

ASX Announcement

Melbourne, Australia, 20 October 2022

UPCOMING WEBINAR

- Webinar: Genetic Medicines and drug-delivery: Understanding the role for exosomes in this growth sector

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: Genetic Medicines and drug-delivery: Understanding the role for exosomes in this growth sector

Time: 8:00 (Melbourne, Victoria, Australia), 21 October 2022

Registration: <https://exo.ph/Genetic-Medicines-and-drug-delivery>

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.

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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:

Genetic Medicines and drug-delivery:

Understanding the role for exosomes in this growth sector

Exo-webinar series : part 2

Thu 20 October 2022 – 17:00 (Boston) / 14:00 (San Francisco)

Fri 21 October 2022 – 08:00 (Melbourne)

Registration: <https://exo.ph/Genetic-Medicines-and-drug-delivery>



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Recap of Webinar One: Genetic disorders & Genetic Medicines ('GMs')

GMs have big potential, but they are held back by the delivery problem

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

GMs may solve the underlying problem patients suffer from (e.g. treating Cystic Fibrosis with additive CFTR) GMs are potentially functional cures

GM includes:

- RNA and DNA Vaccine products



- RNA and DNA Therapeutic products



GM product categories have vastly different requirements:

- **Vaccines** must stimulate an immune response, can only be given a limited number of times, and must meet low-cost, large-volume demand (e.g. <\$10 per dose and billions)
- **Therapeutic products** are much more sophisticated, must avoid an immune response, require multiple or repeat dose flexibility, target a much smaller patient population that influences cost (e.g. could be \$10,000 per dose and treating 200,000 patients worldwide)

Recap of Webinar One: Therapeutic ‘GMs’ Require Special Delivery

Therapeutic GM products require immune-protected delivery to the target

Therapeutic DNA and RNA **cannot** be administered ‘naked’ in the body – they require encapsulation and delivery - like a letter needs an envelope.



An ideal drug-delivery vehicle for therapeutic GMs:

- protects the genetic medicine ‘cargo’ from degradation
- delivers the functional DNA or mRNA efficiently
- is non-toxic and causes no serious adverse effects
- is non-immunogenic (i.e. does not stimulate an adverse immune response or immune-memory)
- is as natural as possible

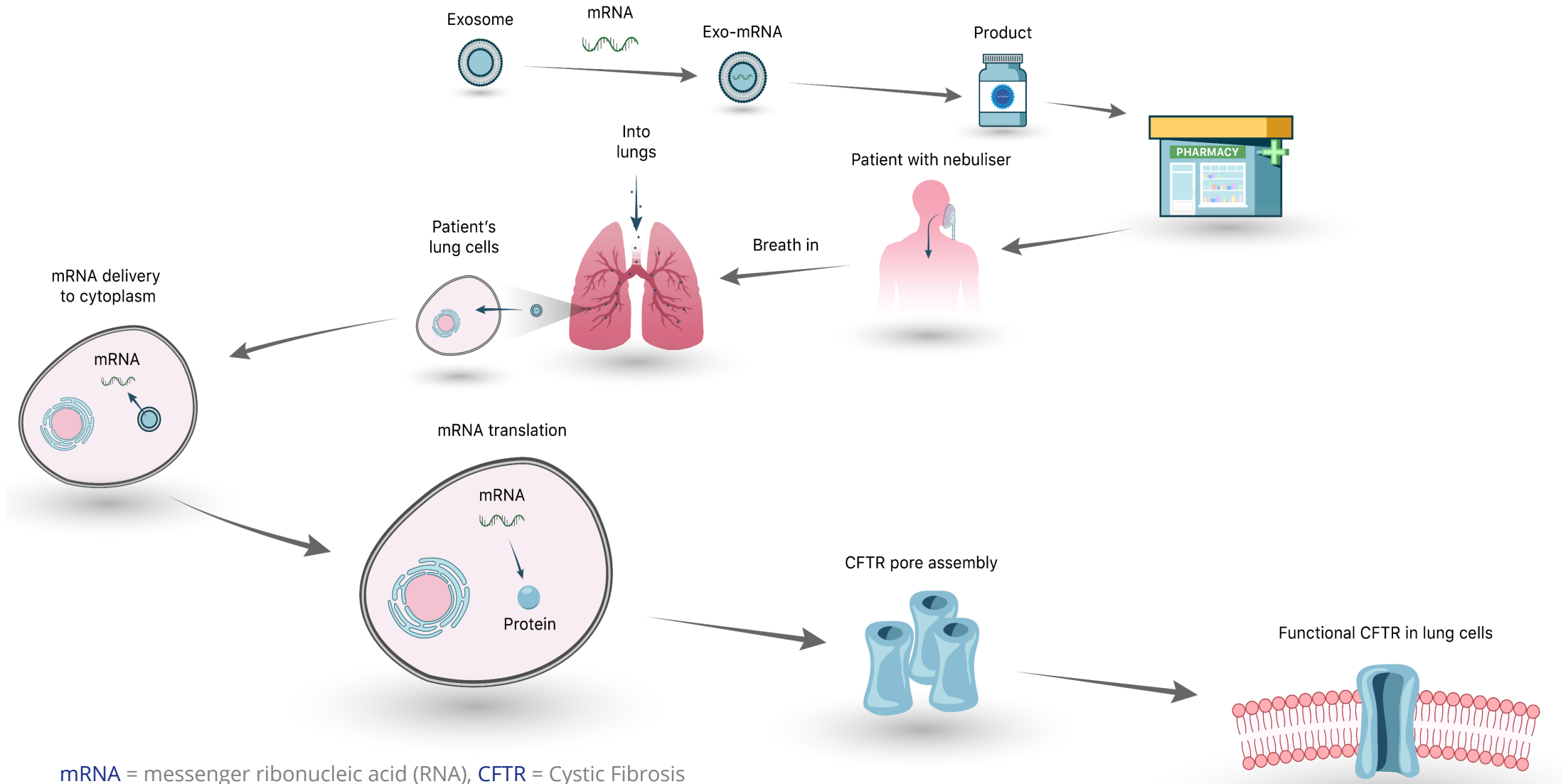
Leading GM delivery technologies are:

- Lipid nanoparticles (LNPs) – delivering mRNA Vaccines
- Exosomes (also known as extracellular vesicles, “EVs”) – can also deliver mRNA and DNA



Recap of Webinar One: How a Therapeutic GM Product Works

Example: GM delivery solution for mRNA to treat Cystic Fibrosis



mRNA = messenger ribonucleic acid (RNA), CFTR = Cystic Fibrosis Transmembrane conductance Regulator

Exosomes and LNPs

Where LNPs are vaccine-suited, Exosomes are ideal for GM therapeutic products

There are three main themes for us to discuss today:

1. GM ***therapeutic products*** have sophisticated drug delivery needs over GM ***vaccines***;
2. GM ***therapeutic products*** can accept higher-price / higher efficiency drug-delivery; and
3. LNPs and Exosomes are processed differently inside a cell – and why that matters for GM ***therapeutic products***



GM products:

GM therapeutic products have sophisticated drug delivery needs over GM vaccines

LNP as GM drug delivery chassis

Advantages

- **Trigger** immune-response
- Low-cost
- Manufacture in a commercial scale
- Well established in vaccine delivery

Disadvantages

- **Trigger** immune-response
- Poor delivery efficiency overall
- Repeat dose limitation
- Not natural, synthetic

LNPs are suited to deliver
vaccine products



GM products:

Therapeutic GM products can accept higher-price / higher efficiency drug-delivery

Exosome as GM drug delivery chassis

Advantages

- Immune-privileged
- Ability to access all body compartments
- Unique target cell entry via membrane fusion
- Multi-dose optionality
- High delivery efficiency overall
- Manufacture at commercial-scale
- Naturally occurring

Disadvantages

- Requires proprietary manufacturing
- Need late-stage clinical study validation

GM therapeutic products require functionality ideally suited to exosomes, even if exosomes were more expensive than LNPs



Therapeutic GMs have special drug delivery requirements

Exosomes emerging as premier delivery chassis for GM therapeutic products



Safety profile:

Given their natural origin, exosomes are considered immune silent, avoiding immune inflammatory triggers. They are also seen as having low toxicity, so doses can be increased



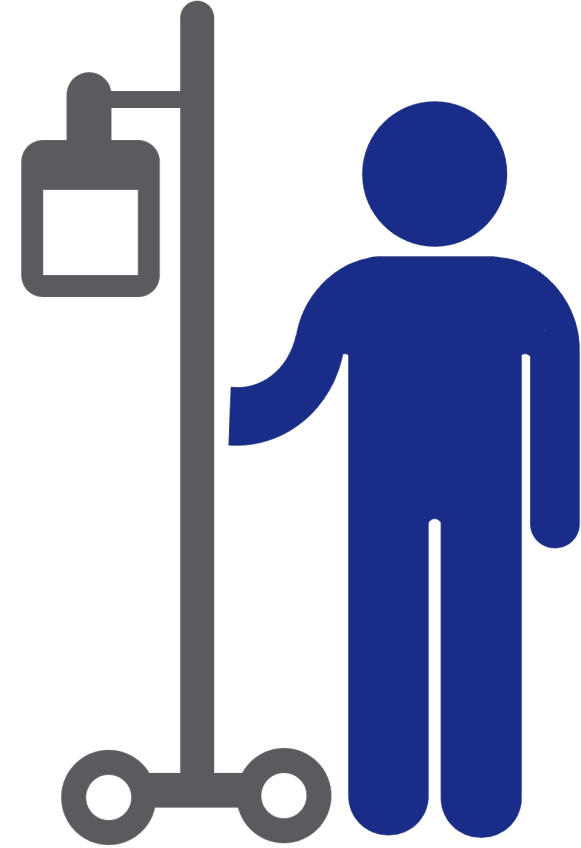
Re-dosing and frequent dosing flexibility:

Exosomes offer the potential of improved efficacy and repeat/multiple dosing



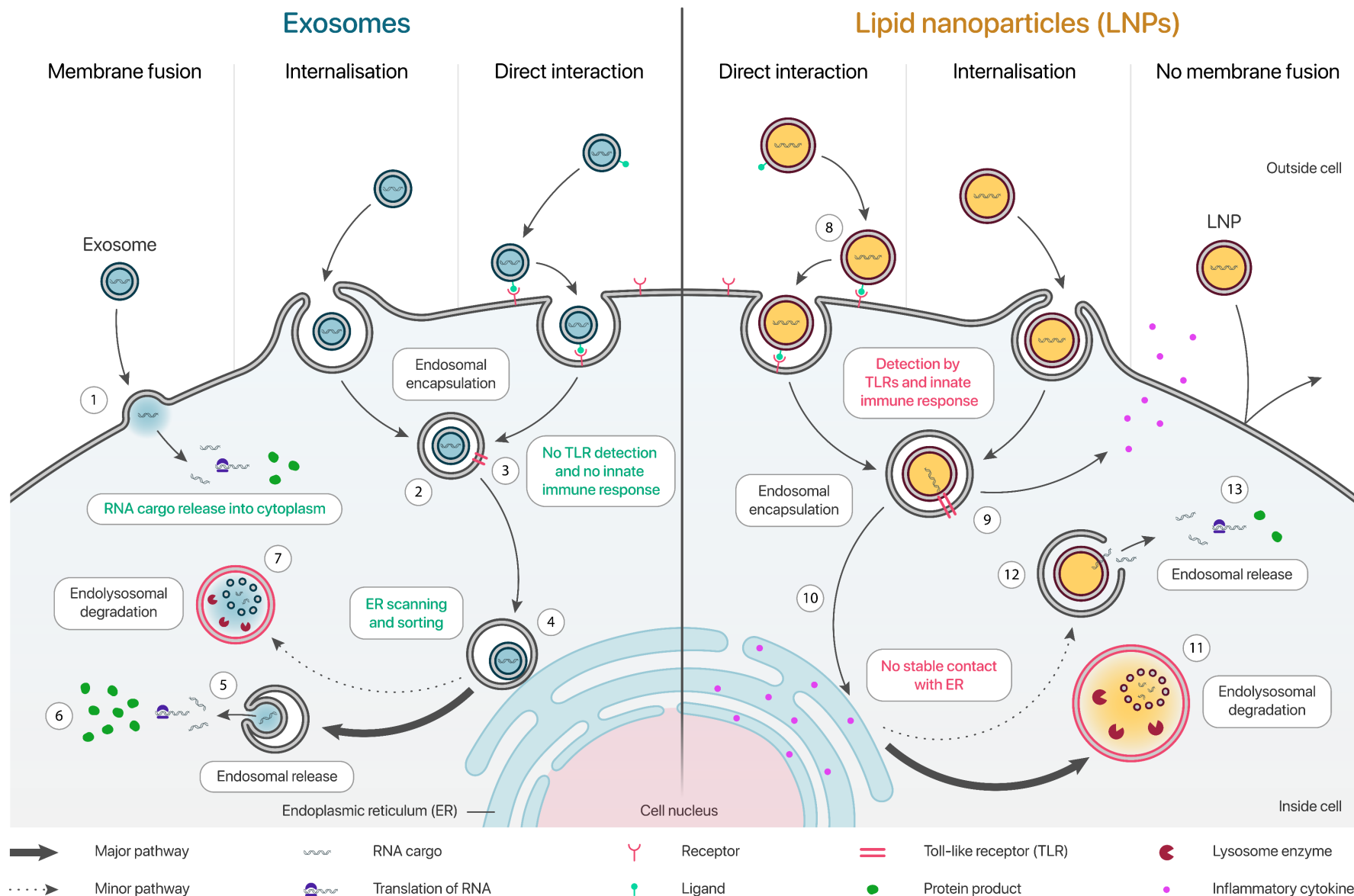
Potential to cross biological barriers:

Exosomes can cross various biological barriers present in the human body – so can deliver the GM cargo to important sites



Exosomes and LNPs as GM Delivery Chassis Options:

LNPs and Exosomes are processed differently inside a cell



Exosomes and LNPs as GM Delivery Chassis Options:

LNPs and Exosomes are processed differently inside a cell

- **Exosomes can fuse with the cell membrane**, releasing GM cargo into cytoplasm for **instant effect**.
- Exosomes are natural and, therefore, efficiently processed inside the cell, leading to **higher delivery efficiency than LNPs**.
- **LNPs** and their synthetic components exhibit **immunogenicity** and **toxicity** within the cell, triggering immune activation **jeopardizing GM cargo**

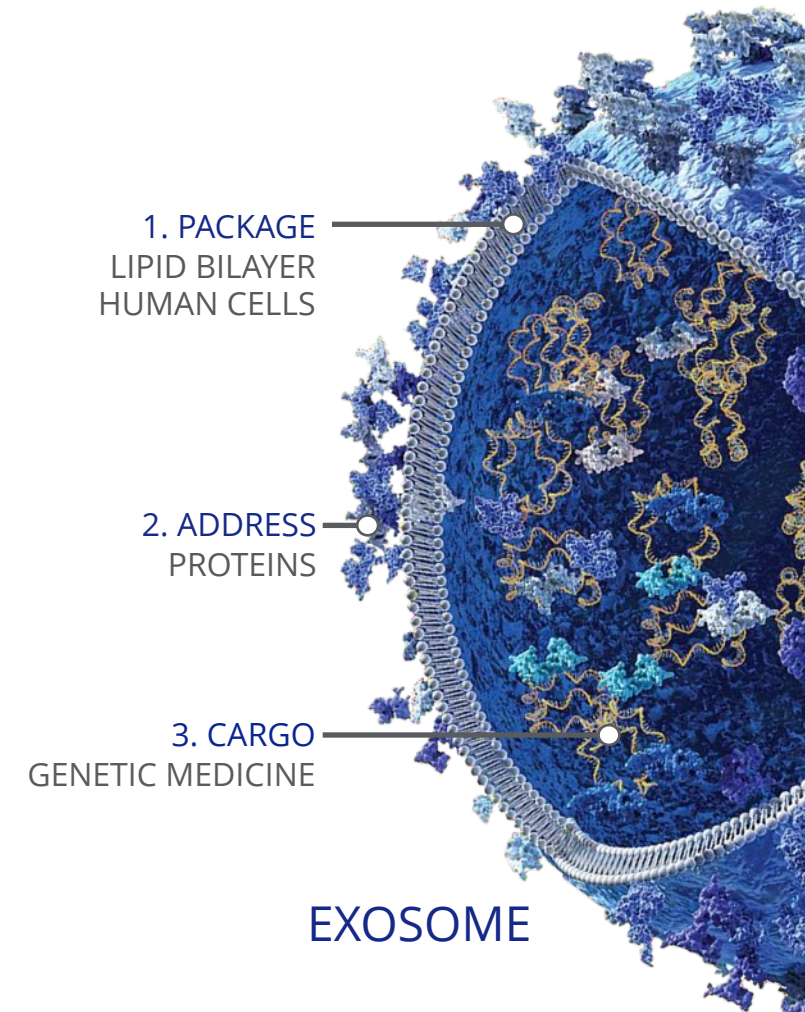


Exosomes Have a Place in GMs

Exosomes are emerging as an ideal GM therapeutic product delivery chassis

Takeaway messages:

1. **Therapeutic GM products** require specialised delivery over GM vaccines
2. **LNPs** are well-suited to deliver GM vaccines that require an immune-response
3. **Therapeutic GM products** require immune-privileged & non-toxic delivery - making exosomes ideal
4. **Exosomes** are efficiently processed inside a cell – optimising the **therapeutic GM delivery efficiency**



Exopharm is making things happen in medicine

Genetic Medicines
(GMs) that are
important

Exosome drug
delivery to make
new GMs work

based on Exosomes: Nature's drug delivery chassis enabled
by Exopharm's manufacturing technologies





Thank you for your participation. Join us again next week at this same time.

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

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