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Market Analysis & Segmentation

Gary Phillips CEO

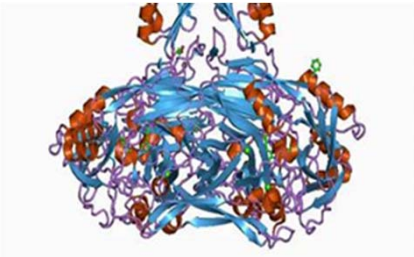
Bioshares 29 July 2016

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.

Pharmaxis 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

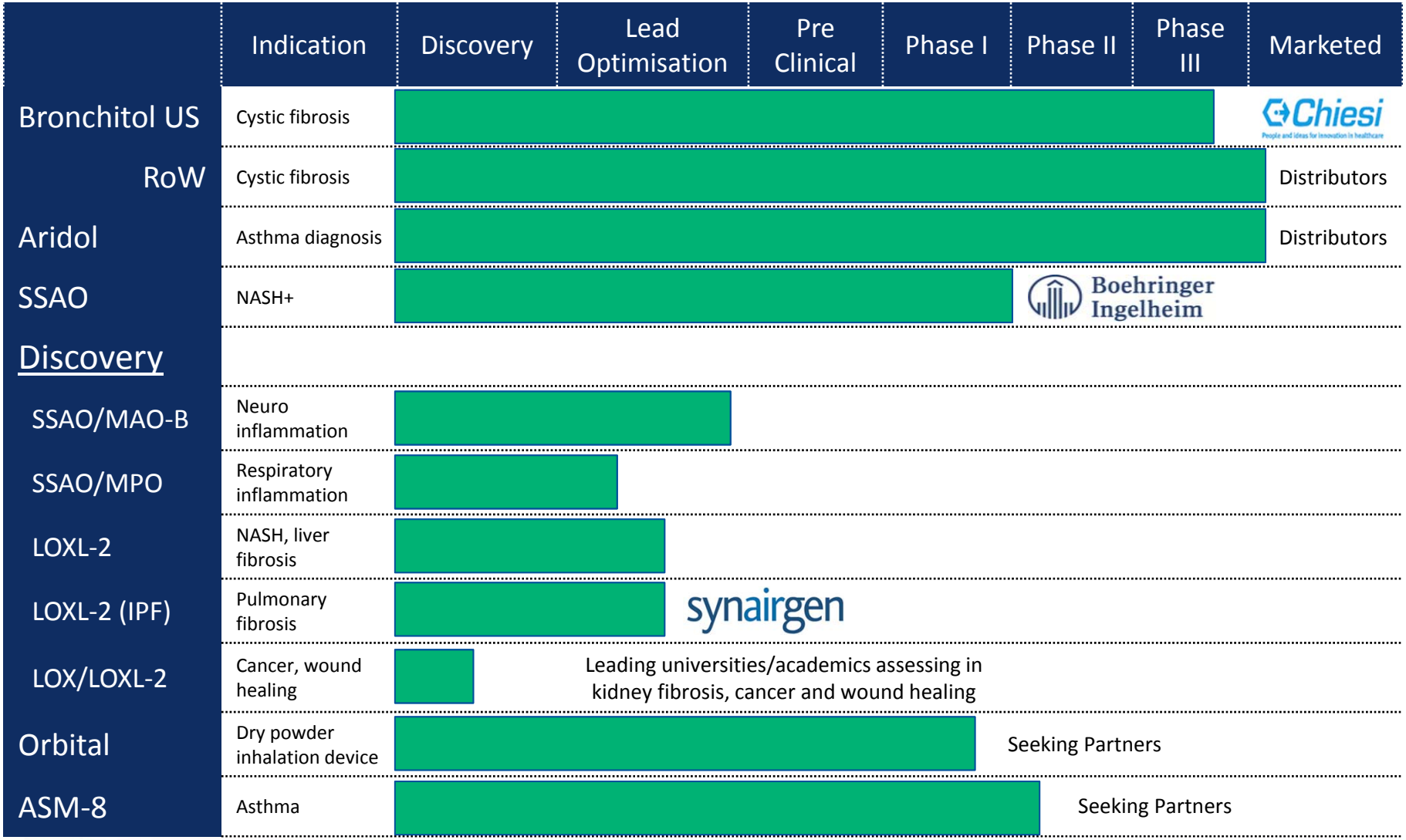
- First drug out licensed to Boehringer Ingelheim in globally competitive deal - total potential deal >A$750m
- Significant value milestones from existing partner deals near term
- Pipeline providing multiple future opportunities
- Synairgen collaboration developing additional indication

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 $95*
- Institutional investor's >45%
- Increasing Bronchitol sales globally in new and existing markets

* Note: Market Cap as of 27/07/16

Pharmaxis product portfolio



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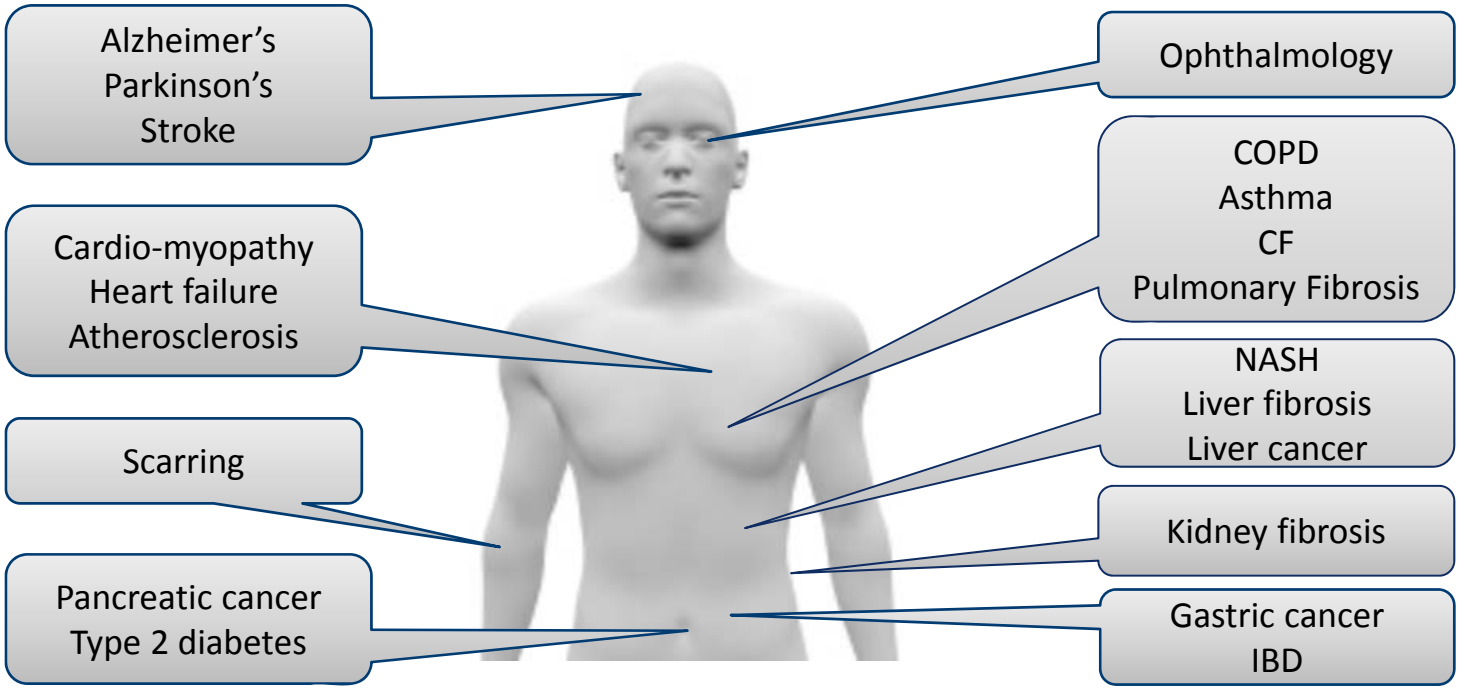
Drug discovery

Applying amine oxidase chemistry to inflammation and fibrosis

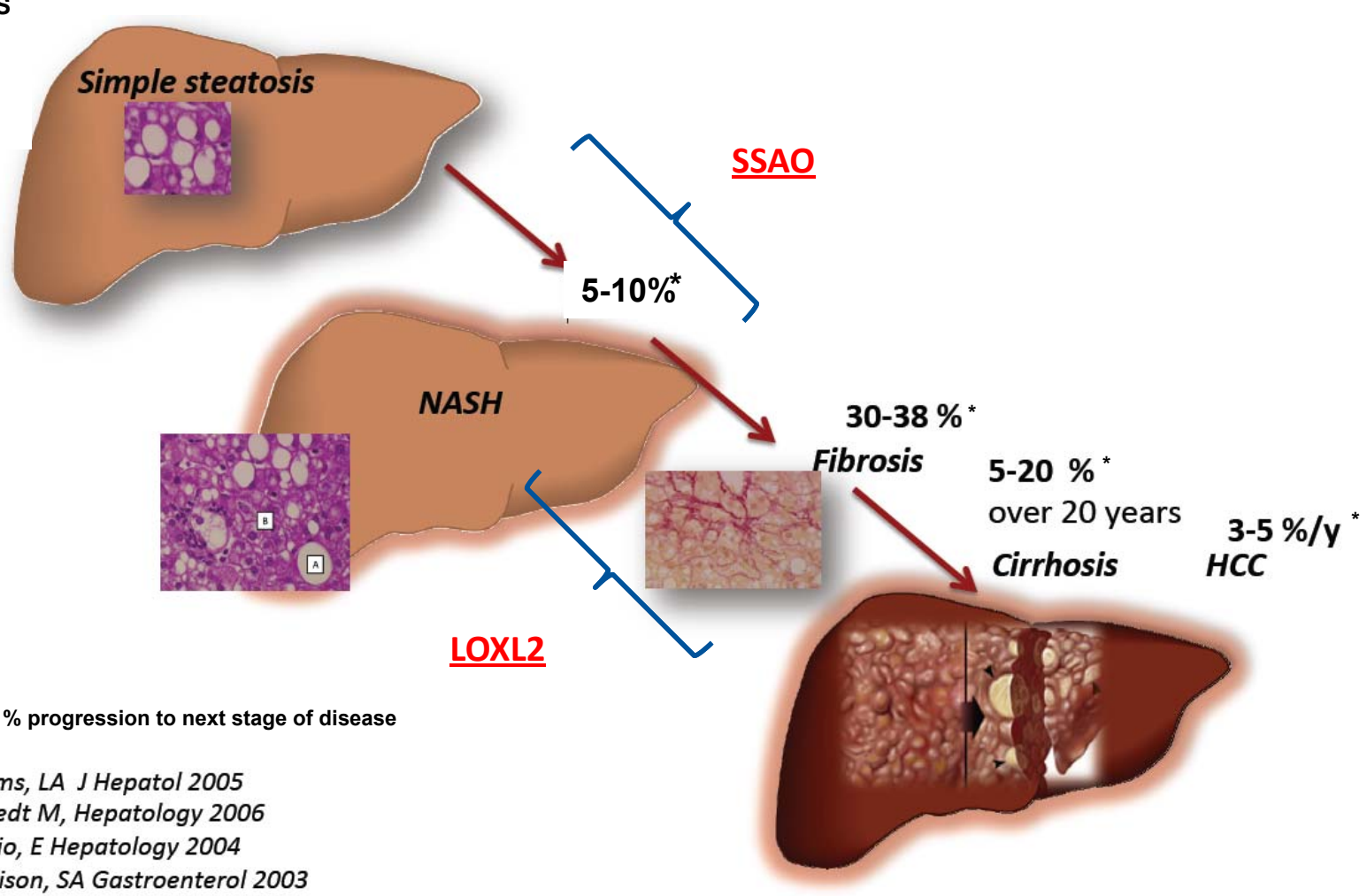


Amine oxidase enzymes are well validated as targets in diseases with a high unmet medical need

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)



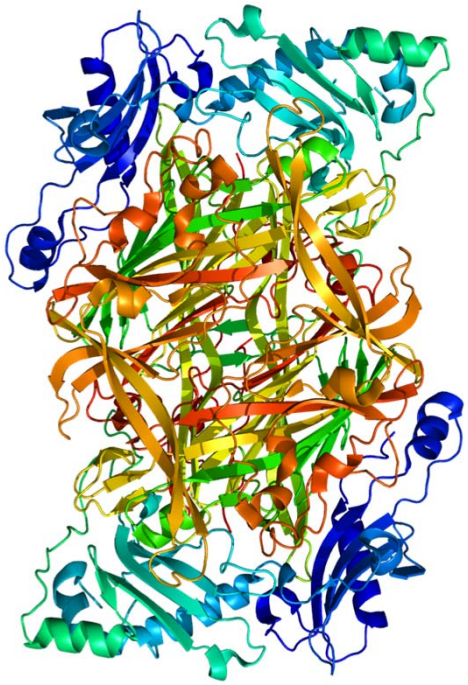
*** % progression to next stage of disease**

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

SSAO for NASH

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
  - Partnered with Boehringer Ingelheim
    - Phase 2 in NASH due to start Q1 2017

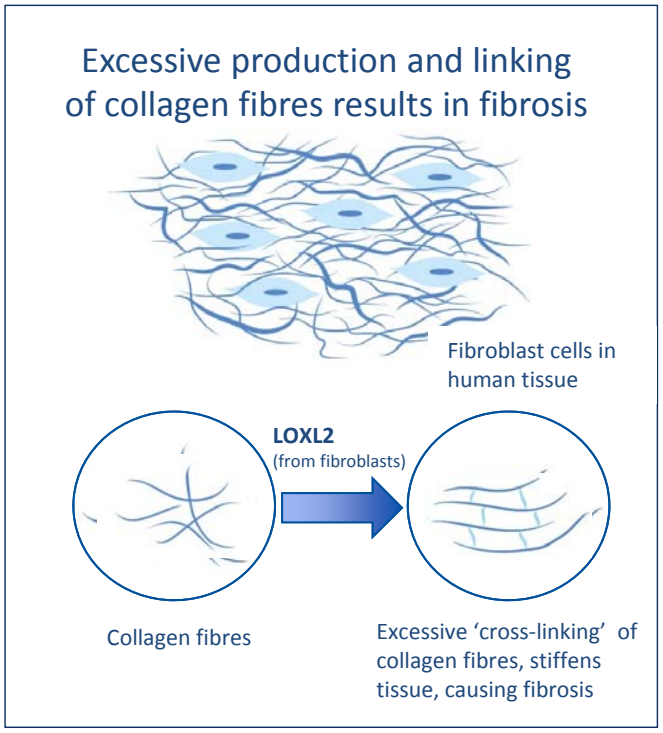


Compelling evidence has been provided that the enzymatic activity of SSAO/VAP-1 is involved in the development of fatty liver disease

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

LOXL2 inhibition for NASH & other fibrotic diseases

An attractive target and development program



- 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

The Competition – NASH

Thomson Reuters Cortellis July 2016

Filter	# Drugs	# mode of action
Discovery	52	35
Phase 1	17	13
Phase 2	25	18
Phase 3	4	4
Total	88	50

Note (All indications):

# of LOXL2 inhibitor programs = 4

# of SSAO inhibitor programs = 3

Phase 1 & 2 fibrosis deals

Large deal values!

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

Commercially focused drug discovery

"This will be the advertising campaign - now it's up to the research dept to come up with something."

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

Key Success Factors

Business Development and Licensing

1. Is your target validated / of known value?
2. Has your scientific team been validated / endorsed?
3. Is your data package sufficient?
4. Do you know who your potential customers are?

Is your target validated / of known value?

Business Development and Licensing – Key Success Factor 1

Validated target?	YES	NO
Pros	• No need to spend money on the target • Companies looking for competitive program	• Limited competition • Built in differentiation
Cons	• Competition could be fierce • Hidden Big Pharma programs	• Competing with other mechanisms • Need to spend money on target validation
Potential Actions	• Must have a competitive pre clinical program with accepted comparators that differentiates your program. • Consider collaboration or early deal so as not to lose the race.	• Support academic target validation and publication with Global KOLs • Present at key academic conferences

Has your scientific team been validated / endorsed?

Business Development and Licensing – Key Success Factor 2

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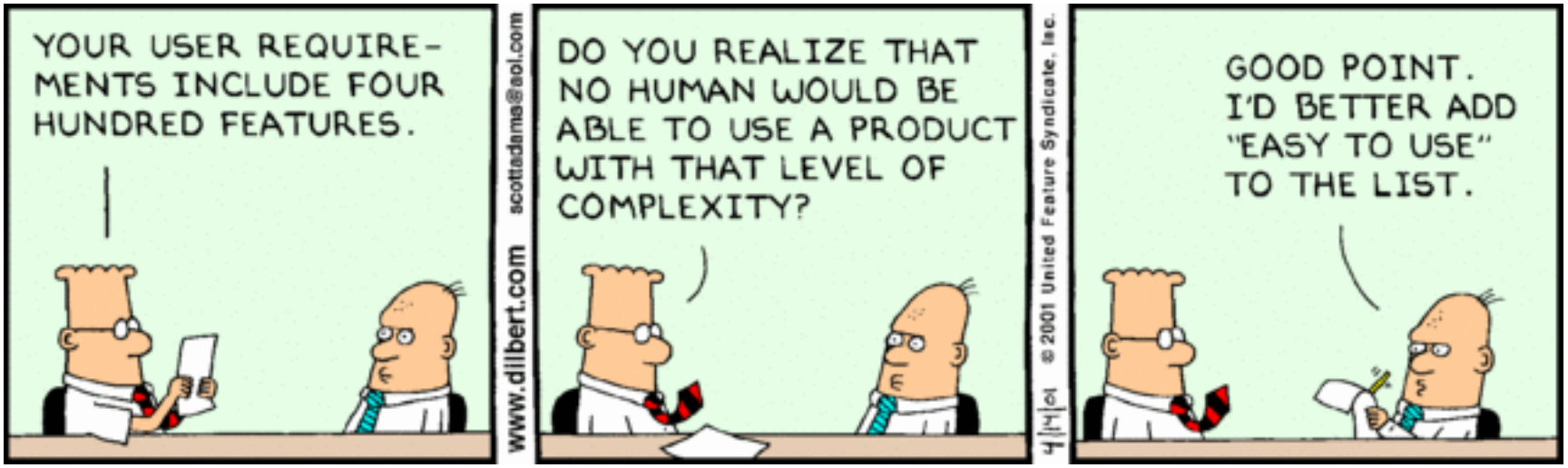
Has your scientific team been validated / endorsed?

Business Development and Licensing – Key Success Factor 2

Types of endorsement	Strength
Previous deals with Big Pharma	10
Reputation of key staff	6
High quality Scientific Advisory Board	5
Successful grants	5
Peer reviewed scientific publications	5

What do your customers consider to be P.O.C.?

Business Development and Licensing – Key Success Factor 3



Is your data package sufficient?

Business Development and Licensing – Key Success Factor 3

Stage of Development	Adding Value
Pre-Clinical	• POC versus accepted comparators in accepted models with reliable vendors • Tox data to de risk the target and drug
Phase 1	• Same as for pre-clinical • Phase 1 design showing target engagement • Phase 1c with biomarkers in patients
Phase 2	• Trial design that covers anticipated changes in standard of care

Do you know who your potential customers are?

Business Development and Licensing – Key Success Factor 4

Look for strong strategic fit

1. Are they committed to the disease / therapeutic area?

2. What do they have in their development pipeline?
   a) Complementary or competing?
   b) What development stage?

3. Do they have marketed products in the disease?
   a) Patent life?
   b) Size of franchise?

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

Key Issues for a LOXL2 inhibitor

Maximising value in pre-clinical

Key Issues	Action
Competing programs?	• Speed to market critical • Consider exit post phase 1 or 2a
Gilead phase 2 failures	• Differentiate from antibody program

Multiple potential indications	• Collaborations to cover the opportunities
	  • Synairgen - IPF
	  • Cancer Institutes

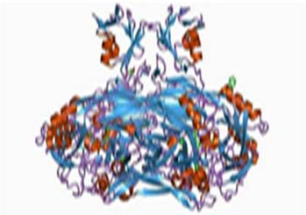
Changing standard of care?	• Pre-clinical models to mimic revised standard of care / combination therapy.
High level of interest	• Disclose when pre-clinical package complete under CDA • Due diligence in parallel with Phase 1 • Run a competitive licensing process

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