



# BBY Life Sciences Conference

Dr. Marie Roskrow, CEO & Managing Director  
November 2013

**ASX: PAB**

# Safe Harbour Statement

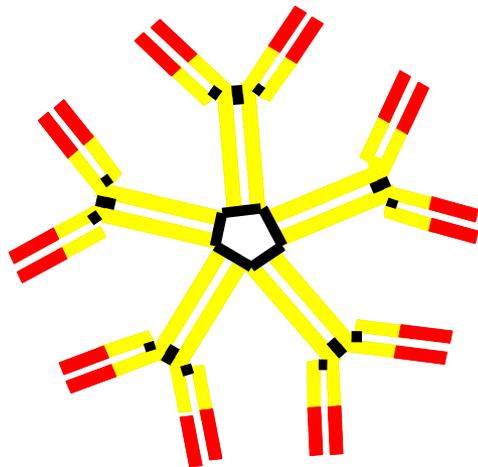
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This presentation contains forward-looking statements that are subject to risks and uncertainties. Such statements involve known and unknown risks that may cause the actual results, performance or achievements of Patrys Limited to be materially different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

# Patrys in 2013

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IgM

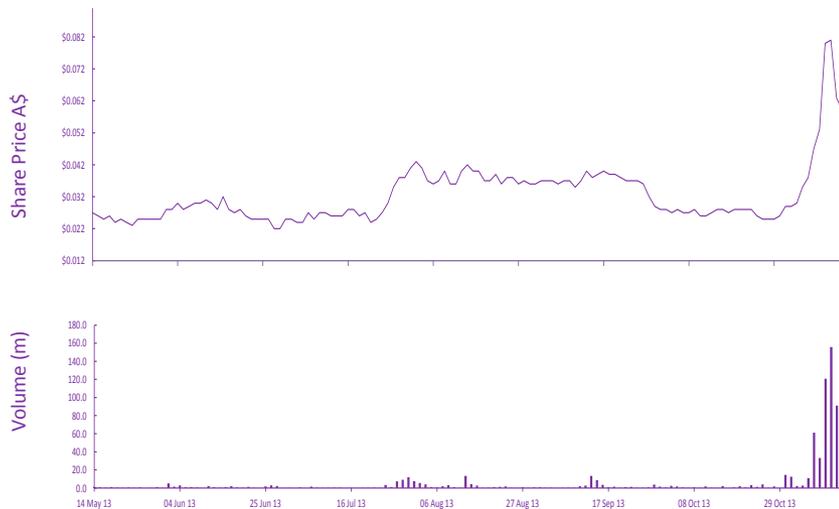
- Oncology-focussed clinical-stage Company
- Deep pipeline of novel cancer-specific IgM monoclonal antibodies
- Treasure-trove of novel cancer targets
- Significant intellectual property portfolio
- Network of Internationally-renowned collaborators
- Experienced Board of Directors and Management Team

# Corporate Overview

## KEY STATISTICS – 15 NOVEMBER 2013 (AUD\$)

ASX code	PAB
Share price	\$0.061
52 week high	\$0.092
52 week low	\$0.024
Shares on issue	524,362,177
Market capitalisation	\$32.0m
Cash 30/9/13	\$3.8m

## SIX MONTH SHARE PERFORMANCE



## RECENT NEWS

Nov. 2013	Key patent granted for PAT-SM6 Rights Issue \$12.5m Clinical trial with Onyx Pharma Key patent granted for PAT-LM1
Oct. 2013	Orphan Drug Status FDA for PAT-SM6
Sep. 2013	Orphan Drug Status EMA for PAT-SM6
Aug. 2013	Final cohort in MM trial commenced
Jul. 2013	ARC Linkage Grant awarded supporting diagnostic development of PAT-SM6
Jun. 2013	Paper published on effectiveness of PAT-SM6 Key patent granted for PAT-LM1
May 2013	Paper published on preclinical work on MM
Apr. 2013	Paper published on preclinical data on PAT-SM6

# Rights Issue - Overview

Offer	<ul style="list-style-type: none"> <li>○ 1 new share for every 2 shares held</li> <li>○ Share Top Up Offer for any shortfall</li> <li>○ Shortfall to be placed within 3 months</li> </ul>								
Pricing	<ul style="list-style-type: none"> <li>○ 5 cents</li> <li>○ 17% discount to 30 day VWAP (6.05 cents)</li> <li>○ 38% discount to last traded price 8.1 cents (12 Nov. 2013)</li> </ul>								
Timing	<ul style="list-style-type: none"> <li>○ Announced – 13 Nov. 2013</li> <li>○ Record – 25 Nov. 2013</li> <li>○ Closing – 11 Dec. 2013</li> <li>○ Allotment – 19 Dec. 2013</li> </ul>								
Issued Shares	<table border="0"> <tr> <td data-bbox="546 862 1566 915">○ Current shares on issue</td> <td data-bbox="1566 862 1860 915">507.4m</td> </tr> <tr> <td data-bbox="546 919 1566 972">○ “In the money” options</td> <td data-bbox="1566 919 1860 972">50.0m</td> </tr> <tr> <td data-bbox="546 976 1566 1029">○ Max. shares under offer</td> <td data-bbox="1566 976 1860 1029">278.6m</td> </tr> <tr> <td data-bbox="546 1033 1566 1100">○ <b>Shares on issue post Rights Issue</b></td> <td data-bbox="1566 1033 1860 1100"><b>836.0m</b></td> </tr> </table>	○ Current shares on issue	507.4m	○ “In the money” options	50.0m	○ Max. shares under offer	278.6m	○ <b>Shares on issue post Rights Issue</b>	<b>836.0m</b>
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○ <b>Shares on issue post Rights Issue</b>	<b>836.0m</b>								
Other	<ul style="list-style-type: none"> <li>○ New shares issued through Rights Issue, Top Up Offer and placement of any shortfall will be fully paid ordinary shares, ranking pari passu with existing shares</li> </ul>								

# Rights Issue – Use of Funds

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## PAT-SM6 Clinical Programme:

- Manufacturing of GMP PAT-SM6 (Onyx Pharma-funded multiple myeloma clinical trial)
- Expand external collaborations; additional preclinical data and intellectual property

## PAT-LM1 Preclinical Programme:

- Scale-up manufacturing to produce GMP material for clinical trial
- Expand external collaborations; additional preclinical data and intellectual property

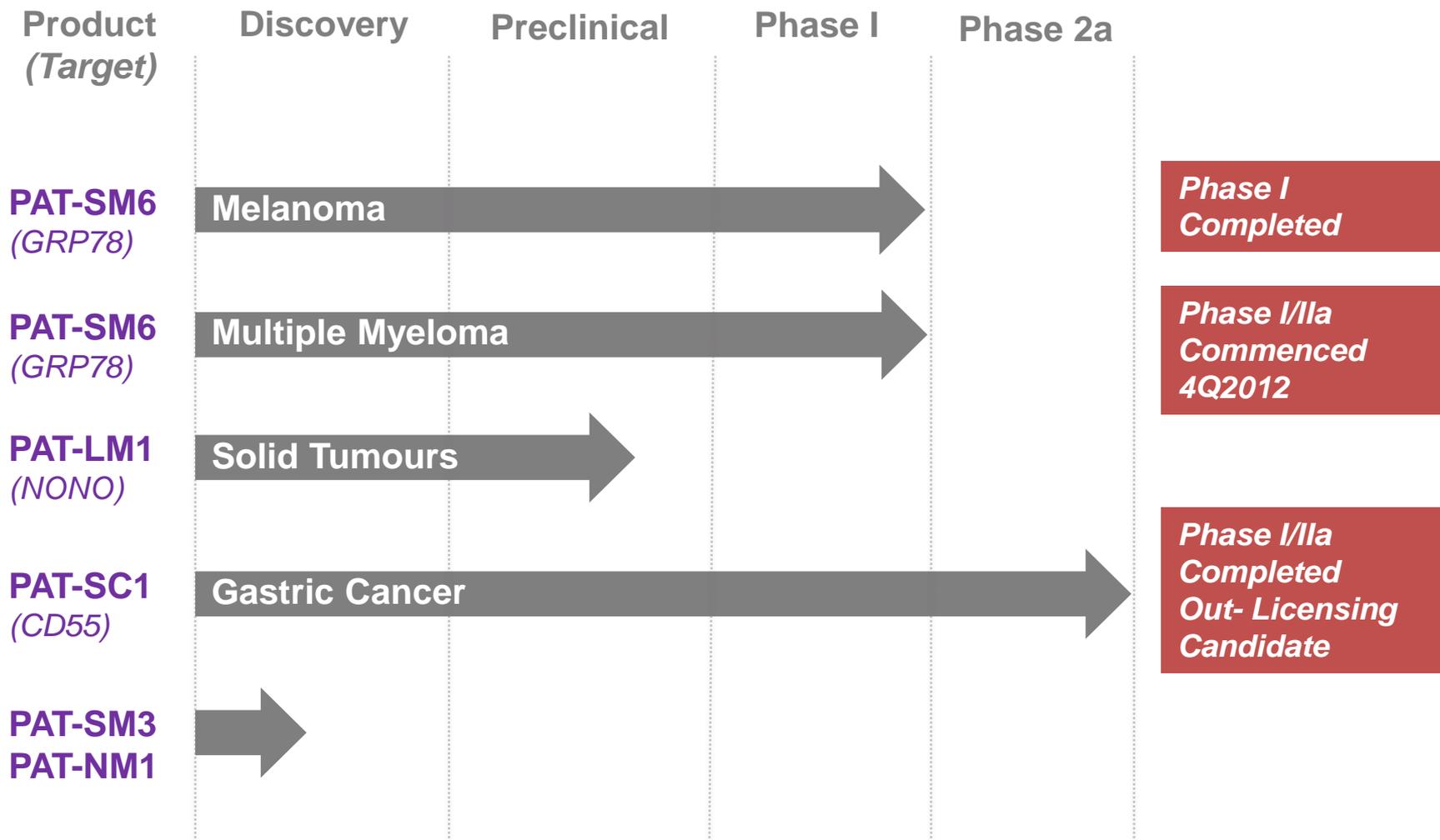
## PAT-SC1 Out-licensing Programme:

- Fund activities related to the ongoing partnering programme

## PAT-NM1, PAT-SM3 Discovery Programmes:

- Preclinical development and target discovery work

# Pipeline



**PAT-SM6**



# 2013 Programme Highlights – PAT-SM6

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## Phase I/IIa multi-dose multiple myeloma trial:

- Conducted at University Hospital, Würzburg, Germany
- Commenced Nov. 2012, estimated 1yr complete enrolment
- Currently in 4<sup>th</sup> (final) dosing cohort
- Full data to be released 1Q 2014

## Other:

- Ongoing data presented at multiple clinical / scientific meetings in Europe, USA and Japan. Data accepted for presentation ASH Dec. 2013
- Received Orphan Drug Designation Europe (Sept. 2013) and US (Oct. 2013)
- Announced Phase I/II combination trial in relapsed / refractory MM to be sponsored by Onyx Pharmaceuticals (Amgen)

## Preclinical:

- 3 publications published in peer-reviewed journals
- ARC linkage grant awarded with Macquarie University
- Expanded external collaborations with CSIRO, University Brussels, Myelomax

# Patrys' Lead Antibody: PAT-SM6

## PAT-SM6:

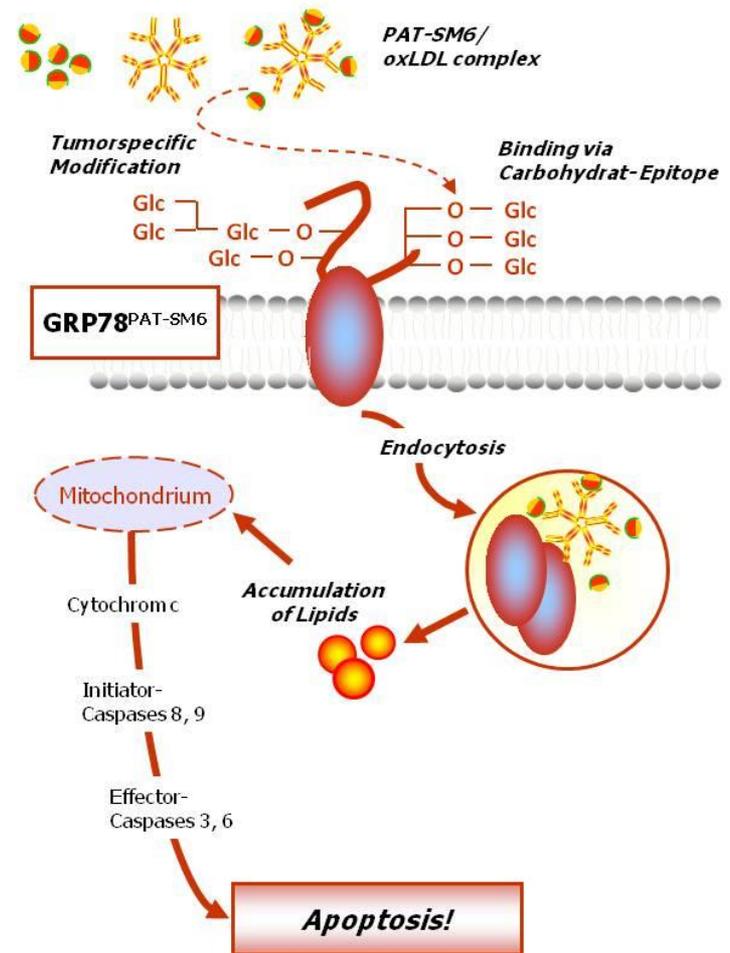
- IgM isotype,  $\lambda$ -light chain
- Isolated from stomach cancer patient
- Targets tumour specific epitope on GRP78
- Binds also to oxidised LDL and VLDL

## 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



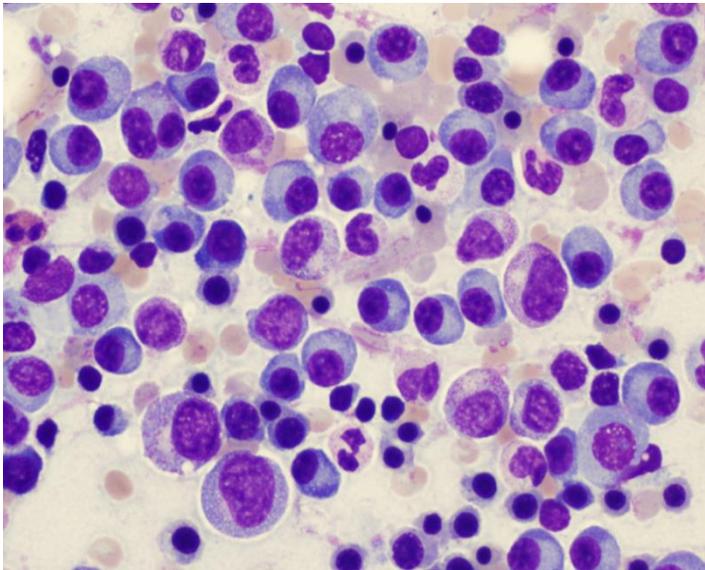
# Multiple Myeloma – Opportunity

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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 ~\$6B (2012) to >\$10B (2018)
- Market currently dominated by 3 products:
  - Revlimid (net sales \$3.7B in 2012)
  - Velcade (net sales \$2B in 2012)
  - Thalidomide (net sales \$302M in 2012)
- 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

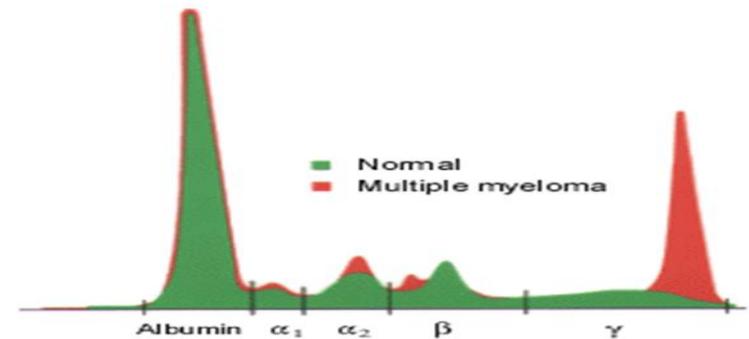
# Multiple Myeloma – Pathology

- 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)

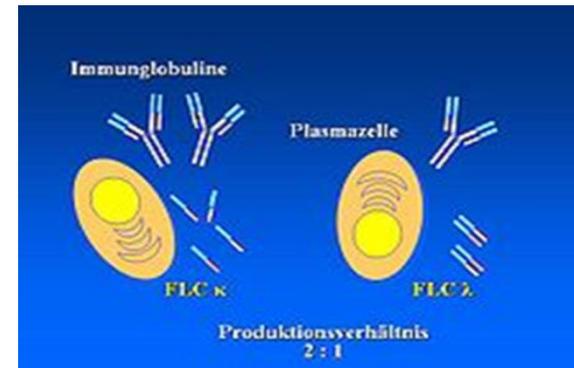


- Monoclonal gammopathy detected by electrophoresis

**Serum Protein Electrophoresis**



- Abnormal proteins (Bence Jones) detected in urine



# Multiple Myeloma – Presentation

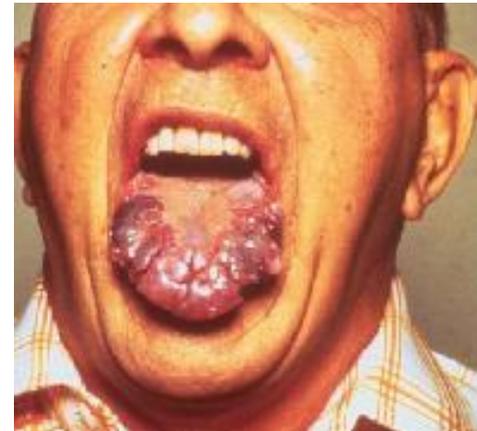
- Bone disease and hypercalcaemia



- Abnormal protein deposits



- Bone marrow failure



# Therapies for Multiple Myeloma

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## Proteasome inhibitors

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

## IMiDs

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

## Chemotherapeutics

- Melphalan
- Cisplatin
- Cyclophosphamide
- Doxorubicin

## Stem cell transplantation

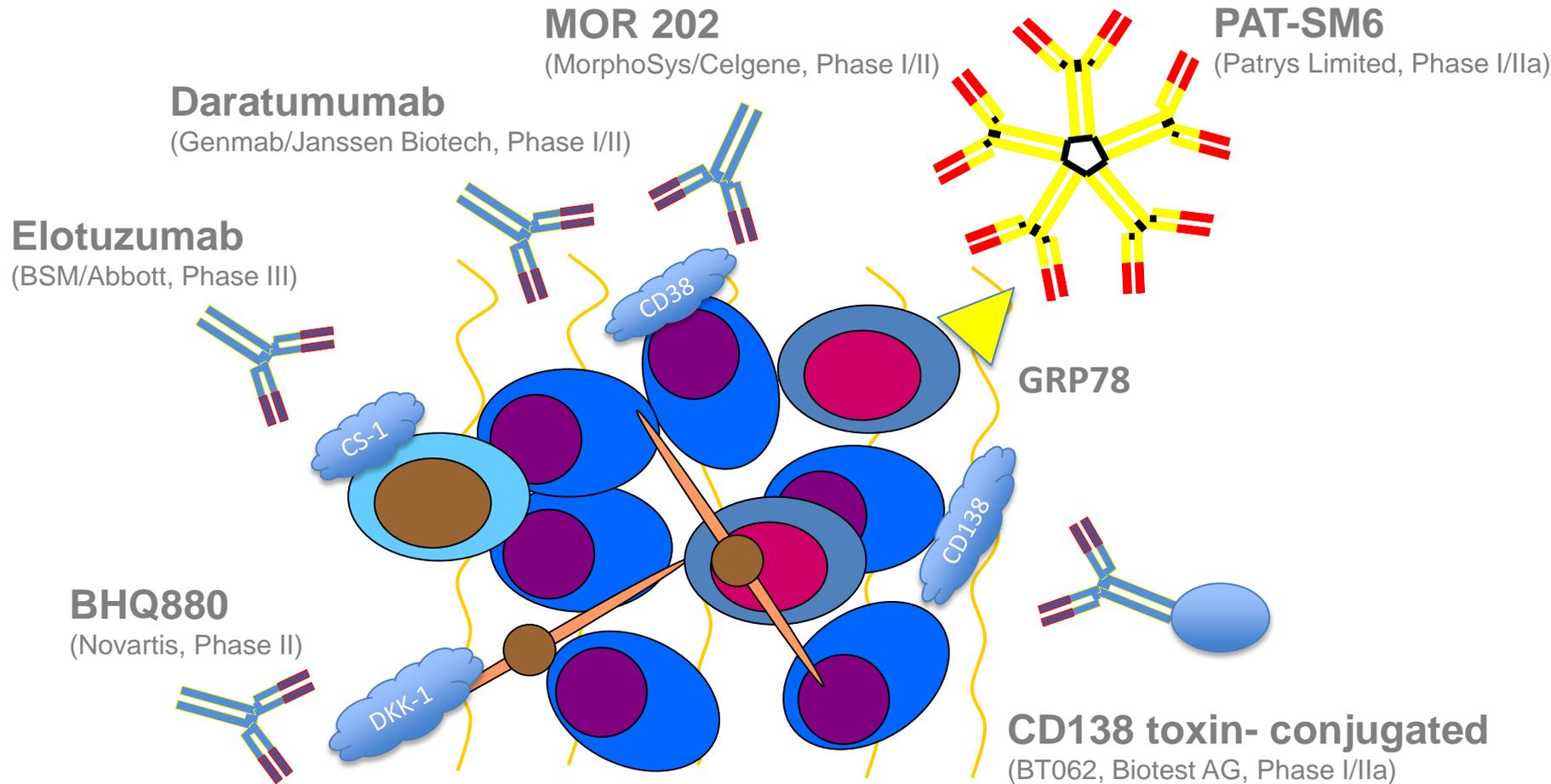
- Autologous
- Allogeneic

## Clinical studies

- Small molecules
- Antibodies, peptides
- Immunotherapeutics



# Antibodies in Clinical Trials for MM

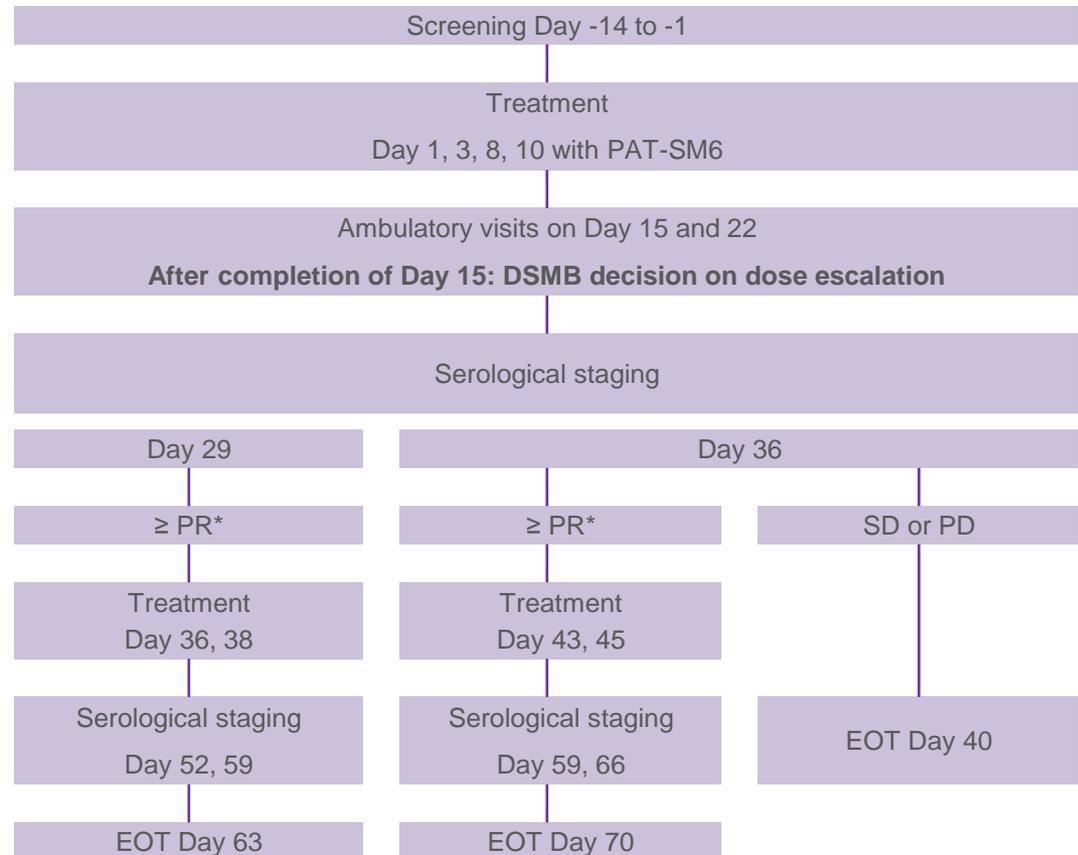


Antibodies in all stages of clinical development

# Ongoing Multiple Myeloma Clinical Trial

- Phase I/IIa open-label multi-dose trial in relapsed and multi-resistant patients (N=12 in 4 escalating dosing groups)
- 4 cohorts (0.3mg/kg, 1mg/kg, 3mg/kg, 6mg/kg)
- Patients receive 4 doses of PAT-SM6 given i.v. over 2 weeks
- Primary endpoint = safety and tolerability
- Secondary endpoints include Pk, immunogenicity, measures of response and Progression Free Survival (PFS)

## Clinical Trial Design



**PR = Partial Response    PD = Progressive Disease**  
**SD = Stable Disease      EOT = End of Trial Visit**

# Initial Clinical Data – I

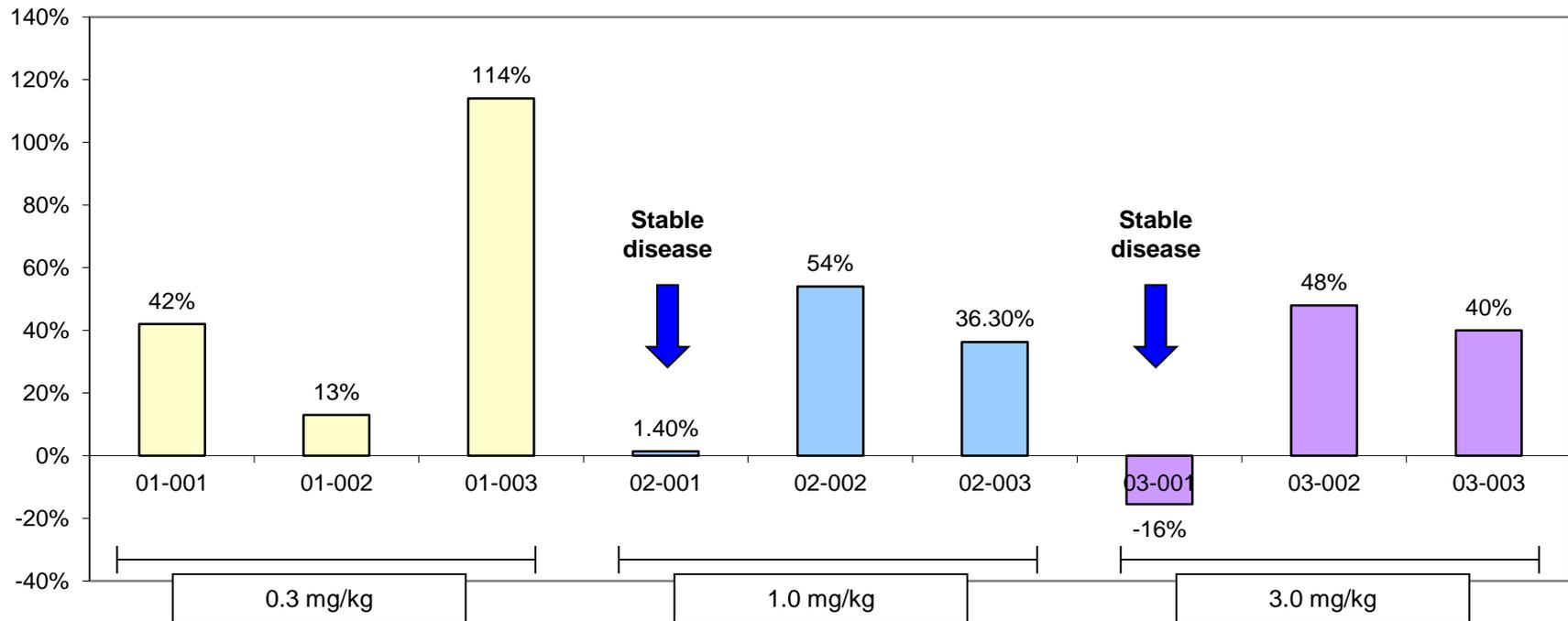
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- To date: 9 patients treated with 4 doses PAT-SM6 in 3 dose cohorts (0.3mg/kg, 1mg/kg, and 3mg/kg). 4th cohort (6mg/kg/dose) currently underway
- PAT-SM6 safe in all patients so far. No dose limiting toxicity (DLT), no related serious adverse events (SAE) and no related adverse event grade  $\geq 4$
- 2 / 9 patients had stable disease (day +35 post treatment) with a significant reduction in protein M levels in the peripheral blood
- Median time to next therapy is 42 days (clinically significant). One patient has stable disease for 127 days post treatment
- 7 / 9 patients responded positively to drugs that they had previously been resistant to (i.e PAT-SM6 makes cancer cells more sensitive to other drugs)

# Initial Clinical Data – II

## Preliminary Efficacy

Changes in M-Protein from baseline at D36/EOT



- 2 patients showed stable disease according to the IMWG criteria

# Initial Clinical Data – III

Patient	Time to next therapy	Salvage regimen	Response to salvage	Novel agents before PAT-SM6
01-001	28 days	VRCD	PR	Velcade
01-002	9 days	Benda, Pred, Thal	VGPR	Revlimid, Bortezomib
01-003	75 days	Treosulfan	SD	Revlimid
02-001	8 days	Benda, Velcade	PR	Pomalyst, Revlimid
02-002	41 days	Benda, Velcade, Dex	SD	Velcade, Thalidomide
02-003	50 days	Velcade, Melphalan	SD	Revlimid
03-001	127 days	Carfilzomib	na	Bortezomib
03-002	43 days	Pomalyst, Dex	PD	Carfilzomib, Revlimid
03-003	12 days	Carfilzomib, Cyclo./Dex	PR	Revlimid

- PAT-SM6 showed a median time to next therapy of 42 days which is a clinical benefit

# Next Steps for PAT-SM6

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- Execute new Phase I/II combination trial in relapsed / refractory MM:
  - Trial to be sponsored by Onyx Pharmaceuticals (Amgen)
  - PAT-SM6 in combination with carfilzomib (Kyprolis), 2nd generation proteasome inhibitor approved in USA July 2012
  - Patrys to provide PAT-SM6 (large-scale GMP production)
- Trial to be conducted in Germany under leadership of Professor Dr. Hermann Einsele (University Hospital, Würzburg)
- Patrys retains worldwide rights to PAT-SM6

# Recent Multiple Myeloma Deals

Partners	Date	Values	Product (Type)	Stage of Development
Amgen & Onyx	Sept. 2013	<ul style="list-style-type: none"> <li>○ \$10.4B cash</li> </ul>	Kyprolis (Proteasome inhibitor)	Marketed USA
Acetylon & Celgene	July 2013	<ul style="list-style-type: none"> <li>○ Exclusive option to buy: \$100M upfront</li> <li>○ Up to \$1.7B if exercise</li> </ul>	ACAY-1215 (HDAC-Inhibitor)	Phase Ib combination with Revlimid
MorphoSys & Celgene	June 2013	<ul style="list-style-type: none"> <li>○ Upfront \$92M</li> <li>○ Milestones \$60M</li> <li>○ Double digit royalties</li> </ul>	MOR202 (Fully human MAb $\alpha$ CD38)	Phase I/IIa for relapsed / refractory myeloma
Genmab & J&J Janssen Biotech	Aug. 2012	<ul style="list-style-type: none"> <li>○ Upfront \$55M</li> <li>○ Milestones \$1B</li> <li>○ Equity \$80M</li> <li>○ Double digit royalties</li> </ul>	Daratumumab (Human MAb $\alpha$ CD38)	Phase I/IIa for relapsed / refractory myeloma

**PAT-LM1**



# 2013 Programme Highlights – PAT-LM1

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- Two key patents granted around use of PAT-LM1 for treatment / prevention of metastatic cancer
- Moved recombinant cell line through development / early scale-up
- Promising preclinical data in blood malignancies
- External collaboration with Bioprocessing Technology Institute, Singapore

# PAT-LM1 Antibody & Target

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## PAT-LM1:

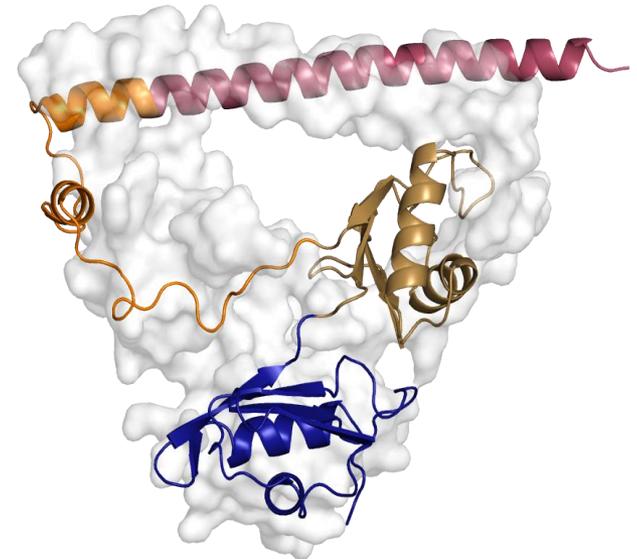
- IgM isotype,  $\lambda$ -light chain
- Isolated from a lung cancer patient
- Targets tumour-specific epitope of surface-expressed NONO (non-POU-domain-containing octamer binding protein)

## Mode of Action:

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

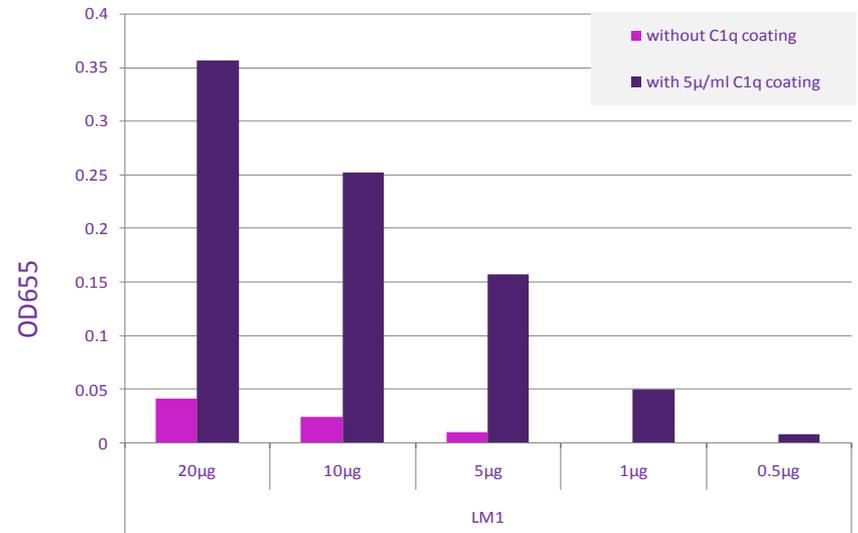
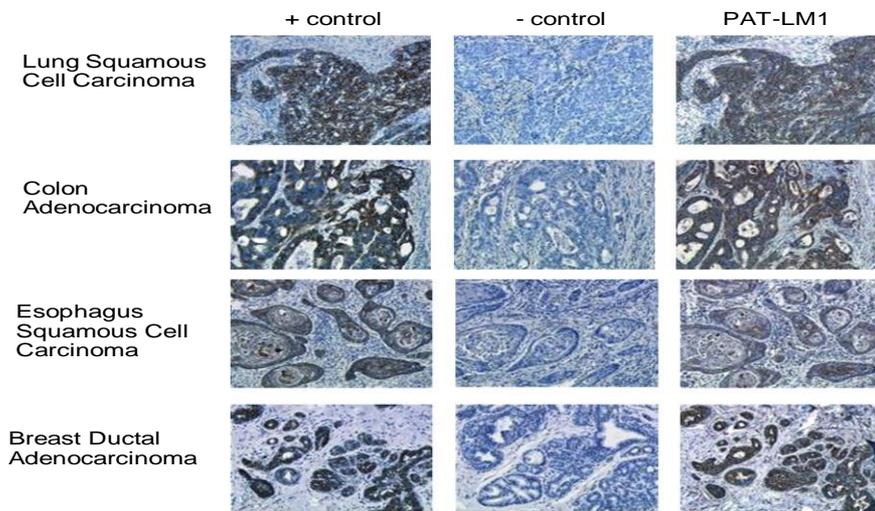
## *In Vivo & In Vitro* Reactivity:

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



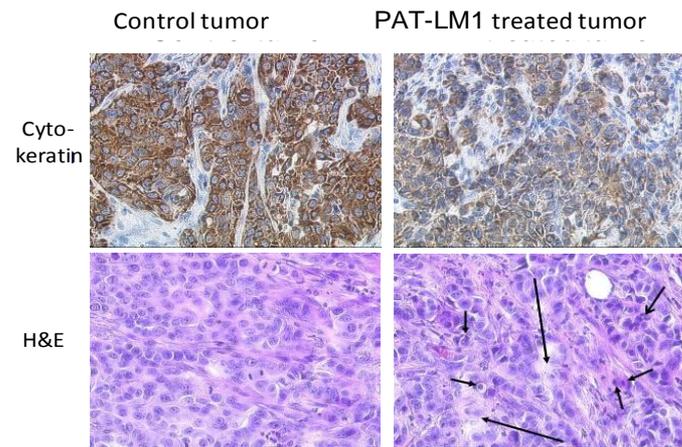
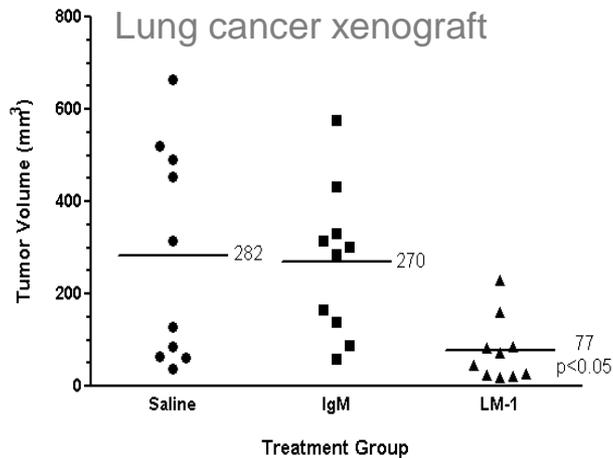
Crystal structure of NONO with PSCP1  
Passon et al PNAS 2012

# PAT-LM1 Preclinical



IHC staining with PAT-LM1 on tumour tissues

PAT-LM1 binds C1q, suggestive of CDC



PAT-LM1 reduced tumour volume & induced cell-death

# End 2013 / 2014 Key Milestones

Key Milestone	Projected Timing (CY)
<b>PAT-SM6:</b> <ul style="list-style-type: none"> <li>Complete Phase I/IIa multiple myeloma (MM) trial enrolment</li> <li>MM data presented at ASH</li> <li>Final results from MM trial</li> <li>Complete GMP manufacturing</li> <li>Commence combination Phase I/II MM trial with Onyx</li> </ul>	4Q, 2013 4Q, 2013 1Q, 2014 1H, 2014 TBA
<b>PAT-LM1:</b> <ul style="list-style-type: none"> <li>Complete manufacturing and scale-up work / Toxicology</li> <li>Preclinical data published</li> </ul>	2H, 2014 1H, 2014
<b>PAT-SC1:</b> <ul style="list-style-type: none"> <li>Preclinical &amp; clinical trial (gastric cancer) data published</li> <li>Out-licensing deal</li> </ul>	2H, 2013 2014
<b>Discovery Abs:</b> <ul style="list-style-type: none"> <li>Target work on PAT-SM3 &amp; PAT-NM1</li> </ul>	2013-2014
<b>Corporate:</b> <ul style="list-style-type: none"> <li>Capital Raising</li> </ul>	4Q, 2013 – 1Q, 2014

# For Further Information

## Contact Details:

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