

# Broker Presentation

**April 2015**

**ASX: PAA**

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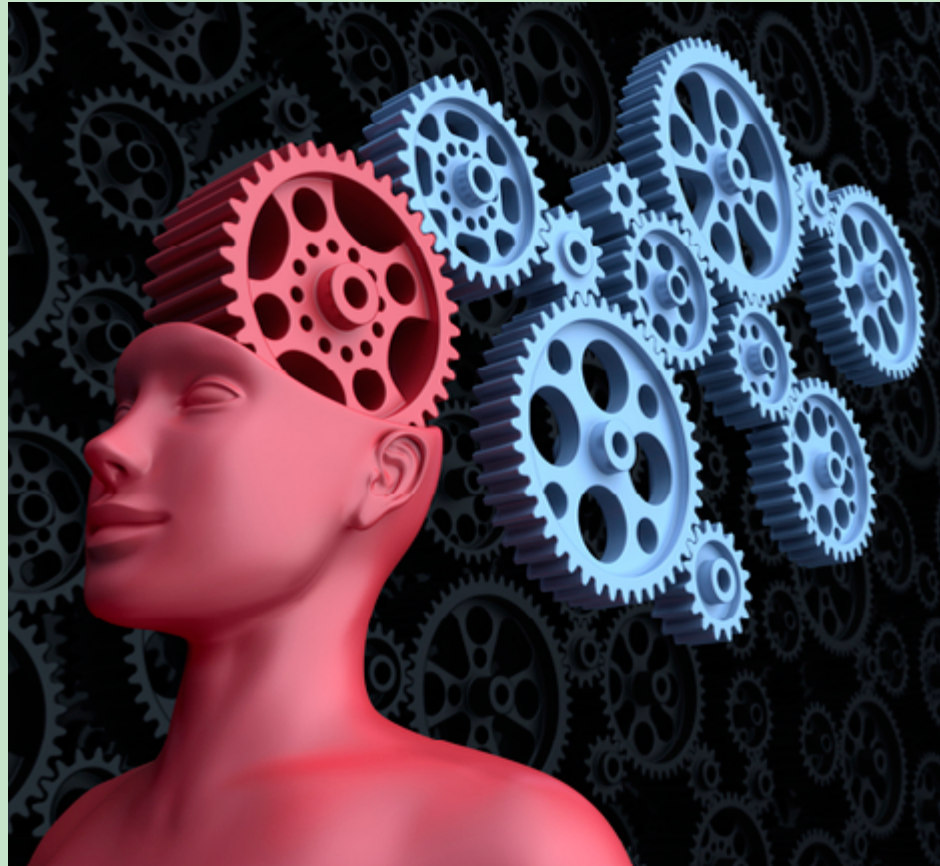
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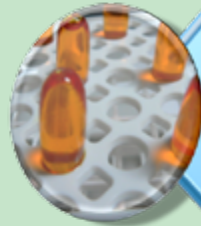


# PHARMAUST LIMITED

...Fast tracking leading edge technology in cancer therapy...



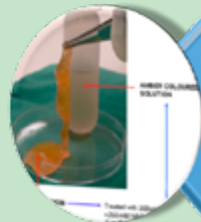
# PharmAust Cancer Programs



**MPL** Breakthrough New Cancer Therapy Drug Class with Reduced Side-Effects



**Albendazole** Reformulation and use of an Approved Drug for Cancer



**Mucin** Dissolution Sensitizing Highly Resistant Cancers to Chemotherapy

# PHARMAUST LIMITED CORPORATE STRUCTURE



# PharmAust -> Challenging Limitations in Cancer Therapy

- Drug Toxicity
- Drug Resistance
- Cancer Recurrence
- Poor Tumour Targeting
- Poor Diagnosis -> Patients Progressed at Treatment
- Optimising Treatment
- Survival vs Quality of Life



# PHARMAUST STRATEGY

Oncology Focus

Leading New Drug for Cancer Management

Phase II Clinical Stage

Strategic Partnerships with two Global Corporations

- Targeting oncology applications of well established drugs and piggy-backing" on substantive programmes of major pharmaceutical companies
- Engaging a leading clinical oncology units (St George Hospital, Sydney) and The Royal Adelaide Hospital
- Granted patents or patent filings (either owned outright or jointly with partner)
- Relationships with major global corporations
- Use of CROs and external R&D suppliers

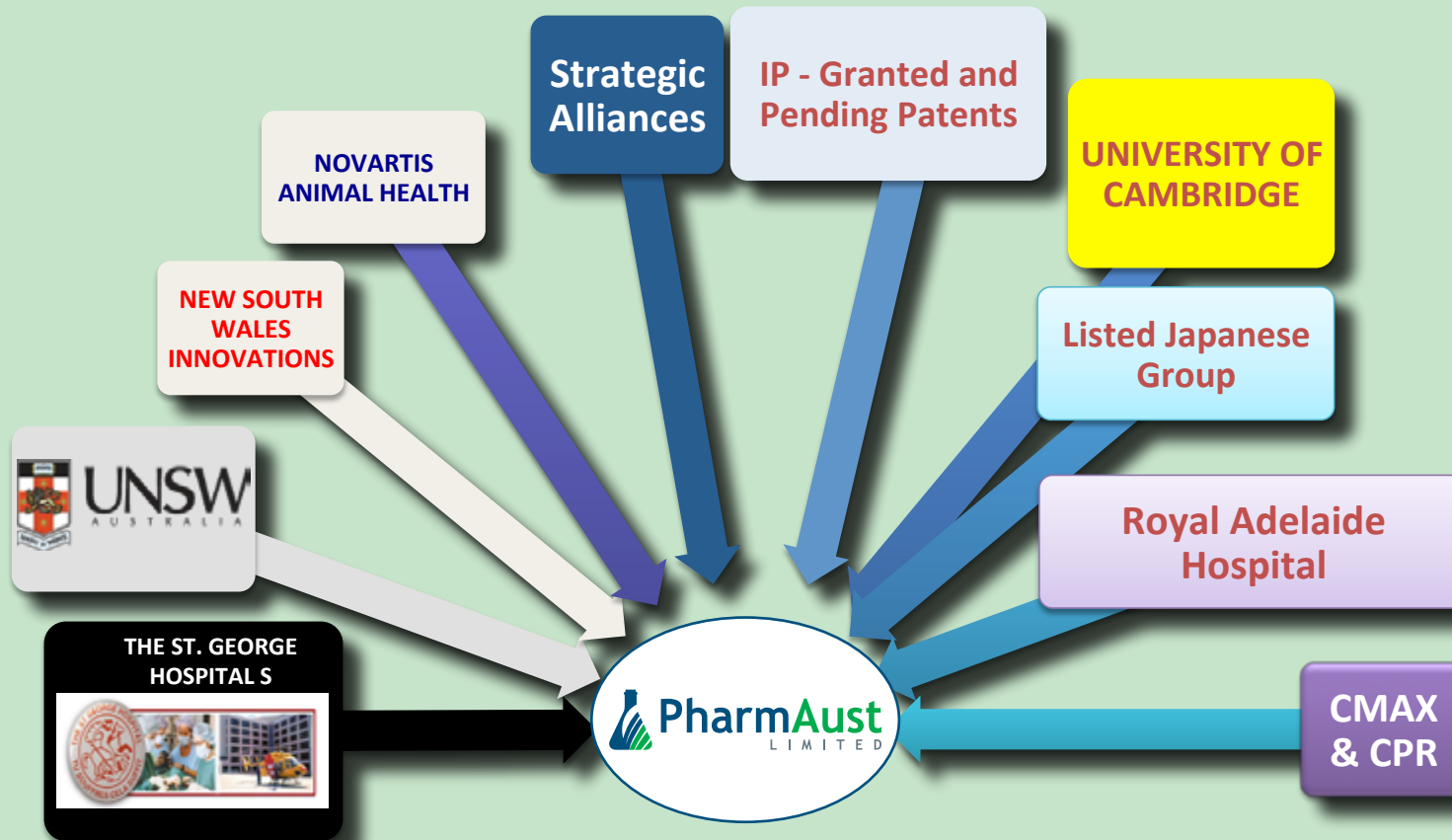


# Targeted Therapies for Cancer

Recent Examples of New Targeted Therapies				
DRUG	TARGET	PEAK ANNUAL SALES	PATIENT COST	COMPANY
HERCEPTIN + PACLITAXEL	BREAST CANCER (HER2-NEU)	\$6.5Bln	\$60,000/ANNUM	GENENTECH/ ROCHE
GLEEVEC	MULTIPLE CANCERS TYROSINE KINASE	\$4.7Bln	\$92,000/ANNUM	NOVARTIS
AVASTIN + 5FU	COLON, RENAL, OVARIAN CANCER VEGF INHIBITOR	\$2.6Bln	\$100,000/ANNUM (US)	GENENTECH
ABRAXANE + GEMCITABINE	PANCREATIC CANCER	\$800M	\$60,000/ANNUM	CELGENE



# PharmAust Limited Foundations, Relationships and Commercial Cornerstones





# **MONEPANTEL (MPL or PPL-1)**

1. MPL is a new class of anticancer drug
2. MPL can act alone or in combination with standard of care chemotherapy for treatment of cancer
3. Pharmaust has the IP for MPL assigned from the University of NSW



# MONEPANTEL'S UNIQUE PROPERTIES

- ❖ Unlike most cancer drugs, MPL has virtually little or no toxicity based on the toxicology undertaken by Novartis Animal Health
- ❖ MPL has shown a high degree of selectivity in killing cancer cells over normal cells in culture
- ❖ Studies in animal models show potent tumour regression without toxicity as measured by weight loss
- ❖ MPL demonstrates a potent synergistic action with many cytotoxic drugs used in "Standard of Care"



# EFFECT OF MPL ON TUMOUR-BEARING MICE

Dose-finding study of IP administered Drug in nude mice bearing Subcutaneous ovarian tumours (OVCAR-3), SC / IP



Control Group VEHICLE



Low dose Group  
MPL (25 mg/kg)  
DRUG



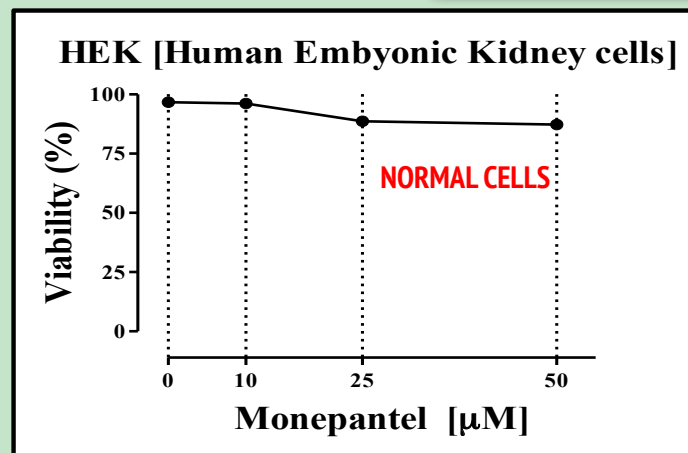
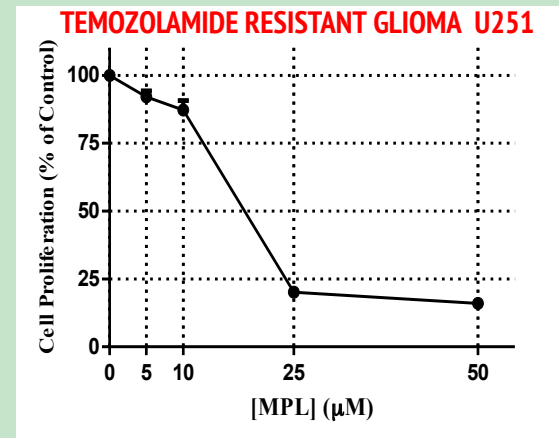
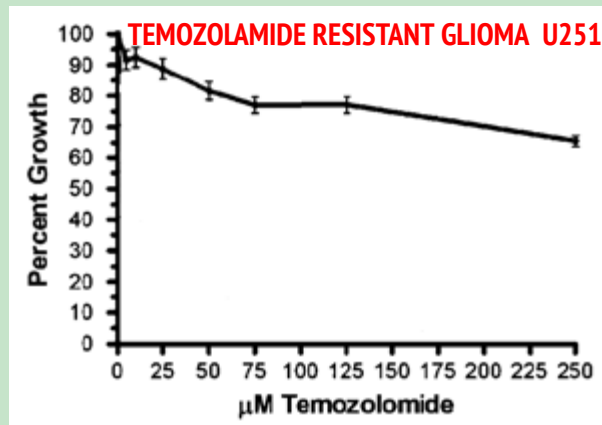
High dose group  
MPL (50 mg/kg)  
DRUG

# Monepantel Kills Drug Resistant Cancer Cells

- Temozolamide resistant cancer cells are effectively killed by MPL
- MPL has little toxicity on “Normal” Human Embryonic Kidney Cells
- MPL offers Treatment Options either singularly or with “Standard of Care”



# EFFECT OF MPL ON CANCER CELLS



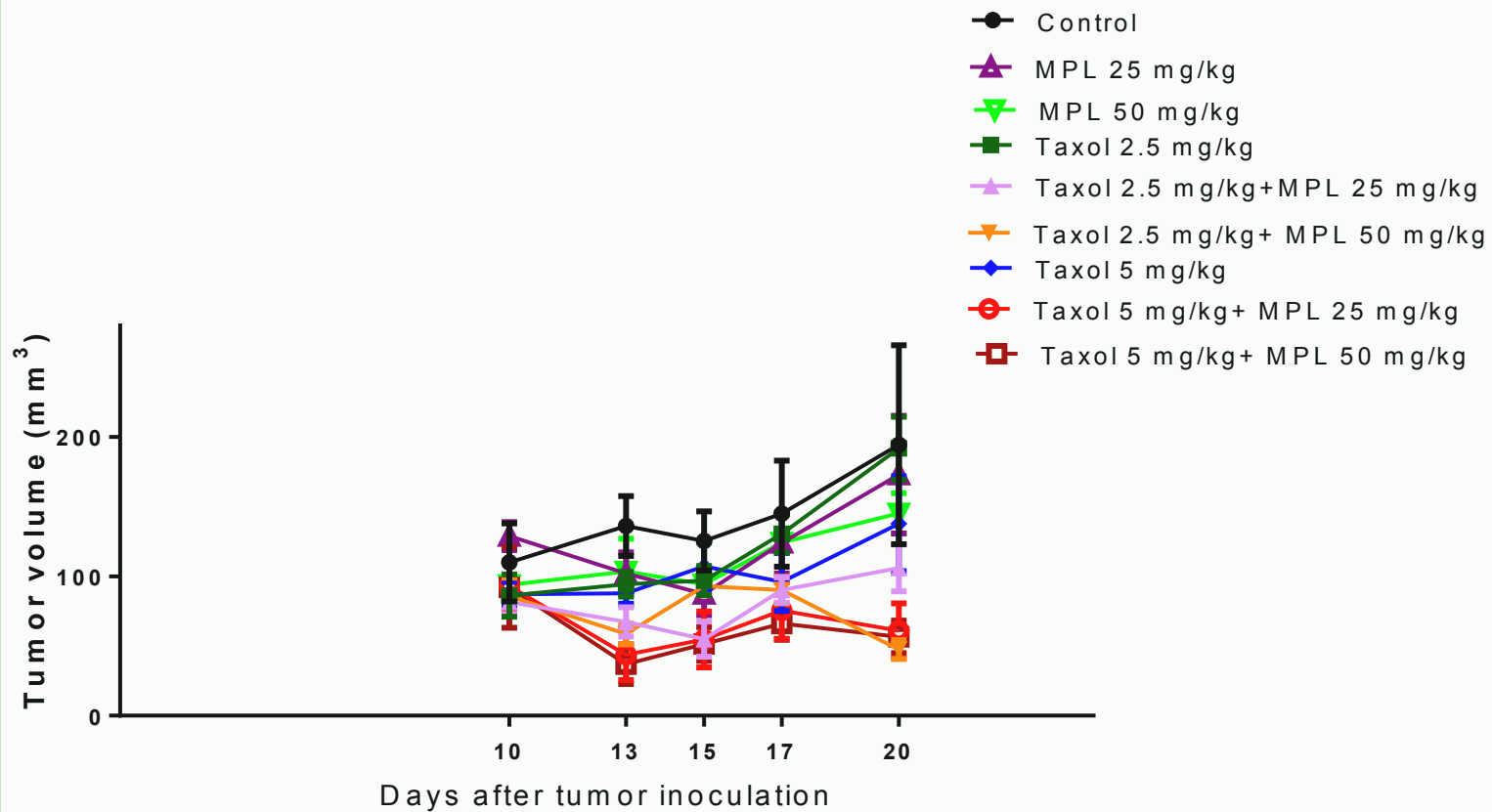
# SYNERGY BETWEEN AMINOACETONITRILES AND CYTOTOXIC DRUGS

- Selective Synergy between MPL and cytotoxic drugs on “cancer” cells
- Little synergy between MPL and cytotoxic drugs on “normal” cells
- Opportunity for “New Class” of drug for cancer
- Therapy in conjunction with standard clinical practice
- Optimization of Synergy



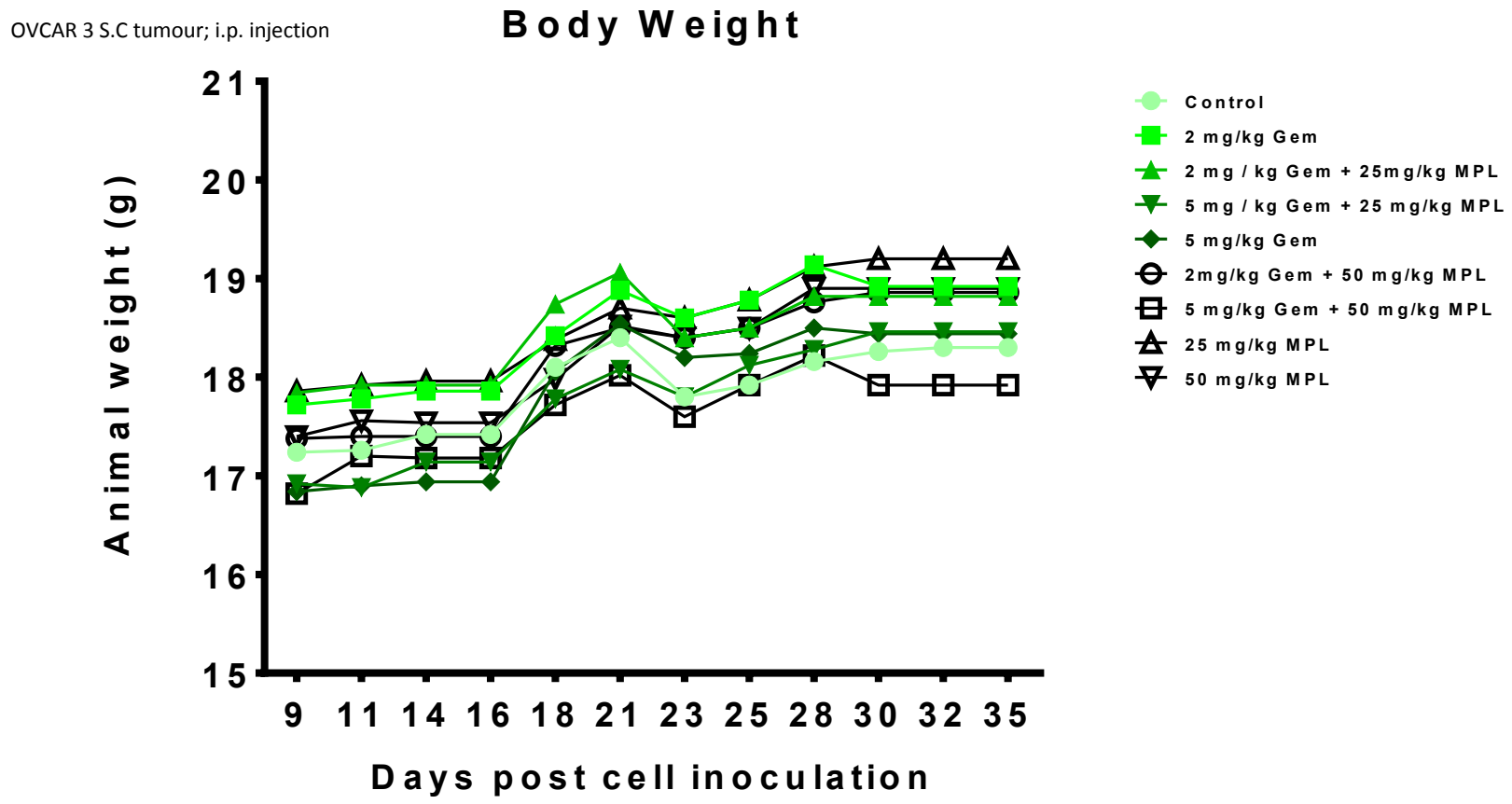


# Synergy with Taxol



Comparison of tumour volume V.s. treatment time in mice treated with MPL/ Taxol alone or in combination (after 5 doses).

# Lack of PPL-1 Toxicity as Measured by Weight Loss



# Human Safety Trial - Monepantel

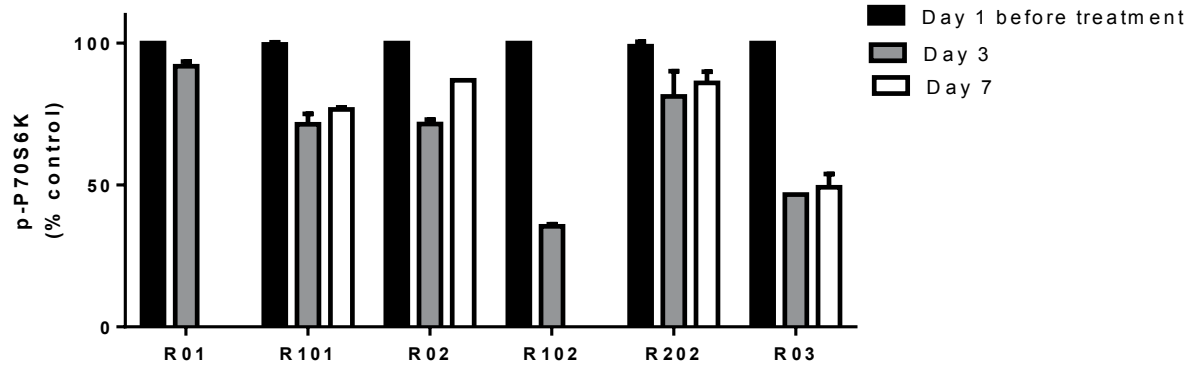
## ACCELERATED ENTRY INTO MAN DUE TO EXTENSIVE TOXICOLOGY BY GLOBAL MAJOR

- Patient #1 -> Discontinued after 3 days (drug-unrelated death)
- Patient #2 -> Completed 28 days
- Patient #3 -> Discontinued after 13 days (urinary tract infection)
- Patient #4 -> Discontinued after 4 days (did not wish to continue)
- Patient #5 -> Completed 28 days
- Patient #6 -> Completed 28 days

**Successful Demonstration of Safety and Indication of Clinical Value by Suppression of Key Cancer Marker**

# p70S6K

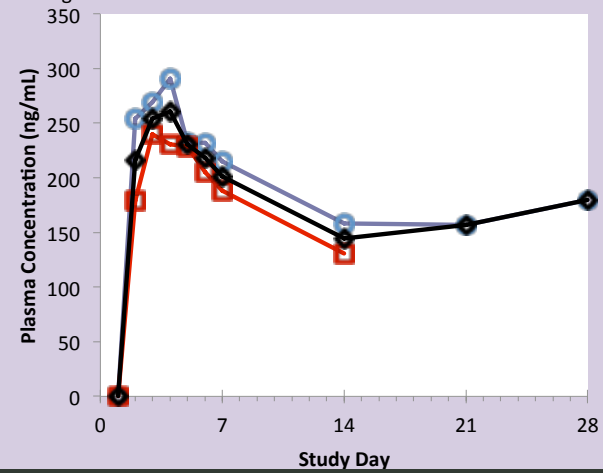
Determination of p-P70S6K in PBMC of patients treated with MPL



Significance at Day 3  $p < 0.0005$

- MPLS, R101
- MPLS, R02
- ◆ MPLS. Average

## PK – BLOOD LEVELS



# Target Markets

Monepantel offers a new therapeutic regimen with or without standard Chemotherapy. The current Chemotherapy market is US\$42 billion/annum.



# Canine Trial

## No Adverse Events Noted

- Four Dogs Received MPL (25mg/kg) compassionately
- Three Dogs treated with the lowest dose of MPL (25mg/kg) as part of trial

Tasteless Formulation  
of MPL in Soft- Gel  
Capsules



# Albendazole for the Treatment of Ascites

Two Clinical Trials Completed Showing:

1. Maximum tolerated dose
2. Benefits in Localised Therapy in the abdomen
3. Significant Inhibition of VEGF



# Albendazole Ascites I

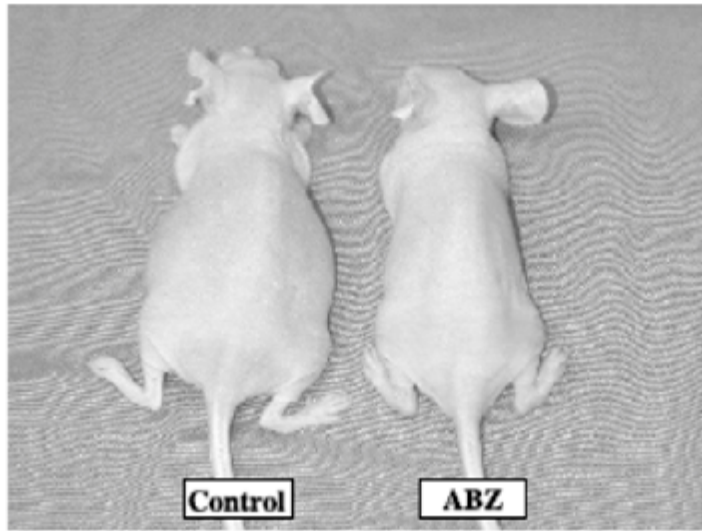


Fig. 1. Effect of albendazole (ABZ) on ascites development. Nude mice inoculated i.p. with OVCAR-3 cells were left to develop ascites and then randomly assigned to one of control or albendazole treatment groups ( $n = 10$  per group). Whereas all mice in the control group developed overt ascites, there were no macroscopic signs of ascites formation in albendazole-treated mice.

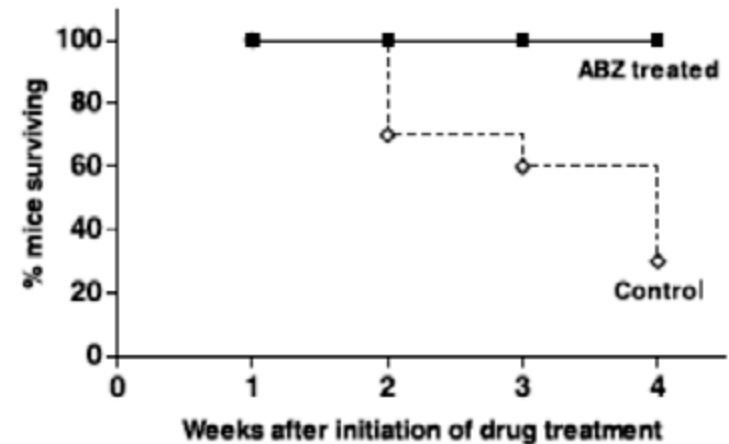
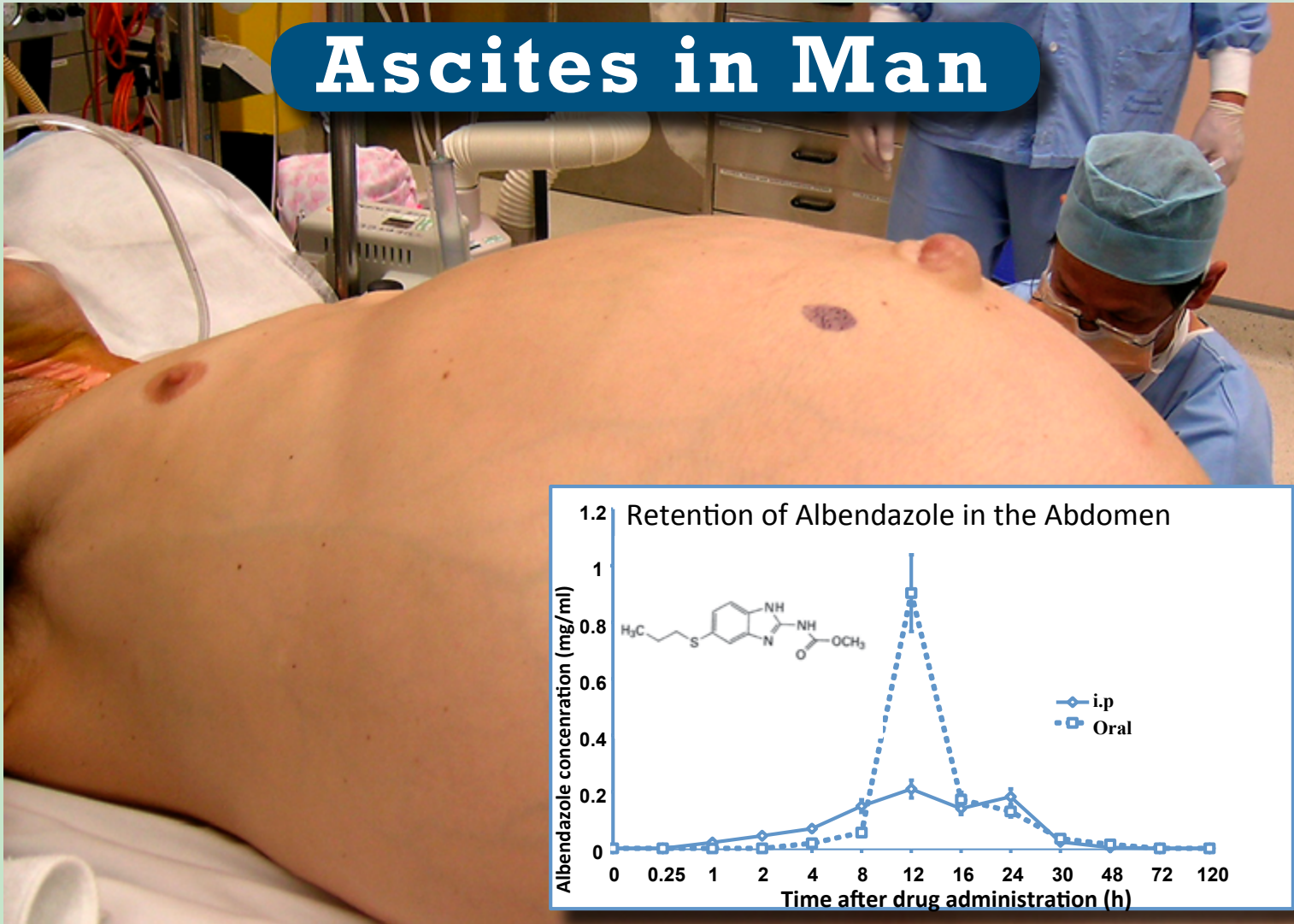


Fig. 3. Survival curve showing the effect of albendazole (ABZ) on survival. Whereas for all animals the intended duration of treatment was 4 weeks, mice (10 per group) were euthanized if due to ill health, they were expected to become moribund within a short time. Survival was calculated as the number of days lapsed between initiation of treatment and euthanasia, and % mice surviving was the number of animals remaining in each group ( $\times 10$ ) at the end of each week following initiation of treatment.

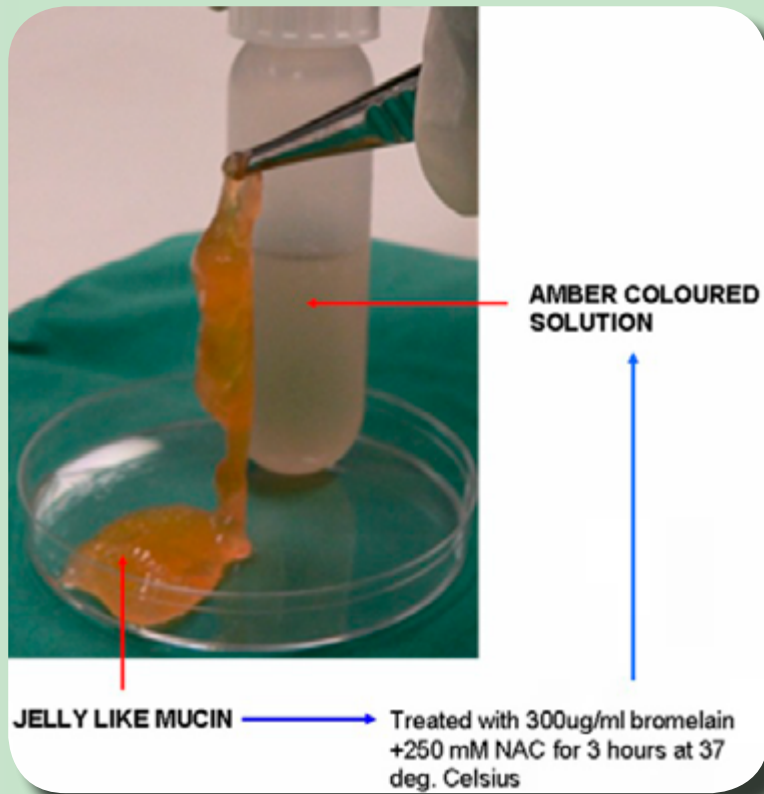


# Ascites in Man



Tumour Growing in the Abdomen and producing large quantities of Ascites fluid which distends the abdomen and reduces life expectancy

# Mucin Dissolution



Efficacy of a Novel Mucolytic Agent on Pseudomyxoma Peritonei Mucin with Potential for Treatment through Peritoneal Catheters.

# EPICHEM

Epichem is a profitable wholly owned subsidiary of PharmAust which provides services in synthetic and medicinal chemistry to the drug discovery and pharmaceutical industries. Epichem turnover is \$2.0M/annum. With two state-of-the-art laboratories in Perth and Melbourne, Epichem serves an international clientele ranging from small operations to large multinational pharmaceutical companies. Epichem's ability to excel in a highly competitive global marketplace was recently recognized when in 2010 it won an Australian Export Award. It has achieved this by combining a world-class team (12 Ph.Ds) of highly innovative chemists and a management committed to customer service.

# Contact Details

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