# ASX ANNOUNCEMENT

## Investor Presentation - Delivering Transformative Medicines

**16 April 2021, Melbourne, Australia:** Exopharm Limited (ASX:EX1), a clinical-stage company at the forefront of developing transformative exosome therapeutics, is pleased to provide the attached Investor Presentation titled "Delivering Transformative Medicines."

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


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## ABOUT EXOPHARM

Exopharm (ASX:EX1) is a clinical-stage biopharmaceutical company using exosomes to deliver a new class of transformative medicines and generate revenue from multiple partnership deals.

Exosomes are seen by the Biopharma industry as a highly differentiated platform with the potential to enhance tissue delivery for a variety of payloads like mRNA and proteins – part of the global market for drug delivery systems which is growing at a compound annual growth rate (CAGR) of 5% and valued at around US$170 billion in 2021.

For some medicines, exosomes are an alternative and superior means for delivery inside the body, alongside technologies such as lipid nanoparticles (LNP), cell penetrating peptides, viral vectors and liposomes.

Exopharm's LEAP technology solves the challenge of purifying clinical-grade exosomes at large scale and low cost.

Exopharm also has two exclusive proprietary technologies that allow advanced customisation of exosomes – the LOAD technology improves loading of nucleic medicines into exosomes and the EVPS technology allows exosomes to be directed towards selected cell types.

Exopharm uses variations and combinations of LOAD and EVPS to enable its Biopharma partners to improve delivery of their drug candidates and help them design and test new exosome medicines aimed at treating a wide scope of medical problems including neurological disease, infectious disease, cancer, and fibrosis.

## 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.


# Exopharm Ltd

## *Delivering Transformative Medicines*

April 2021

Updated Investor Presentation

# IMPORTANT INFORMATION

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# Exopharm Ltd

## Overview

- Australian clinical-stage company at the forefront of developing transformative medicines based upon exosomes (extracellular vesicles, EVs)
- 40 staff based in Melbourne, Australia; 1 based in Europe
- Publicly traded on the ASX (ASX:EX1) (listed Dec 2018) 139.4 million shares on issue, current market cap. ~AU$100 million
- A platform technology company with application to many exosome medicines – using our exclusive LEAP, LOAD and EVPS technologies

## Priorities

- Making exosome medicines available to established biopharmaceutical companies to enable successful delivery of existing drug candidates
- Generating revenue in the near-term through multiple deals
- Building a valuable leadership position in the emerging exosome medicines field through our exosome technologies and exosome medicines


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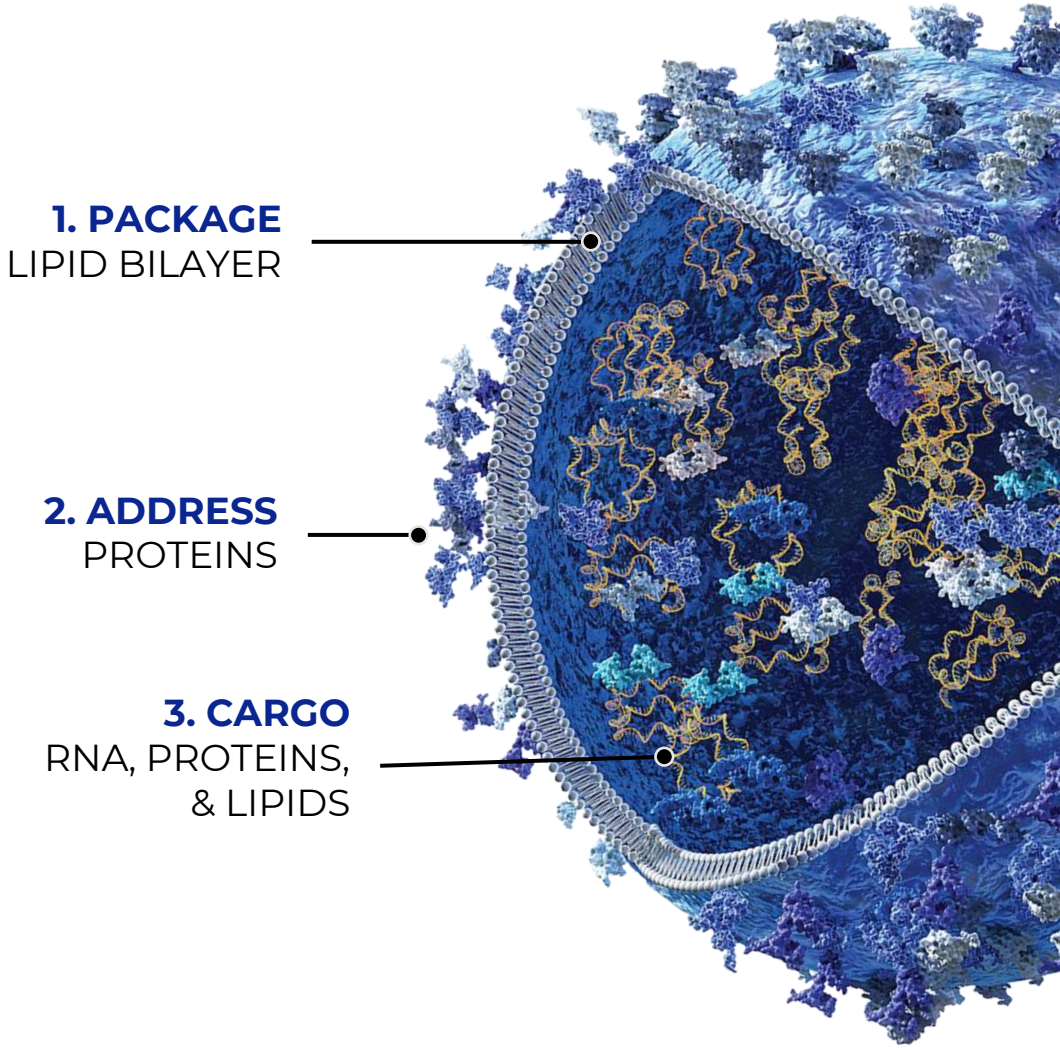
# Exosomes: Nature's Solution to Delivery in the Body

Exosomes (also referred to as extracellular vesicles or EVs) are **natural, multifunctional and stable nanoparticles that transfer cargo and messages between cells.**

Natural exosomes can be produced from cells in a bioprocessing facility.

| | |
|---|---|
| **1. PACKAGE** | Outer membrane that forms the exosome (same membrane as human cells) |
| **2. ADDRESS** | External proteins that improve targeting of exosomes to specific cell types |
| **3. CARGO** | The materials delivered by exosomes, including instructions (RNA) and building materials (lipids, enzymes, proteins) |

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# Uncovered by Scientists in 2007, in 2021 Exosomes are Emerging as a New Frontier in Medicines Delivery

> Exosomes represent a dynamically growing segment in life sciences with opportunities in research, diagnostics and therapeutic applications.
>
> **Dr Uwe Gottschalk, Chief Technology Officer for Lonza (2017)**

> Targeted and non-targeted exosomes offer a highly differentiated platform with the potential to enhance tissue delivery for a variety of payloads like mRNA and proteins.
>
> **Dr Madhu Natarajan, Head of the Rare Diseases Drug Discovery Unit at Takeda (2020)**

> Now is the time for researchers to usher in a new era of therapeutic possibilities using RNA-delivering, natural exosome vesicles.
>
> **Professor Phillip Askenase MD, Yale University School of Medicine (2020)**

**Exopharm is at the forefront of exosome medicines**

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# Many Modern Drugs Need Packaging and Addressing to Solve the '*Delivery Problem*'

| Drug type | Common delivery problems |
|---|---|
| Small molecules | • Inability to cross the blood-brain barrier<br>• Metabolism (degradation) in the blood<br>• Poor delivery to inside the cell<br>• Not targeted |
| Nucleic acids (e.g. mRNA, miRNAs) | • Degradation in the blood<br>• Stimulation of immune response<br>• Poor delivery efficiency |
| Gene therapy | • Inability to reach nucleus of the cell<br>• Degradation in the blood |

The global market for drug delivery systems is projected to reach USD 175 billion by 2021

https://formulations.pharmaceuticalconferences.com

Pharma companies have medicines that need ***better delivery*** to become transformative

There is a USD175 billion p.a. industry around drug delivery and formulation

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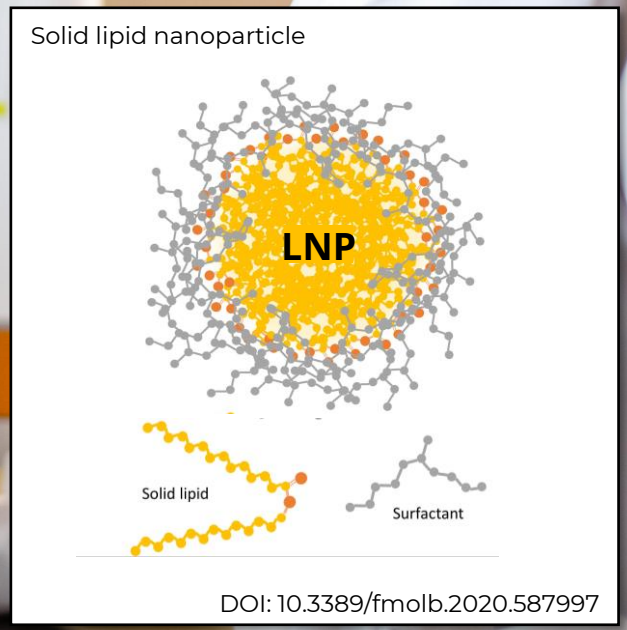
# SARS-CoV-2 RNA Vaccines Use “Artificial Exosomes”

Pfizer and Moderna SARS-2 vaccines package RNA into artificial synthetic lipid nanoparticles (LNPs)

However, LNPs have **major limitations for drug delivery:**

- Toxicity
- Anti-LNP immune response
- Inefficient cellular delivery

Naturally occurring exosomes from human cells have evolved an optimal composition over billions of years **and have none of these limitations**.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# Exosomes Overcome Significant Issues Associated with Other Drug Delivery Technologies

| Delivery technology | Efficient delivery | Non toxic | Tropism | High stability | Multiple dosings |
|---|---|---|---|---|---|
| Lipid nanoparticles (LNPs) | ✗ | ✗ | ✓ | ✓ | ✓ |
| Liposomes | ✗ | ✗ | ✓ | ✓ | ✓ |
| Cell-penetrating peptides | ✗ | ✓ | ✓ | ✗ | ✓ |
| Viral vectors | ✓ | ✗ | ✗ | ✓ | ✗ |
| **Engineered exosomes with EVPS and LOAD** | ✓ | ✓ | ✓ | ✓ | ✓ |

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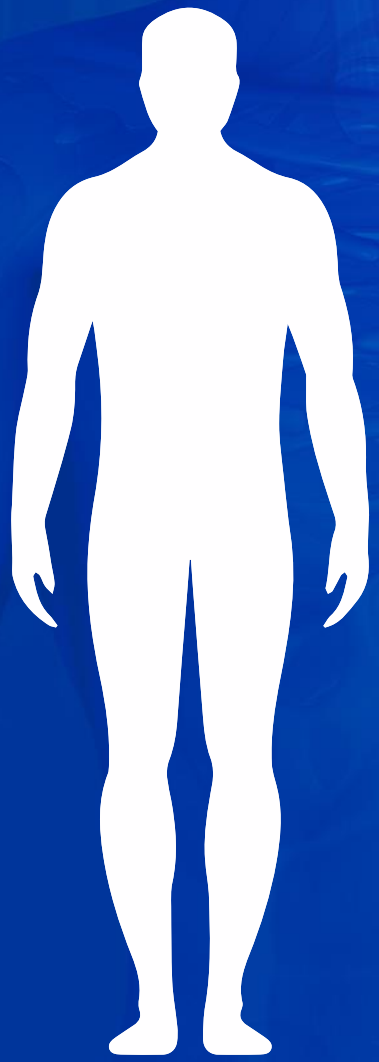
# Exosome Medicines Can Have Broad Application to Solve Many Medical Problems

Orphan indications/ genetic conditions (e.g. cystic fibrosis)

Infectious disease (viral, bacterial, parasite)

Cancers/oncology

Psychiatric & pain



CNS/neurological

Disability (hearing, taste, mobility)

Cardiovascular

Immune conditions (e.g. GvHD, TID, transplant rejection, IBD)

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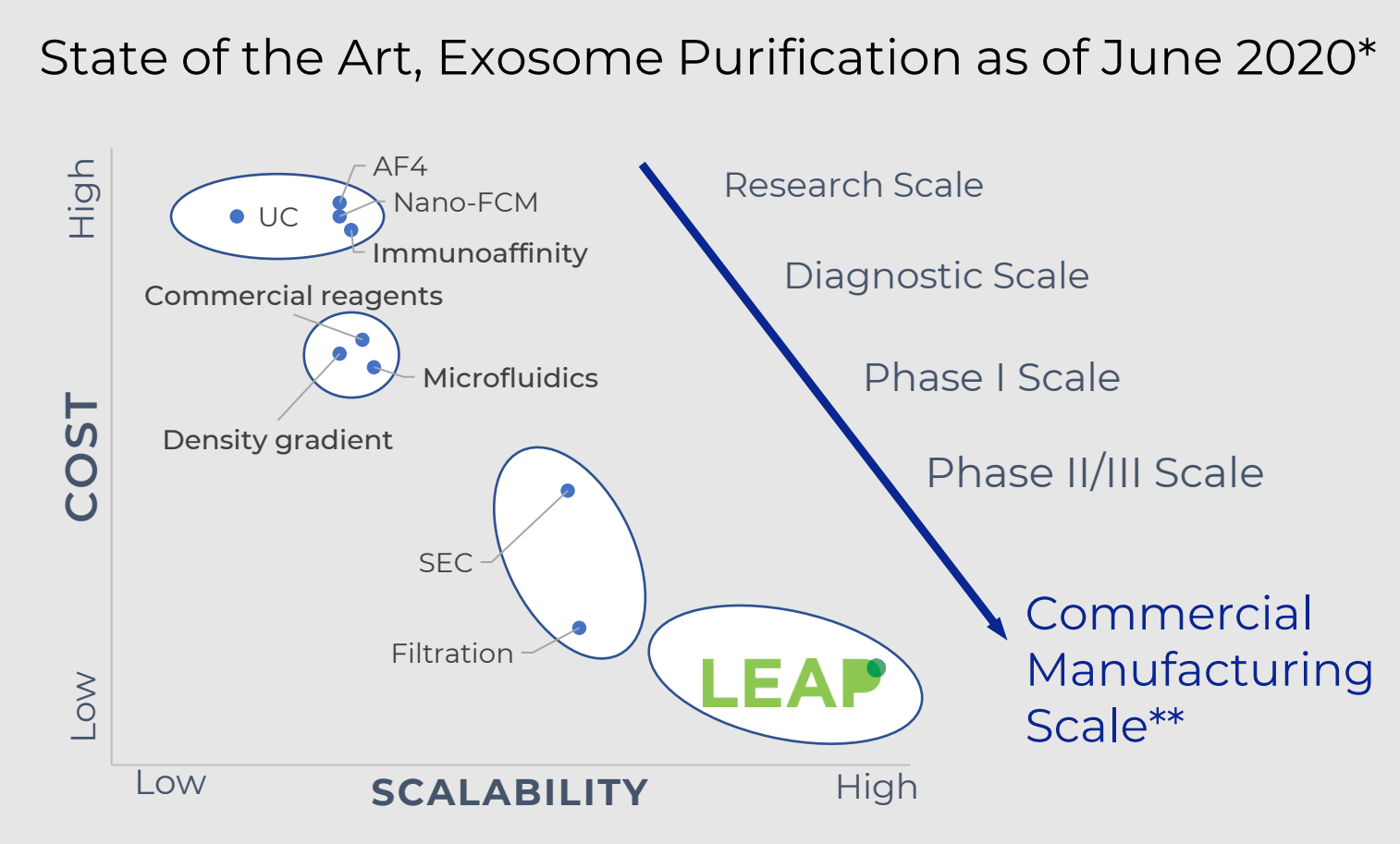
# Exopharm Has the Only Known Technology for Commercial-scale Exosome Medicine Purification




Unlike all alternatives, LEAP technology:

1. Uses industry-standard equipment/processes
2. Uses low-cost, reusable consumables
3. Scales economically beyond thousands of doses
4. Is proprietary (i.e. patent applied for)

**LEAP unlocks the potential of Exosome Medicines**

** LEAP assessment from Exopharm, based on industrial use to date; LEAP Patents processing through National phases at present.
* Adapted from https://doi.org/10.1016/j.tibtech.2020.05.012

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# Three Unique & Powerful Technologies Underpin our Partnering and Exosome Medicines

| | | Exclusivity |
|---|---|---|
| LEAP | Scalable, economical GMP process for purifying exosomes | **100% Exopharm** |
| LOAD | Activity enhancing method for delivering RNA by exosomes | **100% Exopharm** |
| EVPS | Tropism-conferring approach for engineering exosome surface proteins | **100% Exopharm** |

*EVPS US patent is granted, LOAD patent is progressing and LEAP patent is progressing through national phase in USA under fast-track process.*

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# Exopharm is Proud to Be One of Few Exosome Medicine (EM) Companies Worldwide

| Leading EM Company | EM IP Status | Pharma Partner | Date | Area | Upfront | Milestones |
|---|---|---|---|---|---|---|
| evox THERAPEUTICS | ☑ | Lilly | **Jun 2020** | **CNS** | **> $50 million** | **$1.7 billion** |
| | | Takeda | Mar 2020 | Rare diseases | **$63 million** | **$1.3 billion** |
| CODIAK | ☑ | Jazz Pharma | Jan 2019 | Cancer | **$77 million** | **$1.8 billion** |
| | | SAREPTA THERAPEUTICS | **Jun 2020** | **CNS** | **> $100 million** | **Not disclosed** |
| exopharm | ☑ | Discussions are underway with multiple potential partners | | | | |

Other emerging exosome medicine companies include PureTech, ARUNA, Carmine, Mantra, Ciloa, Anjarium, Ilias

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# Exopharm is Building Financial Value via Two Main Streams – *concurrent business opportunities*

## Outlicensing LEAP technology to others for upfront fees, milestone fees and royalties*

- Early revenue from tech transfer & licensing for research & clinical trial exosome medicines
- License to blood plasma, cell product and bioprocessing companies, CMOs, blood centres, etc

thermo scientific  Lonza  REPLIGEN INSPIRING ADVANCES IN BIOPROCESSING

## Developing exosome medicines utilising Exopharm's three technologies (LEAP, LOAD & EVPS)

- Early revenue from partnership deals incl. upfront & milestone payments* – ***Pharma company nominates the drug to deliver and invests in the development themselves***
- Development of our own Exosome Medicines – ***we own and invest in the development of the exosome medicine***

moderna  SAREPTA THERAPEUTICS  Lilly

* Example possible potential partnerships – inclusion of company name and brand does not indicate an actual commercial or legal relationship with Exopharm at this time. The brand remains the property of the company.

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# Right Place, Right Time, Right People

HAEMONETICS®

CSL™

BAYER

PolyNovo

McKinsey &Company

OPTHEA

zoetis

TERUMOBCT

New York Blood Center

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# Thank you

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