# **Annual General Meeting 27 November 2013**



## pharmaxis

An Australian based speciality pharmaceutical company developing therapeutic products for chronic respiratory diseases

Approved Bronchitol® for cystic fibrosis

Aridol® for diagnosis of asthma

Products in Bronchitol® for bronchiectasis

development ASM8: moderate-severe asthma

PXS64: idiopathic pulmonary

fibrosis

PXS4728: anti-inflammatory LOXL2 inhibitor: fibrosis (and

cancer)

Operations Headquartered in Australia with

operations in Europe and US

Production GMP manufacture of respirable

dry powders













products

### Short term value drivers



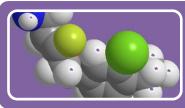
#### **Bronchitol for US**

- •US revenue opportunity US\$160m (adults)
- •Clarity over approval path achieved FDA requirements, time and cost
- Partnering process underway



#### Sales of Bronchitol for CF in rest of world

- •Revenue opportunity (adults) of US\$75m in EU & Australia (direct) and US\$70m RoW (indirect)
- Sales growth in approved/priced markets
- Accessing new markets



### Early stage pipeline

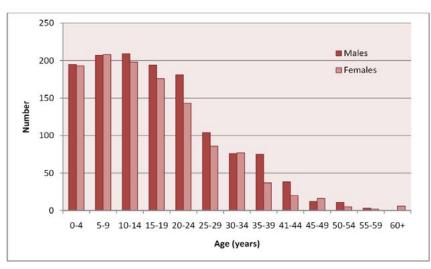
- •External funds for early stage program
- •Retain strategic interest in programs



#### Secure financial footing

- Reduction in cost base
- •Non equity financing provides US\$40m additional funding

## **Cystic fibrosis**

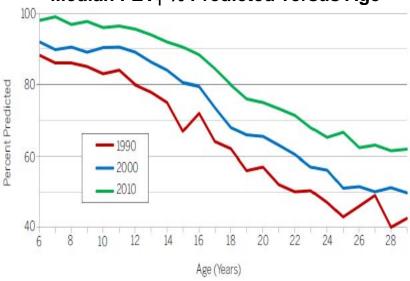


Source: Australian CF Registry

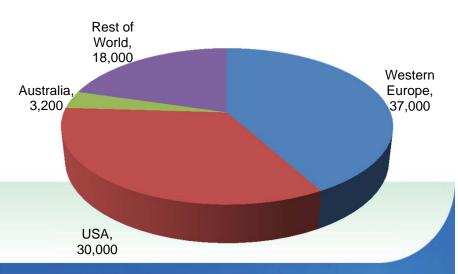
#### **Background**

- Disease characterised by poorly hydrated, tenacious, thick mucus
- Main therapeutics
  - Mostly delivered by nebulizer
  - rhDNase (Pulmozyme®): global sales ~US\$ 500m (2011)
  - Various inhaled antibiotics: global sales ~US\$ 300m

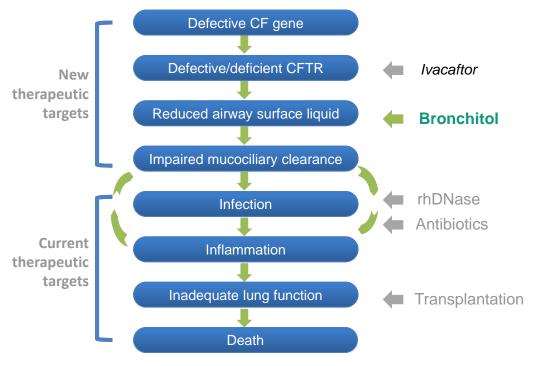
### Median FEV<sub>1</sub> % Predicted versus Age



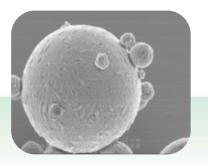
Source: Cystic Fibrosis Foundation Patient registry report, 2010

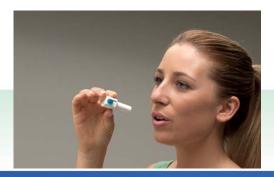


### **Bronchitol for CF**

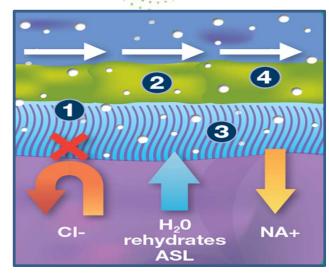








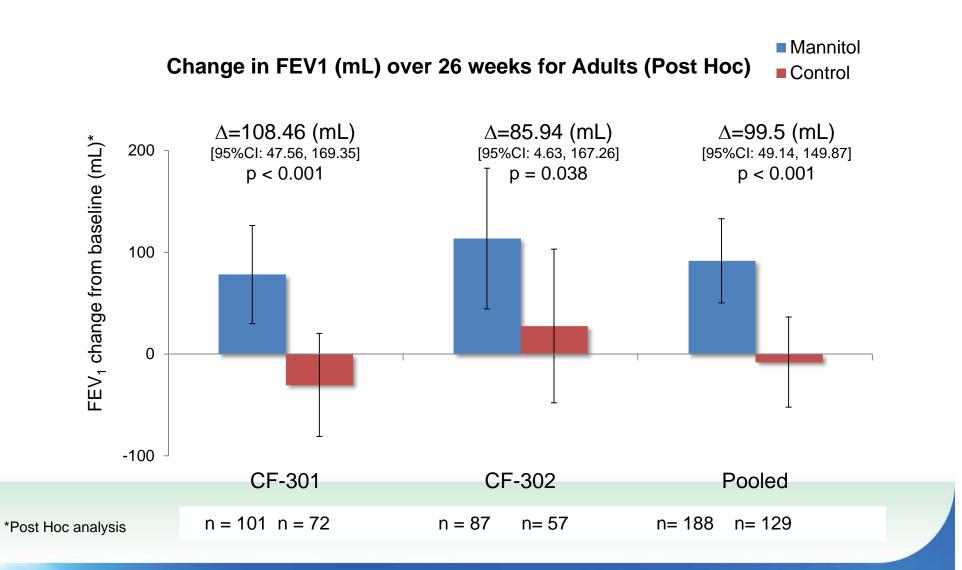




#### **Bronchitol**

- active ingredient mannitol delivered as an inhalable dry powder
- restores airway surface liquid
- mucus flow properties improved
- increases cilia beat frequency
- mucus clearance enhanced

## Predictable results in adult CF patients

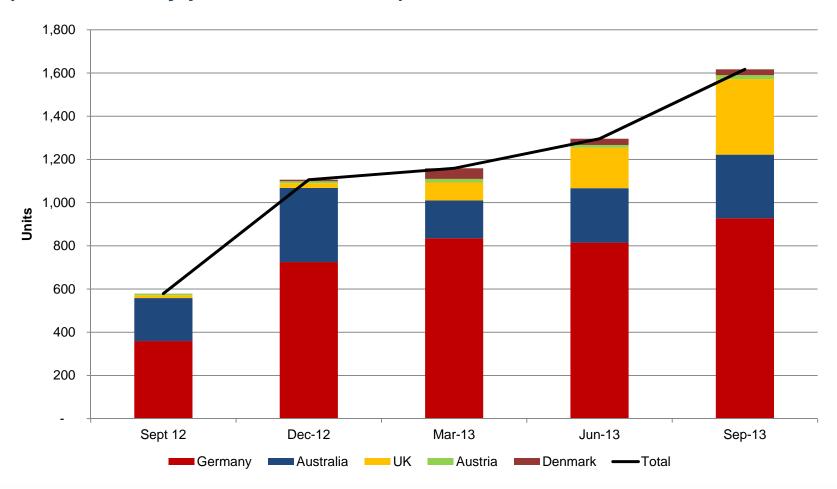


### **Bronchitol US value drivers**

Value Driver	Status	Next
US adult CF market is significant and has a high level of unmet need.	<ul> <li>~50% of US CF patients (15,000) are adults.</li> <li>&gt;60% of Adults have Moderate or Severe disease</li> <li>~80% of all CF patients in US take rhDNase (US\$360m p.a.)</li> </ul>	<ul> <li>Adult patients on ivacaftor still require effective airway clearance.</li> <li>% Adult patients increases each year as life expectancy increases.</li> </ul>
FDA approval of Bronchitol	<ul> <li>FDA and Pharmaxis agreed on route forward.</li> <li>Requirement for adult "tie breaker" study</li> <li>Protocol submitted for review</li> </ul>	<ul> <li>Final protocol comments due Q4 13.</li> <li>Paediatric development plan to be submitted Q1 14.</li> </ul>
Clinical Trial Success	<ul> <li>Adult data from CF301 and CF302 give confidence of positive outcome</li> <li>~350 patients / A\$15-A\$20m</li> </ul>	<ul> <li>CRO selection and contract negotiation</li> <li>CF303 first patient 1H 2014</li> </ul>
Commercial Success	<ul> <li>Low cost of entry (~20 sales reps)</li> <li>Strong support from the CFF</li> <li>Similar to UK market</li> <li>Formal partnering process initiated</li> </ul>	<ul> <li>Negotiations with preferred partner Q1 2014</li> <li>NQ funding enables deferral of partnering for greater partner value</li> </ul>
pharmaxis		7

## **Bronchitol unit sales by country**

(1 unit = 14 day pack of Bronchitol)



	<b>Germany</b>	<u>Australia</u>	<u>UK</u>	<u>Total</u>
Sales increase over				
Sep-12	158%	48%	nm	179%
Jun-13	14%	17%	87%	25%

### Sales of Bronchitol for CF in rest of world

Region Achieved		Next		
1. Europe	<ul> <li>Germany</li> <li>Launched June 2012</li> <li>100% of large/85% of all CF clinics prescribing Bronchitol</li> <li>Patient compliance and adherence suboptimum</li> <li>UK</li> <li>Strong growth seen once funding issues resolved (April 2013)</li> <li>65% of clinics prescribing Bronchitol</li> <li>Good compliance and patient retention</li> <li>Rest of Europe</li> <li>Launched in Austria &amp; Denmark</li> <li>Paediatric patients</li> <li>EU paediatric trial (CF204) commenced recruitment</li> </ul>	<ul> <li>Increase centre penetration</li> <li>Pilot initiatives to reduce patient cycling on and off Bronchitol and improve patient adherence and compliance</li> <li>Increase centre penetration</li> <li>Support adherence</li> <li>Italy – named patient sales Q4</li> <li>Progress pricing applications for remaining EU markets</li> </ul>		

### Sales of Bronchitol for CF in rest of world

Reg	ion	A	chieved	Ne	ext
2. #	Australia	•	Achieved improved PBS reimbursement rules Good patient compliance Steady growth in last two quarters (to Sept 2013)	•	Increase rates of trial of Bronchitol by patients Support adherence
	Rest of world	•	Appointed distributors and commenced local approval/pricing submissions  • Brazil  • Poland and ten other eastern European countries	•	Appoint distributors for Russia, Turkey and Israel Named patient sales commencing Q4 CY 2013 File additional marketing applications as distributors appointed

# Strategic interest in an innovative pipeline of early stage compounds

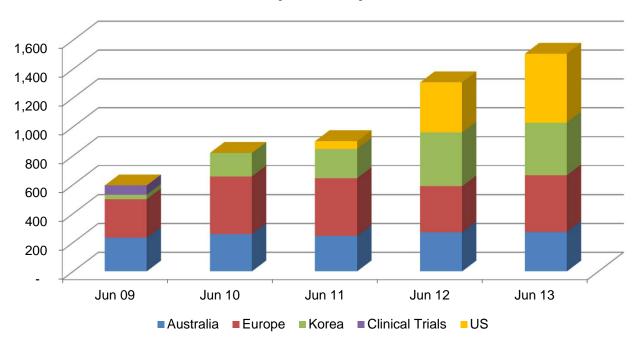
Objective		Achieved	Next	
1.	Pursue multiple strategies to fund early stage development that retain maximum participation for Pharmaxis shareholders  a. Pharma research collaborations	Detailed scientific discussions with pharma companies interested in pipeline assets	<ul> <li>Assess value (Q4 CY2013)</li> </ul>	
	b. Grants c. Spin out of R&D assets	<ul> <li>Awarded two ARC linkage grants, applying for other larger overseas grants</li> <li>Commenced discussions with Australian and international (pharma) VC's</li> </ul>	<ul> <li>Progress grant applications</li> <li>Assess value (Q4 CY2013)</li> </ul>	
2.	Continue to invest selectively in proof of concept H2 CY2013	<ul> <li>LOXL2 program significantly advanced</li> </ul>		

### **Aridol**

Identifies airway hyperresponsiveness which helps physicians in the overall assessment of **asthma** 



## Aridol Sales (A\$'000)



- Sales increased 15% over June 2012
- Minimal sales investment in FTE's: US 2.6; RoW 1.0

## **Secure financial footing**

Objective		Achieved	Next	
1.	Reduce cost base by \$11.8m p.a. (29% of cash costs) by 31 December 2013	a. (29% of cash expenses for Sept 2013	<ul> <li>Further costs to reduce in Q4 CY2013</li> <li>External funding of early stage R&amp;D staff</li> <li>Continued eligibility for R&amp;D tax credit</li> </ul>	
2.	Non equity financing	<ul> <li>Negotiated innovative US\$40m financing from NovaQuest</li> </ul>	<ul> <li>Remaining US\$20 million available from commencement of US clinical trial</li> </ul>	

# **Financial Overview**



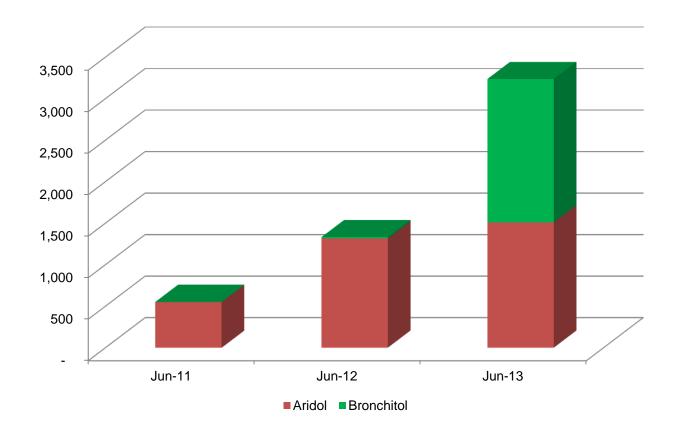
### **Financial statements**

	Year ended	30 June
('000 except per share data)	30-Jun-13	30-Jun-12
	A\$	
Revenue from sale of goods	3,237	1,331
Cost of sales	(1,141)	(522)
Gross profit	2,096	809
Interest income	2,695	3,049
Grant and other income	5,675	3,874
Expenses		
Sales & marketing	(13,893)	(11,073)
Safety, medical and regulatory	(5,581)	(4,904)
Administration	(6,030)	(5,248)
Research & development - Bronchitol	(18,531)	(19,850)
Research & development - new drug development	(5,331)	(4,519)
Finance & royalties	(2,945)	(856)
Restructuring charges	(1,690)	-
Total expenses	(54,001)	(46,450)
Net loss before tax	(43,535)	(38,718)
Income tax expense	(2)	74
Net loss after tax	(43,537)	(38,644)
Basic and diluted earnings (loss) per share - \$	(0.030)	(0.042)

#### Revenue

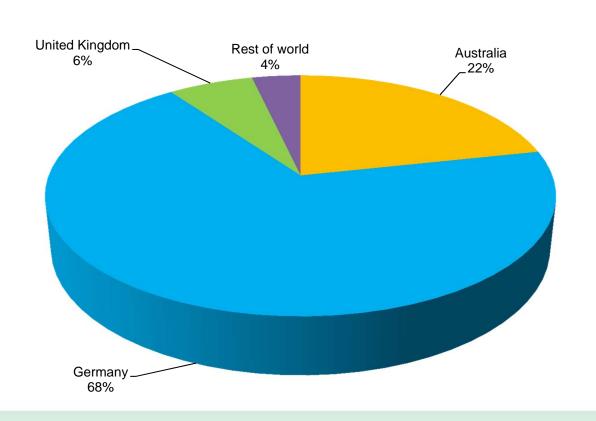
- Sales increase
- R&D tax credit significant

### Sales revenue – FY 2013



- Total sales increased 143% over 2012
- Bronchitol sales
   53% of total
- Aridol sales increased 15% over 2012

## **Bronchitol sales by country – FY 2013 Total: \$1.7m**



Mix represents market size and timing of pricing approval /launch sequence

- Germany June 12
- UK April 13
- Australia Aug 12

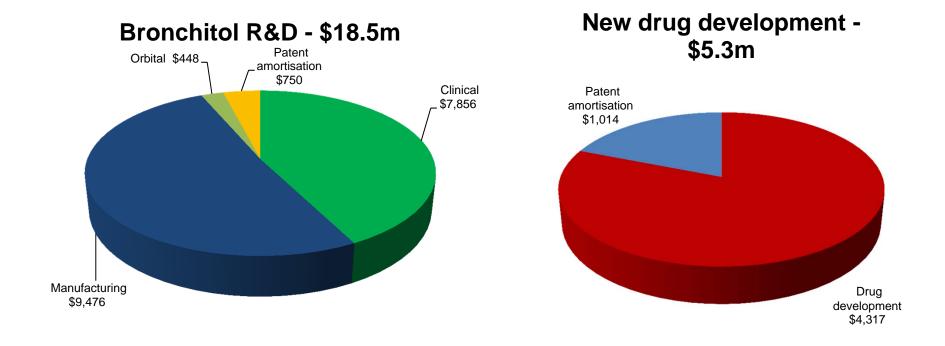
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#### Expenses

- Sales & marketing mainly EU
- Safety includes FDA costs
- Admin consolidation of functions, corp costs
- Finance includes non cash NQ finance expense
- Restructuring charge re CY 2013 actions, paid out FY 2014
- R&D next slide

## Research & development expense (A\$'000)



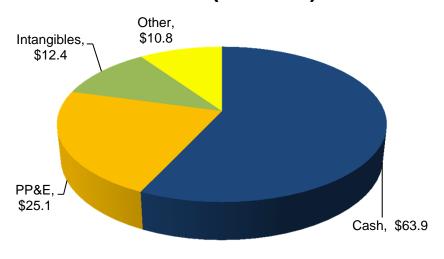
Total R&D tax credit: \$5.3m

Net cash cost of new drug development: \$2.3m

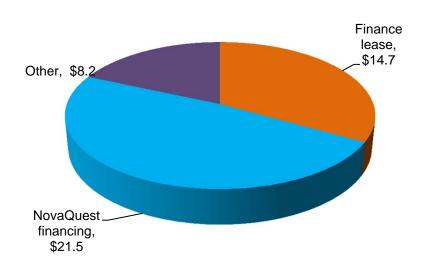
### Balance sheet – 30 June 2013

(A\$ mil)

### **Assets (A\$112m)**



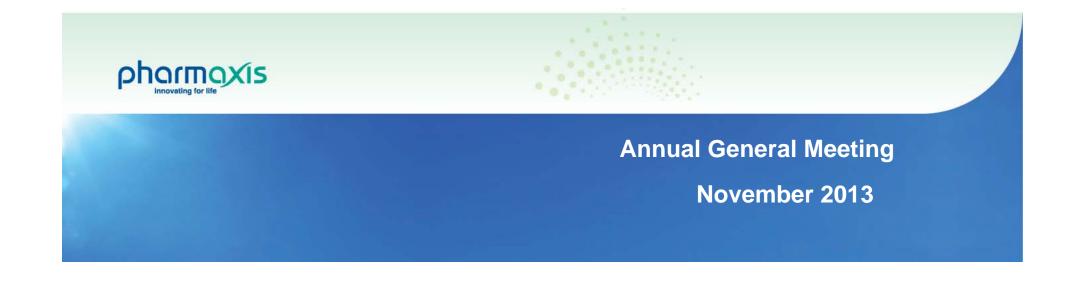
### Liabilities (A\$44m)



- Additional US\$20m available under NovaQuest agreement on commencement of US CF trial
- 2013 R&D tax credit of \$4.8m received Oct 13

- Finance lease is 15 year lease over 20 Rodborough Rd (11 years remaining)
- NovaQuest financing amount received plus accrued charge. Not repayable other than as % of sales

# **Shareholder Questions**



### **Formal Business**



## Resolution 1 – Financial Report, Directors' Report and the Auditor's Report

No shareholder vote is required

### **Resolution 2: Adoption of the Remuneration Report**

### **Resolution:**

"That the remuneration report of the Company for the year ended 30 June 2013 is adopted."

### **Resolution 2: Adoption of the Remuneration Report**

The Company has received:

- 43,085,823 proxy votes in favour of the resolution;
- 8,415,789 proxy votes against the resolution;
- 47,769,637 proxy votes abstaining from the resolution;
- 512,127 proxy votes excluded from voting;
- 1,116,928 proxies able to be voted by the chair which the chair intends to vote in favour of the resolution.

<sup>\*</sup> Voting exclusions apply

## Resolution 3: Re-election of Mr Malcolm McComas as a Non Executive Director

### **Resolution:**

"That Mr Malcolm McComas is re-elected as a non executive director of the Company."

## Resolution 3: Re-election of Mr Malcolm McComas as a Non Executive Director

### The Company has received:

- 93,093,208 proxy votes in favour of the resolution;
- 6,312,013 proxy votes against the resolution;
- 286,297 proxy votes abstaining from the resolution;
- 1,208,786 proxies able to be voted by the chair which the chair intends to vote in favour of the resolution.

## Resolution 4: Grant of Performance Rights to Mr Gary Phillips

### **Resolution:**

"That, for the purposes of Listing Rule 10.14 of the Listing Rules of the Australian Securities Exchange (ASX) Listing Rules), the Corporations Act 2001 (Cth) (Corporations Act) and for all other purposes, approval is given for the grant of 2,000,000 zero grant and zero exercise price employee options (Performance Rights) to Mr Gary Phillips under the Company's performance rights plan, resolved to be granted by the Board in June 2013 and, upon exercise of those Performance Rights, the acquisition of 2,000,000 ordinary shares underlying those Performance Rights, in accordance with the terms of the performance rights plan and the explanatory statement accompanying this notice of meeting."

## Resolution 4: Grant of Performance Rights to Mr Gary Phillips

### The Company has received:

- 90,428,428 proxy votes in favour of the resolution;
- 8,621,428 proxy votes against the resolution;
- 303,106 proxy votes abstaining from the resolution;
- 412,127 proxy votes excluded from voting;
- 1,135,215 proxies able to be voted by the chair which the chair intends to vote in favour of the resolution.

Voting exclusions apply

# Thank you for your participation

