

## ASX ANNOUNCEMENT & MEDIA RELEASE

### Added intellectual property empowers Exopharm's engineered exosome pipeline

#### HIGHLIGHTS

- Engineered exosomes or extracellular vesicles (EEVs) have been the subject of recent partnering deals in the exosome/EV field
- Exopharm has a pipeline of EEV products under development for potential future partnering
- Together, these technologies enable unprecedented capability for manufacturing EEVs for a wide range of therapeutic applications

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**Melbourne, Australia:** Exopharm Limited (ASX:EX1) is now developing EEV exosome medicines to include potential applications for cancer, rare disease, infectious diseases and neurological conditions.

Exopharm now holds a portfolio of exclusive worldwide intellectual property (IP) rights for the design and manufacture of a pipeline of EEV products.

This IP now includes:

**LEAP™**, wholly-owned IP covering the proprietary isolation and purification of all EVs;

**LOAD™**, IP for the insertion of custom-designed nucleic acids, such as messenger RNA (mRNA), interfering RNA (RNAi), microRNA (miRNA) and silencing RNA (siRNA) – into EVs in-licensed from the State University of New York at Buffalo USA; and

**EVPS™**, IP for the attachment of custom proteins to the surface of EVs to enable targeting of EVs to selected cell types, in-licensed from Santa Clara University USA.

"LEAP, LOAD and EVPS IP are a powerful set of tools that enable us to design and make a range of important and novel EEV products to treat a number of medical problems," said Dr Ian Dixon, Managing Director of Exopharm. "The synergy of these three technologies is harnessed in our new prototype product **Fortrexo CoV**, for fighting Coronavirus infection by delivering virus-specific RNAi into at-risk cells, arresting virus replication in the cell."

"Our EEV technology portfolio is exciting and gaining attention," said Dr Chris Baldwin, Chief Commercial Officer at Exopharm. "As we meet with companies worldwide at the (virtual) Bio 2020 event this week, potential partners are interested to use EEVs in everything from rare

genetic diseases to cancer to neurology. With LOAD, we can better deliver new treatments into the body and with EVPS, we can make sure they target the cells that need it.”

Exopharm has exclusive worldwide rights to use or on-license each of the three technologies by indication or by geography. Different EEV products will combine one or more of the technologies so partnerships can be structured in ways that are complementary to Exopharm’s advancement of its fully-owned clinical assets, including Plexaris and Cevaris.

“The in-licensing of LOAD and EVPS has involved an extensive search and due diligence process that has been very deliberate,” said Dr Lieven Huang, Head of Business Development and Licensing at Exopharm. “We have looked at a number of patents and in-licensing opportunities, and this IP places Exopharm in a strong position for EEVs and partnerships.”

Exopharm’s EEV products, including Plexodox and Fortrexo CoV, have potential to treat a range of medical conditions with unmet medical needs – including cancer, cardiac disease, rare diseases and conditions (e.g. cystic fibrosis), infectious diseases and neurological conditions.

As released to the ASX on 28 May 2020, Exopharm is utilizing its manufacturing capability and know-how in new EEV product areas to progress into proof-of-concept *in vitro* testing, then potentially non-clinical and then clinical studies with selected EEV products.

The EVPS IP has been in-licensed from Santa Clara University, USA ([www.scu.edu](http://www.scu.edu)) and the LOAD IP has been in-licensed from the State University of New York at Buffalo, USA ([www.buffalo.edu](http://www.buffalo.edu)). Both license agreements include non-material annual commitments to keep each license and associated patents in good standing as well as standard commercial royalties on product sales.

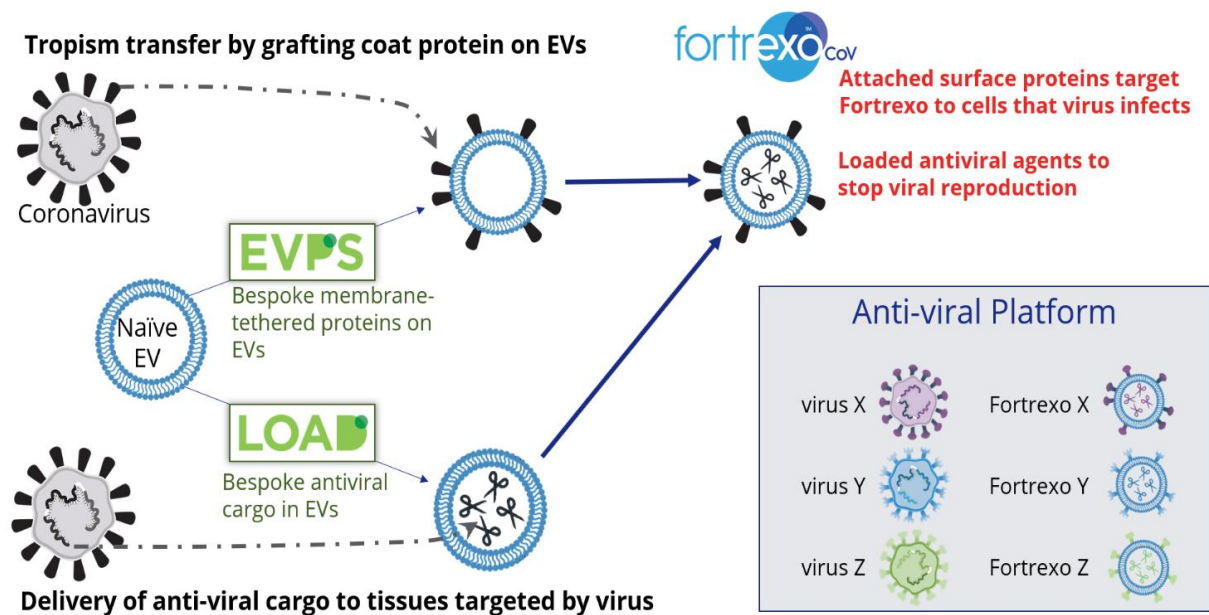


Figure 1. Targeted application of newly in-licensed technologies for the development of an anti-virus treatment for coronavirus and other viruses.

## Glossary

Cevaris	Exopharm's mesenchymal stem cell-derived extracellular vesicle medicine currently in non-clinical efficacy trials.
DNA	deoxyribonucleic acid is the carrier of genetic information
EVPS	Exopharm's in-licensed technology for attaching specific proteins to the surface of EVs. EVPS allows EVs to be targeted to specific cells.
Fortrexo	Exopharm's anti-virus products targeted at a range of viruses
Fortrexo CoV	Exopharm's anti-virus project targeted at stopping the virus responsible for COVID-19, a prototype product which is yet to be tested
LOAD	Exopharm's in-licensed technology for adding selected nucleic acids to the payloads of EVs. Nucleic acids (e.g. mRNA, RNAi and siRNA) can modify how cells operate.
mRNA	Messaging RNA is a single-stranded RNA molecule that encodes a gene product and is complementary to one of the gene's DNA strands
miRNA	miRNA is a short non-coding RNA molecule (around 22 nucleotides in length) that performs RNA silencing and regulation of gene expression
non-coding nucleotides	A nucleic acid that does not code for a gene protein or other product Nucleotides are the basic unit of nucleic acids such as DNA and RNA
Plexaris	Exopharm's platelet-derived extracellular vesicle medicine currently in human safety trials.
Plexodox	Exopharm's formulation of doxorubicin within Plexaris.
RNA	Ribonucleic acid is essential in various biological activity in cells, including the production of proteins and other gene products inside a cell
RNAi	RNA interference is when certain sequences of RNA molecules inhibit gene expression by interfering with targeted mRNA molecules
siRNA	Small interfering RNA are double-stranded RNA molecules, 20-25 base pairs in length, operating similar to RNAi

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

### **Company and Media Enquiries:**

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## **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.