# Quarterly Shareholder Update – March 2017

## "Three Share Price Drivers"

Dear Shareholder,

It was pleasing to read a recent analysis in the industry publication Bioshares entitled "Three Share Price Drivers for Pharmaxis." The article accurately noted that the company has a number of milestones approaching in Q2 which could have a meaningful impact on its share price. While the market will determine the extent to which the Pharmaxis share price reflects value, we are now quickly closing in on three significant and valuable milestones due this quarter:

1. The commencement of a phase 2 NASH trial by Boehringer Ingelheim, triggering the payment of an €18 million (~A$25m) milestone to the Company and further validating our science and business model. We also hope to learn more about Boehringer's plans to commence a phase 2 trial in a second indication – triggering another milestone payment to Pharmaxis and opening up a second stream of potential milestone payments.
2. The completion of preclinical toxicology studies for the LOXL2 program which Pharmaxis is conducting with our UK collaborator Synairgen, the last step before we commence human phase 1 clinical studies in the second half of 2017 and move towards a formal partnering process.
3. Top line results from our international clinical trial of Bronchitol (CF303) designed to meet the remaining clinical requirements of the US Food and Drug Administration (FDA). The trial was completed in February and is scheduled to report top line results by the end of the quarter. The US market is the largest opportunity for Bronchitol and we continue, with our partner Chiesi, to prepare for an FDA submission and the commercial launch at which time Pharmaxis receives a US$10 million milestone payment.

Our progress for the March quarter included a full review of the Pharmaxis drug discovery pipeline by our recently expanded Scientific Advisory Board. The group was enthusiastic about a number of valuable opportunities being developed utilising our expertise in amine oxidase chemistry and LOXL2 research. The SAB members encouraged us to continue to move these assets forward into formal preclinical toxicology studies as soon as possible.

During the quarter the company also completed the first sale of Bronchitol to Russia, a potentially large opportunity and an important step in bringing our Bronchitol business to profitability. As part of our commitment to this market two members of our Australian based team travelled throughout Russia in February meeting with cystic fibrosis clinicians.

The report below outlines our progress in more detail.

Sincerely,

Chief Executive Officer

# Drug discovery

## Boehringer Ingelheim approaches commencement of phase 2 NASH trial

Boehringer acquired PXS-4728A in May 2015 to develop initially as a treatment for non-alcoholic steatohepatitis (NASH). Under the terms of our agreement, Boehringer has total responsibility for the development program and is required to make milestone payments to Pharmaxis as PXS-4728A progresses towards approval as well as other sales related payments post approval.

Boehringer has completed all prerequisite non-clinical safety and pharmacokinetic studies and scaled up drug synthesis and is now working through the logistical steps to commence a phase 2 NASH trial in the current quarter. This will trigger a milestone payment to Pharmaxis of approximately A$25 million.

As reported last quarter Boehringer has also provided a target profile and the clinical development steps for a second indication. Under our agreement with Boehringer, Pharmaxis will receive a lesser but significant payment on commencement of a phase 2 study in a second indication. Pharmaxis expects to receive more information concerning Boehringer's plans for this study in late June. Total milestones through to approval for a second indication are in aggregate the same as for the first indication, but weighted more towards the latter stage of development and approval.

## LOXL2 inhibitor program preclinical candidates in toxicology studies

The Pharmaxis drug discovery group has developed a number of selective inhibitors to the lysyl oxidase type 2 enzyme (LOXL2) utilising the amine oxidase platform that delivered PXS-4728A. LOXL2 is important in the liver disease NASH, cardiac fibrosis, kidney fibrosis, the fatal lung disease idiopathic pulmonary fibrosis (IPF) and also plays a role in some solid cancers.

Pharmaxis is working with its collaborator, UK biotechnology company Synairgen plc (LSE: SNG) to progress at least one drug candidate into the clinic in the second half of 2017. Two drug candidates are presently in 28 day GLP toxicology studies which should both report by mid-2017.

## Large pharma company interest

As the LOXL2 program approaches the clinic, Pharmaxis and Synairgen continue to engage with a number of large pharma companies in addition to the twice yearly meetings at the US industry conferences - JP Morgan each January and US BIO in June. The role of LOXL2 in fibrotic diseases such as NASH and pulmonary fibrosis is of significant interest to many of the big companies who are keen to understand our scientific progress and timing of planned partnering.

## Pharmaxis expands Scientific Advisory Board

Pharmaxis has expanded its Scientific Advisory Board with the appointment of Professor Andrew Boyle, Head of the Cardiovascular Medicine at the University of Newcastle.

Professor Andrew Boyle is a cardiologist who studies left ventricular remodeling, the process by which the heart weakens and becomes ineffective following heart attacks and with advancing age. In particular, his research focuses on the molecular and cellular mechanisms of fibrosis and stem cell function in the heart.

Professor Boyle received his medical degree from Monash University and then completed cardiology advanced training in Melbourne. It was during this time he noticed that early treatments for heart attack were very successful at keeping patients alive, but the late heart failure that ensued was difficult to treat. He became interested in the emerging research field of stem cell therapy for heart disease, with a view to regenerating the damaged heart muscle that occurred during a heart attack. Professor Boyle undertook a PhD at the University of Melbourne studying cardiac regeneration, and then moved to the US and continued this study as a fellow at Johns Hopkins University. He then joined the faculty at the University of California San Francisco, becoming Associate Professor of Medicine, where his laboratory focused on the effects of ageing on left ventricular remodeling, funded by the US National Institutes of Health. After 7 years there, he moved to the University of Newcastle and the Hunter Medical Research Institute (HMRI) where he now continues this research. Professor Boyle has a research laboratory based at HMRI where he studies pre-clinical models of left ventricular

remodeling, and he also performs clinical research at the John Hunter Hospital.

Professor Boyle is ideally suited to provide us with expert advice and we welcome him to the SAB.

Further information concerning the Scientific Advisory Board is available on the Pharmaxis website.

## Scientific Advisory Board reviews drug development pipeline

In February the Pharmaxis Scientific Advisory Board joined the management and drug discovery teams to review the Company's drug development pipeline:

- The LOX inhibitor program has potential anti-fibrotic application in scarring and Pharmaxis is working together with the University of Western Australia, the Fiona Wood Foundation and the Royal Perth Hospital Burns Unit. The research is currently focused on formulation and is scheduled to commence formal preclinical toxicology studies in the second half of 2017. Research work is also exploring other severe fibrotic indications where the Company's LOX inhibitor may also have application.
- The SSAO/MPO program is developing a dual inhibitor with potential anti-inflammatory application in respiratory and cardiovascular disease. The research is currently focused on fully profiling the drugs under development and identifying the appropriate indications to pursue.
- The SSAO/MAOB inhibitor program with potential anti-inflammatory application in a number of indications. Further investment in this project has been postponed whilst awaiting the results from a number of academic collaborators focusing on identifying the appropriate indication.

# Bronchitol for cystic fibrosis

Bronchitol® is an inhaled dry powder for the treatment of cystic fibrosis (CF) and has been the subject of two large scale global clinical trials conducted by Pharmaxis. The product is approved and marketed in Europe, Russia, Australia and several other countries.

## United States

A third international multicentre clinical trial (CF303) designed to meet the remaining clinical requirements of the US Food and Drug Administration (FDA) was completed in February and is scheduled to report top line results in the current quarter. The trial was conducted in 126 sites across 21 countries and enrolled 423 adult CF patients.

Pharmaxis has partnered with Chiesi Farmaceutici SpA (Chiesi) to conduct CF303. Under the terms of the agreement and following a positive outcome of the trial, Chiesi will have responsibility for completing the New Drug Application with the FDA and the commercialisation of Bronchitol in the United States. We continue to work closely with Chiesi on all aspects of securing US marketing approval for Bronchitol.

Subject to a positive trial outcome, we anticipate that an FDA submission would be made in the first half of 2018.

Chiesi has funded US$22 million of the US$26 million total cost of the trial. Milestones of up to US$25 million and mid to high teen percentage royalties are payable to Pharmaxis including US$10 million on the commercial launch of Bronchitol.

## Europe

In the EU, Chiesi has been Pharmaxis' exclusive distributor for the UK and Germany since June 2015. Chiesi is an experienced and respected partner in key global markets and sells Bronchitol as part of its cystic fibrosis portfolio.

Having built local European inventory levels in the previous year, Chiesi had only purchased smaller quantities of Bronchitol in the current financial year. During the quarter regular six monthly supply recommenced with Chiesi purchasing

Bronchitol valued at $850,000. Ongoing Bronchitol sales by Pharmaxis will continue to reflect the expected six monthly ordering by Chiesi rather than in-market use of the product.

Chiesi unit sales in the March quarter in the UK and Germany were marginally above the level of sales in the March 2016 quarter. While sales in Germany continue to fluctuate quarter by quarter, pleasingly unit sales in the UK are approximately 20% higher than the level of sales before Chiesi was appointed.

Pharmaxis also sells Bronchitol in Austria, Denmark and Norway via its German based logistics provider, with sales totaling $37,000 in the March 2017 quarter, $123,000 for the year to date.

## Other territories

Bronchitol is sold in Australia by Pharmaxis and in Turkey and Russia by exclusive distributors.

A highlight of the quarter was the first sales into Russia value at $643,000, following approval in September 2016. While Pharmaxis' application for country wide reimbursement (in both adults and children) will be determined in the second half of 2017, a number of cystic fibrosis clinics in Moscow are authorized to directly purchase Bronchitol.

Sales for the quarter and year to date were as follows:

| A$'000 | Three months | | Nine months | |
|---|---|---|---|---|
| | Mar 17 | Mar 16 | Mar 17 | Mar 16 |
| **Australia** | 177 | 199 | 566 | 554 |
| **Russia** | 643 | - | 643 | - |
| **Turkey** | 91 | 48 | 206 | 48 |

## Aridol

Aridol sales increased 31% for the quarter to $469,000 and 10% year to date to $1.4 million. Pharmaxis is working towards reintroducing Aridol to the United States via a suitably experienced distributor.

# Corporate

## Major shareholders

The holdings of several large Pharmaxis institutional shareholders changed during the quarter and early April.

BVF Partners LP increased its shareholding from 12.9% to 15.9%, having first joined the Pharmaxis share register in May 2015 after the sales of PXS-4728A to Boehringer Ingelheim. BVF Partners LP is a San Francisco-based private investment partnership specialising in fundamentally-driven public biotechnology investments. Its investment approach is to build concentrated, long-term investments in small-cap biotechnology companies while performing rigorous diligence and on-going monitoring.

Australian Ethical Australian Share Fund, a long time shareholder, increased its shareholding from 5.0% to 10.2%.

BVF Partners LP and Australian Ethical Australian Share Fund are now the Company's two largest shareholders.

Information on Pharmaxis shareholders including substantial shareholders is available on the Pharmaxis website.

## Bioshares points to big quarter

Independent investment research publication Bioshares recently included an article "Three Share Price Drivers for Pharmaxis" in its weekly publication, pointing to the Company's upcoming milestones. A copy of the article is available on the Pharmaxis website.

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# Financials

## Key financial metrics

| A$'000 | Three months ended | | Nine months ended | |
|---|---|---|---|---|
| (unaudited) | **31-Mar-17** | **31-Mar-16** | **31-Mar-17** | **31-Mar-16** |
| **Income statements** | | | | |
| Sales | 2,267 | 1,705 | 3,957 | 5,432 |
| Total revenue | 4,146 | 4,854 | 11,056 | 14,225 |
| Total expenses | (7,345) | (8,007) | (25,280) | (28,557) |
| **Net profit (loss) after tax** | **(3,199)** | **(3,153)** | **(14,234)** | **(14,338)** |
| **Segment results – adjusted EBITDA** | | | | |
| Bronchitol & Aridol | (738) | (1,602) | (4,679) | (6,088) |
| New drug development | (1,892) | (870) | (4,313) | (2,717) |
| Corporate | (934) | (1,103) | (3,012) | (2,855) |
| **Total** | **(3,564)** | **(3,575)** | **(12,004)** | **(11,660)** |
| **Statement of cash flows** | | | | |
| Cash inflow/ (outflow) from: | | | | |
| Operations | (2,132) | (4,004) | (11,026) | (10,430) |
| Investing activities | (183) | (143) | (397) | (1,235) |
| Financing activities | (431) | (417) | (1,287) | (1,289) |
| **Total cash used** | **(2,747)** | **(4,564)** | **(12,710)** | **(12,954)** |
| Foreign currency exchange rate changes impact on cash | (73) | 136 | (73) | 324 |
| **Cash at bank** | **26,499** | **41,508** | **26,499** | **41,508** |

## Highlights

- Sales revenue for the quarter benefited from the Chiesi order for the UK and German Bronchitol markets and the first sale of Bronchitol to our Russian distributor.
- Total revenue for the quarter decreased predominantly because of the lower expenditure in relation to clinical trial CF303 which is reimbursable by Chiesi, partially offset by the increase in sales. While at 31 December 2016 Chiesi had contributed all of its US$22 million funding commitment to CF303, for financial reporting purposes the reimbursement is recognised as revenue over the complete term of the clinical trial.
- Underlying core expenses for the quarter were mainly unchanged from the comparable period, however four specific items accounted for a net decrease in total expenses of $661,000.
    - Clinical trial expenses in relation to clinical trial CF303 decreased by $1.7 million.
    - Drug development expenses increased by $0.7 million reflecting increased levels of research activity.

  - Foreign exchange gains of $969,000 million in the March 2016 quarter increased to a gain of $1.2 million in the current quarter, including an unrealised gain of approximately $1.3 million in both periods in relation to the financing agreement with NovaQuest.
  - Other expenses include the net transfer of manufacturing labour and overhead to and from inventory as product is first manufactured and then subsequently sold to distributors and customers. Other expenses were $234,000 in the March 2016 quarter and $751,000 in the current quarter, reflecting a transfer of the manufacturing and overhead associated with the large Bronchitol orders sold to the UK, Germany Russia in the quarter.
- Segment information provided below provides a useful overview of the business. Note that the decrease in the Corporate Adjusted EBITDA reflects foreign exchange losses of $318,000 in the March 2016 quarter reducing to $131,000 in the March 2017 quarter.
- Closing cash for the quarter was $26.5 million. Cash used during the quarter was $2.7 million, $12.7 million for the half year.
- The Company received its R&D tax credit claim for the 2016 financial year of $2.1 million in the March 2017 quarter.
- As noted above the Company also expects to receive approximately $25 million from Boehringer Ingelheim when it commences a phase 2 trial of PXS-4728A in the second quarter of 2017.

## Segment information

| A$'000 | Segment information - three months ended | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| (unaudited) | 31-Mar-17 | | | | 31-Mar-16 | | | |
| **Income statements** | Bronchitol & Aridol | New drug developmt | Corporate | Total | Bronchitol & Aridol | New drug developmt | Corporate | Total |
| **Revenue** | | | | | | | | |
| Sale of Bronchitol | 1,798 | - | - | 1,798 | 1,348 | | | 1,348 |
| Sale of Aridol & other | 469 | - | - | 469 | 357 | | | 357 |
| | **2,267** | **-** | **-** | **2,267** | **1,705** | | | **1,705** |
| Clinical reimbursement | 1,570 | - | - | 1,570 | 2,557 | - | - | 2,557 |
| Tax credit | 16 | 44 | - | 60 | - | - | - | - |
| Other revenue | 6 | (2) | 85 | 89 | 1 | 248 | 86 | 335 |
| | **3,859** | **42** | **85** | **3,986** | **4,263** | **248** | **86** | **4,597** |
| **Expenses** | | | | | | | | |
| Employee costs | (1,526) | (540) | (455) | (2,521) | (1,395) | (424) | (435) | (2,254) |
| Clinical trials | (1,409) | - | - | (1,409) | (3,114) | (12) | | (3,126) |
| Drug development | - | (1,311) | - | (1,311) | | (592) | | (592) |
| Other expenses | (1,662) | (83) | (564) | (2,309) | (1,356) | (90) | (754) | (2,220) |
| Total expenses | (4,597) | (1,934) | (1,019) | (7,550) | (5,865) | (1,118) | (1,189) | (8,172) |
| **Adjusted EBITDA** | **$(738)** | **$(1,892)** | **$(934)** | **$(3,564)** | **$(1,602)** | **$(870)** | **$(1,103)** | **$(3,575)** |

| A$'000 | Segment information - nine months ended | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| (unaudited) | 31-Mar-17 | | | | 31-Mar-16 | | | |
| **Income statements** | Bronchitol & Aridol | New drug developmt | Corporate | Total | Bronchitol & Aridol | New drug developmt | Corporate | Total |
| **Revenue** | | | | | | | | |
| Sale of Bronchitol | 2,508 | - | - | 2,508 | 4,115 | | | 4,115 |
| Sale of Aridol & other | 1,449 | - | - | 1,449 | 1,317 | | | 1,317 |
| | **3,957** | **-** | **-** | **3,957** | **5,432** | | | **5,432** |
| Clinical reimbursement | 5,871 | - | - | 5,871 | 6,952 | - | - | 6,952 |
| Tax credit | 16 | 44 | - | 60 | - | - | - | - |
| Other revenue | 20 | 328 | 250 | 598 | - | 668 | 259 | 927 |
| | **9,864** | **372** | **250** | **10,486** | **12,384** | **668** | **259** | **13,311** |
| **Expenses** | | | | | | | | |
| Employee costs | (4,456) | (1,514) | (1,528) | (7,498) | (4,211) | (1,252) | (1,537) | (7,000) |
| Clinical trials | (6,807) | - | - | (6,807) | (9,392) | (109) | | (9,501) |
| Drug development | - | (2,903) | - | (2,903) | | (1,772) | | (1,772) |
| Other expenses | (3,280) | (268) | (1,734) | (5,282) | (4,868) | (252) | (1,578) | (6,698) |
| Total expenses | (14,543) | (4,685) | (3,262) | (22,490) | (18,471) | (3,385) | (3,115) | (24,971) |
| **Adjusted EBITDA** | **$(4,679)** | **$(4,313)** | **$(3,012)** | **(12,004)** | **$(6,088)** | **$(2,717)** | **$(2,855)** | **$(11,660)** |

## Income statements

| A$'000 | Three months ended | | Nine months ended | |
|---|---|---|---|---|
| **(unaudited)** | **31-Mar-17** | **31-Mar-16** | **31-Mar-17** | **31-Mar-16** |
| **Revenue** | | | | |
| Revenue from sale of goods | 2,267 | 1,705 | 3,957 | 5,432 |
| Clinical trial cost reimbursements | 1,570 | 2,557 | 5,871 | 6,952 |
| Interest | 159 | 257 | 568 | 915 |
| Drug discovery service fee | - | 248 | 330 | 668 |
| Other | 148 | 87 | 330 | 258 |
| Total revenue | **$ 4,146** | **$ 4,854** | **$ 11,056** | **$14,225** |
| **Expenses** | | | | |
| Employee costs | (2,758) | (2,434) | (8,203) | (7,667) |
| Administration & corporate | (333) | (398) | (1,398) | (1,551) |
| Rent, occupancy & utilities | (301) | (351) | (845) | (975) |
| Clinical trials | (1,409) | (3,127) | (6,807) | (9,502) |
| Drug development | (1,311) | (592) | (2,903) | (1,772) |
| Sales, marketing & distribution | (258) | (220) | (715) | (855) |
| Safety, medical and regulatory affairs | (256) | (450) | (949) | (1,363) |
| Manufacturing purchases | (282) | (243) | (1,015) | (1,143) |
| Other | (752) | (235) | (226) | (934) |
| Depreciation & amortisation | (768) | (757) | (2,291) | (2,273) |
| Foreign currency exchange gains & losses | 1,238 | 969 | 549 | (6) |
| Finance expenses | (155) | (169) | (477) | (516) |
| Total expenses | **(7,345)** | **(8,007)** | **(25,280)** | **(28,557)** |
| **Net profit (loss) before tax** | **(3,199)** | **(3,153)** | **(14,234)** | **(14,331)** |
| Income tax expense | - | - | - | (7) |
| **Net profit (loss) after tax** | **$(3,199)** | **$(3,153)** | **$(14,234)** | **$(14,338)** |