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. 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. 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.
Business overview

Built to deliver value

Drug development
- Focus on fibrosis and inflammation
- Strong Pharma interest in validated small molecule technology platform
- Three additional drugs acting on high value targets
  - LOXL2 inhibitor phase 1 ready in H1 2017
  - Two other drugs commencing preclinical tox in H1 2017

Management
- Management and Board with global experience & Pharma network
- Proven capability of executing global BD with major partners
- In house capability to run multi-centre international trials

Partnerships
- First drug out licensed to Boehringer Ingelheim in globally competitive deal - total potential deal >A$750m
- Boehringer developing second indication
- Synairgen collaboration for LOXL2
- Significant value milestones from existing partner deals near term
- Pipeline providing multiple future opportunities

Financial strength
- A$29m cash balance at Dec 2016; average annual cash usage $1.4m/month
- Boehringer NASH phase 2 initiation milestone expected Q2 2017 €18m
- Boehringer phase 2 milestone for second indication
- Significant value milestones from existing partner deals near term
- Pipeline providing multiple future opportunities

Note: Market Cap as of 9/02/17
Senior management

Significant experience in drug development, commercialisation and partnering

Gary Phillips – CEO
- more than 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

Wolfgang Jarolimek – Drug Discovery
- more than 18 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

David McGarvey – CFO
- more than 30 years’ experience building and funding Australian based companies from inception to globally successful enterprises
- joined Pharmaxis as Chief Financial Officer and Company Secretary in December 2002
- commenced career at PriceWaterhouseCoopers

Kristen Morgan – Alliance Management
- responsibility for alliance management and medical and regulatory affairs
- more than 19 years’ experience in the pharmaceutical industry having previously held a senior role in medical affairs at Sanofi-Aventis, and a commercial sales role at GlaxoSmithKline.

Brett Charlton - Medical
- more than 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

Board of Directors
- Malcolm McComas – Chair
  - former investment banker at Grant Samuel, County Natwest and Morgan Grenfell
- Gary Phillips – Managing director
- Will Delaat – Non executive director
  - former CEO of Merck Australia
  - former chair of Medicines Australia
- Simon Buckingham – Non executive director
  - former President Global Corporate and Business Development at Actellon
# Pharmaxis product portfolio

<table>
<thead>
<tr>
<th>Indication</th>
<th>Discovery</th>
<th>Lead Optimisation</th>
<th>Pre Clinical</th>
<th>Phase I</th>
<th>Phase II</th>
<th>Phase III</th>
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<td>Cystic fibrosis</td>
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<td>Cystic fibrosis</td>
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<td>LOXL-2 (other)</td>
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<td>Scarring+</td>
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<td>Seeking Partners</td>
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</table>
Drug discovery

Applying amine oxidase chemistry to inflammation and fibrosis

Amine oxidase enzymes are well validated as targets in diseases with a high unmet medical need
Pharmaxis drug discovery strategy

Building a biotech powerhouse in fibrosis and inflammation

**Strategy**

**Drug discovery:**
- Prioritise validated targets
  - Multiple small molecule drugs from in-house amine oxidase chemistry platform
- Develop to phase 1 or 2

**Partnering:**
- Create value via:
  - Licence out to Big Pharma with attractive 1st in class drugs post phase 1 or 2
  - Collaborate to de-risk and accelerate PXS programs
  - Collaborate on in-licensing programs

**Achievements to date**

**Drug discovery:**
- First in class SSAO inhibitor drug taken to phase 1. Initial indication NASH. Partner developing second indication.
- One lead candidate recently entered preclinical
- Two lead candidates to enter preclinical H1 2017

**Partnering:**
- In house BD expertise achieves valuable deal with Boehringer Ingelheim - A$39m upfront, total potential > A$750m
- Collaboration with Synairgen Research plc for early stage fibrosis program to widen spread of indications, enhance time to value inflection and spread risk
Drug discovery

Our therapeutic focus is inflammation and fibrosis

Pharmaxis drug discovery

- NASH, liver, kidney and cardiac fibrosis (LOXL2)
- Respiratory, cardio vascular (SSAO/MPO)
- Neuro inflammation – Alzheimer’s, Parkinson’s, stroke (SSAO/MAO-b)
- Scarring (LOX)

Collaborations allow us to leverage our platform without losing focus

Collaboration with Synairgen

- Pulmonary fibrosis (LOXL2)

Exploratory academic collaborations (LOX/LOXL2)

- Cancer
9

30-40% of US population have steatosis (fatty liver)
5-10% progress to NASH (Non-alcoholic steatohepatitis)
3-5% progress to hepatocellular carcinoma

Potential Insults

Quiescent State
(healthy liver)

Inflammatory State
5-10% progress to NASH
(Non-alcoholic steatohepatitis)

Fibrotic State
30-38% progress to fibrosis
3-5% progress to hepatocellular carcinoma
Drugs targeting NASH → Cirrhosis

- Neutrophils
- Macrophage
- Endothelial cells

Virus / bacteria → Activated stellate cell
Diabetes → Activated stellate cell
High-fat diet → Activated stellate cell

Chemo/cytokines

- PXS4728 SSAO inhibitor
- Reactive oxygen species
- LOXL2 inhibitor
- Collagen
- LOXL2
- MMPs
- TIMP
- Cross links
- Collagen fibrils

Hepatocytes

Metabolic Modifiers
Anti-Inflammatory Drugs
Anti-Fibrotic Drugs
# Drugs in the clinic targeting NASH

Several large Pharma companies seeking to build competitive portfolios

<table>
<thead>
<tr>
<th>Company</th>
<th>Metabolic modifiers</th>
<th>Anti-inflammatory</th>
<th>Anti-fibrotic</th>
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<tr>
<td>Intercept</td>
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<td>Galmed</td>
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<td>Ph 2</td>
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<td>Gilead</td>
<td>Ph 2 x 2</td>
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<td>BMS</td>
<td>Ph 2</td>
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<td>Ph 1</td>
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<td>Galectin</td>
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<td>Immuron</td>
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<td>Shire</td>
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<tr>
<td>Other</td>
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<td>Ph 2 x 3</td>
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</tbody>
</table>
SSAO for NASH

SSAO inhibitor PXS4728A sold to Boehringer Ingelheim in May 2015

**PXS-4728A**

- **Mechanism based inhibitor of SSAO**
  - Small molecule inhibitor of SSAO (VAP-1)
  - Important inflammatory pathway in several diseases including NASH and COPD

- **Development status**
  - Pharmaxis discovery – patent filed 2012
  - Effective in pre clinical models of NASH and airway inflammation
  - Phase 1 study reported
    - orally bioavailable
    - long lasting enzyme inhibition after single dose
    - progressive dose response
  - Phase 2 scheduled H1 2017

**Competitive deal with Boehringer**

- **Total potential payments to approval for 2 indications: €418.5m (~A$600m)**
  - Upfront (May 2015): €27.5m (~A$39m)
  - Commencement of phase 2 and 3: up to total €55m (~A$80m)
  - Filing, regulatory & pricing approvals: up to total €140m (~A$200m)
  - Second indication: additional total milestone payments (€195m)

- **Earn-out payments on annual net sales**
  - Tiered percentages increasing from high single digits
  - Plus potential sales milestones

External validation of PXS drug discovery and ability to negotiate valuable global deals
LOXL2 inhibition for NASH & other fibrotic diseases

An attractive target and development program

- Potential indications:
  - NASH / Liver Fibrosis
  - Pulmonary fibrosis (IPF)
  - Cancer
  - Kidney
  - Cardiac fibrosis

- Development status:
  - Pharmaxis discovery – patent filed 2016
  - Effective in pre clinical models of fibrosis and cancer
  - Candidate compounds identified
  - Preclinical toxicity studies commenced Q4 2016 (significant de-risking step)

- Competitive profile:
  - Novel target and mechanism of action
  - Once daily oral drug
  - Complete inhibition of LOXL2 versus partial inhibition by antibody
  - Selective inhibition over other amine oxidases Low cost of goods

Fibroblast cells in human tissue

Excessive production and linking of collagen fibres results in fibrosis

Collagen fibres

Excessive ‘cross-linking’ of collagen fibres, stiffens tissue, causing fibrosis

LOXL2 (from fibroblasts)
LOXL2 for pulmonary fibrosis

Collaboration with Synairgen

Idiopathic Pulmonary Fibrosis (IPF)
- IPF primarily affects people over the age of 50
- 5,000 patients have IPF in Australia
- 100,000 people with IPF in the US
- Prognosis is worse than that of many cancers
- Two drugs approved recently
  - Nintedanib (Boehringer Ingelheim)
  - Pirfenidone (Roche)
- Need for new therapies
- Current products expected to produce global revenues > $1.1 billion by 2017

Synairgen collaboration
- Access to
  - Synairgen’s strength in fibrosis biology and respiratory clinical development - BioBank human tissue models technology platform
  - Clinical expertise at University of Southampton
- Faster time to value appreciation and partnering points of phase 1 or 2a
- Synairgen to fund pre clinical tox and phase 1
- Shares risk and reward based on investment in program
- Revenue share for IPF phase 1 partnering deal: 50/50
- Larger value partnering deal(s) from additional indications
# Fibrosis and NASH M&A

Attractive deal values for phase 1 and phase 2 clinical assets

<table>
<thead>
<tr>
<th>Acquirer</th>
<th>Company</th>
<th>Indication</th>
<th>Deal Type</th>
<th>Stage</th>
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<th>Potential (US$M)</th>
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<td>&lt; 2 years ago</td>
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<td>Gilead</td>
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<td>P1</td>
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<td>Allergan</td>
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<td>BMS</td>
<td>Promedior</td>
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<td>BMS</td>
<td>Nitto Denko</td>
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<td>Boehringer</td>
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<td>License</td>
<td>Discovery</td>
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<td>Boehringer</td>
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<td>NASH - inflammation</td>
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<td>&gt; 2 years ago</td>
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<td>License + equity</td>
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</table>
Bronchitol for cystic fibrosis

Overview

Cystic fibrosis
- Patients
  - US: 30,000;
  - Europe: 37,000;
  - Rest of world: 21,000
- Disease characterised by poorly hydrated, tenacious, thick mucus
- Rapid decline in lung function
- Frequent infections

Bronchitol
- Active ingredient mannitol delivered as an inhalable dry powder
- Restores airway surface liquid
- Mucus clearance enhanced
- Improves lung function
- Reduces incidence of lung infections

CF301/2 trial (adult)
- Total 317 adults
- FEV1
  - CF301; p=0.001
  - CF302; p=0.038
  - Pooled; p=0.001 rel % change = 4.7%
- Exacerbations
  - Pooled data
  - 26% reduction
  - 60% reduction in Bronchitol responders

CF204 trial results
- Paediatric age 6-17
  - Placebo-controlled
  - 8 weeks crossover design
  - standard therapy continued
- Primary endpoint:
  - Absolute change in FEV1: 3.42%; p=0.004
- Key secondaries
  - Absolute change in FEF25-75: 5.75%
  (p=0.005)
- Acceptable safety profile
  - Exacerbations and lung infection reduced by 25%
Bronchitol for cystic fibrosis

Partnering for success

**US market**
- Largest CF market by value
- 28,103 CF patients
- 49.7% adults
- Bronchitol price target US$20k per patient/year
- 7 year post launch market exclusivity

**US partner: Chiesi**
- Fund CF303 up to US$22m
- ~A$13m milestone payment on launch, plus sales milestones
- High mid teens royalty % on in-market sales
- Mid teens % uplift on COGs
- Chiesi responsible for regulatory filing & commercialisation

**US trial: CF303**
- Tie-breaker phase 3 trial commenced Q4 2014, managed by PXS
- 423 adult patients
  - 21 countries
  - 126 sites
- Design
  - Full consultation with FDA
  - Similar design to CF301/2
- Fully recruited July 2016
- Results Q2 2017

**Rest of world**
- Sold by Chiesi in UK & Germany
- Sold by PXS in Australia & Denmark
- Russian approval received Oct 2016 – first sale Q1 2017
- Pending approval/pricing/distributor appointments in Israel, Turkey, Brazil, Eastern Europe countries
## Financial highlights

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<tr>
<td><strong>Income statements</strong></td>
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<td>Sales revenue</td>
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<td>Total revenue</td>
<td>1,900</td>
<td>4,551</td>
<td>6,910</td>
<td>9,372</td>
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<tr>
<td>Total expenses</td>
<td>(8,850)</td>
<td>(9,567)</td>
<td>(17,945)</td>
<td>(20,550)</td>
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<td>Net profit (loss) after tax</td>
<td>(6,950)</td>
<td>(5,016)</td>
<td>(11,035)</td>
<td>(11,185)</td>
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<td><strong>Segment results – adjusted EBITDA</strong></td>
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<td>Bronchitol &amp; Aridol</td>
<td>(2,489)</td>
<td>(3,334)</td>
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<td>New drug development</td>
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<td>(863)</td>
<td>(2,421)</td>
<td>(1,847)</td>
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<td>Corporate</td>
<td>(850)</td>
<td>(1,330)</td>
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<td>(1,753)</td>
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<td>Total</td>
<td>(4,642)</td>
<td>(5,527)</td>
<td>(8,440)</td>
<td>(8,085)</td>
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<td><strong>Statement of cash flows</strong></td>
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<td>Cash inflow/ (outflow) from:</td>
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<td>Operations</td>
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<td>(646)</td>
<td>(214)</td>
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<td>Financing activities</td>
<td>(430)</td>
<td>(430)</td>
<td>(856)</td>
<td>(872)</td>
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<td>Total cash used</td>
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<td>(9,964)</td>
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<td><strong>Cash at bank</strong></td>
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<td>29,245</td>
<td>45,936</td>
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Refer December 2016 Quarterly Shareholder Update for additional financial information.
Balance sheet – 31 December 2016

- Finance lease over 20 Rodborough Rd (to 2024)
- NovaQuest financing – not repayable other than as % of Bronchitol revenue
Shareholders (31 Dec 16)

- Shares on issue: 319m
- Employee options: 9.9m
- Institutional shareholders ~50%:
  - Australia - Orbis (16%); Australian Ethical (6%); Other (1%)
  - US - BVF Partners (14%); Other (2%)
  - UK - Montoya Investments (6%); Other (3%)

Shares traded to 31 Dec
- Three months: 12m
- Six months: 34m
- Year: 78m

Market capitalisation
- A$96m (9 February 17)
## News flow

<table>
<thead>
<tr>
<th>Boehringer Ingelheim</th>
<th>CY 2017</th>
<th>CY 2018</th>
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<tbody>
<tr>
<td></td>
<td>PXS4728A Phase 2 commences &amp; ~A$25M milestone payable (H1)</td>
<td>Br advise timetable for second indication</td>
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<td>pharmaxis</td>
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<td>Bronchitol – RoW</td>
<td>EU Paediatric label extension application</td>
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<td>Russian sales commence</td>
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<td>Chiesi</td>
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<td>Bronchitol – US</td>
<td>CF303 – trial completion (Q1)</td>
<td>Bronchitol approval</td>
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<td></td>
<td>CF303 – top line results (Q2)</td>
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<tr>
<td>pharmaxis</td>
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<tr>
<td>New drug development</td>
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</tbody>
</table>

- **LOXL-2**
  - Complete GLP tox program for ≥1 compounds
  - Commence ≥1 phase 1 studies
  - Complete 1 phase 1 study
  - Partner ≥1 compound

- **SSAO/MPO**
  - Commence GLP tox program
  - Complete GLP tox program – phase 1 ready
  - Commence phase 1 study

- **LOX**
  - Commence GLP tox program
  - Commence phase 1 study

- **LOXL-2**
  - Leading universities/academics assessing in kidney fibrosis, cancer and wound healing

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## Pharmaxis opportunities for growth

### Building a biotech powerhouse in fibrosis and inflammation

<table>
<thead>
<tr>
<th>SSAO program for NASH (fatty liver)</th>
<th>LOXL2 program</th>
<th>Discovery pipeline</th>
<th>Bronchitol for CF</th>
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</thead>
<tbody>
<tr>
<td>NASH: US$35B market by 2025</td>
<td>NASH market &gt;$35B</td>
<td>LOX</td>
<td>Access large US CF market with Chiesi</td>
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<tr>
<td>Acquired by BI at phase 1 for A$39m upfront, total &gt;A$750m</td>
<td>Pulmonary fibrosis: market &gt;$1B</td>
<td>– Scarring and severe fibrosis</td>
<td>– CF303 trial reports in Q2 17</td>
</tr>
<tr>
<td>BI to develop for NASH and other inflammatory indications (eg. kidney fibrosis, COPD)</td>
<td>Strong big Pharma interest in LOXL2 and PXS chemistry</td>
<td>– Commence preclinical H1 2017</td>
<td>– ~A$13m milestone payments on launch</td>
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<tr>
<td>Next milestone: ~A$25m at start of phase 2 – Q2 2017</td>
<td>Formal preclinical commenced Q4 2016</td>
<td>SSAO/MPO</td>
<td>– High teens % share of in-market sales</td>
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<td>Next step – phase 1 ready Q2 2017</td>
<td>– Respiratory and cardiovascular inflammation</td>
<td>Growth from existing markets including Russia</td>
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<td>Synairgen collaboration increases value and shares risk</td>
<td>– Commence preclinical H1 2017</td>
<td>– sales commenced Q1 17</td>
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<td>New RoW markets opening over next 24 months</td>
</tr>
</tbody>
</table>
Conclusions

- **Strong therapeutic focus** in area of high unmet medical need and increasing interest to big Pharma
- **Productive R&D engine** and capacity to run multi-centre international studies
- **Track record** of value adding business development
- **Strong news flow** over the next 12 months
- **Strong balance sheet** – A$29m cash at December 16 with a likely milestone of A$25m due in Q2 17