

pharmaxis

# Quarterly Investor Briefing

**Gary Phillips**  
**Chief Executive Officer**  
**11 April 2013**

**Innovative products  
for respiratory diseases**

*SLIDE 1*

# Forward Looking Statements

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This presentation may contain forward-looking statements that are based on management's current expectations and beliefs and are subject to a number of factors and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. The forward-looking statements contained in this presentation include statements about future financial and operating results, results of our clinical trials, status of our regulatory submissions, possible or assumed future growth opportunities and risks and uncertainties that could affect Pharmaxis' product and products under development. These statements are not guarantees of future performance, involve certain risks, uncertainties and assumptions that are difficult to predict, and are based upon assumptions as to future events that may not prove accurate. Therefore, actual outcomes and results may differ materially from what is expressed herein. In any forward-looking statement in which Pharmaxis expresses an expectation or belief as to future results, such expectation or belief is expressed in good faith and believed to have a reasonable basis, but there can be no assurance that the statement or expectation or belief will result or be achieved or accomplished.

We are not under any duty to update forward-looking statements unless required by law. This investor presentation is not an offer of the sale of securities.

# Quarterly Investor Briefing – 10 April 2013

- Key initiatives
  - Defining the path to approval for Bronchitol in the US
  - Phase III clinical trial of Bronchitol in bronchiectasis – B305
  - Bronchitol for CF in the rest of the world
  - Changes to the Company's business model
  - Generating value from the Company's pipeline of early stage assets
- Financial overview

# Bronchitol<sup>®</sup> for cystic fibrosis - US

FDA complete response letter – main concerns:

- frequent early dropouts in trial 301 for which the pre-agreed primary statistical analyses could not account;
- the lack of statistical significance in trial 302 for the primary endpoint;

and

- haemoptysis, particularly in paediatric patients

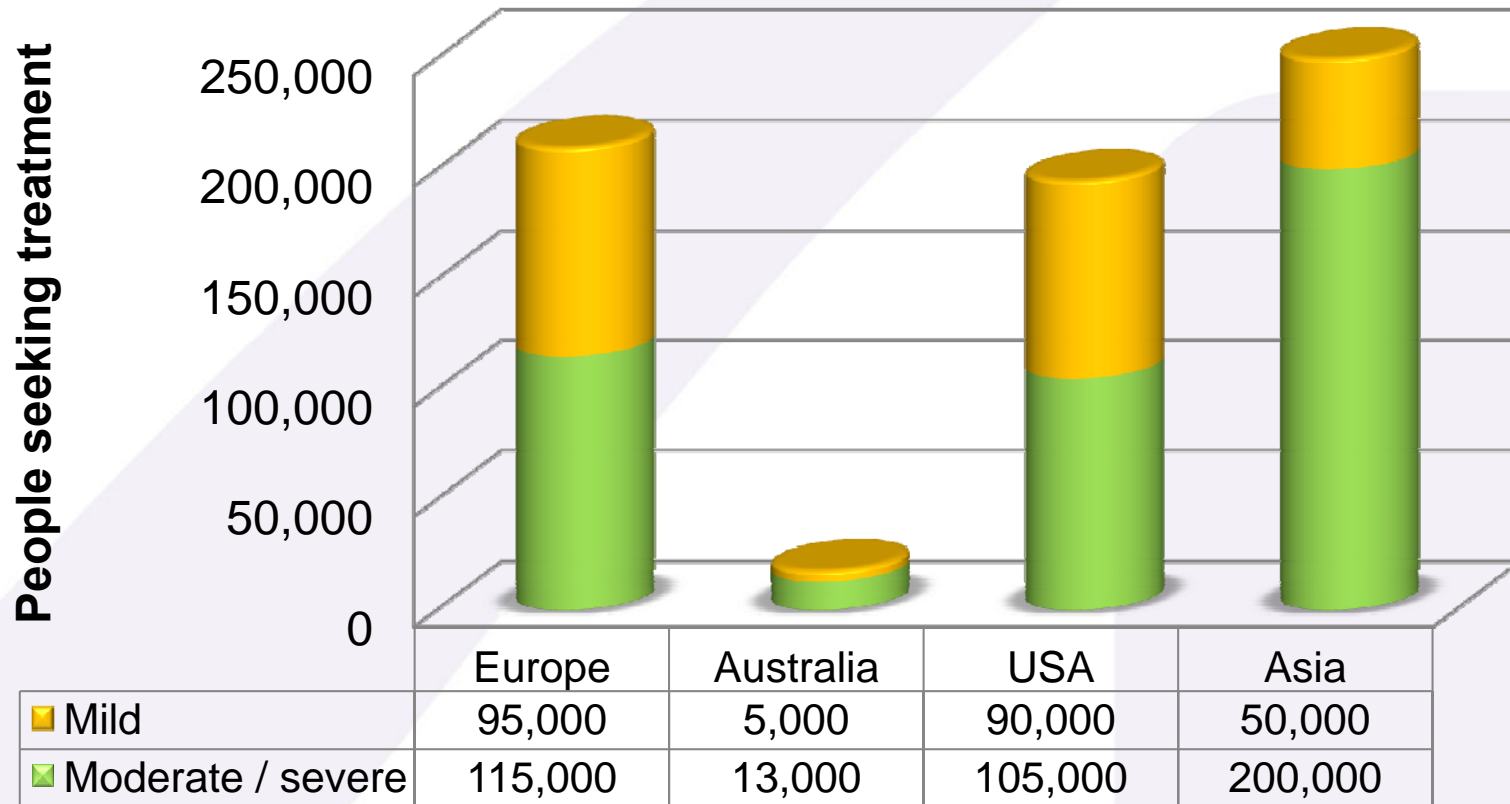


# Bronchitol for cystic fibrosis - US

Pathway to approval of Bronchitol for cystic fibrosis in the US:

- Type A meeting to understand FDA concerns and requirements – Qtr 2
- Complete design of required clinical trial, submitted to FDA – Qtr 4
- Commence clinical trial H1 2014
- Reduce uncertainty – requirements, timelines & cost
- Orphan Drug Status in US – 7 years market exclusivity from approval
- Pulmozyme® sales in US – approximately US\$300m

# Bronchiectasis - patients seeking treatment



- no products have been approved that assist mucus clearance
- no drugs in phase III

Note: Data from Datamonitor research and from Frost & Sullivan research (2007)

\*CHEST, August 2012;142(2):432-439. doi:10.1378/chest.11-2209

# Phase III trial of Bronchitol in bronchiectasis

- **Timeline**

- Last patient completed March 2013
- Top line results Qtr 2 2013

- **Phase III trial**

- 485 patient, controlled, double blind, randomised, 52 week treatment, 83 sites in US, Europe, South America, Australia
- 400mg twice a day

- **Primary endpoint**

- Reduction in the rates of pulmonary exacerbations

- **Secondary endpoints**

- quality of life, sputum weight and spirometric lung function

- **Orphan Drug designation**

USA

7 years marketing exclusivity from approval



# Bronchitol for CF in the rest of the world

Region	Country	Mode to market	Patients	Approval	Pricing
EU	Germany	Direct/launched	7,500	√	√
	UK	Direct/launched	9,000	√	√
	France	Direct/launch Q2	6,300	√	Q2
	Italy	Direct/launch H2	3,800	√	H2
	Spain	Direct/launch 2014	4,500	√	2014
	Austria	Direct/launched	1,000	√	√ <sup>1</sup>
	Denmark	Direct/launched	500	√	√
	Ireland	Direct/launch H2	1,100	√	H2
	Sweden	Direct/launch H2	900	√	H2
	Netherlands	Direct/launch Q2	2,200	√	H2
	EU other (Poland, Czech, Hungary)	Distributor/tba	12,000	√	H2 <sup>1</sup>
Europe other	Russia	Distributor – tba	10,000	tba	tba
South America	Brazil	Distributor – United	3,500	2015	2015
Rest of world	Australia	Direct/launched	3,000	√	√
	Middle East – Israel & Turkey	Distributor - tba	7,000	tba	tba

(1) Named patient reimbursement basis

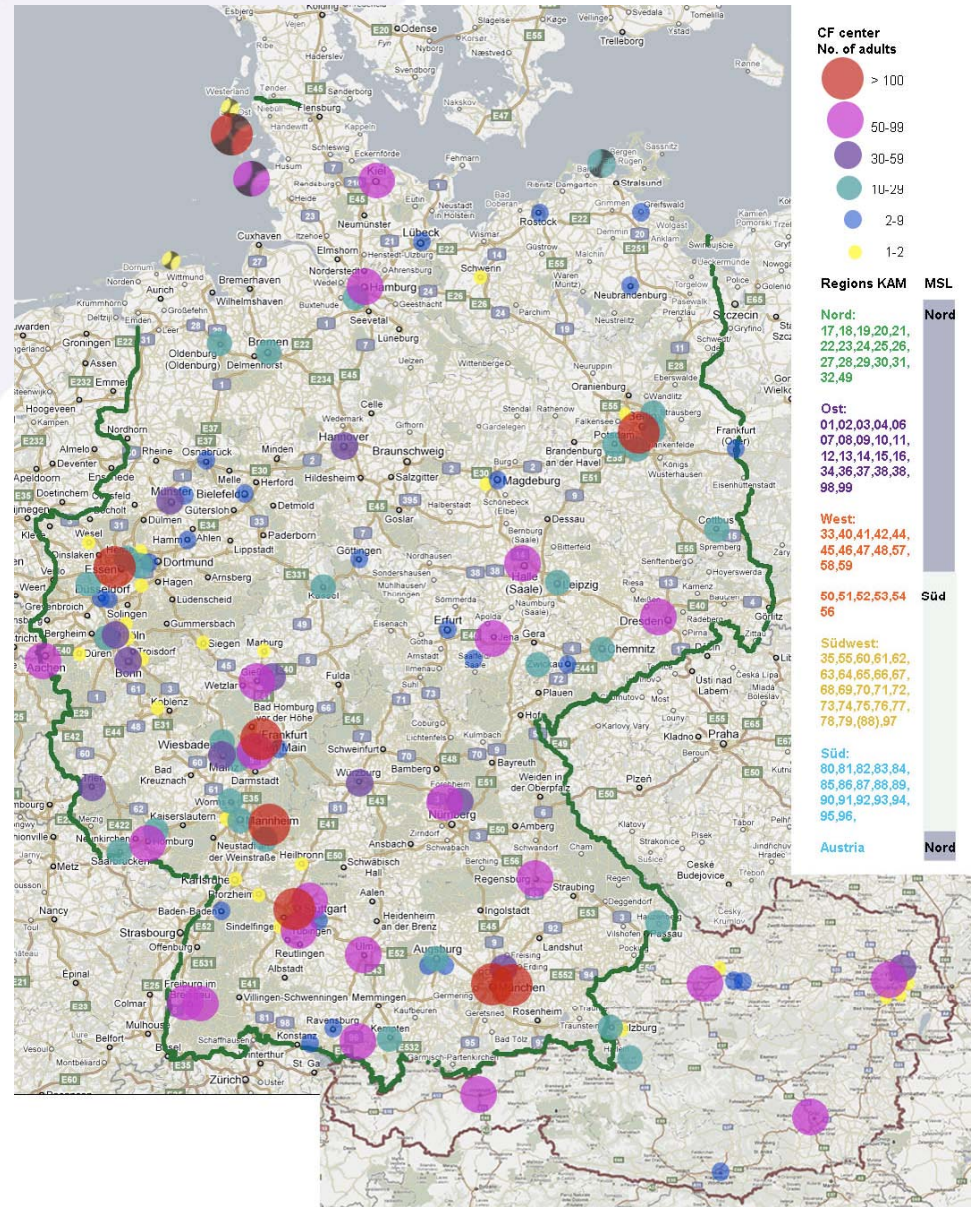


# Brazil

- CF population
  - Population of Brazil – 190 million across 27 states
  - Estimated 3,500 CF patients (70% are under 18 years)
  - 2,500 are diagnosed and undergoing treatment at 32 CF centres
  - 23 patient associations cover top 3 regions and account for ~90% patients
  - Available CF drugs include Pulmozyme, inhaled antibiotics
- Regulatory pathway
  - Based on Australian approval
  - 12 to 18 months from submission
- United Medical Ltda
  - Founded in 1987 - based in Sao Paulo
  - Privately held
  - Core focus in CF since 1999
  - Promotes inhaled tobramycin to CF community

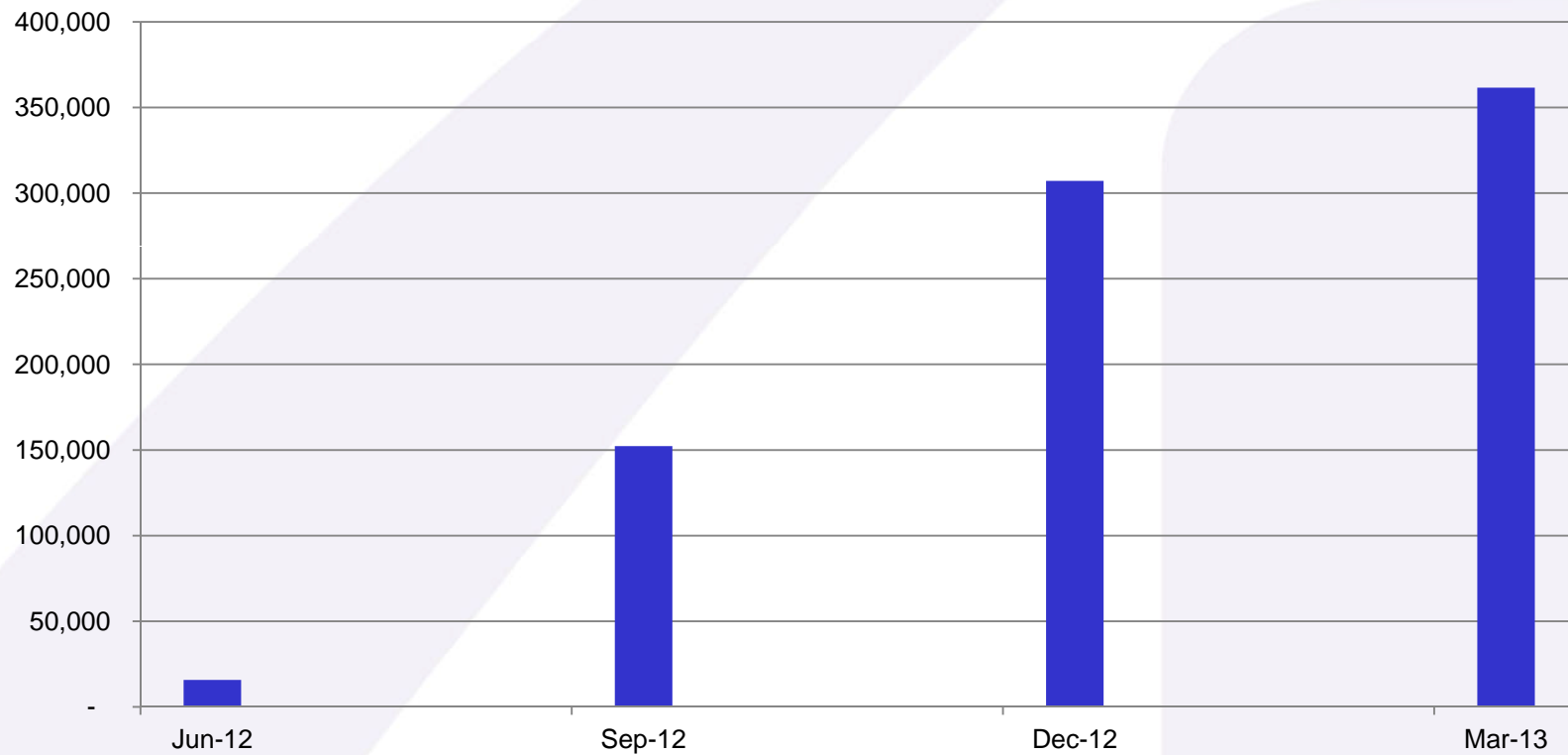
# German CF market – CF centers (incl Austria)

No of adult patients/clinic	No of clinics
>100	8
50 to 99	22
30 to 49	11
10 to 29	29
2 to 9	27
1 to 2	23



# Bronchitol sales in Germany

German Sales A\$'000



# Aridol<sup>®</sup> quarterly sales history since launch



# Changes to business model

## **Our new priorities:**

- **maximise revenue from Bronchitol approvals outside of the US**
- **significantly reduce our cost base**
- **capture the value of**
  - **Bronchitol for CF in the US, and**
  - **Bronchitol for bronchiectasis globally.**

# Pipeline of early stage assets

- Oligonucleotide portfolio - including ASM8 which is in phase 2 clinical development for moderate and severe asthma, and at a preclinical stage, PXS1100 and PXS2200 which are for COPD and asthma.
- Orbital – a new respiratory device uniquely capable of delivering dry powder respiratory drugs requiring high doses (eg mannitol, antibiotics) without needing multiple capsules.
- PXS4728 – an SSAO inhibitor that is in preclinical development as an anti inflammatory/anti fibrotic once a day oral drug for several indications.
- LOX / LOXL2 Inhibitor Program - a small molecule active against a well known target important in several fibrotic diseases and some cancers.
- PXS64/PXS25 - an anti-fibrotic drug that has completed it's preclinical development which inhibits the function of TGFb and is targeting the treatment of lung fibrosis.

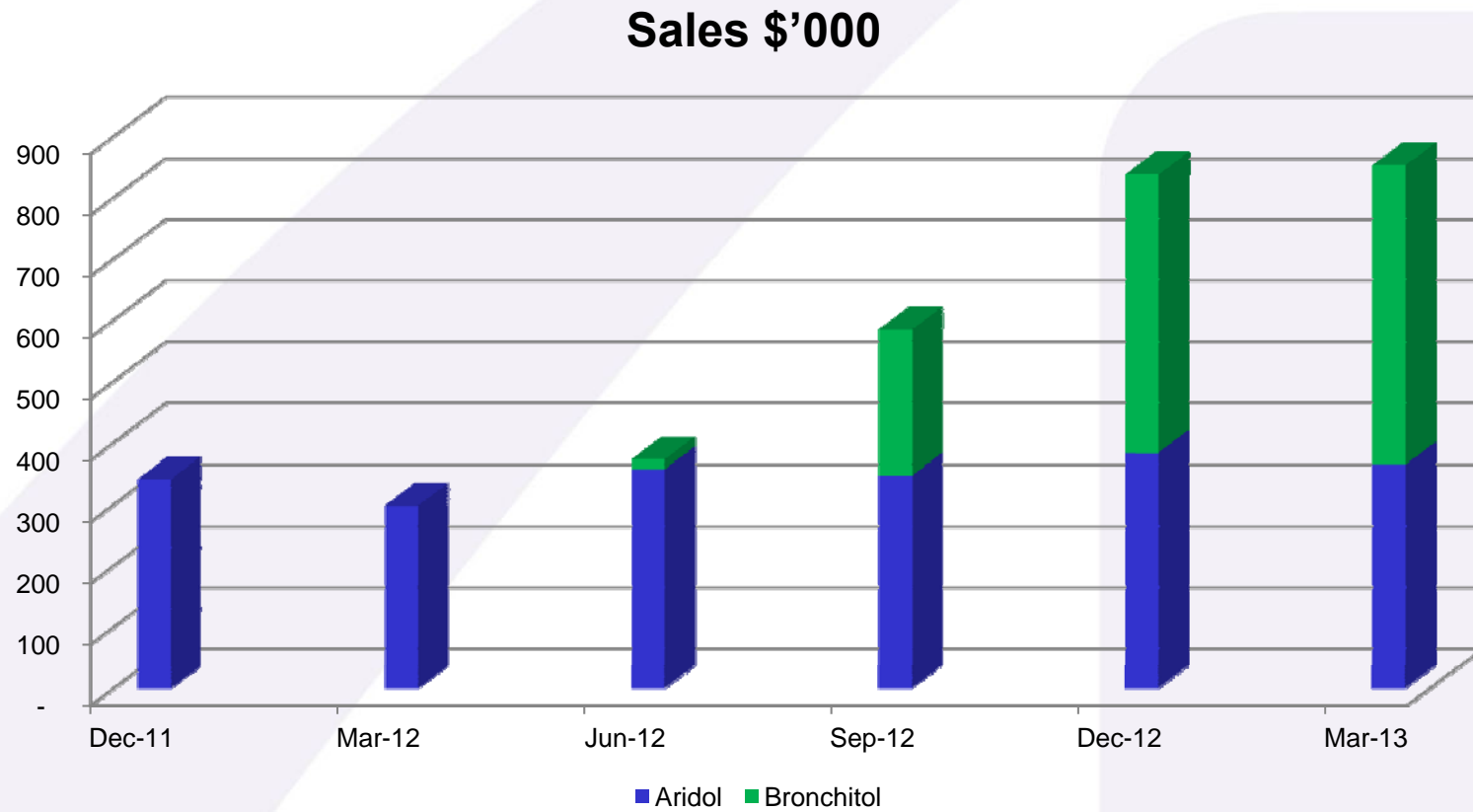


# Financial statements – unaudited

('000 except per share data)

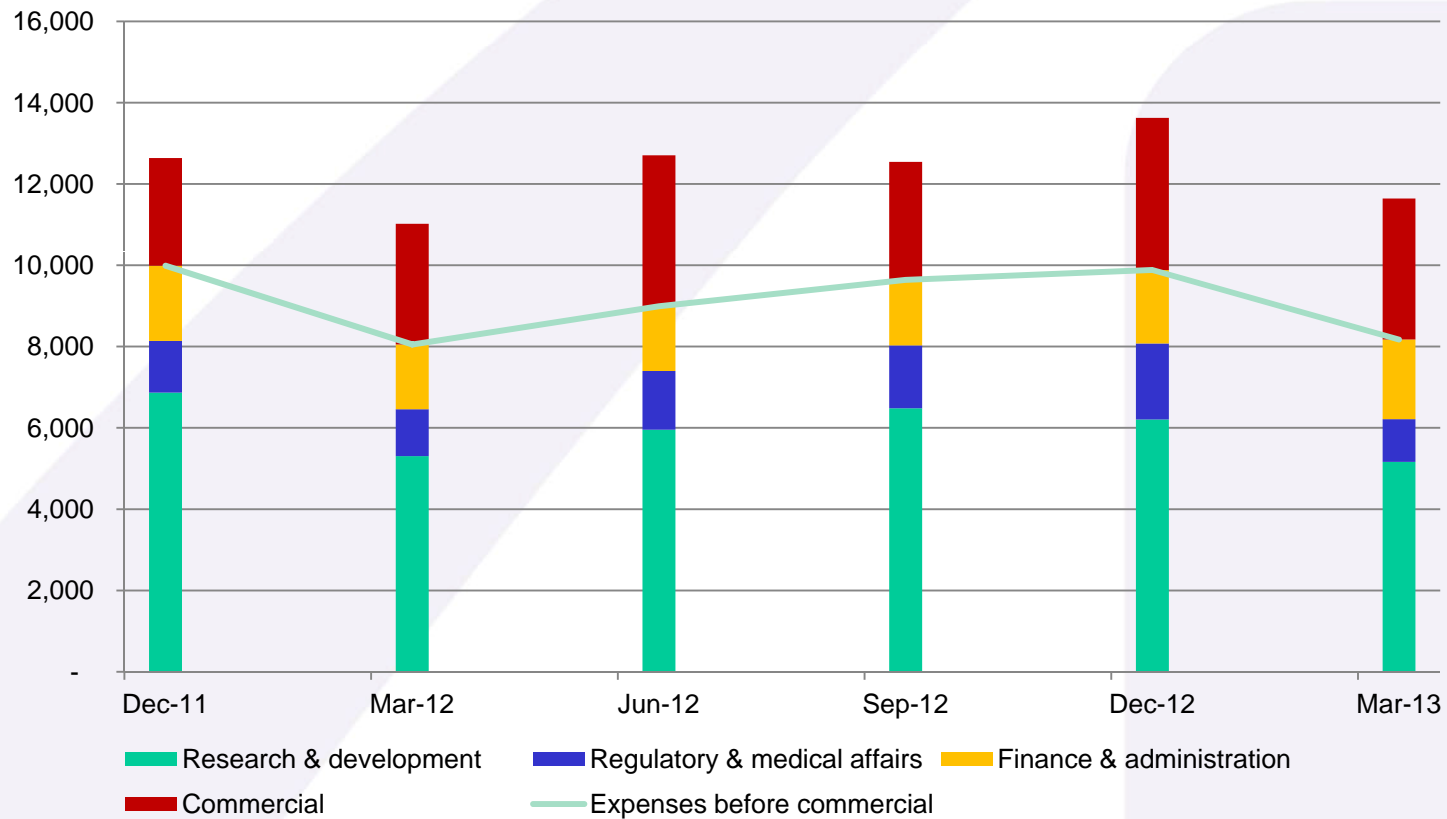
Income Statement Data	Three months ended		Nine months ended	
	31-Mar-13	31-Mar-12	31-Mar-13	31-Mar-12
	A\$	A\$	A\$	A\$
Revenue from sale of goods	853	298	2,275	958
Cost of sales	(306)	(124)	(815)	(379)
Gross profit	547	174	1,460	579
Interest income	675	1,049	1,987	2,081
Grant and other income	776	761	3,988	2,434
Expenses				
Commercial	(3,467)	(2,971)	(10,100)	(7,358)
Regulatory, safety & medical affairs	(1,051)	(1,155)	(4,468)	(3,458)
Finance & administration	(1,960)	(1,592)	(5,379)	(4,565)
Research & development	(5,164)	(5,292)	(17,866)	(18,349)
Total expenses	(11,642)	(11,010)	(37,813)	(33,730)
Loss before income tax	(9,644)	(9,026)	(30,378)	(28,636)
Income tax expense	(22)	29	(64)	123
Loss for the period	(9,666)	(8,997)	(30,442)	(28,513)
Basic and diluted earnings (loss) per share - \$	(0.031)	(0.029)	(0.099)	(0.109)
Depreciation & amortisation	1,144	1,164	3,441	3,511
Fair value of securities issued under employee plans	456	211	1,332	756

# Sales revenue by quarter





# Expenses by quarter

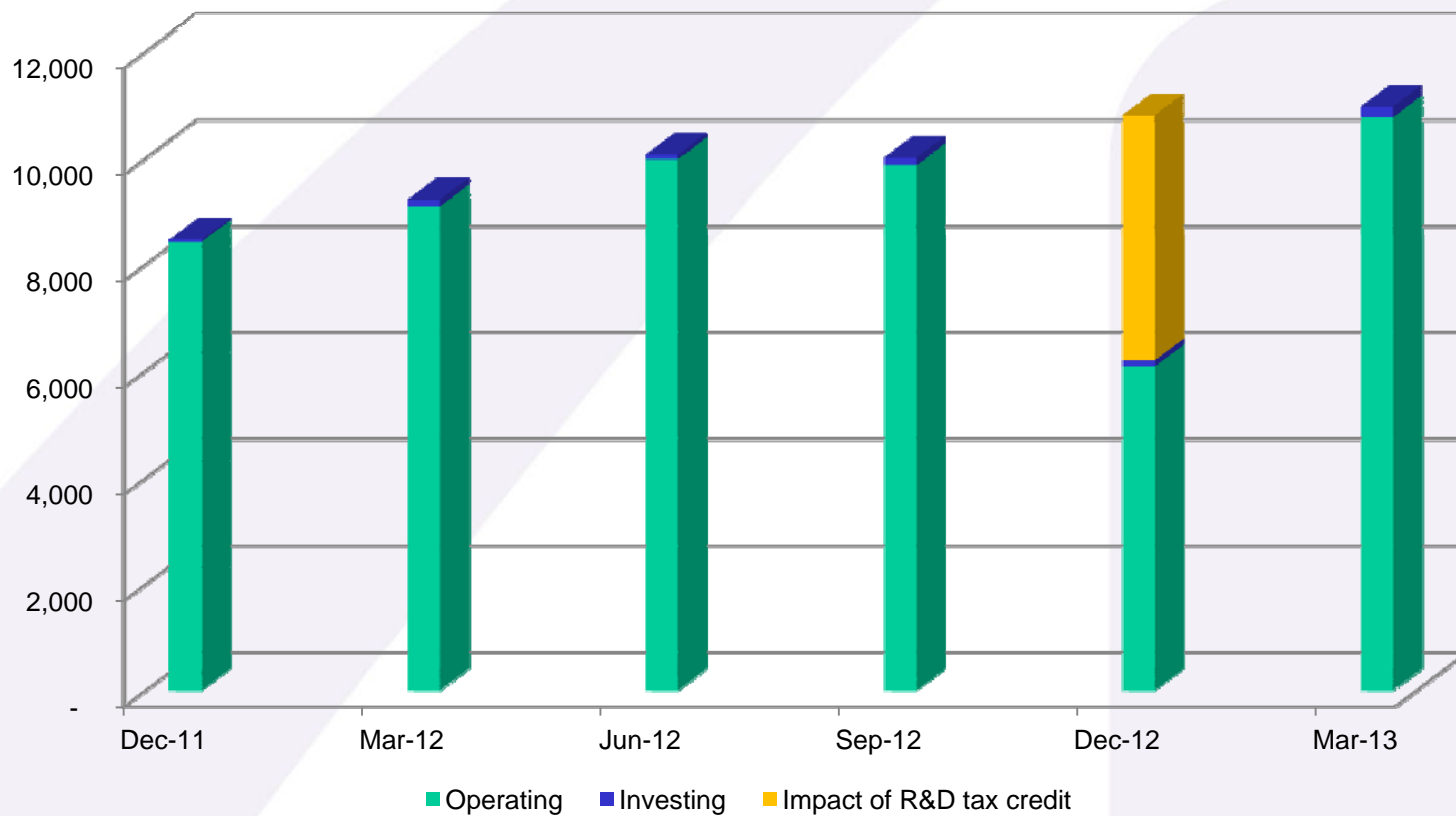


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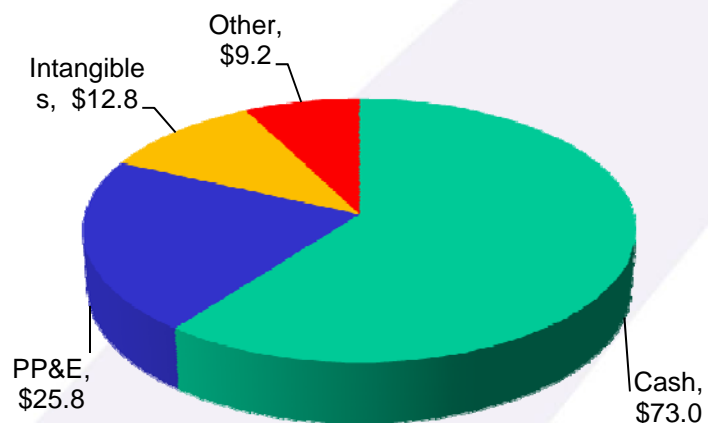
Balance Sheet Data	As at			
	31-Mar-13	30-Jun-12		
	A\$	A\$		
Cash and cash equivalents	73,000	81,475		
Property, plant & equipment	25,813	27,683		
Intangible assets	12,841	14,143		
Total assets	120,810	131,700		
Total liabilities	(39,907)	(21,897)		
Net assets	80,903	109,803		
Cash Flow Data	Three months ended		Nine months ended	
	31-Mar-13	31-Mar-12	31-Mar-13	31-Mar-12
	A\$	A\$	A\$	A\$
Cash flows from operating activities	(10,766)	(9,086)	(26,714)	(28,164)
Cash flows from investing activities	(196)	(130)	(454)	(84)
Cash flows from financing activities	19,125	(423)	18,714	75,445
Impact of foreign exchange rate movements on cash	(26)	(13)	(21)	10
Net increase (decrease) in cash held	8,137	(9,652)	(8,475)	47,207

# Operating & investing cash flow by quarter

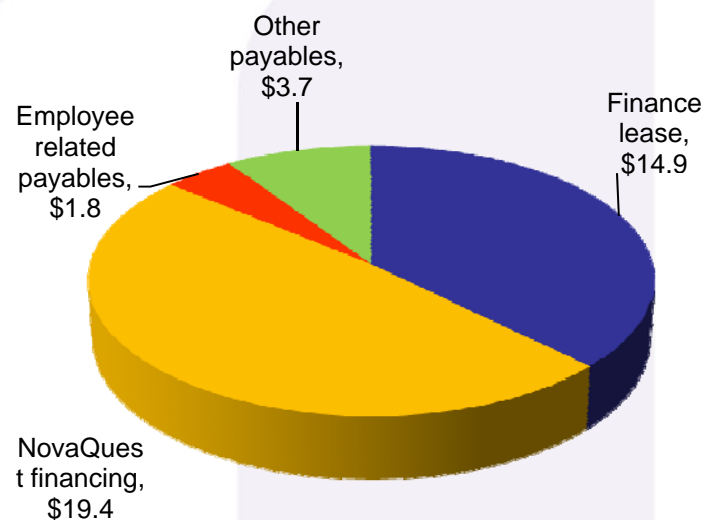


# Balance Sheet – 31 March 2013

## Assets (\$ mil)



## Liabilities (\$ mil)



### 31 March 2013

No of shareholders	7,611
Shares on issue	309 million
Options outstanding	11 million

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