

phormoxis

Investor Presentation | 17 November 2021 Gary Phillips CEO

developing breakthrough treatments for fibrosis and inflammation

Forward looking statement

This document contains forward-looking statements, including statements concerning Pharmaxis' future financial position, plans, and the potential of its products and product candidates, which are based on information and assumptions available to Pharmaxis as of the date of this document. Actual results, performance or achievements could be significantly different from those expressed in, or implied by, these forward-looking statements. All statements, other than statements of historical facts, are forward-looking statements.

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Offer Summary

Offer structure and Size	 Placement to raise approximately \$7.2 million (approximately 68.2 million shares), representing 15% of issued capital, to institutional and sophisticated investors (the Placement) The Placement is issued under the Company's available capacity pursuant to ASX Listing Rule 7.1 Share Purchase Plan (SPP) to raise approximately \$2.0 million to eligible shareholders¹ The Placement and SPP are together referred to as the Offer
Offer Price	 Offer Price of \$0.105 per share represents a: 12.4% discount to the VWAP of \$0.1198 on Friday, 12 November 2021; 12.0% discount to the 5-day VWAP of \$0.1193; and 11.9% discount to the 10-day VWAP of \$0.1191 10.9% discount to the 15-day VWAP of \$0.1178
Use of Funds	Proceeds raised will be used to fund trials and for working capital
Lead Manager	Morgans Corporate Limited
Ranking and Distribution	New shares issued under the Offer will rank pari-passu with existing fully paid ordinary shares on issue

^{1.} Further information regarding the SPP will be provided in the SPP booklet

Indicative Timetable

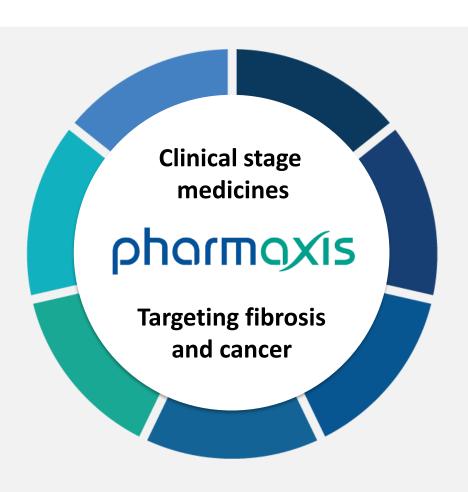
	Event	Date
	Trading Halt	Monday, 15 November 2021
\geq	Record Date for SPP	7.00pm Tuesday, 16 November 2021
	Announce results of the Placement and resume normal trading	Wednesday, 17 November 2021
	Settlement of shares issued under the Placement	Tuesday, 23 November 2021
	Allotment, quotation and trading of shares issued under the Placement	Wednesday, 24 November 2021
	Despatch of the SPP Booklet and SPP Opening Date	Thursday, 25 November 2021
7	SPP Closing Date	5.00pm Wednesday, 15 December 2021
	Announcement of SPP results	Monday, 20 December 2021
	Issue Date	Tuesday, 21 December 2021
	SPP Shares issued under the SPP commence trading on ASX	Wednesday, 22 December 2021
	Despatch of holding statements in respect of the SPP Shares issued under the SPP	In the week commencing Monday, 3 January 2022

Note: Dates and times are indicative only and subject to change without notice. Pharmaxis Ltd reserves the right to alter the dates in this presentation at its discretion and without notice, subject to the ASX Listing Rules and the Corporations Act 2001 (Cth). All dates refer to Sydney, Australia time.



Executive Summary

- Pharmaxis is a clinical stage drug development company targeting fibrosis and cancer indications with first in class or best in class small molecule drugs in markets of high value
- Lead asset PXS-5505 is in phase 2 trial a breakthrough clinical program with disease modifying potential in Myelofibrosis
- IND approval to commence US investigator led phase 2 trial in liver cancer with PXS-5505 as first line treatment added to existing chemotherapy.
- Topical drug PXS-6302 progressing to phase 1c trial in patients with potential to improve function and appearance of scars
- Specific corporate strategy to deliver non-dilutive cash and cost savings from commercial stage mannitol business;
- Pharmaxis is in a strong position to fund its focused clinical program





Shareholders & cash



Financial Information	15 Nov 21
ASX Code	PXS
Share price	\$0.115
Liquidity (turnover last 12 months)	323m shares
Market Cap	A\$52m
Cash balance (30 Sept 2021)	A\$16m
Enterprise value	A\$36m

- Mannitol business* forecast to provide ongoing positive EBITDA growing to \$10m in 5 - 6 years
- R&D tax credits
- Strategy of partnering deals with pipeline assets

Institutional Ownership	30 Sep 21
BVF Partners LP	19%
Karst Peak Capital Limited	12%
D&A Income Limited	7%
Total Institutional Ownership	38%





Experienced Scientific Leadership Team

Significant global experience in drug development, commercialisation and partnering

In senior management



- 20+ years' experience in pharmaceutical drug discovery and published more than 30 peer reviewed articles
- Previously Director of Assay Development and Compound Profiling at the GlaxoSmithKline Centre of Excellence in Drug Discovery in Verona, Italy
- Spent 8 years as post-doc at the Max-Plank Institute in Munich, Germany; Baylor College of Medicine, Houston, Texas; Rammelkamp Centre, Cleveland Ohio; and University of Heidelberg, Germany





Gary Phillips – CEO and Managing Director

- 30+ years' of operational management experience in the pharmaceutical and healthcare industry in Europe, Asia and Australia
- joined Pharmaxis in 2003 and was appointed Chief Executive Officer in March 2013 at which time he was Chief Operating Officer
- Previously held country and regional management roles at Novartis Hungary, Asia Pacific and Australia



Dieter Hamprecht – Head of Chemistry

- 20+ years' experience with small molecule and peptide drug discovery, contributed to greater than 10 drug candidates brought to development and co-inventor of 50 patent families, co-author of 30+ scientific publications
- Previously Managing Director Boehringer Ingelheim's research group in Milan
- Senior medicinal chemistry positions at GSK



Kathleen Metters - Non Executive Director

- Former Senior Vice President and Head of Worldwide Basic Research for Merck
 & Co. with oversight of all the company's global research projects.
- In a subsequent role at Merck & Co she led work on External Discovery and Preclinical Sciences
- Former CEO of biopharmaceutical company Lycera Corp



Brett Charlton - Medical

- 25+ years' experience in clinical trial design and management
- Author of more than 80 scientific papers
- Founding Medical Director of the National Health Sciences Centre
- Previously held various positions with the Australian National University, Stanford University, the Baxter Centre for Medical Research, Royal Melbourne Hospital, and the Walter and Eliza Hall Institute



Neil Graham - Non Executive Director

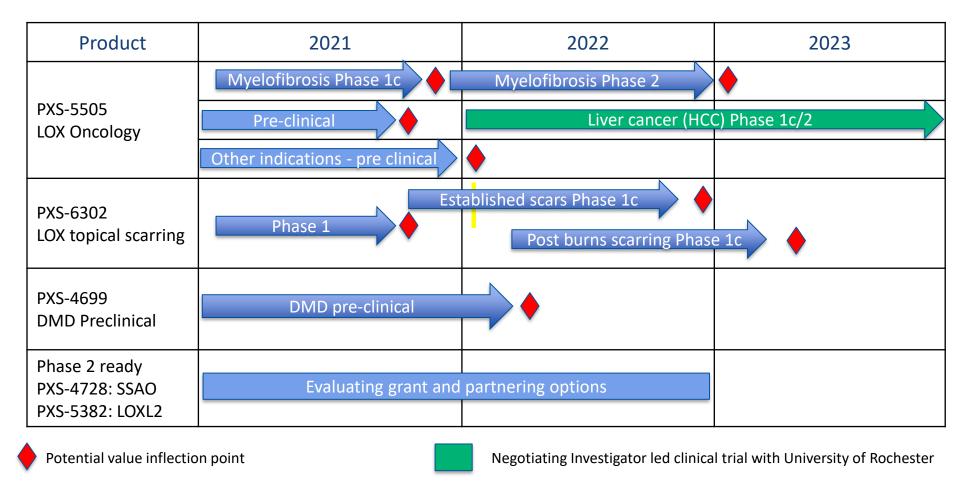
- Former VP of immunology and inflammation responsible for strategic program direction overseeing pipeline development and clinical programs at Regeneron (REGN:US)
- Former SVP program and portfolio management at Vertex Pharmaceuticals
- Former Chief Medical Officer at Trimeris Inc and Tibotec Pharmaceuticals

Multiple potential value inflection points over next two

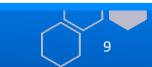
years

Pipeline creates multiple opportunities in high value markets

Target timelines

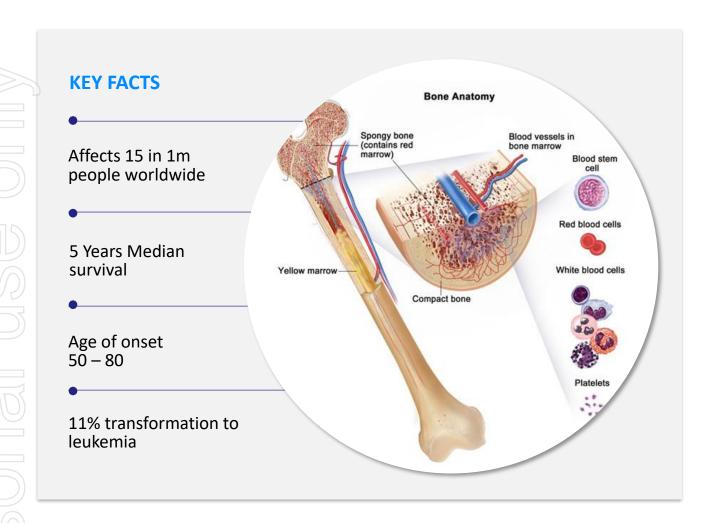






Myelofibrosis background

A rare type of bone marrow cancer that disrupts your body's normal production of blood cells



Primary Myelofibrosis is caused by a build up of scar tissue (fibrosis) in bone marrow reducing the production of blood cells:

- Driven by clonal mutations of a hematopoietic stem cell (JAK, MPL, CALR genes)
- Reduced red blood cells can cause extreme tiredness (fatigue) or shortness of breath
- Reduced white blood cells can lead to an increased number of infections
- Reduced platelets can promote bleeding and/or bruising
- Spleen increases blood cell production and becomes enlarged
- Other common symptoms include fever, night sweats, and bone pain

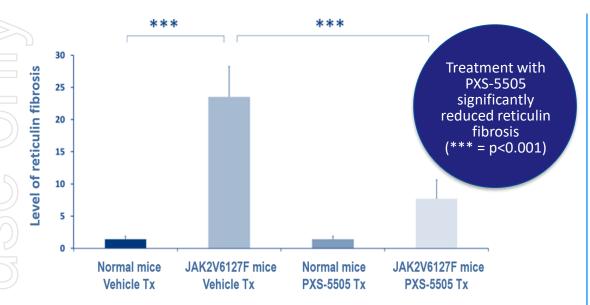
Standard of Care; JAK inhibition

- Current standard of care; revenue ~US\$1b per annum
- Symptomatic relief plus some limited survival improvement. 75% discontinuation at 5 years
- Median overall survival is 14 16 months after discontinuation

PXS-5505; An effective and safe inhibitor of LOX in myelofibrosis

patientsPre clinical and clinical studies strongly support entry into long term phase 2 patient studies

PXS-5505 attenuates hallmarks of primary myelofibrosis in mice

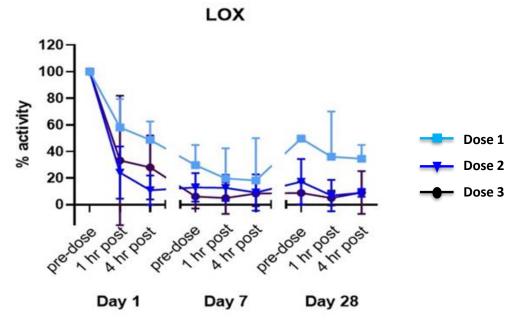


"None of the drugs approved to date consistently or meaningfully alter the fibrosis that defines this disease. PXS-5505 has a novel mechanism of action by fully inhibiting all LOX enzymes.

Preliminary data thus far, demonstrate that PXS-5505 leads to a dramatic, >90% inhibition of LOX and LOXL2 at one week and 28 days. This confirms what's been shown in healthy controls as well as mouse models, that this drug can inhibit the LOX enzymes in patients. Inhibiting these enzymes is a novel approach to the treatment of myelofibrosis by preventing the deposition of fibrosis and ultimately reversing the fibrosis that characterizes this disease"

Dr Gabriela Hobbs¹

PXS-5505 – Phase 1c dose escalation in MF patients



- Open label dose expansion in JAK-inhibitor unsuitable² primary MF or post-ET/PV MF patients
- Maximum of 3 patients on each dose for 28 days
- Good safety profile with no adverse events at highest dose
- >90% inhibition of LOX and LOXL2 at trough on highest dose at day 7 and 28



¹ Assistant Professor, Medicine, Harvard Medical School & Clinical Director, Leukaemia, Massachusetts General Hospital

PXS-5505 Phase 1/2a Trial in myelofibrosis

6 month monotherapy study with meaningful safety and efficacy endpoints (phase 1c complete)

Australia)

STUDY POPULATION **DESIGN** TREATMENT COHORT **ENDPOINTS** JAK-inhibitor unsuitable* Phase 1/2a open **Dose escalation: Primary:** Safety TEAEs label study to primary MF or post-ET/PV **PXS-5505** evaluate safety, MF patients with: 3 ascending doses, 4 weeks **Secondary:** PK/PD, and efficacy (n = 3 to 6 subjects/dose)PK/PD • INT-2 or High risk MF **BMF** Grade requiring therapy **IWG** Response Symptomatic **SVR** BMF Grade 2 or greater **Cohort expansion:** Haematology PXS-5505 (n = 24 subjects) 26 weeks Symptom score Multiple sites across Study recruitment FDA granted orphan drug 4 countries to enhance commenced Q1 2021, designation July 20 and IND trial recruitment Study budget (~US\$6m) study targeted to conclude approved August 2020 (USA, South Korea, Taiwan,

*Unsuitable = ineligible for JAKi treatment, intolerant of JAKi treatment, relapsed during JAKi treatment, or refractory to JAKi treatment. JAKi – Janus Kinase inhibitor, MF myelofibrosis, ET Essential Thrombocythaemia, PV polycythaemia vera, INT intermediate,

BMF bone marrow fibrosis, RP2D recommended phase 2 dose, TEAE treatment emergent adverse event, PK pharmacokinetics, PD pharmacodynamics, SVR spleen volume response, IWG International Working Group Myeloproliferative Neoplasms



H2 2022

Myelofibrosis - examples of other programs

PXS-5505 unique mechanism of action designed for disease modification and good tolerability

Company	Market cap ⁽¹⁾	Bourse	Asset	Description	Clinical phase
KEROS THERAPEUTICS	\$0.9bn	Nasdaq	KER-050	TGF-β ligand trap	Phase 2
Constellation PHARMACEUTICALS	\$1.6bn	Nasdaq	CPI-0610	BET inhibitor	Phase 3
KARTOS THERAPEUTICS	\$0.7bn ⁽²⁾	n.a. – private	KRT-232	MDM2 antagonist	Phase 3
geron	\$0.4bn	Nasdaq	Imetelstat	Telomerase inhibitor	Phase 3
phormoxis	\$43m (A\$57m)	ASX	PXS-5505	LOX inhibitor	Phase 1c/2 commenced

PXS-5505 unique mechanism of action expected to deliver additional efficacy on top of existing standard of care and/or known pipeline drugs without adding to tolerability issues



PXS-5505: Significant opportunity in other cancers

Global academic and clinical interest in LOX inhibition drives development plan

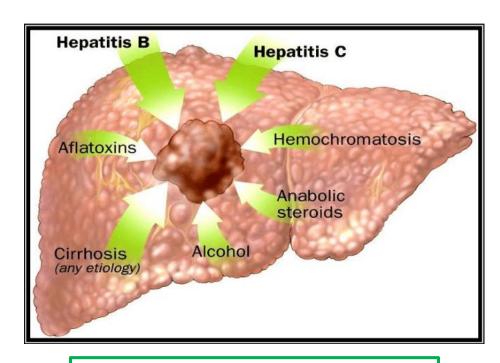
Normal tissue **Pharmaxis Research Collaborations** Myelodysplastic syndrome Collagen Germany **Liver Cancer** Tumour with fibrotic tissue has Rochester (NY) increased tissue stiffness Increased interstitial pressure **Pancreatic Cancer** Sydney, Rochester (NY) Increased Increased angiogenesis **EMT** Melanoma and glioblastoma Houston Increased Decreased Increased **Head and Neck Cancer** drug perfusion Invasion tumour growth Boston, (MA)

Multiple expected benefits from inhibition of LOX enzymes

Hepatocellular Carcinoma (HCC)

4th leading cause of cancer-related mortality worldwide with a 19.6% 5-year relative survival

- Primary liver malignancies have doubled in incidence over the last two decades.
- 4th leading cause of cancerrelated mortality worldwide with a 19.6% 5-year relative survival
- Accumulation of collagen cross-links increases stromal stiffening and interstitial fluid pressure reducing delivery of chemotherapy and immunotherapy
- Current standard of care
 20-30% are resectable at presentation with many patients relying on systemic therapy:
 - Tyrosine kinase inhibitorsPD-L1 inhibitors + anti-VEGF



Commercial Opportunity

Drugs market currently worth ~US\$2bn with rising incidence forecasted to drive growth to ~US\$7bn by 2027

- Pre-clinical data (Rochester Uni; Aug 2021)
 - Tumour tissue specimens show LOX enzymes are significantly elevated in human liver cancer and correlate with poor prognosis.
 - PXS-5505 with or without chemotherapy treatment in a pre-clinical model significantly improves survival, delays tumor growth, and reduces intratumoral pressure.
- Proposed clinical strategy
 - Enhance the intratumoral response to standard of care through the addition of LOX inhibition in human HCC
 - 6 month study combination PXS-5505 on top of standard of care in newly diagnosed unresectable or metastatic hepatocellular carcinoma
 - Investigator led trial University of Rochester. Cost ~US\$2.5m



Hypertrophic and keloid scarring

Cutaneous scarring following skin trauma or a wound is a major cause of morbidity and disfigurement

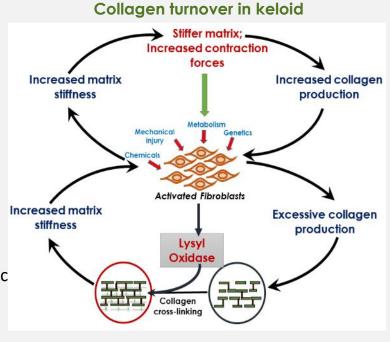
KEY FACTS

100m patients develop scars in the developed world alone each year as a result of elective operations and operations after trauma

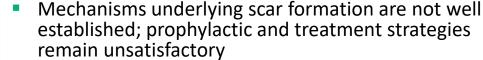
Hypertrophic scars and keloids are fibroproliferative disorders that may arise after any deep cutaneous injury caused by trauma, burns, surgery, etc.

Hypertrophic scars and keloids are cosmetically and functionally problematic significantly affecting patients' quality of life

Total scar treatment market in 2019 exceeded US\$19b. Keloid and hypertrophic scar segment ~US\$



The increase in extracellular matrix is a key factor and this depends on collagen and elastin cross-linking to make them less degradable.



Current standard of care includes:

- Corticosteroids
- Surgical revision
- Cryotherapy
- Laser therapy
- 5-fluorouracil



Pre clinical evidence

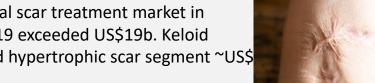
Treatment with PXS-6302 monotherapy demonstrates cosmetic and functional improvements to scarring in pre clinical models (data on file)

Clinical evidence

1 month phase 1a in healthy volunteers demonstrates good tolerability and full inhibition of LOX in skin.

Next Steps

- 3 month study versus placebo in patients with established scars to commence Q4 2021
- Study to investigate scarring subsequent to burn injury to follow in 2022





Anticipated news flow: 2021 - 2022

Multiple anticipated value inflection points

PXS-5505 – anti cancer drug

- PXS-5505 phase 1c liver cancer (HCC) study starts recruitment
- PXS-5505 phase 2a myelofibrosis study fully recruited
- PXS-5505 phase 2a myelofibrosis study safety and efficacy data
- PXS-5505 publications by KOL's in other cancers
- Results of Charlie Teo Foundation funded research into PXS-5505 in glioblastoma

PXS-6302 - scar treatment

- LOX topical drug PXS-6302 commences independent investigator patient studies - established scars
- LOX topical drug PXS-6302 commences independent investigator patient studies - burns scars
- LOX topical drug PXS-6302 patient studies fully recruited – established and burns scars
- PXS-6302 publications by KOL's in scarring

Other

- Mannitol business simplification realising annual cost savings
- Mannitol business appointment of new distributors
- Decision by Aptar whether to exercise (by Aug 22) option to license high payload inhaler for US\$2.5m plus royalties





phormoxis

developing breakthrough treatments for fibrosis and inflammation

Pharmaxis Ltd ABN 75 082 811 630 www.pharmaxis.com.au





Contacts

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David McGarvey Chief Financial Officer david.mcgarvey@pharmaxis.com.au



Mannitol respiratory business (Bronchitol® and Aridol®)

Transformational impact of FDA Bronchitol approval (Oct 2020) – business segment cash flow positive from FY 2021 onwards

Sales

- Bronchitol > 75% of sales
- Strong short term growth from Russia
- Sales growth expected in approved markets as patients access hospitals again post COVID-19 restrictions
- Strong longer term growth contribution expected from US

Expenses

- Relatively fixed production cost base
- Potential for simplified business model to reduce costs

Segment EBITDA

- Forecast ongoing positive EBITDA
- US volumes contribute to mannitol segment generating profit



Bronchitol in US

 US CF market >65% of global market in value

US market doubles global cystic fibrosis patient opportunity with attractive pricing

- Chiesi approval /launch milestone payments US\$10m received FY 2021
- US sales commenced in Q2 CY 2021

 delay in patient initiation due to
 COVID
- High teens % of Chiesi sales + supply contract - ~20% of Chiesi US Bronchitol net sales flow directly to the Pharmaxis bottom line
- Three sales milestones totaling US\$15m payable on achieving annual sales thresholds



Board

Significant international pharmaceutical experience



Malcolm McComas - Chair

- Former investment banker and commercial lawyer
- Former MD Citi Group
- Has worked with many high growth companies across various industry sectors and has experience in equity and debt finance, acquisitions and divestments and privatisations
- Joined Pharmaxis Board in 2003
- Chair since 2012



Will Delaat - Non-Executive Director

- 35+ years' experience in the global pharmaceutical industry
- Former CEO of Merck Australia
- Former chair of Medicines Australia and Pharmaceuticals Industry Council
- Joined Pharmaxis Board in 2008



Dr Kathleen Metters - Non-Executive Director

- Former Senior Vice President and Head of Worldwide Basic Research for Merck & Co. with oversight of all the company's global research projects
- In a subsequent role at Merck &Co she led work on External Discovery and Preclinical Sciences
- Former CEO of biopharmaceutical company Lycera Corp



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- Former VP of immunology and inflammation responsible for strategic program direction overseeing pipeline development and clinical programs at Regeneron (REGN:US)
- Former SVP program and portfolio management at Vertex Pharmaceuticals
- Former Chief Medical Officer at Trimeris Inc and Tibotec Pharmaceuticals

Financials

Income statement highlights

Periods ended (A\$'000)	Sept 2021 Qtr	Sept 2020 Qtr	June 2021 FY	June 2020 FY
Segment Financials				
New drug development				
Oral LOX (external costs)	(1,467)	(777)	(2,521)	(3,124)
Other program external costs (net of grants)	(303)	(297)	(1,850)	(3,315)
Employee costs	(715)	(924)	(3,270)	(3,373)
Overhead	(102)	(93)	(395)	(460)
R&D tax credit	_	148	148	5,159
EBITDA	(2,587)	(1,943)	(7,888)	(5,113)
Mannitol respiratory business				
Sales	3,272	661	6,680	7,027
Other revenue and income	2,342	142	15,985	20
	5,614	803	22,665	7,047
Expenses – employee costs	(1,197)	(1,385)	(5,558)	(5,855)
Expenses – manufacturing purchases	(1,205)	(71)	(1,168)	(1,456)
Expenses – other	(1,103)	(1,212)	(4,483)	(3,713)
EBITDA	2,109	(1,865)	11,456	(3,977)
Corporate – EBITDA	(755)	(860)	(3,795)	(2,990)
Total Adjusted EBITDA	(1,233)	(4,668)	(\$227)	(\$12,080)
Net profit (loss)	(3,179)	(4,981)	(\$2,970)	(\$13,943)

Financials

Cash

Periods ended (A\$'000)	Sept 2021 Qtr	Sept 2020 Qtr	June 2021 FY	June 2020 FY
Cash				
Cash period end	16,131	9,656	18,712	14,764
Cash Flow Statement Highlights Operations				
Receipts from customers	1,156	1,934	7,242	7,775
R&D tax incentive	-	-	5,433	6,271
Chiesi milestone	-	-	13,845	-
Sale of distribution rights	2,342	-	1,365	-
Payments to suppliers, employees etc				
(net)	(5,443)	(6,300)	(24,813)	(27,330)
Total operations	(1,945)	(4,366)	3,072	(13,284)
Investing (capex & patents)	(40)	(100)	(644)	(574)
Finance lease payments ¹	(593)	(574)	(2,305)	(2,232)
Financing agreement payments ²	(3)	(68)	(240)	(270)
Share issue - net		-	4,065	-
Net increase (decrease) in cash	(\$2,581)	(\$5,108)	\$3,948	(\$16,360)

- 1. Lease over 20 Rodborough Rd (to May 2024) total liability at 30 June 2021: \$6.3 million
- 2. NovaQuest financing not repayable other than as % of US & EU Bronchitol revenue up to 7 years

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