

pharmaxis

A close-up photograph of a person's hand holding a small, blue and white inhaler device. The hand is positioned in the center of the frame, with the fingers gripping the device. The background is a plain, light-colored wall.

**Therapeutic products  
for  
respiratory and  
autoimmune diseases**

September 2005



# Investor Presentation Disclaimer

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The following factors, among others, could cause actual results to differ materially from those described in the forward-looking statements: risks associated with preclinical, clinical and sales and marketing developments in the biopharmaceutical industry in general and in particular including, without limitation, the potential failure to meet Aridol revenue goals, the potential failure of Bronchitol to prove safe and effective for treatment of COPD and/or Cystic Fibrosis, determinations by regulatory, patent and administrative governmental authorities, competitive factors, technological developments, costs of developing, producing and selling Aridol, Bronchitol and Pharmaxis’ other products under development; and other economic, business, competitive, and/or regulatory factors affecting Pharmaxis’ business generally, including those set forth in Pharmaxis’ filings with the ASIC, including its Annual Report for its most recent fiscal year and its most recent Quarterly Report, especially in the “Factors Affecting Our Operating Results” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” sections, and its Current Reports. Pharmaxis is under no obligation to (and expressly disclaims any such obligation to) update or alter its forward-looking statements whether as a result of new information, future events, or otherwise.

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# The Business.....



**Manufacture**



**Aridol**

- Fund product development through to registration
- Launch products in accessible markets
- Use distributors for other markets
- Retain full product rights

## **Aridol**

- Diagnosis and management of asthma and chronic obstructive pulmonary disease



**Bronchitol**

## **Bronchitol**

- Treatment of cystic fibrosis and chronic obstructive pulmonary disease



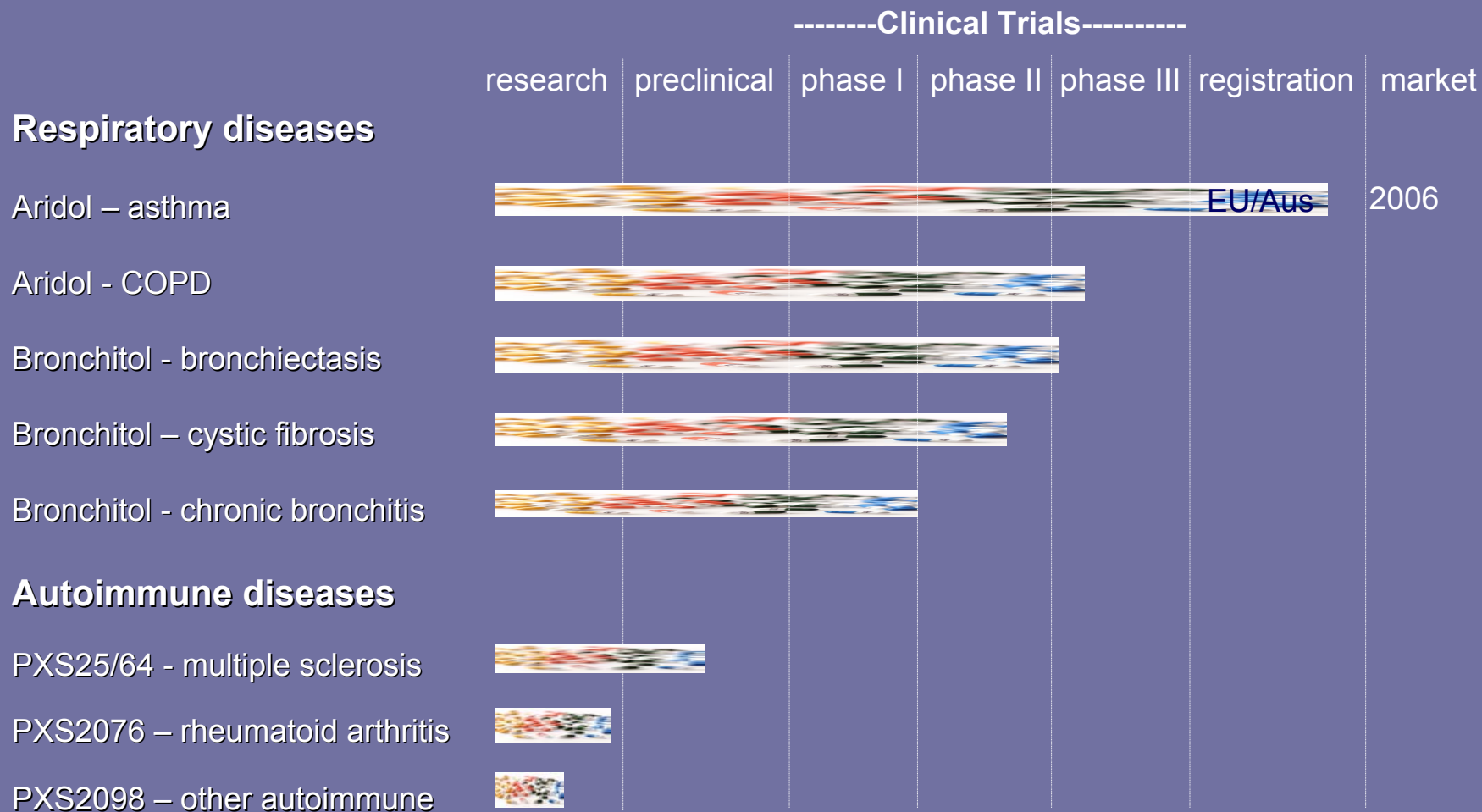
**Autoimmune disease**

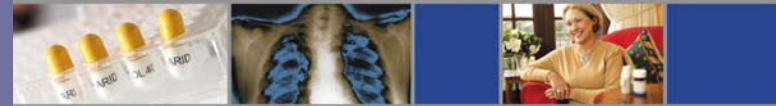
## **PXS64**

- Research into new treatments for multiple sclerosis and rheumatoid arthritis



# The Pipeline....





# The People.....



● Alan Robertson PhD      CEO      Inventor/developer of Zomig



● David McGarvey CA      CFO/Secretary      CFO at Memtec



● Brett Charlton PhD      CMO      Clinical research at Stanford



● Gary Phillips MBA      Commercial      CEO at Novartis Australia



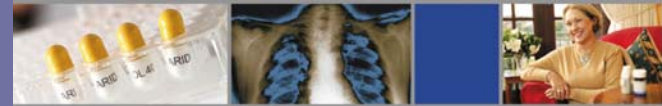
● John Crapper MBA      COO      Managing Director of Memcor



● William Cowden PhD      CSO      Co-inventor of TNF antibodies



● Ian McDonald PhD      CTO      VP Discovery, SIBIA



# The Progress.....

## ● Aridol

- Completed Phase III asthma trial (Aus/EU)
- US Phase III trial ready to commence
- Marketing application lodged - Aus/EU
- Commenced Phase II COPD study

## ● Bronchitol - bronchiectasis

- Completed Phase II trial
- Orphan Drug designation granted by FDA
- Compassionate use granted by TGA
- Eu & Au PIII study ready to commence

## ● Bronchitol – cystic fibrosis

- Completed Phase II trial
- Comparison study ready to commence
- Phase II dosing study ready to commence
- Orphan Drug designation granted by FDA

- Improved oral version of PXS25 discovered

## ● Manufacturing

- TGA approved GMP facility
- Production capacity tripled
- Uneventful TGA facility audit

- Listed on NASDAQ – Aug 05

- A\$20 million placement - Nov 04

- A\$6 million Aus P3 government grant awarded

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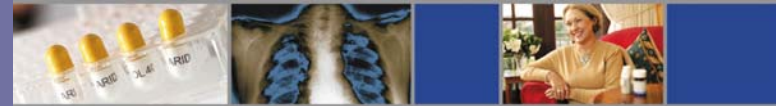
Market capitalization on August 31 2005 ~ A\$325million



# Global Capital Raising

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# Capital raising (structure).....

## Global Capital Raising

- Coordinated bookbuild in Australia and USA
- Common pricing
- Closings are not contingent on one another

Australia (ASX)

Private placement of 17.5 million shares

USA (Nasdaq)

Public offering of 21.0 million shares

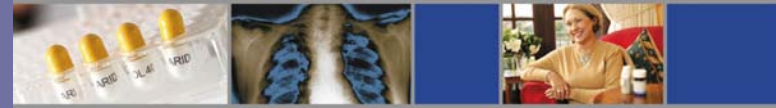
Allowance for increasing  
US offering

Standard feature in US: 4.2 million shares

TOTAL (maximum)

42.7 million shares





# Capital Raising (purpose).....

- Clinical development of Bronchitol for cystic fibrosis
- Broaden the commercial opportunity for Aridol
- International launch of Aridol
- Additional clinical opportunities for Bronchitol
- Clinical development of Bronchitol for chronic bronchitis
- Expansion of manufacturing/company facilities
- Further development of preclinical pipeline
- Raise international profile
- Promote liquidity of Nasdaq/ASX shares



# Capital raising (timing).....

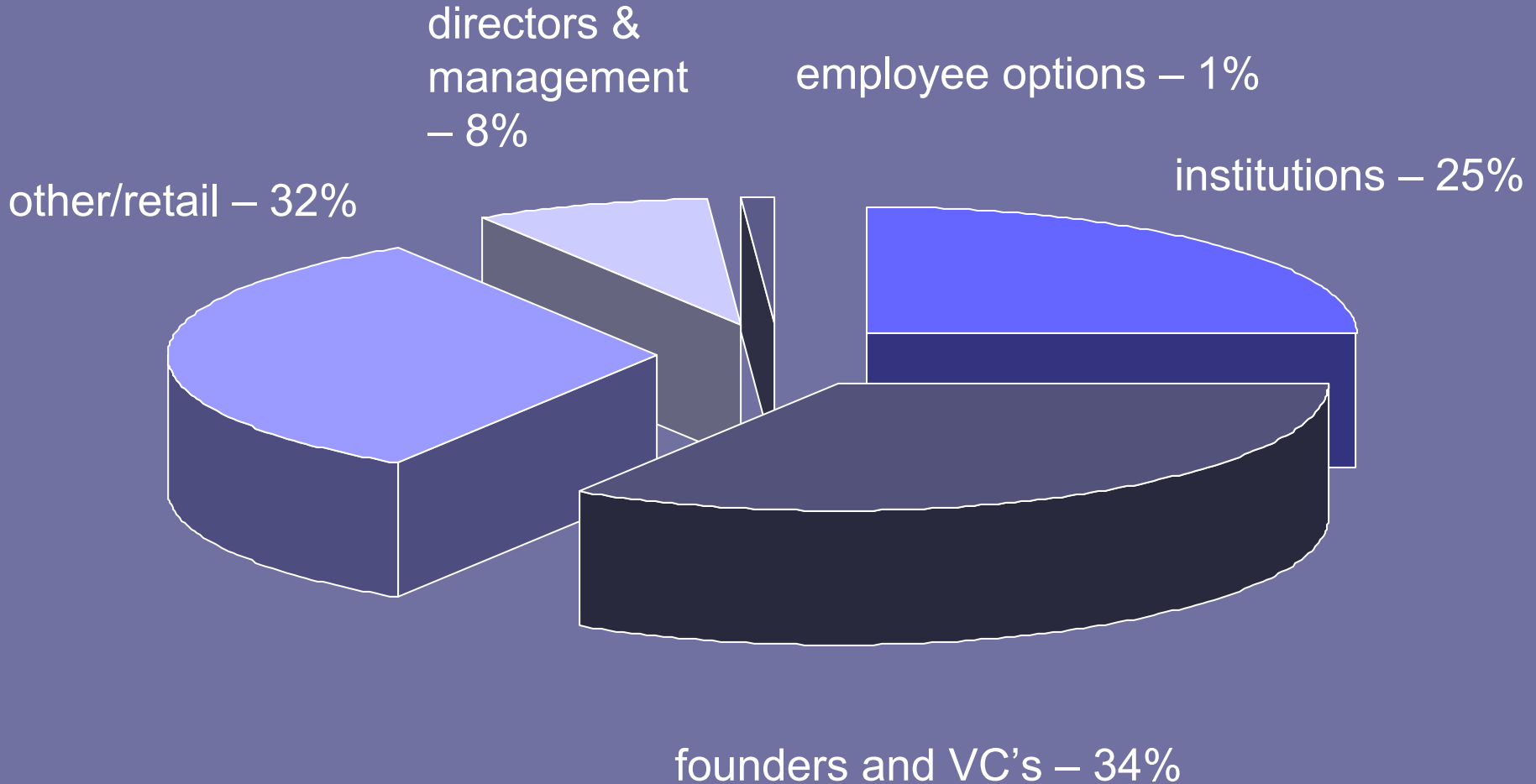


- U.S. SEC review of U.S. prospectus (30-60 days)
  - Lodged 26 September 2005
- Shareholder review and approval
  - General meeting on 28 October 2005
- Anticipated close (subject to shareholder approval and SEC review) – November/December 2005



# Share Capital

(including options)

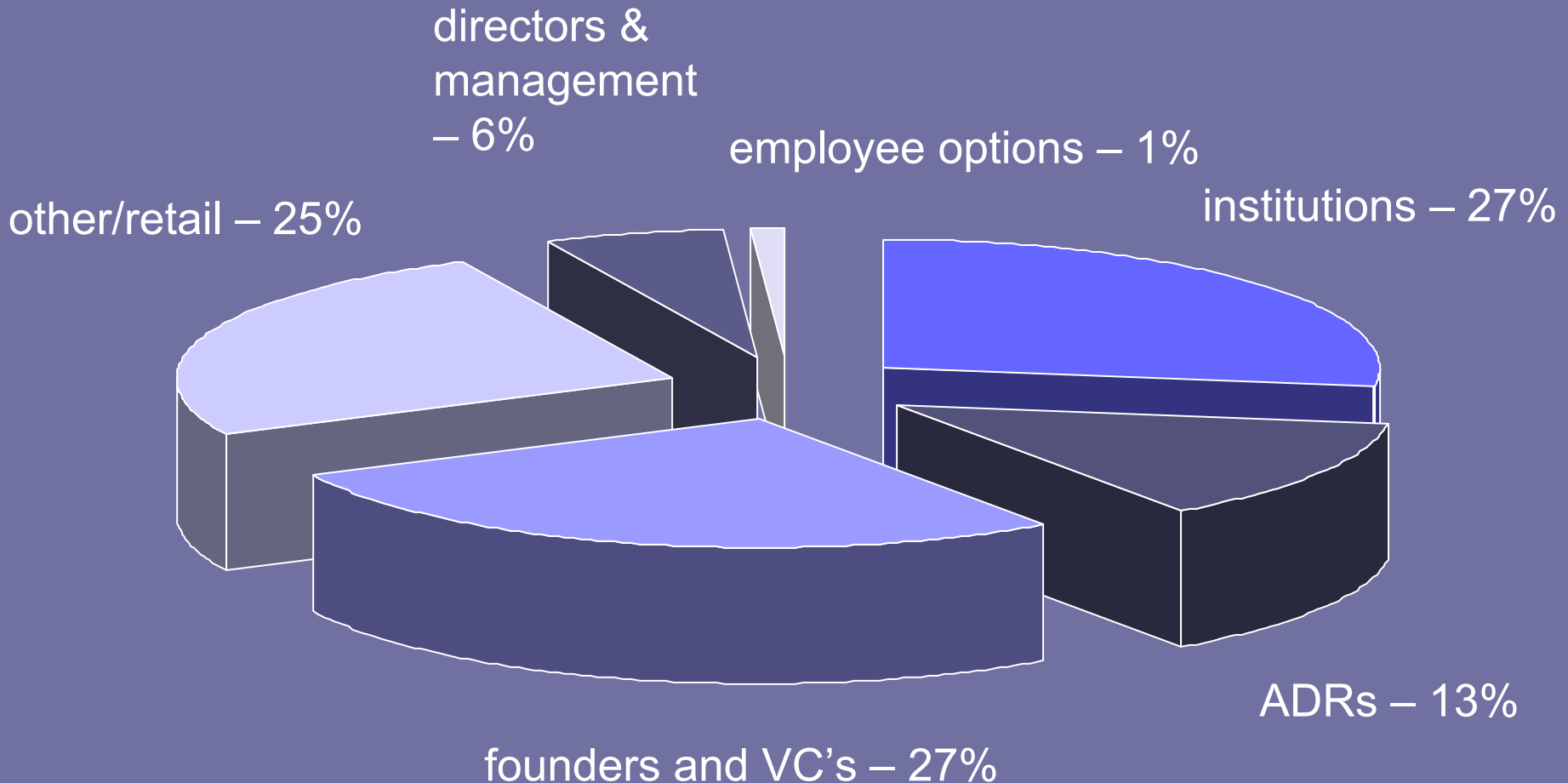


31 August 2005



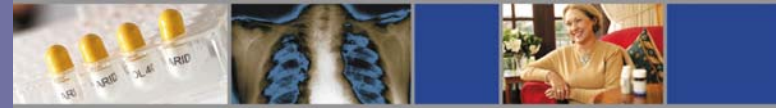
# Share Capital - Proforma

(including options)



31 August 2005

Proforma gives effect to Australian placement of 17.5m shares, US public offering of 21m shares, sale of 3.15m GBS shares



# Bronchitol

cystic fibrosis  
bronchiectasis

chronic obstructive pulmonary disease





# Bronchitol

cystic fibrosis, bronchiectasis and chronic bronchitis

## ● Cystic fibrosis

- Genetic, life limiting, disorder affecting 30,000 in U.S.
- 66% of people with CF are <18
- Annual healthcare cost in the US - >\$1 billion
- Poorly hydrated, difficult to clear, thick mucus



## ● Bronchiectasis

- Abnormal, irreversible dilation of the lower airways
- Daily mucus production, constant coughing, breathlessness
- >60,000 affected in the U.S.
- Treatments only partially effective



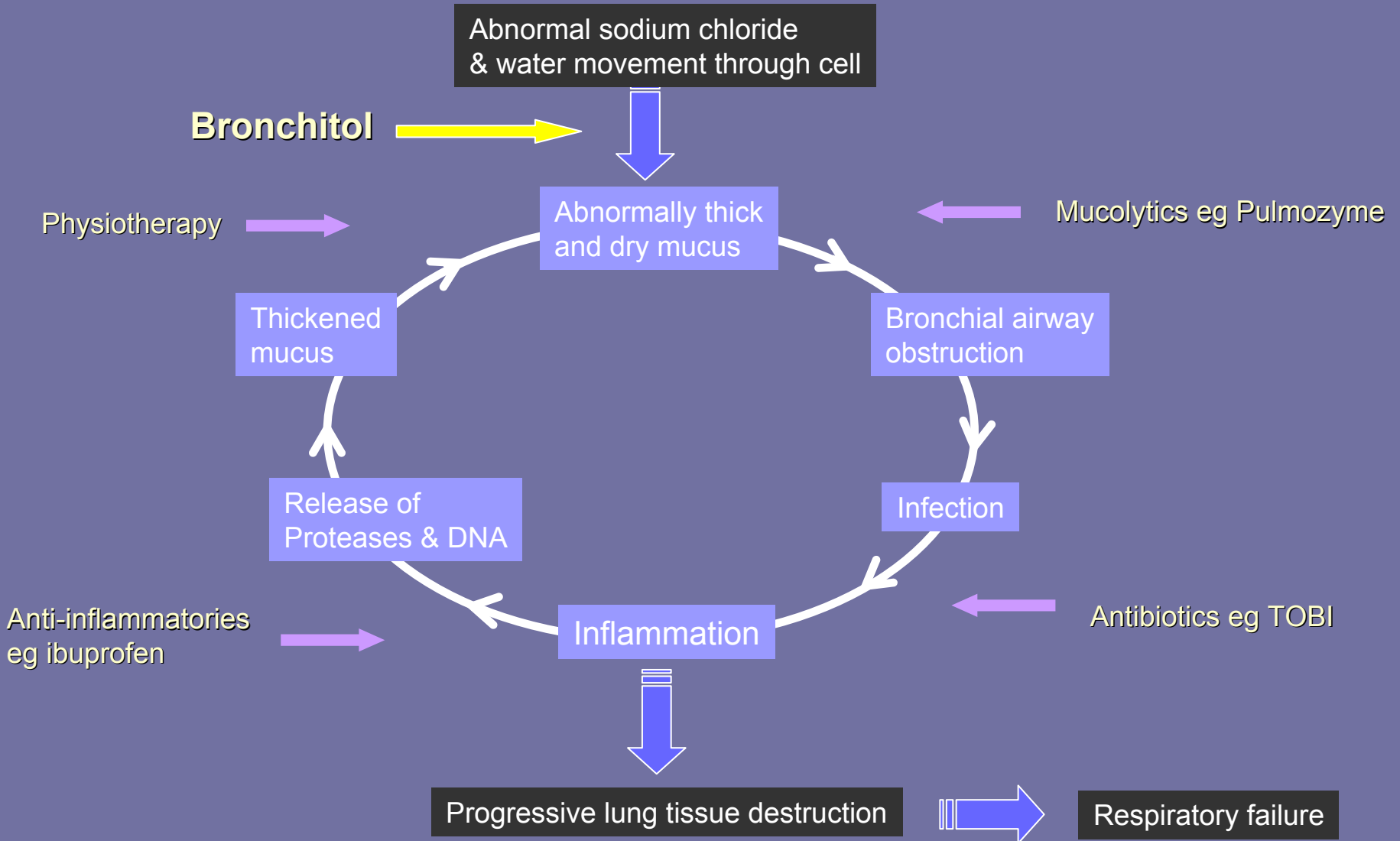
## ● Chronic bronchitis

- Chronic cough, breathlessness, heavy sputum
- >30 million people affected in 7 major pharmaceutical markets

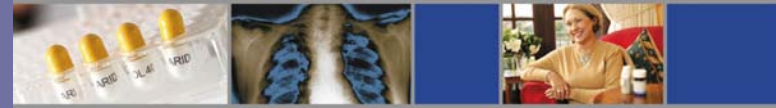




# Thickened mucus begins a vicious cycle....



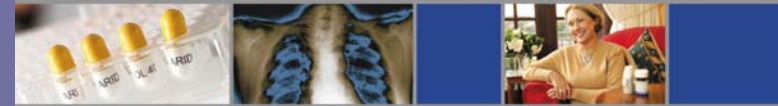




# How Bronchitol works.....



# Bronchitol



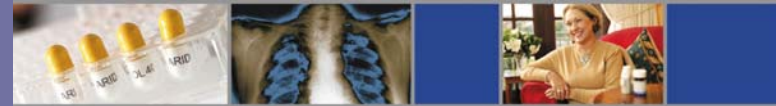
*---cystic fibrosis, bronchiectasis and chronic bronchitis---*

## ● Cystic fibrosis



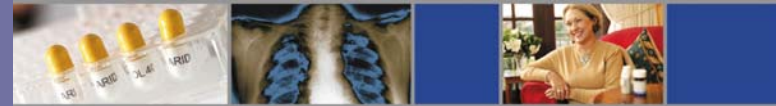
- Acute study completed
- PII 2 week trial completed (CF201)
- PII 2 week dose selection study – Canada
  - To report H1 2006
- PII 3 month comparator study against market leader, Pulmozyme
  - To report 2007
- US Orphan Drug designation granted
- Pivotal pre-registration studies to commence mid 2006
- Targeting market application submission – 2007/2008





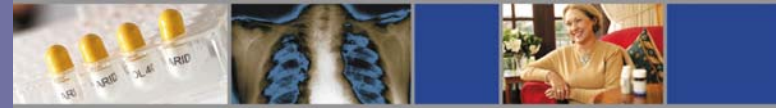
# CF201 study summary....

- Phase II in 39 patients with cystic fibrosis
- Multicentre (8 sites)
- Randomised two week treatment periods
- Placebo controlled
- Double blinded
- Crossover with follow-up

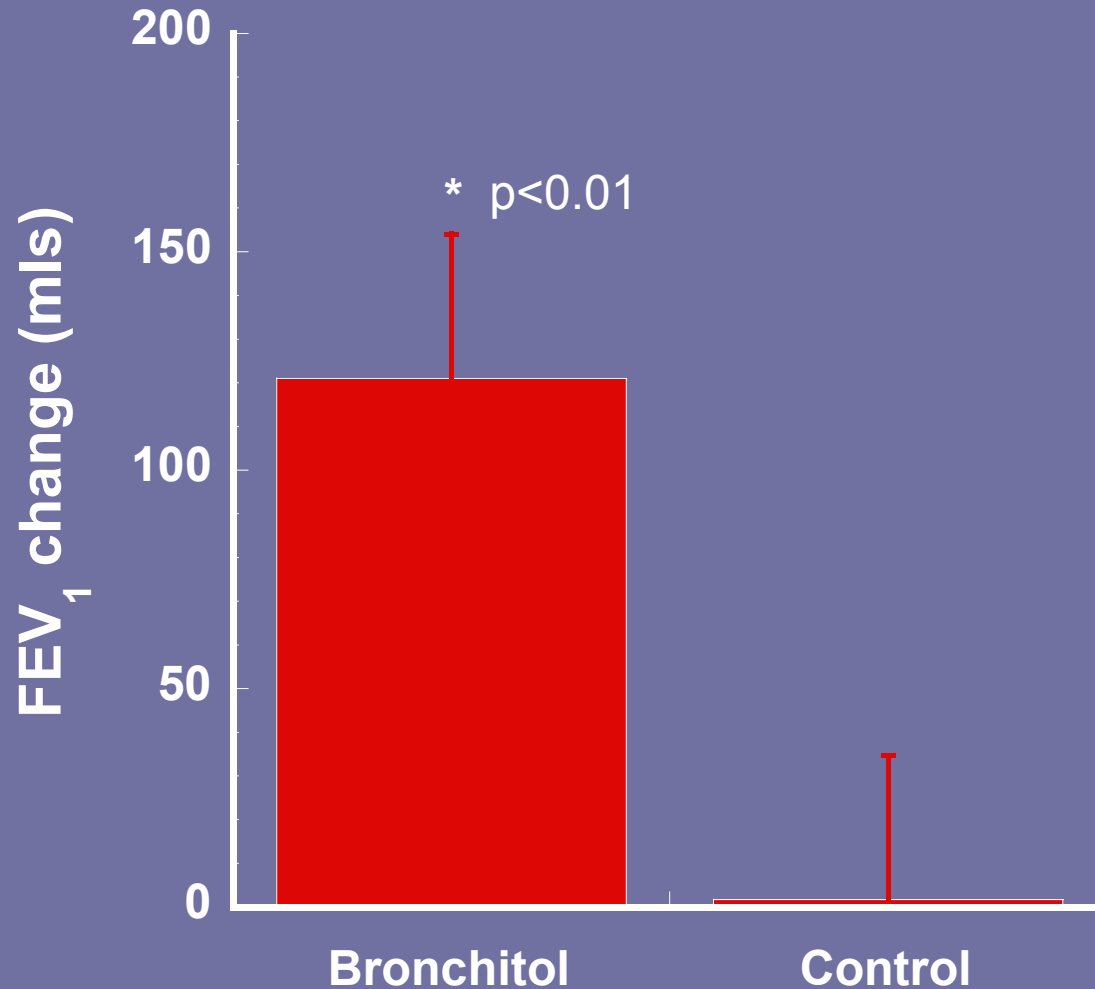


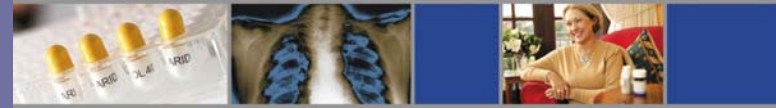
# Primary endpoint

| <b>Change in FEV<sub>1</sub></b> |            |         |
|----------------------------------|------------|---------|
|                                  | Bronchitol | Control |
| Relative (%)                     | 7 ± 2      | 0 ± 2   |
| Versus control                   | p<0.01     |         |



# Primary endpoint - absolute change in FEV<sub>1</sub>





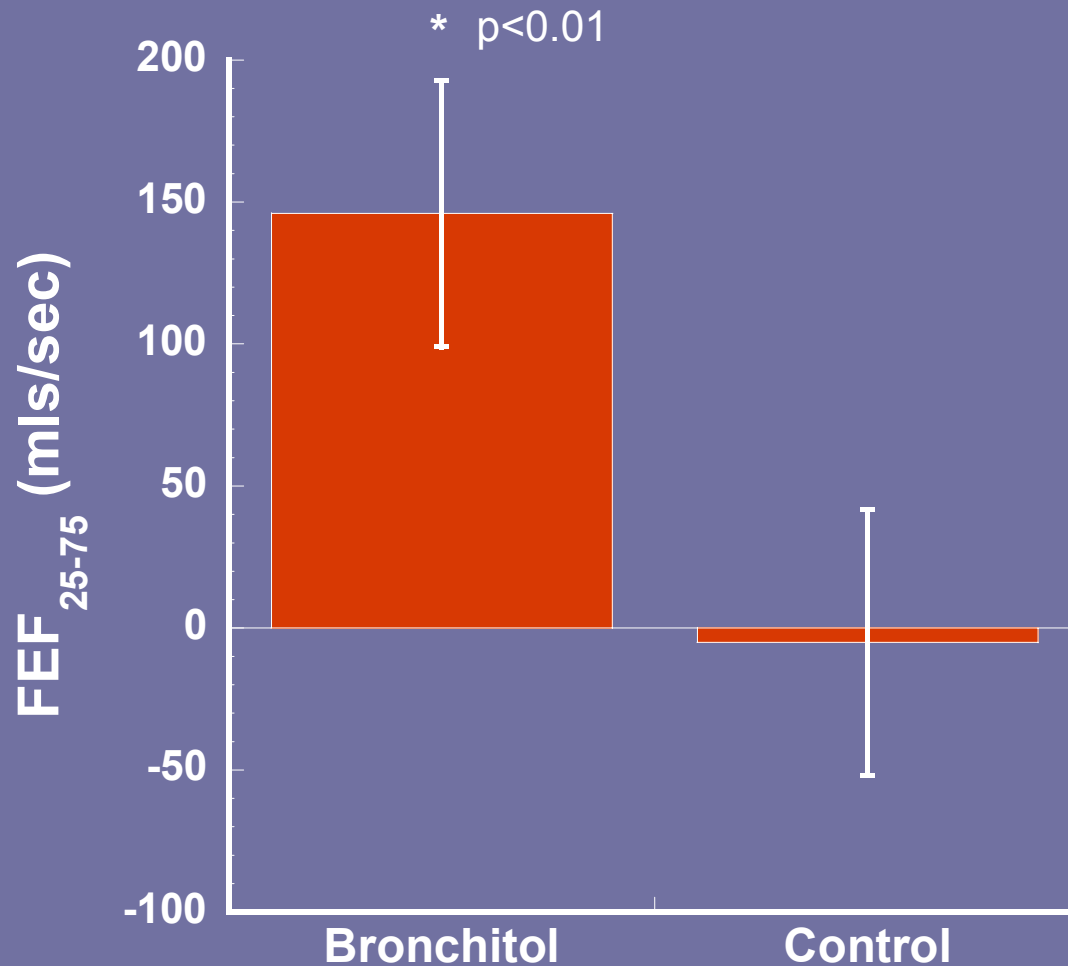
# Secondary endpoints

| Change in $FEF_{25-75}$ |              |             |
|-------------------------|--------------|-------------|
|                         | Bronchitol   | Control     |
| Relative (%)            | $15.5 \pm 5$ | $0.6 \pm 5$ |
| Versus control          | $p < 0.01$   |             |

$FEF_{25-75}$  or MMEF is considered a measure of small airway function



# Secondary endpoints - absolute change in $FEF_{25-75}$





# Bronchitol



cystic fibrosis, bronchiectasis and chronic bronchitis



## ● Bronchiectasis

- PII trials complete
- Supplied in Australia under compassionate use program
- PIII trials (EU & AU) to commence Q4 2005/Q1 2006
- US Orphan Drug designation
- PIII trials (USA) to commence 2006
- Targeting first market authorisation application - 2007





# Phase IIb Clinical Trial Results - bronchiectasis

|                                     |                     |  |
|-------------------------------------|---------------------|--|
| <b>Dropout Rate</b>                 |                     | 3/60 (2 on placebo)  |
| <b>Primary End Points</b>           | Quality of life     | Significant improvement on Bronchitol over baseline ( $p < 0.05$ )     |
|                                     | Sleepiness          | Significant improvement Bronchitol over placebo ( $p < 0.05$ )         |
|                                     | Symptoms            | Highly significant improvement Bronchitol over placebo ( $p < 0.005$ ) |
| <b>Secondary End Points</b>         | Exercise capacity   | Trend to improvement ( $p = 0.07$ )                                    |
|                                     | Lung Function       | No changes   |
|                                     | Sputum microbiology | No changes   |
|                                     | Sputum rheology     |  |
|                                     | Sputum volume       | No changes   |
| <b>Clinical Improvement (all)</b>   | >4.0                | 4.8  |
| <b>Clinical Improvement (43/60)</b> | >4.0                | 6.9  |
| <b>Adverse Events</b>               |                     | None serious   |

Being supplied in Australia on an individual compassionate use basis

# Bronchitol



cystic fibrosis, bronchiectasis and chronic bronchitis

## ● Chronic bronchitis

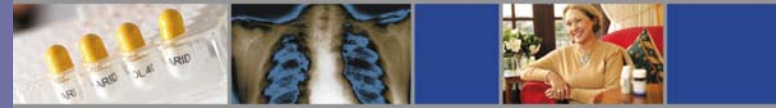


- Disease largely attributable to smoking
- No therapy halts disease progression
  - ⇒ Treatment aimed at symptom relief - bronchodilation



- Acute pilot studies completed
- PII clinical protocol in development
  - ⇒ Quality of Life
  - ⇒ Additional end point
- Study to commence 2006

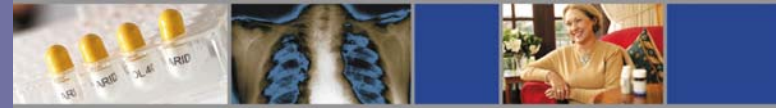




# Aridol™

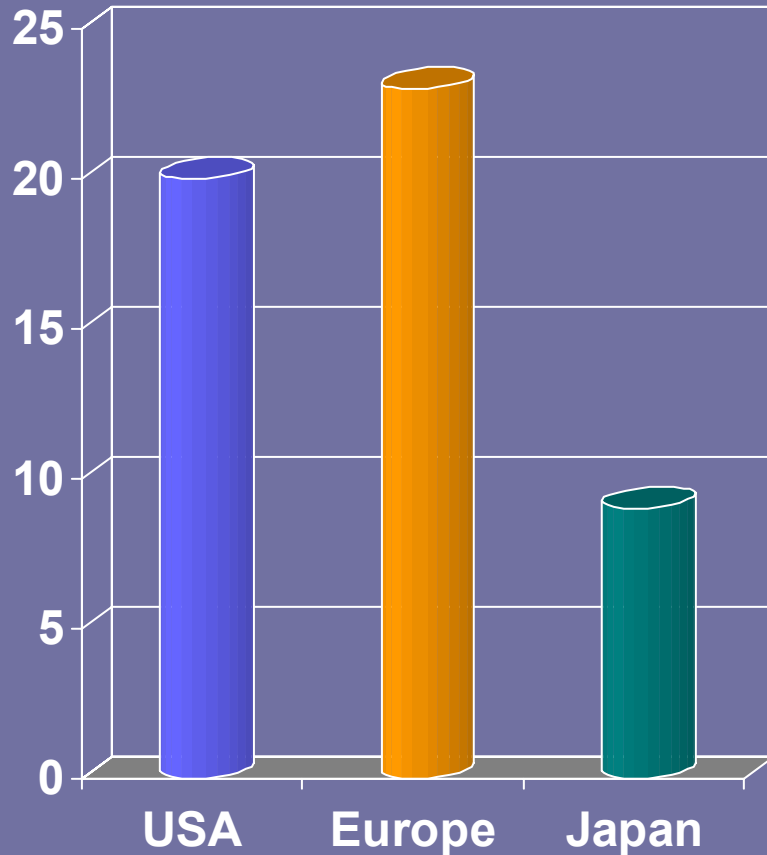


*A rapid and simple test for airways inflammation that facilitates diagnosis and management of asthma and COPD patients.*

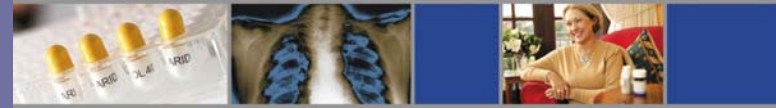


# Asthma – an disease with poor diagnosis

Asthma patients (millions)

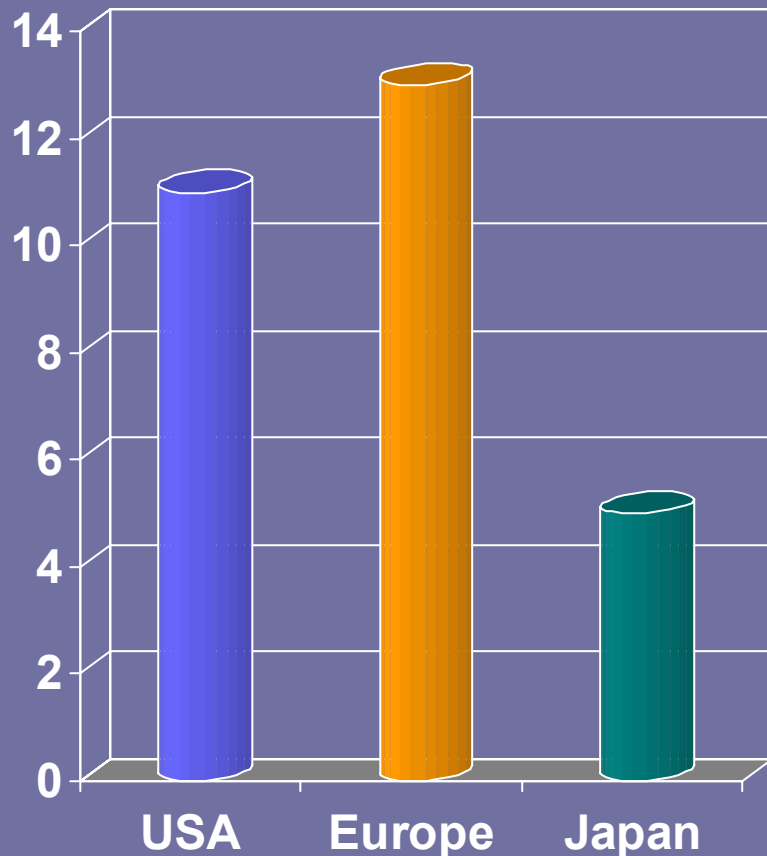


- Asthma has a high prevalence worldwide
- There is no simple test to identify airway inflammation
- The diagnosis rates for asthma remain low, with on average only 57% of the prevalent population diagnosed per country.
- Approximately 15% of people receiving anti-asthma medication do not have asthma.
- Patient management difficult



# COPD – major health problem

COPD patients (millions)

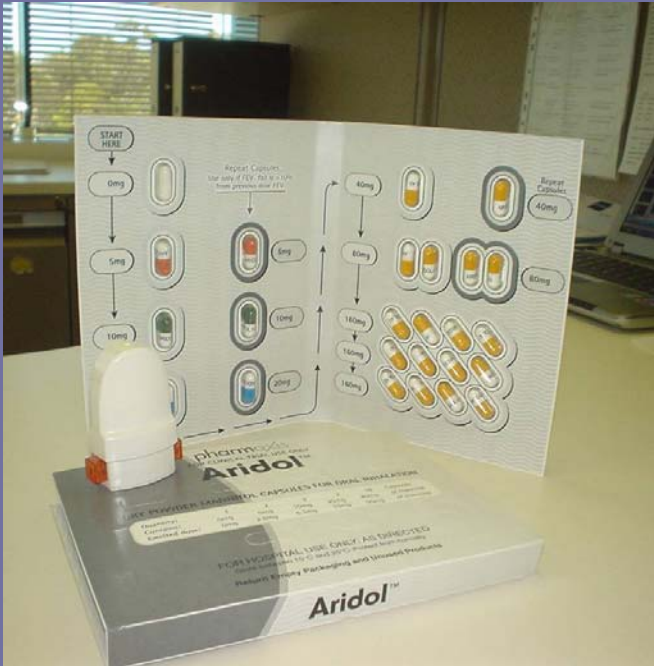


- 30 million people affected in 7 major pharmaceutical markets
- Cost to US healthcare - US\$30 billion pa
- 20-25% respond to inhaled steroids but no test to identify them



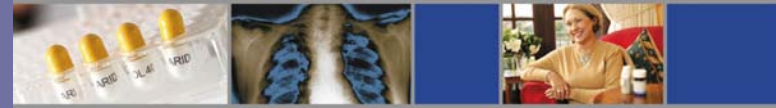
# Aridol™

- Unique clinical applications in the diagnosis and management of Asthma and COPD
- Quick and easy to use – test patients in physicians rooms
- Tested on over 1200 asthma patients



Clinical Trials pack

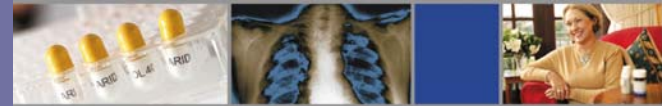






# Phase III trial summary...

- Accurately identifies asthma
- Effective at identifying clinical mis-diagnosis (7%)
  - ⇒ 140,000 Australians
- 20% of subjects over treated and over diagnosed
  - ⇒ 400,000 people in Australia
- 25% of subjects not well controlled
  - ⇒ 500,000 Australian asthmatics

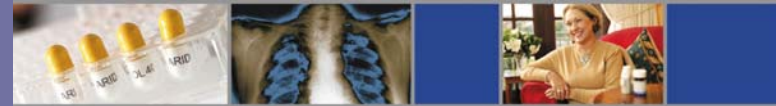
European and Australian marketing authorisation submitted



# Competitor analysis

| Attribute   | Exercise test   | Direct challenge   | eNO   | Aridol  |
|-------------|---|--|---|---|
| Equipment   |  |  |  |  |
| Max Time    | 35 min  | 40 min   | 10 min  | 20 min  |
| Preparation | None  | 30 min   | None  | None  |
| Specificity | EIA   | No   | No  | Yes   |
| Manage Rx   | No  | No   | ?   | Yes   |
| Cost        | \$\$\$  | \$   | \$\$\$\$  | \$  |

***Aridol: First 'point of care' test specific for Asthma***



# Potential clinical applications for Arido<sup>TM</sup>

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

## 1. Asthma diagnosis<sup>1</sup>

- Identifies airway inflammation
- Dose response

## 2. Asthma patient management / response to treatment<sup>2</sup>

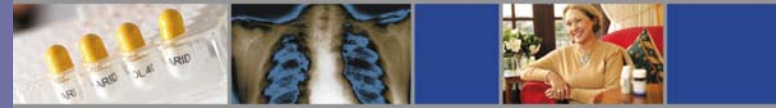
- Negative test = good control of asthma
- Positive test = currently active airway inflammation
- Predict risk of exacerbation when back titrating steroids

## 3. Identification of COPD patients responsive to steroids<sup>2</sup>

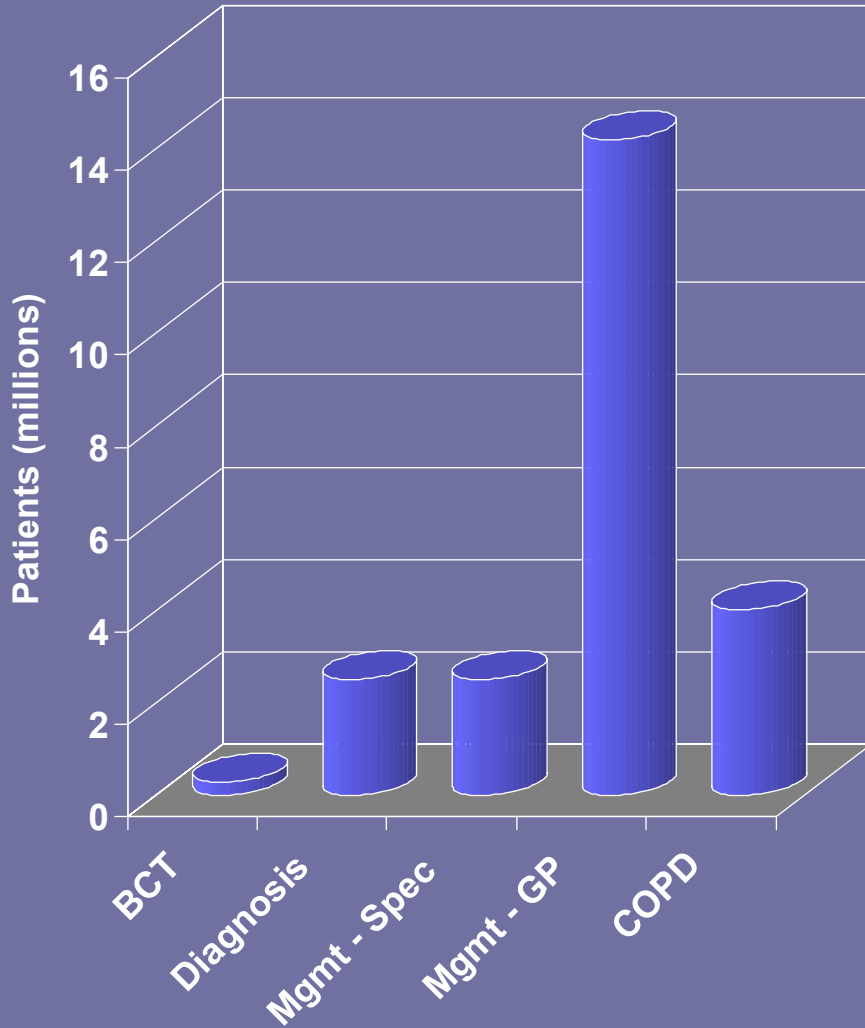
- Confident prescription of appropriate medication.
- Reduce unnecessary steroid usage and healthcare costs.

*NOTES: 1 = Evidence available from pivotal phase 3 study*

*2 = Proof of concept only; definitive studies ongoing / planned*



# Potential market for Aridol™



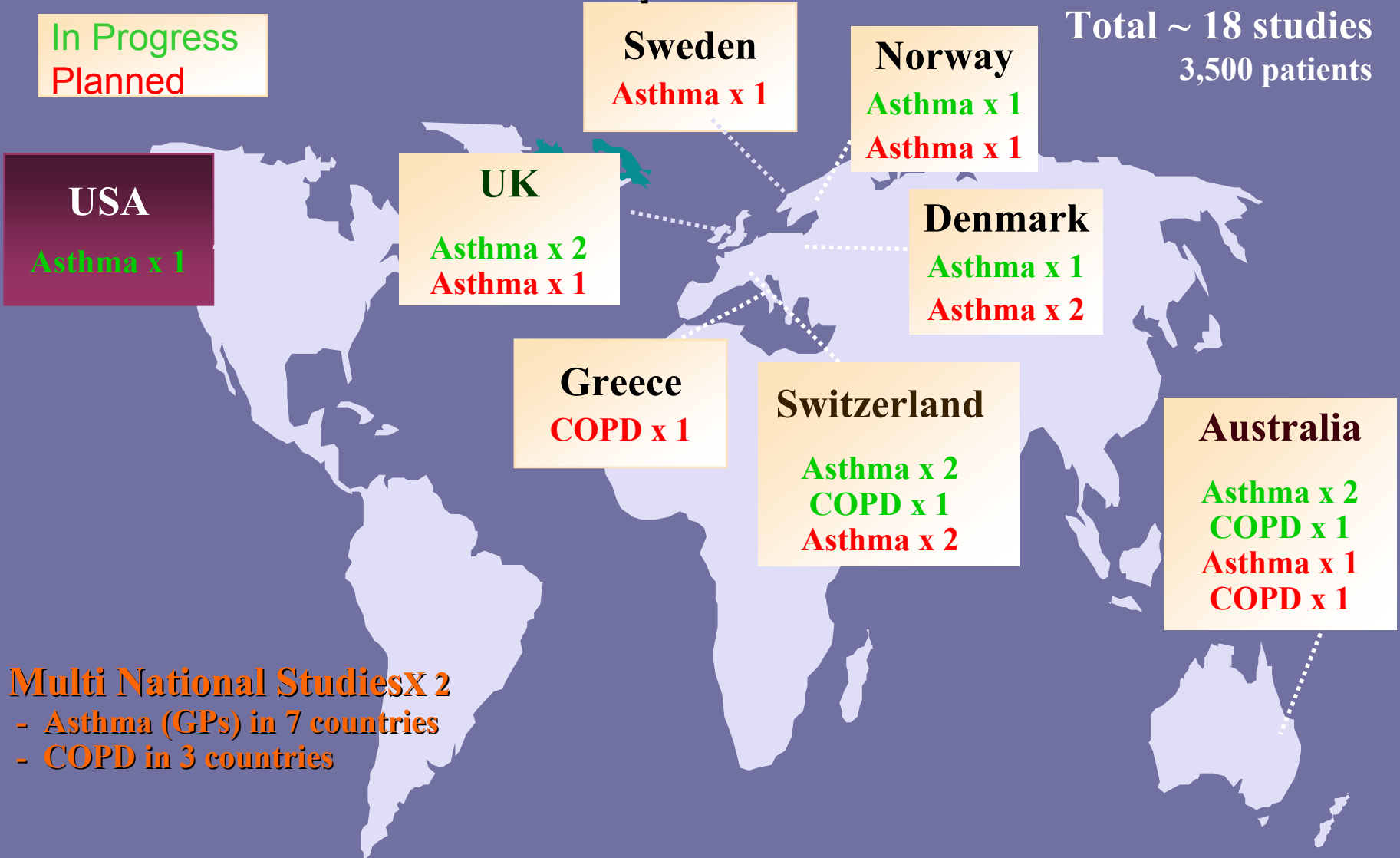
- Replace existing tests
- Asthma diagnosis
- Asthma management
  - Specialists
  - Generalists
- COPD steroid responders



# Worldwide development of Aridol

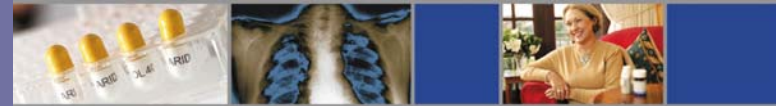
In Progress  
Planned

Total ~ 18 studies  
3,500 patients



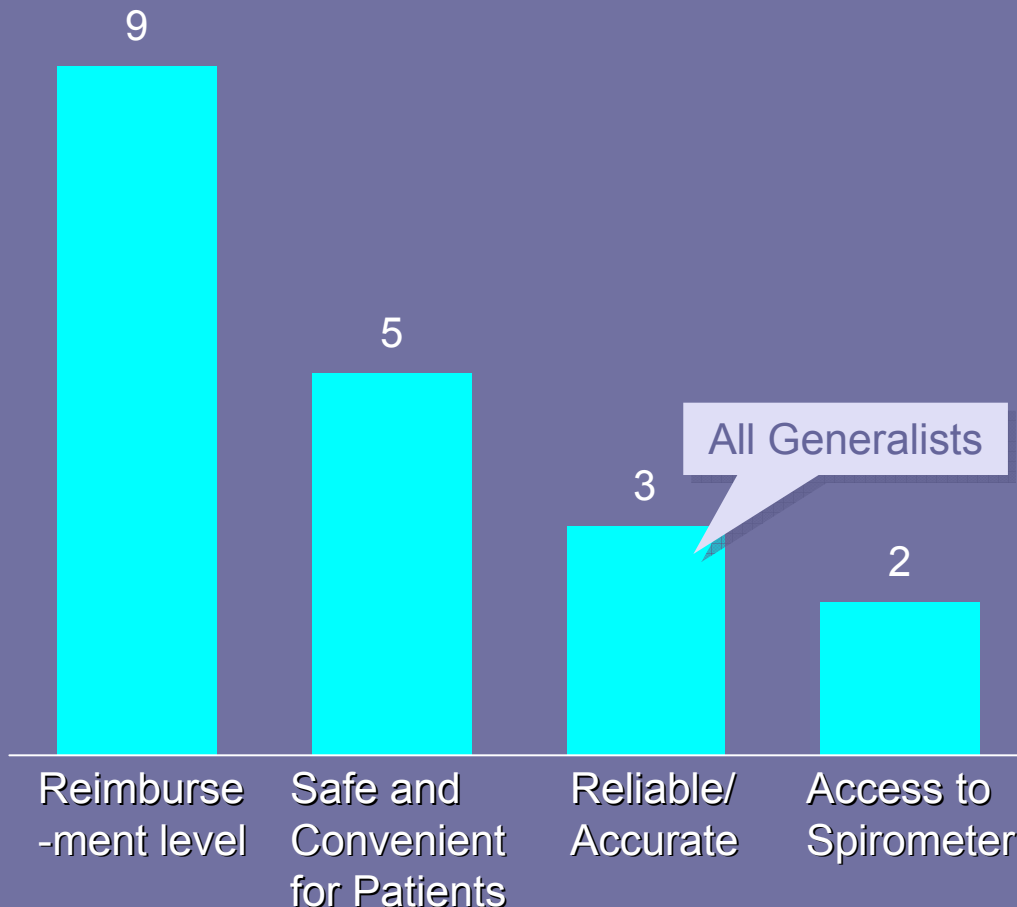
## Multi National Studies X 2

- Asthma (GPs) in 7 countries
- COPD in 3 countries



# Reimbursement and safety / reliability perceptions are the key challenges

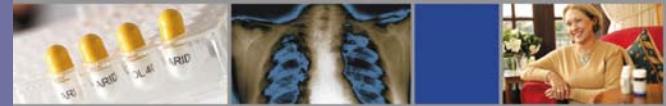
Number of Physicians Who Mentioned\* This Concern About Aridol™ (Out of 50 responders)






**Aridol well placed to overcome challenges**

- US consultant's key finding is that no new procedure codes or modifications to procedure codes are necessary for reimbursement of Aridol
- Completed Aridol phase 3 study designed to answer safety and reliability questions.

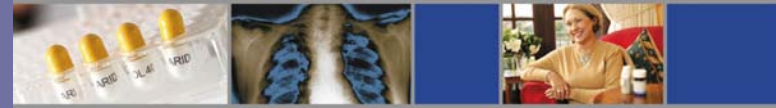
\* Sum of prompted and unprompted responses Source: Physician Interviews; PTD analysis



# Aridol – Commercial key success factors

| Key Success Factor  | Action   | Status  |
|---|--|---|
| First registered indirect challenge test                      | Dossier to EU / TGA<br>FDA trials underway           |  |
| First choice test for Key Opinion Leaders                     | Multiple trials in progress<br>KOL development EU/US |  |
| Labs replace existing tests with Aridol                       | Reimbursement  |  |
| Specialists refer more patients for all indications           | Sign marketing partner<br>(Pharmaxis in Australia)   | Q4 05   |
| Accepted in International Guidelines                          | Publications from studies                            | 2006/7  |
| GPs with asthma clinics commence testing patients with Aridol | Sign marketing partner                               | 2006  |





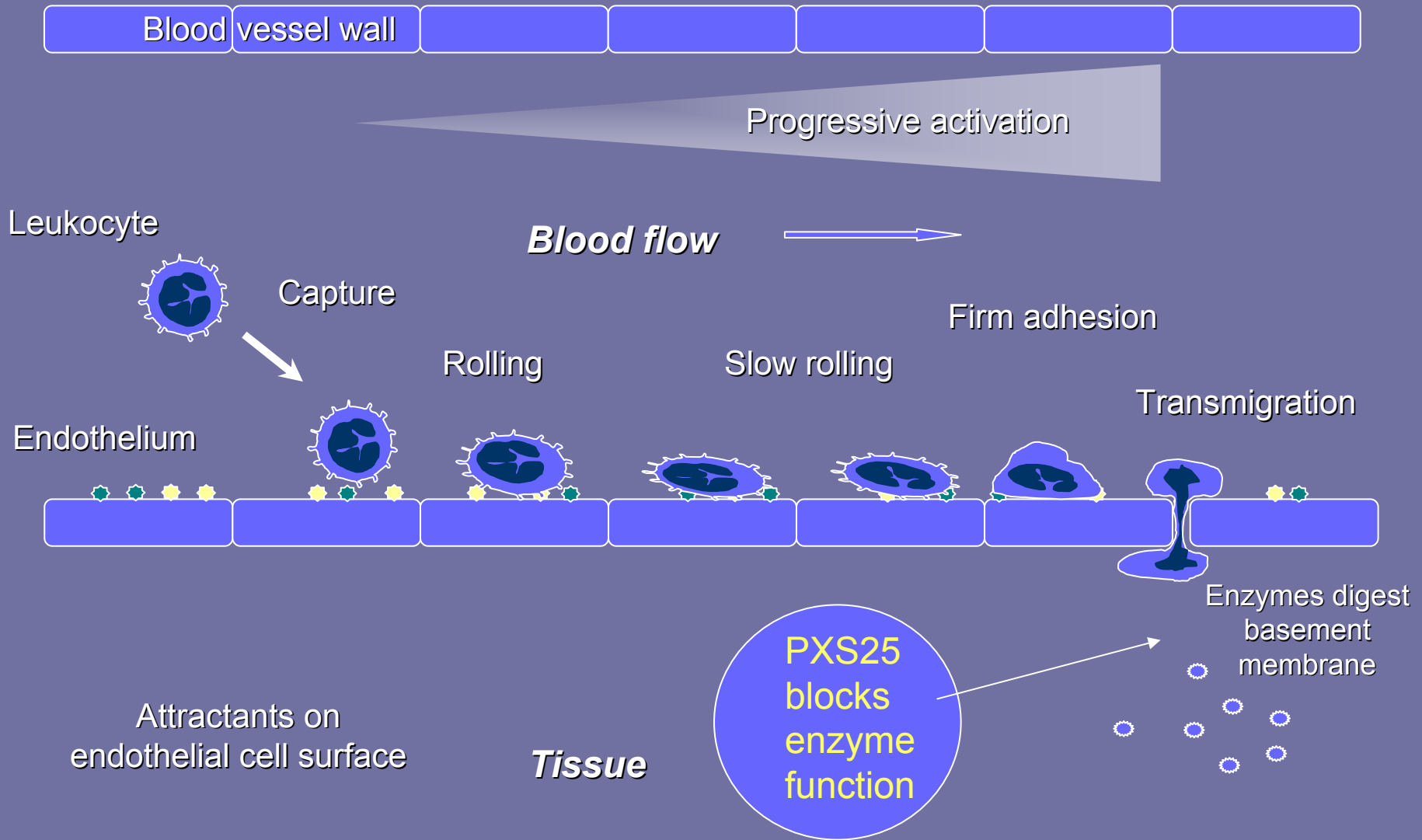
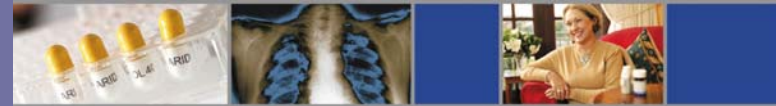
# Autoimmune diseases

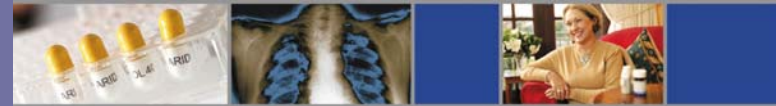
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multiple sclerosis  
rheumatoid arthritis

# Autoimmune Disease

## Inflammation: the leukocyte activation cascade

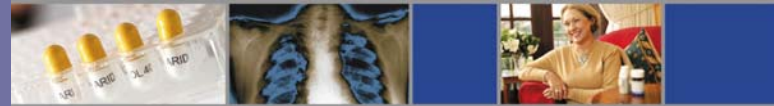




# Autoimmune Disease

- PXS25/64
  - Selective inhibitor of T cell migration
  - Novel mechanism of action
  - Effective in models of multiple sclerosis
  - Complementary with existing treatments
- Competitive Edge
  - Delivery by the oral route
  - Approach clinically validated
- Status
  - Preclinical development
  - Start human PI clinical trials 2006

*Large market opportunity*



# The clinical future .....

2005

2006

Q3

Q4

Q1

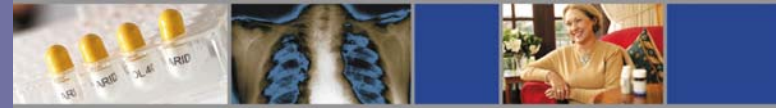
Q2

H2 2006

- Cystic Fibrosis
  - Canadian PIIb dosing study commences
  - UK study versus pulmozyme commences
- Bronchiectasis
  - European PIII study commences
- Aridol
  - US asthma PIII study commences

- Cystic Fibrosis
  - Canadian PII dosing study reports
  - European PIII study commences
  - US PIII study commences
- Bronchiectasis
  - US PIII study commences
- Aridol
  - US asthma study reports
  - Australian COPD study reports

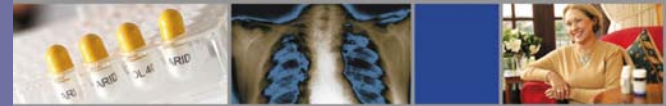
Complete European bronchiectasis Phase III study



# Financials

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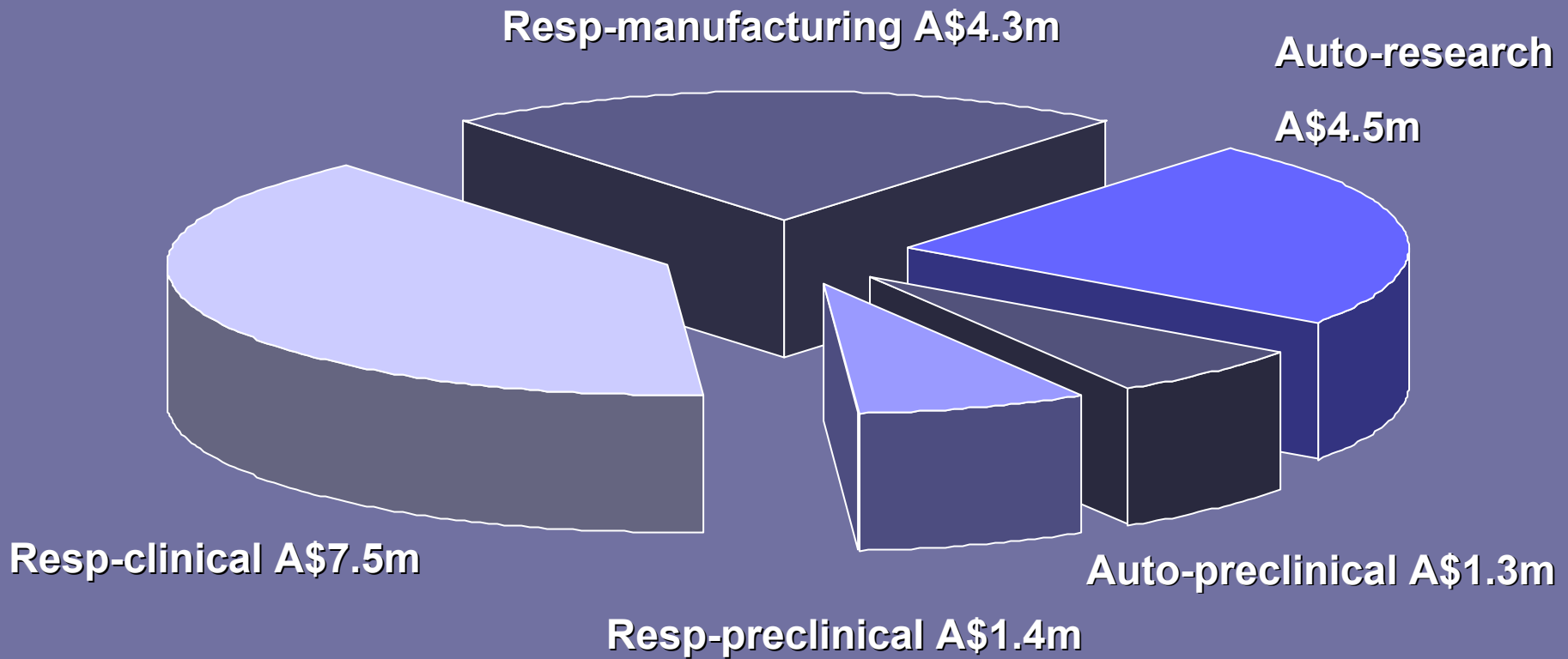
**ASX Investors**

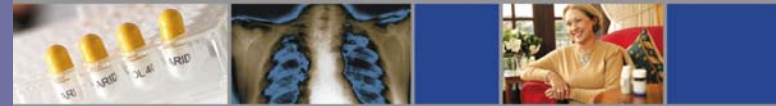


|                                      | Year ended 30 June, |                |                |                |
|--------------------------------------|---------------------|----------------|----------------|----------------|
|                                      | 2005<br>\$'000      | 2004<br>\$'000 | 2003<br>\$'000 | 2002<br>\$'000 |
| <b>Financial Performance</b>         |                     |                |                |                |
| Revenue                              |                     |                |                |                |
| Interest received                    | 1,702               | 1,075          | 284            | 43             |
| Research grants                      | 1,172               | 1,105          | 976            | 646            |
| Other                                |                     | 48             | 43             |                |
|                                      | <u>2,874</u>        | <u>2,228</u>   | <u>1,303</u>   | <u>689</u>     |
| Expenses                             |                     |                |                |                |
| Research & development               | (9,154)             | (6,047)        | (1,790)        | (1,151)        |
| Commercial                           | (847)               | -              |                |                |
| Administration                       | (3,105)             | (2,182)        | (981)          | (140)          |
| Total expenses                       | <u>(13,106)</u>     | <u>(8,229)</u> | <u>(2,771)</u> | <u>(1,291)</u> |
| Net loss before and after tax        | <u>(10,232)</u>     | <u>(6,001)</u> | <u>(1,468)</u> | <u>(602)</u>   |
| Depreciation & amortisation          | 626                 | 410            | 256            | 130            |
| EBITDA                               | (11,308)            | (6,666)        | (1,496)        | (515)          |
| <b>Cash Flows</b>                    |                     |                |                |                |
| Cash flows from operating activities | (9,274)             | (4,652)        | (1,168)        | (363)          |
| Cash flows from investing activities | (1,575)             | (406)          | (1,652)        | (36)           |
| Cash flows from financing activities | 19,021              | 22,891         | 9,453          | -              |
| Net increase (decrease) in cash held | <u>8,172</u>        | <u>17,833</u>  | <u>6,633</u>   | <u>(399)</u>   |



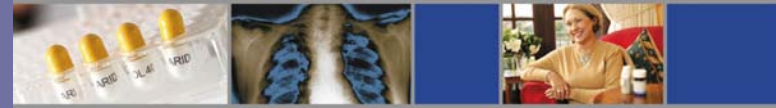
# R&D from Inception to June 30, 2005 (A\$19.2m before R&D Grants of A\$4.6m)



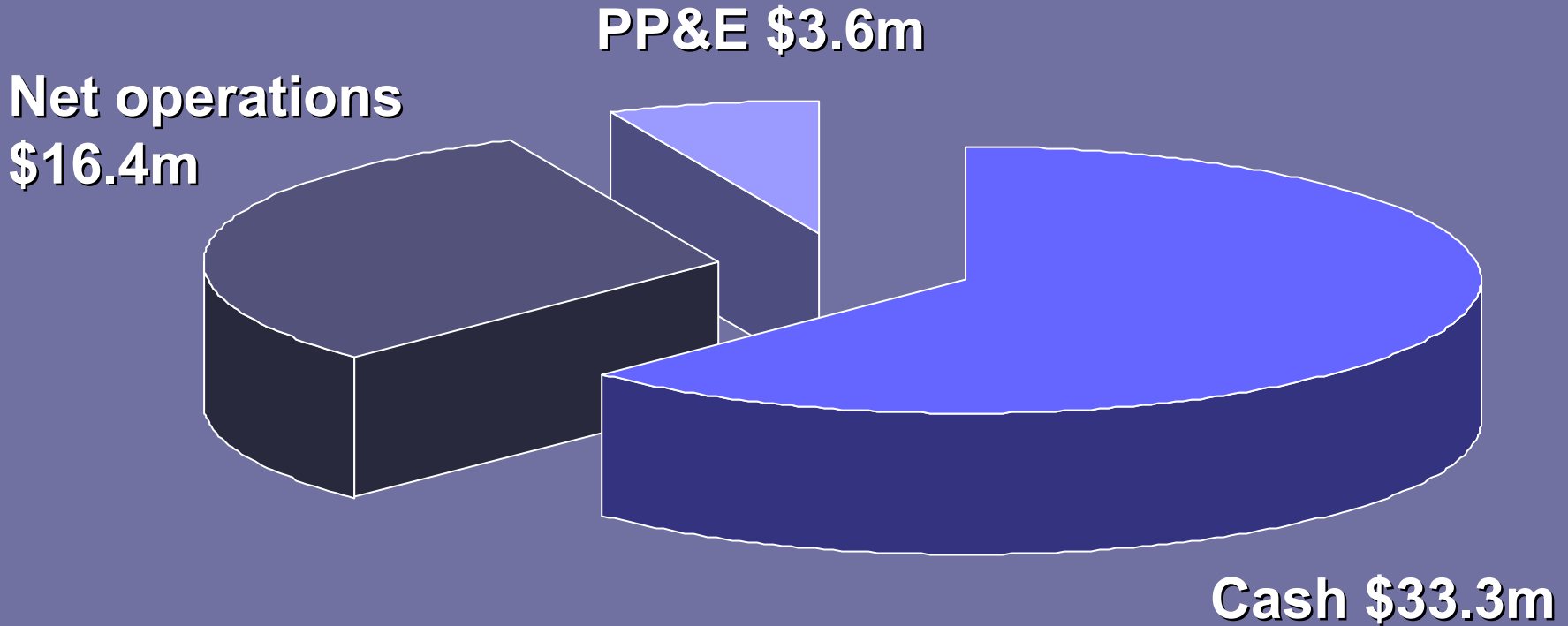


|   | 30 June, |        |
|---|----------|--------|
|   | 2005     | 2004   |
|   | \$'000   | \$'000 |
| <b>Financial Position</b>               |          |        |
| Cash and bank accepted commercial bills | 33,389   | 25,217 |
| Plant & equipment                       | 2,477    | 1,474  |
| Intangible assets                       | 1,106    | 1,162  |
| Total assets                            | 37,937   | 28,261 |
| Total liabilities                       | 2,369    | 1,481  |
| Total shareholders' equity              | 35,569   | 26,780 |





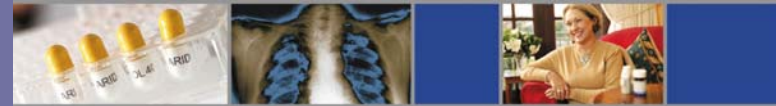
# Total Capital Raised to June 30, 2005 A\$53.3m



# Capitalisation Table



|  | 30-Jun-05      | 30-Sep-05      | Proforma       |
|--|----------------|----------------|----------------|
| <b>Share Capital</b>                         |                |                |                |
| (in thousands except option price/life data) |                |                |                |
| <b>Share Capital</b>                         |                |                |                |
| Shares on issue                              | 134,770        | 134,982        | 134,982        |
| Global Capital Raising                       |                |                |                |
| ASX  |                |                | 17,500         |
| NASDAQ                                       |                |                | 21,000         |
|  | <u>134,770</u> | <u>134,982</u> | <u>173,482</u> |
| Escrowed to 10 November 2005                 | 24,964         | 24,964         | 24,964         |
| Escrowed 90 days post closing                |                |                | 44,713         |
| <b>Options</b>                               |                |                |                |
| Options on Issue                             | 10,914         | 11,302         | 11,302         |
| Vested                                       | 8,792          | 8,792          | 8,792          |
| Escrowed to 10 November 2005                 | 6,720          | 6,720          | 6,720          |
| Escrowed 90 days post closing                |                |                | 9,765          |
| Average Exercise Price                       | 0.31           | 0.39           | 0.39           |
| Average Life - Years                         | 6.7            | 6.7            | 6.7            |



# Summary.....

- Well resourced
- Bronchitol in Phase III for bronchiectasis
  - ✓ Market launch targeting 2007 (if clinical trials successful)
- Bronchitol completed Phase II for cystic fibrosis
  - ✓ Positive results
- Technical risk removed for Aridol
- Aridol asthma launch 2006 (if approved)
- Integrated business
  - ✓ All marketing rights retained
- Pipeline of earlier stage products
  - ✓ R&D phase