Therapeutic products for respiratory diseases

pharmaxis

October 2011

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. There can be no assurance that any forward looking statement will result or be achieved or accomplished. 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.

Company Overview

Objective	The development of products for respiratory diseases	
Lead products	Aridol:	•assessment of asthma and COPD •approved & launched in major markets
	Bronchitol for cystic fibrosis:	•approved in Australia •recommended for approval in EU •US NDA in preparation
	Bronchitol for bronchiectasis:	•Phase III trial in progress
	ASM8 for asthma:	•Phase II trial in progress
Discovery	PXS25 (M6P receptor blocker); SSAO/VAP1 inhibitor	
Listing	ASX (Nov 2003): PXS	
Locations	Sydney, Australia • Philadelphia, USA • London, UK	
Facility	Manufacture of Aridol & Bronchitol	
Employees	129 (30/9/11)	
Cash	A$34 million (30/9/11)	
Shares & Options	Shares outstanding: 229m; Options outstanding: 12m (30/9/11)	
Analyst coverage	SHAW STOCKBROKING; Merrill Lynch A subsidiary of Bank of America Corporation; CREDIT SUISSE; RBS Morgans; BBY; Wilson HTM INVESTMENT GROUP	

bronchitol™

MANNITOL

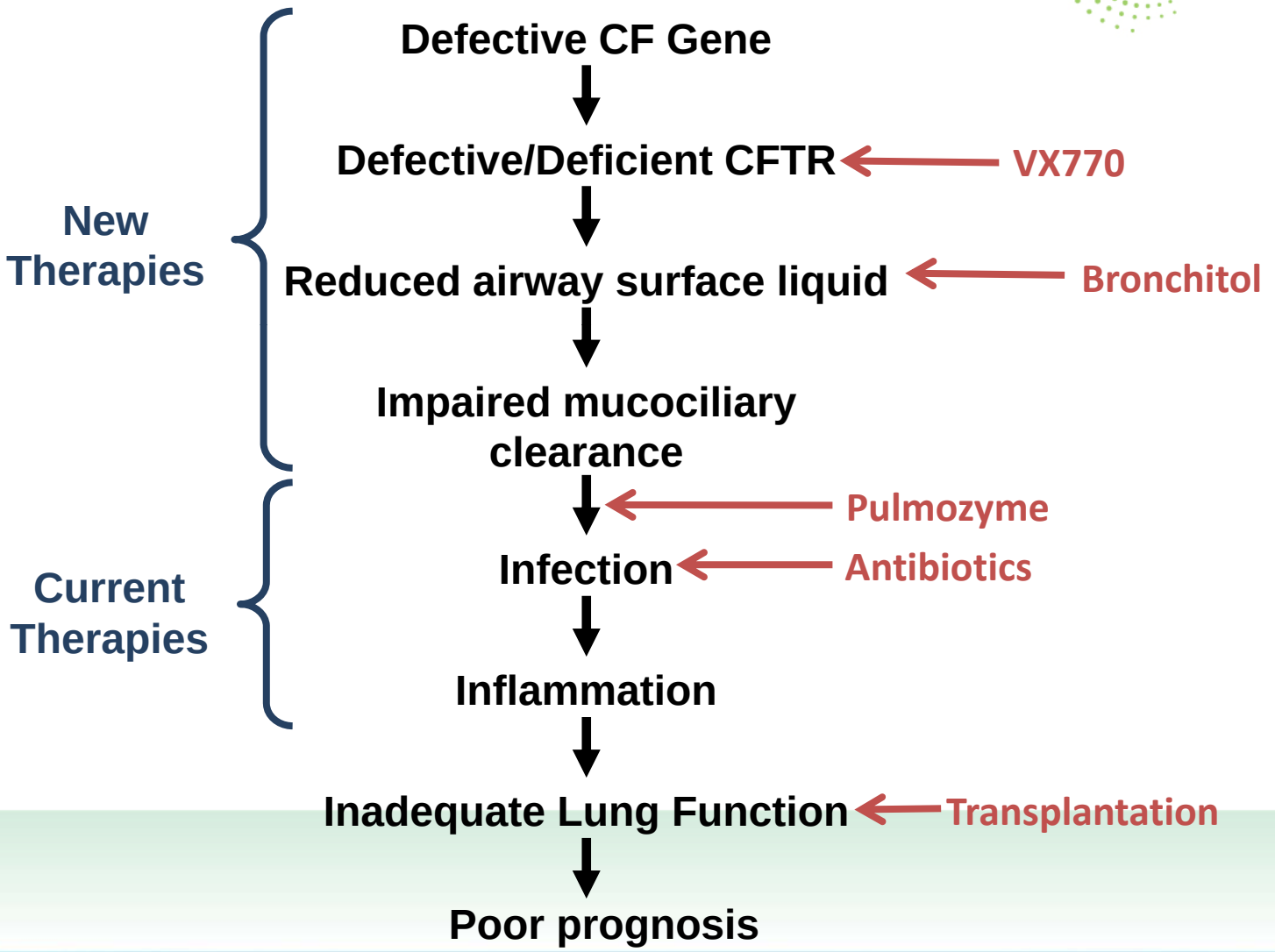
pharmaxis

innovating for life

Cystic Fibrosis

- **Background**
  - Genetic disorder affecting 48,000 in EU and 30,000 in US
  - Poorly hydrated, tenacious, thick mucus
  - Average life expectancy at birth today in the US is 37 years
- **Current Therapies**
  - Mostly delivered by nebulizer (preparation, sterilization)
  - rhDNase (Pulmozyme®): global sales ~US$540m (2010)
  - Tobramycin (Tobi®): global sales ~US$300m (2009)
  - Aztreonam (Cayston®): approved EU: 9/09; US: 02/10

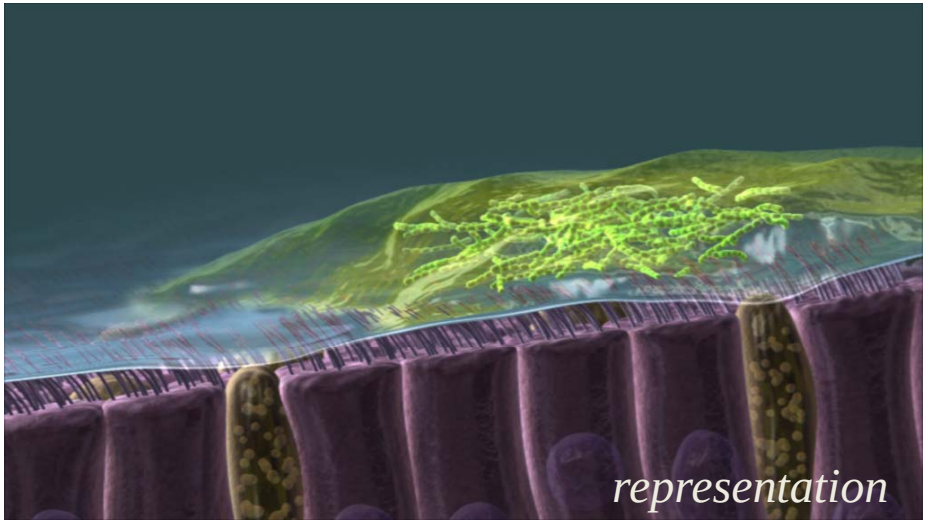
Progression of CF Lung Disease



Clearance of thick mucus

bronchitol™ MANNITOL

Before treatment

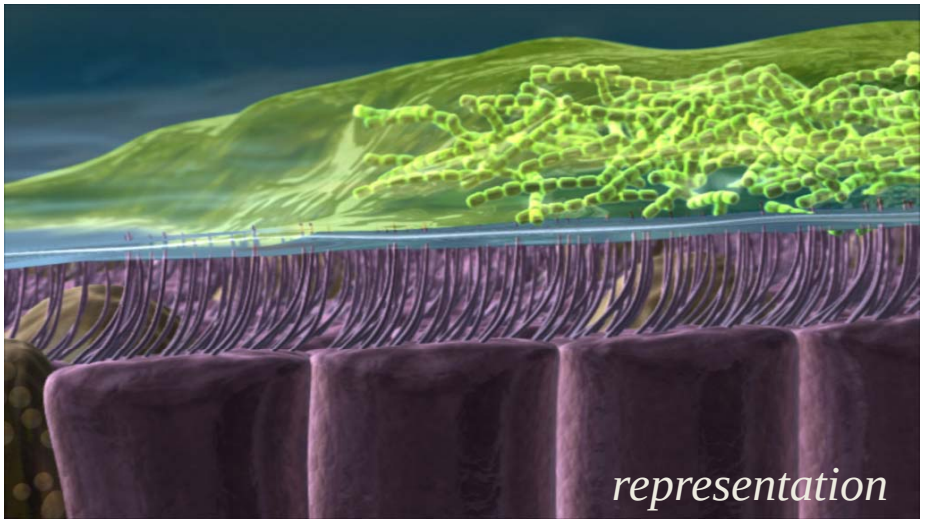


Lung surface dehydrated

Airway surface fluid layer impaired

Lung defense and hygiene compromised

After Bronchitol administration



Lung hydrated

Airway surface liquid restored

Improved lung clearance

Bronchitol

- Portable
  - No nebuliser
  - No refrigeration
- 3-5 minute treatment
  - Minimal set-up
  - Minimal cleaning
  - Minimal maintenance
- Hygienic
  - Weekly disposable device, no sterilisation
- Discreet
  - Inhaler is convenient and unobtrusive
  - No power source

Cystic Fibrosis clinical program

Two Pivotal Phase III trials – same design

- Multicentre, double blind, controlled
- Approx 300 subjects greater than 6 years old per trial
- 6 month treatment, 400mg twice per day followed by 6 month open label
- Primary endpoint:
  - lung function (FEV_1)
- Secondary endpoints:
  - Other lung function measures
  - Mucus clearance
  - Exacerbations
  - Antibiotic use
  - QOL and safety
- CF301: 40 centres in UK, Ireland, Australia & New Zealand
- CF302: 53 centres in US, Canada, Argentina, Germany, France, Belgium, & Netherlands
- Subjects remain on existing best standard of care

Improvements in lung function

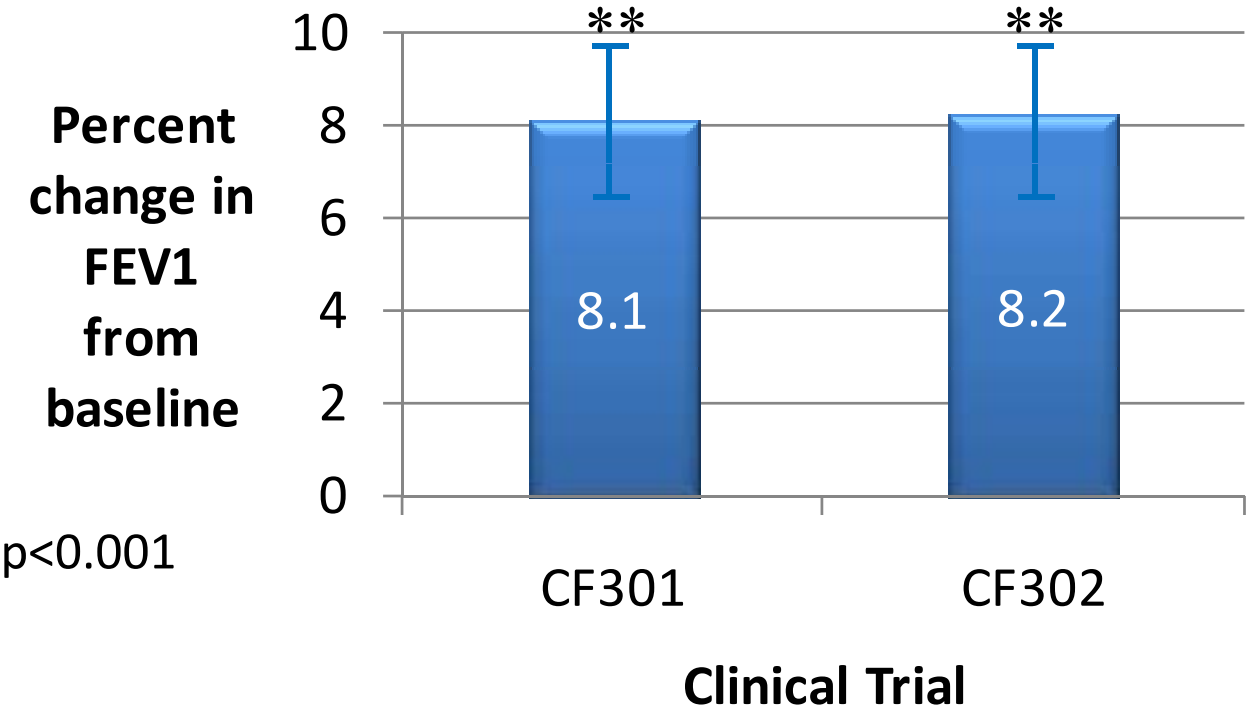
Change in lung function after 6 months Bronchitol treatment

	Results of Clinical Trials CF301 and CF302 - Combined				
	Paediatric (6-11yrs)	Adolescents (12-17 yrs)	Adults (≥18 yrs)	Overall	
	FEV_1	FEV_1	FEV_1	FEV_1	p value
% difference versus control (mL)	4.16	1.25	4.88	3.80	<0.001
% change from baseline (mL)	13.19	8.41	4.68	7.32	<0.001

Overall treatment response (FEV_1) over 26-weeks – all age groups (Pooled Data)

Sustained treatment effect

Change in lung function after 12 months Bronchitol treatment



** denotes p<0.001

CF301 and CF302 – Double Blind for 6 months followed by Open Label for 6 months

Exacerbation incidence reduced

Percentage reduction in exacerbation incidence after 6 months Bronchitol treatment		
CF301	35%	p=0.045
CF302	20%	p>0.05
CF301 +CF302 combined	29%	p=0.039

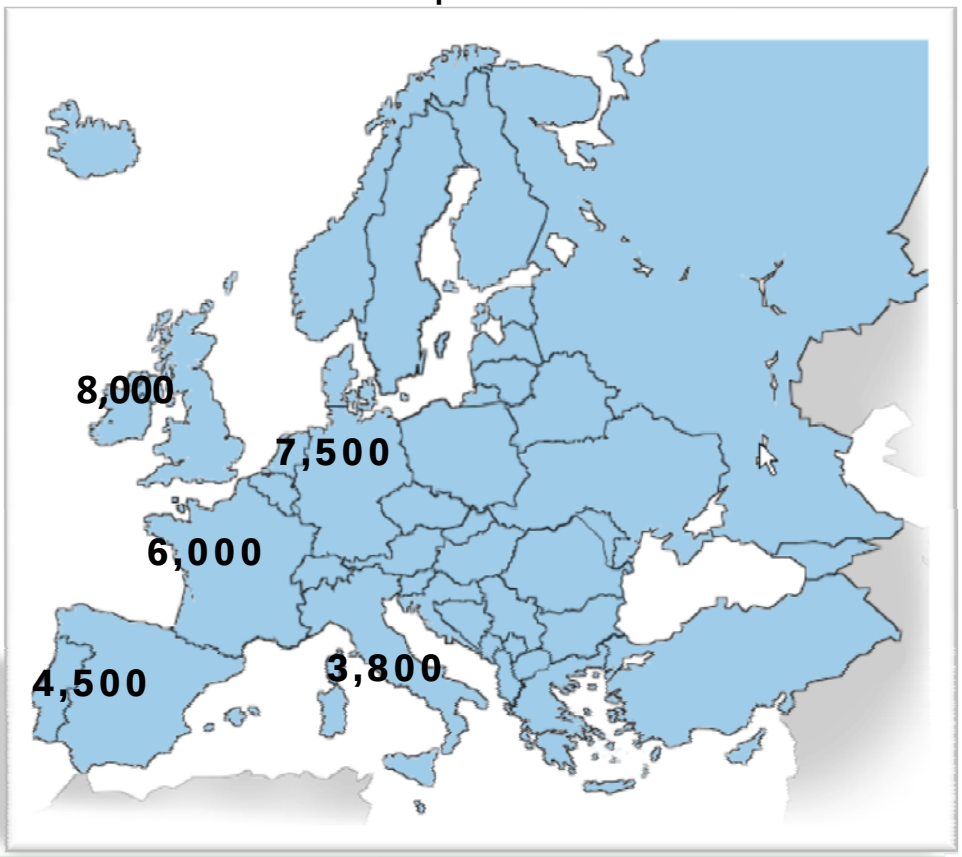
Commercialisation - Europe

- Orphan drug: 10-11 years market exclusivity
- Marketing approval status
  - Positive CHMP opinion received
  - Indicated for patients 18 years and older
  - Indicated as add on therapy to best standard of care
  - Anticipated European Commission approval – Jan 12
- Clinical trial to be conducted in patients under 18
  - Design to be finalised after scientific advice from EMA

Cystic Fibrosis - Europe

CF Patients	
Top 5 France, Germany, Italy, Spain, UK	30,000
Western EU – other	10,000
Central / Eastern EU	8,000
Total EU	**48,000**

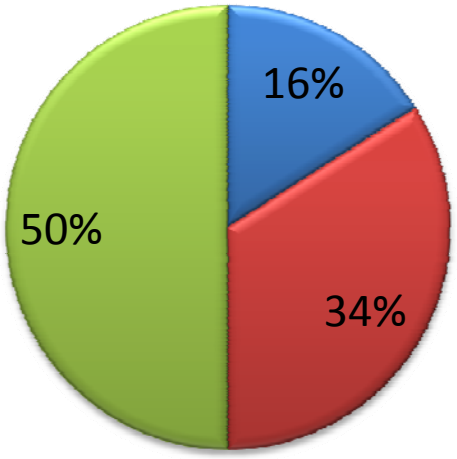
CF Patients in Top 5 EU Countries



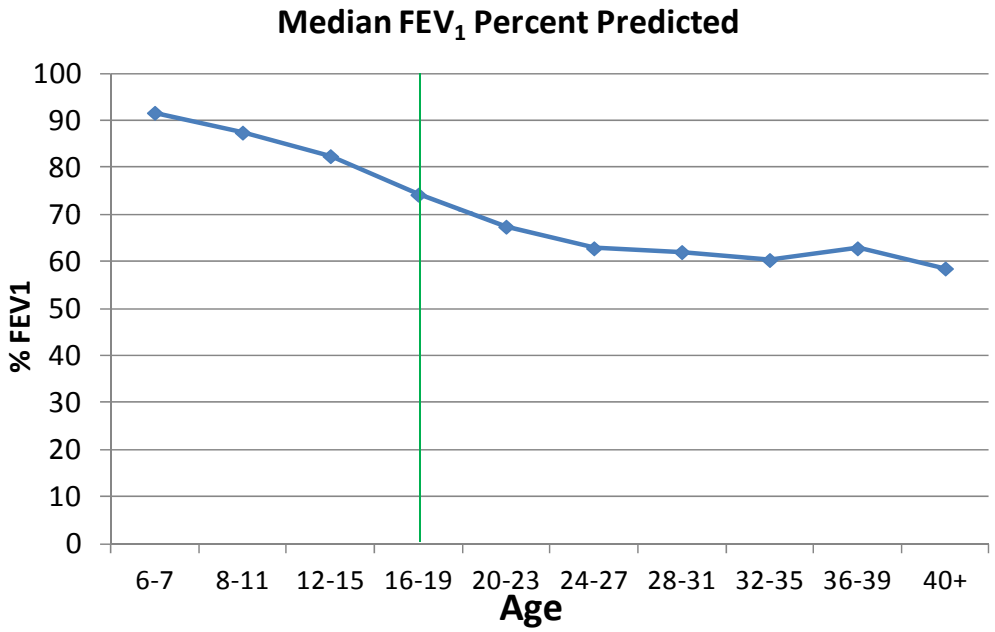
Adult CF population represents major clinical need

bronchitol™ MANNITOL

CF patients in EU by age



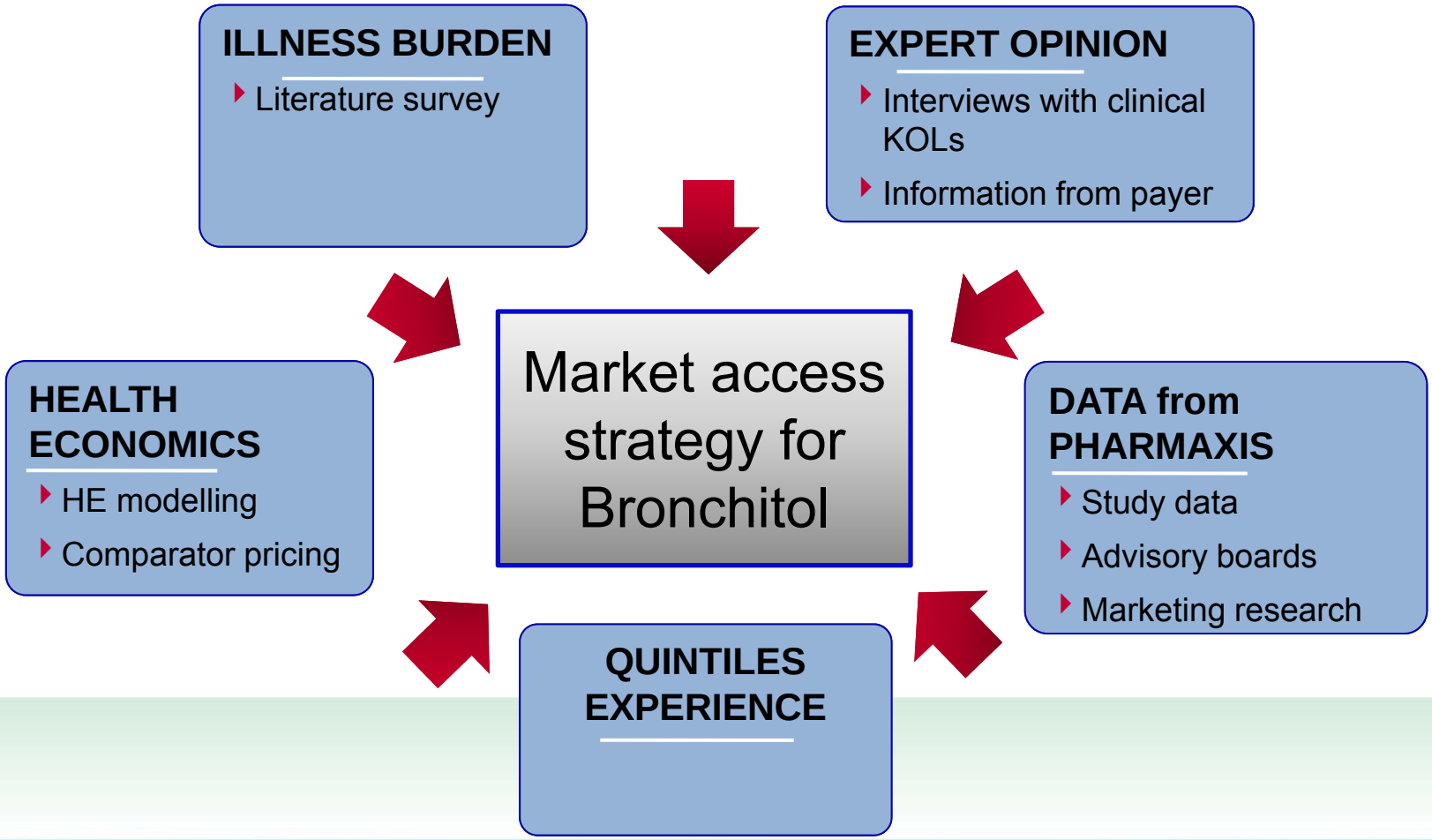
Under 6 6 to 17 Over 18



Source: UK CF Registry

Pricing and reimbursement

Market access strategy for Bronchitol based on multiple information sources:

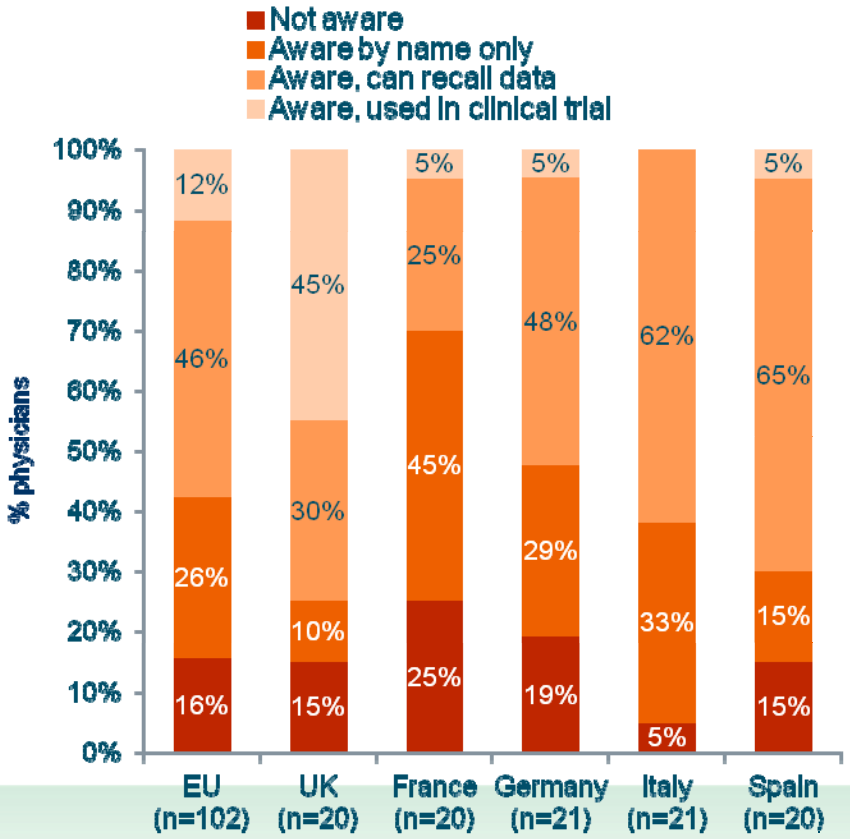


Targeted launch sequence

	Country	Reimbursement process	Targeted launch date (CY)
TOP 5	United Kingdom	Automatic reimbursement but significant national pricing guidance through NICE.	Q2 2012
	Germany	Reimbursement granted with Marketing Authorisation	Q2 2012
	France	Centralised & robust	Q4 2012
	Italy	Centralised with regional implementation	Q1 2013
	Spain	Centralised with regional implementation	Q3 2013
	Rest of Western Europe	Country dependent	Q3 2012 – Q3 2013
	Central & Eastern Europe	Country dependent	From Q3 2013 onwards

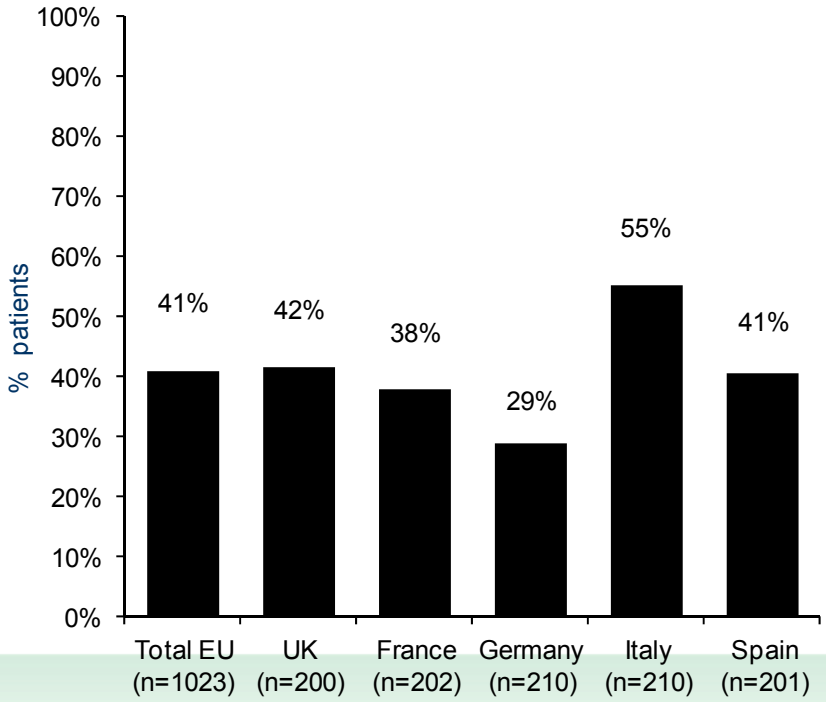
Existing physician awareness survey conducted H1 2010

Survey of Bronchitol awareness by country



Q6: Please consider the list of emerging CF-specific treatments below. For each treatment, please indicate the statement that best reflects your level of awareness.

Indicative prescription of Bronchitol by country



P49: If the following emerging therapies for cystic fibrosis (including Bronchitol) were currently available on the market, which, if any, would you consider prescribing to this patient?

Engagement with EU CF centres and clinicians

	Total	Other EU	Top 5	UK	Germany	Spain	Italy	France
Number of CF centres			294	48	110	35	34	67
Centres participating in clinical trials	46	6	40	24	4	-	-	12
Centres supporting consensus statement sent to CHMP	45	13	32	6	6	1	5	14
KoL's signed Consensus Statement	66	22	44	12	7	1	9	15
Clinicians attended international PXS symposia	344	169	175	110	32	9	11	13
PXS Advisory Boards with KoL's		X		X	X			
Contacts with patient organisations		X		X	X			

Other activities

- Pharmaco-economic models completed
- Reimbursement dossiers completed for NICE and French authorities.
- Exemption from new German process obtained
- Sales team development/recruitment underway in UK and Germany

Direct sales resources required

Country		Sales Force Personnel	Centres/Sales Person
TOP 5	United Kingdom	6	8
	Germany	5	22
	France	5	13
	Italy	4	9
	Spain	3	12
	Rest of Western Europe	7	N/A
	Central & Eastern Europe	Distributor strategy	Distributor strategy

European infrastructure

- UK – Pharmaxis UK subsidiary and sales force in place (currently promoting Aridol)
- Remainder Western Europe (13 countries) – Quintiles:
  - Recruit and manage dedicated Pharmaxis sales force
  - Local market knowledge to speed access
  - Full back office support
  - Satellite model leveraging PXS Top 5 country management infrastructure
- European CF market support – Pharmaxis UK subsidiary
  - Marketing
  - Pricing
  - Medical information, regulatory and pharmacovigilance
- Build to total ~40 people in EU for CF
- Logistics infrastructure by 3rd party

US Cystic Fibrosis opportunity

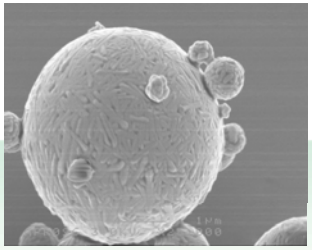
Regulatory

- NDA scheduled submission H1 2012
- Earliest FDA review completed H1 2013
- Orphan drug provides 7 years market exclusivity

Marketing

- 150 CF centres anticipated to require 15 - 25 person field force
- 30,000 people in the US with CF
- Pulmozyme price ~US$22,000 pa

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

Indication	Bronchiectasis (non Cystic Fibrosis)
Target Product Profile	- Effective clearance of mucus - Reduction in exacerbation incidence
Market Size	Affects approximately 600,000 people worldwide
Competitors	Antibiotics & CF drugs; lack of targeted clinical development in this disease state
Status	Phase III trial recruitment ongoing
Next Milestone	Close of recruitment in Phase III trial – Q4 2011 - 474 subjects, 89 sites in US, Europe, South America & Australia

Aridol®

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

- An **easy-to-use test kit** provides rapid results and doesn't require specialized equipment

aridol™

Aridol – commercialisation status

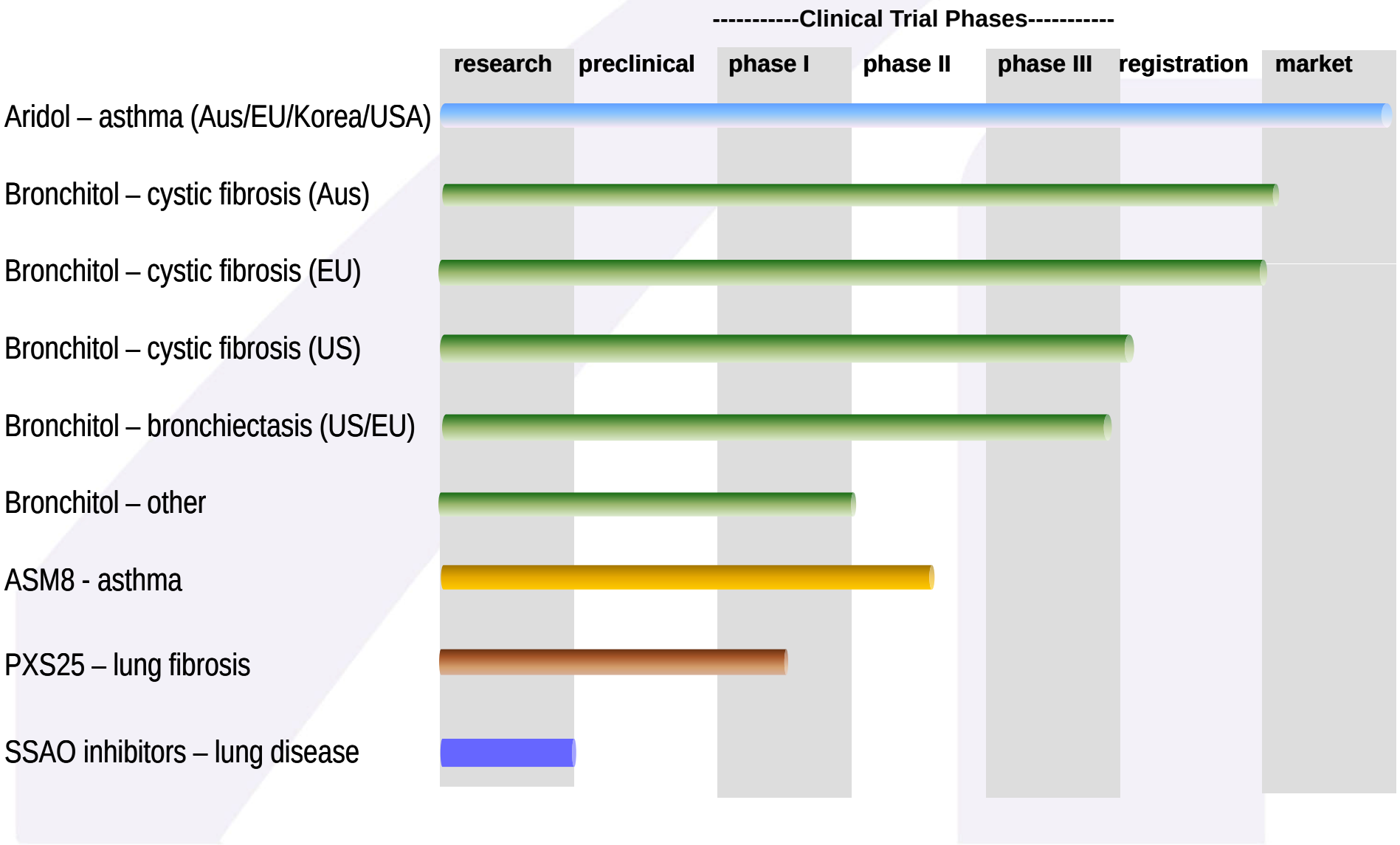
			2008	2009	2010	2011
Sales (A$'000)						
Australia	Launched 2006	Direct	216	232	268	254
Europe	Staggered launch from 2007	Distributors (7); UK – direct;	137	267	398	400
Korea	Launched Oct 2009	Distributor	-	32	162	203
Clinical trials		Direct	174	64	-	-
US	Launched Feb 2011	Direct	-	-	-	53
			527	595	828	910

Potential growth

- US and Korea
- Asthma management
- COPD

aridol™

Development Pipeline



ASM8 – Asthma

Indication	Moderate to severe asthma for patients who do not respond to inhaled steroids or cannot tolerate high doses
Target Product Profile	- Greater efficacy through multi-targeting - Better tolerability & convenience compared with current treatments - Once daily nebulisation
Market Size	Affects approx 12 million people worldwide
Competitors	Singulair (US$4.5B sales, 2008), Xolair (US$730M sales, 2008)
Status	Phase II trial ongoing
Next Milestone	Phase II trial results anticipated – Q1 2012

PXS25 - IPF

Indication	Idiopathic Pulmonary Fibrosis (IPF)
Target Product Profile	- Inhibition of fibrosis and inflammation to lung tissue - Local administration to the lung - Safe & well tolerated in humans
Market Size	Affects approx 90,000 people in the USA
Competitors	Pirfenidone (just launched in EU), immunosuppressives & steroids
Status	Phase I trial complete
Next Milestone	Phase II trial planning – Q1 2012

SSAO – Lung inflammation

Indication	Treatment of inflammatory disease, such as asthma
Target Product Profile	- Target eosinophilic and neutrophilic asthma - Once daily oral dosing
Market Size	Affects approx 20 million people worldwide
Competitors	All in pre-clinical development
Status	Pre-clinical development
Next Milestone	Lead development candidate – Q4 2011

Financial Statements

Financial Statement Data - Unaudited
(International Financial Reporting Standards)
('000 except per share data)

Income Statement Data	**Three months ended**	
	30-Sep-11	**30-Sep-10**
	A$	A$
Revenue from sale of goods	319	202
Cost of sales	(122)	(69)
Gross profit	197	133
Interest	450	937
Other income	74	175
Expenses		
Research & development	(7,221)	(8,768)
Commercial	(1,736)	(1,469)
Administration	(913)	(1,197)
Finance expenses	(210)	(290)
Total expenses	(10,080)	(11,724)
Loss before income tax	(9,359)	(10,479)
Income tax expense	-	(7)
Loss for the period	(9,359)	(10,486)
Basic and diluted earnings (loss) per share - $	(0.041)	(0.046)
Depreciation & amortisation	1,178	1,189
Fair value of securities issued under employee plans	243	440

Financial Statements

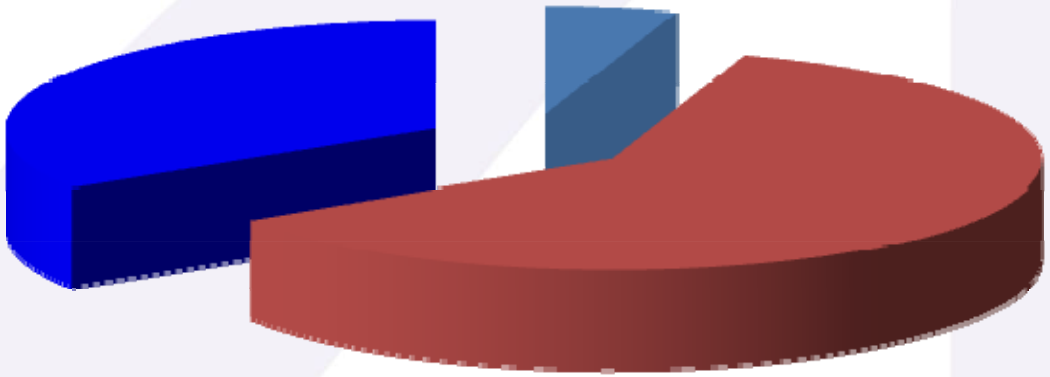
Balance Sheet Data	As at	
	30-Sep-11	30-Jun-11
	A$	A$
Cash and cash equivalents	33,730	44,343
Property, plant & equipment	29,757	30,570
Intangible assets	15,475	15,954
Total assets	82,955	94,572
Total liabilities	(21,046)	(23,742)
Net assets	61,908	70,830
Cash Flow Data	**Three months ended**	
	30-Sep-11	30-Sep-10
	A$	A$
Cash flows from operating activities	(10,657)	(8,795)
Cash flows from investing activities	84	(433)
Cash flows from financing activities	(120)	(288)
Impact of foreign exchange rate movements on cash	79	(440)
Net increase (decrease) in cash held	(10,614)	(9,956)
Share Data	**Ordinary Shares as at**	
	30-Sep-11	30-Jun-11
Ordinary shares on issue	229,116	225,765
Options over ordinary shares outstanding	11,989	13,297

Share Capital
(including options)

Management – 5%

Other/retail – 36%

Institutions – 59%
(62 institutions)



30 September 2011: 229m shares; 12m options

Summary

- Respiratory company with approved products and strong pipeline
- Aridol
  - approved in Australia, SEA, Europe and USA
- Bronchitol
  - approved in Australia for cystic fibrosis
    - Going through pricing / reimbursement
  - recommended for approval in Europe
    - First launch expected 1H 2012
  - USA marketing application to be filed 1H 2012
  - Clinical trials in progress to expand opportunity
- ASM8 for asthma
  - In Phase II

End