



Investor Briefing

November 2016



Pharmaxis pipeline

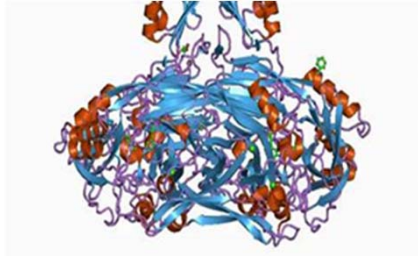
Gary Phillips CEO

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



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\$32m cash balance at September 2016; average 12 month cash usage \$1.5m per month
- Boehringer phase 2 initiation milestone expected H1 2017 ~A\$25m
- Market cap \$83M*
- institutional investor's ~50%
- Increasing Bronchitol sales globally in new and existing markets

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 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



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



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

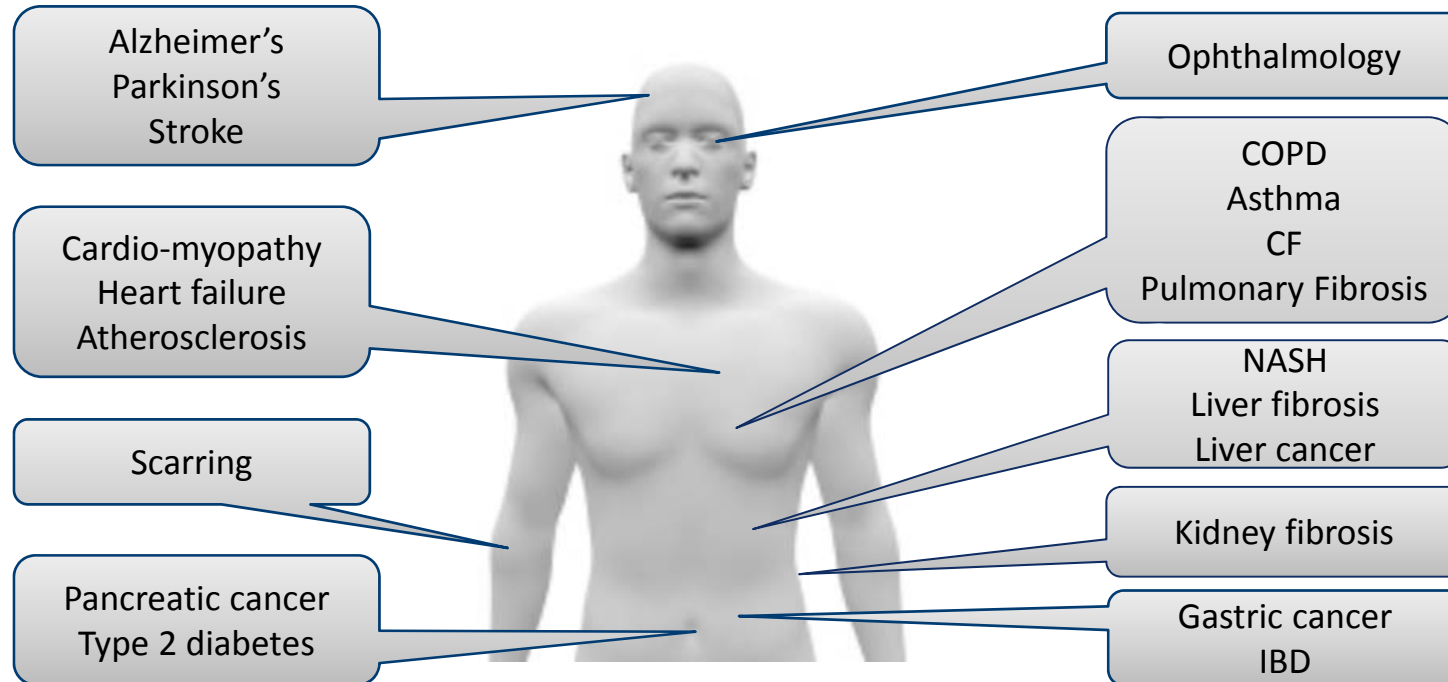
- more than 15 years experience in clinical trial design and management
- author of more than 60 scientific papers
- founding Medical Director of the National Health Sciences Centre
- previously held various positions with the Australian National University, Stanford University, the Baxter Centre for Medical Research, Royal Melbourne Hospital, and the Walter and Eliza Hall Institute

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

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

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

Pharmaxis product pipeline

	Indication	Discovery	Lead Optimisation	Pre Clinical	Phase I	Phase II	Phase III	Marketed
SSAO	NASH+					Boehringer Ingelheim		
<u>Discovery</u>								
SSAO/MAO-B	Neuro inflammation							
SSAO/MPO	Respiratory inflammation							
LOXL-2	NASH, liver fibrosis							
LOXL-2 (IPF)	Pulmonary fibrosis			synairgen				
LOXL-2 (other)	Other fibrotic & cancer			Leading universities/academics assessing in kidney fibrosis and cancer				
LOX	Skin scarring							

Investor Briefing

Agenda

- **Introduction to Pharmaxis Pipeline;**
Gary Phillips
- **Review of the competitive science in NASH and fibrosis;**
Professor Darren Kelly
- **Review of the commercial deal environment in NASH and fibrosis;**
Dr Anthony Brown



Professor Darren Kelly

- More than 20 years of management and research experience in the life sciences sector, particularly relating to drug development.
- Currently CEO and Managing Director of Occurx
- Associate Dean (Innovation and Commercialisation), Professor & Director of Biomedical Research in the Department of Medicine at The University of Melbourne.
- Entrepreneur in Residence with Brandon Capital, Melbourne.
- Former CEO & Director of Fibrotech Therapeutics,



Dr Anthony Brown

- Anthony is currently a partner of advisory firm WG Partners, and has extensive experience across the life sciences sector at both corporate and buy-side levels.
- Winner of the Thomson Extel Award as best buy-side Biotech analyst of 2005.
- In 2007 joined the Abu Dhabi Investment Authority, one of the world's largest sovereign wealth funds, in London.
- In 2011 joined AstraZeneca working directly for global CFO Simon Lowth
- Joined WG Partners in early 2015



Review of the competitive science in NASH and fibrosis

Professor Darren Kelly

What is NASH

(Nonalcoholic steatohepatitis)

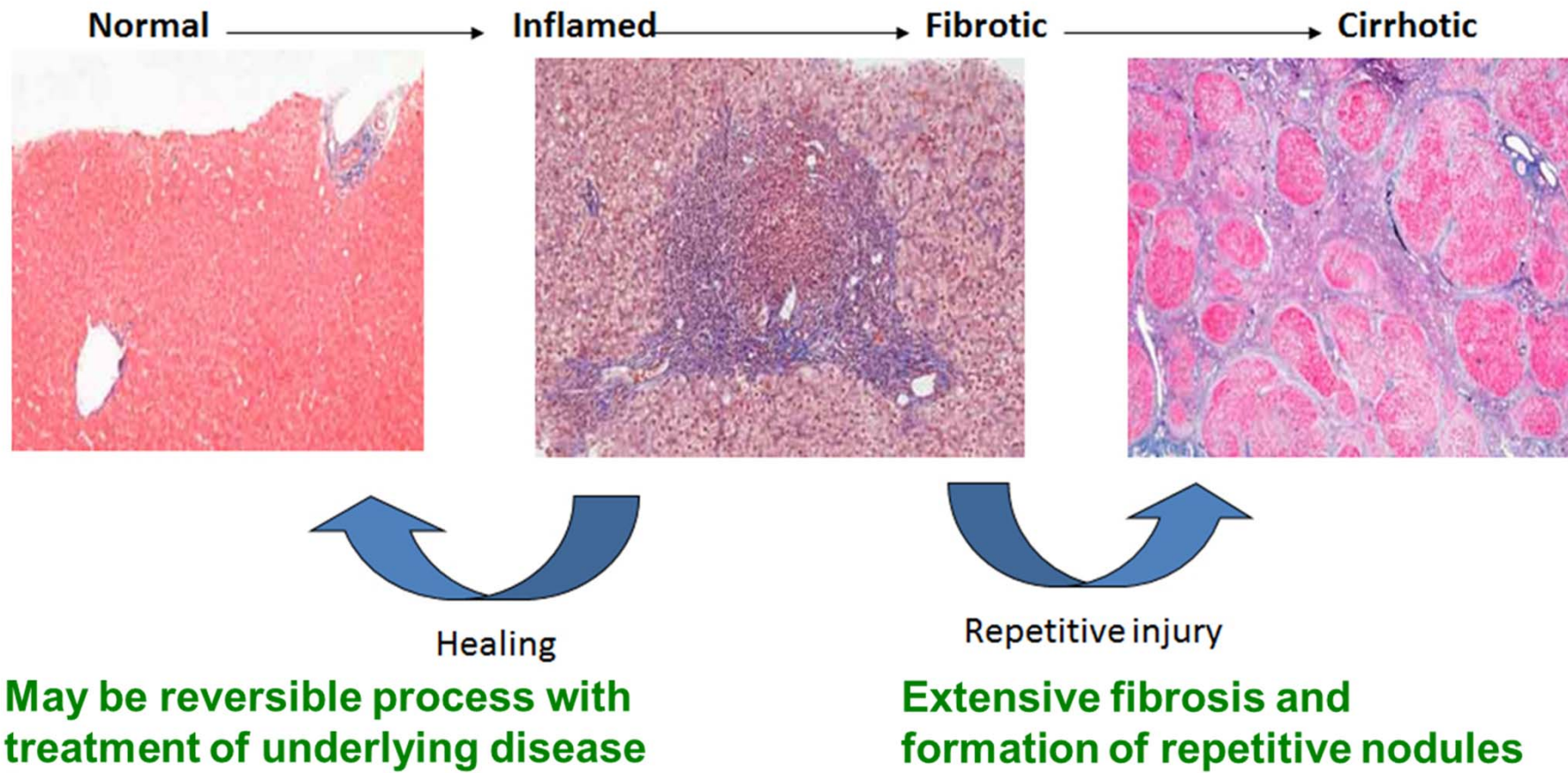
- NASH is a common, often “silent” liver disease.
- Resembles alcoholic liver disease, but occurs in people who drink little or no alcohol.
- Major feature is fat in the liver, along with inflammation and damage.
- Most people with NASH feel well and are not aware that they have a liver problem.
- NASH can be severe and can lead to cirrhosis, in which the liver is permanently damaged and scarred and no longer able to work properly.
- NASH affects 2 to 5 percent of Americans.
- Both NASH and NAFLD (fatty liver) are becoming more common, possibly because of the greater number of Americans with obesity. In the past 10 years, the rate of obesity has doubled in adults and tripled in children.

From NIH website:

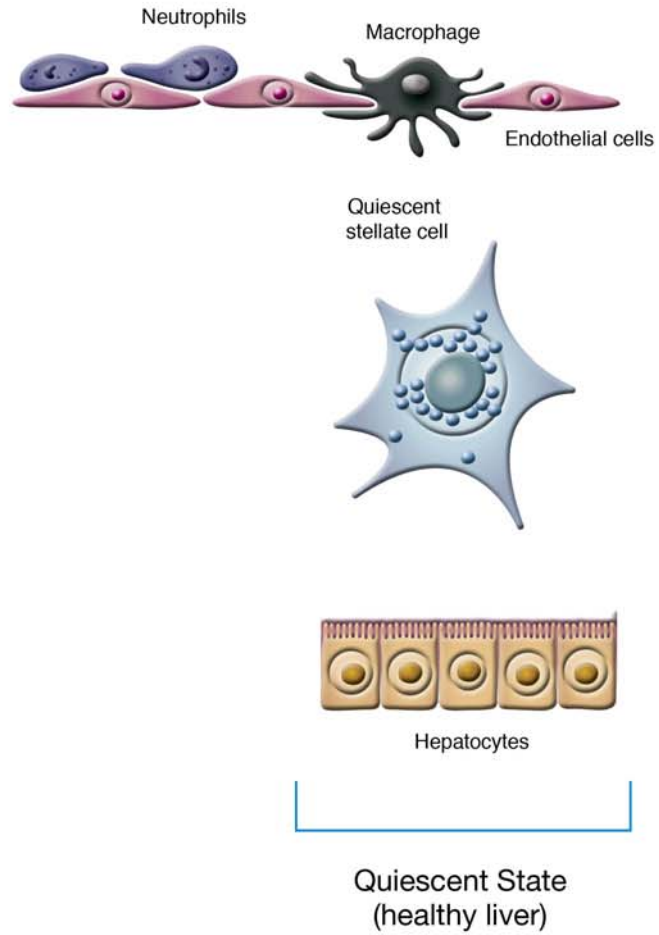
<https://www.niddk.nih.gov/health-information/health-topics/liver-disease/nonalcoholic-steatohepatitis/Pages/facts.aspx>

NASH

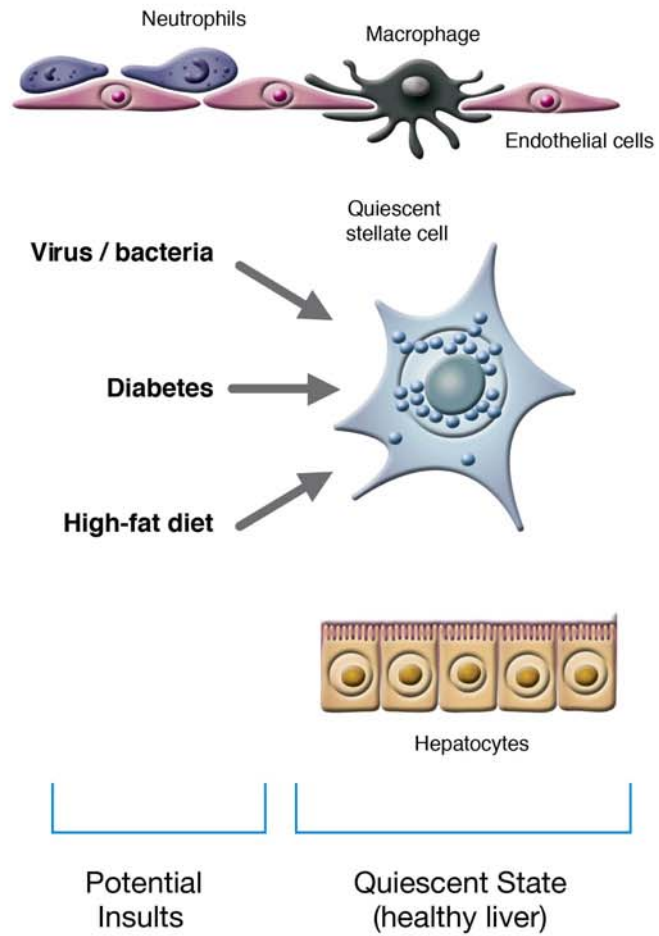
Progression of fibrosis



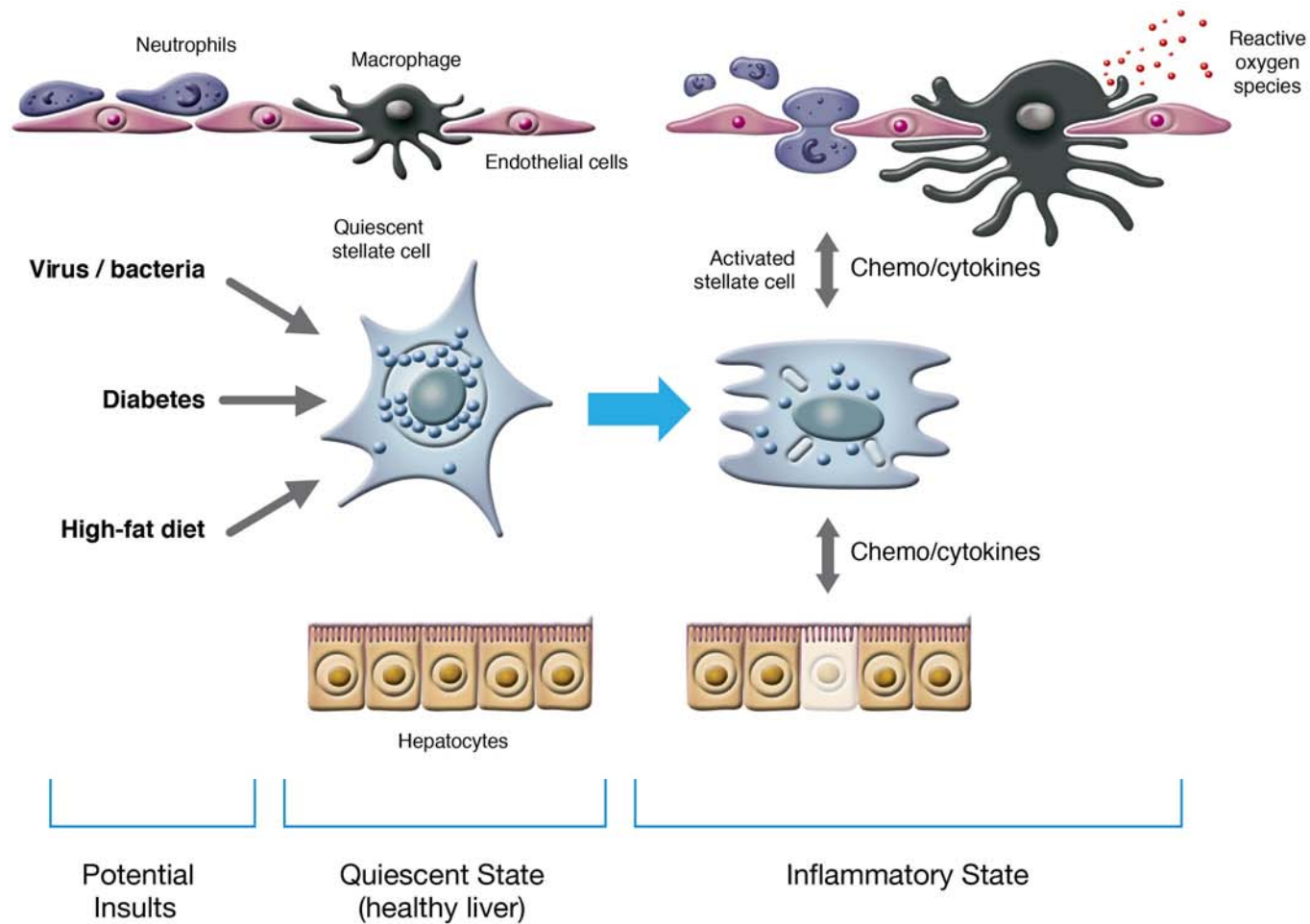
Drugs targeting NASH → Cirrhosis



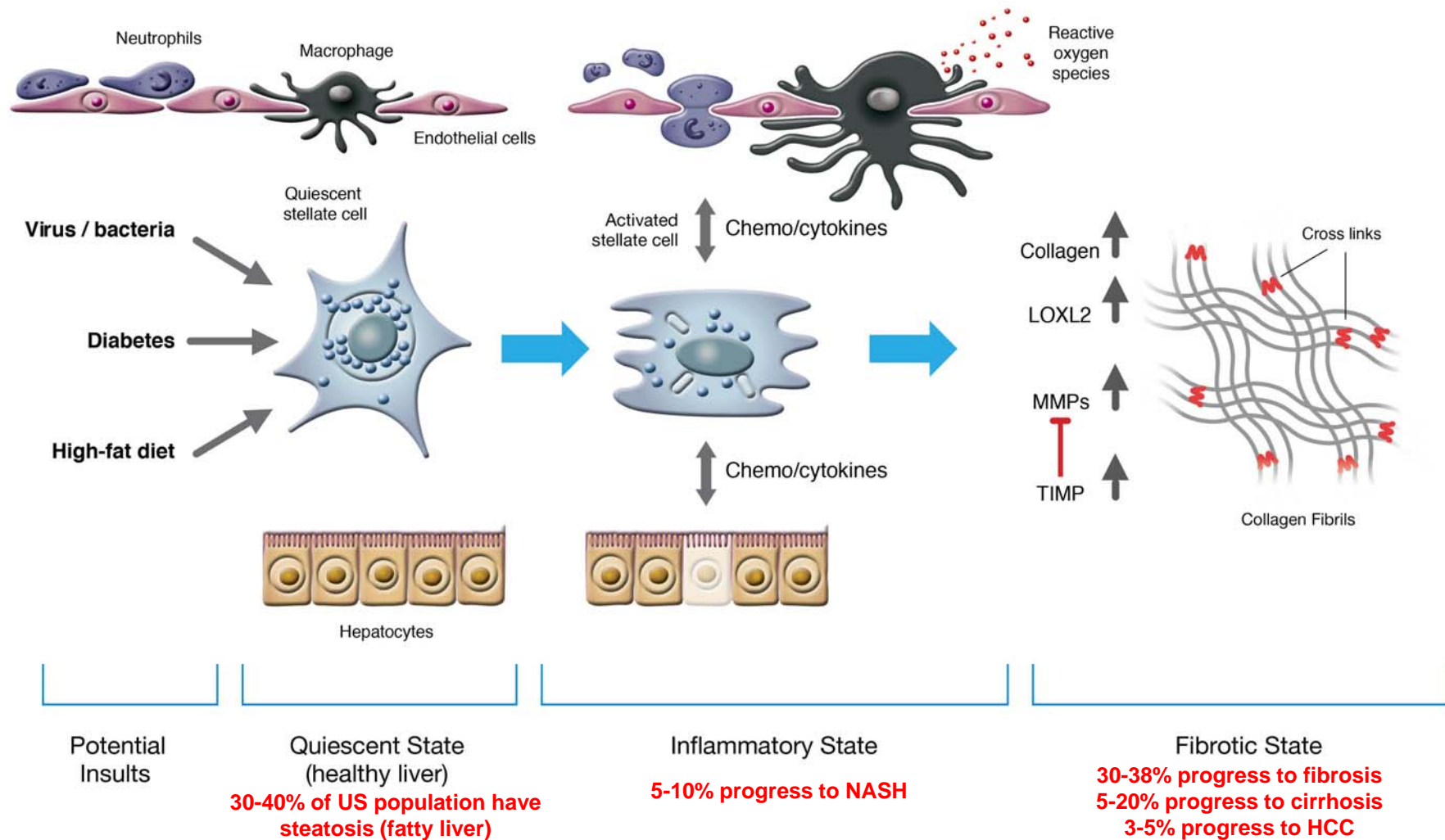
Drugs targeting NASH → Cirrhosis



Drugs targeting NASH → Cirrhosis



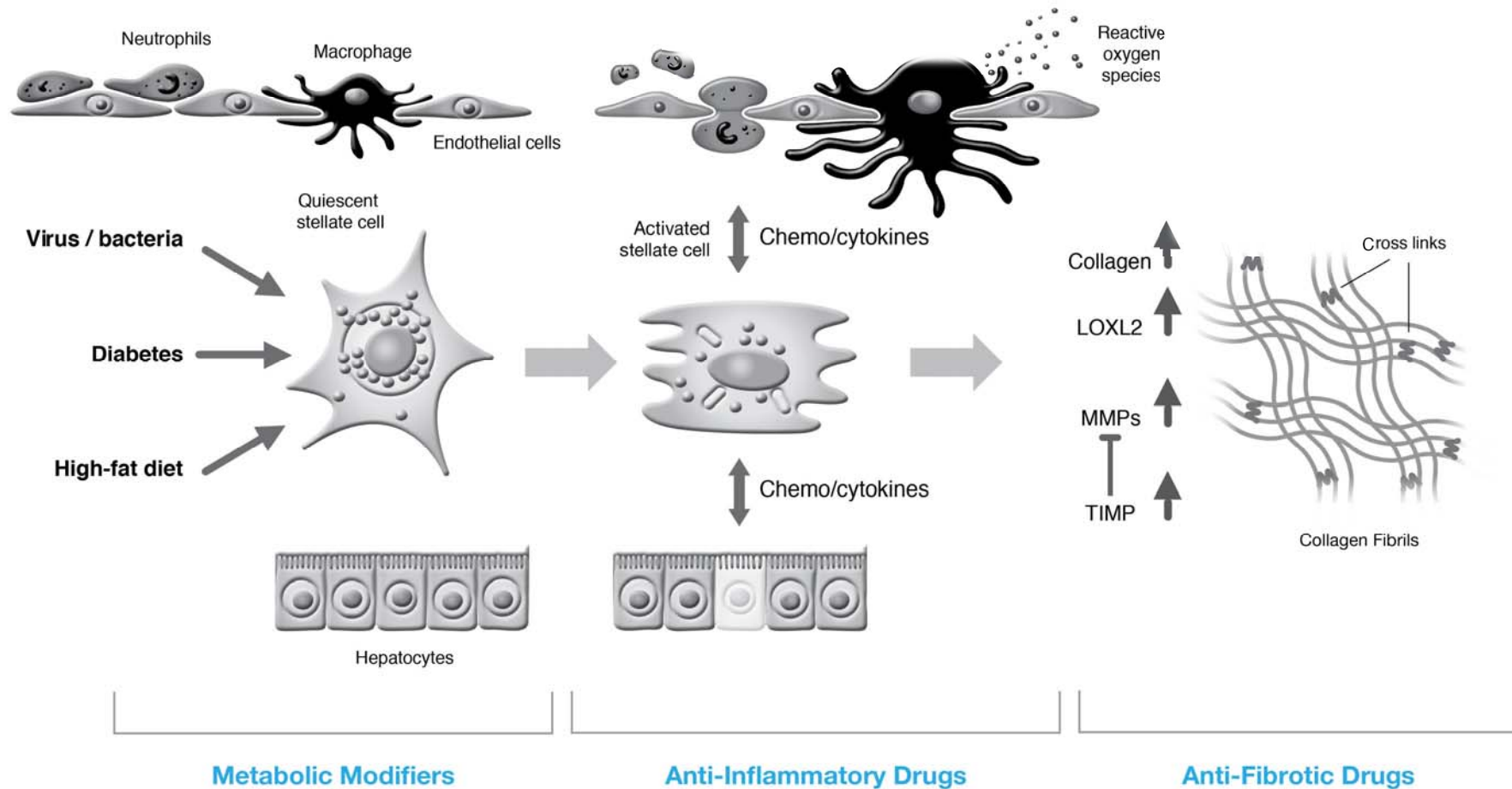
Drugs targeting NASH → Cirrhosis



How can NASH be treated

- Drug development:
 - Metabolic modifiers
 - Anti-Inflammatory drugs
 - Anti-Fibrosis drugs
- Combination therapy?

Drugs targeting NASH → Cirrhosis



Competitors

- Preclinical
- Clinical
- Results to date

Selected NASH products in development

Metabolic modifier

Company	Drug	Mode of action	Highest Phase
Intercept	Obeticholic acid	FXR agonist	Phase III
GFT-505	Elafibranor	PPAR α/δ agonist	Phase III
Galmed	Aramchol	Synthetic fatty acid bile conjugate	Phase II/III
Islet Sciences	Remogliflozin	SGLT2 inhibitor	Type 2 Diab
Novo Nordisk	Liraglutide	GLP1R agonist	Type 2 Diab
Allergan	Evogliptin	DPP-4 inhibitor	Type 2 Diab
Gilead	GS-9674	FXR agonist	Phase II
Gilead	GS-0976	ACC inhibitor	Phase II
Bristol-Myers Squibb	BMS-986036	FGF21 agonist	Phase II
Shire	Volixibat (SHP626)	ASBT inhibitor	Phase II
Arisaph Pharmaceuticals	ARI3037MO	Niacin analogue	Phase II

Selected NASH products in development

Anti-inflammatory

Company	Drug	Mode of action	Highest Phase
Conatus	Emricasan	Pan caspase protease inhibitor	Phase II
Allergan	Cenicriviroc	CCR2 and CCR5 inhibitor	Phase II
Gilead	Selonsertib (GS-4997)	MAPK5 inhibitor	Phase II
MediciNova	Tipelukast (MN-001)	LTD4 receptor antagonist	Phase II
Immuron	IMM 124E	Immunomodulator	Phase II
Cempra	Solithromycin	Macrolide antibiotic	Phase II
Boehringer Ingelheim	PXS-4728A	SSAO inhibitor	Phase I

Selected NASH products in development

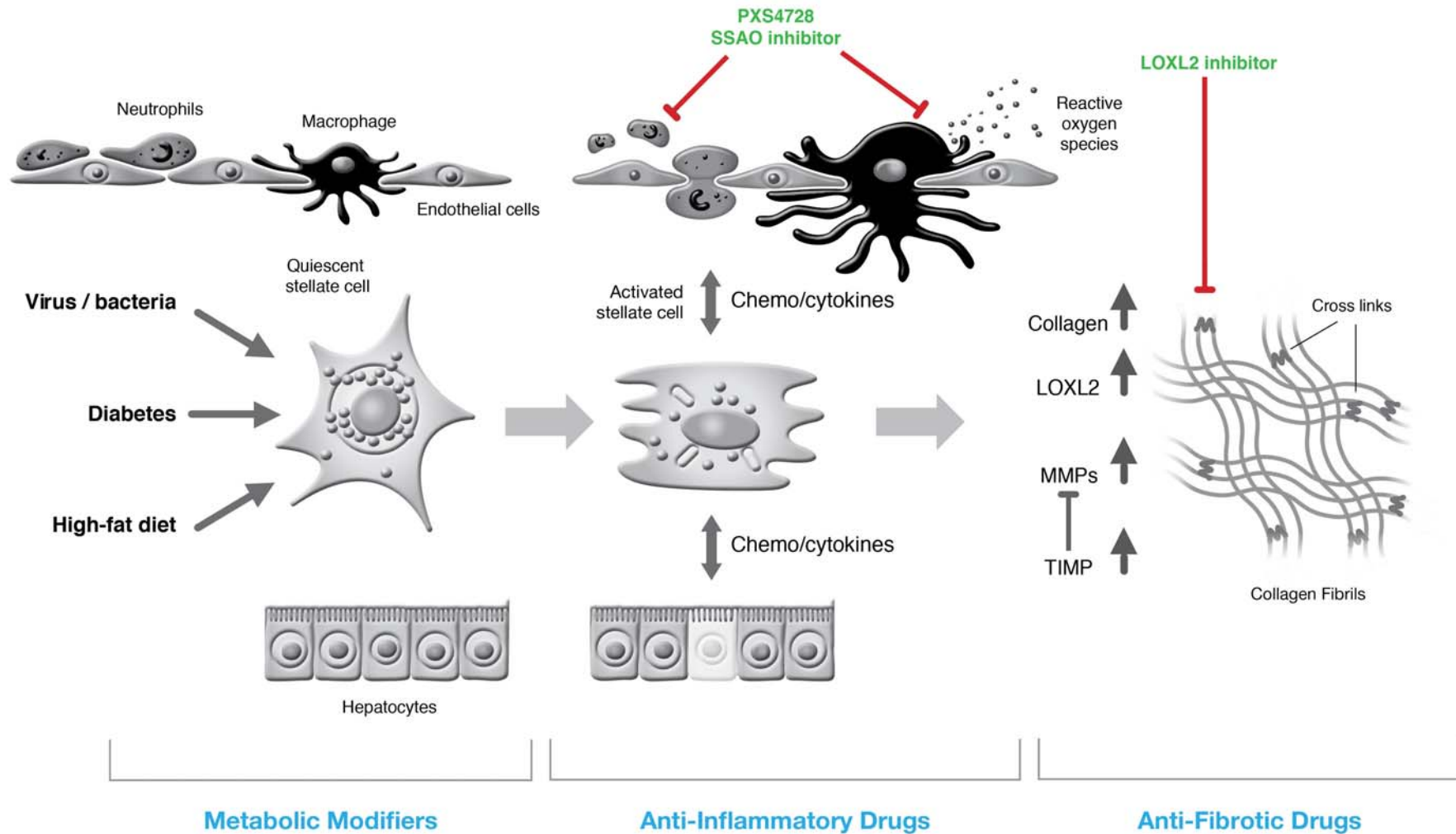
Anti-fibrotic

Company	Drug	Mode of action	Highest Phase
Gilead	Simtuzumab	LOXL2 inhibitor	Phase II
Galectin	GR-MD-02	Galectin-3 inhibitor	Phase II
Bristol-Myers Squibb	ND-L02-s0201	Hsp47 inhibitor	Phase Ib

Pharmaxis compounds

- Mode of action
 - PXS-4728A
 - Small molecule inhibitor of SSAO (VAP-1)
 - Important inflammatory pathway in several diseases including NASH and COPD
 - Being developed by Boehringer Ingelheim after acquisition in Phase I
 - LOXL2 inhibitor
 - Small molecule inhibitors of LOXL2
 - Anti-fibrotic
- Competition

Drugs targeting NASH → Cirrhosis





Review of the commercial deal environment in NASH and fibrosis

Dr Anthony Brown

PXS Investor Presentation

Fibrosis Deal Environment

November 2016



Contents

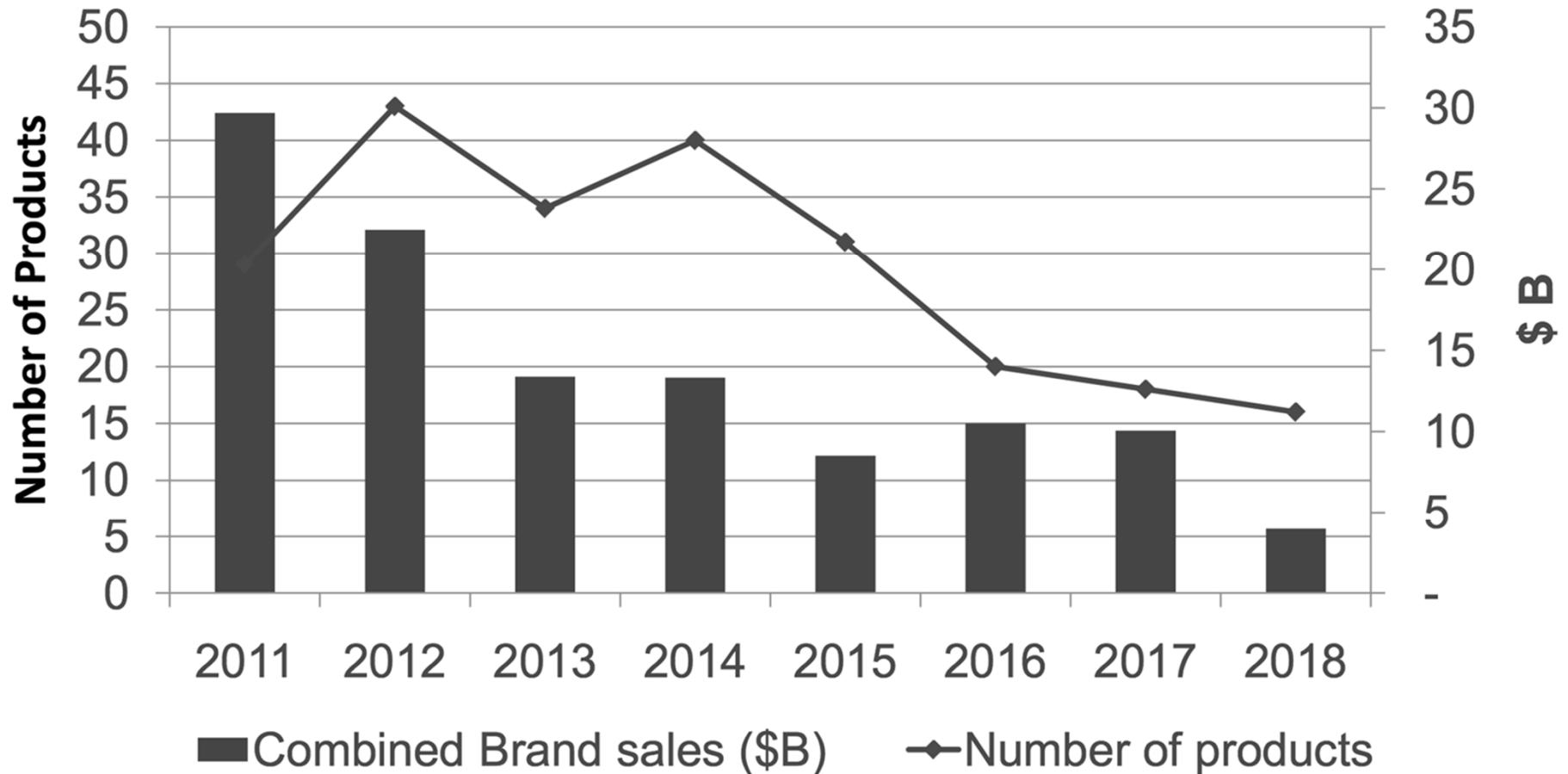
- What is going on in the minds of pharma management?
- Trends in pharma licensing
- Recent fibrosis deals
 - Who is doing them?
 - Where are they focused?
- Conclusions

What motivates pharma management?



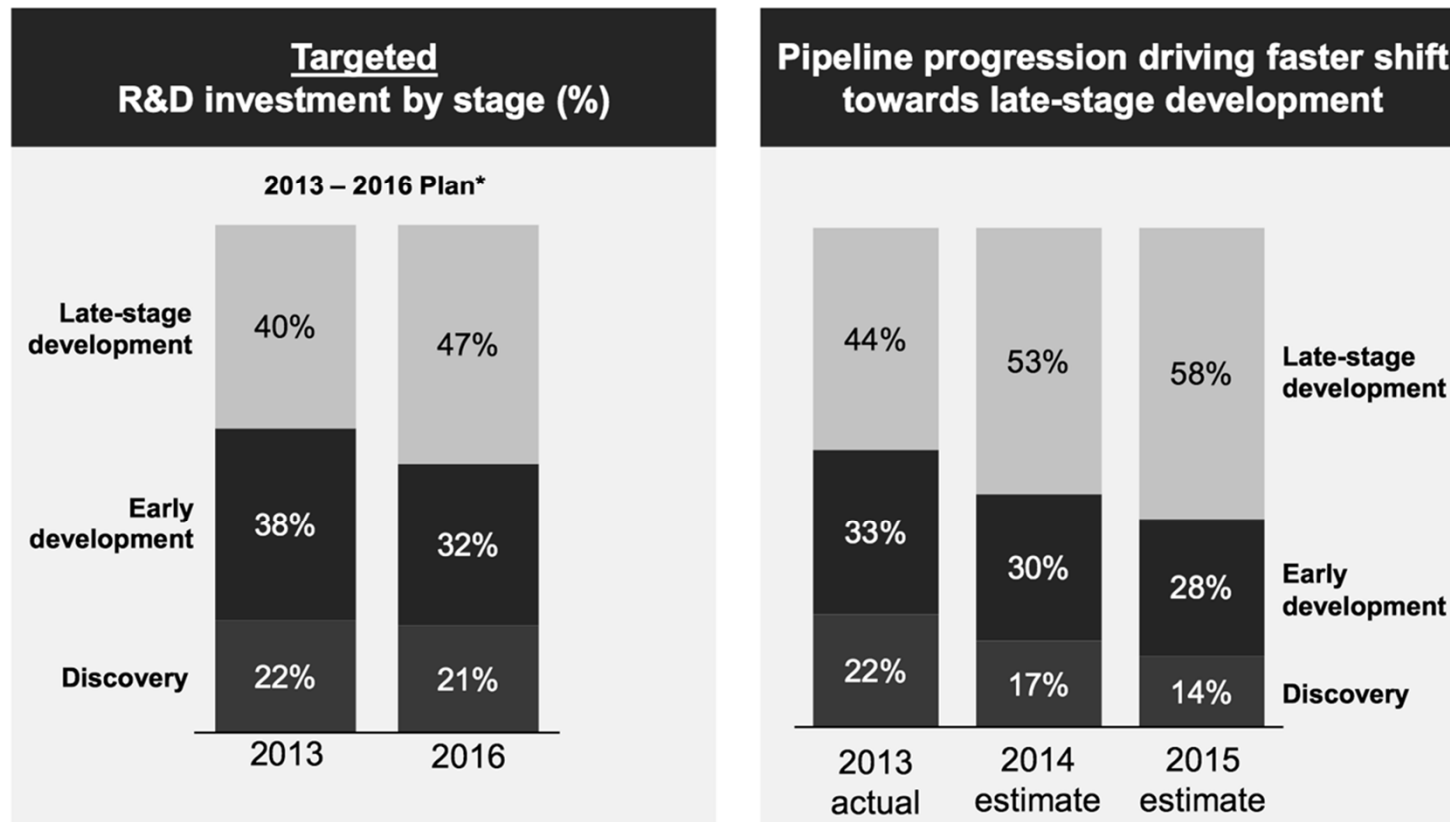
Management operate within certain constraints most importantly the targets for their own remuneration. If these are not aligned with the shareholders interests this will be a problem as that is what management will try to deliver.

Recovering from the patent cliff



Managing the P&L

R&D spend has shifted to support late-stage development

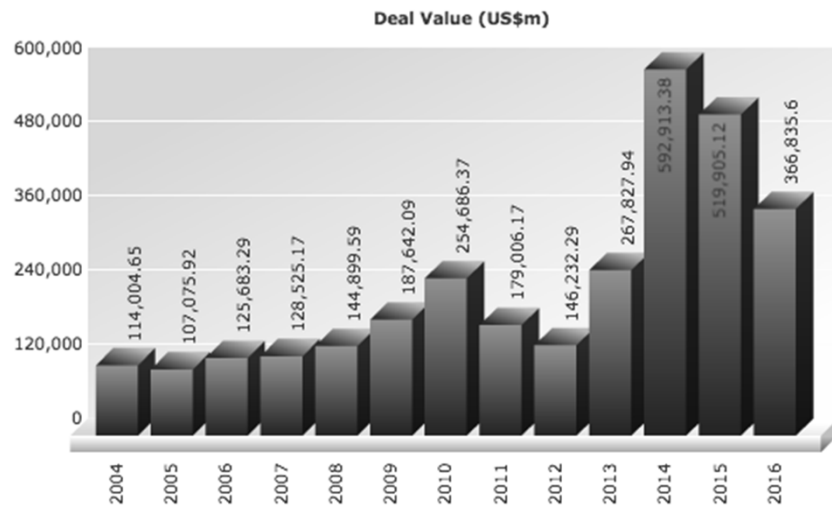


*2013-2016 plan as of March 2013

Source: AstraZeneca Capital Markets Day Nov 2014

Use of the balance sheet

Deals have increased in the wake of the patent cliff



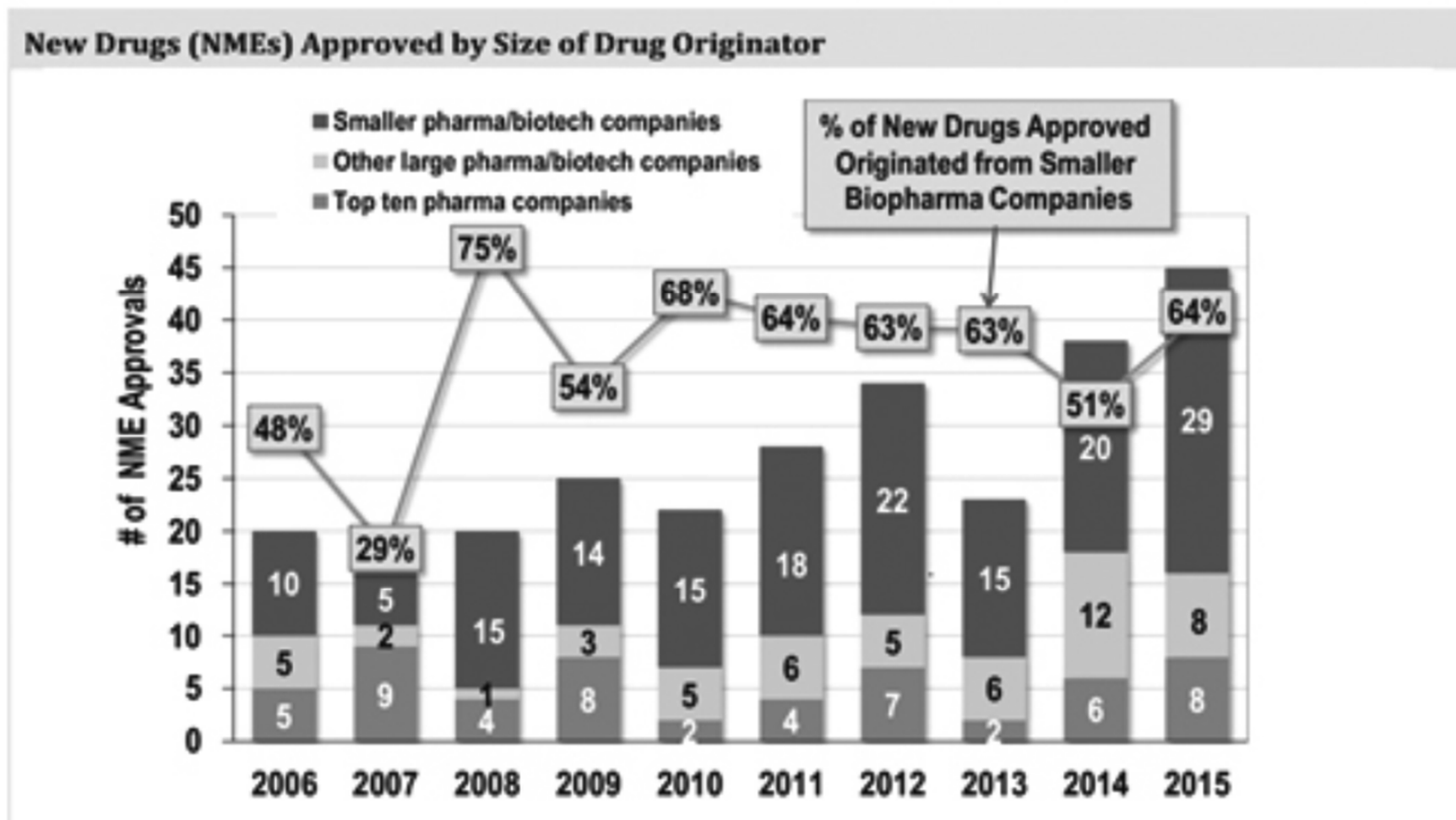
M&A Deals



Licensing Deals

M&A and licensing allows big pharma to either capitalise acquired R&D or structure a deal to ameliorate its impact on the P&L. Most large pharma focus on core EPS which excludes intangible asset write downs and ammortisation.

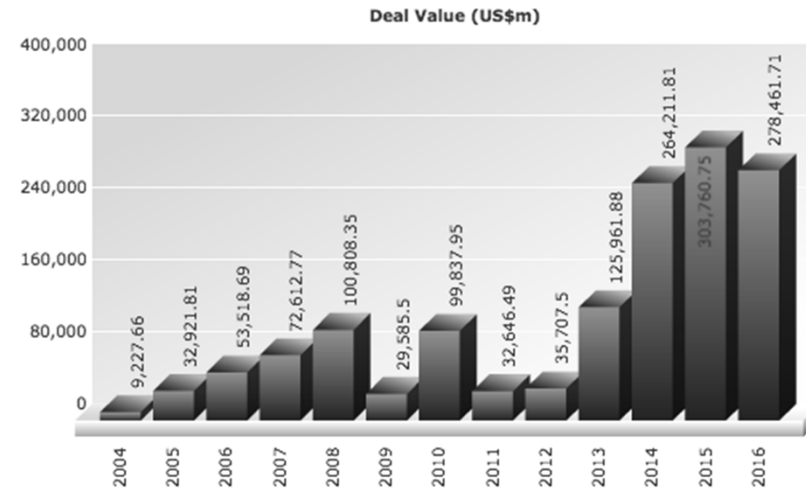
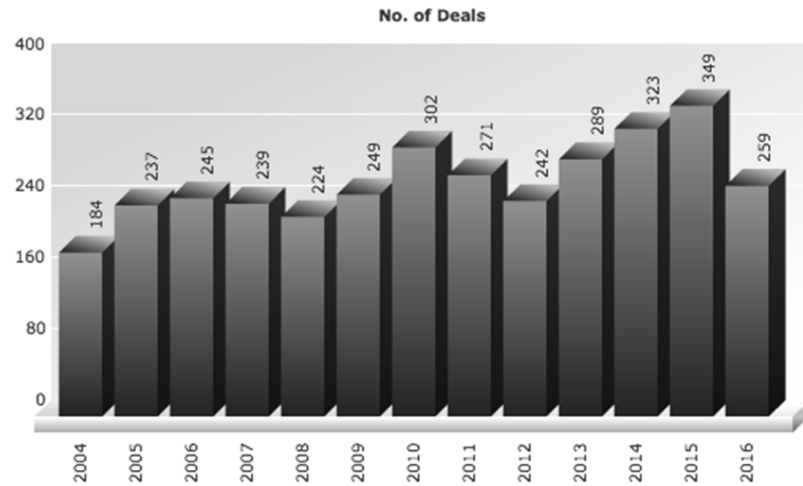
Biotech is already the discovery engine



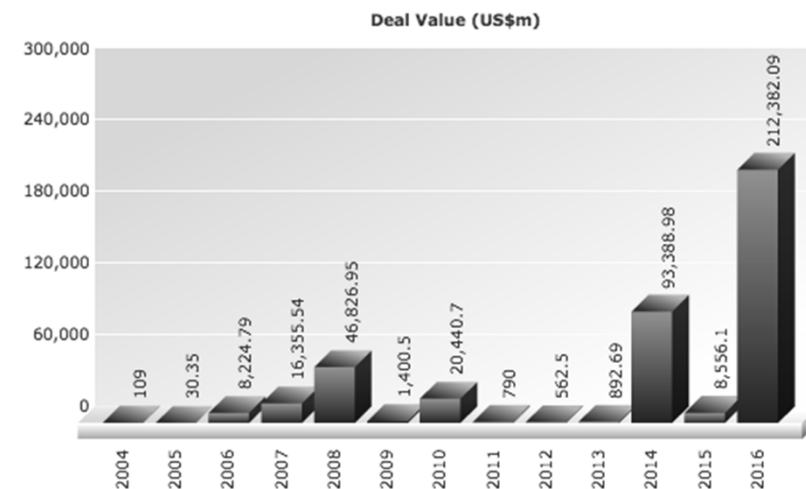
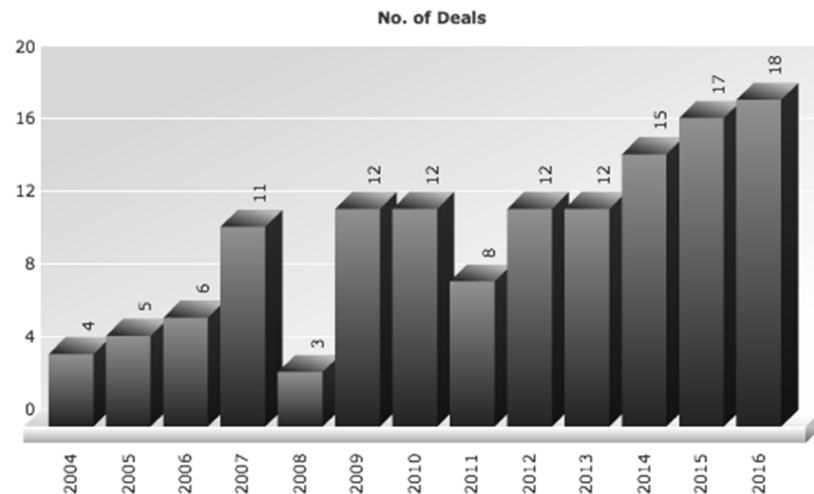
Source: FDA (www.fda.gov), HBM analysis

Areas of Pharma Focus

Oncology



Fibrosis



Fibrosis Deals

Overview

- Strategic trends that favour NASH deals
- Who are playing in the NASH space
- Strategic rationale
 - Dominate liver
 - Extension of other capability (eg fibrosis)
- How are they playing
 - Internal programs
 - Licensing
 - Acquisition
- Where on the disease spectrum are they playing (metabolic, inflammation, fibrosis)
- Who might also play

Industry Trends Favouring NASH

- **Focus on specialty or specialty led away from GP led**
 - Specialty medicines require smaller sales forces
 - Smaller sales forces mean lower fixed costs
 - Specialty medicines tend to be higher priced lower volume so higher margin
 - Sale is data driven so KOL engagement is critical
- **Payer environment reinforces data focus**
 - Pricing power available for innovation but not me toos
 - Data critical to negotiations with insurers in the US and Governments ex-US
 - Data that shows drugs lower overall healthcare burden important e.g. lower ER visits very helpful in negotiations.
- **Treatments increasingly combinations of products**
 - Pharma looking to control key components of combined regimens

NASH clinical development by company

	Metabolic modifiers	Anti-inflammatory	Anti-fibrotic	Comment
Intercept	✓			Biotech - sole NASH focus
Allergan	✓	✓		Recent significant acquisitions
Genfit	✓			Biotech - sole NASH focus
Gilead	✓	✓	✓	Numerous acquisitions – strategic focus on liver
Galmed	✓			
BMS	✓		✓	Strategic fibrosis interest extending from IPF to NASH
Galectin			✓	
Immuron		✓		
Boehringer		✓		
Shire	✓			
Novartis	✓			FXR agonist
Pfizer	?	?	?	New PhI asset but MOA not disclosed
Merck	?	?	?	Merck don't disclose PhI assets
Lilly	?	✓/?	?	CXCR1/2 ligands PhI. Metabolic disease interest.
GSK	?	?	?	Nothing in the clinic for NASH but have an IPF asset

Fibrosis & NASH M&A deals

Acquirer	Company	Indication	Deal Type	Stage	Upfront (\$M)	Potential (\$M)
< 2 years ago						
Gilead	Nimbus	NASH - metabolic	Subsidiary Aqun	P1	400	1,200
Gilead	Phenex	NASH – metabolic	Asset Aqun	P2	U	470
Allergan	Tobira	NASH - inflammatory	Acquisition	P2	400	800
Allergan	Akarna	NASH - metabolic	Acquisition	Pre	50	U
BMS	Promedior	IPF+	Acquisition	P2	150	1,250
BMS	Galecto	IPF	License	P1	U	444
BMS	Nitto Denko	NASH - fibrotic	License	P1	100	U
Boehringer	Inventiva	IPF+	License	Discovery	U	€189+
Boehringer	Pharmaxis	NASH - inflammation	Asset Aqun	P1	A\$40	A\$750+
> 2 years ago						
BMS	Amira	IPF	Acquisition	P1	325	150
Gilead	Arresto	NASH – fibrosis +	Acquisition	P1	225	225
Biogen Idec	Stromedix	IPF	Acquisition	P2	75	487
Shire	Lumena	NASH – metabolic	License	P1	260	U
Shire	Fibrotech	Diabetic nephropathy	Acquistion	P1b	75	482
AZ	Regulus	NASH- metabolic +	License + equity	Pre	U	500

Gilead Acquisitions

- **December 2010** Gilead paid \$225m to acquire Arresto a private company whose lead compound was a PhI humanised monoclonal against the LOXL2 protein.
 - The antibody (sintuzumab) only achieves around 40% inhibition of LOXL2
 - sintuzumab has subsequently failed in IPF and failed to show benefit in NASH in combination with the ASK-1 inhibitor selonsertib so development has been discontinued
- **January 2015** Gilead paid up to \$470m to acquire the Farnesoid X receptor program from Phenex Pharma. This includes two phase II compounds and the preclinical back up compounds.
- **April 2016** Gilead paid \$400m upfront and up to \$800m (of which \$200m has already been paid) in development milestones to acquire Nimbus Apollo a subsidiary of Nimbus Therapeutics. The lead candidate is a PhI NASH asset targeting acetyl CoA Carboxylase (ACC) as well as a number of preclinical assets in fibrosis and oncology.

Gilead's primary focus is NASH as opposed to other fibrotic diseases because of their history in Hepatitis which makes them liver experts with the appropriate networks and KOL connections making it a logical extension of their existing franchise.

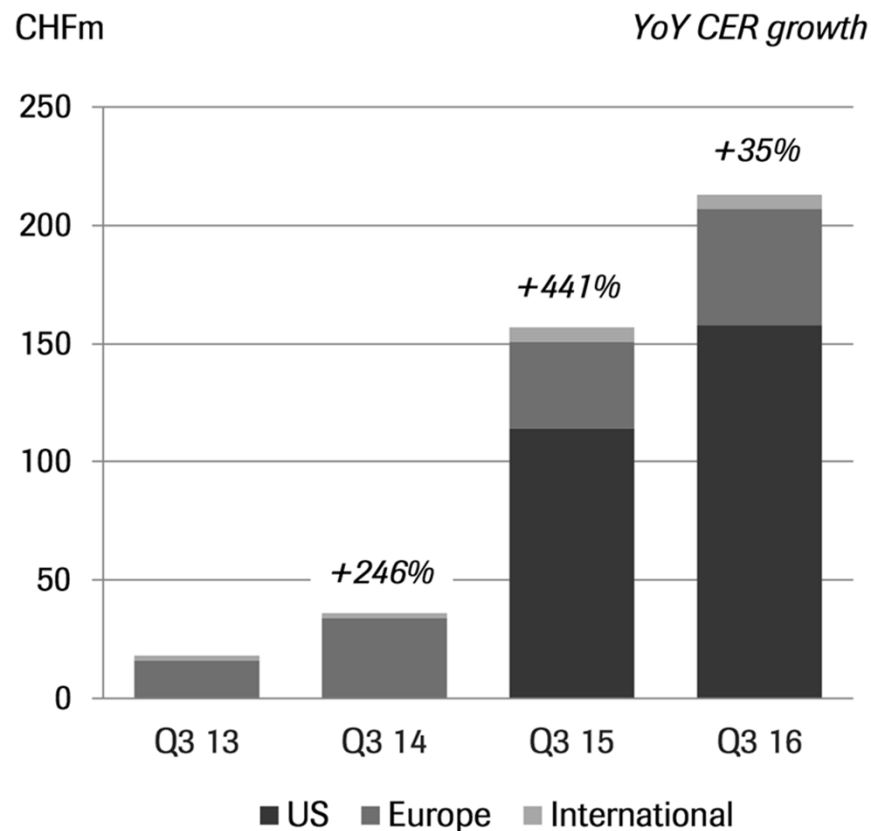
BMS Acquisitions

- **September 2011** BMS acquired the private company Amira Pharmaceuticals for \$475m cash. The lead candidate was an LPA1 receptor antagonist in PhI for IPF.
- **November 2014** BMS takes an option to acquire Galecto Biotech for \$444m and its inhaled Galectin-3 inhibitor for the treatment of IPF.
- **August 2015** BMS takes an option to acquire Promedior for \$1.25bn including \$150m upfront to fund the PhII development of their lead candidate PRM-151 a recombinant form of the human Pentraxin-2 protein. At the time of the deal development was focused on IPF and myelofibrosis.
- **November 2016** BMS paid \$100m to Nitto Denko for its siRNA program against HSP40 the lead candidate was in PhIb for NASH. There are also undisclosed royalties and milestones as well as an option to take the program into other fibrotic diseases.

As of October 1st 2016 BMS has 5 different fibrotic disease programs in the clinic. Fibrotic disease represents one of five therapeutic areas that BMS is specifically focused on. In contrast to Gilead BMS seem slightly more focused on IPF rather than NASH until the most recent acquisition.

Roche Deals

- August 2014** Roche paid \$8.3bn to buy InterMune which had one marketed product Esbriet (pirfenidone) for IPF. Esbriet looks set to be a billion dollar product despite pretty mediocre data due to lack of competition.



Esbriet Q3 2016

US (+38%)

- Growth driven by continued penetration into moderate and severe patient segments, first entries into mild segment

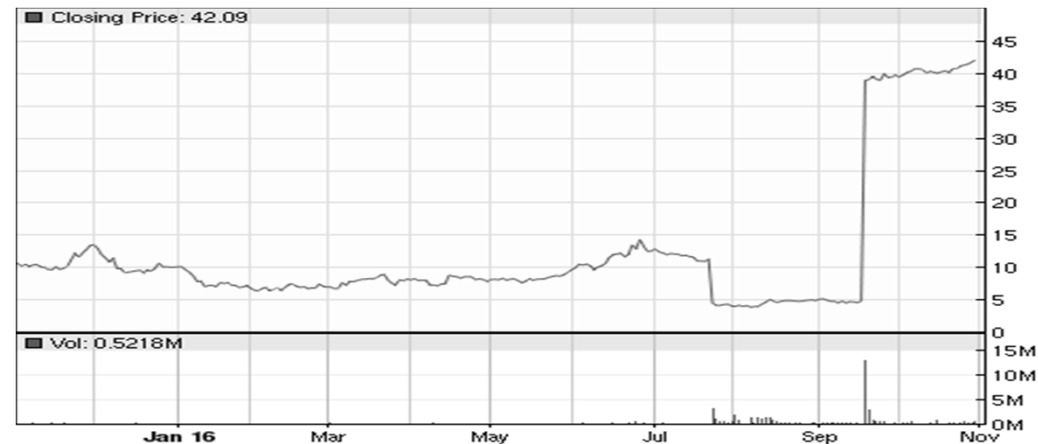
EU (+33%)

- Increasing differentiation due to strengthened label including the pooled 1 year mortality data
- Market leadership in EU5 maintained

Outlook 2016

- Targeting mild and moderate patient segments

Allergan's acquisition of Tobira



- Allergan paid \$28.35 per share plus a CVR of \$49.84 for shares that traded at \$4.59 prior to the announcement
- The share price had halved following the announcement that the lead compound cenicriviroc had failed to hit its primary endpoint in the PhII CENTAUR study.
- The CENTAUR study did hit at least one of its secondary endpoints and there was some early data on the use of cenicriviroc in combination with other agents.

Allergan's willingness to pay 6.2x the closing share price before even considering the CVR indicates this was a competitive auction

Conclusions

- Big pharma has always licensed a lot of products but there is structural pressure that is likely to increase this further.
- Focus on specialty medicines with strong data that will gain reimbursement is also driving interest in diseases such as IPF and NASH which have high unmet need. Sales of Esbriet illustrate the market potential.
- A number of players have started consolidating the space and recent business development deals have clearly been competitive auctions and the number of deals in the space have increased over the last five years.
- PXS have one of the only unencumbered clinical stage assets in the anti-fibrotic space. It is an obvious acquisition target for consolidators looking for combination therapies.

The range of upfronts/acquisition prices paid (before earn outs) for Pre-clinical to PhII assets in the last 2 years has been ~\$50-400m. Pharmaxis' current EV is ~\$60m.

If the market won't value the asset appropriately big pharma will.