



## Media Release

26 October 2012

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### **IMPORTANT MILESTONE: BRONCHITOL APPROVED BY NICE IN UK**

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Pharmaceutical company Pharmaxis (ASX: PXS) is pleased to announce that the National Institute for Health and Clinical Excellence (NICE) in the United Kingdom has issued a positive recommendation in its Final Appraisal Determination for Bronchitol<sup>®</sup>, clearing the way for reimbursement by the National Health Service.

Bronchitol (mannitol dry powder for inhalation), is licensed in the UK for the treatment of adult cystic fibrosis (CF) patients aged 18 years and above as an add-on therapy to best standard of care. The decision by NICE represents an important milestone in marketing of the product.

The National Institute for Health and Clinical Excellence (NICE) provides evidence-based guidance to help resolve uncertainty about which medicines represent the best quality care and the best value for money for the UK's National Health Service.

Pharmaxis CEO Dr Alan Robertson said, "Pharmaxis welcomes this decision from NICE, which recognises Bronchitol as a clinically and cost effective treatment option for CF patients with the most unmet medical need. We have worked with clinicians and patient organisations to respond to questions from NICE during this review process and will continue to work alongside NICE and the CF community to ensure all eligible patients under Bronchitol's licence can benefit from the drug".

Bronchitol is the first and only CF product to have received a positive recommendation from NICE. The recommendation provides a new option for CF patients who cannot use rhDNase (recombinant human deoxyribonuclease) because of ineligibility, intolerance or inadequate response, whose lung function is declining as measured by a fall in forced expiratory volume in 1 second [FEV1] of greater than 2% a year and for whom other osmotic agents are not considered appropriate.

"The clinical studies of Bronchitol showed very promising results in what is a complex therapeutic area", said Dr Diana Bilton, Director of the Adult Cystic Fibrosis Unit at the Royal Brompton Hospital, London, and lead investigator for the first of the Bronchitol Phase III trials. "This new treatment has the potential to delay lung function decline in CF patients, thereby reducing the associated risks of exacerbation and mortality. Bronchitol brings a clear step change in efficacy and ease of use to a patient population that currently spend large amounts of time on laborious nebulised therapies. These patients will be delighted to have a new inhaled treatment option."

Jo Osmond, Director of Clinical Care at the Cystic Fibrosis Trust, said "We are very pleased with this decision from NICE, as we firmly believe that Bronchitol is an important addition to CF care, which will help to relieve the burden of treatment for adults with this debilitating condition".

Bronchitol has Orphan Drug Designation in the U.S., Europe and Australia and is approved for marketing in Australia and throughout the European Union. An application has been submitted for marketing approval in the U.S.

#ENDS#

**SOURCE:** Pharmaxis Ltd, Sydney, Australia

**CONTACT:** Alan Robertson – Chief Executive Officer

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Ph: +61 2 9454 7200 or email [alan.robertson@pharmaxis.com.au](mailto:alan.robertson@pharmaxis.com.au)

**RELEASED THROUGH:**

**Australia:**

Felicity Moffatt, phone +61 418 677 701 or email [felicity.moffatt@pharmaxis.com.au](mailto:felicity.moffatt@pharmaxis.com.au)

**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 sold in key international markets. Its product Bronchitol® for cystic fibrosis is recently launched in Europe and Australia and its development pipeline of products includes, Bronchitol for bronchiectasis, PXS64 for the treatment of lung fibrosis, ASM8 for asthma and PXS4728 for fibrotic disease. Pharmaxis is listed on the Australian Securities Exchange (symbol PXS). The company's head office and manufacturing facilities are located in Sydney. For more information about Pharmaxis, go to [www.pharmaxis.com.au](http://www.pharmaxis.com.au) or contact Investor Relations on phone +61 2 9454 7200.

**About Bronchitol**

Bronchitol has been developed to help clear mucus (a major source of lung infections), improve lung function and reduce exacerbations in patients with cystic fibrosis.

Bronchitol is a proprietary formulation of mannitol administered as a dry powder in a convenient hand-held inhaler. Bronchitol hydrates the lungs, helps restore normal lung clearance, and allows patients to clear mucus more effectively. Clinical studies have shown Bronchitol to be effective and well tolerated in treating patients with cystic fibrosis.

**About Cystic Fibrosis**

In a healthy person, there is a constant flow of mucus over the surfaces of the air passages in the lungs, removing debris and bacteria. In CF, an inherited disease, a defective gene disrupts ion transport across the epithelial membrane within cells. In the lungs, this leads to a depletion of the airway surface liquid that normally bathes the cilia, and a resultant reduction in mucociliary clearance. The result is thick, sticky mucus that clogs the lungs, severely restricting the natural airway-clearing process. It also increases the potential for bacteria to become trapped and for inflammation, thus creating an unhealthy lung environment that leads to life-threatening lung infections.

**Forward-Looking Statements**

Forward-looking statements in this media release include statements regarding our expectations, beliefs, hopes, goals, intentions, initiatives or strategies, including statements regarding the potential for Aridol and/or Bronchitol. All forward-looking statements included in this media 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 cannot guarantee that any product candidate will receive regulatory approval or that we will seek any such approval.

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