

ASX Announcement

2 November 2020

FDA APPROVES BRONCHITOL FOR US MARKET

PHARMAXIS TO MANUFACTURE PRODUCT AND EXPORT TO USA

ACCESS TO US MARKET AND CASH MILESTONES TRANSFORMATIVE FOR PHARMAXIS

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today announced the United States Food and Drug Administration (FDA) has approved Bronchitol® (mannitol) as add-on maintenance therapy to improve pulmonary function in cystic fibrosis (CF) patients 18 years of age and older. The product, developed by Pharmaxis in Australia, has been steered through final stages of FDA approval by US licensee Chiesi Farmaceutici SpA (Chiesi).

A US\$7 million milestone is now payable to Pharmaxis by Chiesi with a further US\$3 million payable on shipment by Pharmaxis of commercial launch stock scheduled for the first quarter of 2021.

Pharmaxis expects Bronchitol sales in the US market to contribute strongly to the product's global sales and profit growth from its launch in Q2 2021, making the mannitol business (Bronchitol® and Aridol®) cash flow positive from FY 2021.

Bronchitol is a precision spray-dried powder which is delivered to the lungs by a portable inhaler and works to rehydrate the airway and lung surface of CF patients.

Pharmaxis Chairman Malcom McComas said, "We are very pleased that Bronchitol, an Australian drug discovery, will now be available for cystic fibrosis patients in the USA. This is a very significant event for everyone who has worked hard to achieve this outcome. Approval in the world's largest market is a testament to the capability of the Pharmaxis clinical team who designed and conducted the three large scale phase 3 clinical trials establishing Bronchitol's safety and efficacy and our particular thanks goes to all the patients and healthcare professionals who made those trials possible."

Pharmaxis Chief Executive Officer Gary Phillips said, "The US market makes up more than 60% of the global CF market by value so today's announcement of FDA approval has important and positive ramifications for Pharmaxis. It justifies the vision we shared with our partner Chiesi to commit to the final phase 3 study requested by the FDA. The additional volume of Bronchitol that Pharmaxis will produce at our Sydney production facility to supply the US, on top of Australia and 17 other international markets, greatly increases capacity utilisation and consequently radically improves the cost of goods."

Following the receipt of the Chiesi approval and launch milestone payments totalling US\$10m, Pharmaxis expects US sales to commence in H1 2021. Pharmaxis will then earn high teens royalties which, allied to a long term supply contract, is forecast to deliver approximately 20% of Chiesi US Bronchitol net sales directly to the Pharmaxis mannitol business segment EBITDA. Three sales milestones totalling US\$15m are also payable on achieving annual sales thresholds.

Mr Phillips continued, "The FDA approval of Bronchitol is transformational for Pharmaxis because the milestone payments from Chiesi together with positive cash flows from the mannitol business

segment allow us to move confidently ahead with the development of our lead clinical development asset PXS-5505 for the treatment of myelofibrosis.

“Clearing this last significant regulatory hurdle for the mannitol business also enables us to progress a number of restructuring initiatives to further extend the company’s cash runway. These will continue to be pursued over the coming months.”

Together with Pharmaxis cash of A\$34 million proforma¹ at June 2020 the contribution from the mannitol business provides a cash runway that covers development of PXS-5505 to conclusion of its phase 2 trial for the treatment of myelofibrosis. Pharmaxis today announced detailed plans for the development of PXS-5505 in a separate announcement “Pharmaxis Prioritises Breakthrough Clinical Program on Myelofibrosis.”

The Company will host an investor conference call at 11.00 this morning (Sydney time) in relation to today’s announcements. A separate announcement will provide access details. A recording of the call will be available later today on the Pharmaxis website.

1. Proforma cash at June 2020 is calculated as follows: \$15m cash at 30 June 2020 plus A\$5m R&D tax credit received 14 October 2020 plus A\$14m Chiesi milestone payments.

#ENDS#

SOURCE: Pharmaxis Ltd, Sydney, Australia

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About Pharmaxis

Pharmaxis Ltd is an Australian pharmaceutical research company developing drugs for inflammatory and fibrotic diseases, with a focus on myelofibrosis. The company has a highly productive drug discovery engine built on its expertise in the chemistry of amine oxidase inhibitors, with drug candidates in clinical trials. Pharmaxis has also developed two respiratory products which are approved and supplied in global markets, generating ongoing revenue.

Pharmaxis is developing its drug PXS-5505 for the bone marrow cancer myelofibrosis which causes a build up of scar tissue that leads to loss of production of red and white blood cells and platelets. The US Food and Drug Administration has granted Orphan Drug Designation to PXS-5055 for the treatment of myelofibrosis and permission under an Investigational Drug Application (IND) to progress a phase 1c/2 clinical trial that is scheduled to begin recruitment in Q1 2021. PXS-5505 is also being investigated as a potential treatment for other cancers such as liver and pancreatic cancer.

Other drug candidates being developed from Pharmaxis’ amine oxidase chemistry platform are targeting fibrotic diseases such as kidney fibrosis, NASH, pulmonary fibrosis and cardiac fibrosis; fibrotic scarring from burns and other trauma; and inflammatory diseases such as Duchenne Muscular Dystrophy.

Pharmaxis has developed two products from its proprietary spray drying technology that are manufactured and exported from its Sydney facility; Bronchitol® for cystic fibrosis, which is approved and marketed in the United States, Europe, Russia and Australia; and Aridol® for the assessment of asthma, which is approved and marketed in the United States, Europe, Australia and Asia.

Pharmaxis is listed on the Australian Securities Exchange (PXS). Its head office, manufacturing and research facilities are in Sydney, Australia. www.pharmaxis.com.au

About Bronchitol

Bronchitol is a precision spray-dried form of mannitol, delivered to the lungs by a specially designed, portable inhaler. Bronchitol works by rehydrating the airway/lung surface and promoting a productive cough. The product is approved for marketing for the treatment of cystic fibrosis patients aged over six years in Australia and Russia and for patients aged 18 years and over throughout the European Union and the United States. In all markets, patients are required to have a tolerance test prior to being prescribed Bronchitol to ensure that they are not hyper responsive to mannitol.

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. For example, despite our efforts there is no certainty that we will be successful in developing or partnering any of the products in our pipeline on commercially acceptable terms, in a timely fashion or at all. 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.