

## Valuable development pipeline with the funds to exploit

Pharmaxis is an Australian based pharmaceutical research company focused on developing innovative treatments for inflammatory and fibrotic diseases with high unmet needs.

A strategic turnaround in recent years has the company on track to become a powerhouse in drug development for inflammatory and fibrotic diseases as evidenced by key deals announced in 2015 with German pharmaceutical company Boehringer Ingelheim (Boehringer), UK based Synairgen plc, and Italian company Chiesi Farmaceutici SpA (Chiesi).

Pharmaxis also manufactures and markets Bronchitol® for cystic fibrosis in Europe and Australia. Opening the US market where a phase 3 study is well advanced and approval in Russia will see this business unit provide cash flow to the business.

With four drug discovery programs underway, a strong balance sheet cash position (A\$42m) and financials evidencing transformation, Pharmaxis is moving into an exciting phase.

### Key points

- **Scientific platform validated** by globally significant deal with Boehringer in 2015 for Phase 1 asset.
- **Multiple potential new drugs in high value markets over next 2 years.**
- **Strong cash position:**
  - o A\$42m cash at 31 March 2016.
  - o Year to date average monthly cash burn of \$1.2m.
  - o Next Boehringer milestone (~A\$25m) expected Q1 2017.
  - o Chiesi milestone on US launch of Bronchitol expected CY 2018.
- **Experienced management** with proven drug development and licensing skills, producing value accretive deals.
- **De-risked business model** focused on investing in validated high value targets and realising value post Phase 1 or 2.
- **Bronchitol.** Opening of new markets (Russia H2 2016) and US (CY2018) – potential to transition business to a cash generator.

### Risks & catalysts

#### Next Boehringer milestone – A\$25m

- Triggered by commencement of phase 2 study.
- Confirmed by Boehringer as on track for Q1 2017.
- Increases probability of Pharmaxis receiving full deal value (~A\$750m).
- Further validates Pharmaxis scientific platform.
- Further strengthens balance sheet and ability to deliver additional drugs.

#### Drug discovery – significant value potential

- 3 small molecule drug candidates with the potential to get to phase 1 trials in the next 12-24 months in therapeutic areas attracting significant deal values.

#### Bronchitol US – key step towards FDA approval

- Tie breaker clinical trial expected to complete recruitment mid-2016, report H1 2017.
- US partner Chiesi responsible for completing NDA and commercial launch.
- Launch milestone ~A\$13m in CY 2018.

#### Bronchitol – new attractive market

- Russian approval expected shortly.
- Russian CF market ~US\$30m annually.
- First sales anticipated Q3 2016.

### Corporate snapshot

- **ASX: PXS**
- **Share price:** A\$0.255 (30 June 2016)
- **Ordinary shares:** 317.2 million
- **Employee option:** 7.5 million
- **Market cap:** A\$80.90m (30 June 2016)
- **12 month share price graph:**



## Product portfolio

**Bronchitol for cystic fibrosis:** phase 3 trial underway in US (partnered with Chiesi), marketed in rest of world by distributors.

**Aridol for asthma diagnosis:** marketed in Australia, EU and South Korea (by distributor).

**SSAO inhibitor for NASH (& other inflammatory indications):** phase 1, sold to Boehringer Ingelheim in 2015.

**SSAO/MAO-B inhibitor for neuro inflammation (e.g Alzheimer's):** lead optimization.

**SSAO/MPO inhibitor for respiratory inflammation:** lead optimization.

**LOXL-2 inhibitor for NASH and liver fibrosis:** lead optimization.

**LOXL-2 inhibitor (IPF) for pulmonary fibrosis:** collaboration with Synairgen, lead optimization.

**LOX/LOXL-2 inhibitor for cancer wound healing & kidney fibrosis:** exploratory assessment by leading universities/academics.

**Orbital dry powder inhalation device:** phase 1 – seeking partner.

**ASM-8 for asthma / orphan eosinophilic respiratory disease:** phase 2 – seeking partner.

## Financials

	A\$'000	Fiscal year ended		Nine months ended
		30-Jun-14	30-June-15	31-Mar-16
(unaudited)				
<b>Income statements</b>				
Sales		5,036	5,999	5,432
Total revenue		10,486	59,247	14,226
Total expenses		(62,201)	(40,739)	(28,557)
Net profit (loss) after tax		(51,818)	18,466	(14,338)
<b>Segment results – adjusted EBITDA</b>				
Bronchitol & Aridol		(22,555)	(10,045)	(5,858)
New drug development		(1,620)	35,068	(1,091)
Corporate		(6,226)	(3,532)	(2,829)
<b>Cash generated (used)</b>		(29,802)	19,725	(12,954)
<b>Cash at bank</b>		34,182	54,138	41,508

## Pharmaxis drug discovery, partnerships and collaborations

Pharmaxis aims to improve success rates, keep drug discovery costs down and speed up development timelines by focusing on drug targets that have been independently validated in diseases with limited treatment options and where its expertise in inflammation and fibrosis using its amine oxidase platform can be utilised.

**Boehringer** acquired Pharmaxis' PXS-4728A in May 2015 primarily for development to treat non-alcoholic steatohepatitis (NASH), a severe, progressive form of fatty liver disease that can lead to scarring and cirrhosis. The deal is potentially worth more than A\$750 million in milestone payments to Pharmaxis if the drug is developed and commercialised with high single digit royalties also payable on sales in a market estimated at more than US\$35 billion by 2025.

**Chiesi Farmaceutici SpA** have partnered with Pharmaxis to conduct a pivotal Phase 3 clinical trial designed to meet the remaining clinical requirements of the US Food and Drug Administration (FDA) for Bronchitol, an inhaled powder for the treatment of cystic fibrosis. Under the partnership, Chiesi will pay Pharmaxis a milestone (~A\$13,000) on commercial launch and then royalties and milestones on sales. Bronchitol is currently on the market in Europe where it is sold via distributors and in Australia.

**Synairgen** and Pharmaxis announced a research collaboration in August 2015 to develop a selective inhibitor to the lysyl oxidase type 2 enzyme (LOXL2) to treat the fatal lung disease idiopathic pulmonary fibrosis (IPF). Under the partnership, Synairgen will fund further development until Phase 1 trials are conducted, when a license partner will be sought. This collaboration exemplifies the Company's flexible approach to share development risks and increase the probability of successfully developing new drugs

<b>Address:</b> Pharmaxis Ltd 20 Rodborough Rd Frenchs Forest NSW 2086, AUSTRALIA	<b>Investors, Corporate and Financial</b> Chief Financial Officer & Company Secretary Pharmaxis Ltd David McGarvey Phone: +61 2 9454 7200 Email: <a href="mailto:david.mcgarvey@pharmaxis.com.au">david.mcgarvey@pharmaxis.com.au</a>	<b>Pharmaxis website</b> <a href="http://www.pharmaxis.com.au">www.pharmaxis.com.au</a>
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