

pharmax is



ABN 75 082 811 630 Human therapeutic products for chronic respiratory and autoimmune diseases

August 2004





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Investment Highlights

- Emerging specialty biotech company with two products in late stage development
 - Aridol Phase III for asthma diagnosis complete, data 9/04
 - Bronchitol expected to report phase II efficacy data in COPD & CF by end 2004
- Targeting large, underserved markets
 - Aridol filling a need for diagnosis and management of asthma
 - Bronchitol offers treatment for CF & COPD lung diseases
- All product marketing rights have been retained
- Strong intellectual property granted in US/Pending in Europe
- Multiple near-term value driving milestones
- Experienced management





Product Pipeline

	research	preclinical	phase I	phase II	phase III	registration	market
Respiratory diseases							
Aridol – airway function							Mid 2005
Bronchitol - bronchiectasis							Early 2008
Bronchitol - cystic fibrosis							Late 2007
Bronchitol - chronic bronchitis*							Late 2008
Autoimmune diseases							
PXS25 - multiple sclerosis							
PXS2030 – multiple sclerosis							
PXS2076 – rheumatoid arthritis							

* CB trial pending outcome of bronchiectasis trial

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- Build a fully integrated specialty pharmaceutical company spanning research, development and commercialization
- Focus on attractive product development and commercialization opportunities
- Undertake product development and commercialization
- Focus on respiratory and autoimmune markets
- Expand R&D pipeline through research and licensing





Respiratory market opportunity

Product	Target Application	Current Market (US\$)	Patients diagnosed	Potential Market	Projected penetration
Aridol	Diagnostic/Theranostic	\$100 M	52 M	31 M	High
Bronchitol	COPD (bronchiectasis and chronic bronchitis)	\$399 M	30 M	15 M	Low
Bronchitol	Cystic Fibrosis	\$294 M	75,000	75,000	Moderate

¹ Dollar figure based on current 400,000 bronchial challenge tests at \$250 charge / test





Aridol



Aridol



- New product for the diagnosis and management of Asthma and COPD
- Indirect airway provocation for accurately measuring level of ongoing inflammation
 - Current standard for diagnosis in Australia
 - Proposed replacement for direct provocation with methacholine in the US
- Quick and easy to use ideal for PCP outpatient clinic setting
- Phase III completed in July 2004, results in September
- Supported by international opinion leaders in respiratory medicine





Market Opportunity

Addressable Market (000 pts)



- Significant addressable market, 31 M patients
- Estimated 400,000 bronchial provocation tests used in 2003 in major pharmaceutical markets
- Methacholine provocation test is currently reimbursed in the U.S. (\$150 - \$300 per test)

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Phase II Clinical Trial Design: Asthma

Progressive Protocol: Diagnostic Measurement: Positive Diagnosis: Time taken: Numbers: Clinical Sites:

Recovery:

0, 5, 10, 20, 40, 80, 160, 160, 160 mg FEV₁ 1 minute post dose Fall in FEV₁ >14.9% 10 minutes (Mean positive test with PD_{15}) > 750 pts Worldwide 20 minutes (negative test with PD_{15}) Spontaneous recovery to baseline FEV₁ in 30 minutes (or Bronchodilator)



Arido



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harm

Phase II Trial Results

Measuring the effectiveness of inhaled steroid therapy



Effect on response to Aridol challenge of 8 weeks Rx with Budesonide



Phase III Clinical Trial Design

Progressive Protocol: Diagnostic Measurements: Positive Response: Number: Clinical Sites: Time taken:

Recovery:

Results Expected:

0, 5, 10, 20, 40, 80, 160, 160, 160 mg FEV₁ 1 minute post dose Fall in FEV₁ >14.9% or >9.9% 600 pts 12 10 minutes (Mean positive test with PD_{10}) 20 minutes (negative test with PD_{10}) Spontaneous recovery to baseline FEV₁ in 30 minutes (or Bronchodilator)

September 2004





Next steps in clinical development

• Aridol as an asthma diagnostic

- Results Nov 04
 - IST in 50 patients vs methacholine for clinical diagnosis in asthma
- Sept 04 Phase III clinical trial results
 - Aridol vs hypertonic saline and physician diagnosis in 600 patients
- Aridol as an asthma management tool
 - Results Nov 05
 - IST in 300 patients with 12 month follow up using Aridol to guide steroid dosage
 - Endpoint is number of exacerbations with Aridol vs standard British Thoracic guidelines
- Aridol as a COPD management tool
 - Results Jun 05
 - Investigator sponsored 100 patient study to determine sensitivity / specificity of Aridol test in identifying COPD patients who will respond to steroids (smokers)
 - Investigator sponsored 40 patient study to determine sensitivity of Aridol in identifying steroid responsive COPD patients (ex-smokers)

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Bronchitol





Product Detail





- Inhaled mannitol for CF & COPD
 - Phase IIb for COPD
 - Phase IIa for CF
 - Patents granted in the U.S. and Australia,
 - pending in Europe and Japan
- Uniform, respirable osmotically active sugar
 - Delivers compound into deep, smaller airways
 - Simple dry-powder delivery without need for nebulizer
- Therapeutic benefits
 - Reduce exacerbations
 - Reduce hospitalizations
 - Extend life expectancy

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Detailed Mechanism of Action

Lung defense (normal)



Cilia

Goblet Cell

`@

Submucosal glands

H,O

Ciliated Cel

Airway Surface Liquid

Restores Airway Surface Liquid

Non-absorbable sugar creates osmotic gradient
No risk of post-receptor effects limiting chronic utility

Changes rheologic properties

 Correction of mucus rheology increases action of ciliary elevator

Increases ciliary beat frequency

Decreased infection and chronic inflammation
 Increased pulmonary function

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Lung defense (compromised)



Airway Surface Liquid (thin/viscous)



Chronic bronchitis without Mannitol







Chronic bronchitis with Mannitol (400mg)







Bronchitol

Status in COPD





Chronic Obstructive Pulmonary Disease

• Epidemiology

- COPD represents emphysema, chronic bronchitis and bronchiectasis
- 4th leading cause of death in US estimated 12 M affected, 1.4 M diagnosed
- Direct cost to US healthcare estimated at \$18 billion per year
- Disease progression
 - Airway hyper-reactivity / obstruction (overlaps asthma)
 - Chronic infections
 - Irreversible lung tissue damage
 - Hypoxemia, pulmonary hypertension, right heart failure
- Current Management
 - Bronchodilators
 - Mucoactive agents
 - Oral corticosteroids (1 in 5 respond to steroids)
 - Prevention / Treatment of infections
 - Supplemental oxygen

Source: National Heart, Lung, and Blood Institute. Morbidity and Mortality: 2002







Proof-of-Concept Data - Bronchiectasis

Right Peripheral Region of Lung







Proof-of-Concept Data - Bronchiectasis





Phase IIb Clinical Trial Design

Study Population: Study Protocol: Dosage: Numbers Clinical Sites: Primary Endpoint: Additional Endpoints:

Results Expected:

Patients with known bronchiectasis Blinded, multicenter, cross-over trial 400 mg twice daily for 14 days 60 pts 4 QOL (St. George questionnaire) FEV₁, sputum microbiology, rheology and 24hr sputum volume

September 2004





Bronchitol

Status in CF





Cystic Fibrosis

- Epidemiology
 - Genetic disease affects or 1000 new infants per year in the U.S.
 - 30,000 children and adults affected in the US
 - Median survival now early-thirties
 - Disease progression
 - Abnormal pulmonary mucus secretions
 - Early infections with S. aureus, or H. influenza
 - Chronic infections with Pseudomonas aeruginosa and Burkholderia
 - Lung tissue damage (bronchiectasis), hypoxia, death
- Current Management
 - Pulmozyme a mucolytic agent
 - TOBI inhaled Tobramycin
 - Other antibiotics oral
 - Bronchodilators
 - Inhaled and oral corticosteroids





Proof-of-Concept Data - CF



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Phase IIa Clinical Trial Design

Study Population:

Study Protocol:

number: Clinical Sites: Dosage: Primary Endpoints: Additional Endpoints:

Evaluating children and adults with cystic fibrosis in the hospital setting Randomized, double blinded, multicenter, placebo controlled, crossover study 60 pts 4 420 mg twice per day for 14 days FEV₁ QOL (St. George questionnaire), lung function and safety

Results Expected:

November 2004





Next steps in clinical development

Phase IIb Trial

Study Population:

Study Protocol:

Dosage: Primary Endpoints: Additional Endpoints:

Results Expected:

Evaluating children and adults with cystic fibrosis in the hospital setting Head-to-head, parallel trial versus Pulmozyme in 30 patients 400 mg twice per day for 3 months QOL (St. George questionnaire), and FEV₁ Safety, sputum microbiology, and rheology

November 2005

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Preclinical Pipeline

Product Detail





Autoimmune Disease

• PXS25

- Orally available compound effective in MS and RA models
- IGF-2 receptor antagonist
- Inhibits diapedesis, immune cell trafficking
- Human studies Q2 2005
- Pro-drug under development with excellent PK profile
- PXS2030
 - Orally available compound targeting symptoms of MS
 - Peripheral cannabinoid receptor agonist
 - Inhibitor of T cell migration & B cell proliferation

• PXS2076

- Effective in RA models
- Believed to act through intracellular kinase pathways
- Inhibits TNF release from immune cells
- BA of >80%

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Summary



Upcoming Milestones

Aug 04 Aridol investigator sponsored trials commence Management of asthma with steroids – UK general practice \checkmark Management of COPD with steroids – Sydney \checkmark Bronchitol in COPD, Phase IIb trial results Sep 04 Sept 04 Aridol Phase III clinical trial results - asthma Oct 04 Aridol in COPD with steroids – Switzerland Submit IND - Aridol study in USA Oct 04 Aridol v methacholine in asymptomatic pts – Denmark Oct 04 Nov 04 Bronchitol in CF - Phase IIa trial results Nov 04 Aridol registration in Aus/EU Early 05 **Commence US Aridol study** Mid 05 Aridol launch Aus/EU Phase I study with PXS25 pro-drug Mid 05 Bronchitol in CF vs pulmozyme, Phase IIb trial results Nov 05 Bronchitol in COPD, Phase III results Late 06 Early 07 Bronchitol in CF, Phase III results ۲

Finances

	Year to 30 June 03	Year to 30 June 04
Income Statement	US\$'000	US\$'000
Revenues		
Grants	571	789
Interest	166	768
Other	25	34
	762	1,591
Expenditures		
Research and Development	(1,047)	(4,317)
Administration	_(574)	(1,557)
Net loss before and after tax	_(858)	(4,284)
Depreciation & amortisation	150	350
EBITDA	(875)	(4,702)
Balance Sheet		
Cash & equivalents	4,931	17,416
Total assets	7,009	19,518
Long term debt	-	-

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Management

Alan Robertson BSc, PhD Brett Charlton MBBS, PhD William Cowden BSc, PhD David McGarvey BA, CA John Crapper BAS, MBA Gary Phillips BPharm, MBA

28 employees, 24 in R&D, 4 in G&A

Chief Executive Medical Director Chief Scientist Finance Operations Commercial

Wellcome/Faulding/amrad
Baxter/Stanford/ANU
Progen/Peptech/ANU
PWC/Memtec/US Filter
Syntex/Memtec/US Filter
Novartis

