Media Release

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PHARMAXIS AWARDED $1M AUSTRALIAN GOVERNMENT FUNDING TO PROGRESS DUCHENNE MUSCULAR DYSTROPHY DRUG INTO THE CLINIC

Pharmaceutical research company Pharmaxis Ltd (ASX: PXS) today announced it has been awarded $1 million funding from the Biomedical Translation Bridge (BTB) program to significantly advance work on the company's drug discovery for the treatment of the devastating genetic disorder Duchenne Muscular Dystrophy (DMD).

Pharmaxis was selected following a highly competitive review conducted by an independent expert evaluation committee as part of the BTB program, which is administered by MTPConnect. The Australian government matched funding will allow the company to take another one of its pipeline of amine oxidase inhibitors (PXS-4699) through to the commencement of human clinical trials. A planned comprehensive program of pre-clinical studies will build on the pioneering work already conducted on Pharmaxis compounds by independent international researchers focused on Duchenne Muscular Dystrophy. PXS-4699 is a dual amine oxidase inhibitor which is expected to protect muscle and reduce inflammation as well as organ fibrosis in DMD. It is hoped this could result in better daily functioning for patients, improved quality of life and longer life expectancy.

"The Pharmaxis research has potential to make a difference to patients with Duchenne Muscular Dystrophy (DMD), a debilitating genetic disorder affecting thousands of Australians. It's an example of the high-quality research translation being generated in Australia which will progress to clinical trials," said MTPConnect Managing Director and CEO, Dr Dan Grant.

Professor Steve Wilton, Director Centre for Molecular Medicine & Innovative Therapeutics, Murdoch University commented, "New treatments, including gene and molecular therapies to restore dystrophin are showing promise for DMD. However, DMD and other muscular dystrophies are multisystem disorders with complex pathologies, and there is an urgent need for additional or complementary treatments to reduce the fibrosis and inflammation that are hallmarks of the disease. PXS-4699 is a powerful new tool in the repertoire of potential treatments for DMD, and targets the inflammatory pathway and fibrosis that are so detrimental in dystrophies. We see PXS-4699 as an additional or adjunct therapy for muscular dystrophies."

The BTB co-funded project is expected to complete all the steps necessary to commence phase 1 healthy volunteer studies within 12 months and a further study in DMD patients scheduled to start within 18 months.

Chief Executive Officer of Pharmaxis, Mr Gary Phillips, said, "Over recent years Pharmaxis investment in drug discovery has progressed three pipeline drugs through pre-clinical testing to the commencement of human clinical trials and beyond. Non-dilutive funding sources, such as this one provided by the Australian Government's BTB grant program, will allow us to similarly progress PXS-4699 in an orphan disease with high unmet need whilst not detracting from the focused investments we are making in a myelofibrosis treatment and as we await the upcoming FDA decision to grant a marketing authorisation of our cystic fibrosis treatment for patients in the United States."

Delivered by MTPConnect, the Australian Government's BTB program is a $22.3 million Medical Research Future Fund initiative that provides up to $1m in matched funding to nurture the translation of new therapies, technologies and medical devices through to proof of concept to turn innovative medical ideas into reality.

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SOURCE: Pharmaxis Ltd, Sydney, Australia

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About Pharmaxis

Pharmaxis Limited is an Australian pharmaceutical research company and a global leader in drug development for inflammation and fibrotic diseases. The company has a highly productive drug discovery engine, drug candidates in clinical trials and significant future cash flows from partnering deals.

Leveraging its small-molecule expertise and proprietary amine oxidase chemistry platform, Pharmaxis has taken four in-house compounds to Phase 1 trials in just five years. Boehringer Ingelheim acquired the Pharmaxis anti-inflammatory AOC3 inhibitor in 2015 to develop it (BI 1467335) for two diseases: the liver condition Non-alcoholic Steatohepatitis (NASH) and diabetic retinopathy (DR).

The company's successor amine oxidase program has developed an oral anti-fibrotic LOXL2 inhibitor, aimed at NASH, pulmonary fibrosis (IPF) and other high-value fibrotic heart and kidney diseases, with a commercial partnering process underway, a systemic pan-LOX inhibitor for acute fibrosis and cancer that will enter a phase 2 study in 2020 and a topical pan-LOX inhibitor for scarring that is expected to commence phase 1 studies in 2H 2020. Pharmaxis' Mannitol platform has yielded the products Bronchitol® for cystic fibrosis, which is marketed in Europe, Russia and Australia, with United States FDA approval pending; and Aridol® for the assessment of asthma, which is sold 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. http://www.pharmaxis.com.au/

What is Duchenne Muscular Dystrophy (DMD)?

Duchenne muscular dystrophy (DMD) is a genetic condition that affects the muscles, leading to muscle wasting that gets worse over time. DMD occurs primarily in males, though in rare cases may affect females. The symptoms of DMD include progressive weakness and loss (atrophy) of skeletal and heart muscles. Early signs of DMD may include delayed ability to sit, stand, or walk and difficulties learning to speak. Muscle weakness is usually noticeable in early childhood. Most children with DMD use a wheelchair by their early teens. Heart and breathing problems also begin in the teen years and lead to serious, life threatening complications. DMD is caused by genetic changes (DNA variants) in the DMD gene. DMD is inherited in an X-linked recessive pattern and may occur in people who do not have a family history of DMD. While there is no known cure for DMD, there are treatments that can help control symptoms. Duchenne muscular dystrophy is estimated to occur in about 16 live male births per 100,000 in the USA and about 20 live male births per 100,000 in the United Kingdom.
Source: https://rarediseases.info.nih.gov/diseases/6291/duchenne-muscular-dystrophy#ref_7593

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 partnering our LOXL2 program or any of the other 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.