



# Investor Presentation

Dr. Marie Roskrow, CEO & Managing Director  
September 2014

**ASX: PAB**

# Safe Harbour Statement

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This presentation contains forward-looking statements that are subject to risks and uncertainties. Such statements involve known and unknown risks that may cause the actual results, performance or achievements of Patrys Limited to be materially different from the statements in this presentation.

Actual results could differ materially depending on factors such as the availability of resources, the results of clinical studies, the timing and effects of regulatory actions, the strength of competition and the effectiveness of the Company's patent protection.

# Highlights of 2014

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## 3<sup>rd</sup> Quarter (July – September):

- Released details of the planned PAT-SM6 & carfilzomib Phase Ib/IIa clinical trial due to start end of 2014
- Reported significant progress on all preclinical programmes
- Progressed the GMP manufacturing of PAT-SM6

## 2<sup>nd</sup> Quarter (April – June):

- Reported the granting of a third US patent for PAT-SM6
- Released clinical data showing that a patient with end-stage multiple myeloma responded positively to treatment with PAT-SM6 in combination with other marketed drugs
- Reported the first plant-based production of PAT-SM6

## 1<sup>st</sup> Quarter (January to March):

- Reported final data from successful Phase I/IIa PAT-SM6 single-agent clinical trial
- Rights issue completed: Total fundraising of \$7.7 million
- PAT-SC1 10yr follow-up clinical trial data in patients with gastric cancer published

# Corporate Overview

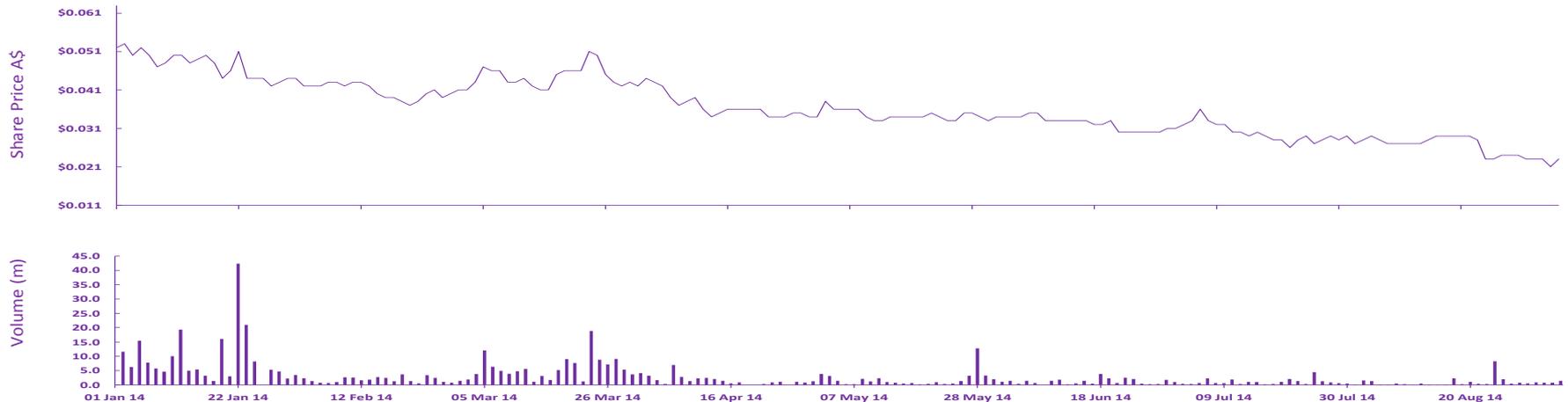
## KEY STATS & FINANCIALS – 17 SEPT. 2014 (AUD\$)

ASX Code	PAB
Share price	\$0.021
52 week high	\$0.092
52 week low	\$0.021
Shares on issue	697,060,986
Market capitalisation	\$14.6m
Cash @ 30/6/14	\$8.6m
Funding Since Listing	\$54m

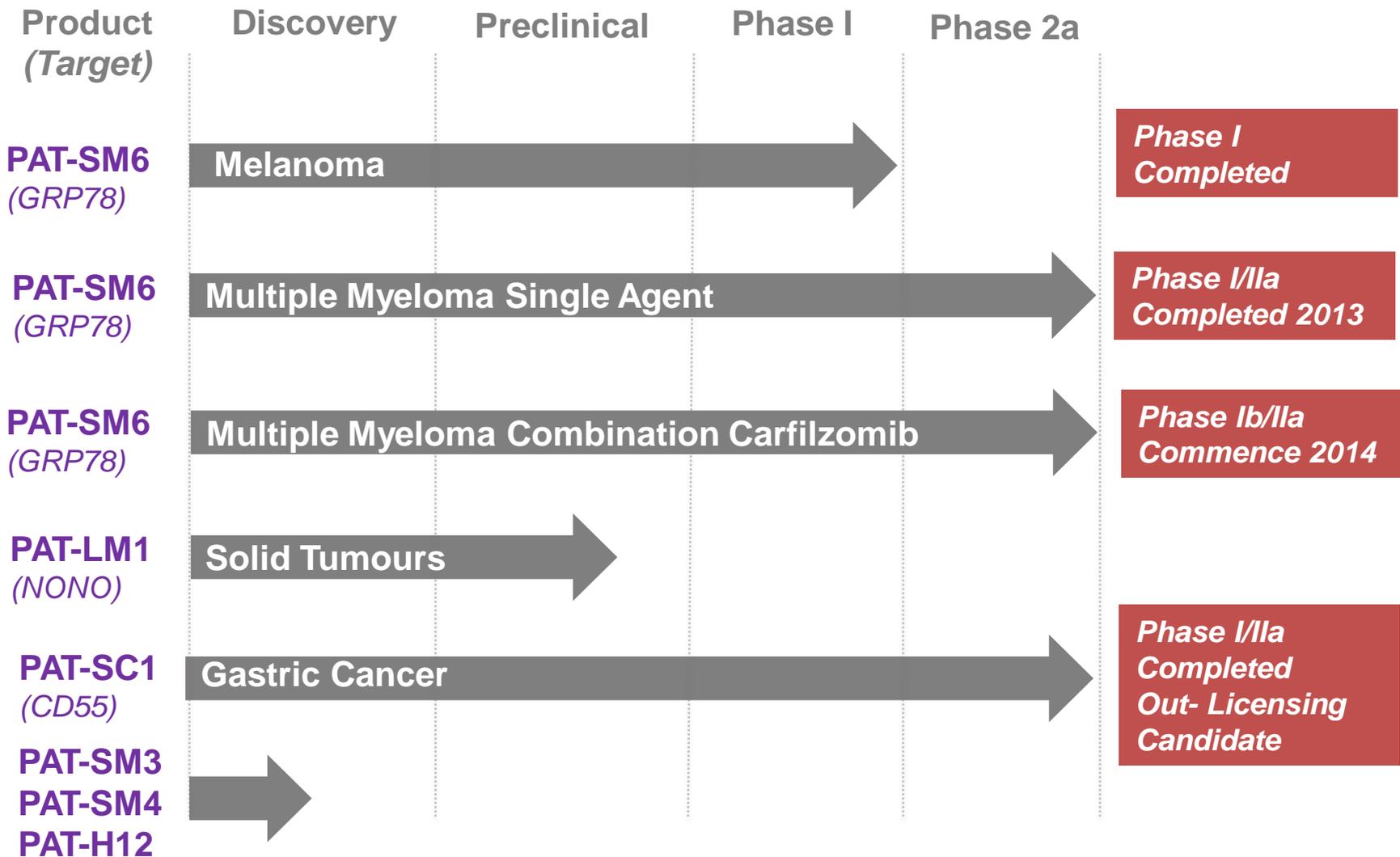
## MANAGEMENT & BOARD

<b>Marie Roskrow, BSc</b> (Hons), MBBS, PhD	<i>Managing Director, CEO</i>
<b>Roger McPherson,</b> CPA, GAICD	<i>CFO, Company Secretary</i>
<b>Frank Hensel, PhD</b>	<i>Vice President R&amp;D</i>
<b>John Read, BSc</b> (Hons), MBA, FAICD	<i>Non-Executive Chairman</i>
<b>Mike Stork, BBA</b>	<i>Non-Executive Director</i>
<b>Suzy Jones</b>	<i>Non-Executive Director</i>

## CURRENT YEAR SHARE PERFORMANCE



# Pipeline



**PAT-SM6**



# Patrys' Lead Antibody: PAT-SM6

## PAT-SM6:

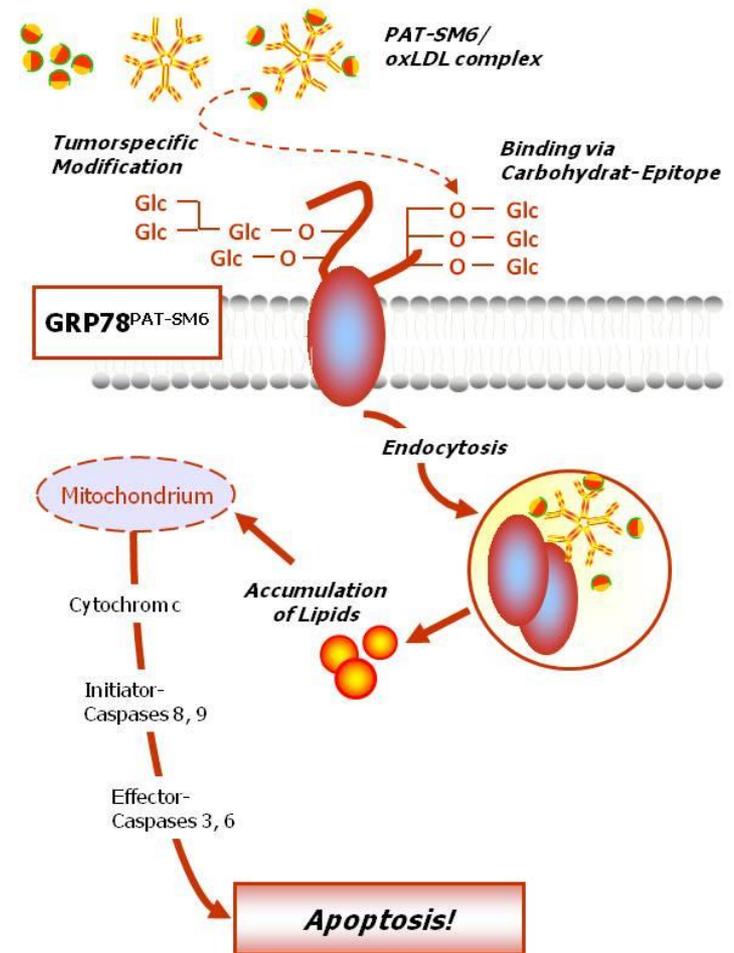
- IgM isotype,  $\lambda$ -light chain
- Isolated from stomach cancer patient
- Targets tumour specific epitope on GRP78
- Binds also to oxidised LDL and VLDL

## Mode of Action:

- Internalisation upon binding of oxidised LDL & GRP78<sup>PAT-SM6</sup>
- Internalisation triggers apoptosis

## *In vivo* & *In vitro* Reactivity:

- Effective in multiple xenograft models
- Expression data show specific expression in wide range of tumours incl. melanoma and myeloma



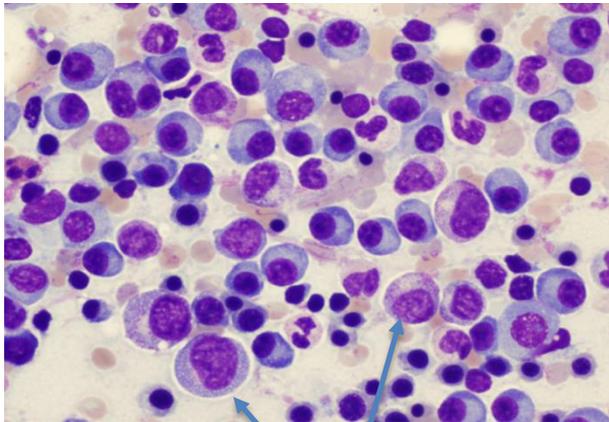
# Multiple Myeloma – Opportunity

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- Cancer of the plasma cells in bone marrow. Cells grow out of control and form tumours in solid bone, cause damage to other organs
- Estimated to be more than 220,000 cases worldwide and incidence increasing
- 5 year survival of ~30%
- Market expected to increase from ≈\$8B (2013) to >\$15B (2018)
- Market currently dominated by 3 products:
  - Revlimid (net sales \$4.28B 2013)
  - Velcade (net sales \$3.77B 2013)
  - Kyprolis® (carfilzomib) (net sales \$272M 2013)
- Several MAbs currently in clinical development but none approved to date. Likely to be used in combination therapies
- Significant interest in MM from both large pharmaceutical and biotechnology companies

# Multiple Myeloma – Pathology

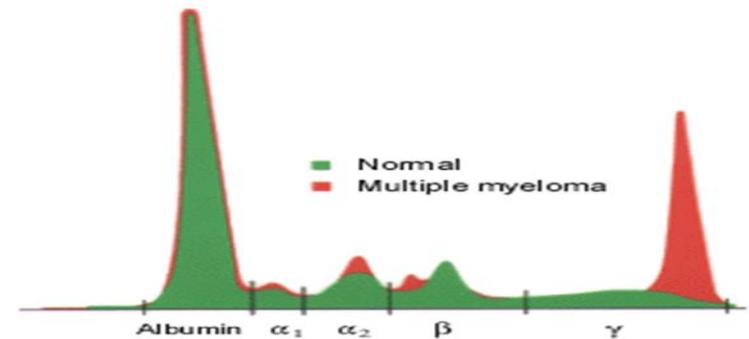
- Abnormal plasma cells (myeloma cells) secrete lots of “useless” antibodies (M proteins)
- Myeloma cells crowd out other blood cells resulting in anaemia, thrombocytopenia (bleeding) and leucopenia (infections)



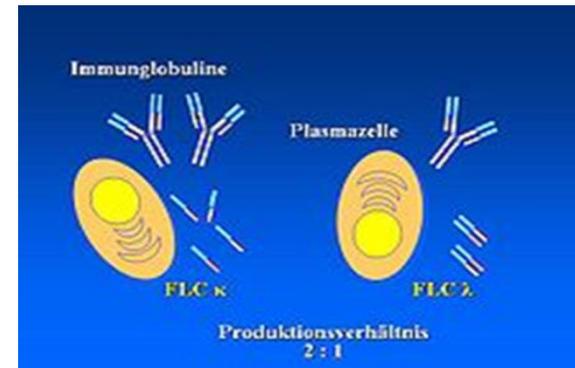
Myeloma cells

- Abnormal proteins in serum detected by electrophoresis

**Serum Protein Electrophoresis**

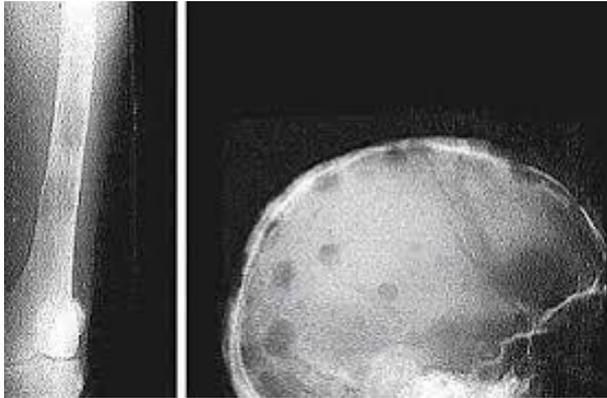


- Abnormal proteins (Bence Jones) detected in urine



# Multiple Myeloma – Clinical Presentation

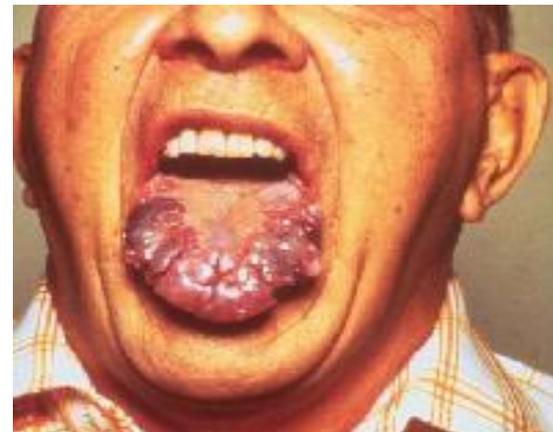
- Bone disease and hypercalcaemia



- Abnormal protein deposits



- Bone marrow failure and infections



# Therapies for Multiple Myeloma

## Proteasome inhibitors

- Bortezomib (Velcade)
- **Carfilzomib (Kyprolis)**

## Immunomodulators (IMiDs)

- Lanalidomide (Revlimid)
- Pomalyst (Pomalidomide)
- Thalidomide

## Chemotherapeutics

- Melphalan
- Cisplatin
- Cyclophosphamide
- Doxorubicin

## Stem cell transplantation

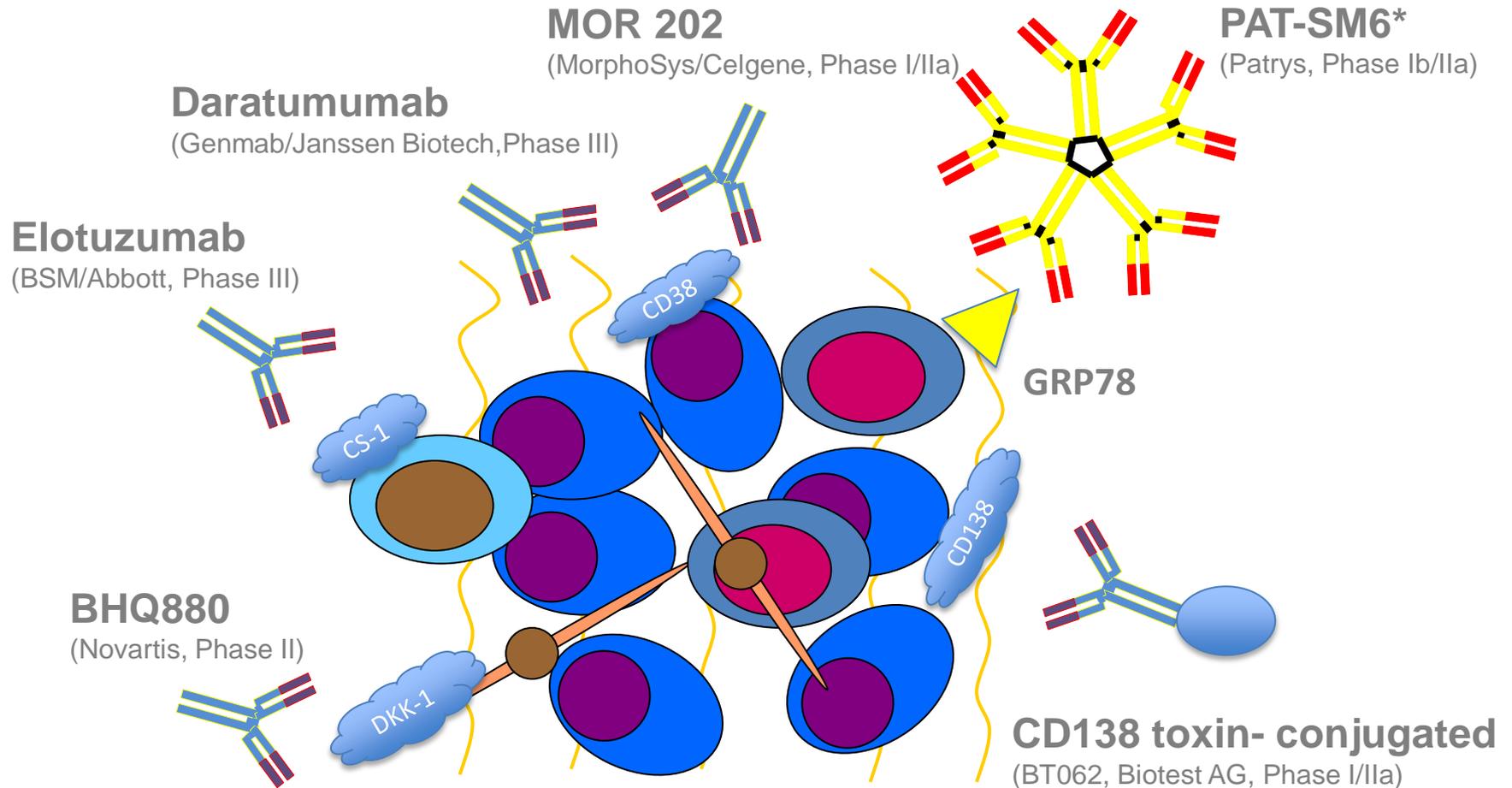
- Autologous
- Allogeneic

## Clinical studies

- Small molecules
- Antibodies, peptides
- Immunotherapeutics



# Antibodies in Clinical Trials for MM



## Antibodies in all stages of clinical development

\*Upcoming trial to begin end of 2014 with PAT-SM6 in combination with carfilzomib and dexamethasone

# PAT-SM6 Monotherapy Trial

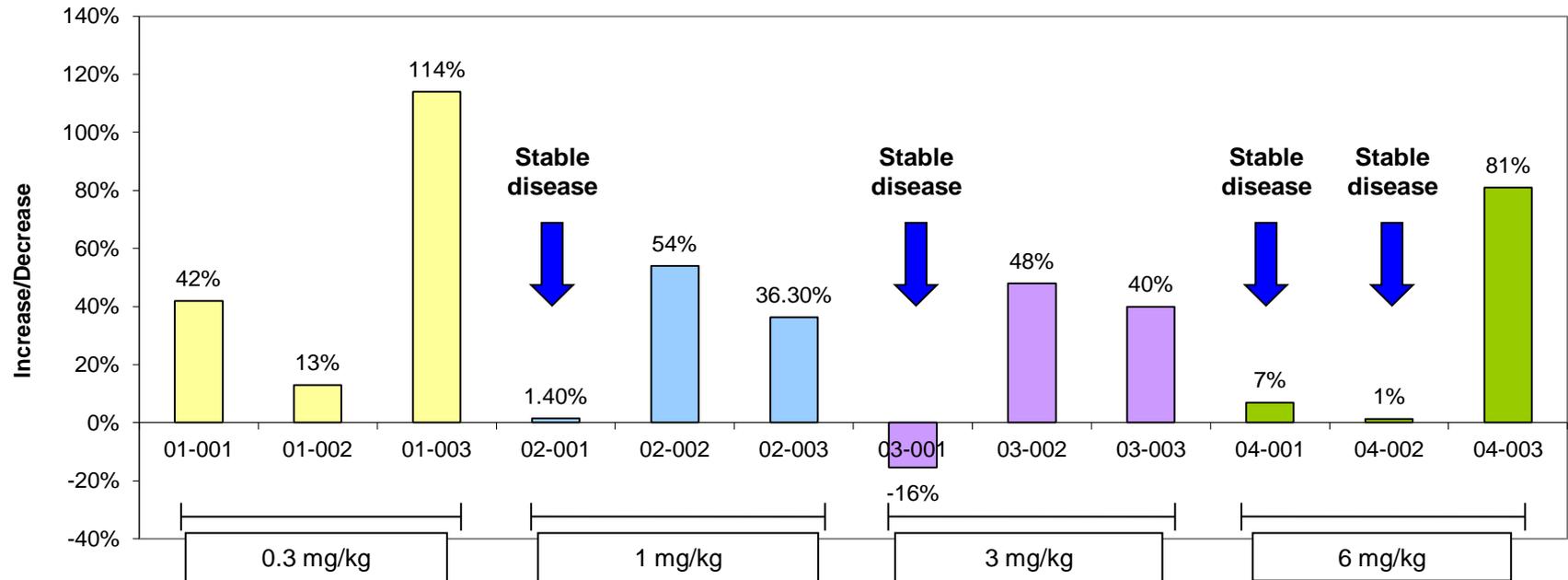
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- 12 patients treated with 4 doses PAT-SM6 in 4 dose cohorts (0.3mg/kg, 1mg/kg, 3mg/kg and 6mg/kg/dose) evaluated at day +36 (end of trial)
  - 10 male, 2 female: median age 71 yrs
  - Received average 5 prior lines of therapy
- PAT-SM6 safe in all patients. No dose limiting toxicity (DLT), no related serious adverse events (SAE) and no related adverse event grade  $\geq 4$
- No evidence of immunogenicity. PK analysis showed half-life of 7 hrs
- PAT-SM6 specifically targets MM cells *in vivo* and stimulates significant immune responses
- 4/12 patients had stable disease (day +36 post treatment) with a significant reduction in protein M levels in the peripheral blood
- Patients who received prior treatment with proteasome inhibitors responded best to PAT-SM6
- Mean time to next therapy is 51 days (clinically significant). Two patients had stable disease for >130 days post treatment

# Clinical Data

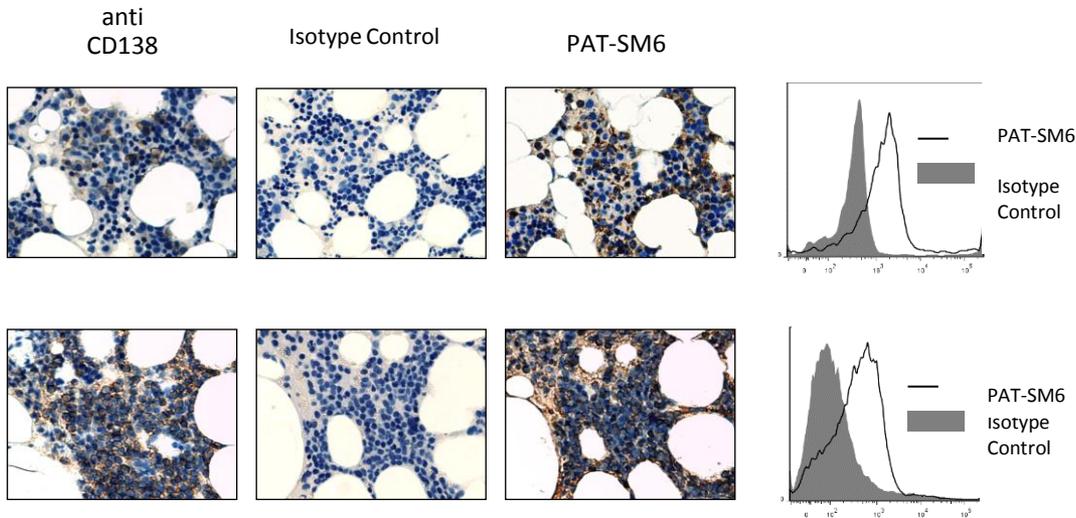
## Preliminary Efficacy

### M Protein Changes from Baseline (%)



4 patients showed stable disease according to the IMWG criteria

# Clinical Data



**Immunohistochemical staining with PAT-SM6 on paraffin embedded tumour biopsies from MM patients**

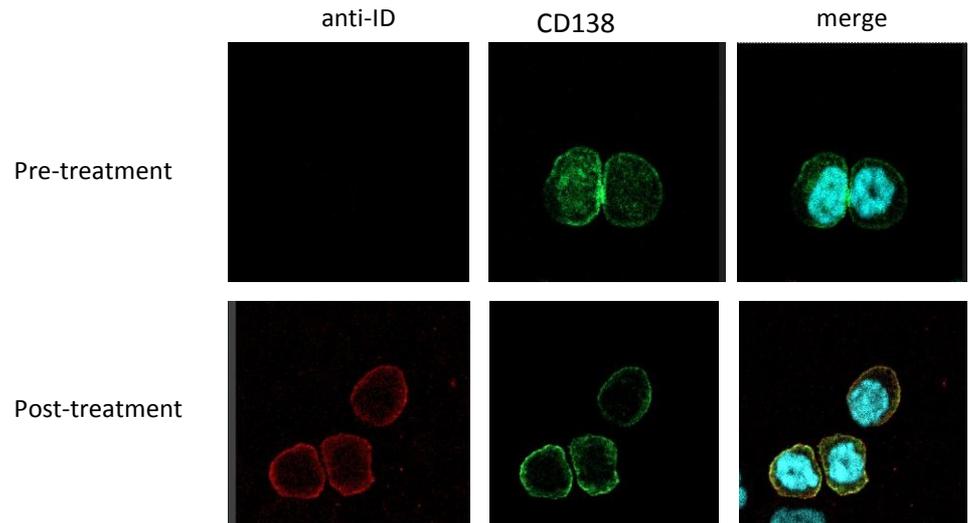
The positive control (anti-CD138) and PAT-SM6 bound to patient tissues

**Flow cytometry on MM patient bone marrow**

Cells from MM patients displayed cell surface binding for PAT-SM6 suggestive the target (GRP78) is present on the MM cells

## Detection of PAT-SM6 on circulating MM cells

Before and after dosing, patient blood was collected and circulating MM cells were purified using CD138 magnetic beads. PAT-SM6 binding was confirmed using an anti-idiotypic antibody specific for PAT-SM6 (anti-ID) by confocal microscopy. In the post-treatment sample, red stained MM cells indicate the presence of PAT-SM6. CD138 was used as a positive control



# PAT-SM6 & Carfilzomib Combination Trial

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## Endpoints:

- *Primary:* The safety and tolerability of PAT-SM6, given in combination with carfilzomib and dexamethasone in patients with multiple myeloma who are refractory and/or intolerant to bortezomib, a currently-marketed proteasome inhibitor
- *Secondary:* Overall response rate, duration of response, time to progression and a series of well-established assays will measure immunological and disease parameters

## Design:

- Single-arm study with a 2-stage design: after treating 9 pts in the 1<sup>st</sup> stage, if > 2 respond (a partial response or better), the trial will continue to the 2nd stage and a total of 24 pts will be treated
- Each pt will be treated with up to 4 cycles of PAT-SM6 (6 mg/kg/dose) + carfilzomib (20-27 mg/m<sup>2</sup>) + dexamethasone (40 mg)
- Each cycle will consist of 3 doses of PAT-SM6, 6 doses of carfilzomib and 4 doses of dexamethasone administered over one month

## Timelines:

- Trial projected to start end of 2014 at 2 clinical centres in Germany (Würzburg & Dresden)
- Progressive recruitment of up to 24 pts estimated to take 15-18 months. Data from first 9 patients will be released

# Cell Lines Used in Manufacturing Antibodies

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## PER.C6® Cell Line:

- Patrys uses PER.C6® human cell line for GMP production:
  - Only human cell line commercially available
  - Over 6300 patients have been treated with PER.C6® products – safe profile
  - PER.C6® cell line has received regulatory approval in all major markets
  - PER.C6® derived products have not yet gained marketed approval: Currently in Phase III
  - Capable of producing high yields of both IgM and IgG antibodies. Cost of goods similar to CHO system

## Chinese Hamster Ovary (CHO) Cell Line:

- CHO is a commonly used cell non-human cell line:
  - CHO is more familiar to big Pharma / Biotech companies
  - Numerous antibody therapeutics produced in CHO are on the market
  - Patrys has initiated a feasibility study with CHO cells in order to deliver multiple options to potential partners

**PAT-LM1**



# PAT-LM1 Antibody & Target

## PAT-LM1:

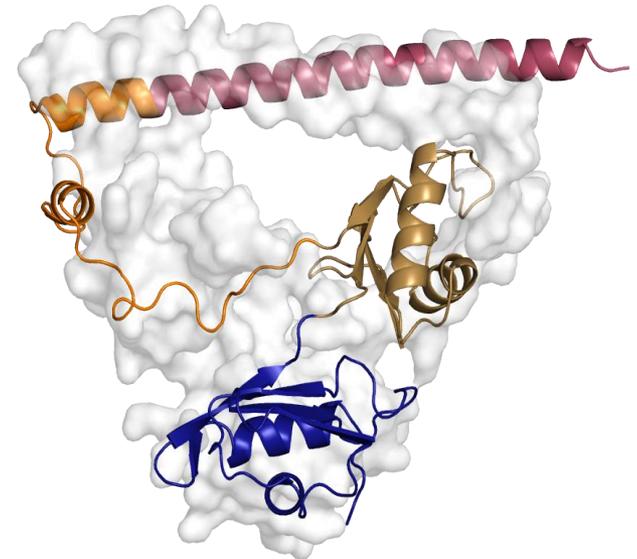
- IgM isotype,  $\lambda$ -light chain
- Isolated from a lung cancer patient
- Targets tumour-specific epitope of surface-expressed NONO (non-POU-domain-containing octomer binding protein)

## Mode of Action:

- NONO mainly found in nucleus: involved in transcriptional & post-transcriptional gene regulation
- Unknown mechanism-of-transport to cell membrane

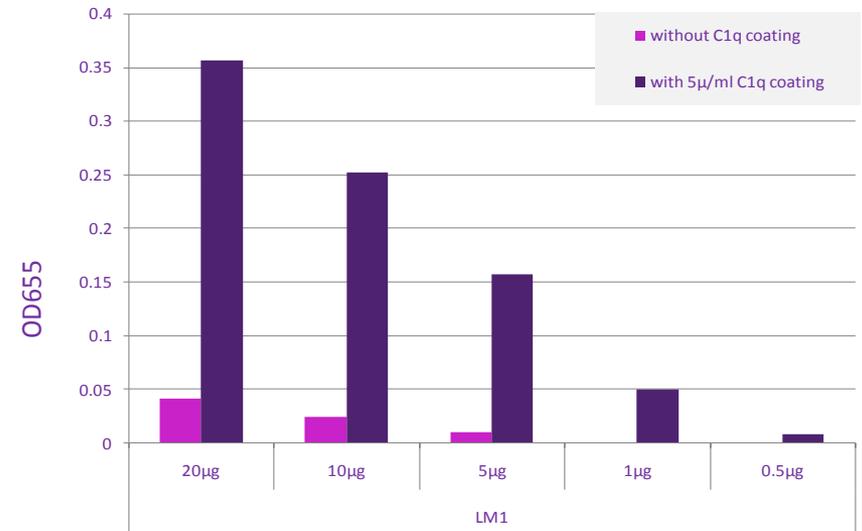
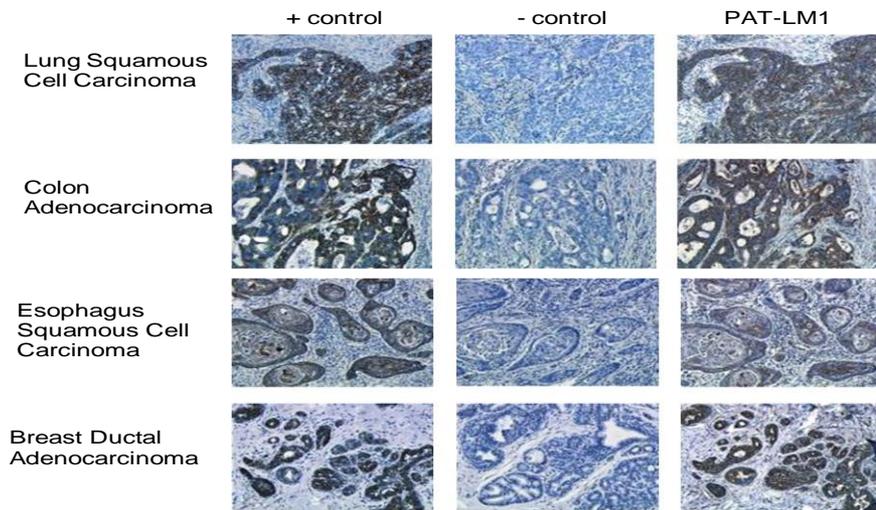
## *In Vivo & In Vitro* Reactivity:

- Effective in several xenograft models
- Expression data show specific expression in a wide range of tumors incl. lung, pancreas, colon, leukaemias



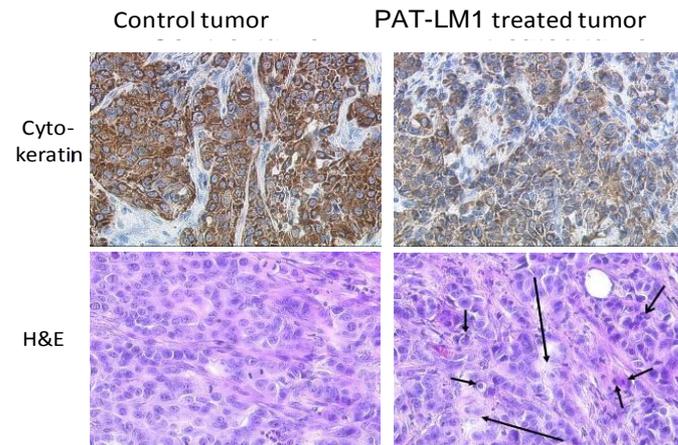
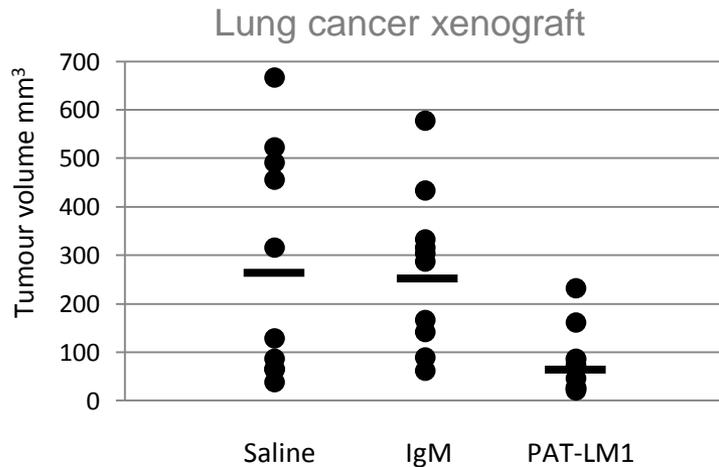
Crystal structure of NONO with PSCP1  
Passon et al PNAS 2012

# PAT-LM1 Preclinical Data



IHC staining with PAT-LM1 on tumour tissues

PAT-LM1 binds C1q, suggestive of CDC



PAT-LM1 reduced tumour volume & induced cell-death

- Apoptotic cells
- Regression
- Necrosis

**PAT-SC1**



# PAT-SC1 (Gastric Cancer)

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## PAT-SC1:

- Pentameric IgM isotype,  $\lambda$ -light chain
- Isolated from a stomach cancer patient
- Targets isoform of CD55 (Decay Accelerating Factor) expressed on surface of multiple types of cancer cells

## POC Trial Results:

- Phase I/IIa (POC) open-label trial conducted 1997-2001 (Germany)
- Safe in 51 pts receiving single 20 mg dose PAT-SC1
- Significant 10 year survival benefit for 30 pts with minimal residual disease (R0) post-surgery vs. untreated pts (historic control)

## Current Stage/Competition:

- Currently in out-licensing process
- Conversion from hybridoma to recombinant PER.C6® cell line completed
- Orphan designation by the FDA for use in gastric cancer
- No other know clinical products targeting CD55

## Intellectual Property:

- Key patent granted in various jurisdictions

# 2014 Key Milestones

Key Milestone	Projected Timing (CY)	
<p><b>PAT-SM6:</b></p> <ul style="list-style-type: none"> <li>○ Final data from monotherapy MM trial</li> <li>○ Complete GMP manufacturing for combination MM trial</li> <li>○ Commence combination Phase Ib/IIa MM trial</li> <li>○ Preclinical and clinical data published</li> </ul>	<p>1Q, 2014</p> <p>2H, 2014</p> <p>4Q, 2014</p> <p>4Q, 2014</p>	<p>✓</p>
<p><b>PAT-LM1:</b></p> <ul style="list-style-type: none"> <li>○ Complete manufacturing process development ready for scale-up</li> <li>○ Preclinical data published</li> </ul>	<p>2H, 2014</p> <p>2H, 2014</p>	
<p><b>PAT-SC1:</b></p> <ul style="list-style-type: none"> <li>○ Preclinical &amp; clinical trial (gastric cancer) data published</li> <li>○ Out-licensing deal</li> </ul>	<p>1Q, 2014</p> <p>2014</p>	<p>✓</p>
<p><b>Discovery Abs:</b></p> <ul style="list-style-type: none"> <li>○ Target work on PAT-SM3, PAT-SM4, PAT-H12</li> </ul>	<p>2014</p>	

# For Further Information

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