



ASX/ Media release

31 August 2005

## ***BRONCHITOL CYSTIC FIBROSIS TRIAL RESULTS POSITIVE***

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*Bronchitol™ was well tolerated and demonstrated statistically significant improvement in lung function relative to placebo.*

Pharmaxis (ASX:PXS, NASDAQ:PXSL) is pleased to announce results from its Phase II trial, DPM-CF-201, in patients with cystic fibrosis.

The trial achieved its primary end point of improvement in lung function as measured by FEV<sub>1</sub>. At the end of the two week treatment periods, patients receiving Bronchitol had statistically significantly improved lung function compared to two weeks of placebo treatment (p=0.008). Spirometry was used to assess lung function.

Pharmaxis Chief Executive Officer Alan Robertson said 'This excellent result suggests that Bronchitol may be an important new treatment for cystic fibrosis patients. We look forward to the results from additional studies and to working with the cystic fibrosis community and regulatory agencies around the world to bring Bronchitol to the market as rapidly as possible.'

The study was a double blind, placebo controlled, randomised comparison of 420mg of Bronchitol to placebo in 49 patients with cystic fibrosis at 8 centres across Australia and New Zealand. Bronchitol or placebo was administered twice a day for 14 days in a crossover design.

Secondary endpoints of the study include quality of life measures. Significantly better respiratory symptoms were achieved among patients when taking Bronchitol compared to when on placebo (p < 0.02). Additionally, Bronchitol had no deleterious effect on the microbiology of the sputum.

No serious adverse events were related to Bronchitol use.

Pharmaxis has received Orphan Drug Status from the Food and Drug Administration (FDA) for Bronchitol in cystic fibrosis.

Pharmaxis plans to present additional data from the study at the internationally important 19<sup>th</sup> Annual North American Cystic Fibrosis Conference to be held in Baltimore, MD in October.

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**About the trial**

The following information is provided in accord with the ASX and AusBiotech draft Code of Best Practice for Reporting by Biotechnology, Medical Device and other Life Sciences Companies.

Name of Trial	DPM-CF-201 (a Phase II study with Bronchitol)
Blinding Status	Double blind
Placebo Controlled	Yes
Treatment Method	
Route	Inhalation
Frequency	Twice daily
Dose levels	420mg for 2 weeks, 2 weeks washout, 2 weeks placebo (or vice versa)
No of subjects enrolled	49
No of subjects treated	39
Subject Selection Criteria	Known diagnosis of cystic fibrosis; either gender; aged 8 or older; baseline FEV <sub>1</sub> 40-80% of normal or decline of 20% in the last 12 months if greater than 80%; not pregnant or breast feeding, not participating in any other clinical trial.
Trial Location	Australia and New Zealand
Commercial partners involved	None
Duration	18 months
<u>Primary end point:</u>	
<input type="checkbox"/> FEV <sub>1</sub> versus placebo;	7% increase on Bronchitol. No change on placebo (p=0.008)
<u>Secondary end points:</u>	
<input type="checkbox"/> Other lung function measures;	15.5% increase in maximum midexpiratory flow (MMEF) [vs placebo p<0.02]. MMEF is the most important parameter to determine small airway function.
<input type="checkbox"/> Respiratory symptoms score;	Bronchitol improved relative to placebo (p<0.02)
<input type="checkbox"/> Nasal/sinus symptoms	No change
<input type="checkbox"/> CF Questionnaire;	No change overall
<input type="checkbox"/> Sputum microbiology	14 years or older - better respiratory quality of life (p < 0.02)
<input type="checkbox"/> Safety/adverse events	No change
	No significant adverse events related to Bronchitol use

## **About Pharmaxis**

Pharmaxis (ACN 082 811 630) is a specialist pharmaceutical company involved in the research, development and commercialization of therapeutic products for chronic respiratory and autoimmune diseases. Its development pipeline of products include Aridol™ for the management of asthma, Bronchitol™ for cystic fibrosis and chronic obstructive pulmonary disease (COPD) and PXS25 for the treatment of multiple sclerosis.

Founded in 1998, Pharmaxis was listed on the Australian Stock Exchange in November 2003 (symbol PXS), and on NASDAQ (symbol PXSL) in August 2005. The company is headquartered in Sydney at its TGA-approved manufacturing facilities.

For more information, go to [www.pharmaxis.com.au](http://www.pharmaxis.com.au) or call +61 2 9454 7230.

## **About Bronchitol**

Pharmaxis Ltd is developing Bronchitol™ for the management of chronic obstructive lung diseases including cystic fibrosis, bronchiectasis and chronic bronchitis.

Bronchitol™ is a proprietary formulation of mannitol administered in a convenient hand-held, pocket-sized inhaler. Its formulation as a dry powder with four-way action is designed to help restore normal lung clearance mechanisms.

Clinical studies have shown Bronchitol™ to be well tolerated and to stimulate mucus hydration and clearance in people with chronic obstructive lung diseases. In particular, Bronchitol™ has been shown to increase mucus clearance from the lungs and significantly improve quality of life for people with bronchiectasis.

Longer term clinical studies involving Bronchitol™ in chronic obstructive lung diseases are underway. These studies aim to demonstrate an improvement in the quality of life, a reduction in the number of bacterial infections and the need for physiotherapy and hospitalisation; an improvement in oxygen delivery from the lungs, exercise capacity and the quality of sleep; and an overall improvement in lung function.

## **About cystic fibrosis**

Cystic Fibrosis (CF) is a hereditary, life-limiting disease that affects the body's exocrine glands which produce mucus, saliva, sweat and tears. In this disease, a genetic mutation disrupts the delicate balance of sodium, chloride and water within cells, causing the exocrine glands to secrete fluids that are thick, sticky and poorly hydrated. This leads to chronic problems in various body systems, especially the lungs and pancreas, and the digestive and reproductive systems.

The thick mucus in the lungs severely affects the natural airway-clearing processes and increases the potential for bacteria to become trapped, resulting in respiratory infections that may require hospitalisation. Impairments to these essential lung defence mechanisms typically begin in early childhood and often result in chronic secondary infections, leading to progressive lung dysfunction and deterioration, and eventually, death.

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The average life expectancy for people with CF is only 31 years of age, with most patients dying from respiratory failure. In Australia, 2,500 people are living with CF, about one fifth of whom are children under five years of age. In the U.S., over 30,000 people are affected.

Pharmaxis is dedicated to developing products to treat this debilitating disease

**Forward-Looking Statements**

The statements contained in this press release that are not purely historical are forward-looking statements within the meaning of Section 21E of the Securities Exchange Act of 1934, as amended. Forward-looking statements in this press release include statements regarding our expectations, beliefs, hopes, goals, intentions, initiatives or strategies, including statements regarding the safety and effectiveness of Bronchitol in treating cystic fibrosis or the timing or ability of the Company to obtain regulatory approval of Bronchitol or to obtain orphan drug exclusivity in the U.S. All forward-looking statements included in this press release are based upon information available to us as of the date hereof, and we assume no obligation to update any such forward-looking statement as a result of new information, future events or otherwise. We can not guarantee that any product candidate will receive FDA or other regulatory approval or that we will seek any such approval. Factors that could cause or contribute to such differences include, but are not limited to, factors discussed in the "Risk Factors and Other Uncertainties" section of our Form 20-F filed with the U.S. Securities and Exchange Commission on August 22, 2005.

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