

pharmaxis

**Therapeutic products  
for respiratory and  
autoimmune diseases**

October 2007

# Forward Looking Statements






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.

Factors that could cause or contribute to such differences include, but are not limited to, factors discussed in the "Risk Factors and Other Uncertainties" section of our Form 20-F filed with the US Securities and Exchange Commission

We are not under any duty to update forward-looking statements unless required by law.

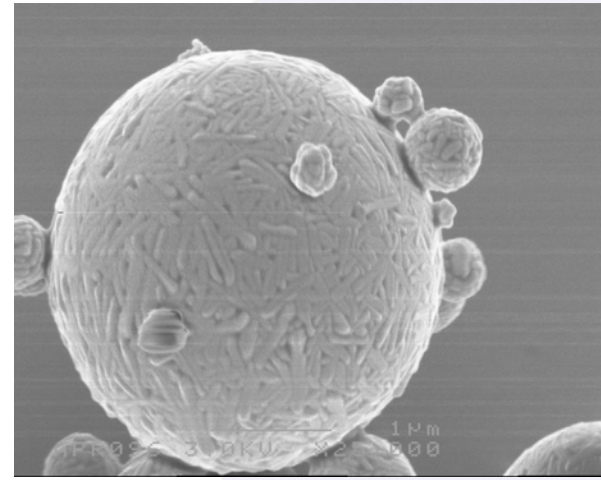
This investor presentation is not an offer of the sale of securities.

# Summary.....

Objective	The development of products for respiratory and autoimmune diseases
Lead products	Aridol: management of asthma and COPD Bronchitol: therapeutic for cystic fibrosis and COPD
Discovery	PXS64 - multiple sclerosis
Listings	ASX (Nov 2003): PXS; NASDAQ (Aug 2005): PXSL
Location	Sydney, NSW, Australia
Facility	GMP Manufacture of lead products
Employees	70
Cash (30/6/07)	A\$76 million
Shares outstanding	178m (11.8m ADS)
Options outstanding	11.4m
Key patents	Aridol & Bronchitol granted in USA, Australia, Asia; pending in EU, Canada and Japan
Analyst coverage	    



# Bronchitol

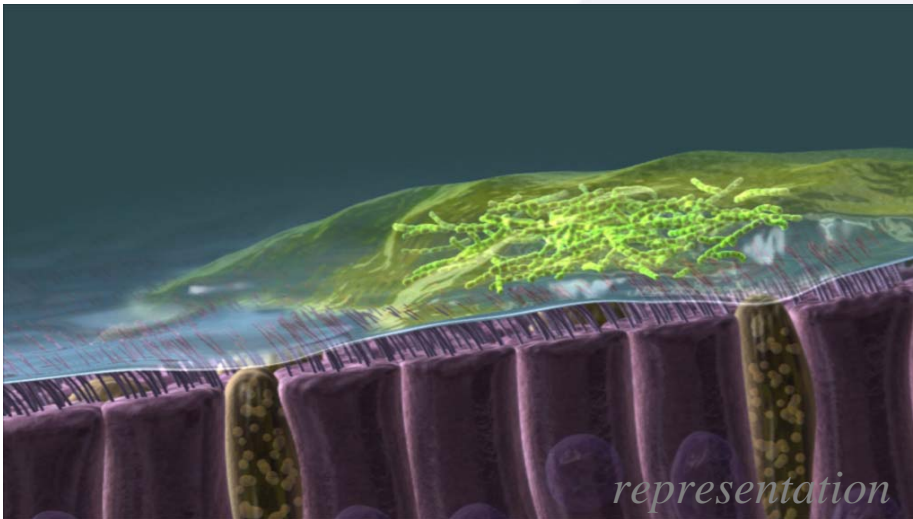


***Mucus clearance:***

***Cystic fibrosis  
Chronic Obstructive Pulmonary Disease  
Bronchiectasis***

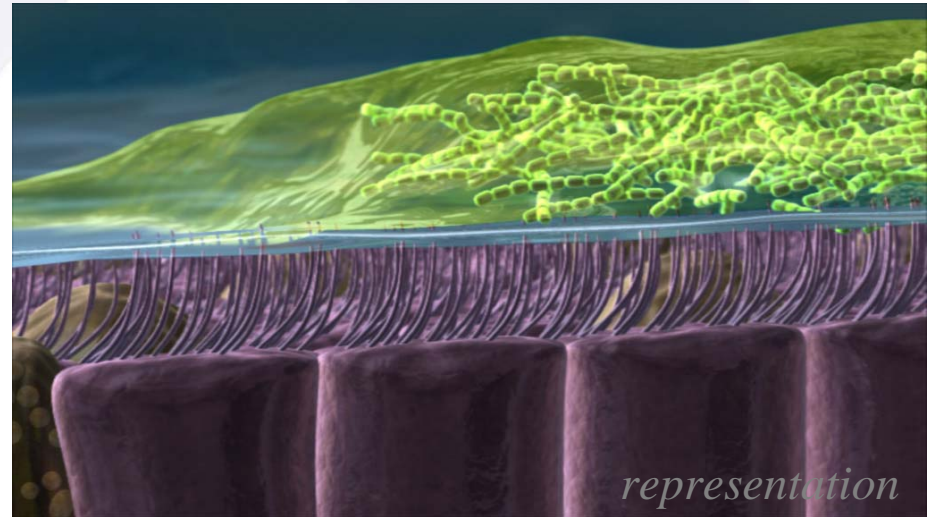
# Osmotic clearance of abnormal mucus.....

Before treatment



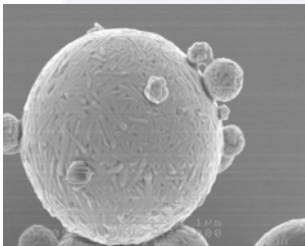
Lung surface dehydrated  
Airway surface fluid layer impaired  
Lung defense and hygiene compromised

After Bronchitol administration



Lung hydrated  
Airway surface liquid restored  
Normal lung clearance

# Bronchitol - bronchiectasis



- Abnormal, irreversible dilation of the lower airways
- Daily mucus production, constant coughing, breathlessness, recurrent acute bronchitis with infective exacerbations : low quality of life
- In 30-50% of cases, the cause is unknown
- Normal lung clearance impaired
- Current treatments: bronchodilators, antibiotics
- No drugs proven effective to clear mucus

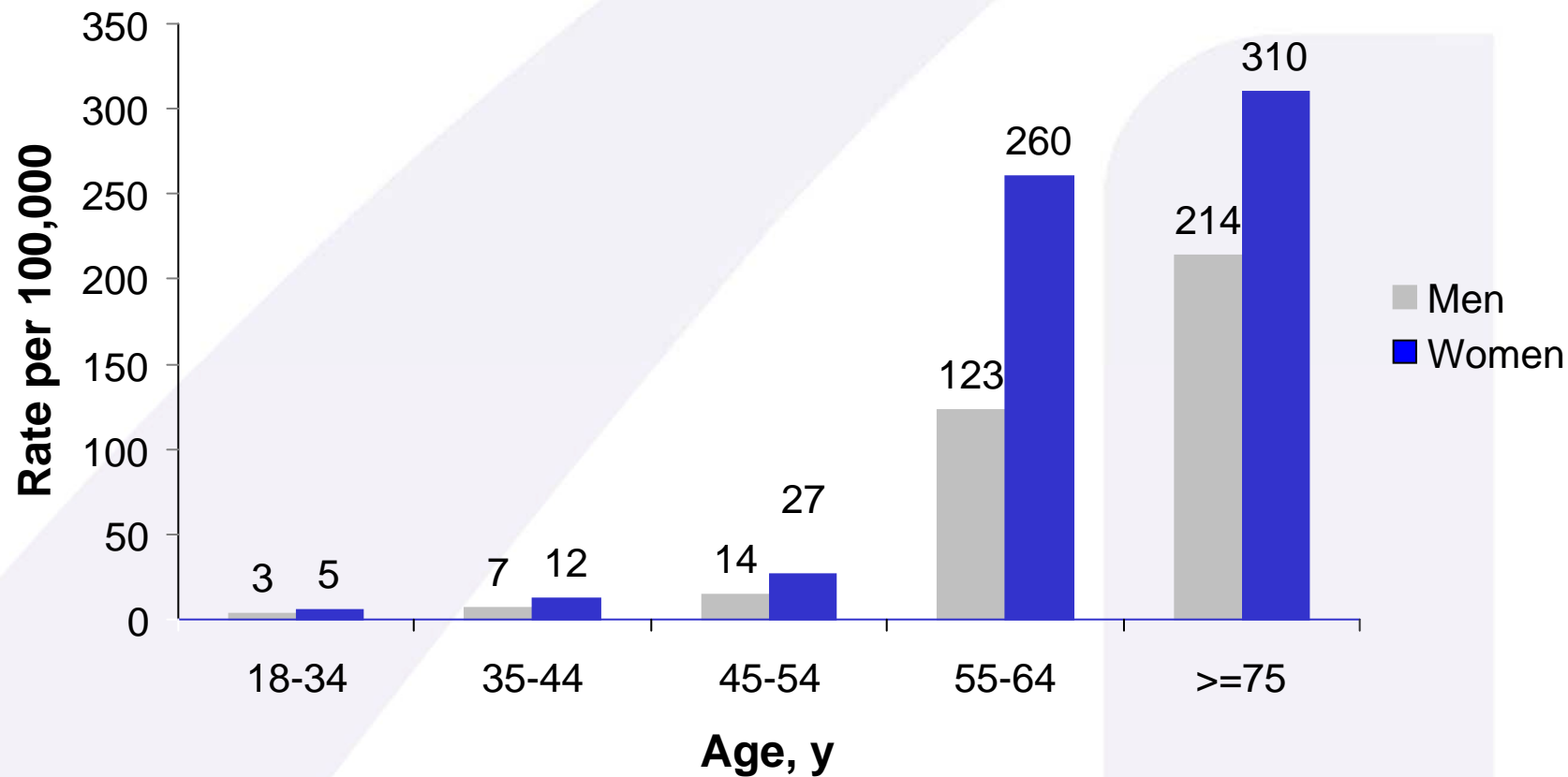
# Number of bronchiectasis sufferers seeking treatment

	EU	Australia	USA	Asia	Total
<b>% of pt pool seen by respiratory specialists</b>	Average 14%	9%	N/A	Average 5%	
<b>Trend</b>	Stable or increasing	Stable	Increasing	Stable or decreasing	
<b>Mod/Severe</b>	55%	70%	55%	75%	
<b>Patients seeking treatment</b>	210,000	18,000	110,000	250,000 ++	600,000+

Prevalence: Much higher. Bronchiectasis is often missed but has been measured as >10% of COPD patients in a US patient cohort ~ 800k

Note: US Data comes from Datamonitor research, other data from Frost & Sullivan research

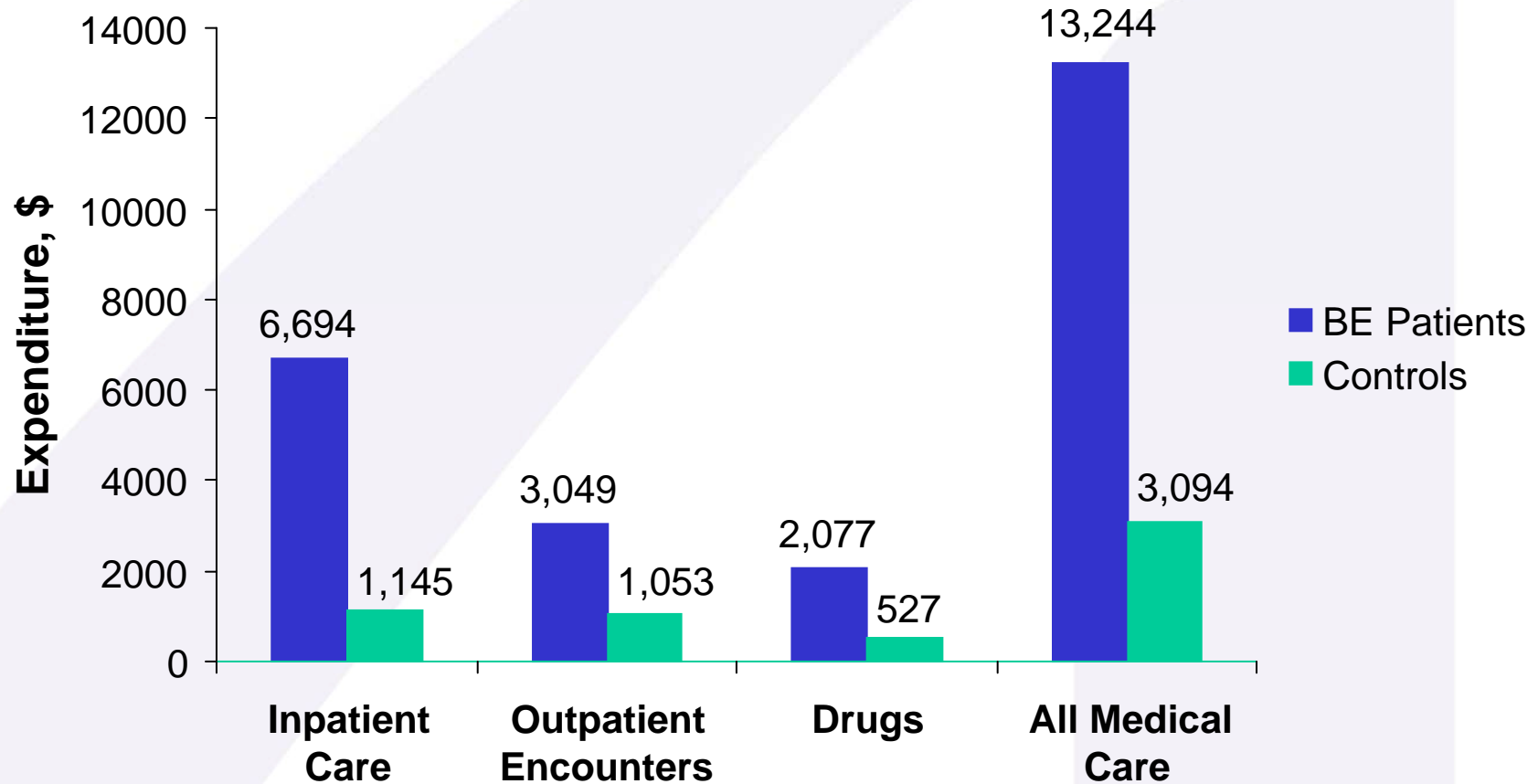
# US bronchiectasis prevalence



Estimated US prevalence: 52 cases per 100,000

Weycker D, et al. *AJRCCM* 2004;169: A330.

# Expenditure on bronchiectasis per patient per year



Source: Weycker et al. *AJRCCM* 2004;169: A330.

# Bronchitol - bronchiectasis



## •Phase II clinical trial

- 60 patient, double-blind, crossover, placebo-controlled
- 400mg twice a day for 14 days
- Primary end point – quality of life



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- Improvement in quality of life ( $p < 0.05$ ) – Patient Reported Outcome
  - Improvement in sleep ( $p < 0.02$ ) – Patient Reported Outcome
  - Improvement in chest congestion ( $p < 0.05$ ) – Physician Reported
  - Improvement in small airway function ( $p < 0.05$ ) - Oscillometry



# Bronchitol - bronchiectasis



- Phase III trial (Europe, Australia, NZ)
  - 363 patient, placebo controlled, double blind, randomised 12 week treatment. 12 month Open Label Extension
  - 320mg twice a day



- Primary endpoints
  - quality of life – validated Patient Reported Outcome
  - mucus clearance – 24hr sputum volume



- Primary Analysis
  - Quality of Life SGRQ,  $p < 0.001$  versus baseline  
SGRQ,  $p < 0.05$  versus placebo
  - Mucus Clearance  $\uparrow 30\%$ ,  $p < 0.001$  versus placebo
  - Adverse Events not significant versus placebo

## Secondary endpoints (1)

<b>Endpoint</b>	<b>Comment</b>
Bronchiectasis symptom score	Highly significant SGRQ impact score
Leicester Cough Questionnaire score	Mixed profile
Exercise tolerance (shuttle test score)	No change
Spirometry: FEV <sub>1</sub> , FVC, FEV <sub>1</sub> /FVC, FEF <sub>25-75</sub> (mean % change baseline)	No change
Change from baseline in other lung function parameters – Diffusing Capacity of the Lung for Carbon Monoxide; Forced Oscillation Technique; Multi Breath Nitrogen Washout	Under analysis

## Secondary endpoints (2)

<b>Endpoint</b>	<b>Comment</b>
Lab safety tests – Full Blood Count, Urea, Electrolytes, Liver Function Test, C-Reactive Protein	Under analysis
No of pulmonary exacerbations	No change
Antibiotic use – no of agents, courses, days of use	Under analysis
Adverse Events	No difference
Change in High Resolution CT scans pre & post Rx	Under analysis
Quantitative & qualitative sputum microbiology	Under analysis



# Bronchitol – cystic fibrosis

## •Background

Postural drainage is a technique for loosening mucus in the airway so that it may be coughed out



## •Current treatments: rhDNase and tobramycin

- Genetic disorder affecting 75,000 worldwide (30,000 in U.S.)
- Poorly hydrated, tenacious, thick mucus
- Current life expectancy is 31 years
- Delivered by nebulizer (preparation, sterilization)
- rhDNase (pulmozyme): US\$265mm @ ~30% penetration
- Tobramycin: US\$233mm

# Phase II cystic fibrosis trial.....



- Crossover, 8 site study in 39 CF patients
- Randomised two week treatment periods
- Double-blind, placebo controlled
- Primary Endpoint:
  - Change in FEV<sub>1</sub>
- Secondary Endpoints:
  - Effect on other lung function measures
  - Effect on symptoms/signs
  - Effect on Quality of Life
  - Safety (including microbiology)

## CF Phase II trial results – lung function.....

	Bronchitol	Control	p value
Change in FEV <sub>1</sub>	7 ± 2%	0 ± 2%	0.008
Change in FEF <sub>25-75</sub>	15.5 ± 5%	0.6 ± 5%	< 0.01

Includes patients being treated with rhDNAse

FEF<sub>25-75</sub> is a measure of small airway function

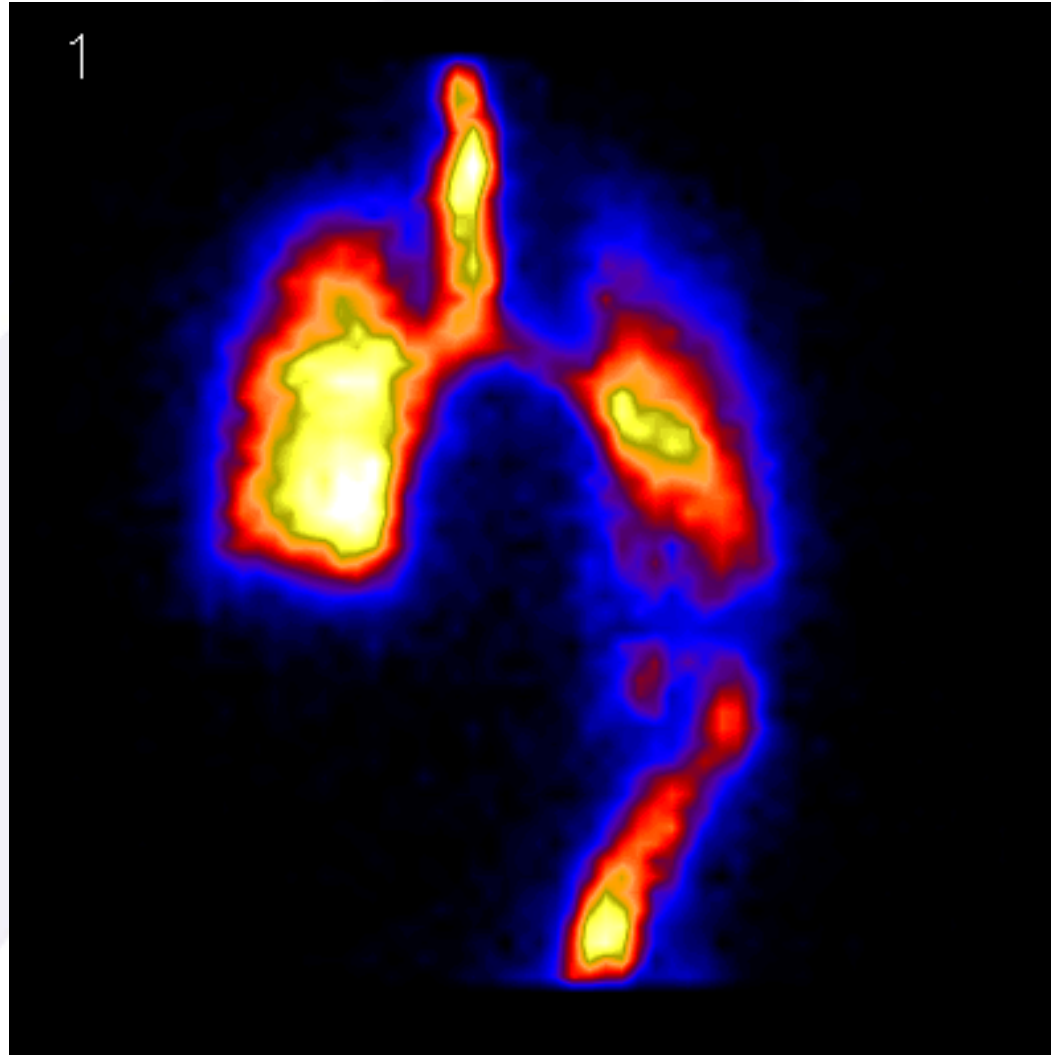
# Bronchitol – cystic fibrosis registration.....

- Phase III trial (EU, AUS, NZ):
  - Now enrolling - 80 out of 250 subjects enrolled
  - Primary endpoint: - lung function (FEV1)
  - Placebo-controlled, 6 month dosing, 400mg bd
  - Scheduled completion 2H 2008
- Phase III trial (USA) to commence 1Q 2008
  - Similar size, design to EU trial
  - Scheduled completion 1H 2009
- Orphan drug designation – EU and USA
- Fast track designation – USA



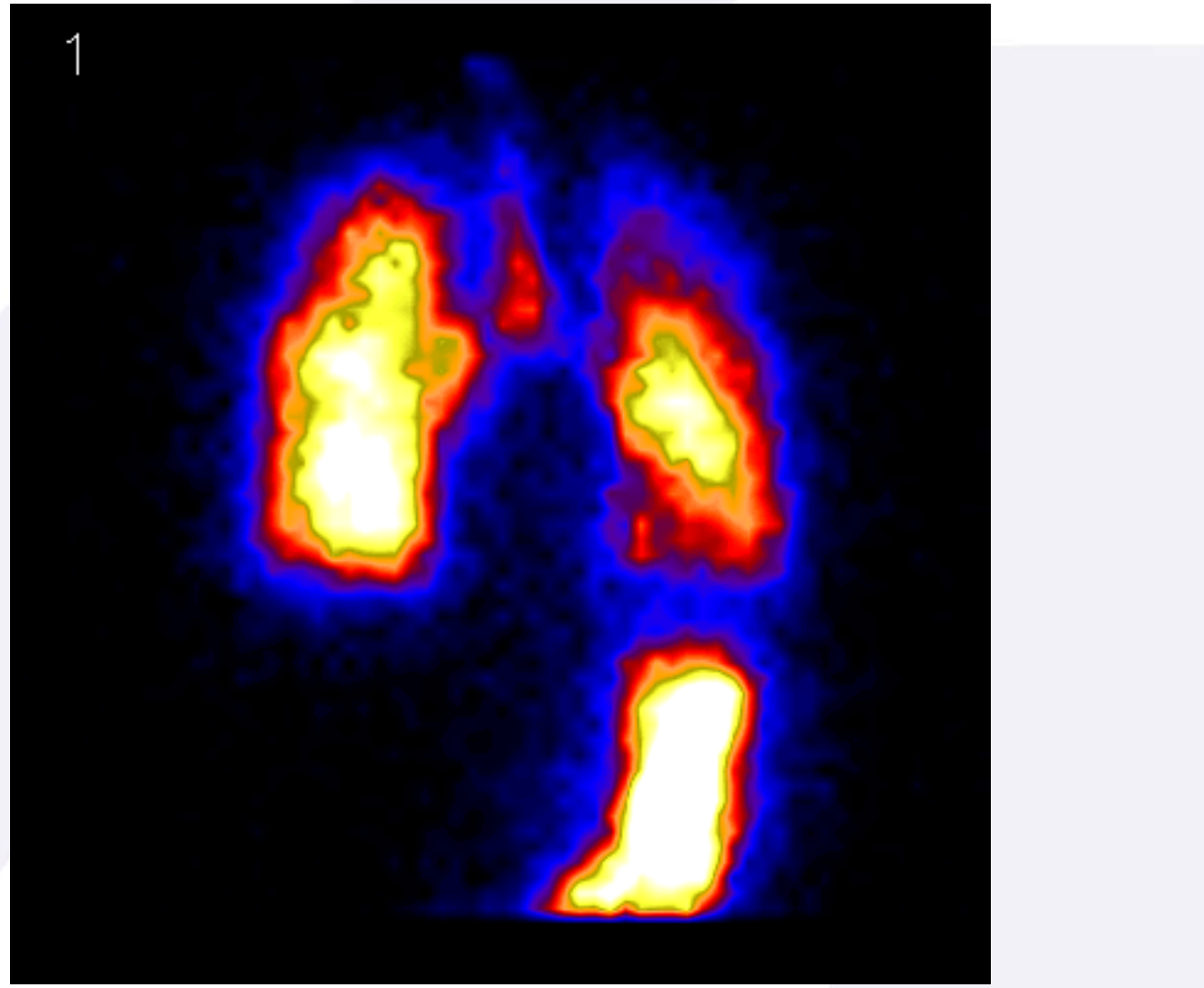
# Bronchitol in the clinic.....

Chronic bronchitis – without Bronchitol



# Bronchitol in the clinic.....

Chronic bronchitis – with 400 mg Bronchitol



# Bronchitol – clearance of lung secretions



- Proof of concept demonstrated with ICU patients
  - Currently supplied on individual compassionate use basis
- Clinical conditions include:
  - asthma, COPD, cystic fibrosis, secondary respiratory disease, neurogenic disorder
  - 30 million COPD exacerbations per year in the U.S.<sup>1</sup>
  - 1 million U.S. emergency room visits per year
- Complete acute care pilot trial (COPD) 1H 2008

# Aridol™



***A rapid and simple test for airways inflammation that facilitates diagnosis and management of asthma.***



# Clinical applications for Aridol

An easy to use, 'point of care' test with a high degree of sensitivity (85%) and specificity (95%) for airway inflammation

- 1. Asthma diagnosis and assessment of disease severity<sup>1</sup>**
- 2. Monitor patient's disease / managing effectiveness of treatment<sup>2</sup>**
- 3. Identification of COPD patients who will respond to steroids<sup>3</sup>**

*NOTES: 1 = Evidence available from phase III study  
2 = Proof of concept only; definitive studies ongoing  
3 = Evidence available from phase II study*



# International Regulatory Status



- **Australia**

- Launched

June 2006

- **Europe**

- Approved for marketing (Sweden)

October 2006

- Launched

January 2007

- Approved European Union (MRP)

May 2007

- Launch

September 2007



- Submitted – Switzerland

- Regional marketing partners appointed

- **South East Asia**

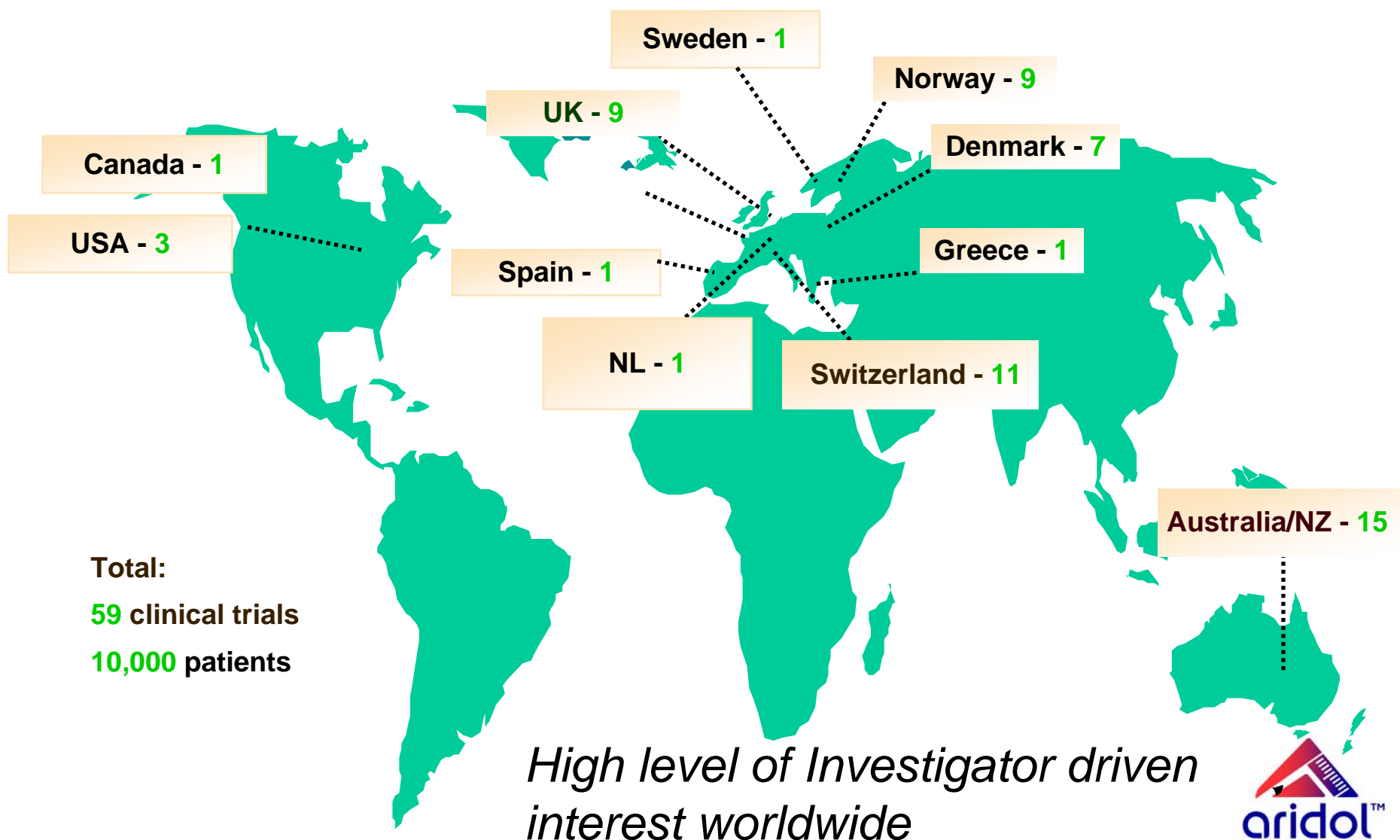
- Submitted - Korea

- **USA**

- Phase III completed. NDA to be filed 1Q08



# Worldwide development of Aridol.....



# Near term catalysts ahead.....

Milestone	4Q-07	1Q-08	2Q-08	3Q-08
<b>Bronchitol - bronchiectasis</b> Finalise US protocol with FDA Commence Phase III (US) File 1 <sup>st</sup> marketing appln (Aus)				
<b>Bronchitol – cystic fibrosis</b> PII dosing trial data (Can/Arg) Close EU P III trial recruitment Commence US Phase III trial				
<b>Aridol</b> File NDA (US)				
<b>Facilities</b> New facility construction begins				
<b>PXS25/64</b> Complete preclinical studies				

# Financial Statements – Australian GAAP

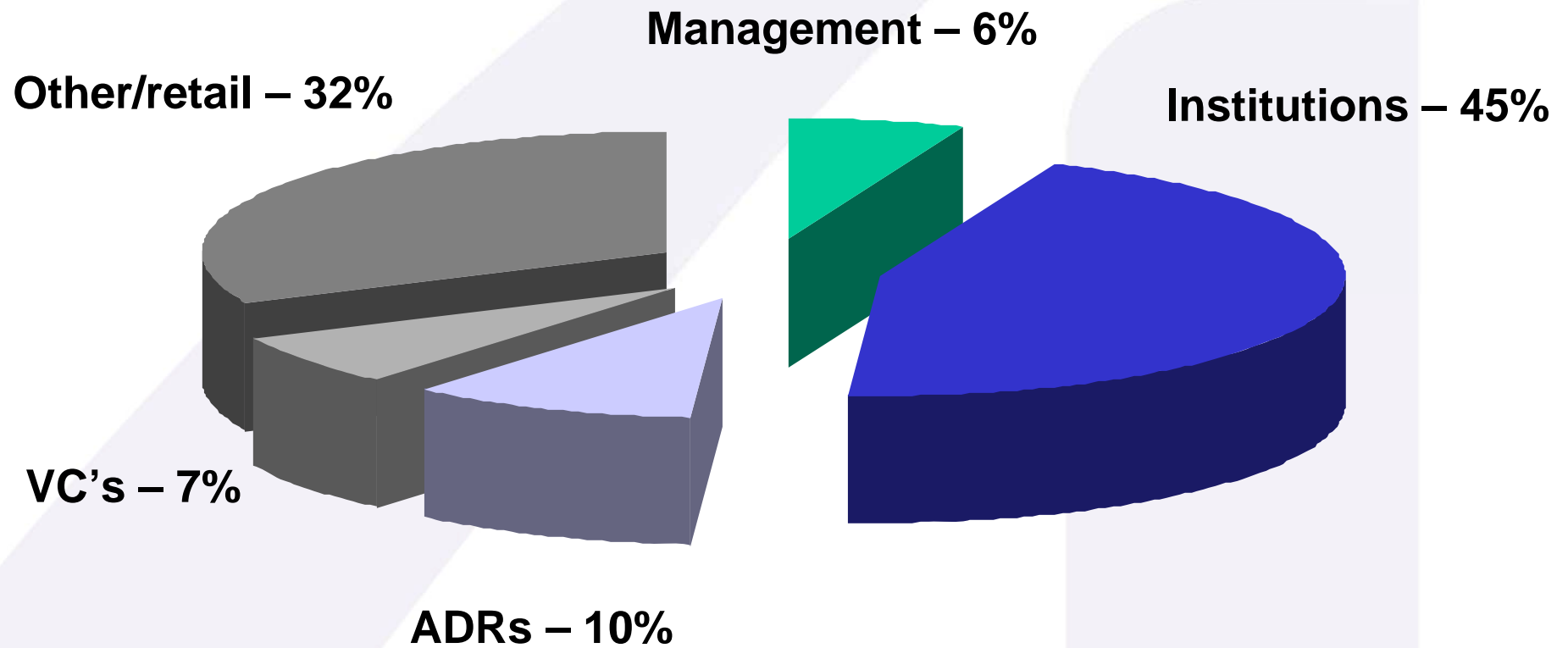
	<u>Year ended 30 June</u>		
	<u>2005</u>	<u>2006</u>	<u>2007</u>
	<u>A\$'000</u>	<u>A\$'000</u>	<u>A\$'000</u>
<b>Income Statements</b>			
Revenue from sale of goods	-	8	205
Cost of sales	-	(2)	(49)
Gross profit	-	6	156
Other income			
Interest	1,702	4,282	5,278
Grant income	1,219	1,299	2,152
Other	-	-	-
Expenses			
Research & development	(9,269)	(16,978)	(23,840)
Commercial	(3,134)	(4,386)	(3,240)
Administration	(963)	(1,951)	(4,619)
Foreign exchange (gains)losses	-	-	(47)
Total expenses	(13,366)	(23,315)	(31,746)
Net loss before tax	(10,445)	(17,728)	(24,160)
Income tax expense	-	(5)	(19)
Net loss after tax	(10,445)	(17,733)	(24,179)
Basic and diluted earnings (loss) per share - \$	(0.084)	(0.111)	(0.136)
Depreciation & amortisation	646	947	939
Fair value of employe options issued	260	1,488	1,488

# Financial Statements – Australian GAAP

	<u>30-Jun-05</u>	<u>As at</u> <u>30-Jun-06</u>	<u>30-Jun-07</u>
<b>Balance Sheets</b>	<u>A\$'000</u>	<u>A\$'000</u>	<u>A\$'000</u>
Cash and cash equivalents	33,390	97,840	76,182
Plant & equipment	2,477	3,205	3,521
Intangible assets	37,937	1,195	1,239
Total assets	2,470	104,267	82,648
Total liabilities	35,467	-5,379	(6,089)
Total shareholders' equity	35,467	98,888	76,559
<b>Share Data</b>	<u>'000</u>	<u>'000</u>	<u>'000</u>
Ordinary shares on issue	134,770	176,904	177,949
Options on issue	10,914	9,692	9,836

# Share Capital

(including options)



30 Sep 2007: 178m shares; 11.4m options