

pharmaxis

# Therapeutic products for respiratory and autoimmune diseases

March 2006

# 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.

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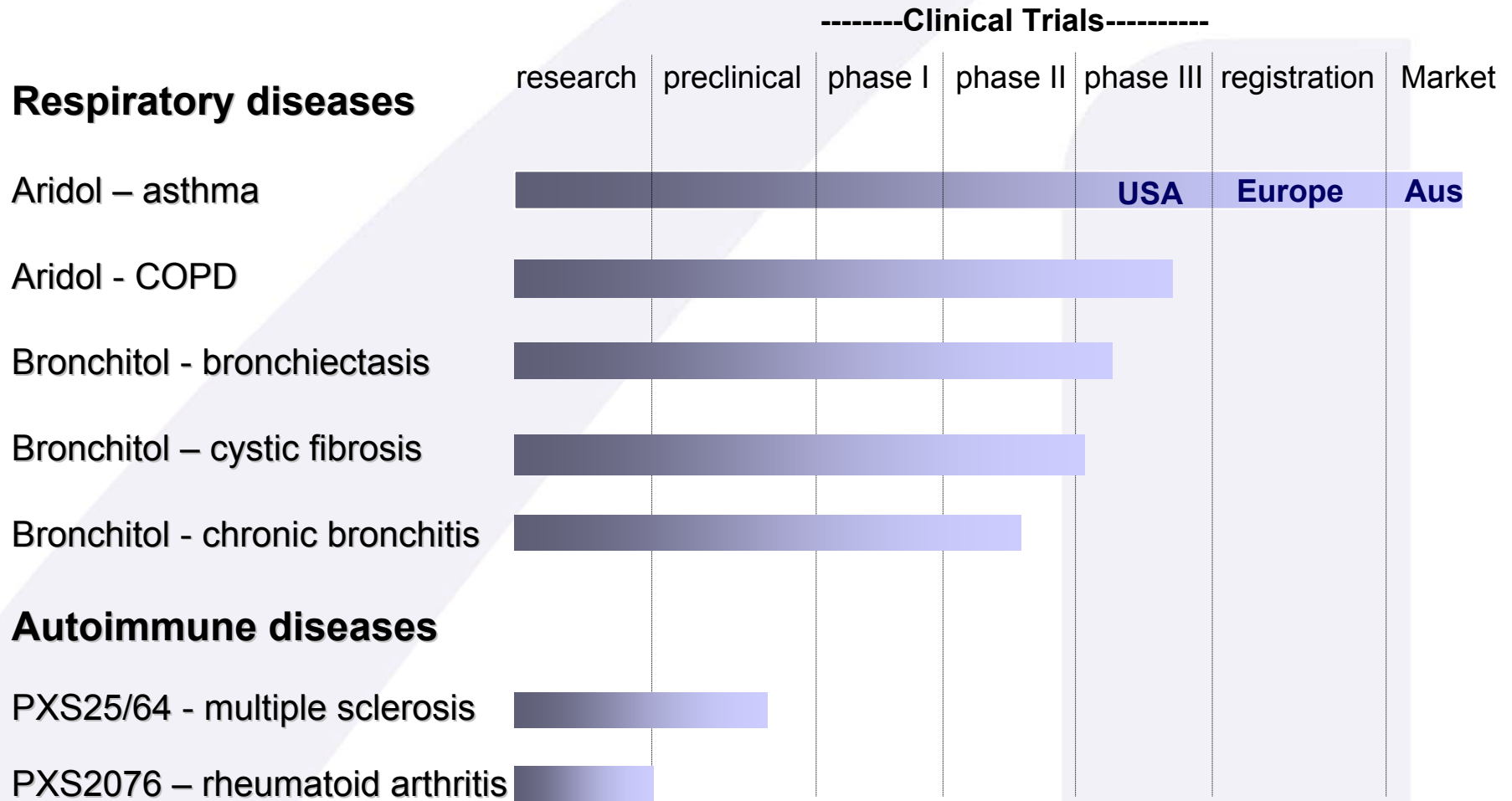
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# Summary

Mission	To research, develop and bring to market innovative therapeutic products for the treatment and management of 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 (28/2/06)	50
Cash (31/12/06)	A\$106.4m
Shares outstanding Options outstanding	174.4m (11.6m ADS) 11.7m
Key patents	Aridol & Bronchitol granted in USA, Australia, Asia; pending in EU and Canada
Analyst coverage	  

# Pipeline

## Pulmonary and Autoimmune Focus



# Recent Highlights....

## •Aridol



- Completed Phase III - asthma management (Aus and EU)
- Positive ADEC opinion received on market application (Aus)
- Market application lodged in Europe (April 2005)
- US Phase III study in progress - completion 2006
- Two marketing/distribution partners appointed in Europe
- Australian launch at TSANZ – 28 March?

## •Bronchitol



- Entering Phase III studies in Europe and USA
  - cystic fibrosis and bronchiectasis
- Orphan Drug designation for CF, bronchiectasis (U.S.)
- Orphan Drug designation for CF (Europe)

# Management



**Alan Robertson PhD** **CEO**  
*Wellcome (GSK); Faulding;  
Amrad; Inventor of Zomig*



**David McGarvey CA** **CFO**  
*CFO, Memtec (NYSE);  
CFO, US Filter Filtration Group*



**Brett Charlton, PhD, MBBS** **CMO**  
*Stanford; ANU*



**Gary Phillips, MBA** **CCO**  
*CEO, Novartis Australia*



**John Crapper, MBA** **COO**  
*Managing Director, Memcor;  
Syntex (Roche)*



**William Cowden, PhD** **CSO**  
*ANU; Co-inventor of TNF mAb's*



**Ian McDonald, PhD** **CTO**  
*VP Discovery, SIBIA (Merck);  
VP Discovery, SGX*

# Aridol™



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

# Aridol

## Asthma and COPD Opportunity

### •Asthma

- 51mm patients in 7 major markets
- No *simple* test
- ~34% of people diagnosed with asthma do not have the disease
- Ongoing patient management difficult

### •Chronic Obstructive Pulmonary Disease

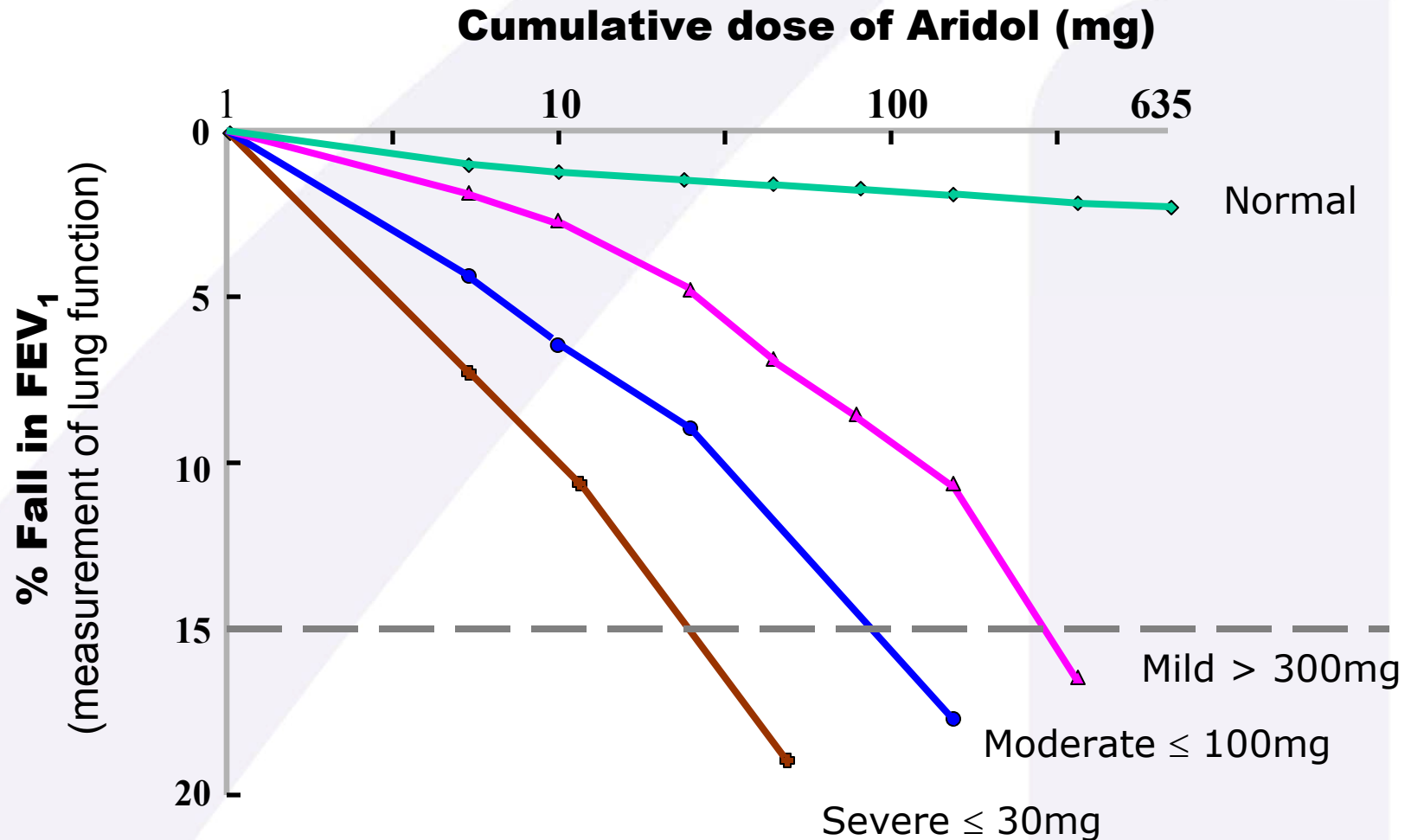
- 30 million people affected in 7 major pharmaceutical markets
- Cost to US healthcare - US\$30 billion pa
- 20-25% respond to inhaled steroids but no test to identify them





# Aridol

## Measurement of airway hyper-responsiveness



# Aridol

## Current regulatory status



- **Phase III results (646 patient study)**
  - Effective at identifying clinical mis-diagnosis (7%)
  - 20% of subjects over treated and over diagnosed
  - 25% of subjects not well controlled
- **European marketing authorization submitted**
  - Anticipated approval – 1H 2006
- **Australian marketing authorization submitted**
  - Positive ADEC opinion received
  - Anticipated approval – 1Q 2006
- **US Phase III trial commenced**
  - Scheduled completion mid – 2006
  - Subjects enrolled at March 15: 103/280

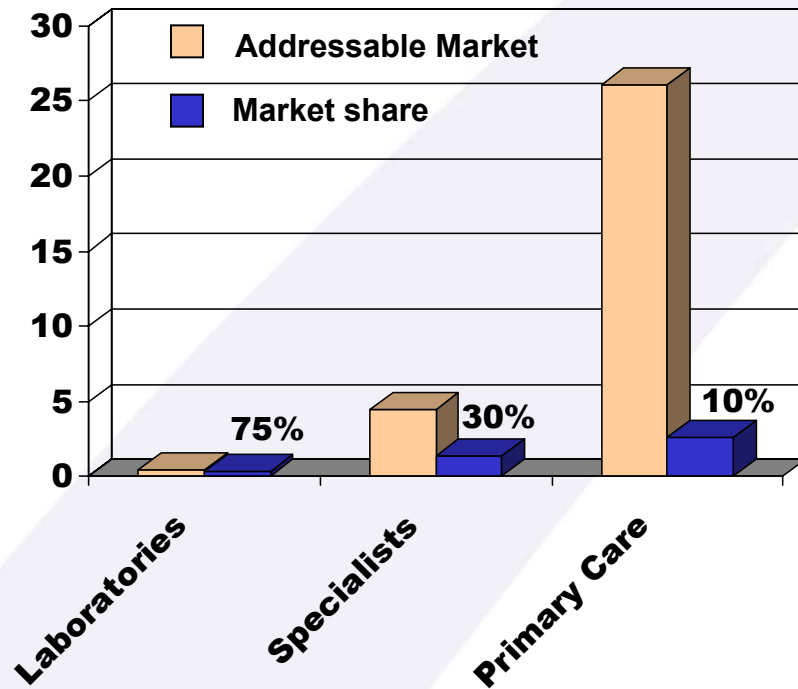
# Aridol

## Marketing plans - Australia

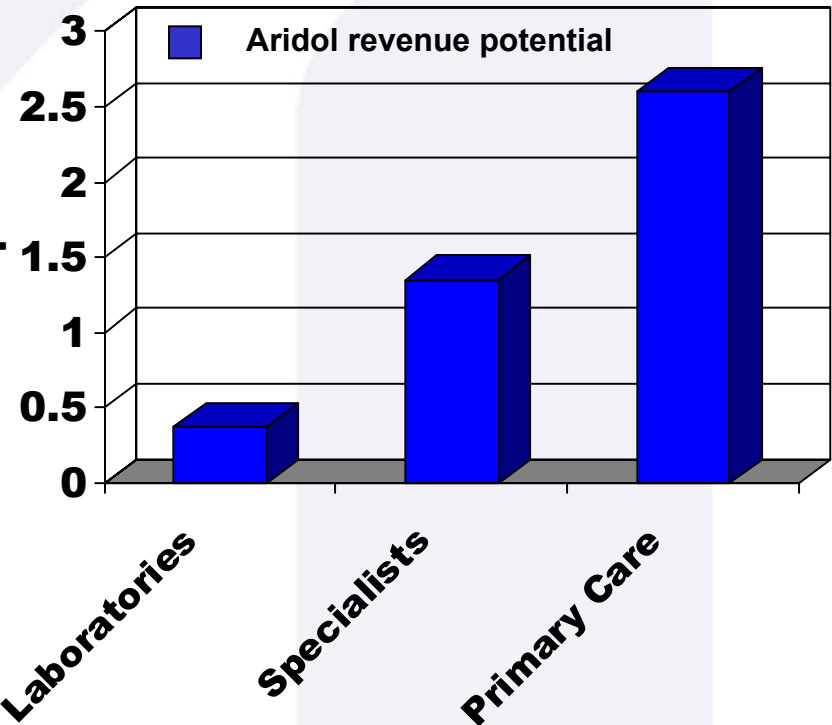
- **Build Marketing and Sales Force:**
  - Recruit and train sales team
  - Market research completed
- **Prepare Promotional Materials**
  - Aridol a global brand with consistent promotional claims
- **Market Introduction**
  - Respiratory laboratories
  - Respiratory specialists - Aridol to be included in Hospital Formulary
  - Primary Care Physicians
- **Reimbursement is available under existing treatment code**

# Aridol opportunity by market segment

Patient Numbers in Millions



No of tests per annum



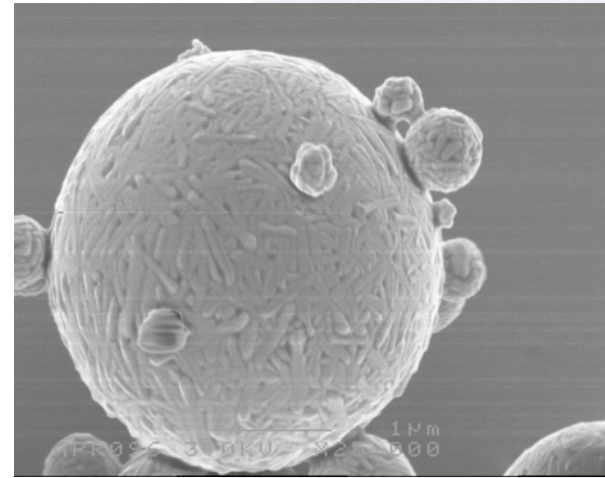
- Replace old tests
- Increase referrals
- New indications
- Create 'practice' test market

**Total Opportunity = \$210m+**

# Bronchitol



***Mucus clearance:***



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

# Bronchitol

## cystic fibrosis

### •Background

- Genetic disorder affecting 75,000 worldwide (30,000 in U.S.)
- Poorly hydrated, tenacious, thick mucus
- Current life expectancy is 31 years

### •Current treatments: rhDNase and tobramycin

- Delivered by nebulizer (preparation, sterilization)
- rhDNase (pulmozyme): US\$265mm @ ~30% penetration
- Tobramycin: US\$233mm

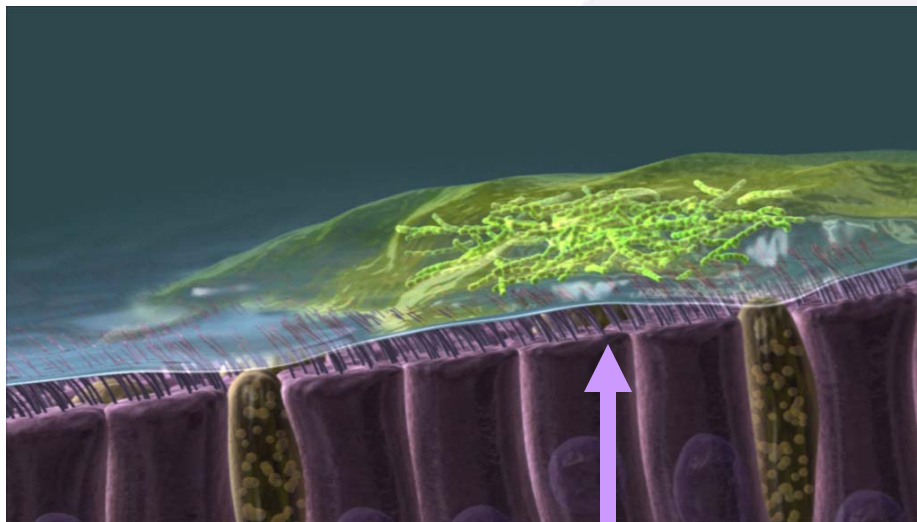
Postural drainage is a technique for loosening mucus in the airway so that it may be coughed out



# How Bronchitol works.....

## Osmotic clearance of abnormal mucus

Before treatment

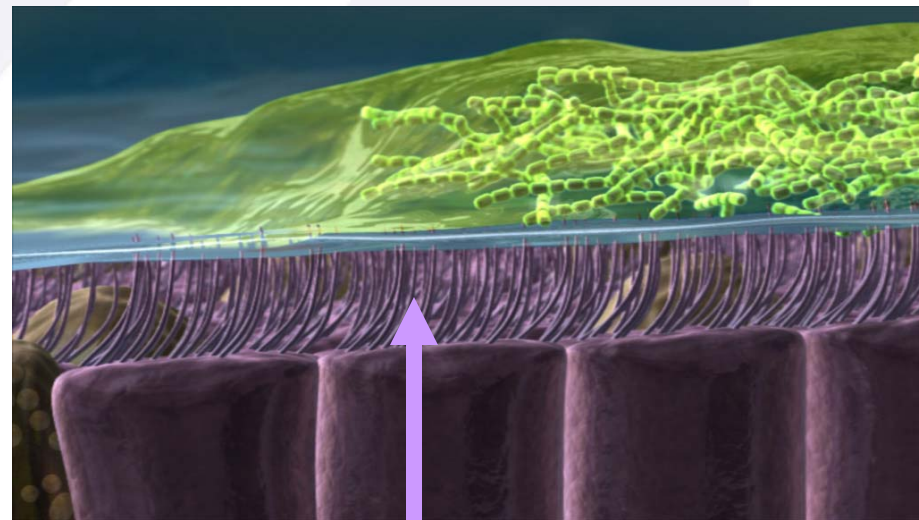


Lung surface dehydrated

Airway surface fluid layer impaired

Lung defense and hygiene compromised

After Bronchitol administration



Lung rehydrated

Airway surface liquid restored

Normal lung clearance



# Bronchitol

## 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  $FEV_1$
- Secondary Endpoints:
  - Effect on other lung function measures
  - Effect on symptoms/signs
  - Effect on QoL
  - Safety (including microbiology)



# Bronchitol

## CF Phase II Results: Change in 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 pulmozyme

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

# Bronchitol

## cystic fibrosis registration strategy



- Phase III trial (EU & Aus):

- Commence mid-2006
- Primary endpoint: Same as Phase II ( $FEV_1$ )
- Placebo-controlled, 6 month dosing, finalising design with EMEA

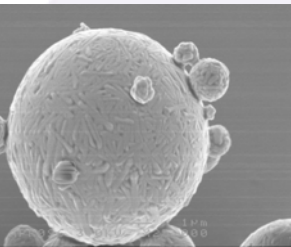
- Phase III trial (US) to commence 2006

- Similar size, design to EU/Aus trial
- End of Phase II meeting held with FDA
- Commence 2006

- Orphan drug designation – EU and USA

# Bronchitol

## bronchiectasis



### •Background

- Abnormal, irreversible dilation of the lower airways
- Daily mucus production, constant coughing, breathlessness: major quality of life impact
- Normal lung clearance impaired
- 500,000 affected worldwide (100,000 in the U.S.)

### •Current treatments: bronchodilators, antibiotics

- No drugs effective to clear mucus

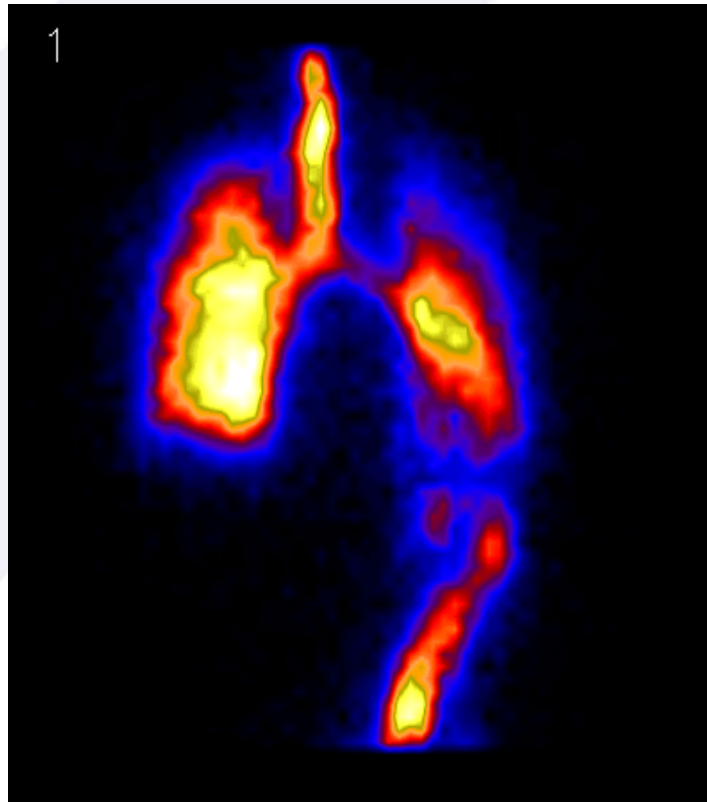
# Bronchitol

## bronchiectasis

- Phase II Trial results
  - 60 patient, double-blind, crossover, placebo-controlled
  - All patients – 4.5 unit improvement in QOL impact score
  - Patients with unclear chests – 6.9 unit improvement in QOL impact score
  - Well tolerated, no adverse events
- Phase III Trials
  - To commence 1Q06 in Australia, EU
  - Initiate US pivotal trial mid-2006
- Supplied on compassionate-use basis in Australia

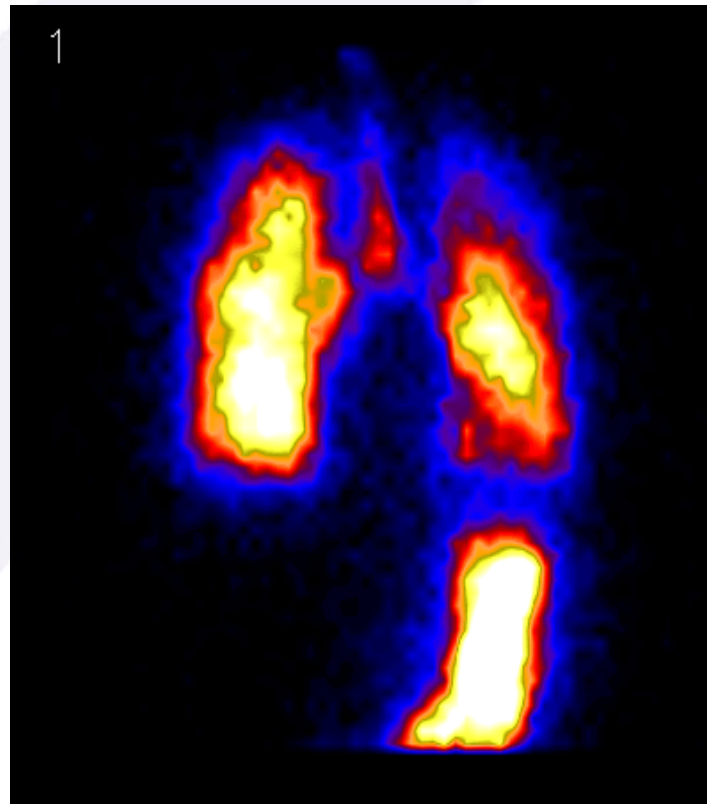
# Bronchitol in the clinic.....

Chronic bronchitis – without bronchitol



# Bronchitol in the clinic.....

Chronic bronchitis – with 400 mg bronchitol





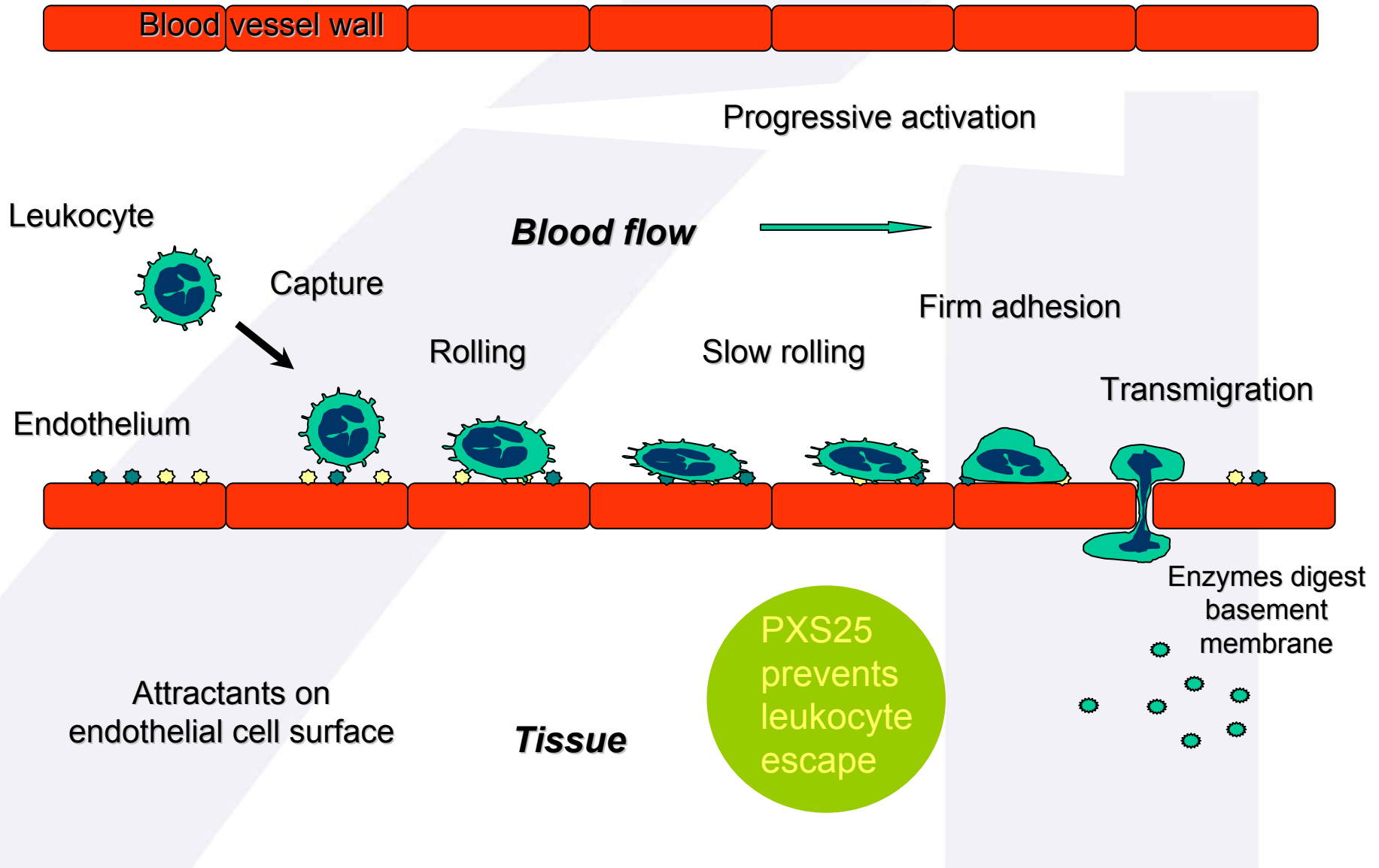
# Autoimmune diseases

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multiple sclerosis  
rheumatoid arthritis

# Autoimmune Disease

## *Inflammation: the leukocyte activation cascade*





# Autoimmune Disease

PXS64

- Selective inhibitor of T cell migration
- Novel mechanism
- Effective in animal models of multiple sclerosis
- Oral prodrug of PXS25
- Current status: preclinical development, start human Phase I clinical trials 1H06

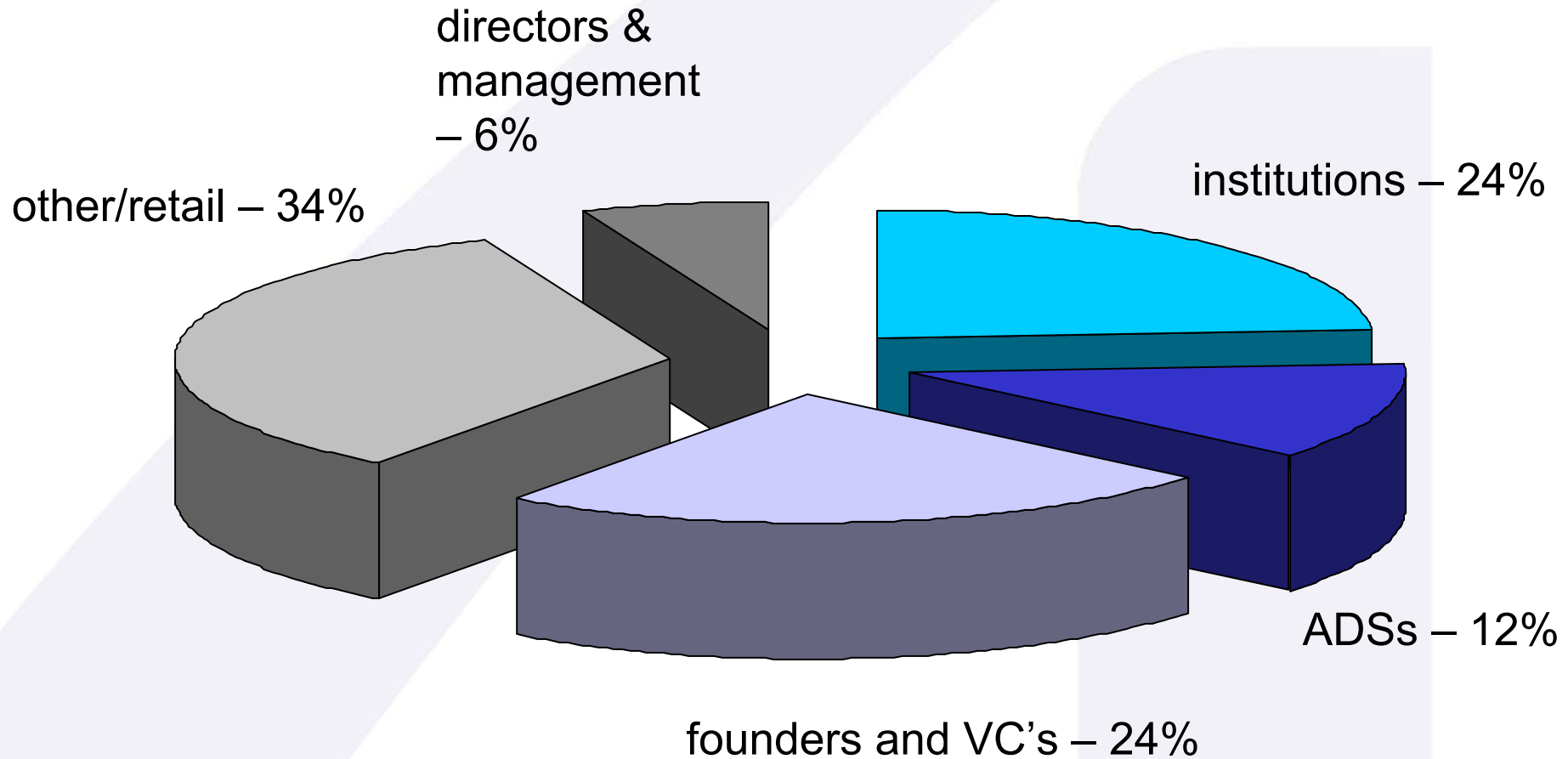


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Financials

# Share Capital

(including options)



31 December 2005: 174m shares; 12m options

**Market Cap: \$380m**

**Volume (ASX/Nasdaq): 250k**

# Australian GAAP

Unaudited - A\$'000 (except per share data) A\$ ~ US\$ 0.75

## Balance Sheet Data

	As at	
	31-Dec-05	30-Jun-05
Cash and cash equivalents	106,434	33,389
Plant & equipment	2,950	2,477
Intangible assets	1,077	1,106
Total assets	111,875	37,937
Total liabilities	2,969	2,470
Total shareholders' equity	108,906	35,467

## Income Statement

	Half year ended	
	31-Dec-05	31-Dec-04
	YTD '06	YTD '05
Revenue		
Interest	1,436	711
Grant income	430	490
	1,866	1,201
Expenses		
Research & development	(5,646)	(4,279)
Commercial	(603)	(320)
Administration	(2,182)	(1,596)
Total expenses	(8,431)	(6,195)
Net loss before and after tax	(6,565)	(4,994)
Basic and diluted loss per share	(0.045)	(0.044)

# US GAAP

Unaudited - A\$'000 (except per share data) A\$ ~ US\$ 0.75

## Balance Sheet Data

	As of	
	Dec-31-05	Jun-30-05
Cash and cash equivalents	106,434	33,268
Plant & equipment	2,873	2,376
Intangible assets	1,077	1,106
Total assets	111,797	37,836
Total liabilities	2,891	2,369
Total shareholders' equity	108,906	35,467

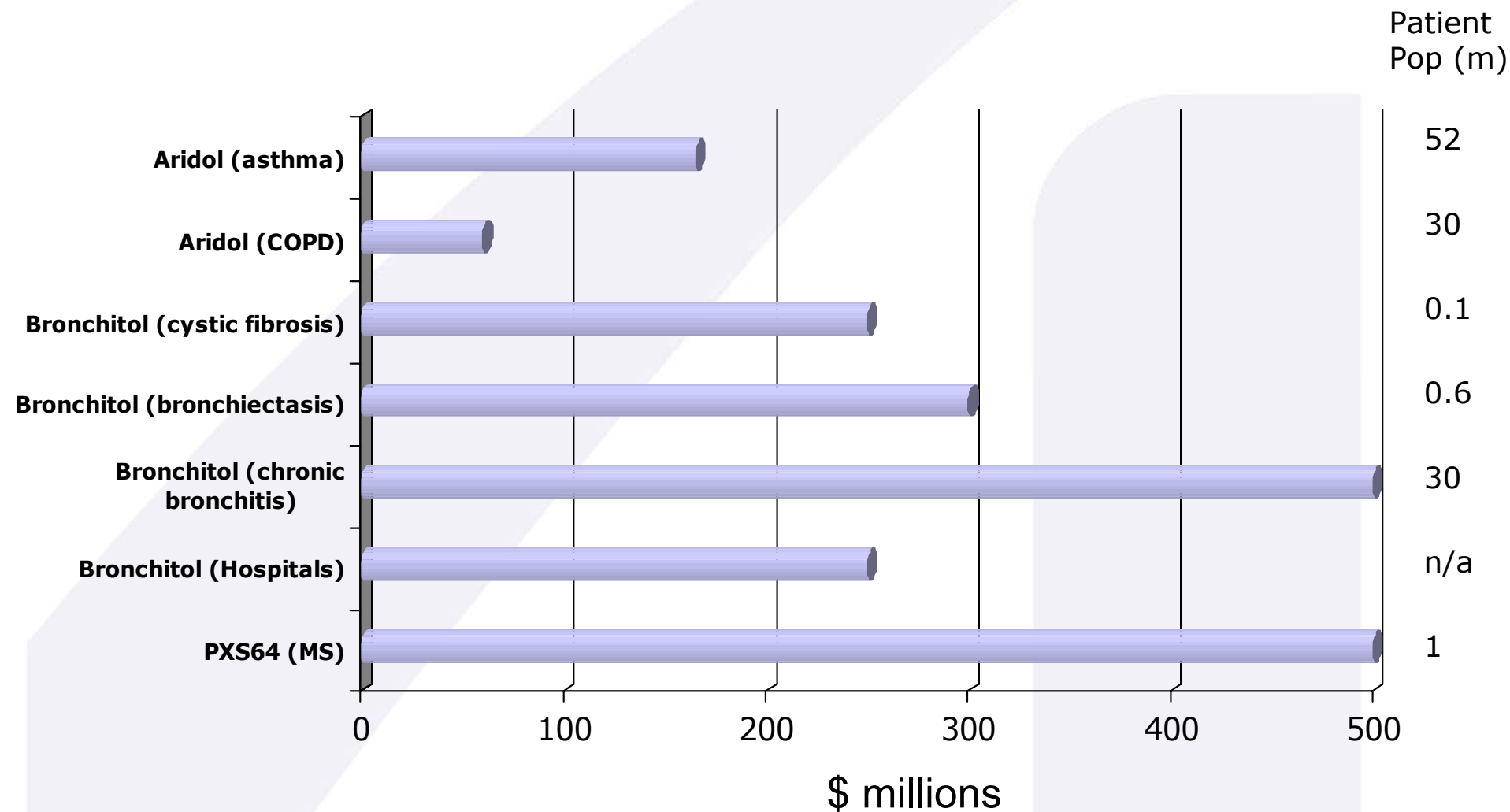
## Statement of Operations

	Six months ended	
	Dec-31-05	Dec-31-04
Revenue	-	-
Operating expenses		
Research & development	4,965	3,711
Commercial	538	320
General and administrative	2,049	1,535
Amortization of intangible assets	46	45
Fair value of stock options issued to employees	403	94
Total operating expenses	8,001	5,705
Loss from operations	(8,001)	(5,705)
Interest and other income	1,436	711
Net loss	(6,565)	(4,994)
Basic and diluted net loss per ADS	(0.045)	(0.044)

# Statement of Operations Profile

- Revenue
- Gross Margin
  - Healthy
  - Low direct material and labour
  - Modest capital cost
  - Capacity utilization/volume
- Selling Costs
  - Centrally managed patient populations
  - Small/focussed sales teams
- Cash Burn
  - 50+ employees
  - Core burn (salaries, rent, fixed costs etc)
  - Plus clinical, preclinical
  - Cash funds sufficient for international approval and launch Aridol and Bronchitol (CF and BCS), and facility expansion

# Revenue Opportunity.....















# Statement of Operations Profile

- Revenue
- Gross Margin
  - Healthy
  - Low direct material (commodities) and labour
  - Modest capital cost
  - Capacity utilization/volume
- Selling Costs
  - Centrally managed patient populations
  - Small/focussed sales teams
- Cash Burn
  - 50+ employees
  - Core burn (salaries, rent, fixed costs etc)
  - Plus clinical, preclinical, market launch
  - Cash funds sufficient for international approval and launch Aridol and Bronchitol (CF and BCS), and facility expansion



# Significant Milestones ahead

Milestone	1Q-06	2Q-06	3Q-06	4Q-06
<b>Aridol</b> Approval – Aus Approval – EU Launch – Aus Launch EU COPD clinical data Ph III US clinical data		    		
<b>Bronchitol – cystic fibrosis</b> Dosing study data Commence PIII trial Pulmozyme trial enrolled				
<b>Bronchitol – bronchiectasis</b> Commence Ph III trial				
<b>Bronchitol – chronic bronchitis</b> Commence Ph II trial				
<b>PXS64</b> Complete preclinical studies			