
PHARMAXIS COMPLETES RECRUITMENT OF PIVOTOL BRONCHITOL CYSTIC FIBROSIS CLINICAL TRIAL FOR US MARKET

Pharmaceutical research company Pharmaxis (ASX: PXS) today announced it has completed recruitment of its international clinical trial evaluating Bronchitol® (mannitol) in adults with cystic fibrosis. The results of the trial (CF303) are expected to be reported in the second quarter of 2017.

The Phase 3 trial is being conducted in accordance with the requirements of the US Food and Drug Administration (FDA) to gain approval for Bronchitol to treat cystic fibrosis (CF) in the United States in adult patients. Subject to a positive trial outcome, the Company will submit a response to the FDA and a decision on approval can be expected in 2018.

As guided by the FDA, the trial is a 26 week randomised, double-blind parallel group investigation of Bronchitol administered twice daily in patients aged 18 and over with cystic fibrosis to assess improvements in lung function, pulmonary exacerbations and safety. Subject to final randomisation of patients screened by the trial sites, the final enrollment is expected to reach 420 adult CF patients.

Pharmaxis has partnered its work on Bronchitol for the United States with global pharmaceutical company Chiesi Farmaceutici SpA (Chiesi). Chiesi is responsible for funding up to US\$22 million of the cost of the trial, the total cost of which is expected to be approximately US\$26 million. Chiesi is also responsible for completing the Bronchitol New Drug Application with the FDA. Subject to approval in the United States, Bronchitol will be sold as part of Chiesi's cystic fibrosis portfolio. Milestones totaling up to US\$25 million are payable including US\$10 million on the launch of Bronchitol.

Pharmaxis will manufacture Bronchitol on commercial terms for Chiesi with Pharmaxis retaining a high teens percent share of sales revenue as its margin.

Gary Phillips, Pharmaxis Chief Executive Officer said "We are pleased to have attained this significant milestone in such a large undertaking securing more than 400 study participants at 126 sites in 21 countries. The US is the largest CF market and we now have greater certainty around when the study will report and, subject to that report being positive, when the FDA will complete its consideration of our new drug application.

"The clinical study protocol closely follows the design of the two large scale clinical trials already undertaken by Pharmaxis (CF 301 and CF 302) in which a post hoc analysis of the subgroups of adult patients (307 in total) showed a significant improvement in FEV₁. I'd like to thank the CF community for its support and participation. We look forward to working with Chiesi to make this new treatment option available for CF patients in the United States."

The CF303 trial is being managed by INC, a global contract research organisation with significant experience running international trials in the cystic fibrosis community.

Bronchitol is a precision spray-dried form of mannitol, delivered to the lungs by a specially designed, portable inhaler. The product is approved for marketing for the treatment of cystic fibrosis patients aged over six years in Australia and for patients aged 18 years and over throughout the European Union and in Israel.

Trial Design	
Name of trial	DPM-CF-303: Long Term Administration of Inhaled Mannitol in Cystic Fibrosis – A Safety and Efficacy Trial in Adult Cystic Fibrosis Subjects
Primary endpoint	The mean change in FEV ₁ (mL) from baseline (Visit 1) over the 26-week treatment period (to Visit 4)
Secondary endpoints	<ul style="list-style-type: none"> • Mean change from baseline FVC (mL) over the 26-week treatment period; • Time to first pulmonary exacerbation over the 26-week treatment period; • Rate of pulmonary exacerbation over the 26-week treatment period • Number of days in hospital due to pulmonary exacerbations; • The incidence of pulmonary exacerbations; • Number of days on antibiotics (oral, inhaled or IV) due to pulmonary exacerbations; • Ease of expectoration measured using a visual analogue scale; <i>and</i> • CFQ-R respiratory domain score
Blinding status	Double blind
Placebo controlled	Yes
Trial design	Randomised, multicentre, double-blind, controlled, parallel group. 26 weeks duration
Treatment route	Inhalation
Treatment frequency	Twice daily
Dose level	400mg mannitol or control
Number of subjects	404 to 424
Subject selection criteria	<ul style="list-style-type: none"> • Confirmed diagnosis of cystic fibrosis • Be aged at least 18 years old, male and female • Predicted FEV₁ of ≥ 40% and ≤ 90% • Pass mannitol tolerance test
Trial locations	125 sites across Europe, North America, South America, Australia, Russia & New Zealand
Commercial partners involved	Chiesi Farmaceutici SpA

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About Pharmaxis

Pharmaxis (ACN 082 811 630) is an Australian research pharmaceutical company with a portfolio of products at various stages of development and approval. Its product Bronchitol® for cystic fibrosis is marketed in Europe and Australia and a phase 3 trial to enable completion of an NDA for the US market is underway. Its product Aridol® for the assessment of asthma is sold in Europe, Australia and Asia. The company's development pipeline is centred on its expertise in amine oxidase chemistry and includes Semicarbazide-Sensitive Amine Oxidase Inhibitors (SSAO) for Non-alcoholic Steatohepatitis (NASH) and inflammatory diseases including Chronic Obstructive Pulmonary Disease (COPD), and Lysyl Oxidase Inhibitors (LOX) targeting fibrotic diseases including pulmonary fibrosis and some cancers. In May 2015, Boehringer Ingelheim acquired the Pharmaxis investigational drug PXS4728A, to develop it for the treatment of the liver-related condition NASH. Pharmaxis is listed on the Australian Securities Exchange (symbol PXS). The company's head office, manufacturing and research facilities are located in Sydney, Australia. For more information about Pharmaxis, please see www.pharmaxis.com.au.

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 of products and drug candidates. All forward-looking statements included in this media release are based upon information available to us as of the date hereof. Actual results, performance or achievements could be significantly different from those expressed in, or implied by, these forward-looking statements. These forward-looking statements are not guarantees or predictions of future results, levels of performance, and involve known and unknown risks, uncertainties and other factors, many of which are beyond our control, and which may cause actual results to differ materially from those expressed in the statements contained in this document. Except as required by law we undertake no obligation to update these forward-looking statements as a result of new information, future events or otherwise.