# Media Release

**30 October 2014**

## PHASE 3 CLINICAL TRIAL IN CYSTIC FIBROSIS BEGINS

Pharmaceutical company Pharmaxis (ASX: PXS) today announced that it has enrolled the first subject in its international clinical trial evaluating Bronchitol® in adults with cystic fibrosis (CF303).

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 (mannitol) to treat cystic fibrosis in the United States. As guided by the FDA, the clinical study protocol closely follows the design of the two large scale clinical trials already undertaken by Pharmaxis (CF 301 and CF 302). The trial is a 26 week randomised, double-blind parallel group investigation of Bronchitol administered twice daily in patients with cystic fibrosis. The trial will enroll between 350 and 440 cystic fibrosis patients aged 18 years and older, and will assess improvements in lung function, pulmonary exacerbations and safety.

Management of the trial is outsourced to INC, a global contract research organisation with significant experience running international trials in the cystic fibrosis community. More than 100 sites across 19 countries will participate in the study and recruitment is expected to take twelve months to complete.

Pharmaxis CEO Mr Gary Phillips said, "This Phase 3 trial in adults has been carefully designed with the benefit of our two previous CF trials (CF301 and CF 302) where a post hoc analysis[1] of the subgroups of adult patients showed a significant improvement in $FEV_1$. The design of the trial also incorporates the very clear guidance provided by the FDA concerning what is required in order to gain approval for Bronchitol in the US. We are very pleased to have enrolled the first patient."

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 patients aged over six years in Australia and for patients aged 18 years and over throughout the European Union and in Israel.

| **Trial Design** | |
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| 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 | • Mean change from baseline FVC (mL) over the 26-week treatment period:<br>• Time to first pulmonary exacerbation over the 26-week treatment period;<br>• Rate of pulmonary exacerbation over the 26-week treatment period<br>• Number of days in hospital due to pulmonary exacerbations;<br>• The incidence of pulmonary exacerbations;<br>• Number of days on antibiotics (oral, inhaled or IV) due to pulmonary exacerbations;<br>• Ease of expectoration measured using a visual analogue scale; *and*<br>• CFQ-R respiratory domain score |
| Blinding status | Double blind |
| Placebo controlled | Yes |
| Trial design | Randomised, multicentre, double-blind, controlled, parallel group.<br>26 weeks duration |
| Treatment route | Inhalation |
| Treatment frequency | Twice daily |

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| Dose level | 400mg mannitol or control |
| Number of subjects | 350 to 440 |
| Subject selection criteria | • Confirmed diagnosis of cystic fibrosis<br>• Be aged at least 18 years old, male and female<br>• Predicted $FEV_1$ of ≥ 40% and ≤ 90%<br>• Pass mannitol tolerance test |
| Trial locations | Europe (12 countries), North America (2 countries), South America (2 countries), South Africa, Australia & New Zealand |
| Commercial partners involved | No commercial partner |
| Expected enrolment period | 12 months |

1. Ref: Bilton D, Robinson P, Cooper P, et al. *J Cystic Fibrosis* 2011, Suppl 20 (A78) and Data on File Pharmaxis

For enquiries about the clinical trial please email med.info@pharmaxis.com.au

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**About Pharmaxis**
Pharmaxis (ACN 082 811 630) is a specialist pharmaceutical company involved in the research, development and commercialisation of therapeutic products for chronic respiratory disorders. Its product Bronchitol® for cystic fibrosis is marketed in Europe and Australia. Its product Aridol® for the assessment of asthma is sold in key international markets. The company's development pipeline of products includes Lysyl Oxidase Inhibitors (LOX) targeting fibrotic diseases including pulmonary fibrosis and some cancers and Semicarbazide-Sensitive Amine Oxidase Inhibitors (SSAO) for inflammatory disease including Chronic Obstructive Pulmonary Disease (COPD) and Non-alcoholic steatohepatitis (NASH). Pharmaxis is listed on the Australian Securities Exchange (symbol PXS). The company's head office and manufacturing facilities are located in Sydney, Australia.

**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 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.