

# Quarterly Investor Briefing

**Gary Phillips**  
**Chief Executive Officer**  
**20 February 2014**

**pharmaxis**  
Innovating for life



# Forward looking statements

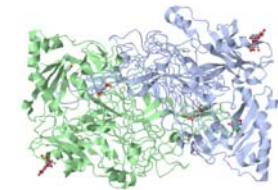
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We are not under any duty to update forward-looking statements unless required by law.  
This investor presentation is not an offer of the sale of securities.

# pharmaxis

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

- Approved products      Bronchitol® for cystic fibrosis  
                                   Aridol® for diagnosis of asthma
- Products in development      LOXL2 inhibitor: fibrosis, cancer  
                                   PXS4728: SSAO inhibitor; anti-inflammatory  
                                   ASM8 / PXS2200: Antisense oligonucleotides; asthma, COPD
- Operations                      Headquartered in Australia with operations in Europe and US
- Production                      GMP manufacture of respirable dry powders



# Business plan objective: pursue short term value drivers to reshape the business model



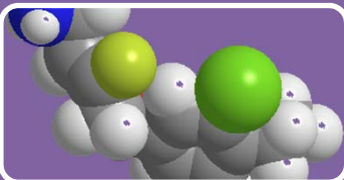
## Bronchitol for US

- The US revenue opportunity<sup>1</sup> is US\$160m (adults)
- Objective 1: Clarify the US approval path – FDA requirements, time and cost to conduct clinical study
- Objective 2: Conduct a partnering process



## Sales of Bronchitol for CF in rest of world

- The revenue opportunity<sup>1</sup> for adults: US\$75m in EU & Australia (direct); US\$70m in RoW (indirect)
- Objective 1: Achieve sales growth in approved/priced markets
- Objective 2: Access new markets – obtain pricing approvals in approved markets; appoint distributors and seek approvals in new markets



## Early stage pipeline

- Objective 1: Identify and secure external funding for early stage research program while retaining a strategic interest in the programs
- Objective 2: Select investment in pipeline to advance near term value and success of funding



## Secure financial footing

- Objective 1: Achieve a reduction in the cost base

1. Revenue opportunity is based on sales of rhDNase

# Business plan objective: achievements & conclusions



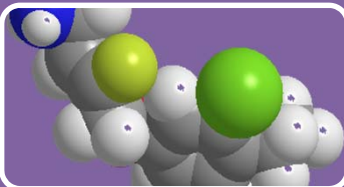
## Bronchitol for US

- Bronchitol is an asset valued by Pharma companies with a strategic interest in CF
- Agreed the study design required for approval with the FDA and engaged a CRO - currently preparing to commence the study in H1 CY2014
- Term sheets currently being received/evaluated



## Sales of Bronchitol for CF in rest of world

- Good patient uptake of Bronchitol occurs in well supported/resourced CF centres once funding hurdles (national & local) are resolved – focus resources on larger clinics
- Developed toolkit of resources to enable specific solutions in other centres (especially Germany)
- New pricing approvals will soon open new EU countries (e.g. Netherlands)
- New distribution agreements in place for Eastern Europe and other countries to provide future growth



## Early stage pipeline

- Both the LOXL2 and SSAO programs are valued by Pharma
- LOXL2 is a well validated target attracting Big Pharma interest
- It is possible to fund an aggressive research program and retain a significant strategic interest for PXS
- Term sheets currently being received/evaluated



## Secure financial footing

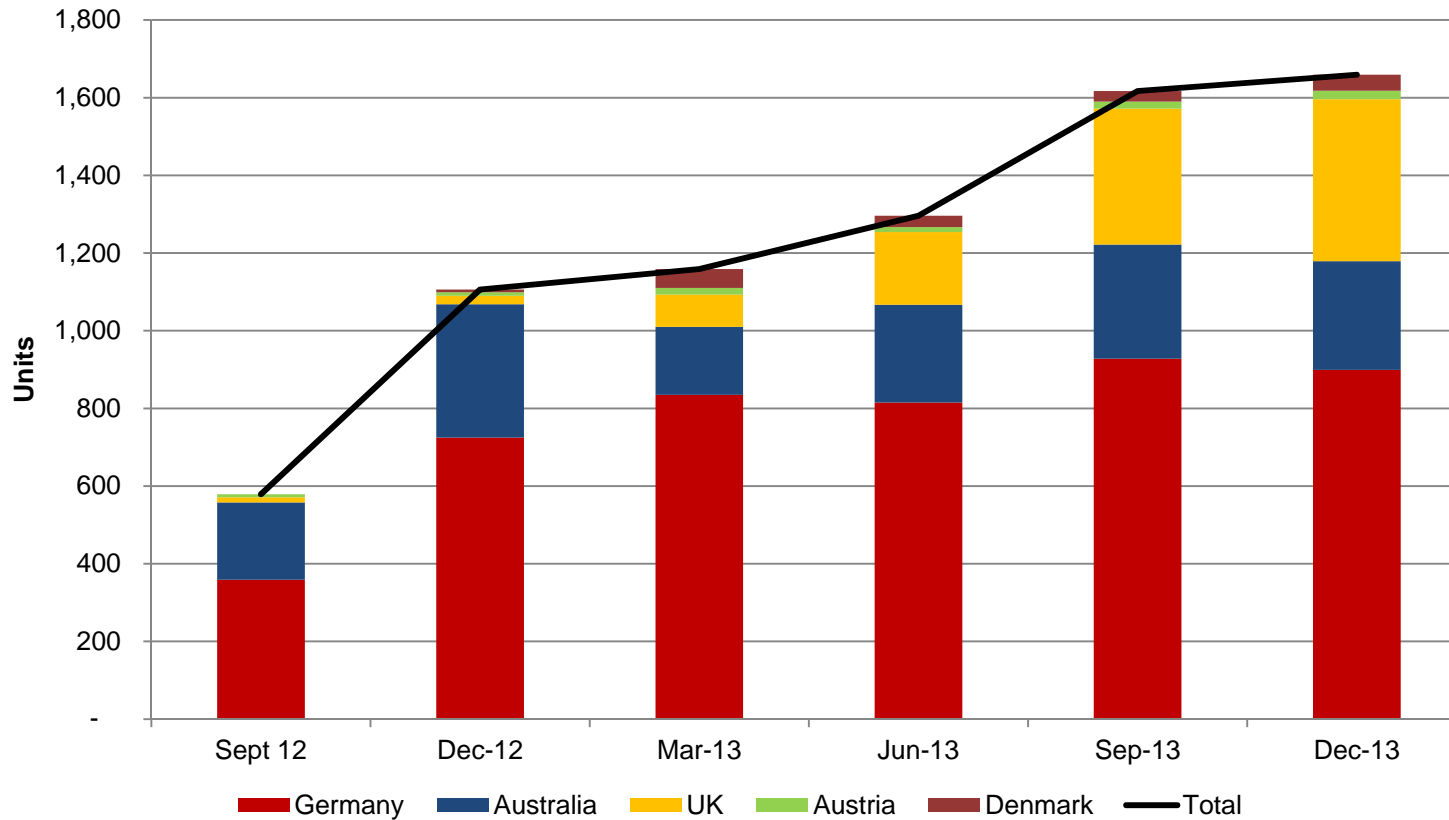
- Cost reduction targets will be achieved – ongoing process
- Successful partnering of Bronchitol and early stage pipeline will reduce expenditure and introduce milestone payments to Pharmaxis

# Bronchitol for US

Objective	Progress in Q4 CY 2013	Next
1. Clarify the US approval path		
<ul style="list-style-type: none"> <li>FDA requirements for CF303 – a “tie breaker” study</li> </ul>	<ul style="list-style-type: none"> <li>The FDA reviewed the study protocol</li> <li>Minor comments have been addressed</li> <li>The trial design remains very close to previous trials</li> </ul>	<ul style="list-style-type: none"> <li>Study protocol completed in January 2014</li> <li>Paediatric development plan to follow</li> </ul>
<ul style="list-style-type: none"> <li>Time and cost to conduct clinical study</li> </ul>	<ul style="list-style-type: none"> <li>Completed evaluation of contract research organisations to conduct trial</li> <li>Selected INC</li> <li>Costs within expected range (A\$15-20m)</li> <li>Study start up preparations underway</li> </ul>	<ul style="list-style-type: none"> <li>CF303 first patient H1 CY2014</li> </ul>
2. Conduct a partnering process		
	<ul style="list-style-type: none"> <li>Formal partnering process progressed – a number of companies have substantially completed diligence</li> <li>Term sheets now being received (Q1 CY2014)</li> </ul>	<ul style="list-style-type: none"> <li>Negotiations with preferred partners</li> <li>NovaQuest funding enables deferral of partnering for greater value at later date</li> </ul>

# Bronchitol unit sales by country

(1 unit = 14 day pack of Bronchitol)

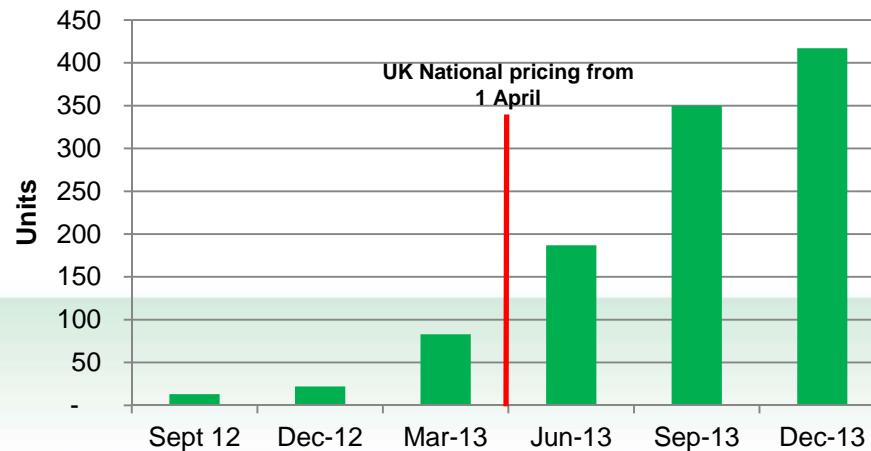


Unit sales increase over	Bronchitol packs (14 days)			
	Germany	Australia	UK	Total
Dec-12	24%	-18%	1795%	50%
Sep-13	-3%	-5%	19%	3%

# Sales of Bronchitol - UK

Status/Progress in Q4 CY 13	Next
<ul style="list-style-type: none"> <li>• Sales growth of 19% over September 13</li> <li>• 65% of clinics prescribing Bronchitol – a 45% increase in the quarter</li> <li>• Centres continue to introduce patients to Bronchitol</li> <li>• Patient feedback and centre staff remain very positive</li> <li>• Good compliance and patient retention</li> <li>• Experienced former CF Trust staffer joins European team as patient advocacy manager – working with individual centres on funding and adherence</li> </ul>	<ul style="list-style-type: none"> <li>• Resolve remaining funding issues in a number of centres arising from new national guidelines</li> <li>• Continue to support adherence</li> </ul>

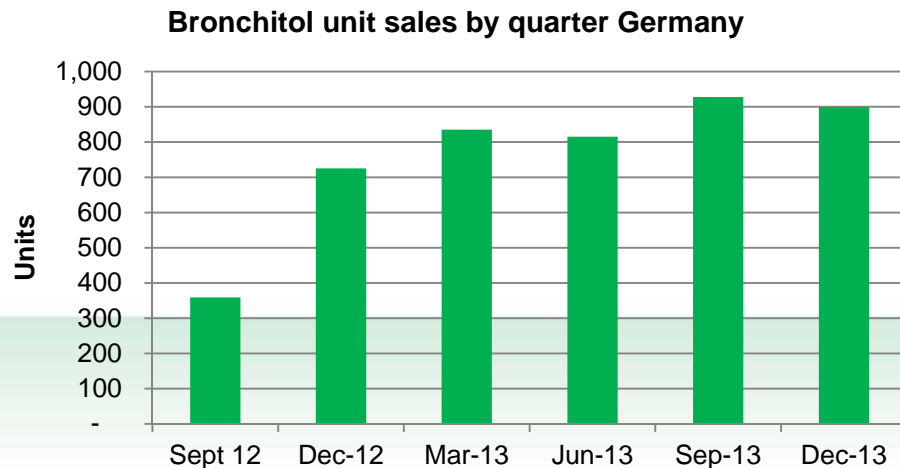
**Bronchitol unit sales by quarter  
United Kingdom**





# Sales of Bronchitol - Germany

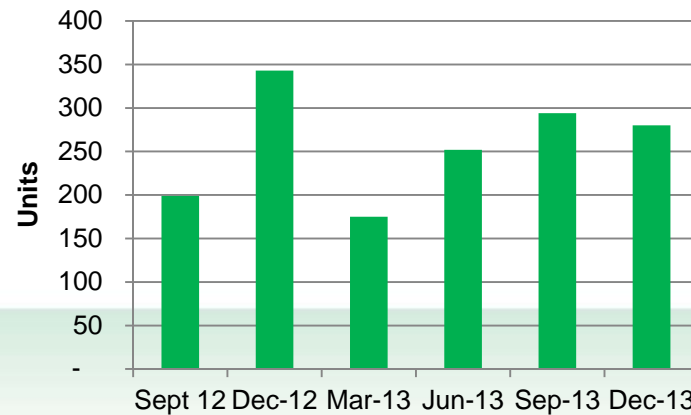
Status/Progress in Q4 CY 13	Next
<ul style="list-style-type: none"> <li>• Sales did not meet expectations – no increase in unit sales over Sept 13</li> <li>• Physio support program effective in pilot centres – significant improvement in patient retention (~80%)</li> <li>• Completed a full review of our approach to the market and resource allocation in December/January               <ul style="list-style-type: none"> <li>• No single issue constraining centres – need to give greater attention to each clinic’s specific issues</li> <li>• Requirement for a “toolkit” approach to support specific programs for each centre</li> <li>• Requirement to change the skill set of the team</li> </ul> </li> </ul>	<p>Implementation of revised approach:</p> <ul style="list-style-type: none"> <li>• Jan 14: redirected sales &amp; marketing resources</li> <li>• Focus on 40 larger clinics (&gt;80% of patients) – develop business plans for each centre</li> <li>• Toolkit includes physio support program, Bronchitol champions in each centre, adherence program and smartphone App.</li> <li>• Mar 14: national roll out of adherence programs</li> </ul>



# Sales of Bronchitol - Australia

Status/Progress in Q4 CY 13	Next
<ul style="list-style-type: none"> <li>Sales growth did not meet expectations – some seasonal impact</li> <li>PBS changes effective 1 November 2013</li> <li>Revised PBS rules introduced to all centres by 31/1</li> <li>Developed new selling position &amp; materials</li> <li>Patient compliance near optimum</li> </ul>	<ul style="list-style-type: none"> <li>Increase rates of trial of Bronchitol by patients</li> <li>Support adherence</li> </ul>

**Bronchitol unit sales by quarter Australia**



# Sales of Bronchitol – rest of world

Status/Progress in Q4 CY 13	Next
<ul style="list-style-type: none"> <li>• Scottish Medicines Consortium (SMC) accepted Bronchitol – SMC influential in other EU markets. First sales made in Scotland</li> <li>• Netherlands agree to waiver of full pricing submission</li> <li>• Ireland – Key opinion leaders and pricing model support broad pricing approval</li> </ul>	<ul style="list-style-type: none"> <li>• Netherlands – launch expected Q3 CY2014</li> <li>• Ireland – pricing submission Q1 CY2014</li> <li>• Denmark – application to improve pricing status</li> <li>• Progress pricing applications for remaining EU markets</li> </ul>
<ul style="list-style-type: none"> <li>• Appointed distributors and commenced local approval/pricing submissions               <ul style="list-style-type: none"> <li>• Russia (Australian label for over 6 yrs)</li> <li>• Czech Republic &amp; Slovakia</li> <li>• Turkey</li> </ul> </li> </ul>	<ul style="list-style-type: none"> <li>• Appoint distributor for Israel</li> <li>• File additional marketing applications</li> </ul>
<ul style="list-style-type: none"> <li>• Italian business manager employed as named patient program commences in Italy</li> </ul>	<ul style="list-style-type: none"> <li>• Italian named patient program commence Q1 2014 – first step in formal pricing approval process</li> </ul>

# Pharmaxis drug discovery assets

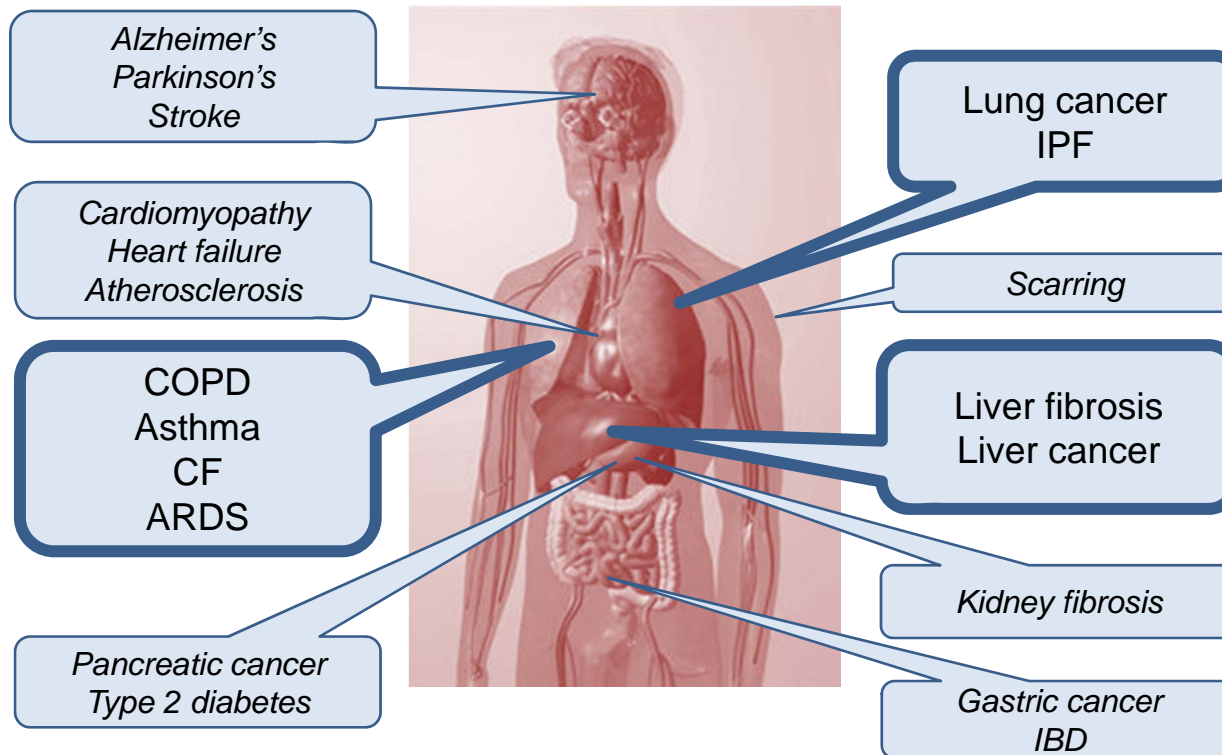
**Pharmaxis has a globally competitive research program based on an amine oxidase platform**

- Experienced discovery and clinical teams
- Pharmaxis drug discovery comprises state of the art facilities opened 2009
- Global leaders in amine oxidase chemistry
- Investment focus on value adding to clinical proof of concept for two lead compounds for inflammation and fibrosis



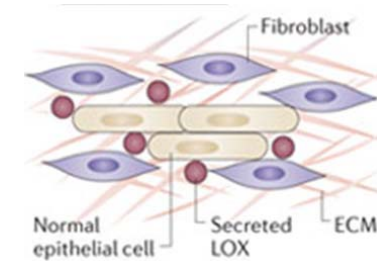
# Early stage pipeline - potential indications

Broad clinical indication opportunities exist for the amine oxidase inhibitors



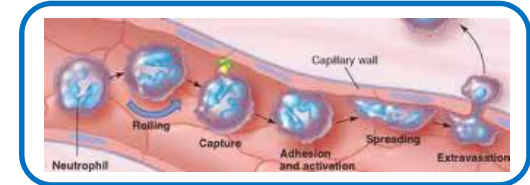
**The amine oxidase platform technology and its associated strong IP position is a source of long term value for Pharmaxis**

# LOXL2 inhibitor

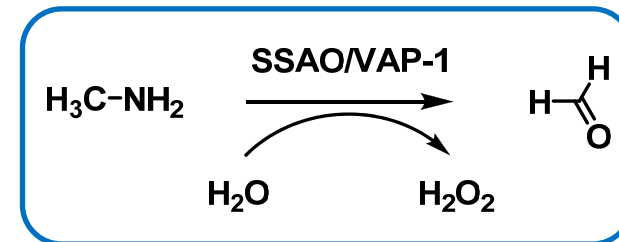


- LOXL2 is an enzyme linked to various human diseases that have high levels of unmet need and significant patient numbers
  - E.g. Pulmonary fibrosis, liver fibrosis, solid tumour cancers
- Pharmaxis LOXL2 inhibitor is a small molecule with anti-fibrotic properties
- Limited competitor activity other than Gilead who paid US\$225m to acquire Arresto's LOXL2 phase 1 antibody program in 2010
- Expected competitive product profile
  - Oral / Once-a-day
- Development Status
  - Discovery – 3<sup>rd</sup> generation compounds with broad IP protection (filed 2013)
  - Lead compounds show excellent efficacy in in-vivo models of fibrosis
  - Requires preclinical safety before proceeding into man
- Commercial status
  - Several Big Pharma companies at advanced stages of due diligence
  - Term sheets being received
- Additional scientific information – refer PXS website

# SSAO inhibitor – PXS4728A



- SSAO / VAP-1 is an enzyme that is up-regulated in neutrophilic inflammation, liver and kidney fibrosis, cardiovascular diseases, cancer and metabolic disorders
- PXS-4728A is a mechanism-based potent and selective SSAO/VAP-1 inhibitor with an excellent drug profile.
- Competitive product profile
  - Oral / Once-a-day
- Limited competitor activity
- Development Status
  - Discovery – Pre clinical candidate identified with broad IP protection (filed 2012)
  - PXS-4728 show excellent efficacy in in-vivo models of fibrosis and neutrophilic inflammation
  - Requires preclinical safety before proceeding into man
- Commercial status
  - Several Big Pharma companies engaged in due diligence
  - Seeking research collaboration with suitably qualified Pharma
- Additional scientific information – refer PXS website



# Early stage pipeline

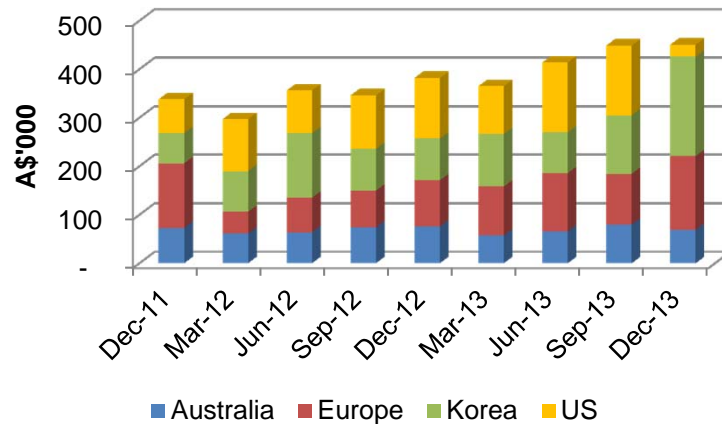
Objective	Progress in Q4 CY 13	Next
1. Identify and secure external funding for early stage research program while retaining a strategic interest in the programs		
Pursue multiple strategies: a. Pharma research collaborations b. Grants c. Spin out of R&D assets	<ul style="list-style-type: none"> <li>Received term sheets which meet our objectives of both funding and retained strategic interest</li> <li>Recent significant interest by large Pharma companies now well advanced in diligence</li> <li>Spinout alternative still a viable alternative</li> <li>Continue to apply for grants</li> </ul>	<ul style="list-style-type: none"> <li>Receive additional term sheets</li> <li>Assess value</li> <li>Negotiate with preferred partner(s)/collaborator(s)</li> </ul>
2. Select investment in pipeline to advance short term value and success of funding		
	<ul style="list-style-type: none"> <li>LOXL2 program significantly advanced:               <ul style="list-style-type: none"> <li>Selected pre-lead candidate</li> <li>Additional Proof of Concept underway</li> </ul> </li> <li>No investment in other early stage programs</li> <li>R&amp;D tax credit significantly subsidises expenditure</li> </ul>	<ul style="list-style-type: none"> <li>Minimal investment pending completion of R&amp;D collaboration</li> </ul>



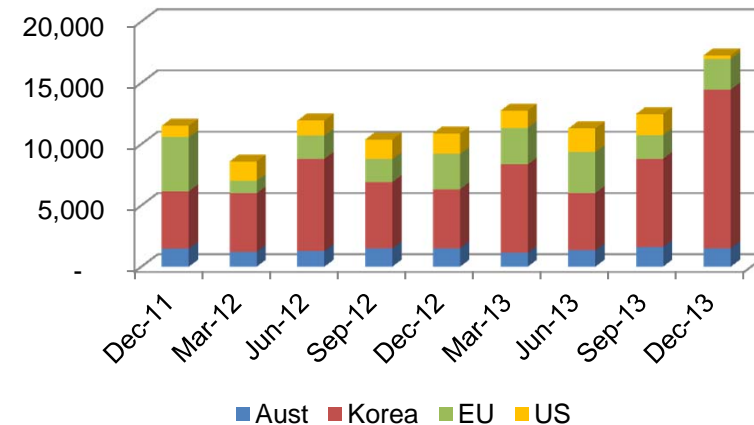
# Aridol

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

### Quarterly Aridol Sales



### Quarterly Unit Aridol Sales



- Sales increased 18% compared with Dec 2012, flat with Sept 13
- Unit sales increased 59% over Dec 2012, 38% over Sep 2013
- Change in sales mix from US to Korea (distributor)
- Minimal sales investment of 3.0 FTE's
- Improved EU profitability – selling directly in larger Scandinavian market; rationalising EU countries we supply
- US sales expected to recommence H1 CY2014

# Secure financial footing

Objective	Achieved	Next
1. Reduce cost base by \$11.8m p.a. (29% of cash costs) including total FTE reduction of 34%	<ul style="list-style-type: none"> <li>• Staff numbers reduced by 36% at 31 December.</li> <li>• Exited 10 Rodborough Road facility</li> <li>• Subletting additional factory space at 20 Rodborough Road</li> </ul>	<ul style="list-style-type: none"> <li>• On track for cost reductions of \$11.8m vs March 13</li> <li>• Continued eligibility for R&amp;D tax credit</li> <li>• Ongoing review of efficiencies</li> </ul>
2. Non equity financing	<ul style="list-style-type: none"> <li>• Additional US\$20m financing from NovaQuest available from start of CF303 – 4 quarterly instalments<sup>1</sup></li> </ul>	<ul style="list-style-type: none"> <li>• Successful partnering initiatives will improve financial position</li> </ul>

1. Refer to ASX announcement dated 30 October 2013 for further important detail

# Financial Overview

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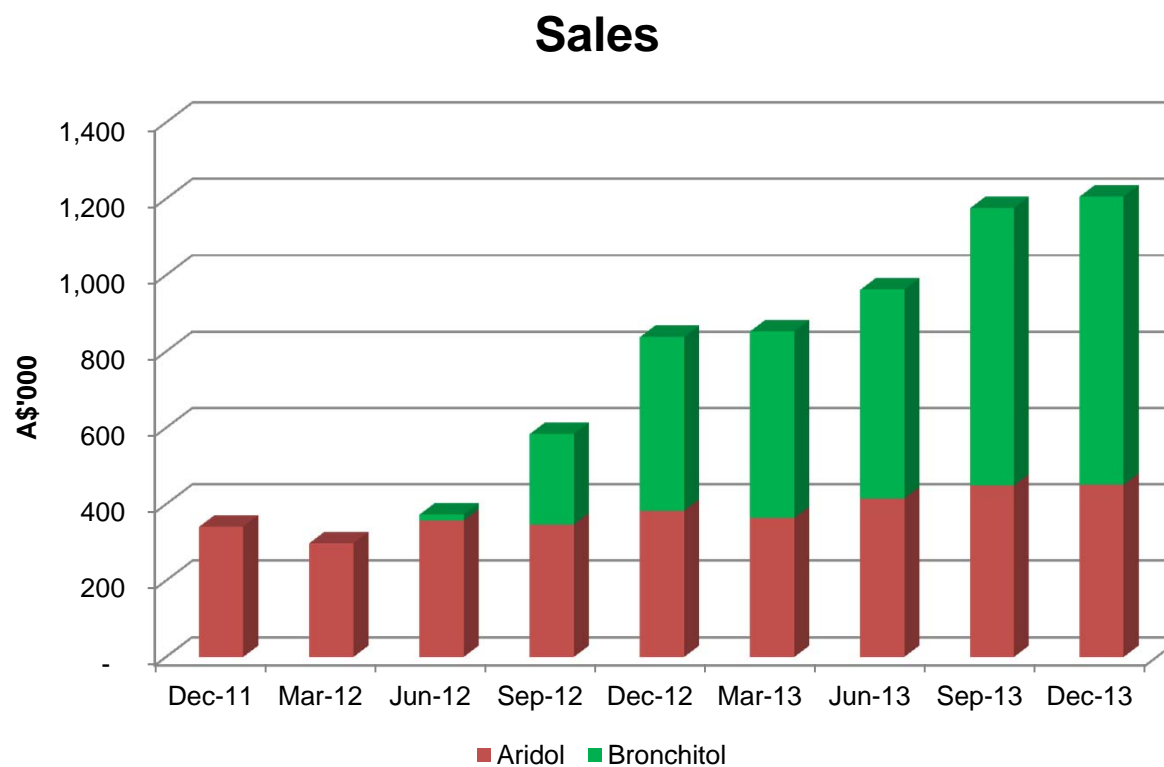


# Financial statements – unaudited

('000 except per share data)

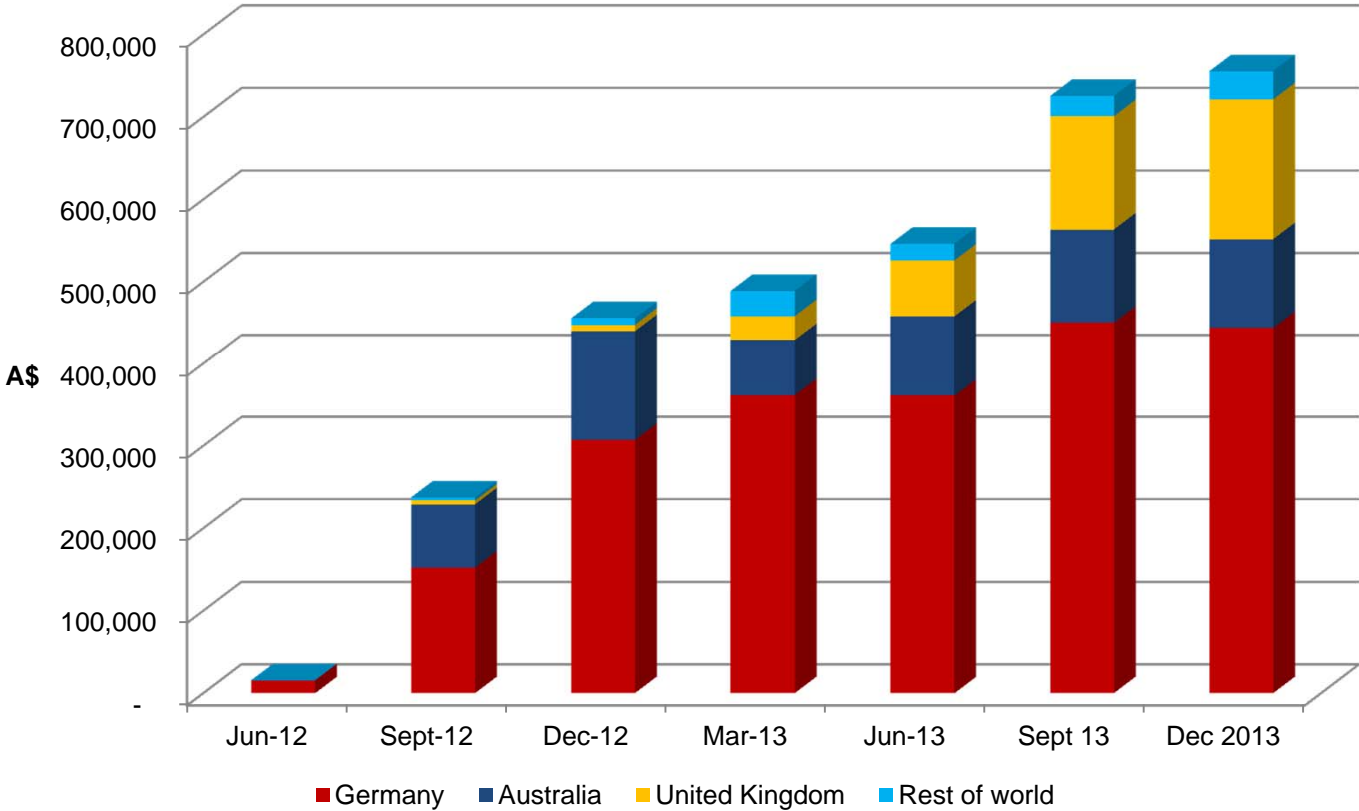
Income statement - unaudited ('000 except per share data)	Three months ended		Year-to-date	
	31-Dec-13	31-Dec-12	31-Dec-13	31-Dec-12
Revenue	A\$	A\$	A\$	A\$
Sales	1,206	838	2,382	1,422
Cost of sales	(470)	(282)	(876)	(509)
Gross profit	736	557	1,506	913
Interest income	466	612	1,010	1,311
Grant and other income	1,150	1,435	1,978	3,212
Expenses				
Sales & marketing	(2,926)	(3,735)	(5,196)	(6,632)
Regulatory, safety & medical affairs	(1,258)	(1,869)	(2,191)	(3,417)
Administration	(1,980)	(1,598)	(4,078)	(2,990)
Available manufacturing capacity	(996)		(2,401)	
Research & development - Bronchitol	(2,941)	(4,673)	(4,342)	(9,969)
Research & development - new drug development	(1,394)	(1,531)	(2,151)	(2,721)
Finance & royalties	(2,388)	(221)	(4,772)	(440)
Restructuring charges	-	-	-	-
Total expenses	(13,882)	(13,628)	(25,131)	(26,169)
Net loss before tax	(11,530)	(11,023)	(20,637)	(20,733)
Income tax expense	(43)	(26)	(61)	(42)
Net loss after tax	(11,573)	(11,050)	(20,698)	(20,775)
Basic and diluted earnings (loss) per share - \$	(0.037)	(0.037)	(0.067)	(0.067)

# Sales revenue by quarter



Sales increase over	Bronchitol	Aridol	Total
Dec-12	66%	18%	44%
Sep-13	4%	0%	3%

# Bronchitol sales by country



Sales increase over	Germany	Australia	UK	Total
Dec-12	45%	-19%	2042%	66%
Sep-13	-1%	-5%	23%	4%

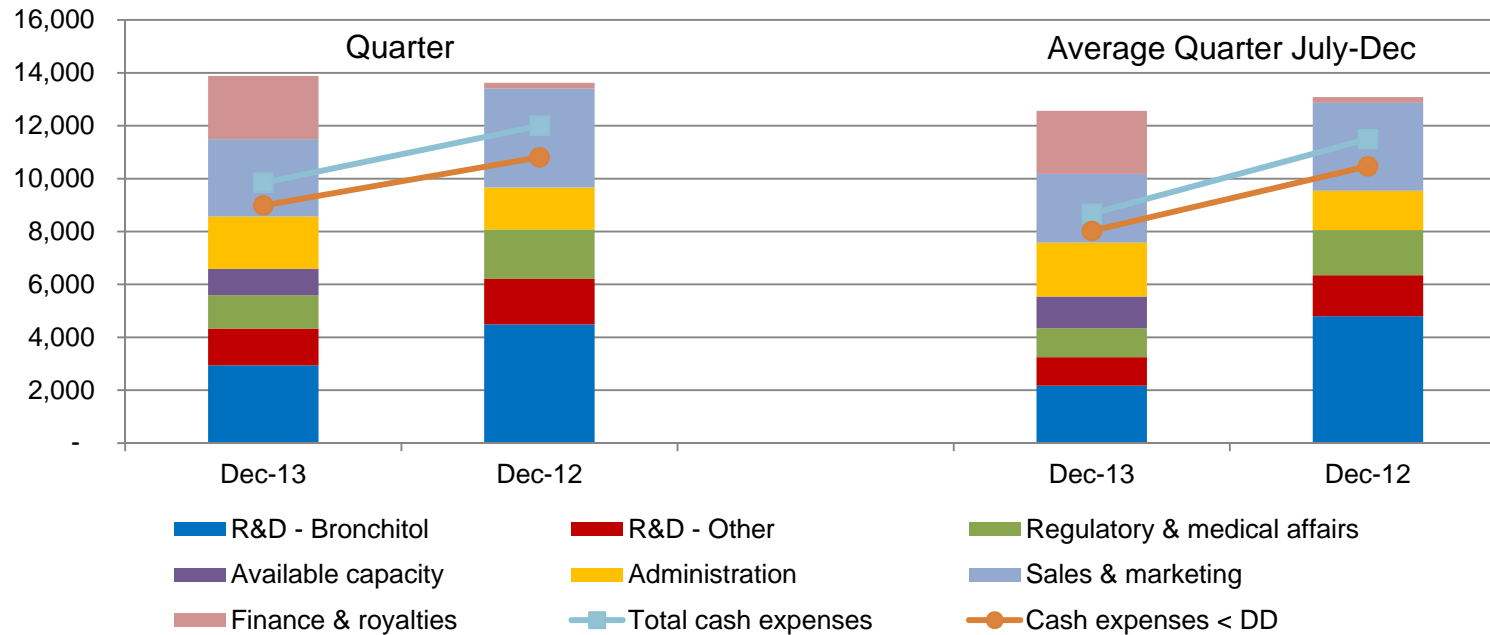
# Cash loss and expenses

(A\$'000)

Normalised cash loss - unaudited ('000 except per share data)	Three months ended		Year-to-date	
	31-Dec-13	31-Dec-12	31-Dec-13	31-Dec-12
Net loss after tax	(11,573)	(11,050)	(20,698)	(20,775)
Non cash expenses				
Depreciation	483	516	984	1,029
Amortisation	856	634	1,488	1,269
Share based compensation	557	477	1,029	876
NovaQuest finance charge <sup>(1)</sup>	2,145	-	4,290	-
	4,041	1,626	7,792	3,174
Restructuring charges	-	-	-	-
Net cash loss before restructuring expenses	(7,532)	(9,424)	(12,907)	(17,601)
Total cash expenses before restructuring expenses	9,841	12,002	17,340	22,996

1. Payments to NovaQuest will be made quarterly from April 2014 based on EU sales of Bronchitol in the previous quarter

# Expenses by quarter (excluding restructuring costs)



Annualised reduction in cash expenses before drug discovery (~\$4m pa) of \$7.3m for quarter, \$9.8m YTD

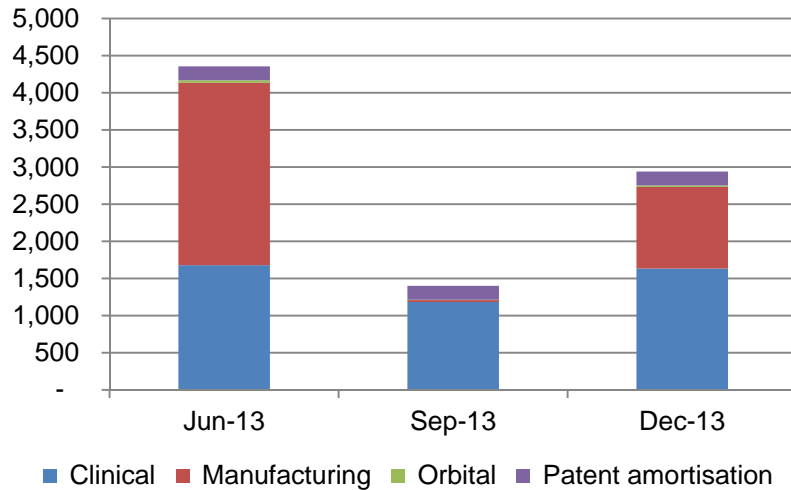
Quarterly highlights:

- R&D – Bronchitol. Clinical trial related costs increased. Also includes costs to manufacture clinical trial material (see below)
- R&D – new drug development. Selective investment in LOXL2 program and increased patent amortisation (see below)
- Regulatory – costs vary by quarter with annual fee payments. 2013 included US NDA costs
- Available manufacturing capacity includes costs classified as R&D until June 13.
- Administration – unchanged but includes costs of ongoing BD initiatives and all employee equity plan costs from FY14
- Sales & marketing – current quarter includes external promotional expenditure including German market initiatives and Australian relaunch
- Administration cost increase attributable to consolidation of certain commercial management functions and all equity compensation cost into administration
- Finance & royalties – includes non-cash accrual of current estimated future payments under the NQ financing agreement booked commencing June quarter 2013



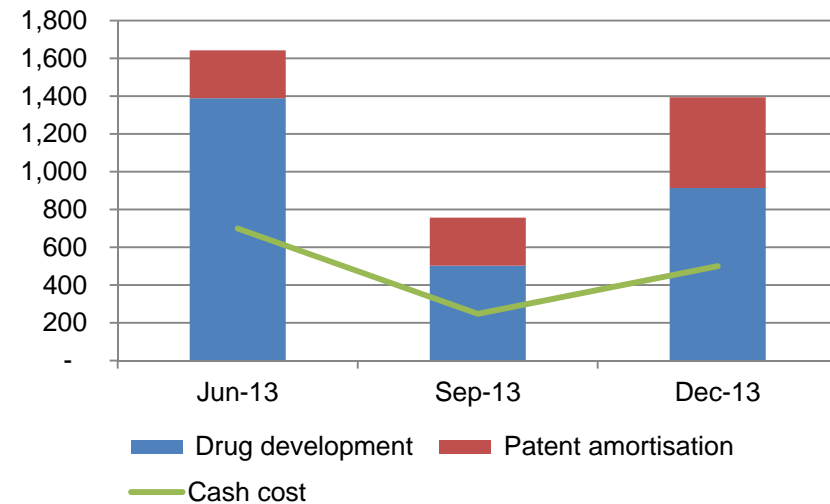
# Research & development expense (A\$'000)

## Bronchitol R&D



- December manufacturing R&D predominantly cost of producing study drug for CF204
- Increase in December quarter clinical expenses reflects expansion of CF204 and initial costs on CF303 offset by staff reductions

## New Drug Development

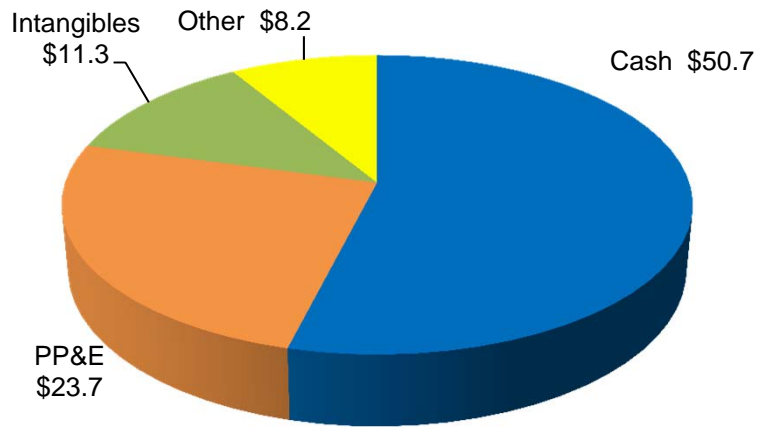


- Net cash cost for December quarter: \$0.5m
- Increased amortisation (non cash)
- Increased external costs advancing LOXL2 program

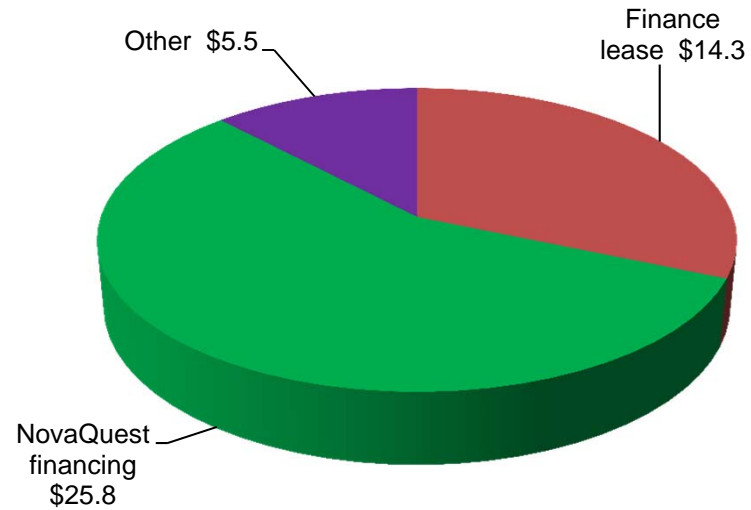
# Balance sheet – 31 December 2013

(A\$mil)

### Assets (\$94m)



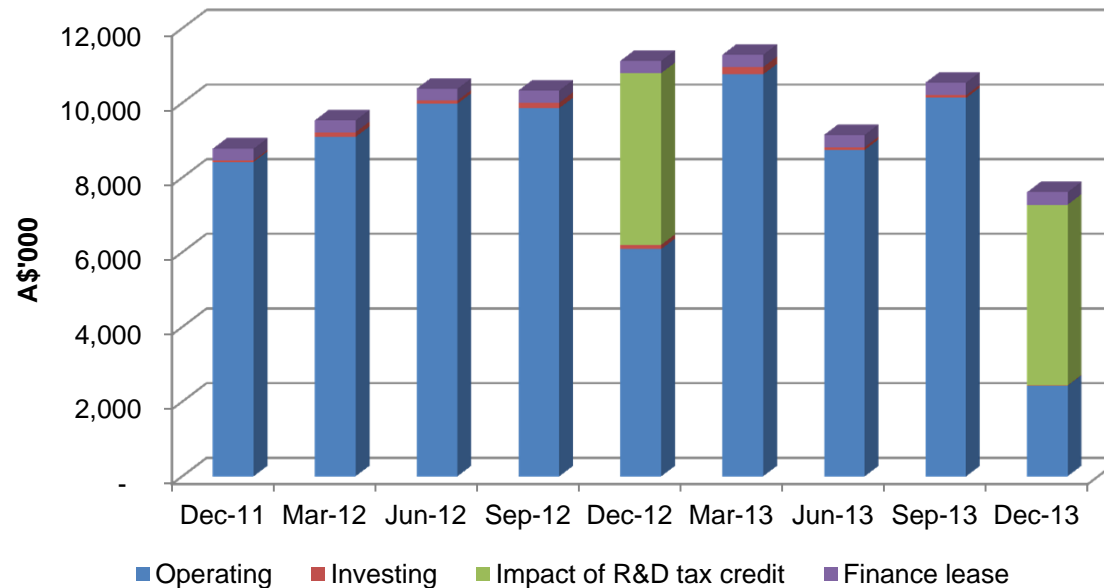
### Liabilities (\$46m)



Additional US\$20m available under NovaQuest agreement on commencement of US CF trial<sup>1</sup>

1. Refer to ASX announcement dated 30 October 2013 for further important detail

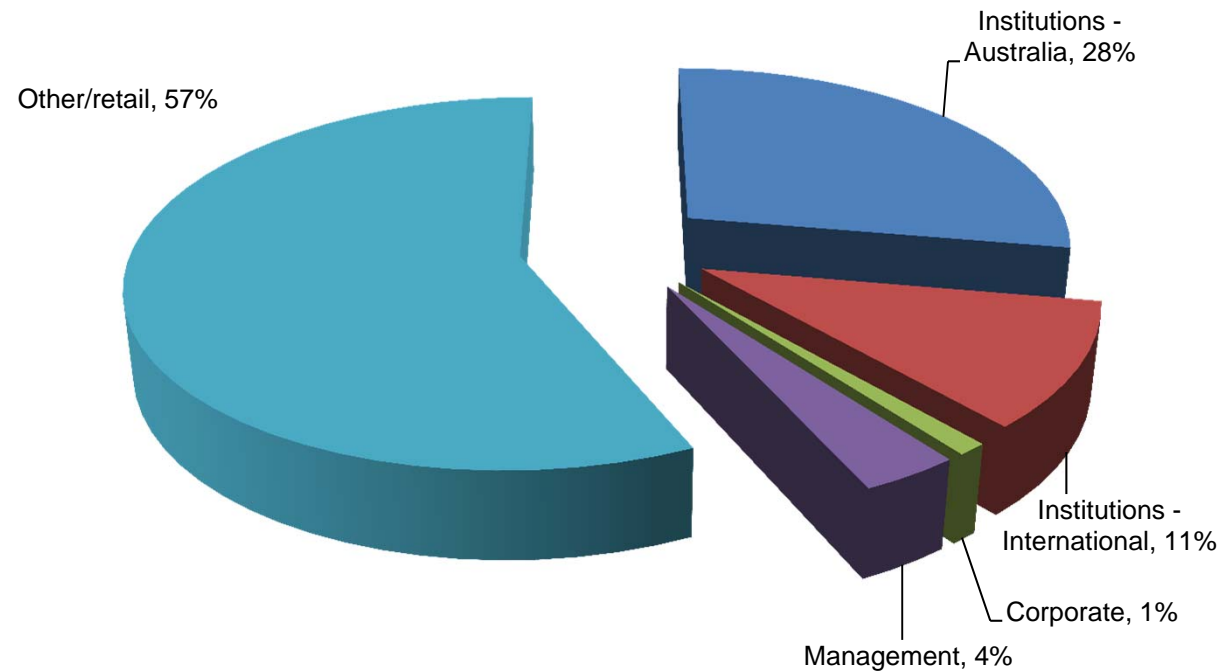
# Operating & investing cash outflow by quarter



- Operating cash outflow for quarter of \$2.4m after receipt of \$4.8m R&D tax credit
- Cash flow includes restructure & redundancy payments of \$360k
- R&D tax credit of \$1.7m for Q3 & Q4 2013 receivable Q3 2014
- Quarterly amounts continue to be influenced by recurring annual receipts and payments (eg R&D tax credit) as well as investment in items such as clinical trials, early stage pipeline, etc

# Share Capital

(including options)



No of shareholders - 18 February 2014	7,355
Shares on issue – 31 December 2013	309 million
Options outstanding – 31 December 2013	20 million

# Objectives for upcoming quarter

## (March 2014)

- Bronchitol sales
  - Continued growth in UK
  - Germany and Australia back on growth
  - Complete preparation for CF303 trial
- Negotiations with selected partner for LOXL2 program
- Negotiations with selected partner for Bronchitol in US
- Advancement of potential R&D collaborations for other pipeline assets SSAO program, Orbital inhalation device, ASM8
- Continue expanding distribution arrangements in non EU countries

**END**

