

pharmaxis

Therapeutic products for respiratory diseases






June 2010

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.

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Company Overview

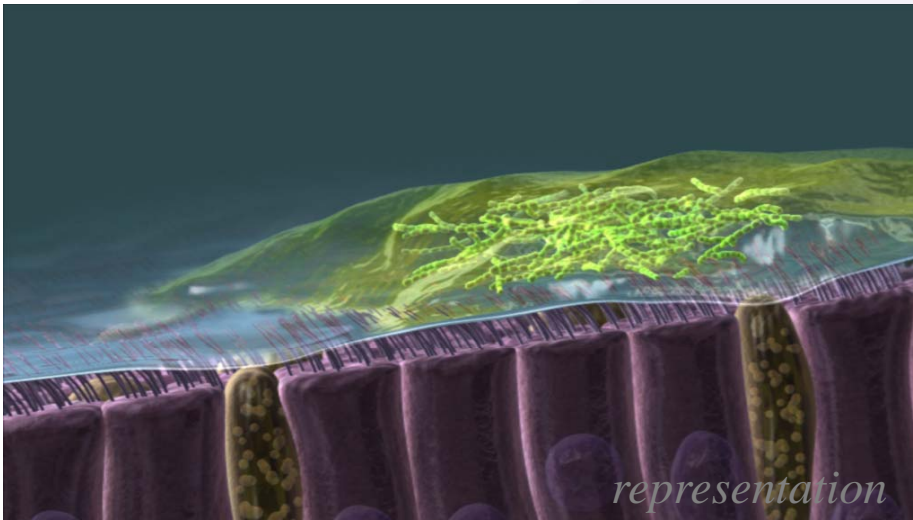
Objective	The development of products for respiratory and inflammatory diseases
Lead products	Aridol: management of asthma and COPD Bronchitol: therapeutic for cystic fibrosis and COPD ASM8: therapeutic for asthma
Discovery	PXS25 (M6P receptor blocker); PXS4206 (VAP1 inhibitor)
Listing	ASX (Nov 2003): PXS
Locations	Sydney, Australia • Exton, USA • Slough, UK • Montreal, Canada
Facility	GMP Manufacture of lead products
Employees	135
Cash (31/3/10)	A\$96 million
Shares & Options	Shares outstanding: 225m; Options outstanding: 13m
Key patents	Bronchitol & Aridol granted in USA, Australia, Asia, Canada, Japan; pending in EU, Japan.
Analyst coverage	    

Bronchitol for Cystic Fibrosis



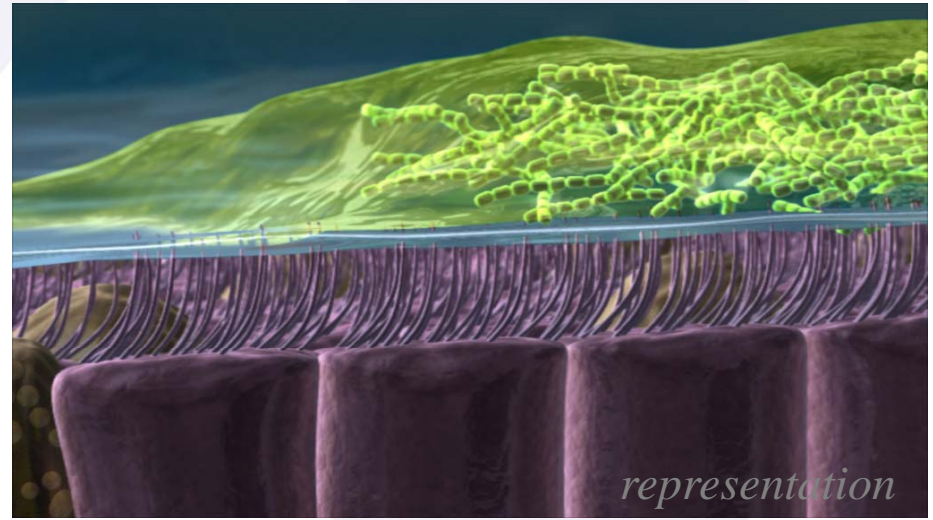
Osmotic clearance of abnormal mucus

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 – cystic fibrosis

- **Background**



- Genetic disorder affecting 75,000 worldwide (30,000 in US)
- Poorly hydrated, tenacious, thick mucus
- Current life expectancy is 37 years (US)



- **Current treatments: rhDNase and tobramycin**

- Delivered by nebulizer (preparation, sterilization)
- rhDNase (Pulmozyme®): global sales US\$460mm (2009)
- Tobramycin (Tobi®): global sales US\$233mm (2007)



Bronchitol – cystic fibrosis clinical program

2 Pivotal Phase III trials – same design



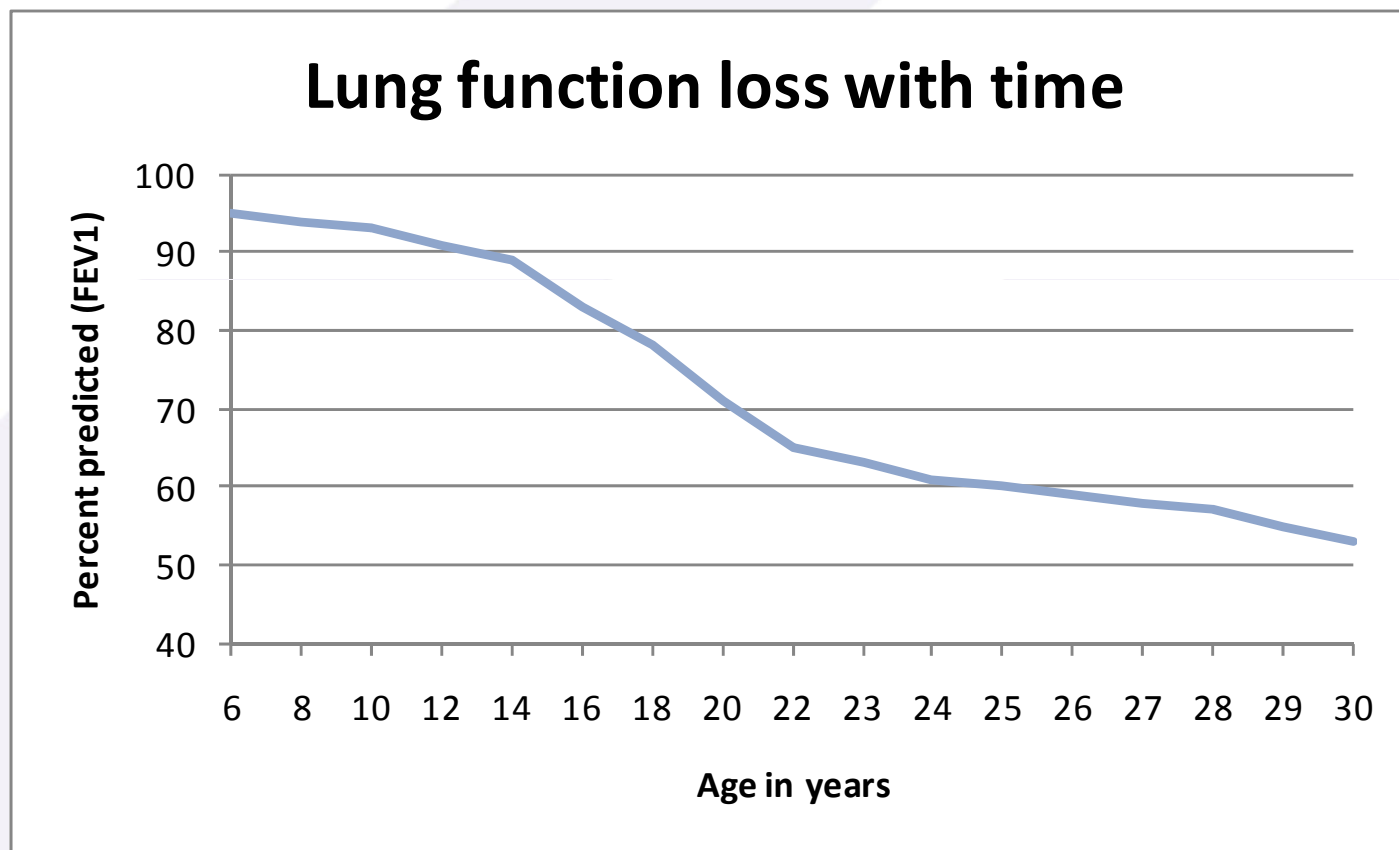
- Multicentre, double blind, placebo controlled
- Approx 300 subjects greater than 6 years old
- 6 month treatment, 400mg twice per day followed by 6 month open
- Primary endpoint:
 - lung function (FEV_1)
- Secondary endpoints:
 - Other Lung Function measures
 - Lung function (FEV_1) in patients on rhDNase
 - exacerbations
 - antibiotic use
 - QOL and safety

Cystic Fibrosis Trial Demographics



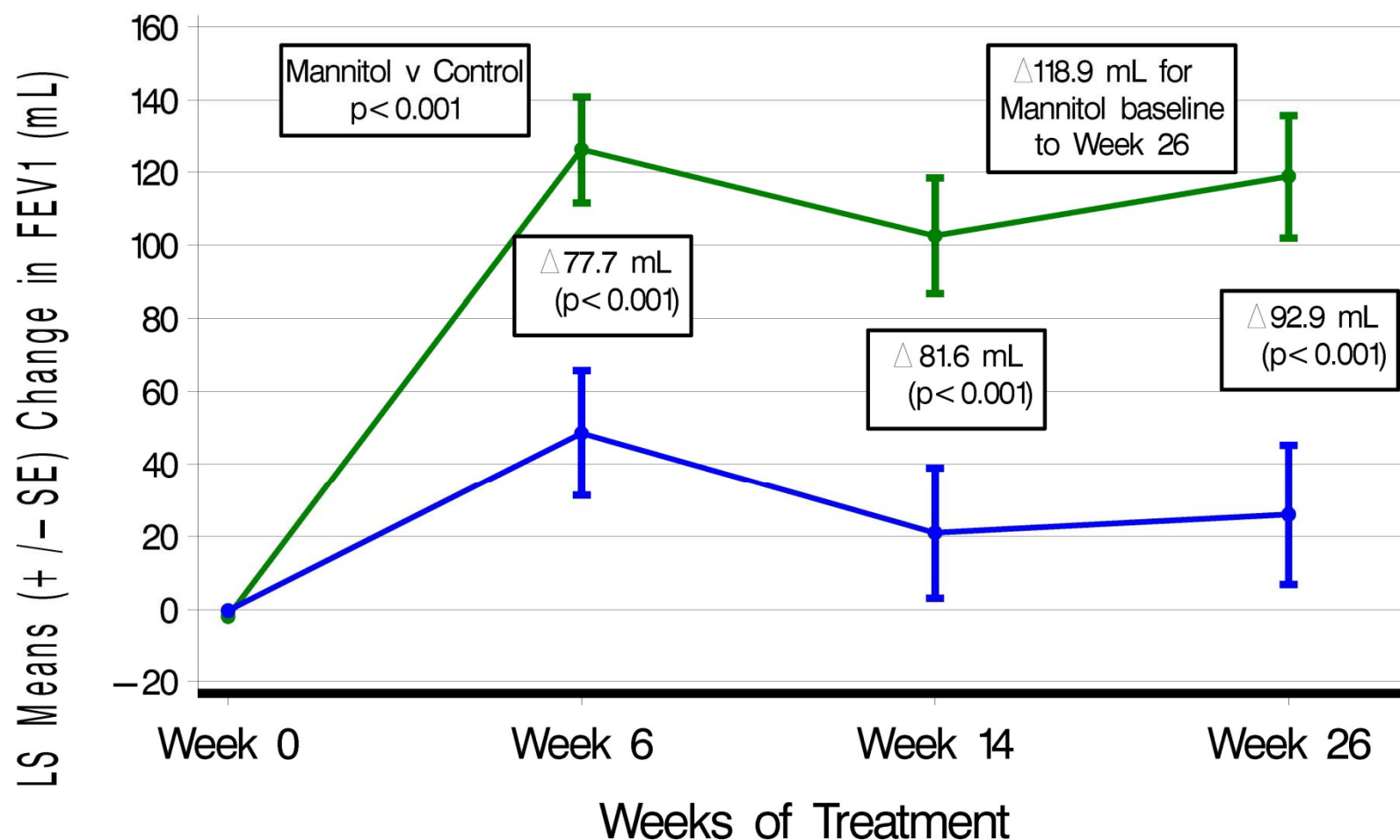
Criteria	CF301	CF302
Patients failing mannitol tolerance test	7%	7%
Patients randomised	324	318
Received treatment	295	305
Withdrawal rate	33%	14.8%
Average age	23	20
Age range	6 - 56	6 - 53
Mean predicted FEV ₁ on entry	62%	65%
Predicted FEV ₁ range	26% - 94%	34% - 96%
% patients on dornase alfa	55%	75.1%

Average lung function decline in CF patients



Source: Cystic Fibrosis Foundation Patient registry, 2004

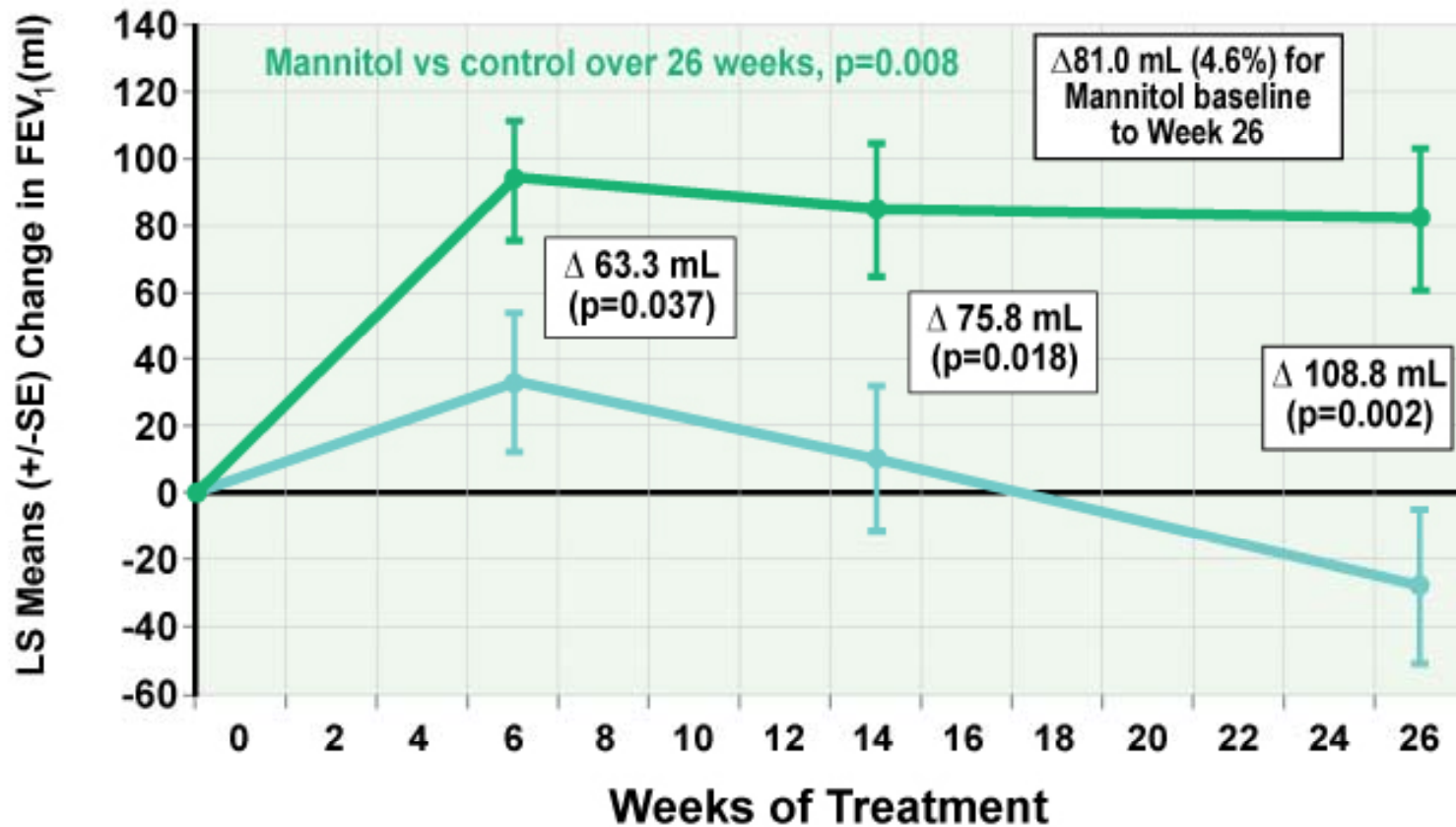
CF-301 Absolute mean change (mL) in FEV₁



Model: delta_FEV1=treatment group + RhDNase Use + Age + Week of Study + baseline FEV1 + Sex + Baseline FEV1 percent predicted+ region + treatment*week
Covariance Autoregressive (1)

Treatment Used ●—● Mannitol ●—● Control

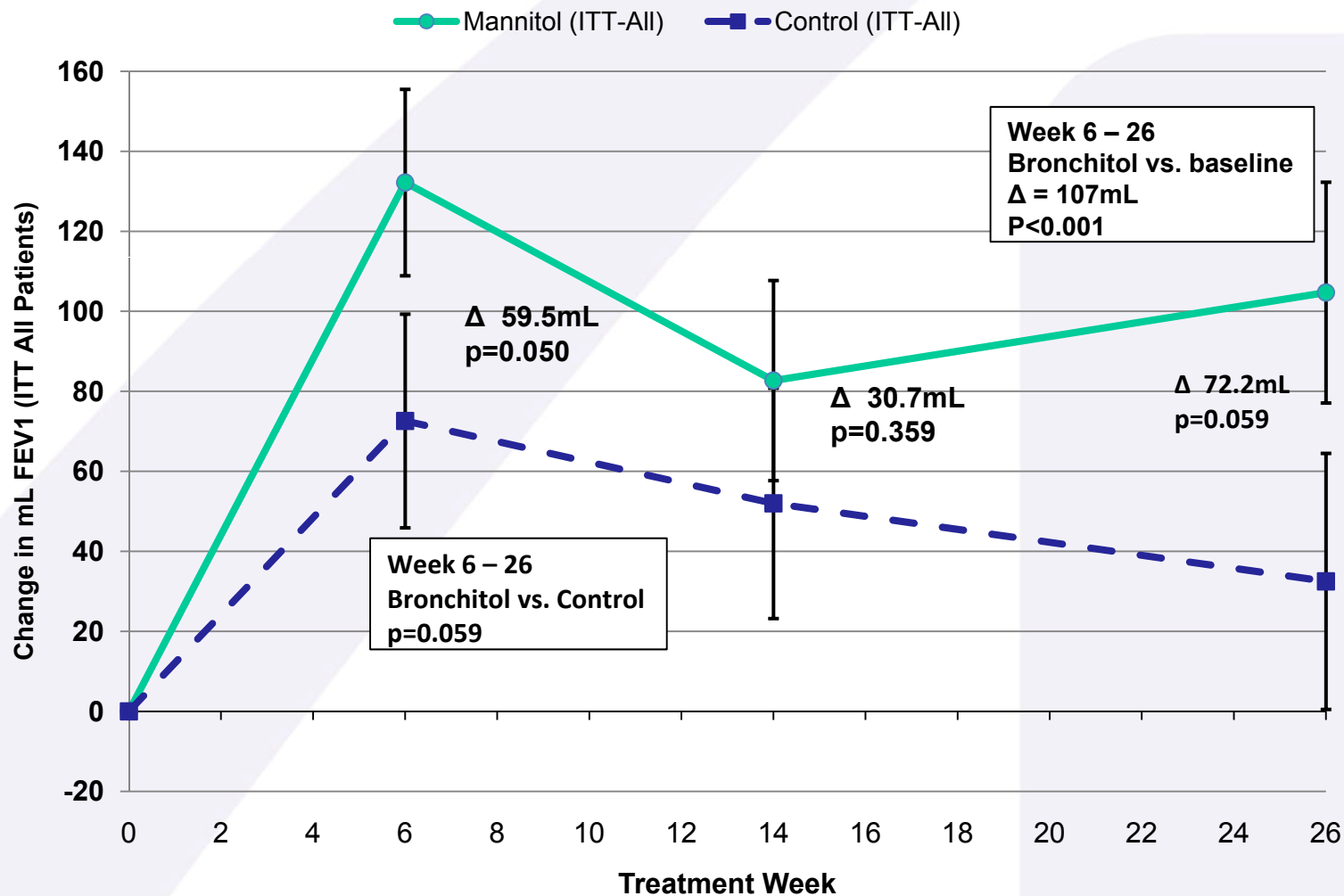
CF301 FEV1 mean change (mL) in rhDNase patients



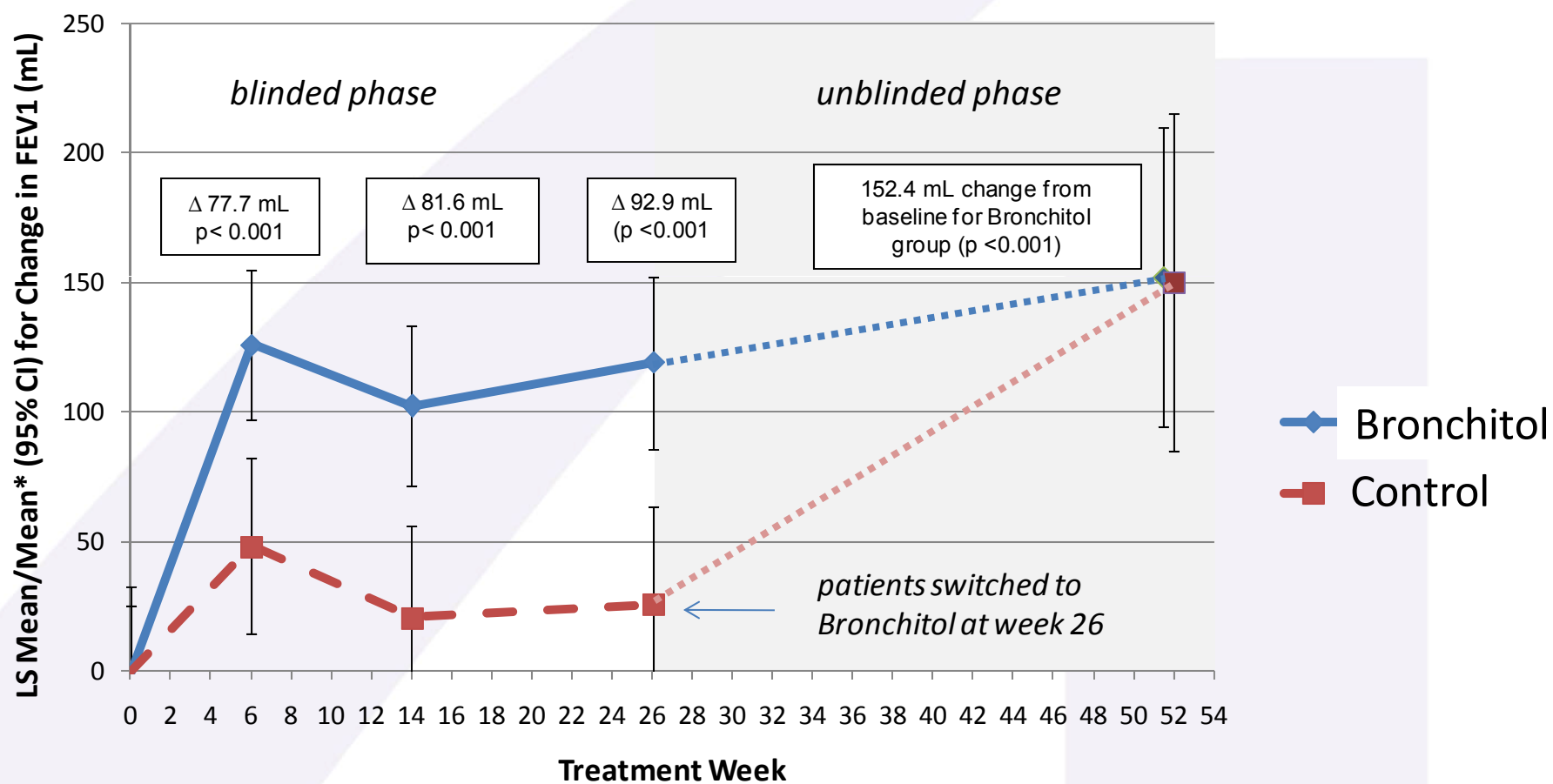
Model: $\Delta FEV_1 = \text{treatment group} + \text{RhDNase Use} + \text{Age} + \text{Week of Study} + \text{baseline FEV}_1 + \text{Sex} + \text{Baseline FEV}_1 \text{ percent predicted} + \text{region} + \text{RhDNase use "treatment" week}$
Covariance Autoregressive (1)

Treatment Used —●— Mannitol —●— Control

CF-302 Absolute mean change (mL) in FEV₁

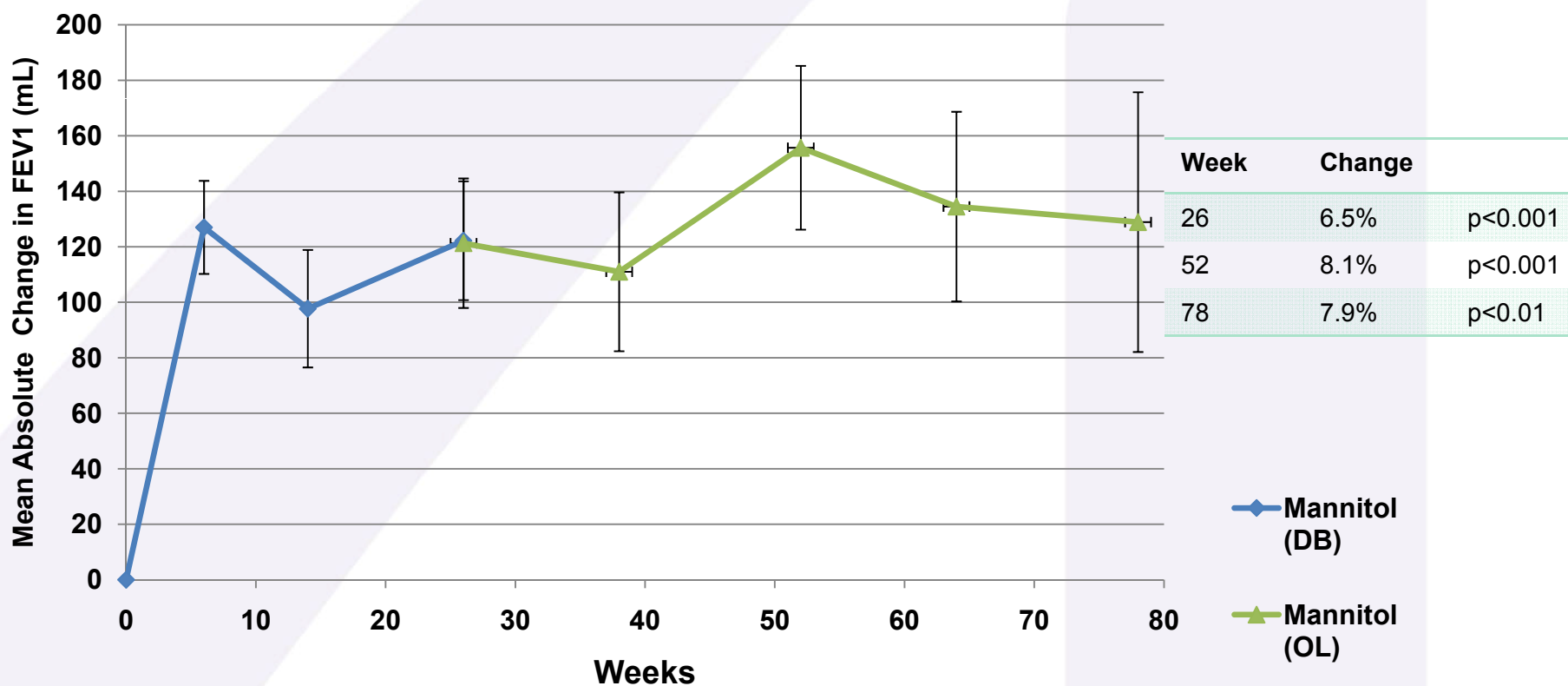


CF301 – lung function changes at 12 months



CF301 Bronchitol Arm (DB and OL for 18 months)

CF301 Change in FEV1 Summary Statistics for Bronchitol (DB patients only) over 18 months

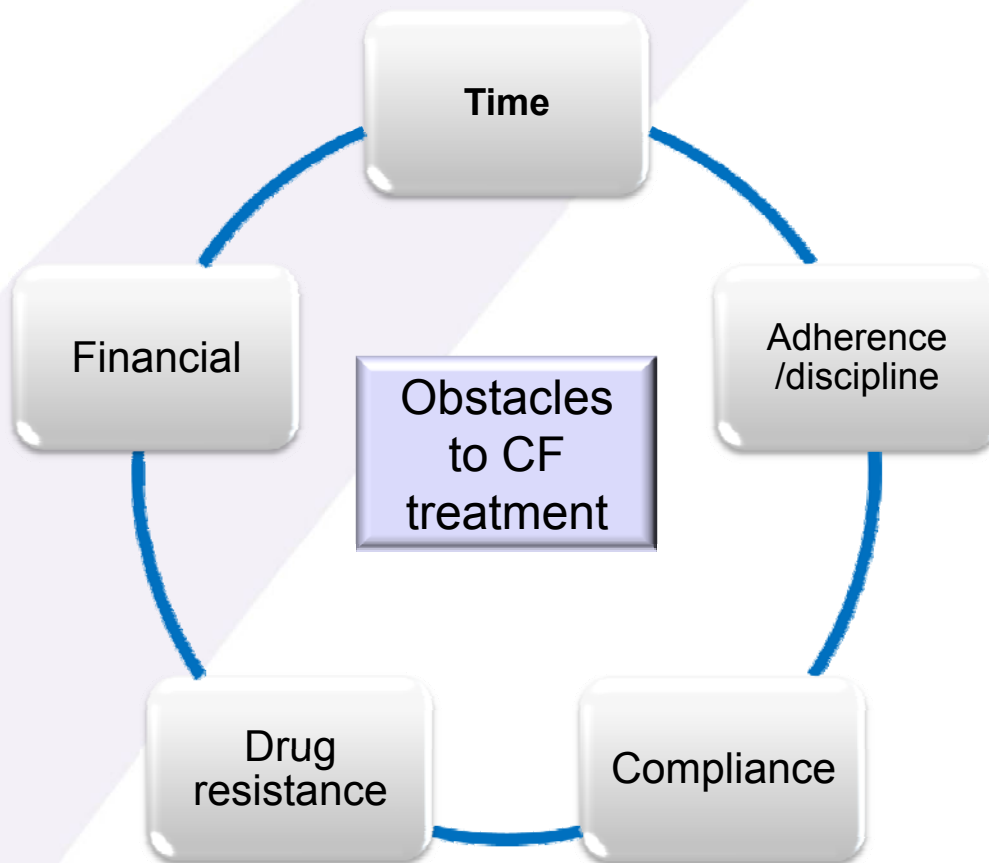


CF302 – Summary

- Primary endpoint of lung function (FEV_1) improvement compared to control measured in mLs - narrowly missed statistical significance ($p=0.059$)
- Lung function (FEV_1) improvement compared to control expressed as percentage - similar to CF301 and statistically significant ($p=0.029$)
- Lung function improvement of 8.2% over baseline exceeds improvement demonstrated in CF301
- Early and sustained lung function improvement over baseline as seen in CF301
- Secondary endpoints analysed to-date positive and consistent with CF301
- Safety – well-tolerated as in CF301, supportive of EU MAA
- Compelling body of evidence ready to discuss with the FDA

Cystic Fibrosis market research

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



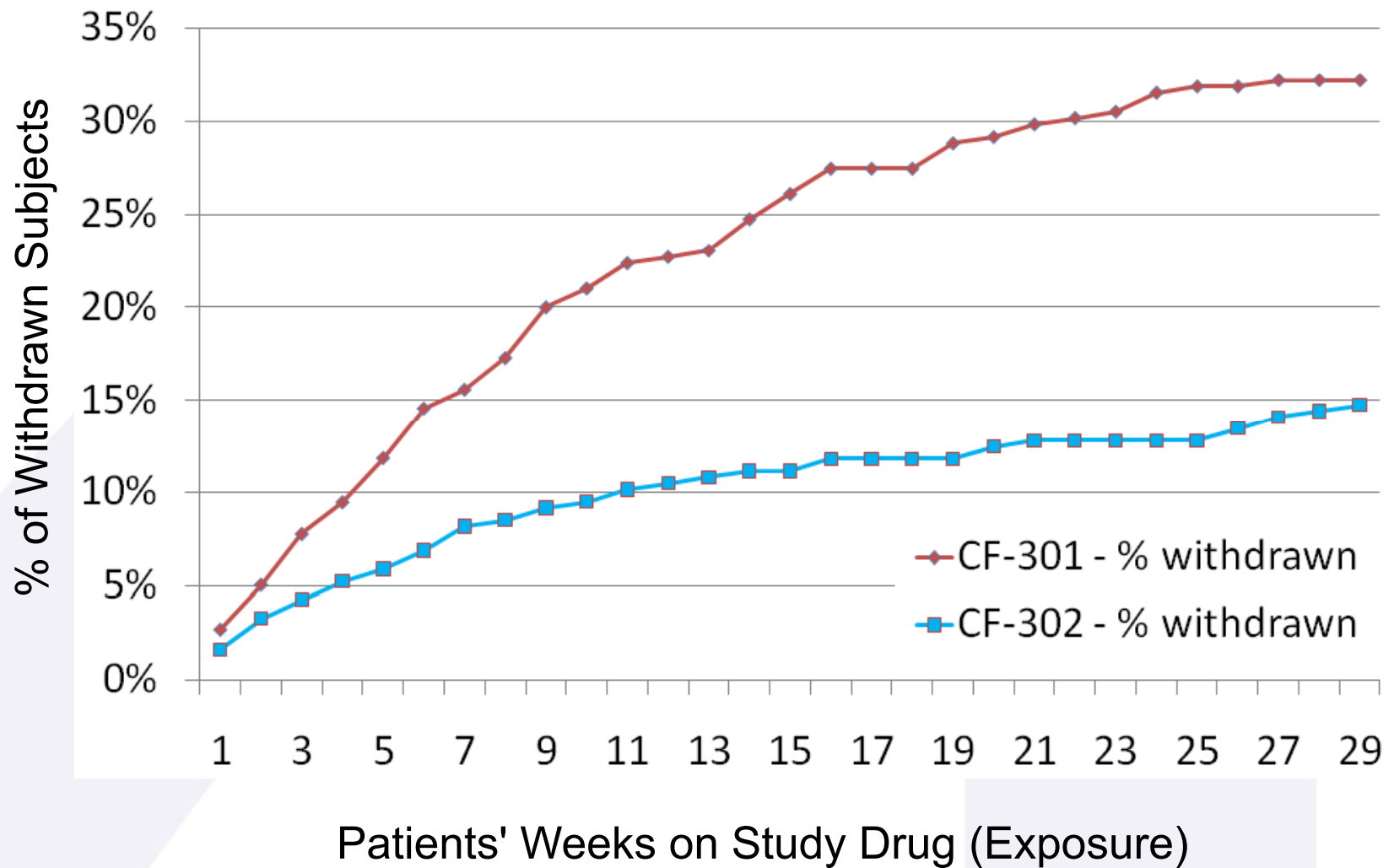
- Time requirements and adherence to therapy are pervasive challenges
 - "the treatments take time. Although the payback is longevity and QOL, at the moment the treatments can take up a large part of the day."
 - "patients feel very pressed for time."
 - "Because of the time requirement, you have to 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 Delivery

- **Treatment burden a higher concern to many families than reduced life expectancy**
- **Twice a day dosing**
- **2 - 5 minutes delivery time**
- **Convenient and portable**
- **Does not rely on a power source**
- **No cleaning / maintenance/ sterilisation required**



CF-301 vs. CF-302 withdrawals of dosed subjects during blinded phase



Bronchitol – cystic fibrosis registration

Europe



- Orphan drug designation
- Headline data
- EMA submission
- EMA response anticipated

May 2009

Oct 2009

Q4 2010



USA

- Orphan drug designation
- Headline data
- FDA pre NDA meeting

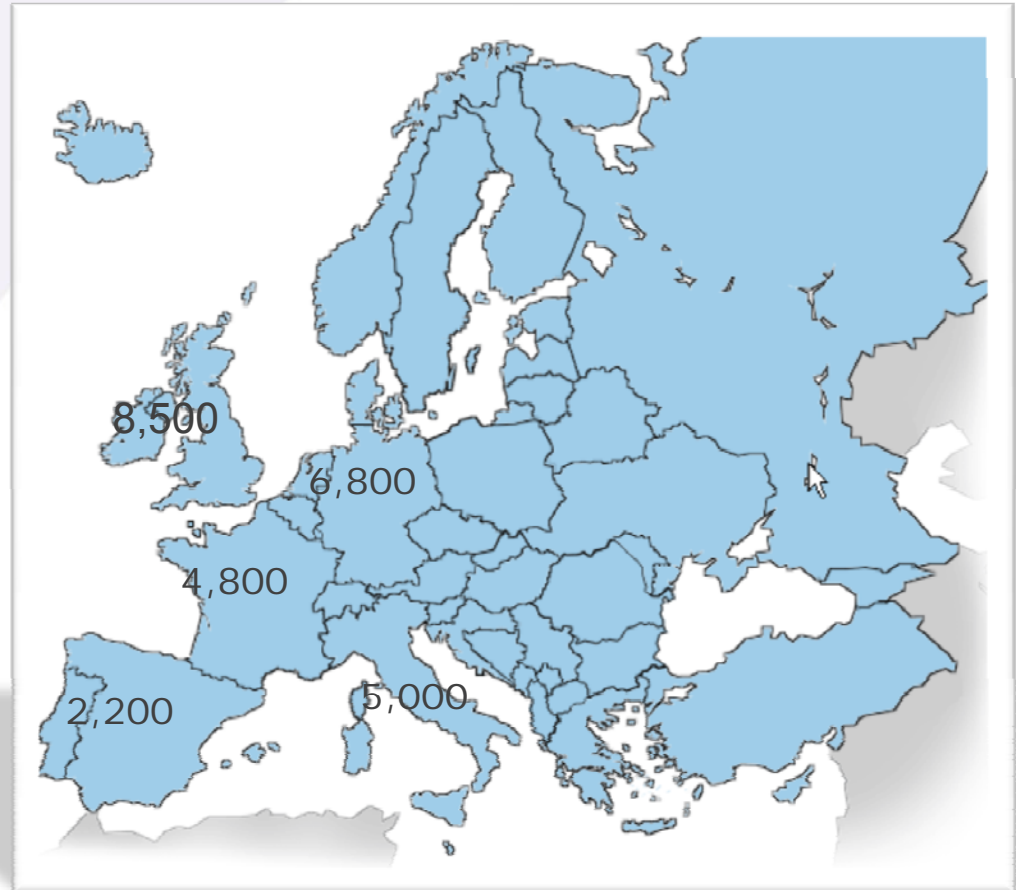
June 2010

Q3 2010



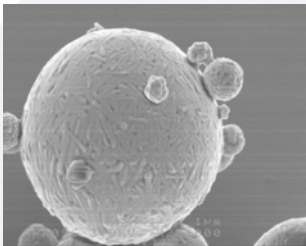
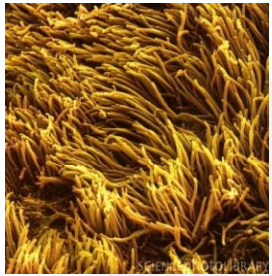
Bronchitol – commercialisation in EU

- Orphan drug – up to 12 years exclusivity
- Promotion by PXS in Western Europe (14 countries)
- Distributor for Central / Eastern Europe
- Centralised approach to pricing
 - Core pricing dossier Q3 2010
 - Market access staff Q3 2010
- First launch UK / Germany Q1 2011



27,000 people with CF in top 5 EU countries

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
- Affects 600,000 people worldwide

Bronchitol – bronchiectasis registration

- **1st 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



Bronchitol – bronchiectasis registration



- **2nd Phase III trial**

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

- **Primary endpoint**

- Reduction in number of exacerbations

- **Secondary endpoints**

- Exercise, mucus clearance, antibiotic use
- Quality of life

- **Status**

- Special Protocol Assessment concluded with U.S. FDA
- Orphan Drug designation
- First patient enrollment
- Data

USA

October 2009

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



- **Europe**

- Approved European Union (MRP)
- Staggered launch through distributors
- Launched in major EU countries except Germany

May 2007



- **South Korea**

- Approved for marketing
 - Pricing approval completed
 - Launched

Jan 2008

Sep 2009

Oct 2009

- **USA**

- NDA under review
- Positive recommendation by FDA Advisory Committee
- Response Letter received
- Process expected to conclude

Nov 2009

Dec 2009

2H 2010

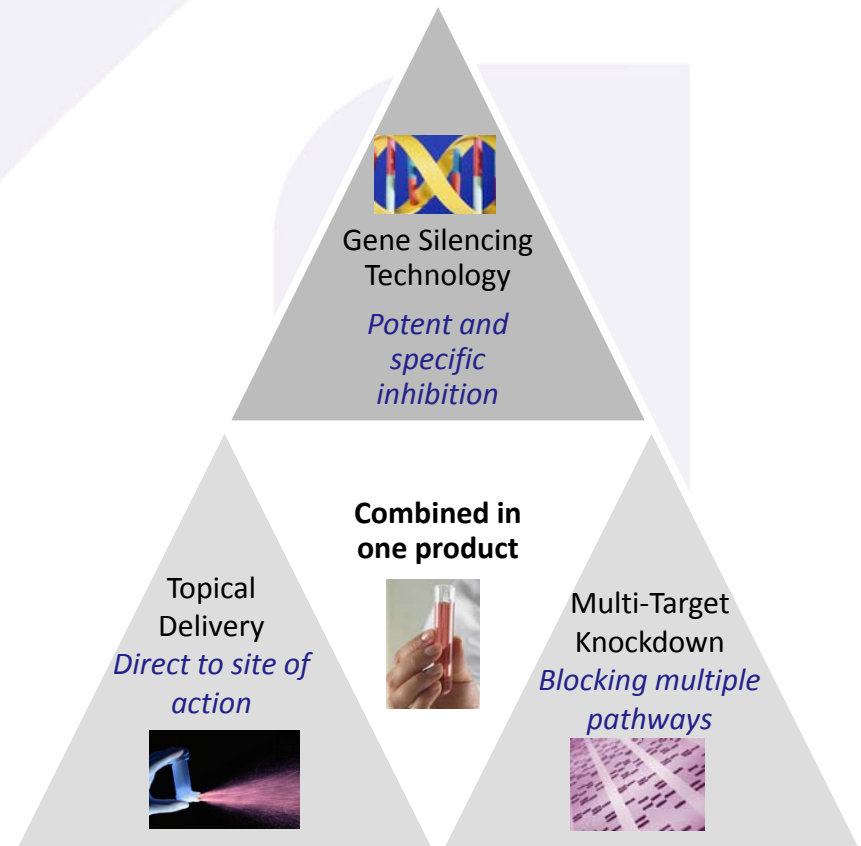


-
- Sales for 9 months to 31 March - \$0.64 million

ASM8 : A new approach for uncontrolled asthma



- Targeting severe asthma
 - affects ~6 million people
 - major cause of ER visits
 - limited treatment options
- Once daily by inhalation
- Improved side effect profile
 - Low systemic exposure
- Improved effectiveness
 - Targets multiple inflammatory proteins
- Inhibits protein synthesis



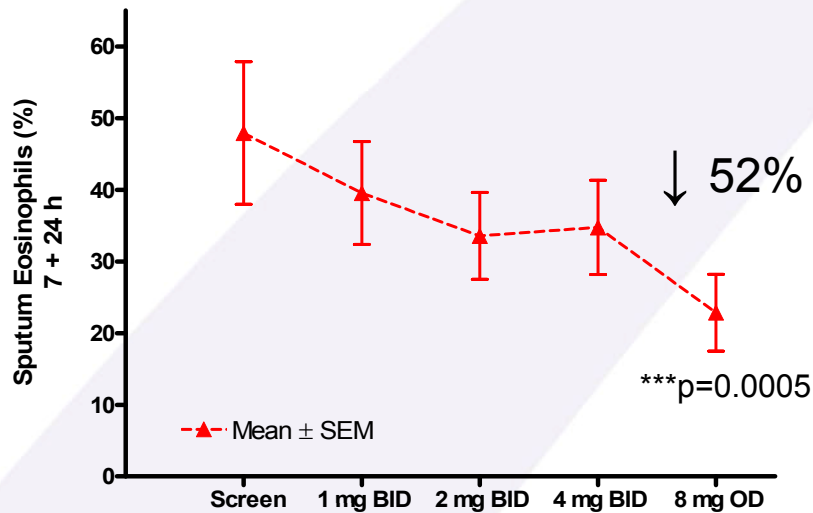
There exists an unmet medical need in patients with severe asthma

ASM8: Clinical studies completed

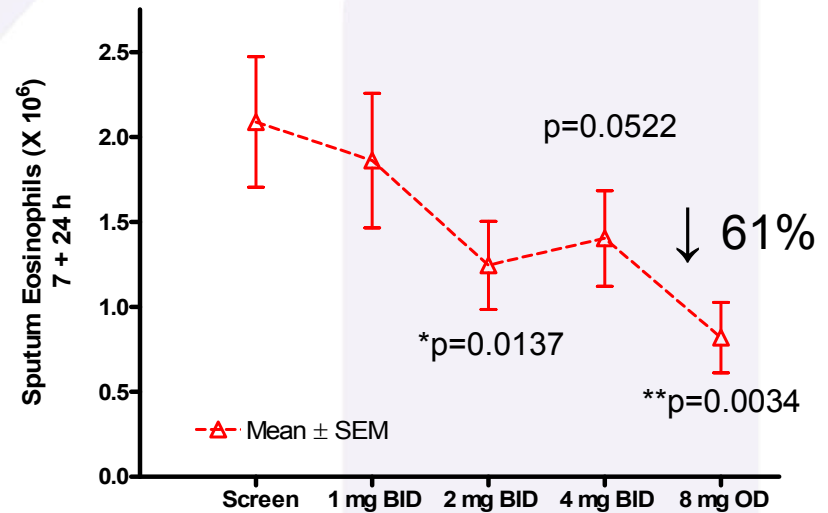
Phase 1 Safety	Phase 2a Allergen Challenge (4-day study)	Phase 2a Allergen Challenge (14-day study)	Phase 2a Dose Profiling
Single ascending dose comparison TPI ASM8 versus placebo (up to 6 mg)	Placebo-controlled, 4-day cross-over study (1.5 mg* Aerogen neb)	Placebo-controlled, 14-day cross-over study (1 mg* Respironics neb)	Ascending dose 1mg bd, 2mg bd, 4mg bd and 8mg od for 4 days. Allergen challenge
10 healthy subjects per dose, 5 doses	17 subjects with mild allergic asthma	18 subjects with mild allergic asthma	12 subjects with mild allergic asthma
Primary objective: <ul style="list-style-type: none"> • Safety Secondary objective: <ul style="list-style-type: none"> • Pharmacokinetics 	Co-primary objectives: <ul style="list-style-type: none"> • Late asthmatic resp • Safety Secondary objectives: <ul style="list-style-type: none"> • Early asthmatic resp • Inflammatory cells • Target mRNA • Pharmacokinetics * Metered dose	Same as 4-day study in Canada * Metered dose	Primary endpoint: <ul style="list-style-type: none"> • Sputum eosinophils • Safety Secondary objectives: <ul style="list-style-type: none"> • LAR • EAR • Target mRNA

ASM8: Sputum Eosinophils (sum of 7h and 24h)

% Eosinophils

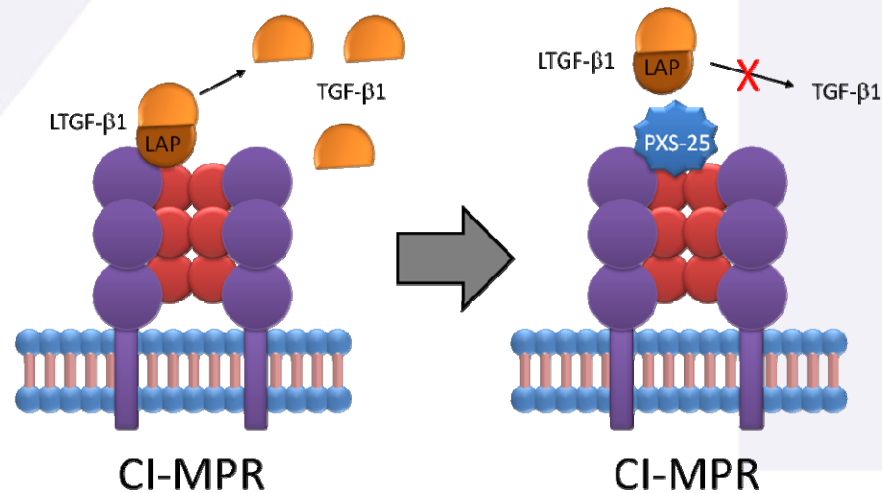
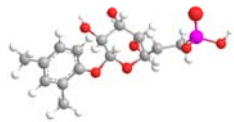


Absolute # Eosinophils



PXS 25 for fibrosis

- ❑ Inhibits cleavage of latent TGF β to active TGF β
 - Targeting Idiopathic Pulmonary Fibrosis
 - Affects >500,000 people worldwide
 - Small molecule with robust pharmaceutical profile
- ❑ Phase I trial completed
 - Safety, pharmacokinetics in healthy subjects



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
 - Complete process validation – mid 2010
 - Capacity
 - Initial capacity - 1 spray drier: 40,000 patients p.a.
 - Expanded capacity – 2nd spray drier: 80,000 patients p.a.

Financial Statements

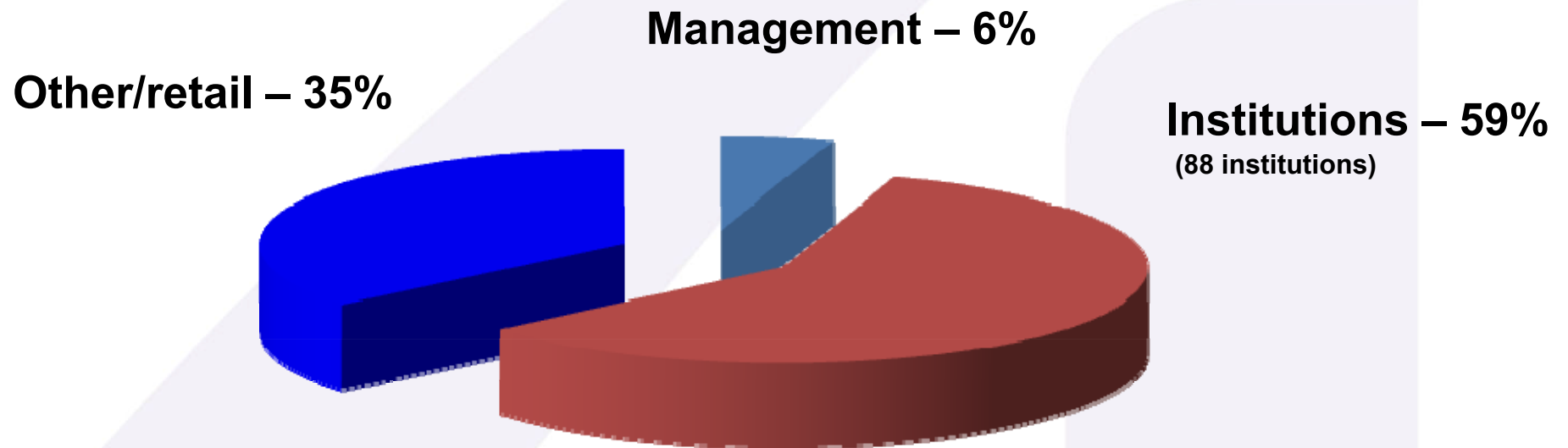
Financial Statement Data - Unaudited				
(International Financial Reporting Standards)				
('000 except per share data)				
Income Statement Data	Three months ended		Nine months ended	
	31-Mar-10	31-Mar-09	31-Mar-10	31-Mar-09
	A\$	A\$	A\$	A\$
Revenue from sale of goods	282	144	636	453
Cost of sales	(125)	(35)	(232)	(113)
Gross profit	157	109	404	340
Interest	1,003	927	2,933	4,584
Other income	123	132	288	276
Expenses				
Research & development	(8,991)	(7,193)	(26,287)	(20,780)
Commercial	(1,261)	(1,449)	(3,725)	(4,339)
Administration	(4,631)	(1,336)	(8,165)	(4,258)
Finance expenses	(148)	-	(656)	-
Total expenses	(15,031)	(9,978)	(38,833)	(29,377)
Loss before income tax	(13,748)	(8,810)	(35,208)	(24,177)
Income tax expense	-	1	(42)	(27)
Loss for the period	(13,748)	(8,809)	(35,250)	(24,204)
Basic and diluted earnings (loss) per share - \$	(0.063)	(0.045)	(0.162)	(0.124)
Depreciation & amortisation	689	271	1,836	789
Fair value of options issued under employee plan	719	650	1,872	1,801

Financial Statements

Balance Sheet Data	As at			
	31-Mar-10	30-Jun-09		
	A\$	A\$		
Cash and cash equivalents	95,904	124,993		
Property, plant & equipment	32,934	32,698		
Intangible assets	12,594	1,193		
Total assets	148,152	163,997		
Total liabilities	(28,821)	(26,306)		
Net assets	119,331	137,691		
Cash Flow Data	Three months ended		Nine months ended	
	31-Mar-10	31-Mar-09	31-Mar-10	31-Mar-09
	A\$	A\$	A\$	A\$
Cash flows from operating activities	(11,554)	(4,515)	(31,898)	(16,343)
Cash flows from investing activities	5,515	(3,655)	3,282	(9,742)
Cash flows from financing activities	(181)	-	(492)	11
Net increase (decrease) in cash held	(6,220)	(8,170)	(29,108)	(26,074)

Share Capital

(including options)



Institutions – 59%
(88 institutions)

31 March 2010: 225m shares; 13m options

END

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