

pharmaxis

# Therapeutic products for respiratory diseases

October 2009

# Forward Looking Statements

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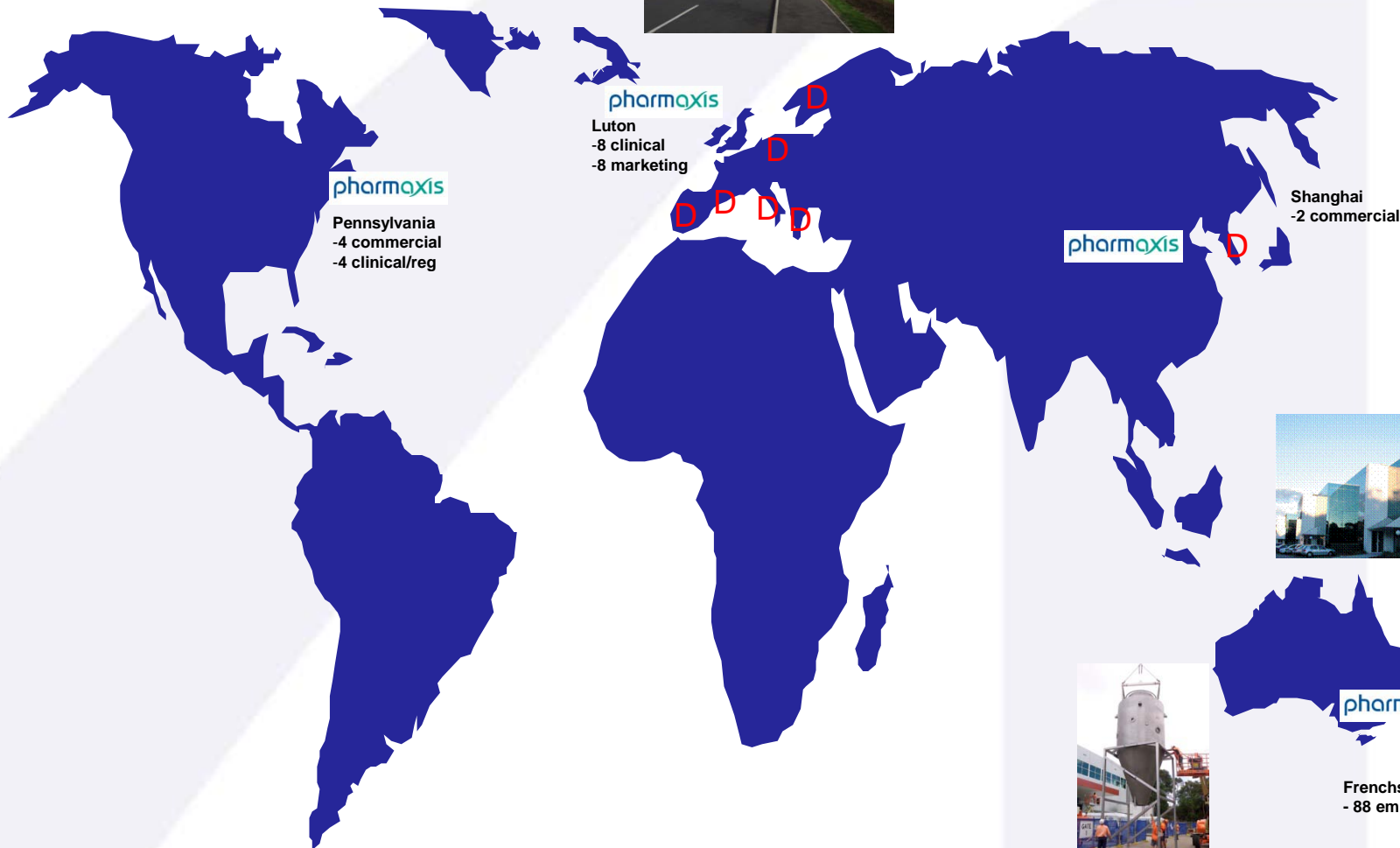
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# Pharmaxis Global Operations 2009



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Pennsylvania  
-4 commercial  
-4 clinical/reg

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Luton  
-8 clinical  
-8 marketing

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Shanghai  
-2 commercial



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Frenchs Forest  
-88 employees



# Development Pipeline

-----Clinical Trial Phases-----

Research    preclinical    phase I    phase II    phase III    registration    market

Aridol – asthma (Aus/EU/Korea)

Aridol – asthma (US)

Bronchitol – bronchiectasis (Aus)

Bronchitol – bronchiectasis (US/EU)

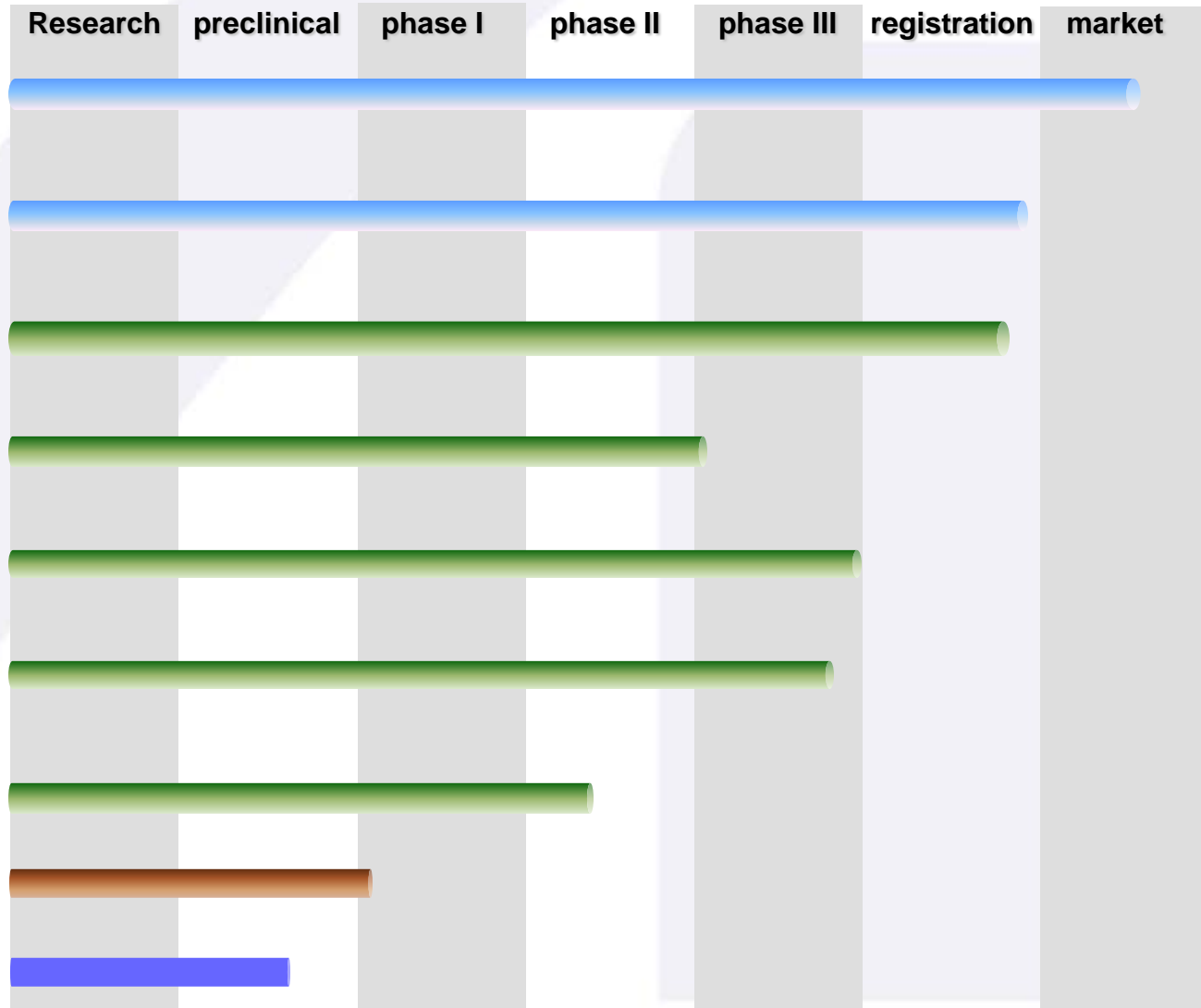
Bronchitol – cystic fibrosis (EU/Aust)

Bronchitol – cystic fibrosis (US)

Bronchitol – acute indications

PXS25 – lung fibrosis

PXS4159 – asthma



# Bronchitol

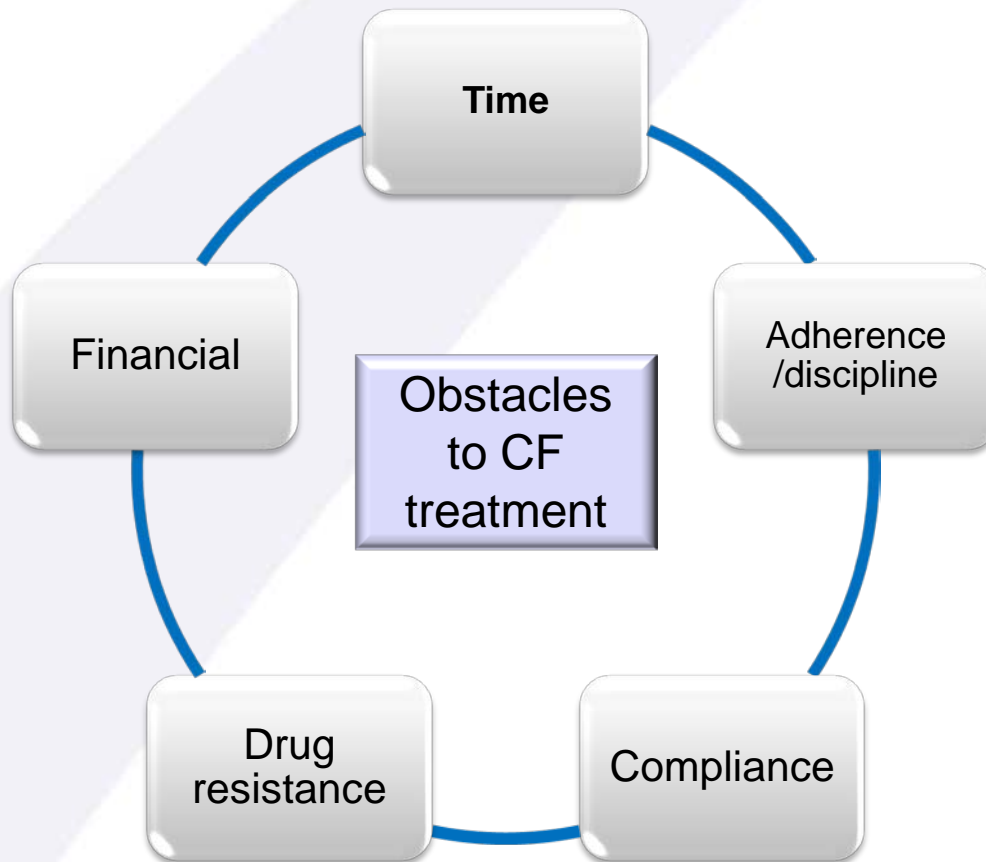


***Mucus clearance:***

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

# Cystic Fibrosis market research

The time commitment to treatment is the biggest challenge to physicians and patients

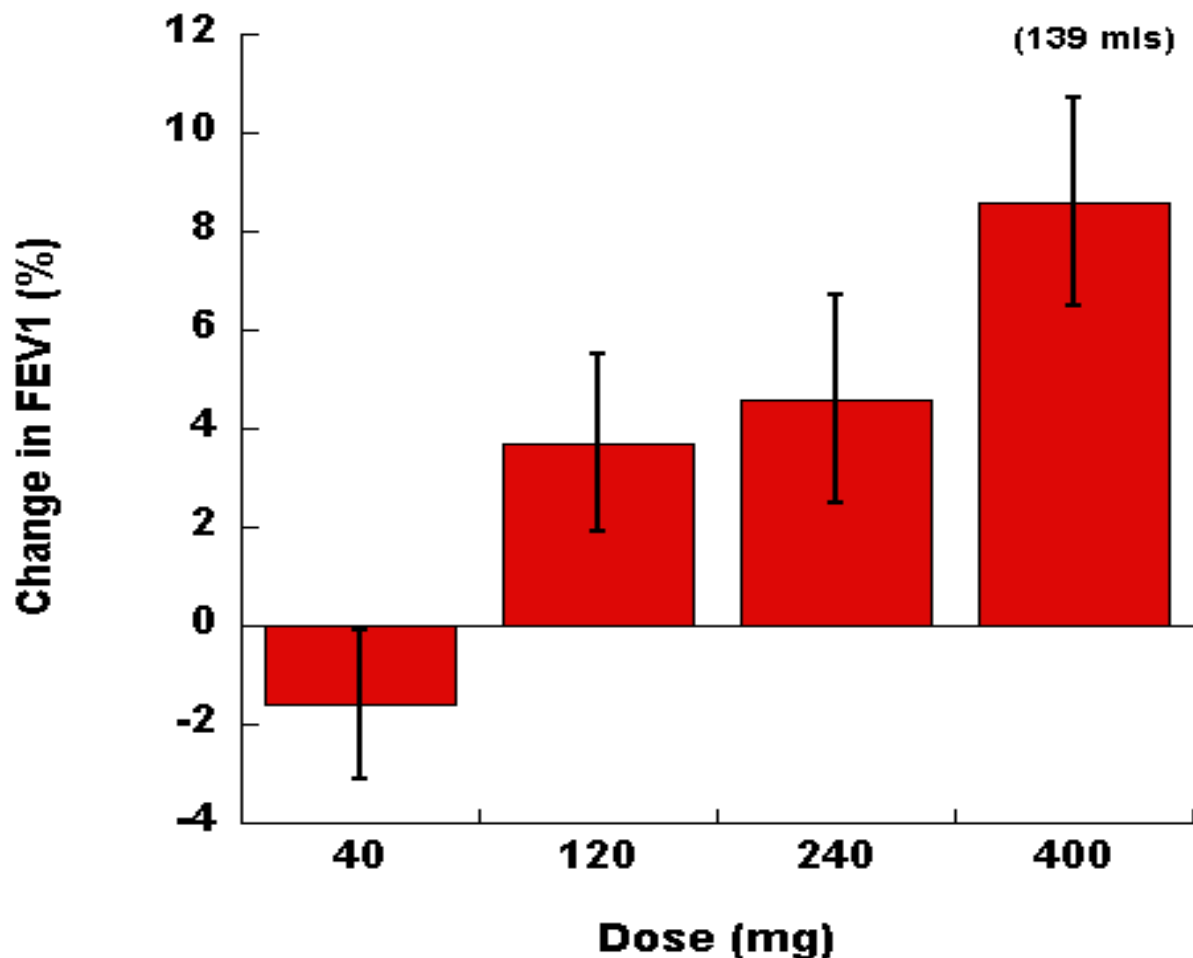


- Time requirements and adherence to therapy are pervasive challenges
  - "the treatments take time. Although the payback is longevity and QOL, at the moment the treatments can take up a large part of the day."*
  - "patients feel very pressed for time."*
  - "Because of the time requirement, you have to prioritise meds sometimes. Do the biggest bang for the commitment buck."*
  - "The time element is the key to adherence."*
  - "Therapy gets in the way of daily activities – 50 minutes two times a day!"*
- Treating resistance to antibiotics is another challenge for physicians

# Bronchitol – the first CF specific dry powder



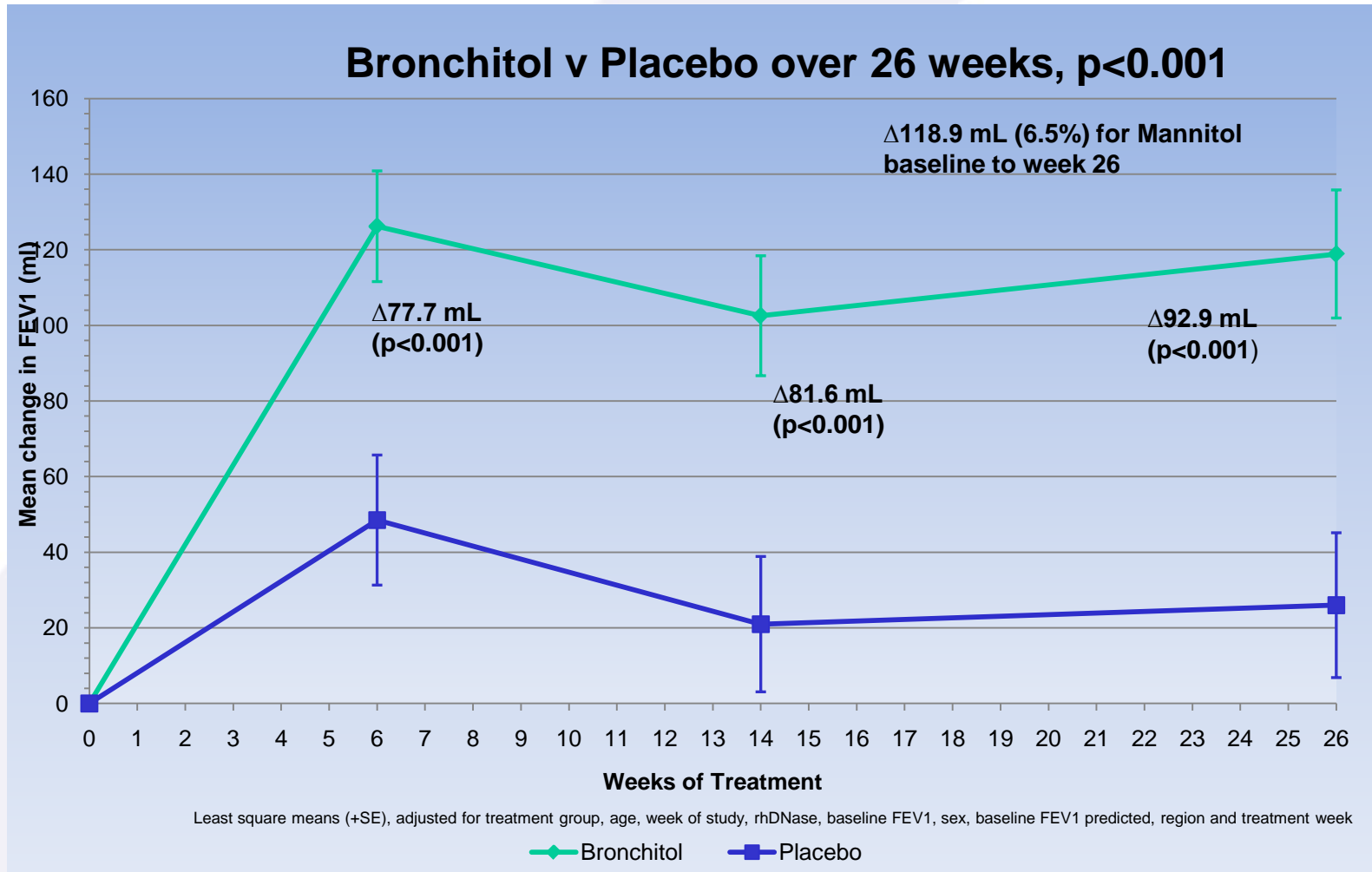
# CF-202 Dose Response 400 mg Selected



- 48 subjects
- Open label multidose study
- 400mg twice a day, then 40, 120, 240mg twice a day for 14 days in a random order
- Washout between doses



# CF-301 Absolute mean change (mL) in FEV<sub>1</sub>



# Bronchitol; TPP → Commercial reality in CF



## Bronchitol:

- An easy, quick, portable dry powder inhaled drug that won't interrupt cystic fibrosis patient's daily schedules.
- Suitable for all ages and stages of cystic fibrosis
- Acts quickly to help clear mucus, producing a lasting benefit to lung function, reducing exacerbations and improving quality of life.

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## Market access Milestones:

- Phase 3 delivers Target Product Profile – May 2009
  - Presentation of clinical trial results
    - Oral presentation at EU CF meeting
    - Oral presentation at Au CF meeting
    - Oral presentation at European Respiratory Society meeting
    - Oral presentation at North American CF meeting (October)
  - EMEA submission
    - Request for accelerated review submitted – September 2009
    - Marketing application submission – October 2009



# Bronchitol – cystic fibrosis registration

- **2<sup>nd</sup> Pivotal Phase III trial**

- Two pivotal trials required by the FDA
- Protocol review through Special Protocol Assessment (FDA)
- Double blind, placebo controlled
- 319 subject 6 years and older
- 400mg, twice per day for 6 months
- 1<sup>o</sup> endpoint - lung function by spirometry (FEV1)
- 2<sup>o</sup> endpoints – antibiotic use, exacerbations, lung function, QoL

- **Enrolment closed at 319 subjects**

**Sep 2009**

- **Headline data**

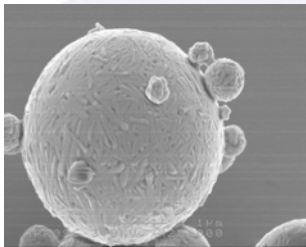
**H1 2010**

- **Orphan drug designation – U.S.**

- **Fast track designation – U.S.**



# 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

# Bronchitol – bronchiectasis registration (I)...

- 1<sup>st</sup> Pivotal Phase III trial



- 363 patient, placebo controlled, double blind, randomised 12 week treatment (twice per day) + 12 month open label extension

- 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
- antibiotic use reduction  $p < 0.05$  versus placebo
- adverse events (52 wks) cough 9%, sore throat 5%  
no SAE attributed to treatment



- First marketing application filed (Aus) in Sep 2008

# Bronchitol – bronchiectasis registration (II)....



- **2<sup>nd</sup> Pivotal Phase III trial**

- 350 patient, placebo controlled, double blind, randomised, 52 week treatment
- 400mg twice a day

- **Primary endpoint**

- Reduction in number of exacerbations
- Quality of life



- **Secondary endpoints**

- Exercise, mucus clearance, antibiotic use

- **Status**

- Special Protocol Assessment concluded with FDA
- Orphan Drug designation USA
- Commencement October 2009
- Data 2011



# Aridol™

- Identifies airway reactivity (active airway **inflammation**) which helps physicians in the diagnosis and management of **asthma**
- An **easy-to-use test kit** provides rapid results and doesn't require specialized equipment



# International regulatory status - Aridol

- **Australia**

- First market to launch
- 50% penetration in 2 years

June 2006

- **Europe**

- Approved European Union (MRP)
- Regional marketing partners appointed
- Launches underway

May 2007

- **South East Asia**

- Approved for marketing – Korea
  - Pricing approval completed mid-2009

Jan 2008

- **USA**

- NDA submitted. Complete response expected

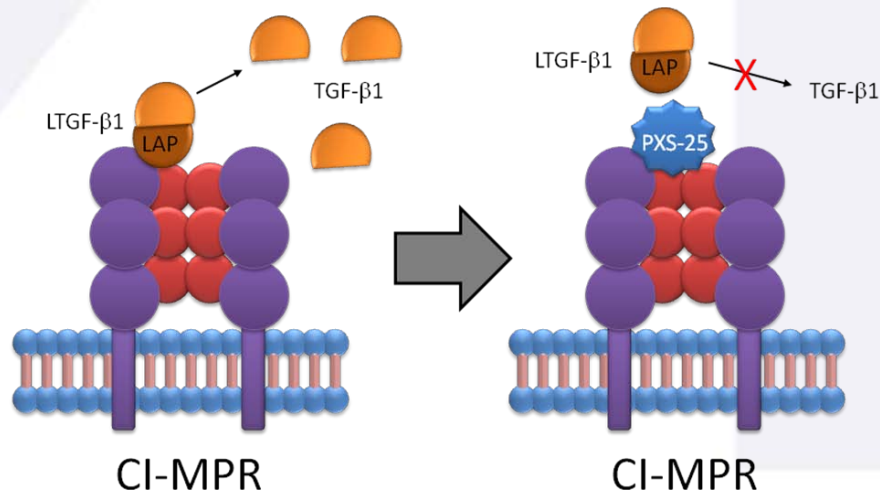
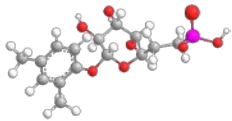
Dec 2009





# R&D - Status (PXS-25)

- Inhibits cleavage of latent TGF $\beta$  to active TGF $\beta$ 
  - anti-fibrotic agent with anti-inflammatory properties
  - small molecule with robust pharmaceutical profile
  - clinical target is pulmonary fibrosis
    - *500,000 cases and no approved drugs*
- Phase 1 trial commenced October 2009
  - data due end 2009



# Manufacturing Capacity

- Current GMP facility

- Manufactures Aridol for sale in EU, Asia & Australia
- Manufacture Bronchitol for clinical trials

- New facility

- Relocated May 2009
- Equipment installation & validation complete - Q3 2009
- Complete process validation – Q2 2010
- Capacity
  - Initial capacity - 1 spray drier: 40,000 patients p.a.
  - Expanded capacity – 2nd spray drier: 80,000 patients p.a.



# Financial Statements

Income Statement Data	Three months ended	
	30-Sep-09	30-Sep-08
	A\$	A\$
Revenue from sale of goods	183	106
Cost of sales	(47)	(29)
Gross profit	136	77
Interest	952	2,076
Other income	88	3
Expenses		
Research & development	(8,111)	(5,960)
Commercial	(1,251)	(1,371)
Administration	(1,721)	(1,283)
Finance expenses	(286)	-
Total expenses	(11,369)	(8,614)
Loss before income tax	(10,193)	(6,458)
Income tax expense	(11)	(6)
Loss for the period	(10,204)	(6,464)
Basic and diluted earnings (loss) per share - \$	(0.047)	(0.033)
Depreciation & amortisation	505	252
Fair value of options issued under employee plan	604	611

# Financial Statements

<b>Balance Sheet Data</b>	<b>As at</b>	
	<b>30-Sep-09</b>	<b>30-Jun-09</b>
	A\$	A\$
Cash and cash equivalents	113,438	124,993
Property, plant & equipment	32,559	32,698
Intangible assets	1,189	1,193
Total assets	151,822	163,997
Total liabilities	(23,508)	(26,306)
Net assets	128,314	137,691
<b>Cash Flow Data</b>	<b>Three months ended</b>	
	<b>30-Sep-09</b>	<b>30-Sep-08</b>
	A\$	A\$
Cash flows from operating activities	(10,025)	(4,877)
Cash flows from investing activities	(1,276)	(1,430)
Cash flows from financing activities	(189)	11
Net increase (decrease) in cash held	(11,537)	(6,296)

**END**

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