

# Quarterly Investor Briefing

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**18 July 2013**

**pharmaxis**  
Innovating for life



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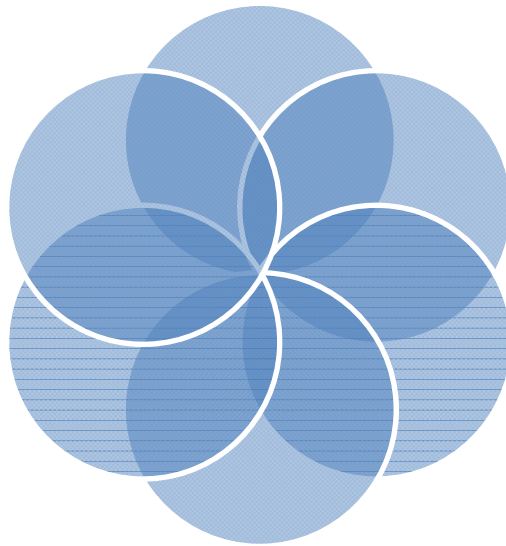
# Major events for June quarter

1 April	Bronchitol for CF listed on UK national funding guidelines
10 April	United Medical Ltda appointed exclusive distributor for Brazil
24 April	Phase 3 trial in bronchiectasis reported
21 May	Meeting with FDA clarifies clinical trial requirements for CF approval in US
22 May	PharmaSwiss appointed exclusive distributor for Poland and other Eastern European territories
28 May	Business review completed
28 May	FDA import restriction on Aridol
15 June	Completed downsize of US office
18 June	PXS presents new analyses of pooled CF data at EU Meeting
20 June	European CF trial in paediatric patients begins
30 June	Initial downsizing of clinical capability – Australia & UK
30 June	Final EU approval for producing Bronchitol at 20 Rodborough Rd

# Business plan

**A speciality pharma company with a global interest in CF and other specialist respiratory diseases**

Direct commercial interest in EU and Australian CF markets



Strategic interest in an innovative pipeline of early stage compounds

Seeking to partner assets for CF and bronchiectasis in USA and other markets of interest

# Bronchitol partnering

Process Q2- Q4 2013	Progress in June quarter
1. Maximise valuation a) agree development path b) finalise trial protocol and feasibility	<ul style="list-style-type: none"> <li>• Agreed clinical development plan for CF with FDA               <ul style="list-style-type: none"> <li>• One trial in adults</li> <li>• FEV1 endpoint</li> <li>• Trial design very close to previous trials</li> </ul> </li> <li>• Protocol drafting well advanced</li> <li>• First patient expected H1 2014</li> <li>• Paediatric plan discussion with FDA to follow review of adult trial</li> </ul>
2. Shortlist potential partners	<ul style="list-style-type: none"> <li>• Contacted all companies having current or potential interest in CF and/or other respiratory diseases</li> </ul>
3. Marketing and due diligence a) CF b) Bronchiectasis	<ul style="list-style-type: none"> <li>• Process underway – discussions commenced</li> <li>• Reviewing bronchiectasis data base</li> </ul>
4. Assess value	Quarter 4

# Direct commercial interest in CF

Plan	Progress last quarter
1. European sales	<ul style="list-style-type: none"> <li>• Continued penetration of CF centres</li> <li>• Initiated comprehensive program to increase patient retention and compliance</li> <li>• UK pricing from 1 April – key account managers change focus from NHS reimbursement applications to product attributes</li> <li>• French pricing negotiations continue</li> <li>• Progress pricing applications for remaining EU markets</li> <li>• CF204 begins as scheduled</li> </ul>
2. Australian sales	<ul style="list-style-type: none"> <li>• PBAC considered revised continuation rule at July meeting</li> <li>• Planning marketing efforts to maximise opportunity from updated PBS listing</li> </ul>
3. Rest of world	<ul style="list-style-type: none"> <li>• Distributor appointed for Poland</li> <li>• Distributor appointed for Brazil</li> <li>• Filed marketing approval application in Israel</li> <li>• Preparing marketing approval application for Russia</li> <li>• Preparing marketing approval application for Brazil</li> </ul>
4. Leverage sales infrastructure	<ul style="list-style-type: none"> <li>• Launched sale of special CF vitamins in UK</li> </ul>

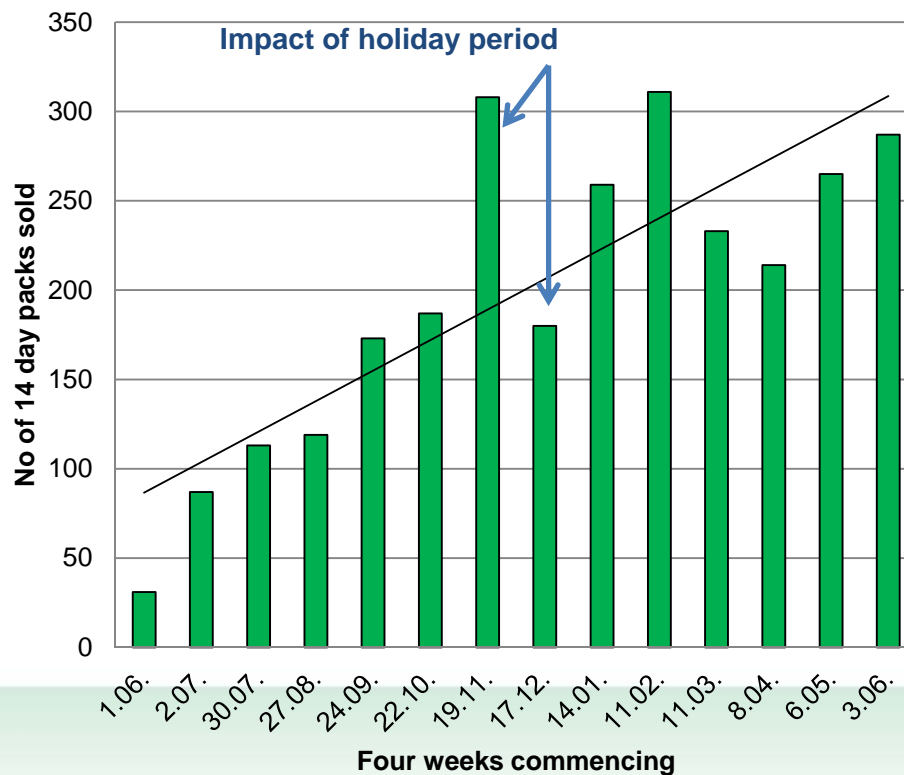
# Germany

- Strong uptake of Bronchitol by clinics across Germany
  - Over 90% of larger clinics have prescribed Bronchitol
  - 70% of all clinics have prescribed Bronchitol
  - Patient market share in larger clinics
    - >30% in ¼ of centres
    - > 10% in more than ½ of centres
- Patients on Bronchitol continues to increase, but cycling on/off drug is a concern for all CF drugs
- Market priorities
  - Increase centre penetration
  - Reduce patient cycling on / off drug
  - Improve adherence

# Germany 4-weekly sales

## growth slower than anticipated

### 14 day pack sales per 4 week periods



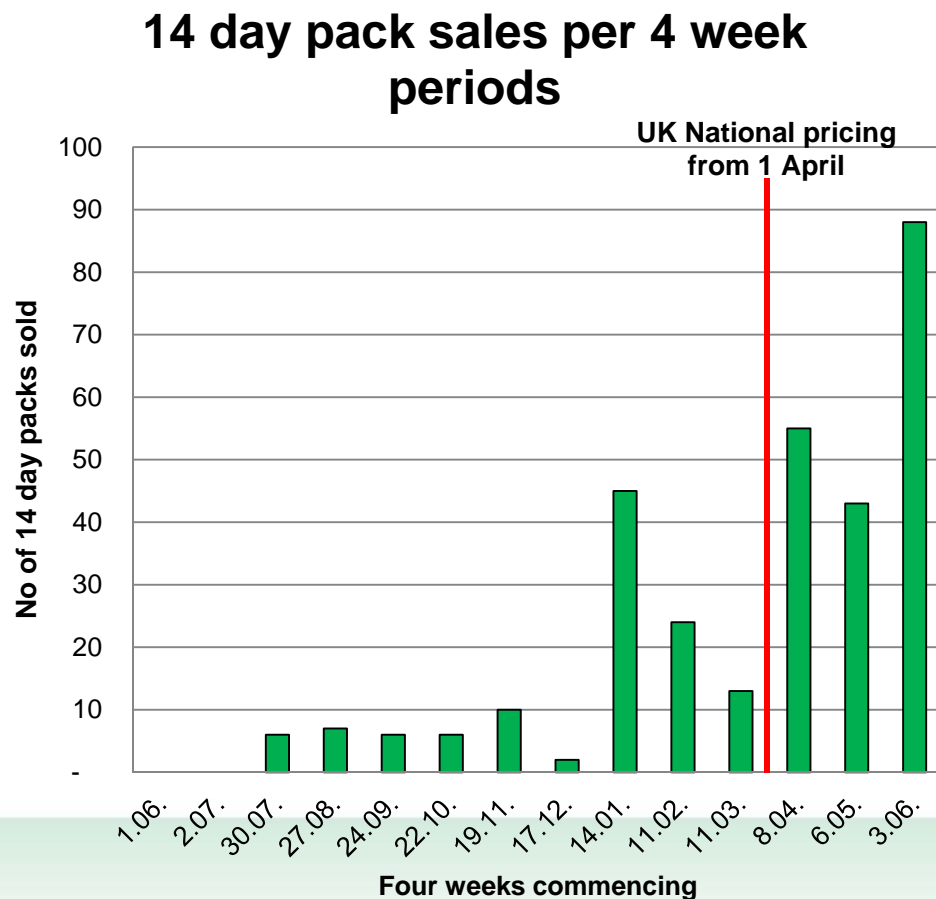
### Marketing Imperatives

- Increase centre penetration
  - Establish Bronchitol as the standard
  - Support peer-to-peer communication – locally, CF conferences
- Reduce patients cycling on and off Bronchitol (impact Q3/4)
  - Physio Pilot with 5 centres in Germany that currently provide no physio support
  - Emphasis on long term benefits of Bronchitol therapy
- Improve adherence (impact Q4)
  - Behavioural training for Sales team and healthcare professionals
  - New support materials
  - Pilot behavioural intervention



# UK 4-weekly sales

majority of sales from top 3 centres



## Marketing Imperatives

### Increase centre penetration

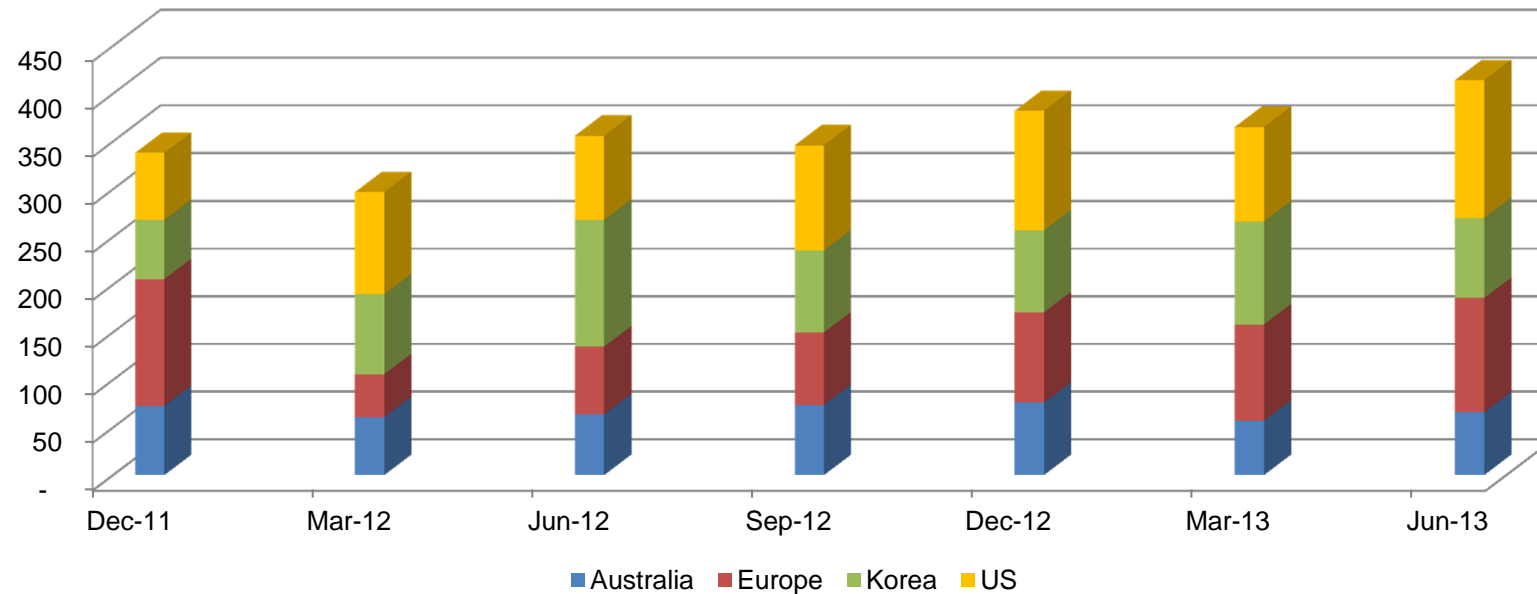
- Sharing positive experiences
  - Physiotherapist meetings
  - Posters and presentations at UK CF meetings
- Increasing share of voice
  - Increase in sales resource
- Improved health economic tools
  - Account level data presentation
- Adherence rates are good

# Australia

- Sales growth disappointing
  - All but two clinics have prescribed Bronchitol
  - Adherence rates are OK
  - Rates of trial are lower than expected due in the main to PBS restrictions
- Marketing imperative – increase rates of trial
  - Seek change to PBS prescribing restrictions
  - Share positive experiences from heavy users
  - Exploit large pool of treatment failures

# Aridol

## Quarterly Aridol Sales (A\$'000)



- Sales increased 17% over June 2012, 14% over March 2013
- Investing in US sales growth – (full year 38%), costs reduced June quarter
- Ex US maintains sales with minimal investment (1 FTE)

# R&D strategy

## 1. Pursue multiple strategies to fund development from Q1 2014

### a. Pharma research collaborations

- Contacted all companies having current or potential interest in targets
- Process underway – discussions commenced

### b. Grants

- Awarded two ARC linkage grants
- Applying for other larger overseas grants

### c. Spin outs (VC,IPO)

- Discussions commenced with interested VC's

## 2. Continue to invest selectively in proof of concept H2 2013

# SSAO candidate: PXS4728A

- PXS-4728A is an anti-inflammatory drug, particularly in neutrophilic inflammation, and has anti-fibrotic properties. Both conditions are linked to various human disease including
  - COPD: > 30 million people; \$9b annual spend; sales ~\$200m
  - Cystic fibrosis: 75,000 people
  - Liver fibrosis: >30 million patients; \$12.4b annual spend; sales ~\$300m
- PXS-4728A is a mechanism-based potent and selective SSAO/VAP-1 inhibitor with an excellent developability profile.
- Competitive product profile
  - Oral
  - Once-a-day
- Limited competitor activity/attracting pharma interest
- Status
  - Preclinical
  - Investment for remainder of CY: proof of concept (COPD/asthma)
- Additional information – refer PXS website

# LOXL2 inhibitor pre-candidate: PXS-5033A

- LOXL2 is upregulated in fibrosis and various cancers
- PXS-5033A is a small molecule with anti-fibrotic properties. Linked to various human disease including
  - Lung fibrosis
  - Liver fibrosis: >30 million patients; \$12.4b annual spend; sales ~\$300m
  - Cancers
- PXS-5033A is a mechanism-based potent and selective LOXL2 inhibitor with a promising developability profile.
- Expected competitive product profile
  - Oral
  - Once-a-day
- Limited competitor activity other than Gilead who paid \$225m to acquire Arresto's LOXL2 antibody program in 2010
- Status
  - Discovery
  - Investment for remainder of CY: proof of concept (lung/liver)
- Additional information – refer PXS website

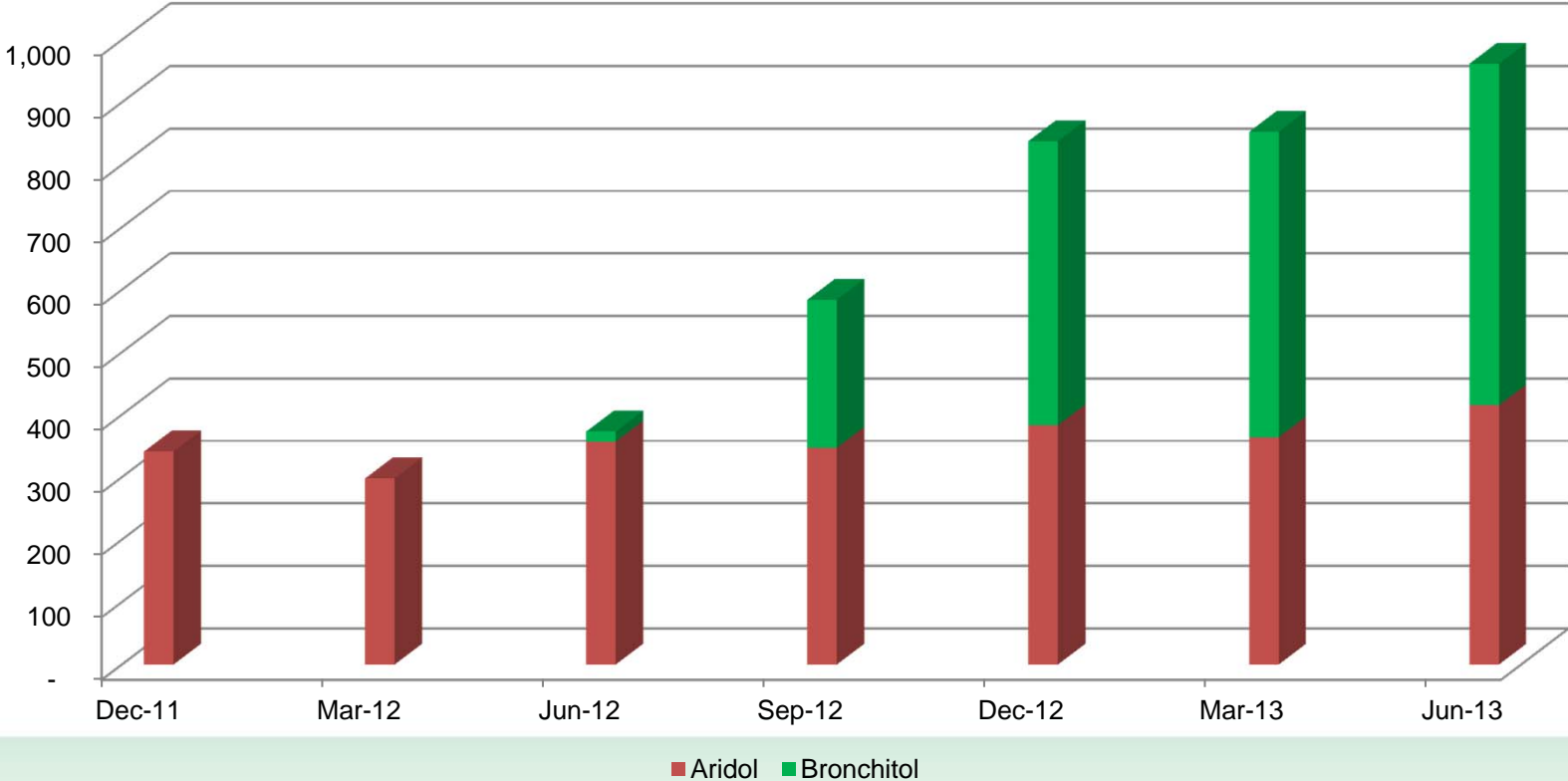
# Financial statements – unaudited

('000 except per share data)

Income statement - unaudited (‘000 except per share data)	Three months ended		Year-to-date	
	30-Jun-13	30-Jun-12	30-Jun-13	30-Jun-12
	A\$	A\$	A\$	A\$
Revenue from sale of goods	962	373	3,237	1,331
Cost of sales	(326)	(143)	(1,140)	(522)
Gross profit	636	230	2,096	809
Interest income	708	968	2,695	3,049
Grant and other income	1,687	1,440	5,675	3,874
Expenses				
Sales & marketing	(3,793)	(3,715)	(13,893)	(11,073)
Regulatory, safety & medical affairs	(1,113)	(1,446)	(5,581)	(4,904)
Administration	(1,303)	(1,372)	(6,030)	(5,248)
Research & development - Bronchitol	(4,351)	(4,695)	(18,531)	(19,850)
Research & development - new drug development	(1,645)	(1,270)	(5,331)	(4,519)
Finance & royalties	(2,293)	(222)	(2,944)	(856)
Restructuring charges	(1,690)	-	(1,690)	
Total expenses	(16,188)	(12,720)	(54,001)	(46,450)
Net loss before tax	(13,158)	(10,082)	(43,535)	(38,718)
Income tax expense	62	(49)	(2)	74
Net loss after tax	(13,095)	(10,131)	(43,537)	(38,644)
Basic and diluted earnings (loss) per share - \$	(0.042)	(0.033)	(0.141)	(0.142)

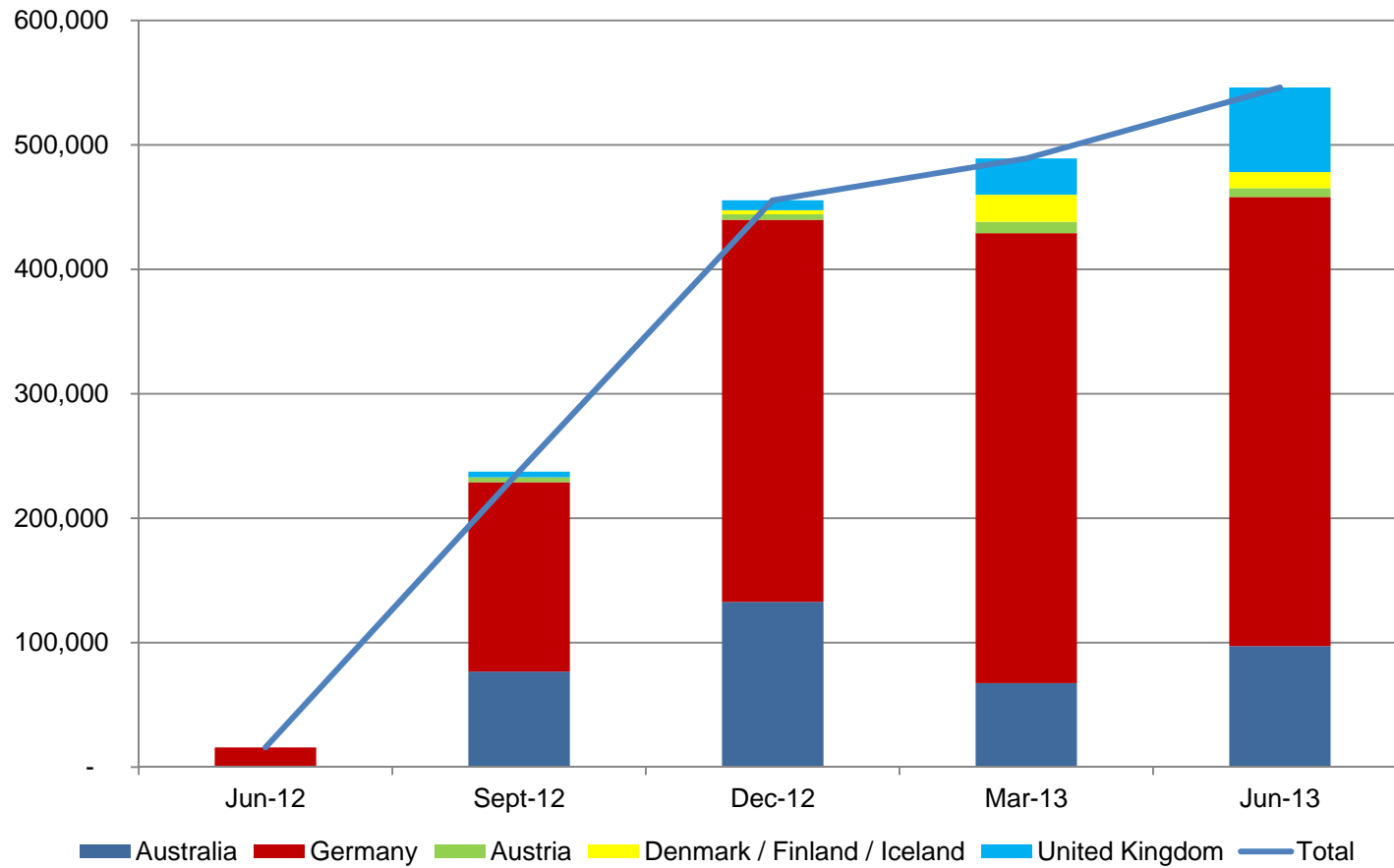
# Sales revenue by quarter

Quarterly Sales  
(A\$'000)





# Bronchitol sales by country (A\$)



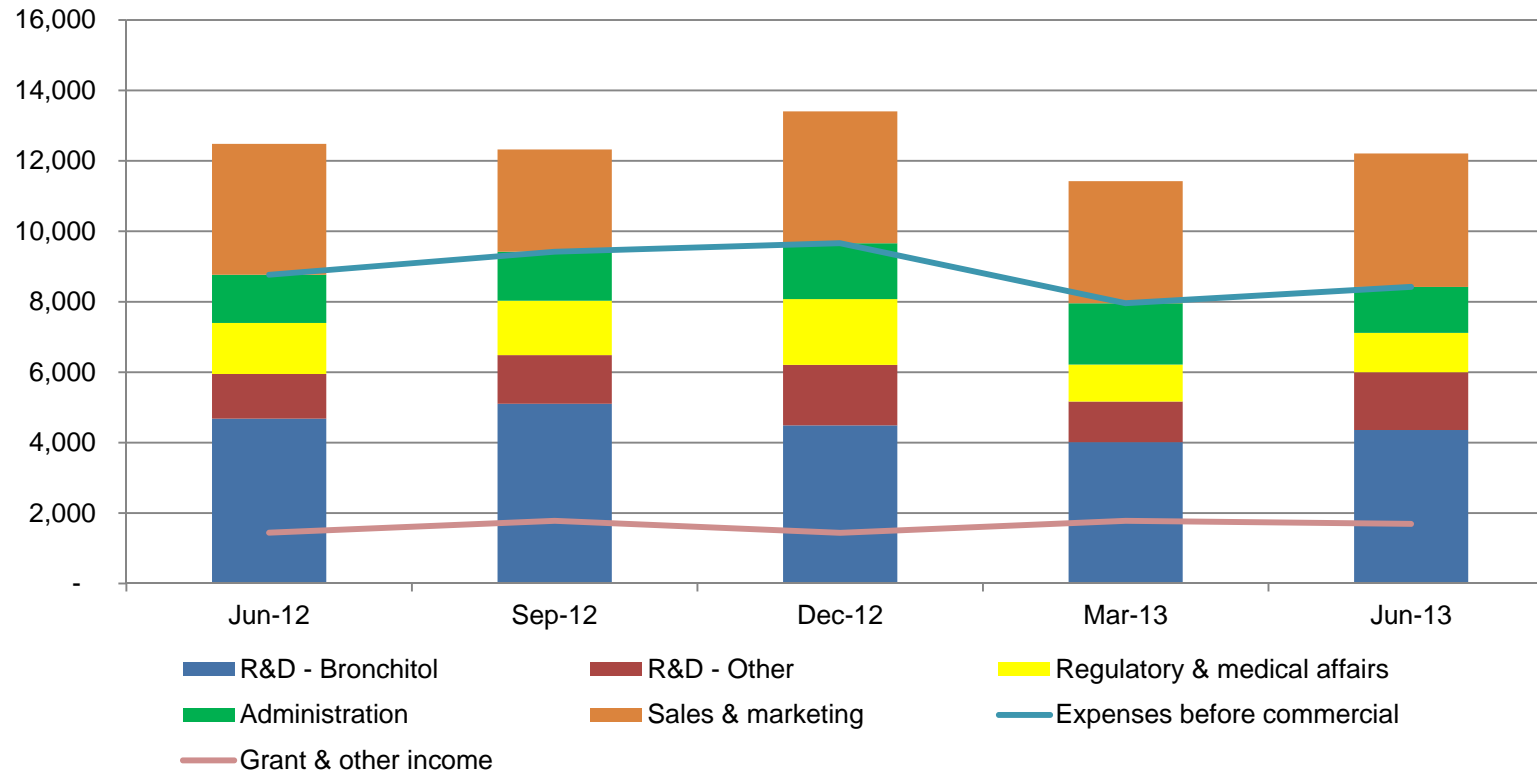
# Financial statements – unaudited

('000 except per share data)

Normalised cash loss & expenses - unaudited ('000 except per share data)	Three months ended		Year-to-date	
	30-Jun-13	30-Jun-12	30-Jun-13	30-Jun-12
Net loss after tax	(13,095)	(10,131)	(43,537)	(38,644)
Non cash expenses				
Depreciation	505	518	2,044	2,108
Amortisation	636	637	2,538	2,557
Share based compensation	39	201	1,370	957
NovaQuest finance charge	2,043	-	2,043	-
	3,222	1,356	7,995	5,622
Restructuring charges	1,690	-	1,690	
Net cash loss	(8,182)	(8,775)	(33,852)	(33,022)
Total cash expenses	11,275	11,365	44,316	40,928

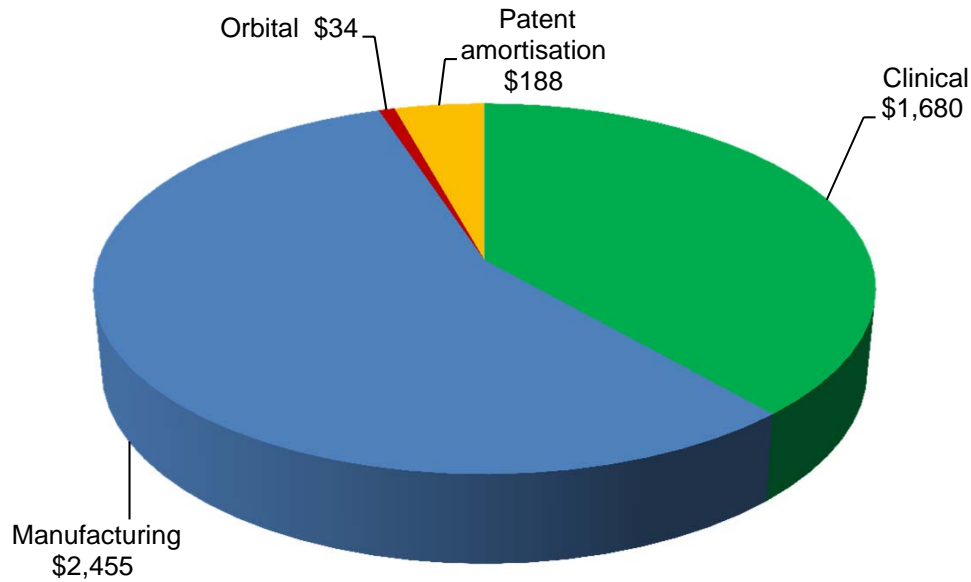
# Expenses by quarter

(excluding restructuring and finance costs)

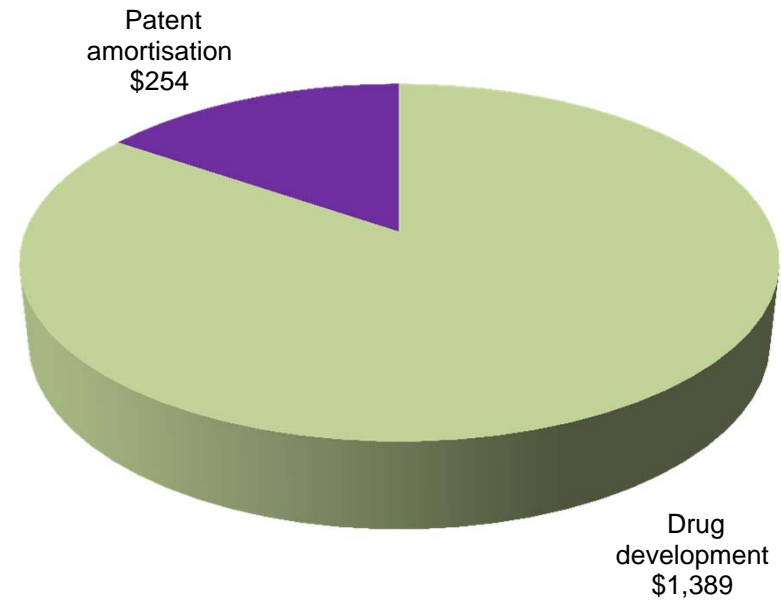


# Research & development – June quarter (A\$'000)

**Bronchitol R&D (\$4,356k)**



**New Drug Development (\$1,643k)**



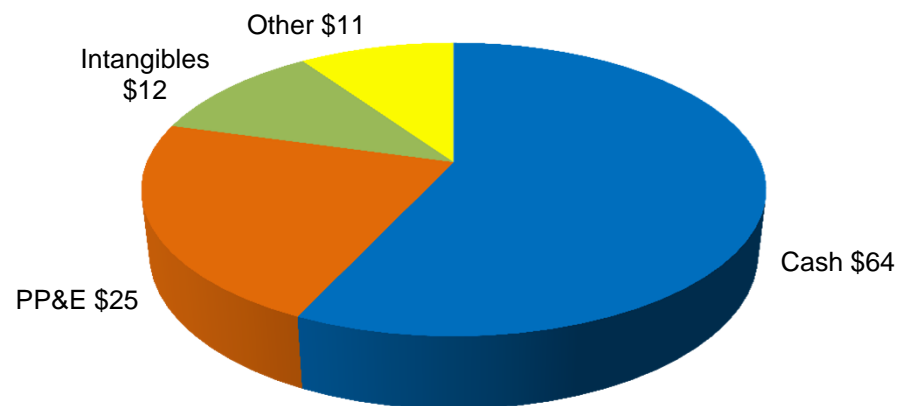
Net cash cost after tax  
credit: \$2,765k

Net cash cost after tax  
credit: \$847k

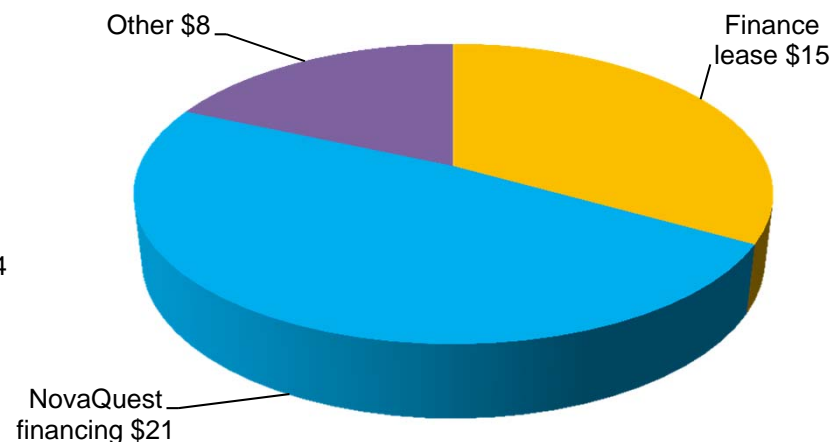
# Balance sheet – 30 June 2013

(A\$mil)

### Assets (\$112 mil)



### Liabilities (\$44 mil)

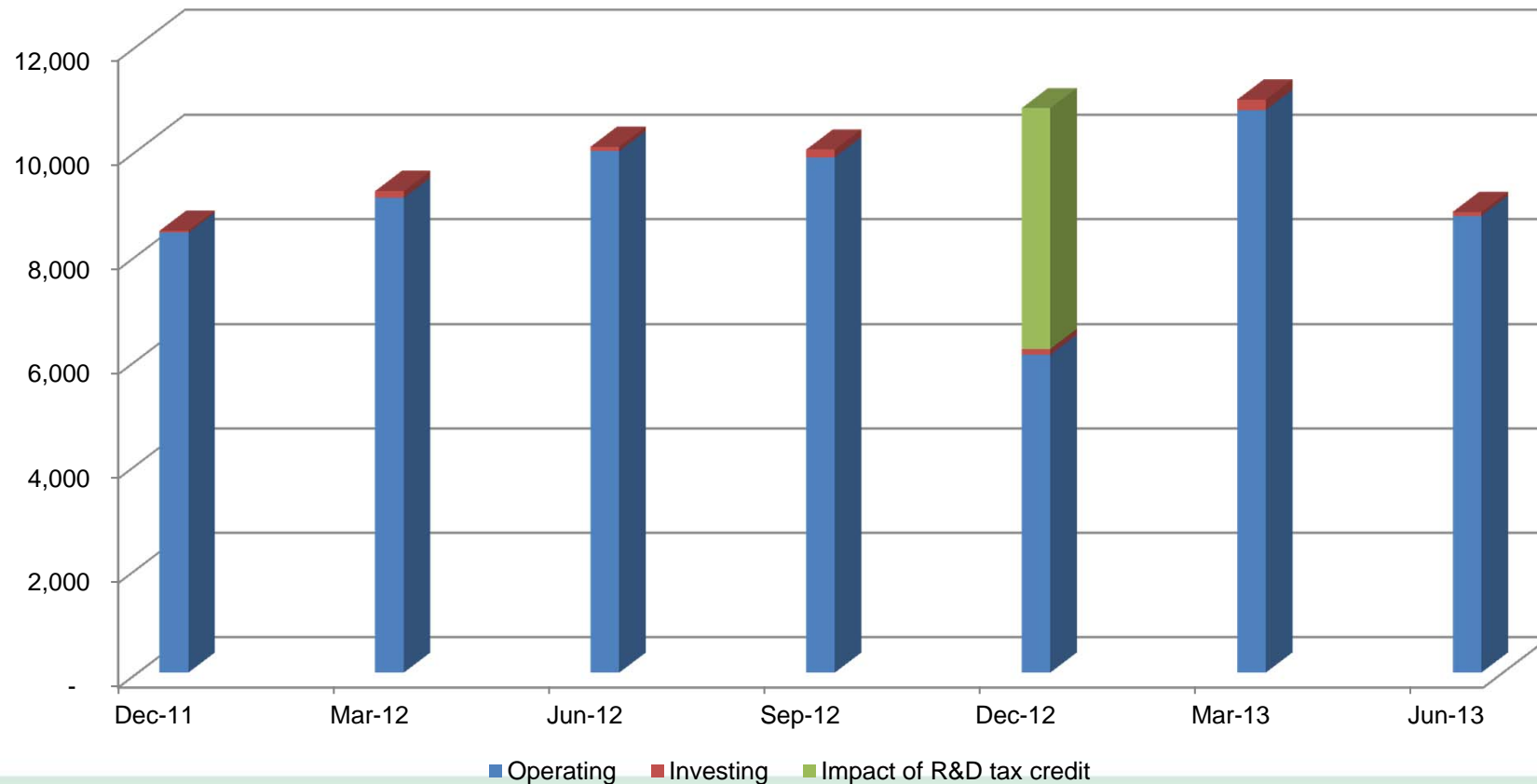


Additional US\$20m available under NovaQuest agreement on commencement of US CF trial

No of shareholders	7,500
Shares on issue	309 million
Options outstanding	19 million

# Operating & investing cash flow by quarter

(A\$'000)



# Cost reduction progress

Department	Plan	Costs reductions implemented at 30 June		
		<u>\$mil</u>	<u>Action</u>	<u>Headcount</u>
Sales & marketing	3.9	US sales staff & office	10	2.7
Clinical	2.0	Reduction in employees	15	1.8
Manufacturing	3.5	Notice given to exit 10 Rodborough Road	-	0.5
Corporate/admin	0.4	Headcount & other	2	0.4
Drug development	2.0	Refer R&D strategy above	-	
Other	-		1	0.2
<b>Total</b>	<b>\$11.8</b>		<b>28</b>	<b>\$5.6m</b>

# The way forward

(Business Review 28 May)

1. Reduce cash burn on non essential capabilities
  - Implemented half of plan by 30 June
2. Retain / invest in capabilities that are fundamental to value generation
  - Investment in partnering projects, growing sales, select drug development
3. Partnership strategy to mitigate short term risk on cash intensive activities
  - Process well underway - encouraged by initial responses
4. Alternative funding to maintain progress in R&D programs
  - Pursuing multiple strategies - encouraged by initial responses



**END**

