income	-1.6	-1.4	-1.3	-1.7	-6.2
Growth (%)	38.5	12.2	1.8	-27.7	-263.6
Balance Sheet (A$m)					
Cash & STI	2.1	2.9	3.0	2.4	2.7
Total Assets	8.6	10.7	11.0	10.1	6.1
Total Debt	0.3	1.5	1.3	1.4	1.0
Net Debt	-1.8	-1.4	-1.7	-1.0	-1.7
Total Liabilities	1.1	2.2	2.1	2.2	2.2
Total Shareholders' Equity	7.5	8.5	8.9	7.8	3.9
Cash Flow (A$m)					
Net Operating Cash Flow	-1.6	-1.3	-0.9	-1.3	-1.6
Capital Expenditures	-0.1	0.0	-0.1	0.0	0.0
Net Investing Cash Flow	-0.1	0.0	-0.1	0.0	0.0
Net Financing Cash Flow	1.9	2.1	1.2	0.8	1.9
Free Cash Flow	-1.7	-1.3	-1.0	-1.4	-1.6
Profitability (%)					
Gross Margin	6.8	-13.3	-36.0	-6.6	-32.9

Period	FY19	FY20	FY21	FY22	FY23
Profitability					
Operating Margin	-59.9	-67.9	-130.3	-81.3	-128.3
Net Margin	-42.3	-42.6	-62.5	-50.5	-220.0
Return on Assets	-18.3	-14.1	-12.3	-16.2	-76.6
Return on Equity	-21.3	-17.0	-15.4	-20.4	-105.9
Return on Invested Capital	-21.0	-15.8	-13.6	-18.1	-90.9
Coverage (x)					
Net Debt/EBITDA	0.0	0.0	0.0	0.0	0.0
Valuation					
Sales per Share	0.0	0.0	0.0	0.0	0.0
EPS (diluted)	0.0	0.0	0.0	0.0	0.0
Growth (%)	*54.5*	*35.2*	*8.7*	*-28.6*	*-259.3*
Dividends per Share	0.0	0.0	0.0	0.0	0.0
Growth (%)	*0.0*	*0.0*	*0.0*	*0.0*	*0.0*
Book Value per Share	0.0	0.0	0.0	0.0	0.0
Growth (%)	*-18.2*	*6.1*	*-0.7*	*-11.7*	*-54.8*
Shares					
Diluted Shares	219.3	296.0	315.7	316.9	321.0
Ratios (x)					
Price / Sales	2.3	14.4	13.4	6.6	8.5
Price / Earnings	-	-	-	-	-
Price / Book Value	1.4	5.5	3.2	2.8	6.7
Price / Tangible Book Value	2.4	8.6	5.0	4.7	33.3
Price / Cash Flow	-5.4	-36.1	-30.6	-16.6	-15.4
Price / Free Cash Flow	0.0	0.0	0.0	0.0	0.0
Dividend Yield (%)	0.0	0.0	0.0	0.0	0.0
Dividend Payout Ratio (%)	0.0	0.0	0.0	0.0	0.0
Enterprise Value / EBIT	-4.0	-20.9	-9.7	-7.7	-6.7
Enterprise Value / EBITDA	-4.4	-23.9	-10.9	-8.7	-7.4
Enterprise Value / Sales	2.4	14.2	12.7	6.3	8.6
EBIT / Interest Expense	-46.0	-18.3	-35.3	-31.1	26.5

Source: Factset, company data

Board and management

Figure 6: Key management personnel

Name	Position	Brief bio
Dr Michael Thurn	MD & CEO	Dr Michael Thurn brings broad experience in drug discovery, development, regulation and commercialisation, acquired through leadership roles in research organisations and industry, including early stage, fast-growing, private and publicly listed biotechnology companies. His previous responsibilities have included leading a variety of US Food and Drug Administration (FDA) Investigational New Drug (IND) applications across a range of therapeutic areas and the evaluation of drugs and vaccines for registration in Australia as a part of the Drug Safety Evaluation Branch (DSEB) of the Therapeutics Goods Administration (TGA).
Mr John Clark	COO	John Clark joined PharmAust with over 20 years of pharmaceutical industry experience in phase I – IV clinical trials across numerous therapeutic areas and multiple geographical regions. Most recently, John served as Senior Project Manager at a Global CRO, leading the Clinical Operations team and providing cross-functional oversight on a national CNS trial. Before that, John held various clinical operations leadership roles responsible for implementing clinical programs. John has a proven project management and stakeholder engagement record, with a thorough knowledge of ICH-GCP and regulatory requirements.
Dr Nicky Wallis	CSO	Dr Wallis is a neuroscientist and brings over 12 years of global expertise in clinical development, spanning pre-clinical through to Phase 3 drug and device development. She has a proven track record in managing multi-site, international clinical studies across the US, Europe, Australia, and New Zealand, with a strong focus on regulatory submissions and compliance, cross-functional collaboration, and vendor management. Prior to joining PharmAust, Dr Wallis provided clinical development consulting services to both private and public biotechnology companies in Australia, with a focus on central nervous system diseases. Her extensive experience includes roles such as Clinical Trials Program Specialist at the Australian Clinical Trials Alliance, Vice President of Clinical Operations at Lateral Pharma Biotech, and Clinical Project Manager at Orygen Youth Mental Health Research. Dr Wallis holds a PhD from The University of Melbourne/The Florey, where her research focused on understanding the pathogenesis of MND/ALS and Alzheimer's Disease.
Dr Herbert Brinkman	Head of Manufacturing	Dr Herbert Brinkman, based in Denver, Colorado, has over 30 years of experience in the pharmaceutical industry. Dr. Brinkman has prepared over 25 Chemistry Manufacturing and Control (CMC) sections and updates for multiple Investigational New Drug (IND), New Drug Application (NDA), supplementary NDA (sNDA), Investigational Medicinal Product Dossier (IMPD), and Abbreviated NDA (ANDA) filings for United States Food and Drug Administration (FDA) and European regulatory agencies. Dr Brinkman has filed and commercially launched nine products encompassing oncology, metabolic, dermatology, and endocrinology therapeutic areas and contributed to filing 21 ANDAs for various semi-solid and parenteral products. He is also an inventor on 14 patents. His expertise includes current Good Manufacturing Practice (cGMP) systems applied to API manufacture / Drug Product manufacture and addressing regulatory issues. Dr Brinkman's previous position was Executive Director of Product Development at NASDAQ-listed company Arcutis Biotherapeutics, Inc. (NASDAQ: ARQT), where he was responsible for the successful commercial launch of ZORYVE (roflumilast).

Source: Company data

Figure 7: Board of directors

Name	Position	Brief bio
Mr Sergio Duchini	Chairman	Mr Duchini brings over a decade of board-level experience with expertise spanning professional services, life sciences, biotechnology, banking, finance, and the not-for-profit sector. His extensive background includes roles such as Chair, Executive and Non-Executive Board Director, Risk & Audit Committee Chair, and Chief Strategy Officer. Mr Duchini has previously sat on the AusBiotech Board of Directors for nine years, where he played a pivotal role in the development of two national life science industry strategies. Mr Duchini's executive experience includes 23 years at Deloitte Australia where he held multiple senior positions as an equity partner.
Dr Michael Thurn	MD & CEO	As in KMP
Dr Katie MacFarlane	NED	Dr Katie MacFarlane has over 30 years of experience in the development and commercialisation of pharmaceutical products and devices. She is the Founder and President of SmartPharma, a commercial and strategic consulting firm that specialises in market and product assessments, market sizing and forecasting, pre-launch preparation and launch and marketing of pharmaceutical products for biopharmaceutical companies. Katie also currently is the Head of Commercial for Arkayli Biopharma, a startup developing a treatment for a rare pediatric disease. Previously, she was Chief Commercial Officer at Agile Therapeutics, Vice President of Marketing, Sales and New Product Planning at Warner Chilcott, and Senior Director of Marketing at Parke-Davis (now Pfizer). Dr MacFarlane is a member of the Board of Directors of Mayne Pharmaceuticals
Mr Marcus Hughes	NED	Mr Hughes brings more than 20 years' experience with listed companies. He possesses extensive corporate finance experience, having led project financing and capital raisings in the industrial sector. He has held senior managerial, tax and finance roles with multi-national companies including Lend Lease, Fortescue Metals and Rio Tinto.

Source: Company data

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