

ASX Announcement

Melbourne, Australia, 13 October 2022

UPCOMING WEBINAR

- Webinar: Exosomes: Unleashing Genetic Medicine Potential

Genetic medicine and exosome-based drug-delivery company Exopharm Limited (ASX:EX1) releases notice of, and information to be covered in an upcoming webinar.

Title: Exosomes: Unleashing Genetic Medicine Potential

Time: 8:00 (Melbourne, Victoria, Australia), 14 October 2022

Registration: <https://exo.ph/Exosomes-Unleashing-Genetic-Medicine-Potential>

By the Managing Director – this release has been authorised by the Managing Director.

COMPANY AND MEDIA ENQUIRIES:

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ABOUT EXOPHARM

Exopharm (ASX:EX1) is a leader in advancing Genetic Medicines and other exosome-based medicines using exosomes or extracellular vesicles (EVs) as a chassis for improved and non-viral drug-delivery.

Exopharm (ASX:EX1) is pursuing a product pipeline-driven platform strategy. Exosomes can be loaded with a variety of active pharmaceutical ingredients (APIs) and can be targeted to selected cell-types and tissue types, improving the safety-profile of the APIs and providing better treatments. Exosomes can be used to deliver small molecule drugs, mRNA, DNA and other types of APIs.

Exosomes are an alternative means of drug-delivery inside the body, alongside technologies such as lipid nanoparticles (LNP), cell-penetrating peptides, viral vectors and liposomes.

Exopharm's exosome technologies solve important needs for the success of exosome medicines – **LEAP** manufacturing technology, **LOAD** API loading technologies and **EVPS** tropism technologies.

Exosome-based medicines could improve the treatment of many chronic or inherited medical conditions.

Exopharm is making its proprietary technologies available to pharmaceutical and biotechnology companies that want to harness exosome-delivery for their own products.

In addition, Exopharm is using its technology platform to enable its own product development programs - each aimed at delivering a transformative medicine for an unmet medical need.

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.



Webinar:

Exosomes: Unleashing Genetic Medicine Potential

Thu 13 October 2022 – 17:00 (Boston) / 14:00
(San Francisco)

Fri 14 October 2022 – 08:00 (Melbourne)

Registration:

<https://exo.ph/Exosomes-Unleashing-Genetic-Medicine-Potential>



Dr. Anna Cifuentes-Rius,
Research Innovation
Manager



Mr. David Oxley,
President International



Ms. Mehak Bhola,
Biotechnology Manager

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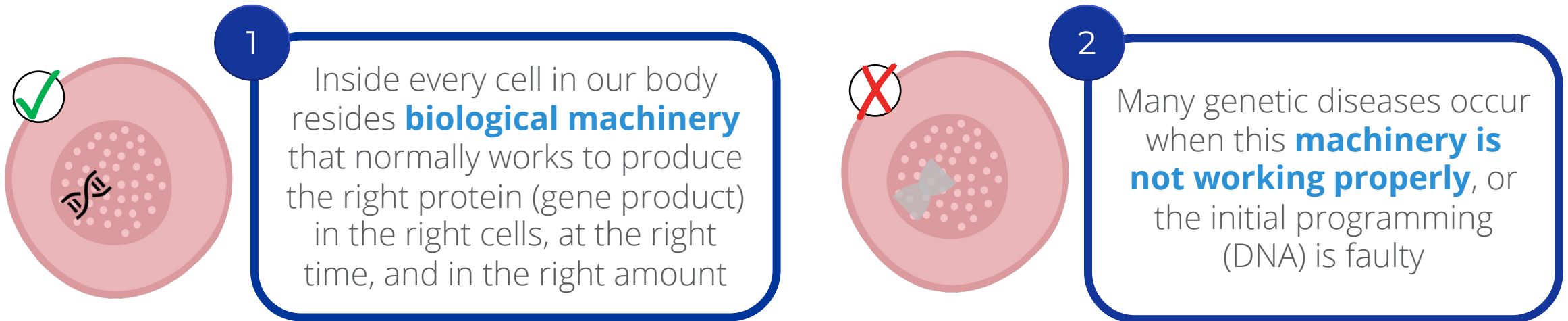


Globally, genetic disorders remain a major challenge

Growing need for the development of functional cures

Global concern: >400 million patients are suffering from >7,000 genetic disorders worldwide

- The cause of these diseases is linked to abnormal genes (the instruction material in our cells)

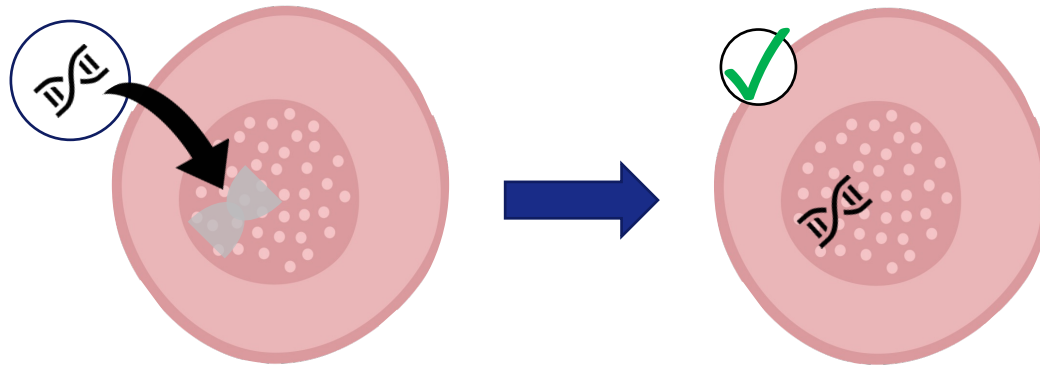


- Less than 5% of these diseases have an approved treatment
- The available treatments often target the symptoms rather than the cause

Genetic medicines: acting at the root of the cause

Potential to achieve functional cures

- Treatments that target these genetic defects are now emerging and fast gaining attention within the biotech and pharmaceutical industry and with regulatory bodies due to:
 - ✓ Promise to provide a functional cure by treating the 'cause' and not the symptoms
 - ✓ Potential to treat greater number of patients
 - ✓ Further improve the quality of life for patients



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Genetic medicines can now alter how this biological machinery works inside the patient's cells and to **act at the root cause**

Example: Cystic fibrosis is a genetic disease with large unmet need

An ideal target where genetic medicines can make a transformation impact

Disease Burden

- Most common fatal inherited disease in U.S.
- Mucus accumulation in the lungs due to defects (mutations) in the CFTR gene, which leads to the production of a defective protein
- ~40,000 patients in U.S. 105,000+ worldwide
- ~900 new cases diagnosed annually
- Severely compromised quality of life
- 34.1 yrs median age at death in U.S. in 2020

Significant Unmet Need

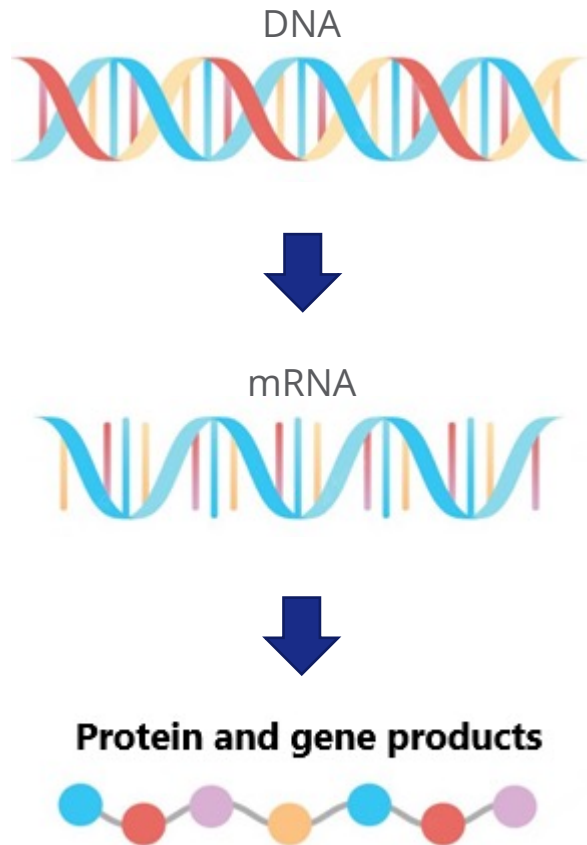
- Current treatments modify the disease and target the defective proteins of only a subset of patients
- Genetic medicines that allow production of correct proteins are needed to treat a broader patient population



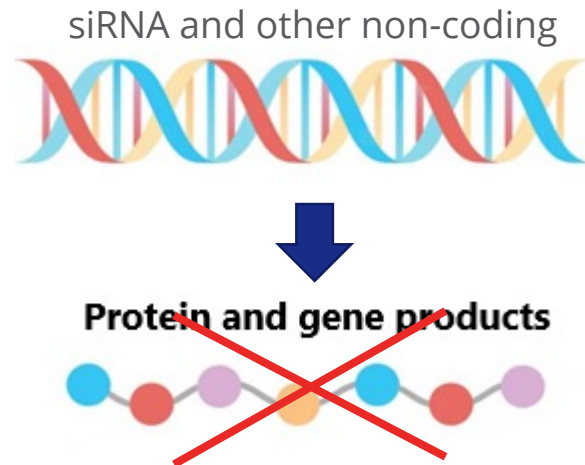
How do these genetic technologies work

A mechanism of action, with functional cure potential

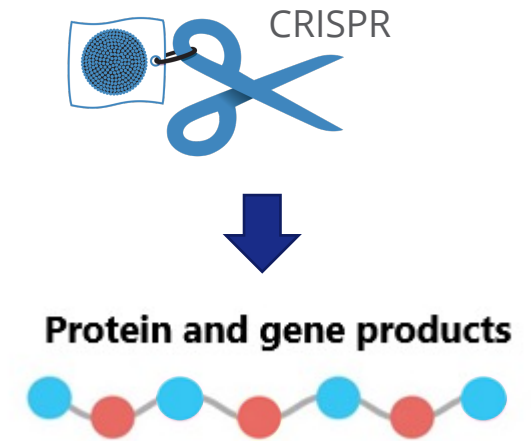
Expressing genes



Silencing genes



Editing genes

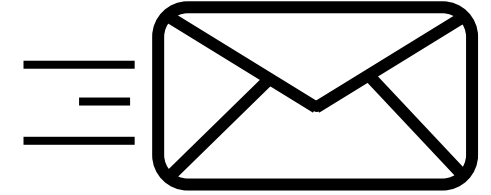


Genetic medicines can **target any gene in different ways**, which may fix many medical problems rather than just treating the symptoms

What challenges or barriers must be overcome

Enabling delivery to the right destination

DNA and RNA **cannot** be administered 'naked' into the body – they need to be packaged and delivered to the right disease destination – like a letter needs an envelope.

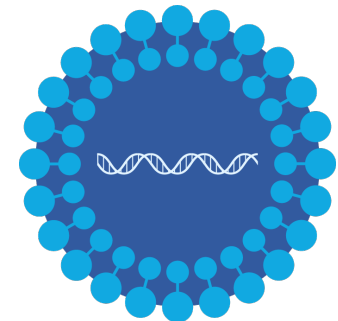


An ideal delivery vehicle:

- ✓ **Protects** the genetic medicine from degradation
- ✓ **Delivers** the medicine to correct destination in the body and cells
- ✓ Is **non-toxic** and causes no serious adverse effects



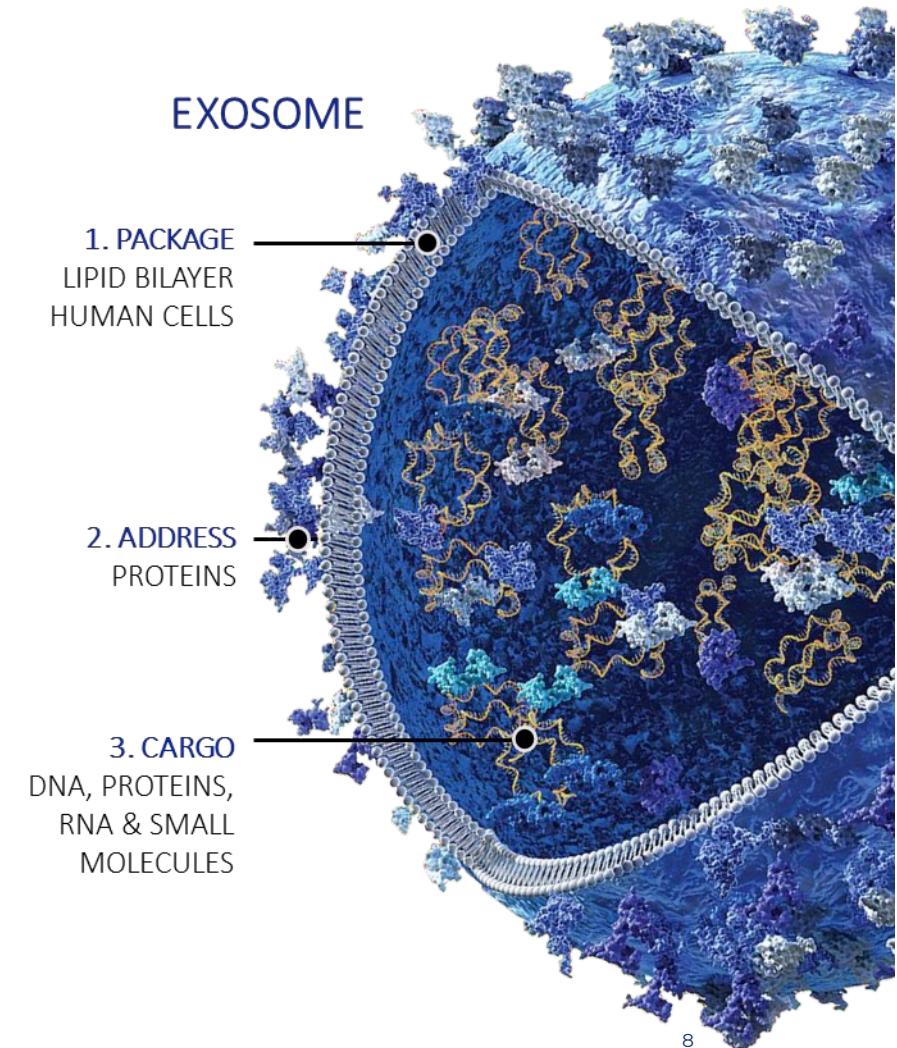
A compelling delivery vehicle, fast gaining attention are **extracellular vesicles (EVs)**, or exosomes



Extracellular vesicles (EVs), or exosome

Nature's drug delivery system

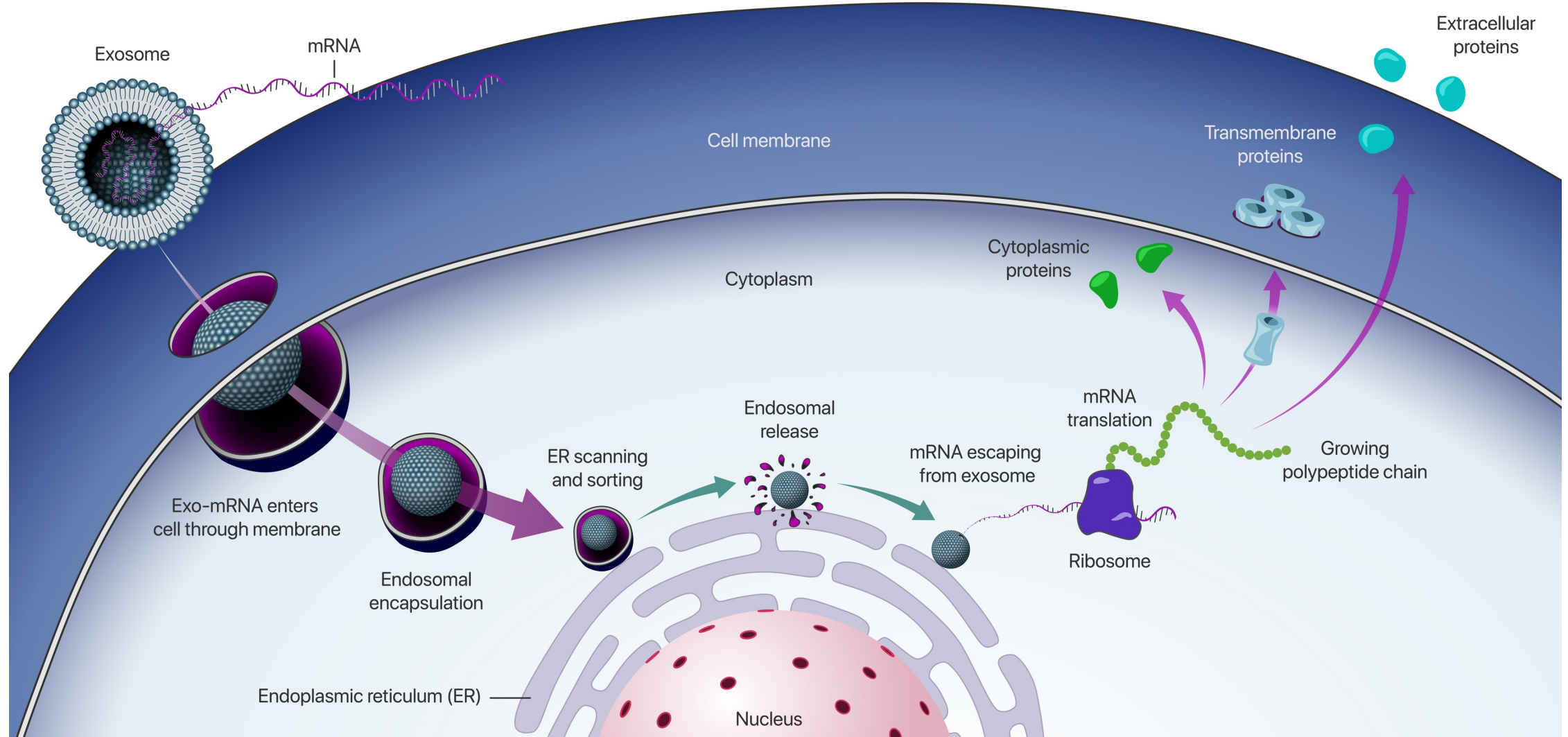
- Nanoparticles released by cells
- Natural mechanism of cell-to-cell signalling and bioactive cargo (drug) delivery
- Non-immunogenic (fully human)
- When harnessed from cells, display broad drug delivery utility (e.g. AAV, CRISPR, DNA, CD24, IL-12, mRNA)
- Can be engineered for selected cell delivery (tissue tropism)
- Compatible with normal drug supply chain
 - can be lyophilised and transported



EVs = extracellular vesicles; **DNA** = deoxyribonucleic acid; **CRISPR** = clustered regularly interspaced short palindromic repeats; **AAV** = adeno associated virus; **mRNA** = messenger ribonucleic acid

How does the exosome transport its drug cargo

Mechanism of delivery illustrated



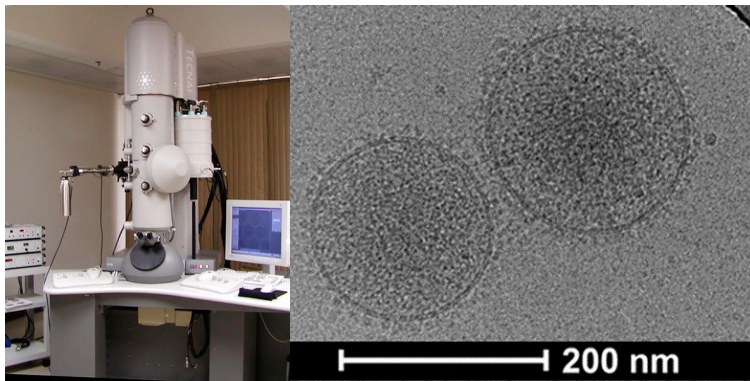
mRNA = messenger ribonucleic acid (RNA)

Exopharm (ASX:EX1)

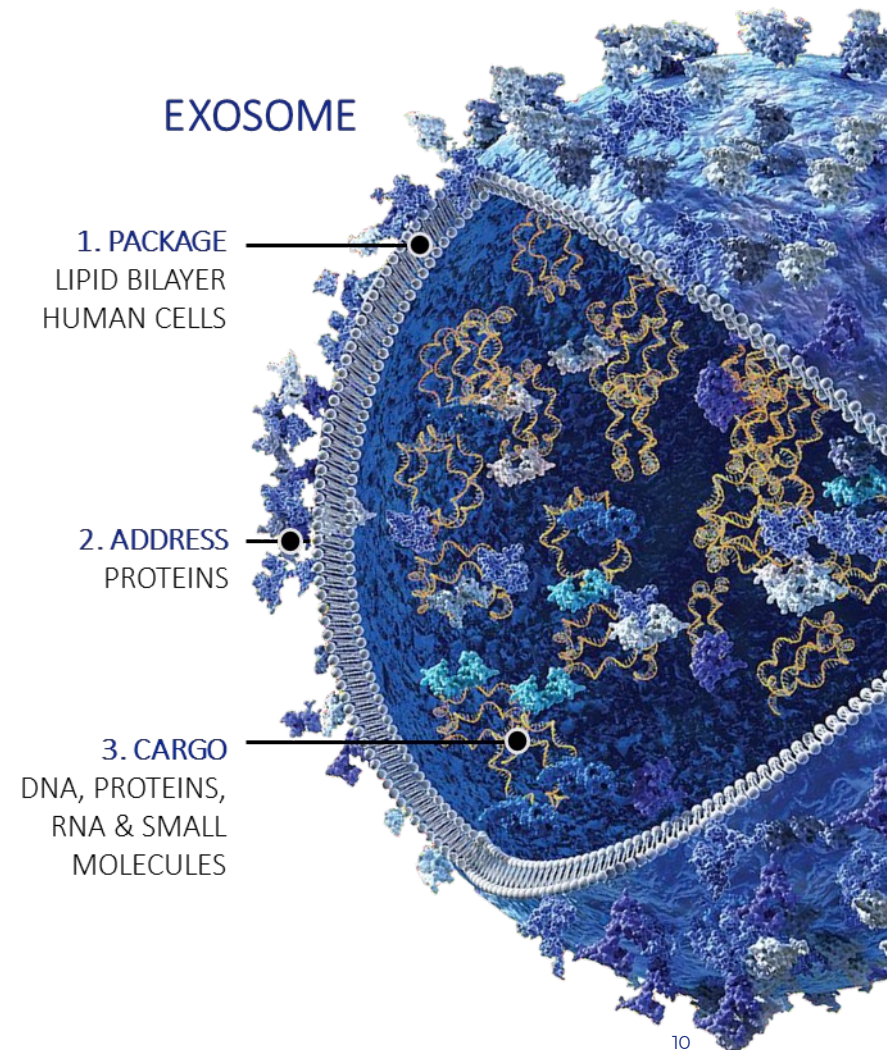
Enabling immune-silent, systemic delivery of genetic medicines in novel ways

Exopharm (ASX:EX1) is a leader in the use of exosomes or extracellular vesicles (EVs) to enable improved drug-delivery in the body.

Exopharm is now developing a new class of transformative medicines that will use EVs, **nature's own drug delivery vehicles**, to distribute drugs inside the body.



EVs as seen under a cryogenic electron microscope



EVs = extracellular vesicles (or exosomes), **GM** = genetic medicines

Exopharm technology platforms

Proprietary innovations that unleash genetic medicine development

Purification technology

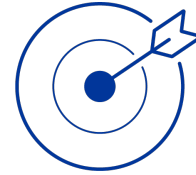


LEAP

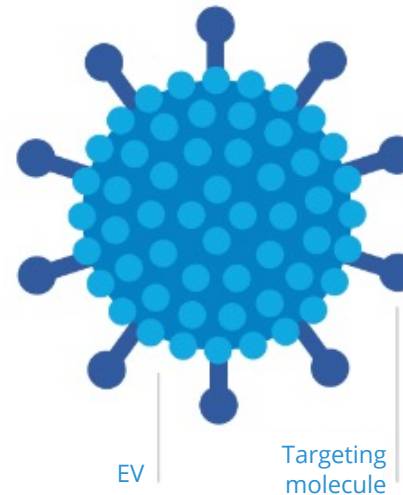


Exopharm's patented LEAP manufacturing technology allows for EVs to be readily purified at commercial scale and low cost

Tissue tropism technology



EVPS

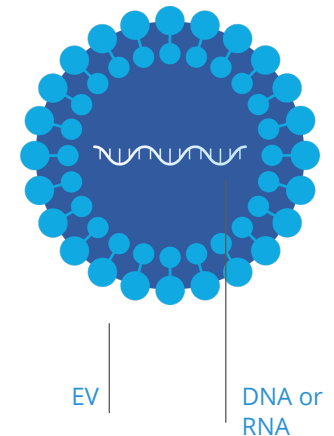


Exopharm's EVPS technology platform targets EVs to specific tissues

API loading technology



LOAD



With Exopharm's LOAD technology, EVs can be loaded with a variety of genetic medicine APIs, including DNA, mRNA, antisense oligonucleotides (ASOs), CRISPR gene editing constructs and more



API = active pharmaceutical ingredient; **DNA** = deoxyribonucleic acid; **mRNA** = messenger ribonucleic acid;
ASO = antisense oligonucleotides; **CRISPR** = clustered regularly interspaced short palindromic repeats



Thank you for your time.

Any questions? Please add your questions in the Q/A chat box.

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