

# **ASX ANNOUNCEMENT**

# SHAREHOLDER UPDATE: EXOPHARM IN 2020 - NEWS, DEALS AND CLINICAL TRIALS

10 February 2020

### **HIGHLIGHTS**

- Exopharm is now a clinical stage company and the first company in the world to test a proprietary exosome product in human clinical trials for regenerative medicine
- 2020 set to be a year of inflexion points with newsflow, partnering activity and clinical trials
- Seeking to build significant shareholder value

Exopharm Limited (ASX:EX1) is a young clinical stage Australian company based upon using exosomes from cells as cell-free medicines. Exopharm (with \$5.8m in the bank as at December 2019) has raised ~\$14m, has made significant progress in its first year as a listed company and is steadily building value.

# 2020 for Exopharm

2020 is a year of inflexion points at Exopharm in three key areas :-

**Newsflow** – non-clinical testing (in vitro, ex vivo and in vivo) is building in momentum. We expect more announcements of test results through 2020. Testing in non-clinical models of erectile dysfunction, ocular disease, ageing, hearing and wound healing is underway. We're also looking at ways to use our 'engineered' exosomes to solve some significant medical problems including some selected genetic orphan diseases and cancer.

**Deals** – partnering discussions started in 2018 and increased pace in 2019. As a leader in the clinical use of exosomes, Exopharm is attracting the attention of larger companies wanting to enter the exosome field. Exopharm's professional approach, leading manufacturing capability and our LEAP Technology are the standout attractions for potential partners.

**Clinical trials** – we are planning clinical trials using allogeneic (off-the-shelf) exosomes. Areas of particular interest include post-operative erectile dysfunction, ocular disease, osteoarthritis and tendinopathy. Completion of Phase II studies are aimed at building value over the next few years. We are also preparing for interactions with the FDA in 2020, building upon our Australian PLEXOVAL study.

## **Achievements in 2019**

One standout advancement through 2019 was the growing Exopharm team. Exopharm now has a full-time team of 23 people – covering commercialisation and partnerships, manufacturing, analytics, development (i.e. testing), new product development and

management. Our employees have extensive biotechnology experience and previously worked for companies such as CSL, Polynovo, Opthea, Cynata, McKinsey & Company, Bayer, Amgen, Terumo and Zoetis.

Exopharm also won approval for the PLEXOVAL study in 2019, with first dosing occurring early in 2020.

# Clear strategy to build value

Over the past 6 months Exopharm has refined its strategy and focus, as detailed in the updated investor presentation. The aim is to maximise shareholder value over the long term and capitalise upon the power of the LEAP purification technology owned by Exopharm.

The LEAP technology triggered a burst of innovation in exosome manufacture that has in turn propelled Exopharm to the front-runner position in clinical trials and the potential use of exosomes in many clinical applications. But exosomes have so many potential applications and Exopharm is a new company with limited resources.

To focus our attention and use of capital, Exopharm has decided on a clear development and partnership strategy:-

**Core programs** [partnering after Phase II human trials] –fund our own clinical trials through to the end of Phase IIb only in **mobility** and **sensory** deficit areas (e.g. treatments for osteoarthritis, tendinopathy, bone repair, muscle, dry age-related macular degeneration, hearing and erectile dysfunction)

**Non-core programs** [partnering early with non-clinical in vitro or in vivo data] – fund early proof of concept tests in areas outside of mobility and sensory (e.g. cardiac repair, neurodegeneration, autoimmune disease, transplant rejection, cancer and autosomal dominant conditions)

'Engineered exosomes' are also part of our non-core programs and are intended to be spun out or partnered early.

Partnerships have the potential to deliver upfront fees, milestone payments and royalties.

## Recent deals in the exosome field

Commercial activity is already strong in the exosome field. Some example deals are listed below:

Established	<b>EV Partner</b>	Interaction
Company		
Jazz Pharmaceuticals	Codiak Biosciences	Access to Codiak's exosome platform for
(NASDAQ: JAZZ)	Private	cancer therapies
US\$8.6 bln	US	Undisclosed upfront with milestones
		exceeding\$1billion

Roche Puretech Health PLC Milk-derived exosome technology (SWX: ROG) (LON: PRTC) US\$36mln upfront with milestones

CHF 271 bln GBP 860 mln **exceeding \$1billion** 

Bio-Techne Corp Exosome Diagnostics Acquisition of diagnostic technology (NASDAQ: TECH) Inc. US\$250mln upfront with additional

US\$8.3 bln Acquired **US\$325mln in milestones** 

# Cell therapy companies pivoting to exosomes

While cellular therapies in regenerative medicine are gaining approvals and revenue is building, the mechanism by which these therapies work has led a growing number of stem cell companies to see the light and shift their development focus from stem cell products to exosome products.

Recent research points to stem cell-derived exosomes being responsible for the healing effects of cellular therapy, so the commercial success of cell therapy companies could bolster the interest in exosomes as medicines.

Pivoting cell therapy companies are potential partners for Exopharm.

By the Board - this announcement has been authorised for release by the board.

## **Company and Media Enquiries**

For further information about this announcement, please contact:

Join our mailing list to receive updates: info@exopharm.com <a href="https://www.exopharm.com">www.exopharm.com</a>

P: +61 (0)3 9111 0026

Rudi Michelson Monsoon Communications Tel: +61 (0)3 9620 3333

#### **ABOUT EXOPHARM**

Exopharm Limited (ASX:EX1) is a clinical-stage Australian regenerative medicine company developing therapeutic exosome products as alternatives to stem-cell therapies.

Exosomes are small particles naturally produced by cells, which deliver therapeutic 'cargoes' to other cells to reduce inflammation and promote regeneration. Exosomes are plentiful in our youth but decline with age. Recent research points to exosomes as a way to extend the number of healthy, functional years (extending health span).

Exosomes secreted by stem cells could be used instead of stem-cell therapy with equal or greater benefit – and without the problems of stem-cell therapies. They could be used to deliver targeted 'novel' drugs and have potential as diagnostics.

While trillions of exosomes are produced by stem cells, the real challenge is to 'purify' them as drug products. Exopharm owns a purification technology called Ligand-based Exosome Affinity Purification (LEAP). LEAP technology and associated know-how places Exopharm at the forefront of this emerging field worldwide. Exopharm is at clinical stage with pending and current trials for wound healing, dry aged-related macular degeneration and osteoporosis.

Exopharm was founded in 2013 by Dr Ian Dixon, co-founder of the ASX-listed stem-cell therapy developer Cynata Therapeutics. He was also a director of Cell Therapies, which produced adult stem cells for ASX-listed stem cell company Mesoblast. Exopharm listed on the ASX in December 2018.

#### FORWARD LOOKING STATEMENTS

This announcement contains forward-looking statements which incorporate an element of uncertainty or risk, such as 'intends', 'may', 'could', 'believes', 'estimates', 'targets', 'aims', 'plans' or 'expects'. These statements are based on an evaluation of current corporate estimates, economic and operating conditions, as well as assumptions regarding future events. These events are, as at the date of this announcement, expected to take place, but there cannot be any guarantee that such events will occur as anticipated or at all given that many of the events are outside of Exopharm's control or subject to the success of the Development Program. Furthermore, the Company is subject to several risks as disclosed in the Prospectus dated 6 November 2018.

#### INHERENT RISKS OF INVESTMENT IN BIOTECHNOLOGY COMPANIES

There are a number of inherent risks associated with the development of biopharmaceutical products to a marketable stage. The lengthy clinical trial process is designed to assess the safety and efficacy of a drug prior to commercialisation and a significant proportion of drugs fail one or both of these criteria. Other risks include uncertainty of patent protection and proprietary rights, whether patent applications and issued patents will offer adequate protection to enable product development, the obtaining of necessary drug regulatory authority approvals and difficulties caused by the rapid advancements in technology. Companies such as Exopharm are dependent on the success of their research and development projects and on the ability to attract funding to support these activities. Investment in research and development projects cannot be assessed on the same fundamentals as trading and manufacturing enterprises. Therefore, investment in companies specialising in drug development must be regarded as highly speculative. Exopharm strongly recommends that professional investment advice be sought prior to such investments.