pharmaxis

Investor Presentation

Gary Phillips CEO

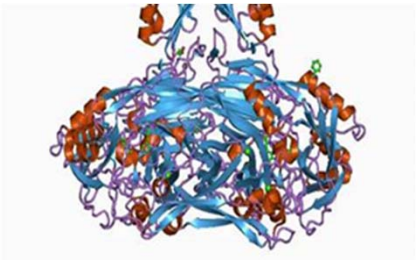
6 September 2016

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 approaching the clinic over next 24 months

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

Boehringer Ingelheim

Chiesi

People and ideas for innovation in healthcare

Partnerships

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

Financial strength

- A$39m cash balance at June 2016; average monthly cash usage $1.3m
- Boehringer phase 2 initiation milestone expected Q1 2017 ~A$25m
- Market cap $94M*
- institutional investor's >45%
- Increasing Bronchitol sales globally in new and existing markets

* Note: Market Cap as of 31/08/16

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

David McGarvey – CFO

- more than thirty 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
- previously Chief Financial Officer of the Filtration and Separations Division of US Filter (1998-2002), and Memtec Limited (1985-1998)
- commenced career at PriceWaterhouseCoopers

Brett Charlton - Medical

- more than 15 years experience in clinical trial design and management
- author of more than 60 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

Wolfgang Jarolimek – Drug Discovery

- more than 15 years' experience in pharmaceutical drug discovery and published more than 20 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

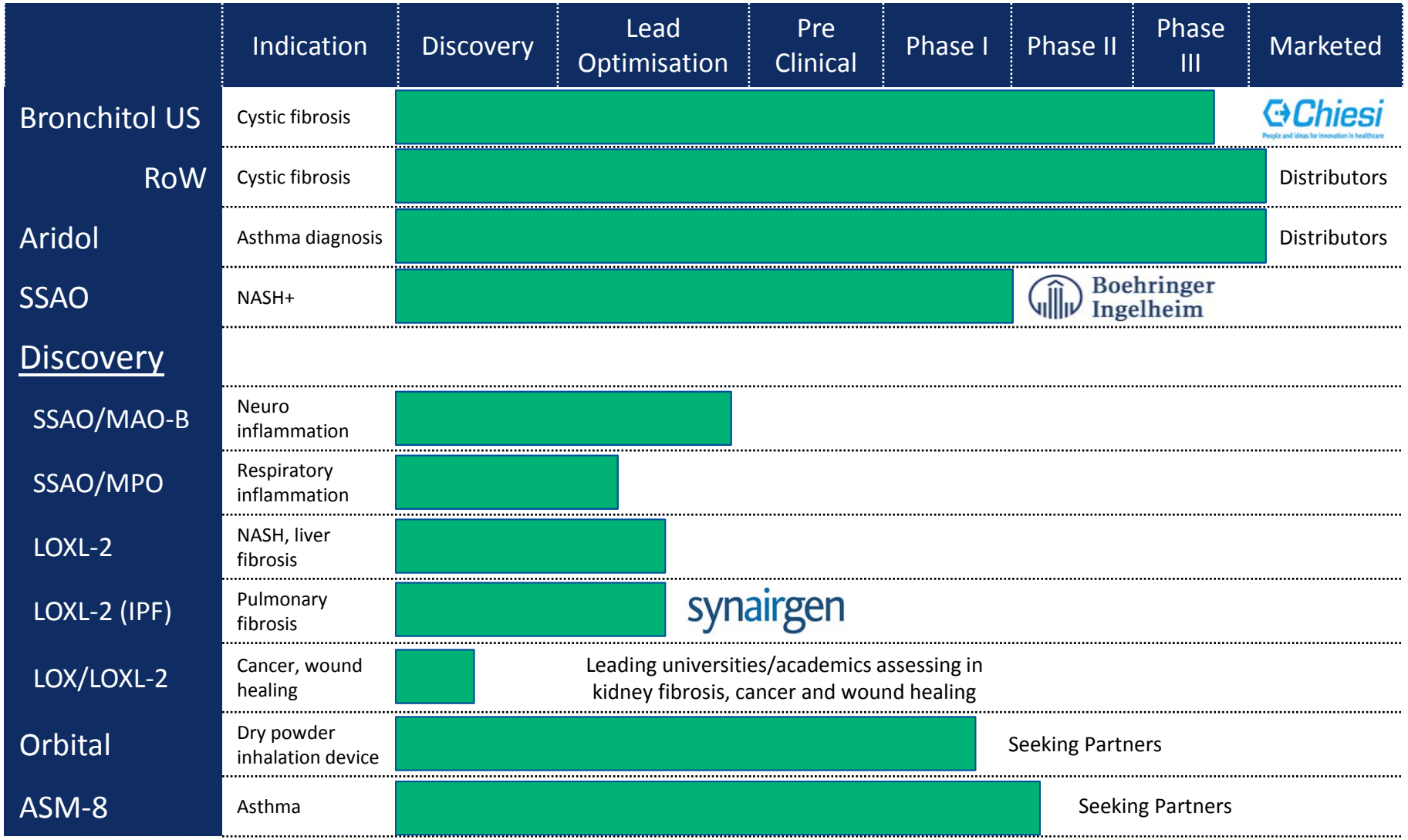
Kristen Morgan – Alliance Management

- 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.

Board of Directors

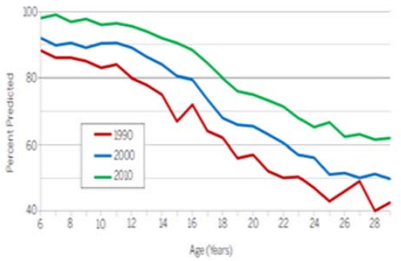
- **Malcolm McComas** – *Chair*
  - former investment banker at Grant Samuel, County Natwest and Morgan Grenfell
- **Gary Phillips** – *Managing director*
- **Will Delaat** – *Non executive director*
  - former CEO of Merck Australia
  - former chair of Medicines Australia
- **Simon Buckingham** – *Non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

Pharmaxis product portfolio

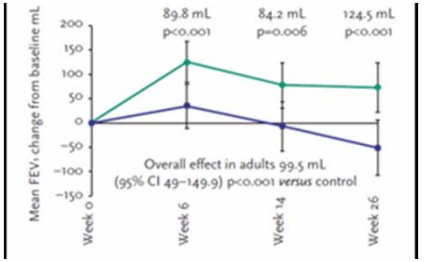


Bronchitol for cystic fibrosis

Overview



Median FEV_1 % Predicted versus Age



Pooled adult data from CF301 and CF302

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%

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Bronchitol for cystic fibrosis

Partnering for success

Chiesi
People and ideas for innovation in healthcare

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

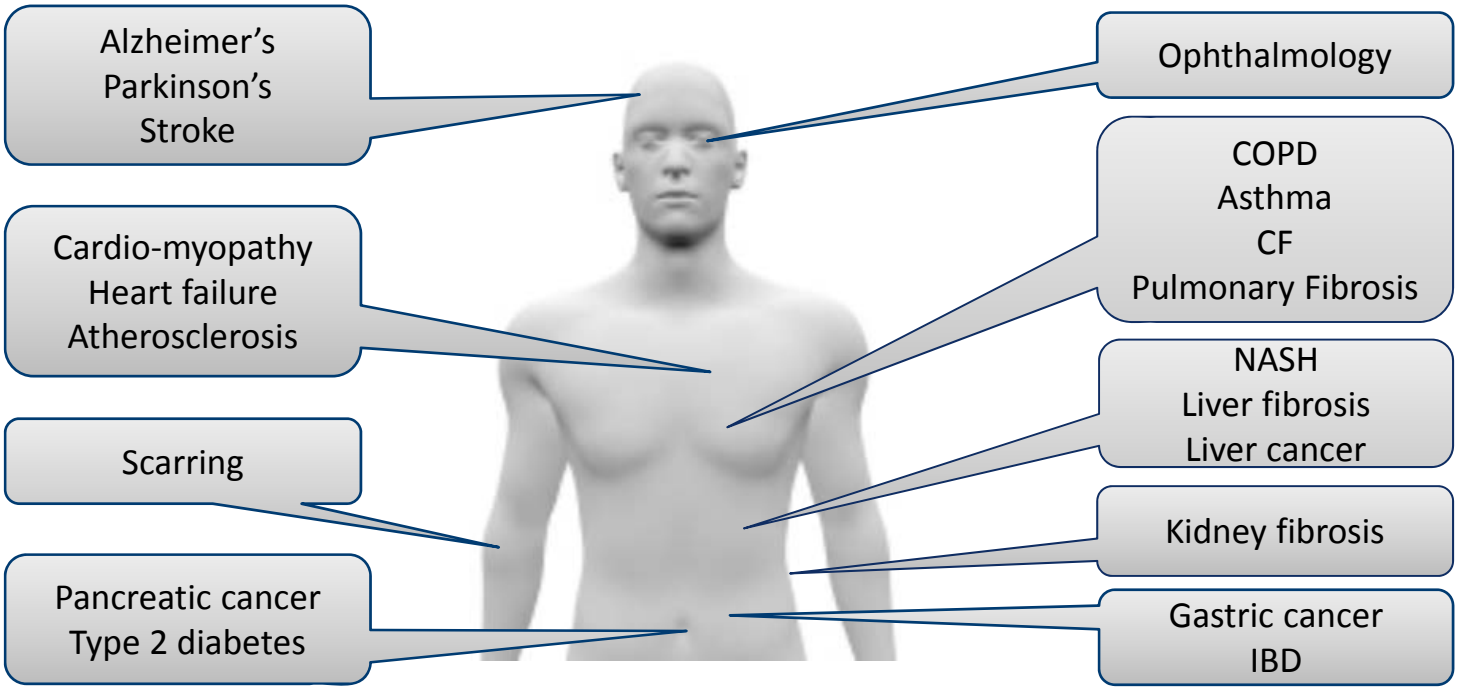
- Tie-breaker phase 3 trial commenced Q1 2015, managed by PXS
- 440 adult patients
  - 20+ countries
  - 130 sites
- Design
  - Full consultation with FDA
  - Similar design to CF301/2
- Fully recruited July 2016
- Results H1 2017

Rest of world

- Sold by Chiesi in UK & Germany
- Sold by PXS in Australia & Denmark
- Pending approval/distributor appointments in ten countries including Russia, Israel, Turkey, Brazil, Eastern Europe
- Additional distributors being appointed

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:

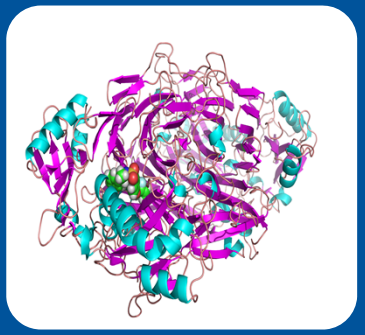
- First in class NASH drug taken to phase 1
- Two further candidates in lead optimisation phase
- One lead candidate moving to preclinical

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



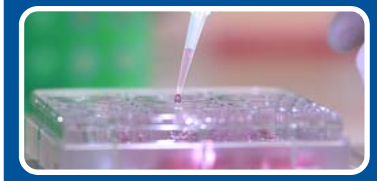
Pharmaxis drug discovery

- NASH & liver fibrosis (LOXL2)
- Respiratory – COPD, asthma, cystic fibrosis (SSAO/MPO)
- Neuro inflammation – Alzheimer's, Parkinson's, stroke (SSAO/MAO-b)

Collaborations allow us to leverage our platform without losing focus

Collaboration with Synairgen

- Pulmonary fibrosis (LOXL2)



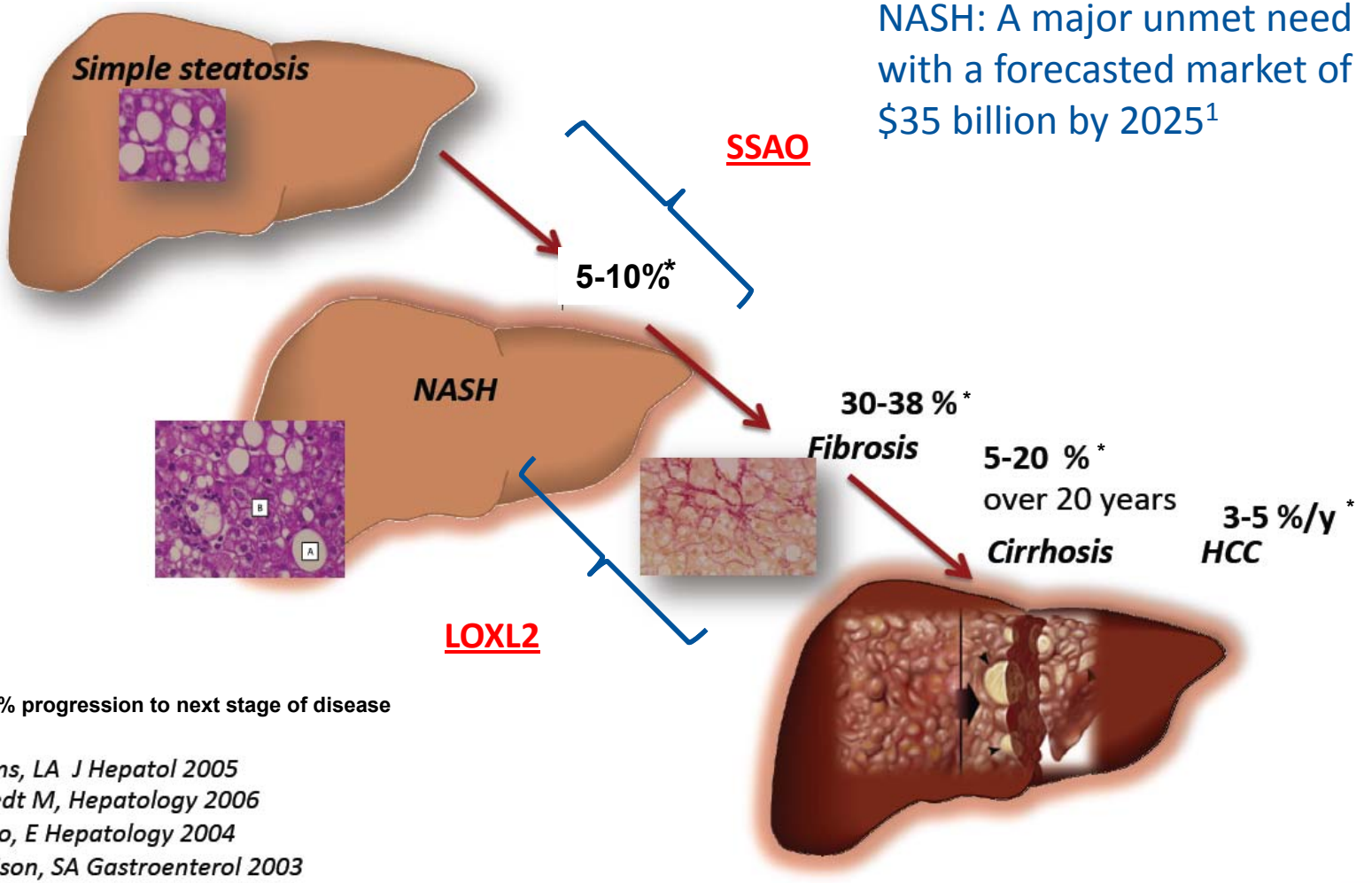
Exploratory academic collaborations (LOX/LOXL2)

- Scarring
- Kidney fibrosis
- Some cancers

Our therapeutic focus in NASH

Two complementary targets in the progression of non alcoholic fatty liver disease

30-40% of US population have steatosis (fatty liver)



NASH: A major unmet need with a forecasted market of $35 billion by 2025[1]

*** % progression to next stage of disease**

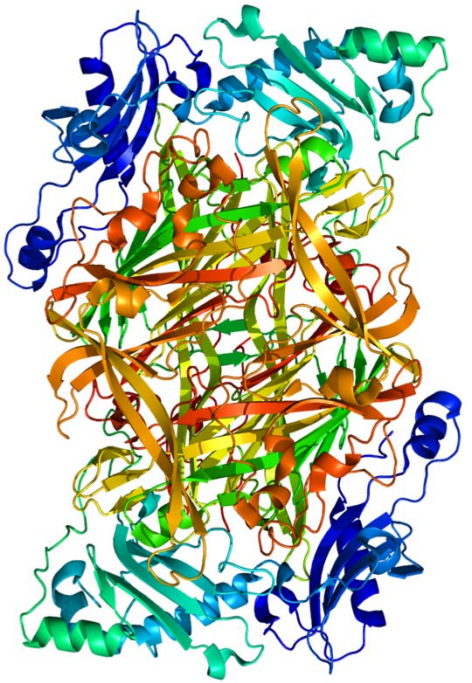
Adams, LA J Hepatol 2005
Ekstedt M, Hepatology 2006
Fassio, E Hepatology 2004
Harrison, SA Gastroenterol 2003

SSAO for NASH

Boehringer Ingelheim

SSAO inhibitor PXS4728A sold to Boehringer Ingelheim in May 2015

- 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
- Competitors:
  - Genefit – GF505 Phase 2b NASH (reported)
  - Intercept - OCA (FXR agonist) Phase 2b NASH (reported)
  - Gilead – FXR agonist in pre clinical



Compelling evidence has been provided that the enzymatic activity of SSAO/VAP-1 is involved in the development of fatty liver disease

(Weston et al., J Clin Invest. 2015;125(2):501–520. doi:10.1172/JCI73722).

Boehringer Ingelheim

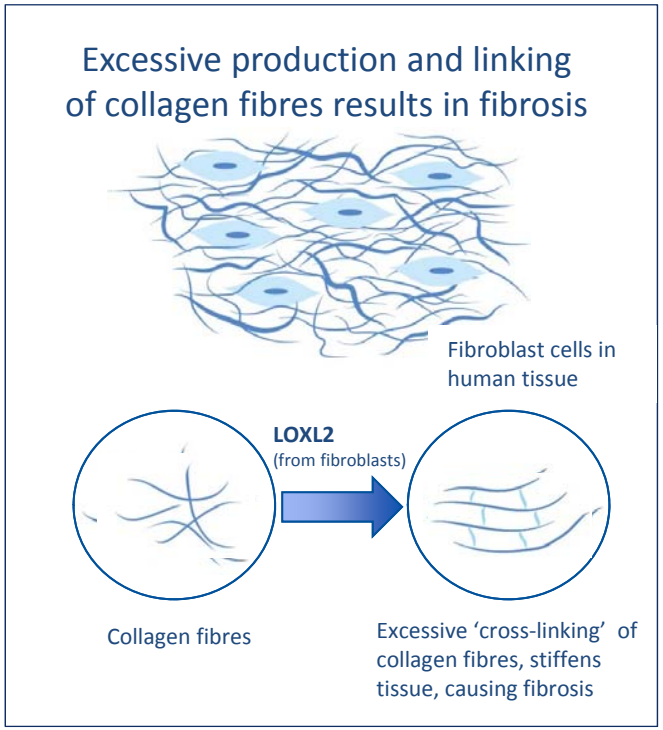
SSAO inhibitor PXS4728A sold to Boehringer Ingelheim in May 2015

- Excellent partner:
  - Boehringer leaders in metabolic disease
  - Industry leading development times
  - Boehringer responsible for all development, and commercialisation activities
- Competitive deal:
  - 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



Phase 1 & 2 fibrosis deals

Companies	Upfront	Potential
Gilead/Nimbus – P1 acquisition (Apr 16)	$400m	$1,200m
BMS/Promedior – P2 option/license (Aug 15)	$150m	$1,250m
Gilead/Phenex – P2 acquisition (Jan 15)	undisclosed	$470m
BMS/Galecto – P1 option to license (Nov 14)	undisclosed	$444m
Gilead/Arresto – P1 acquisition (Dec 12)	$225m	$225m
Shire/Fibrotech – P1 acquisition (May 14)	$75m	undisclosed

- Potential indications:
  - NASH / Liver Fibrosis
  - Pulmonary fibrosis (IPF)
  - Cancer
  - Wound healing

Significant market opportunity

- Development status:
  - Pharmaxis discovery – patent filed 2016
  - Compounds with differentiated PK / PD profile identified
  - Effective in pre clinical models of fibrosis and cancer

- 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

LOXL2 for pulmonary fibrosis

synairgen

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

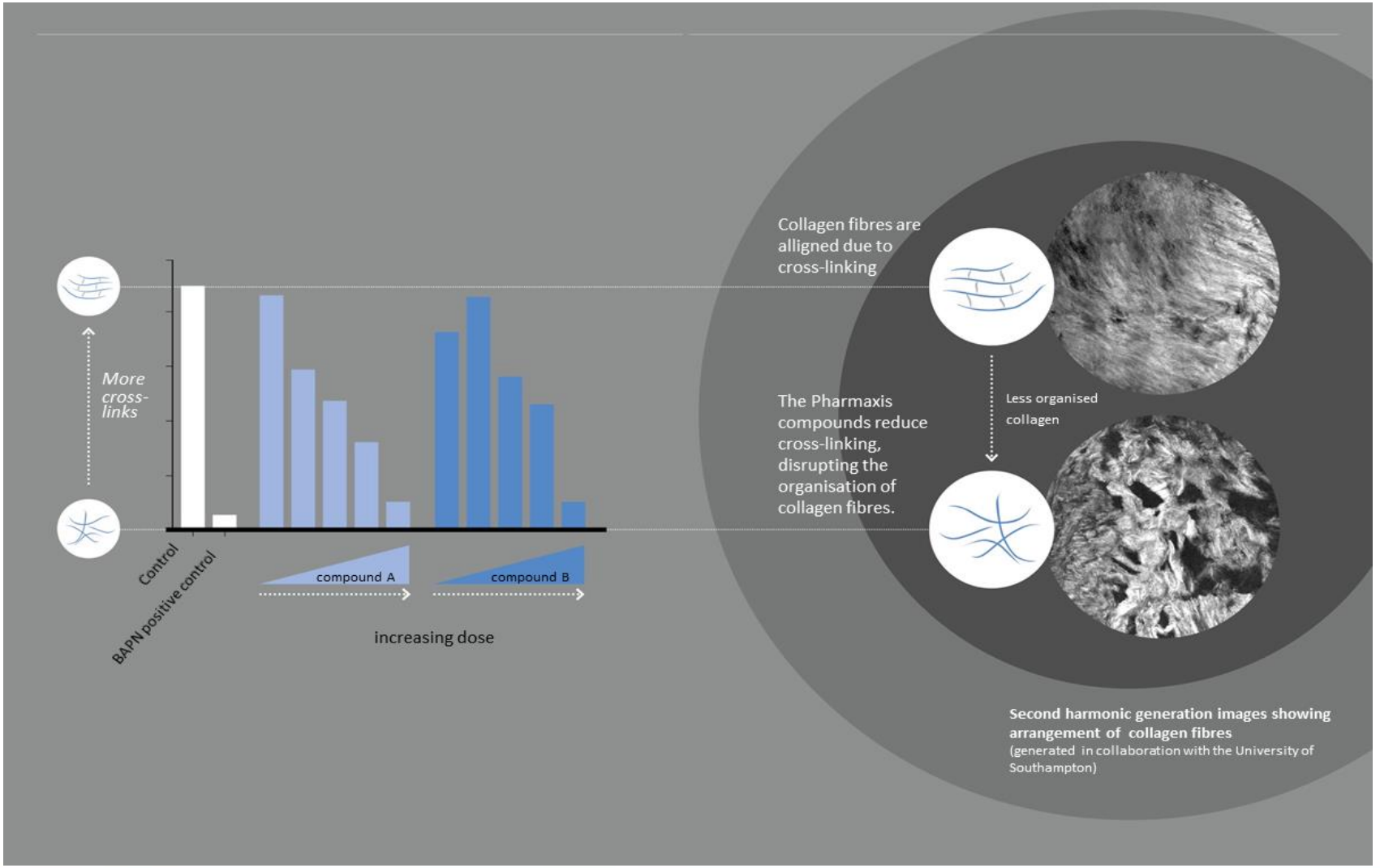
Synairgen collaboration

- Access to
  - Synairgen's strength in fibrosis biology and respiratory clinical development - BioBank human tissue models technology platform
  - 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
- Allows PXS to pursue further indications in parallel

Synairgen collaboration

synairgen

Disease-relevant activity of the Pharmaxis inhibitors in the in vitro model of lung fibrosis



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Financials – key statistics

30 June 2016

A$'000	Fiscal year ended		
(unaudited)	**30-Jun-14**	**30-June-15**	**30-June-16**
Income statements			
Sales	5,036	5,999	6,135
Total revenue	10,486	59,247	19,020
Total expenses	(62,201)	(40,739)	(35,476)
Net profit (loss) after tax	(51,818)	18,466	(16,463)
Segment results – adjusted EBITDA			
Bronchitol & Aridol	(22,555)	(10,045)	(8,228)
New drug development	(1,620)	35,068	(2,625)
Corporate	(6,226)	(3,532)	(3,988)
Total	(30,401)	21,491	(14,841)
Statement of cash flows			
Cash generated by/ (used in):			
Operations	(28,132)	21,780	(11,989)
Investing activities	(313)	(264)	(1,381)
Financing activities	(1,357)	(1,791)	(1,714)
Total cash used	(29,802)	19,725	(15,084)
Foreign currency exchange rate changes impact on cash	41	231	155
Cash at bank	34,182	54,138	39,209

Refer Annual Report and Quarterly Shareholder Update for additional financial information

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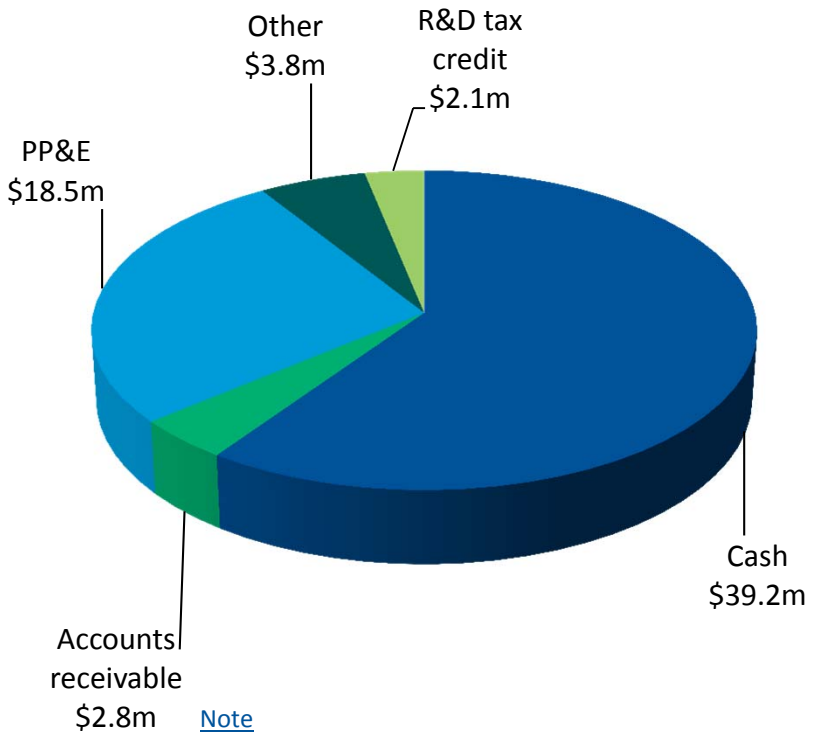
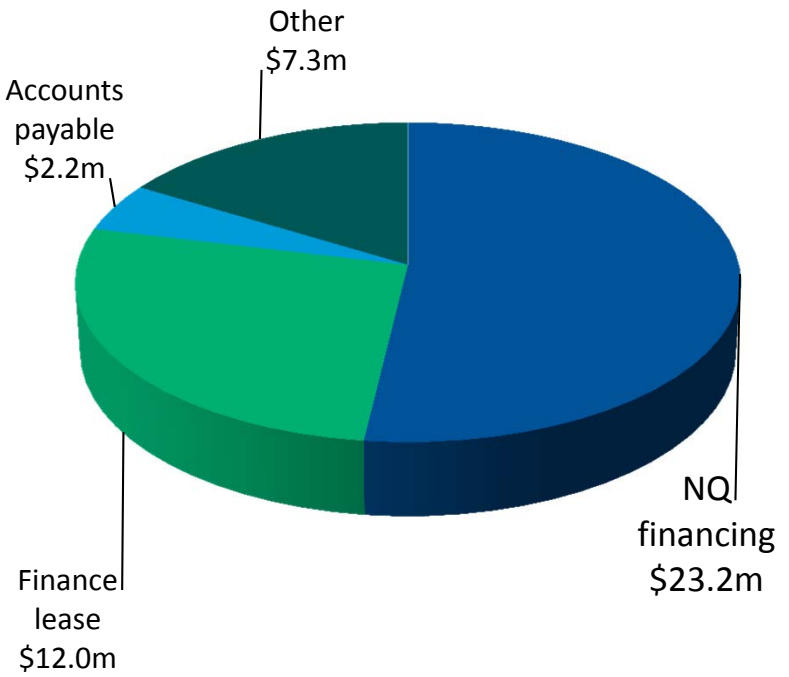
Balance sheet

30 June 2016

Assets (A$65.7m)



Liabilities (A$44.7m)



Note

- Finance lease over 20 Rodborough Road Frenchs Forest (to 2024, break possible in 2019) including deferred lease incentive of $1.9m
- NovaQuest financing – amount received plus accrued charge. Not repayable other than as % of Bronchitol revenue - average of mid-single digit % of net in-country sales by distributors in US (7 years from launch) and EU (to March 2020) and share of any sales milestones received from Chiesi

Shareholders & trading

ASX code: PXS

Shareholders (26 July 16)

- Shares on issue: 317m
- Employee options: 11.3m
- Institutional shareholders ~50%:
  - Australia - Orbis (17%), Australian Ethical (5%)
  - US - BVF Partners (14%)
  - US – other (2%)
  - UK - Montoya Investments (6%)
  - UK – other (3%)

Shares traded to 31 August

- Three months: 21m
- Six months: 41m
- Year: 96m

Market capitalisation

- A$94m (31 August 16)

News flow

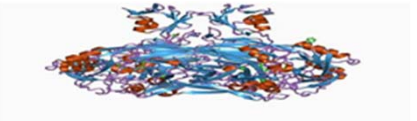
	2016	2017	2018
Boehringer Ingelheim		PXS4728A Phase 2 commences & ~A$25M milestone payable (Q1)	
pharmaxis Bronchitol – RoW	Russian marketing & orphan drug approval & first sales (H2)	EU Paediatric label extension application (H1)	
Chiesi People and ideas for innovation in healthcare Bronchitol - US		CF303 – trial completion and report (H1)	Bronchitol commercial launch milestone ~A$13m
pharmaxis New drug development			
SSAO/MAO-B	Selection of indication – before starting GLP tox	Complete phase 1: ≥1 programs Commence preclinical studies in ≥1 programs	Partner ≥1 programs Complete phase 1: ≥1 programs
SSAO/MPO	Select compound to move into preclinical studies		
LOXL-2 LOXL-2 IPF Synairgen collaboration	Nominate LOXL2 candidate and commence full preclinical studies ≥1 programs Complete PoC studies ≥1 programs Commence GLP tox ≥1 programs		
LOX/LOXL-2	Leading universities/academics assessing in kidney fibrosis, cancer and wound healing		

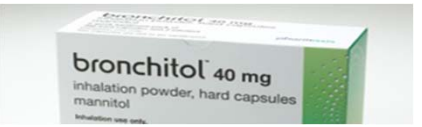
Pharmaxis opportunities for growth

Building a biotech powerhouse in fibrosis and inflammation

Boehringer Ingelheim

synairgen





SSAO program for NASH (fatty liver)

- NASH: US$35B market by 2025
- Acquired by BI at phase 1 for A$39m upfront, total >A$750m
- BI to develop for NASH and other inflammatory indications (eg. kidney fibrosis, COPD)
- Next milestone: ~A$25m at start of phase 2 – Q1 2017

LOXL2 program for pulmonary fibrosis

- Pulmonary fibrosis: market >$1B
- Collaborate to phase 1 or 2 then seek partner
- Revenue share for phase 1 partnering deal: 50/50
- Next milestone – commencement of formal preclinical program H2 2016

LOXL2 for NASH and other diseases

- Big Pharma interest in NASH, LOXL2 and PXS chemistry
- Complimentary to SSAO program acquired by BI
- Next milestone – commencement of formal preclinical program H2 CY 2016

Bronchitol for CF

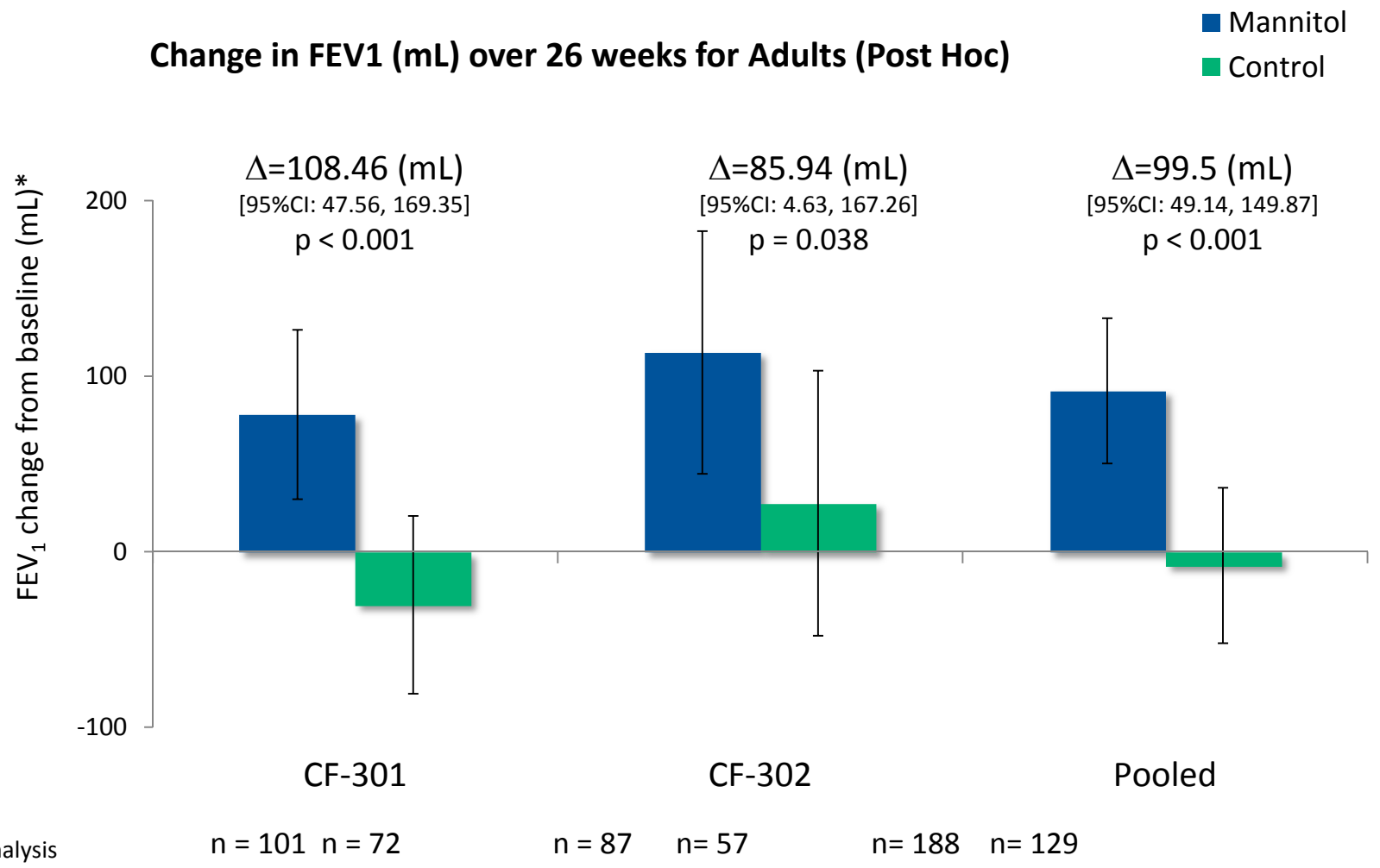
- Access large US CF market with Chiesi
  - Chiesi funding CF303 to a cap of US$22m
  - ~A$13m milestone payments on launch
- High teens % share of in-market sales
- Growth from existing markets
- New markets opening over next 24 months, including large Russian market

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 18 months
- **Strong balance sheet** – A$39m cash at June 16 with a likely milestone of A$25m due in Q1 17

Appendix

CF303: predictable results in adult CF patients



*Post Hoc analysis

Bilton D, Robinson P, Cooper P, et al. *J Cystic Fibrosis* 2011, Suppl 20 (A78) and Data on File Pharmaxis

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