



*PharmAust is a clinical-stage company developing novel targeted cancer, neurodegenerative and antiviral therapeutics for humans and cancer therapeutics for animals. The company specializes in repurposing marketed drugs lowering the risks and costs of development*

**ASX: PAA**

ACN 094 006 023

# PharmAust

## Company Snapshot

### Summary

ASX Code:	PAA
Market Cap at \$0.091	\$28.87M
Cash (June 2021)	3.02M
Debt	\$0k
Total Shares on Issue	317M
Options (Unlisted)	5.3M
Top 20 Own	37%
Board/Exec Own	9.3%

### Share Price Movement/ Daily Volume



# Monepantel (MPL) and mTOR

## MPL from Sheep Drench to Anticancer, Anti-neurodegenerative and Antiviral Agent

### ~Repurposing

1. MPL: AChR - mTOR
2. Viagra: cardiovascular - men's health
3. Tamoxifen: contraceptive - anti-cancer
4. Exanatide: diabetes – weight loss

Anti-Wormicide: AChR



Rufener et al., 2013

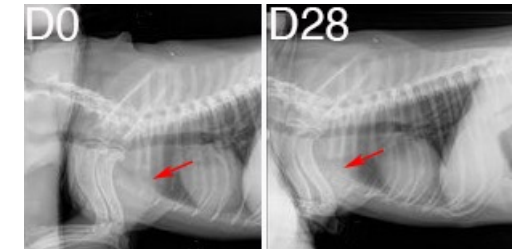


Easter Island: mTOR



Aurbina

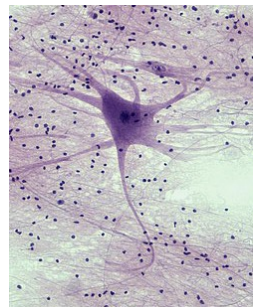
Anti-Cancer: dog and human



PharmAust

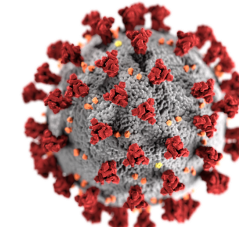


Anti-MND



Berkshire Community College

Anti-Covid19; Anti-HTLV1



Eckert and Higgins

# PharmAust Patent Portfolio

## Patents and Expiry

1. USE OF AMINOACETONITRILES (MPL) IN  
CANCER  
~2032

2. TREATMENT OF DISEASES WITH  
AMINOACETONITRILES OPERATING THROUGH  
mTOR PATHWAY  
~2032

3. MPL IN COMBINATION WITH OTHER  
CANCER DRUGS  
~2034

4. MPL ANALOGUES IN CANCER  
~2034

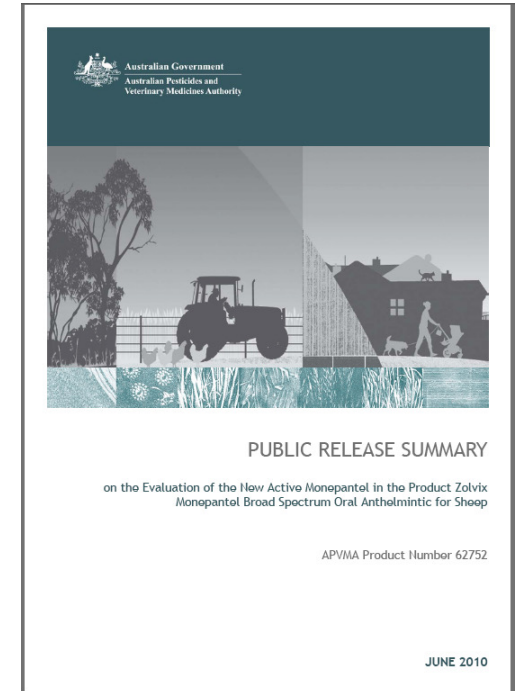
5. MPL IN VIRAL DISEASE  
COVID-19 and HTLV1  
~2040

MPL composition of matter patent owned by major company expires in 2024 in most jurisdictions.

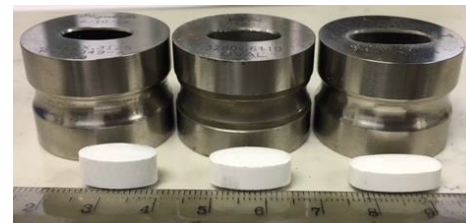


# Dosage Form

## From Sheep Drench to At-Home Tablet



Exceptional Safety Profile



Convenient at-home tablet

# PharmAust: Canine Anticancer

## Pet Dog Phase 2 Trial

Participant	Plasma Drug	Weight loss	Outcome Observer	Outcome Veterinary	How red?	Mpl Dose
001-001	↑	Some to Minor			ambiguous	Adverse Events
001-002					ambiguous	
001-003						
004-003						
007-001					clear	
004-002	↑	None			marginal	Optimal Dose and Outcome
002-002						
003-001						
002-004						
004-006						
005-002					marginal	
004-005						
005-001	↑	None or Not Determined			clear	Suboptimal Efficacy
002-003					clear	
002-001					clear	
006-001					clear	
006-002					clear	

- Prednisolone alone = 4 to 6 weeks OST
- Post trial MPL + prednisolone, 4 of 6 dogs OST > 14 weeks
- High QOL indices
- CHOP alternative = 26 weeks @ \$10 000 and many side effects



Dogs on trial going camping and playing in the park



# PharmAust: Motor Neurone Disease/ Fight MND

## MND/ALS

### Motor Neurone Disease

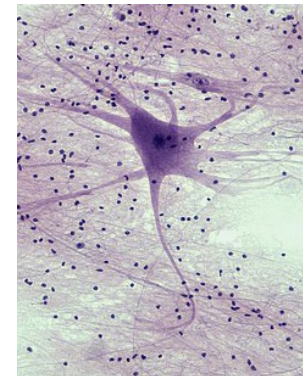
- Amyotrophic lateral sclerosis (ALS); Lou Gehrig's Disease
- Brain and spinal nerve stop working.
- Associated with “proteinopathy” or toxic protein build up in nerve cells.
- Life Expectancy 1-5 years
- About 1 in 400 people
- There are no cures

### Preclinical Data

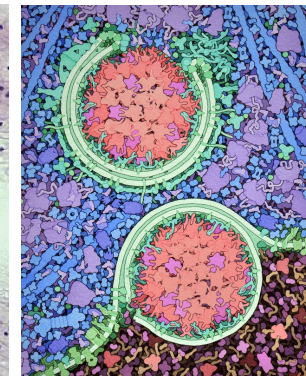
- Hypothesis: MPL induces autophagy to clear protein build up in nerve cells

### FightMND

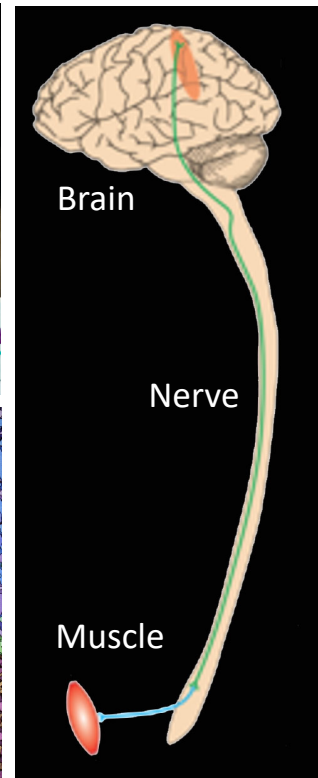
- AUD 881 085 funding for a Phase I/II clinical trial
  - Associate Professor Susan Mathers, Calvary Health Care Bethlehem, Melbourne
  - Professor Dominic Rowe, Macquarie University
- Commencing early next year



Berkshire Community College



David Goodsell, Cell, 2016



FightMND

# PharmAust: Antiviral

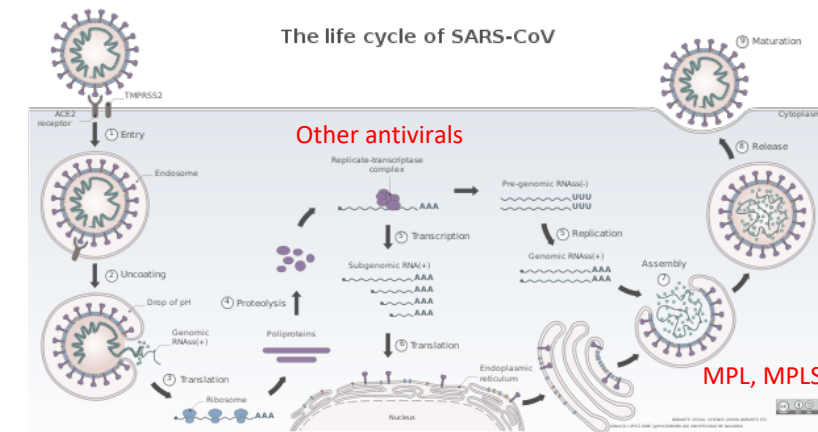
## COVID-19/ HTLV-1

### Preclinical Data: SARS-CoV2

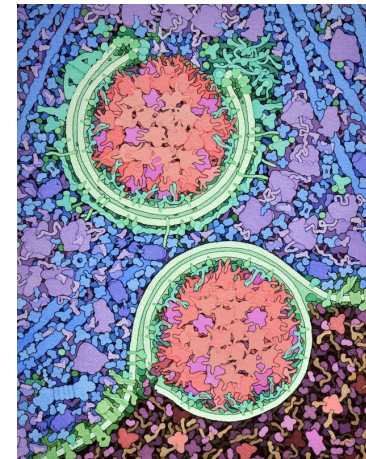
- Three laboratories: MPL and MPLS both protect against SARS-CoV2 induced cell death
- Two of two laboratories: MPL and MPLS reduce viral RNA in culture media
- Two of two laboratories (similar conditions): MPL and MPLS reduce viral secondary infection
- Very different conditions in third laboratory give no effect on secondary infection – good data for determining method of action
- Combined: secondary infection data suggest late-stage viral lifecycle inhibitor
- Consistent with hypothesis for autophagy-mediated mechanism
- Hypothesis: MPL competes to prevent hijacking of host autophagy machinery and permits virus destruction

### HTLV-1

- Retrovirus – like HIV
- But makes blood cells divide like cancer
- 20 million people worldwide; certain Australian indigenous populations
- Very good preclinical models
- Hypothesis: MPL competes to prevent hijacking of host autophagy machinery and permits virus destruction while also exerting anticancer effects



NIAID



David Goodsell, Cell, 2016

Arsensio, Nature, 2020



# PharmAust: Human Anticancer

## Human Phase I, Moving to Phase II

### Clinical Data: Phase I

- Treatment refractory progressing cancer patients
- Three of four Level 1 patients had stable disease by CT scans
- Three of four Level 1 patients had reduced mTOR (increased autophagy) markers
- 92% of adverse events were mild
- No myelosuppression (immune system still intact unlike chemo)
- Very good blood plasma drug data

### Phase II: Set up

- Anticancer activity plasma levels from tablets in pet dogs
- Plasma levels from FightMND and Covid trials for tablet dosing
- Preclinical data shows very effective combination
- Physicians excited by combination: pancreatic, gastric and esophageal

Cancer Chemotherapy and Pharmacology  
<https://doi.org/10.1007/s00280-020-04146-5>

#### ORIGINAL ARTICLE

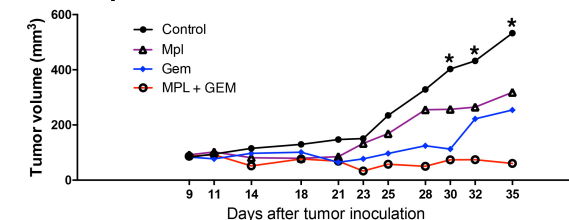
### A preliminary assessment of oral monepanel's tolerability and pharmacokinetics in individuals with treatment-refractory solid tumors

Anna Mislang<sup>1</sup> · Richard Mollard<sup>2,3</sup> · Gonzalo Tapia Rico<sup>1,8</sup> · W. Douglas Fairlie<sup>4,5,6</sup> · Erinna F. Lee<sup>4,5,6</sup> · Tiffany J. Harris<sup>4</sup> · Roger Aston<sup>2</sup> · Michael P. Brown<sup>1,7,8,9</sup> 

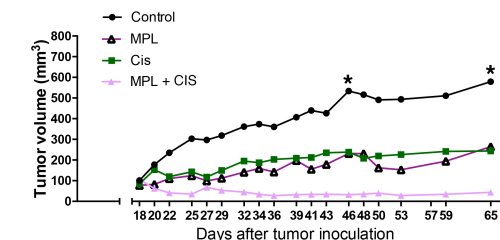
Received: 18 April 2020 / Accepted: 8 September 2020

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#### Monepanel + Gemcitabine: OVCAR3 Xenografts



#### Monepanel + Cisplatin: OVCAR3 Xenografts



# PharmAust: Clinical Programs

## Target Activity Timelines

	Sept 2021- March 2022	March 2022	April 2022	October 2022
<b>Activity</b>	Ongoing Pet Dog Cancer Trials	Phase 3 Pet Dog Cancer Trials	FightMND and COVID19 Human Trials	Human Cancer Trials
<b>Indication</b>	Lymphoma	Lymphoma	ALS/MND, SARS-CoV2 infections	Pancreatic, Gastric, Esophageal
<b>Source</b>	Existing Mpl; Existing Catalent tablets	Existing Mpl; New Syngene tablets	New Mpl; New Catalent tablets	New Mpl; New Syngene tablets
<b>Trial Phase</b>	Phase II/III	Phase III	Phase I/II	Phase I/II
<b>Tablet No</b>	~ 12 000	~ 25 000	~ 11 000	~ 25 000
<b>Treatment</b>	CHOP, pred and Mpl	e.g. CHOP, pred and Mpl	MND: Riluzole COVID: e.g. Remdesivir	e.g. Gemcitabine e.g. Platinum drugs
<b>General Market size</b>	US\$ 500M – 1000M	US\$ 500M – 1000M	MND: US\$ 1B COVID: US\$ 7.7B	US\$ 125B

# EXPERIENCED BOARD AND EXECUTIVE

## Dr Roger Aston, Executive Chairman & CEO

- > 35 years experience in the pharmaceutical & healthcare industries
- Director or chairman on a number of boards carrying out late-stage drug development
- Former CEO of Mayne Pharma Ltd

## Robert Bishop, Executive Director

- > 35 years experience in corporate finance & equity capital markets
- Lawyer & investment banker

## Sam Wright, Finance Director & Co Sec

- > 20 years experience in biotech & healthcare
- Extensive experience in relation to public company responsibilities, including ASX & ASIC compliance, corporate governance & statutory financial reporting

## Neville Bassett, Non-Executive Director

- Member of the Order of Australia (AM)
- > 35 years working in accounting, finance & stockbroking
- Chairman of Westar Capital Ltd

## Dr Richard Mollard, Chief Scientific Officer (CEO Pitney Pharmaceuticals)

- > 25 years national and international pharmaceutical and biotech
- CEO of subsidiary Pitney Pharmaceuticals Pty Limited
- Honorary Fellow, Faculty of Veterinary and Agricultural Science, University of Melbourne