Media Release

1st August 2012

AUSTRALIAN DRUG DISCOVERY LISTED ON THE PBS FOR CYSTIC FIBROSIS

A drug discovered and developed in Australia has been listed on the Pharmaceutical Benefits Scheme (PBS) for the treatment of cystic fibrosis: one of the world's most common life-shortening genetic diseases.

Bronchitol® (mannitol) is the first of a new class of cystic fibrosis medicines designed to tackle one of the fundamental problems that leads to loss of lung function. It has been the subject of one of the largest clinical trial programs undertaken in cystic fibrosis - conducted in over 600 patients in 10 countries around the world and co-ordinated by scientists based in Australia.

Cystic fibrosis affects many organs but most vitally the lungs, where the disease causes the accumulation of thick, sticky mucus that results in loss of lung function. Children and adults with cystic fibrosis suffer repeated infections, inflammation and severe lung infections which can cause irreversible lung damage and respiratory failure, ultimately leading to a shortened life-span.[1] There are approximately 3,000 Australians with the genetic lung disease.

Two large Phase 3 trials with Bronchitol have demonstrated improvements in lung function and a reduction in lung infections. In one clinical trial, improvements in lung function were maintained for one and a half years– which represented the length of the trial for those patients who continued treatment. Side effects reported in patients taking Bronchitol were generally comparable to those who received the control treatment.[2 3]

The most common adverse reaction in the trials was cough. Although reported as a common adverse event, a productive cough is a beneficial component of mucus clearance.

Bronchitol® has been developed by Australian pharmaceutical company Pharmaxis (ASX:PXS) and is manufactured at the company's purpose built factory in Sydney.

Bronchitol® is a spray-dried form of mannitol, delivered to the lungs by a specially designed, portable inhaler.
Bronchitol® is approved in Australia for the treatment of cystic fibrosis in adults and paediatric patients aged over six years as either an add-on therapy to dornase alfa, or in patients intolerant of, or inadequately responsive to, dornase alfa. Bronchitol® is PBS listed from August 1st 2012.

Bronchitol may cause bronchial hyperresponsiveness in some patients, and so all patients are required to undergo an initiation dose assessment prior to starting therapy. Patients with asthma or a history of significant episodes of haemoptysis should be closely monitored by their physician.

Professor John Wilson, Head, Cystic Fibrosis Service The Alfred Hospital, Melbourne, said: "New treatments in cystic fibrosis are vitally important and this particular treatment is a very good example of translation of research into a clinical product that has a good outcome for our patients. This is something that has been developed in this country, it adds to the armamentarium of treatments that we now have for CF."

Mr David Jack, CEO of Cystic Fibrosis Australia said: "New therapeutic advances are desperately needed, and the PBS listing of Bronchitol is good news. I am pleased for the CF Community that a product of Australian innovation and research has brought an advance in patient care. Improving lung function and preventing lung infections are key to maintaining good health, so new treatment options have the potential to make a real difference in the lives of CF patients."

Pharmaxis has been supported by federal government grants for research and development in the field of respiratory medicine and biotechnology innovation.

** End **

PBS Listing Information:

Section 100 Public Hospital Authority Required (STREAMLINED) (HSD)

Section 100 Private Hospital Authority Required (HSD)

For more information: http://www.pharmaxis.com.au/

For Bronchitol Approved Product Information:
http://www.pharmaxis.com.au/assets/pdf/BRO026_Bronchitol_AU_PI_1_HR.pdf

For Bronchitol Consumer Medicine Information:
http://www.pharmaxis.com.au/assets/pdf/BRO024_Bronchitol_CMI_1_HR.pdf

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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 disorders. Its product Aridol® for the assessment of asthma is launched in a number of key markets. Its development pipeline of products includes, Bronchitol for cystic fibrosis, bronchiectasis and chronic obstructive pulmonary disease (COPD), PXS25 for the treatment of lung fibrosis and ASM8 and PXS4159 for asthma. Pharmaxis is listed on the Australian Securities Exchange (symbol PXS). The company is headquartered in Sydney at its TGA-approved manufacturing facilities. For more information about Pharmaxis, go to www.pharmaxis.com.au

Minimum Product Information:

Please review full Product Information which accompanies this item before prescribing. Full Product Information is available on request from Pharmaxis Pty Ltd. ABN 75 082 811 630. 20 Rodborough Rd, Frenchs Forest, NSW 2086, or on the TGA website. INDICATIONS: Treatment of cystic fibrosis (CF) in both paediatric and adult populations six years and above, as either an add-on therapy to dornase alfa, or in patients intolerant to, or inadequately responsive to, dornase alfa. DOSAGE AND METHOD OF USE: Inhale contents of ten 40 mg capsules via the inhaler device, twice a day. CONTRAINDICATIONS: Hypersensitivity to mannitol or to any of the capsule ingredients. Bronchial hyperresponsiveness to inhaled mannitol. PRECAUTIONS: Asthma: Patients with asthma must be carefully monitored for worsening signs and symptoms after the initiation dose of Bronchitol. Patients must be advised to report worsening signs and symptoms of asthma to their physician. Hyperresponsiveness to inhaled mannitol: Patients must be monitored for bronchial hyperresponsiveness before commencing the therapeutic dose regimen of Bronchitol. Bronchitol may

cause bronchoconstriction requiring treatment, even in patients who were not hyperresponsive to the initiation dose of inhaled mannitol. Impaired Lung Function: Safety and efficacy have not yet been demonstrated in patients with a FEV1 <30% of predicted. Impaired Hepatic / Renal Function: Bronchitol has not formally been studied in patients with impaired renal or hepatic function. Haemoptysis: Patients with a previous history of significant episodes of haemoptysis (>60 mL) should be carefully monitored. Bronchitol has not formally been studied in patients with a history of haemoptysis in the previous 6 months. ADVERSE REACTIONS: Very Common: Cough. Common: Decreased appetite, headache, haemoptysis, bronchospasm, wheezing*, asthma*, condition aggravated, pharyngolaryngeal pain, productive cough, chest discomfort, bacteria sputum identified*, vomiting, post-tussive vomiting. * Note: Frequency of adverse reaction lower than noted in the control group. DATE OF PREPARATION: February 2011.

REFERENCES:

1.Ramsey B. Management of pulmonary disease in patients with cystic fibrosis. NEJM 1996;335(3):179-188.

2.Bilton D et al. Inhaled dry powder mannitol in cystic fibrosis: an efficacy and safety study. Eur Respir J 2011;38:1071–1080

3.Aitken M et al. Long-Term Inhaled Dry Powder Mannitol in Cystic Fibrosis. Am J Respir Crit Care Med 2012;185(6):645–652