



# Shareholder Update

Monsoon "Twilights"  
22 July 2020

Chris Baldwin



# IMPORTANT INFORMATION



For personal use only

**Purpose of presentation:** This presentation (including this document, any related video or oral presentation, any question and answer session and any written or oral material discussed or distributed in relation to this presentation) has been prepared by Exopharm Limited (ACN 163 765 991) (**Exopharm or Company**). This presentation is intended for sophisticated or professional investors (as those terms are defined in the *Corporations Act 2001* (Cth)), and their professional investment advisors and has been prepared for the sole purpose of providing general high-level information on Exopharm and its operations.

**Not an offer or solicitation:** This presentation is not investment advice nor an offer to subscribe for securities or otherwise invest in Exopharm, and it should not be relied upon to make any investment decision.

**Nature of presentation:** This presentation is not a prospectus, product disclosure statement or other investment disclosure document, and the level of disclosure in this presentation is less than such disclosure documents. This presentation does not purport to contain all of the information that a prospective investor may require to make an evaluation of Exopharm or its business activities and nothing in this presentation is, or is intended to be, a recommendation to invest in Exopharm. Exopharm does not purport to give financial or investment advice. No account has been taken of the objectives, financial situation or needs of any recipient of this presentation.

**Forward-looking statements:** This presentation may contain forward-looking statements which may be predictive in nature and incorporate an element of uncertainty or risk, such as 'intends', 'may', 'could', 'believes', 'estimates', 'targets' or 'expects'. These statements are based on an evaluation of current economic and operating conditions, as well as assumptions regarding future events. These events are, as at the date of this presentation, expected to take place, but there cannot be any guarantee that such will occur as anticipated, or at all, given that many of the events are outside Exopharm's control. The stated events may differ materially from results ultimately achieved. Accordingly, neither Exopharm nor any of its directors, employees, contractors or advisors make any warranty or assurance that the results, performance or achievements expressed or implied by the forward-looking statements contained in this presentation will actually occur. Further, other than as required by law, Exopharm may not update or revise any forward-looking statement if events subsequently occur or information subsequently becomes available that affects the original forward-looking statement.

**Disclaimer:** Neither Exopharm nor its officers, employees, contractors or advisors give any warranty or make any representation (express or implied) as to the accuracy, reliability, relevance or completeness of the material contained in this presentation. Nothing contained in this presentation is, or may be relied upon as a promise, representation or warranty, whether as to the past or the future. Except for statutory liability which cannot be excluded, Exopharm, its officers, employees, contractors and advisors expressly disclaim any responsibility for the accuracy or completeness of the material contained in this presentation and exclude all liability whatsoever (including in negligence) for any loss or damage which may be suffered by any person as a consequence of any information in this presentation or any error or omission therefrom.

**Professional advice:** Recipients of this presentation should consider seeking appropriate professional financial, taxation and legal advice in reviewing the presentation and all other information with respect to Exopharm and evaluating its business, financial performance and operations.

**Confidentiality and copyright:** While this is a non-confidential presentation, it still provides confidential and commercially sensitive information which is provided for the intended recipient only. Organisations or Persons viewing this presentation must not disclose the presentation or its contents to any third parties other than external consultants of the recipient for the purposes of obtaining a professional review, financial, taxation or legal advice, or as required by law or court order. Exopharm holds the copyright in this paper. Except as permitted under the *Copyright Act 1968* (Cth), this paper or any part thereof may not be reproduced without Exopharm's written permission.

# Exopharm Ltd – Summary



## Overview

- Exopharm is an Australian clinical stage biotechnology company with a rapidly-developing technology platform for turning exosomes into medicines
- 26 staff (currently) based in Melbourne, Victoria
- Publicly-traded on the ASX (ASX:EX1) (listed Dec 2018)

## Financials (as of 01/01/2020)

- 95.47 million shares outstanding with a market capitalisation of ~ \$30 million
- Cash balance of \$5.88 million (per Mar 4C)
- Quarterly burn rate (per Dec 4C) of \$1.85 million

## Priorities

- Leverage our unique access to exosomes to partner with established biopharmaceutical and cell therapy companies
- Advance novel products through early phase clinical development
- Accelerate the development of our exosome generation, manufacturing and characterization technologies
- Add institutional investors/strategic partners to the share register

# Three Development Areas



## Naïve EVs (NEVs)

- EVs from stem cells and blood donations
- Well-established safety profile from millions of transfusions and adult stem cells
- A replacement for stem cell therapies
- Potential to treat age-related degeneration



## Engineered EVs (EEVs)

- EVs that deliver drugs, proteins and/or nucleic acids into specific cells
- Nearly unlimited potential to treat untreatable diseases
- Clear pathway to partnership deals



## Production, purification, formulation, engineering

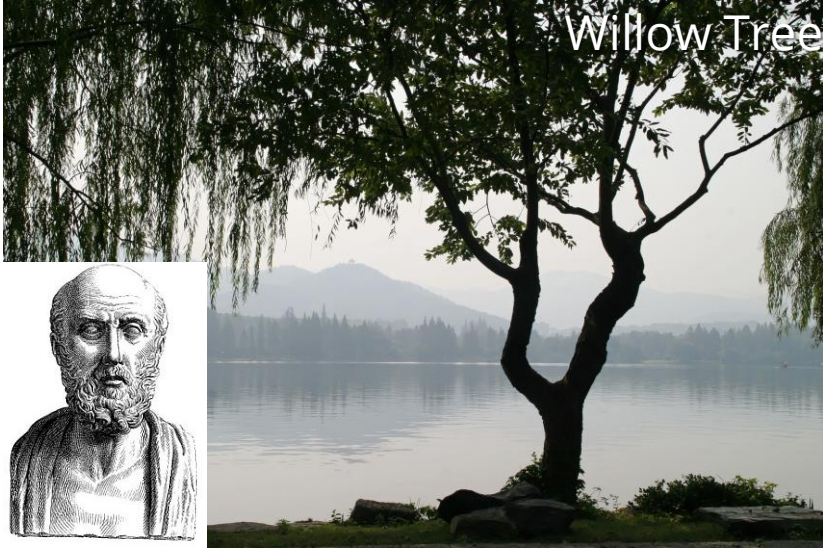
- LEAP™ technology provides unique, high volume process for proprietary EVs
- Access to abundant EVs creates a virtuous circle of innovation within manufacturing and EV drug development





# From Bark to Medicine

400 BC



Willow Tree

Hippocrates



Natural source of salicin

1899



Manufacture of  
acetylsalicylic acid (aspirin)

**Same active ingredient,  
different commercial  
outcome**

- Once the active ingredient in willow bark was identified, Bayer discovered how to manufacture it
- Bayer's aspirin fuelled its growth into one of the world's greatest pharma companies

# From Cells to Medicine



## 2000 AD

Mesenchymal stem cells regenerate tissue, so grow them and transplant them into patients



"Off-the-shelf" stem cells

## 2020's

MSC EVs are the active ingredient, with the potential to deliver a truly off-the-shelf treatment

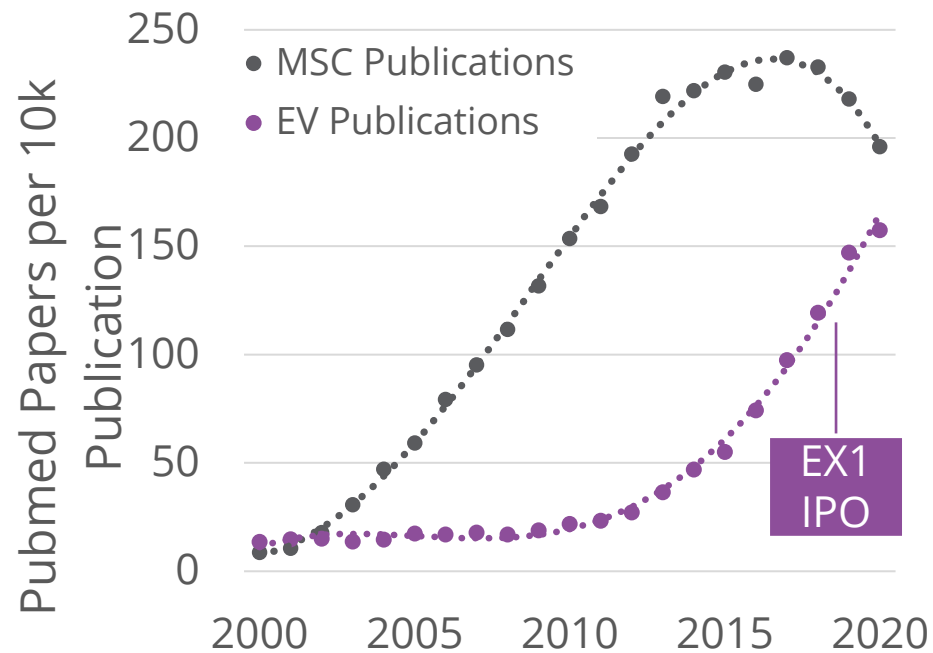


Potential EV Medicine

**Same active ingredient,  
different commercial  
outcome**

- Exopharm solved core manufacturing problem with LEAP
- Let adult stem cell clinical companies identify uses and resolve regulatory pathways
- Follow quickly with safer, more economical offering

# Naïve EV's are the **Future of Regeneration**, Exopharm is the World Leader in NEVs



## Naïve EVs (NEVs)

- Stem cell benefits, without the risks and costs
- Targeted to mobility and sensory indications (sight, osteoarthritis hearing, neural degeneration)

## Exosome redux

news feature

Adult stem cell companies are pivoting their businesses to commercialize exosomes as therapeutics.

NATURE BIOTECHNOLOGY | VOL 37 | DECEMBER 2019 | 1395-1400 | [www.nature.com/naturebiotechnology](http://www.nature.com/naturebiotechnology)

### December 2019

- 14** Exosome companies reviewed globally
- 5** Companies planning Clinical Trials with exosome medicines
- 1**  **exopharm** FIRST and ONLY reported human dosing (Jan 2020)

## EX1 Basic Strategy

- When adult stem cell companies score wins, follow with more economical offering (e.g. ARDS, GVHD, CLI, Cardiac)
- Partner with Pharma with a more 'drug-like' product

Potential markets >>\$10 billion/yr

## EX1 Milestones

### Naïve EVs (NEVs)



Autologous Phase I



**Allogeneic Phase I**



Allogeneic Phase I



= Achieved in CY 2020



= Targeted for CY 2020



# Engineered EVs: Key Exopharm IP Assets\*



For personal use only

## LEAP

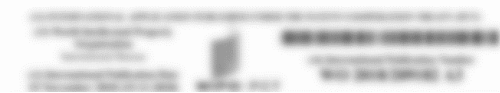
*Purification of EVs at Scale*



- Developed internally – **exclusive** ownership and use
- Comprehensive application to EVs
- Family of resins and potential resins await development

## LOAD

*Addition of nucleic acids to EV Payloads*



- Global, **exclusive** in-licensing agreement (2020)
- Comprehensive application to EVs
- Custom medicines loaded into EEVs

## EVPS

*Addition of proteins to EV Surfaces*

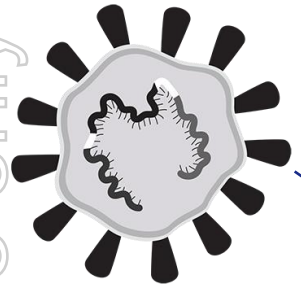


- Global, **exclusive** in-licensing agreement (2020)
- Comprehensive application to EVs
- Custom addressing of EEVs to selected cells

# Engineering Platform Example: CoV Specific Anti-viral “Fortrexo CoV”



For personal use only

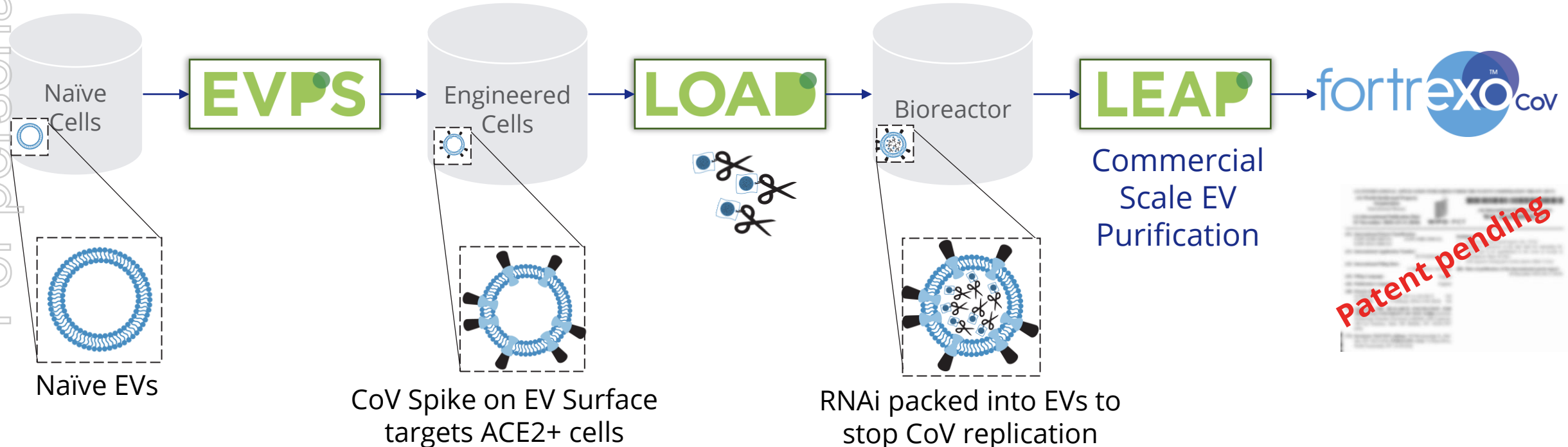


The SARS-CoV-2 virus has spike proteins on the outside and a nucleic acid payload on the inside. The spike protein targets cells with ACE2 receptor on the outside and delivers the particle into the ACE2+ cell. The payload enables viral particle replication inside an infected cell



EV with spike protein attached using EVPS

Exopharm has designed an EEV to reduce viral replication in ACE2+ cells by delivering RNAi that inhibit viral particle replication in an infected cell



# Engineered EVs: the **Future of Precision Medicine**



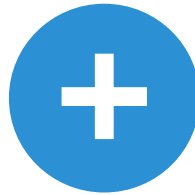
For personal use only

## Natural characteristics of Naïve EVs

EVs cross barriers (including blood-brain)

EVs naturally reprogram cells

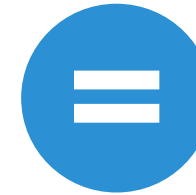
EVs control immune responses



## Engineered EVs

Custom medicines 'Drug' cargoes of many types e.g. small molecules, RNAi, siRNA, mRNA

Custom addressing - targeting EEVs to selected cell types e.g. brain cells, cancer cells



## Potential markets for 'untreatable' diseases

>>\$100 billion/year

Cancers

Viruses

Rare diseases

Age-related illnesses

### EEV Opportunities

- Multiple companies to do deals with
- Multiple EEV products and variations
- Multiple applications

# Engineered EV Deals



Only three companies globally have announced proprietary platforms for EEV Development:

EV Company	IP Status	Pharma Partner	Date	Area	Upfront	Milestones
	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>	Jun 2020	CNS	> \$50 mln	\$1.7 billion
		<input checked="" type="checkbox"/>	Mar 2020	Rare diseases	\$63 mln	\$1.3 billion
	<input checked="" type="checkbox"/>	<input checked="" type="checkbox"/>	Jan 2019	Cancer	\$77 mln	\$1.8 billion
		<input checked="" type="checkbox"/>	Jun 2020	CNS	> \$100 mln	Not disclosed
	<input checked="" type="checkbox"/>	<input type="checkbox"/>	Target: 2021			

As of Jun 2020



# Engineered EVs Conclusion



For personal use only

## Basic Strategy

- Demonstrate power of EVPS and LOAD with Fortrexo CoV
- Partner with multiple pharma companies with EEV products
- Use proceeds to fund NEV clinical trials

**Potential markets >>\$100 billion**

## Milestones

### Engineered EVs (EEVs)

- ☒ Assembly of IP
- ☒ **Demonstrate Proof of Concept**
- ☐ First Partnering Deal



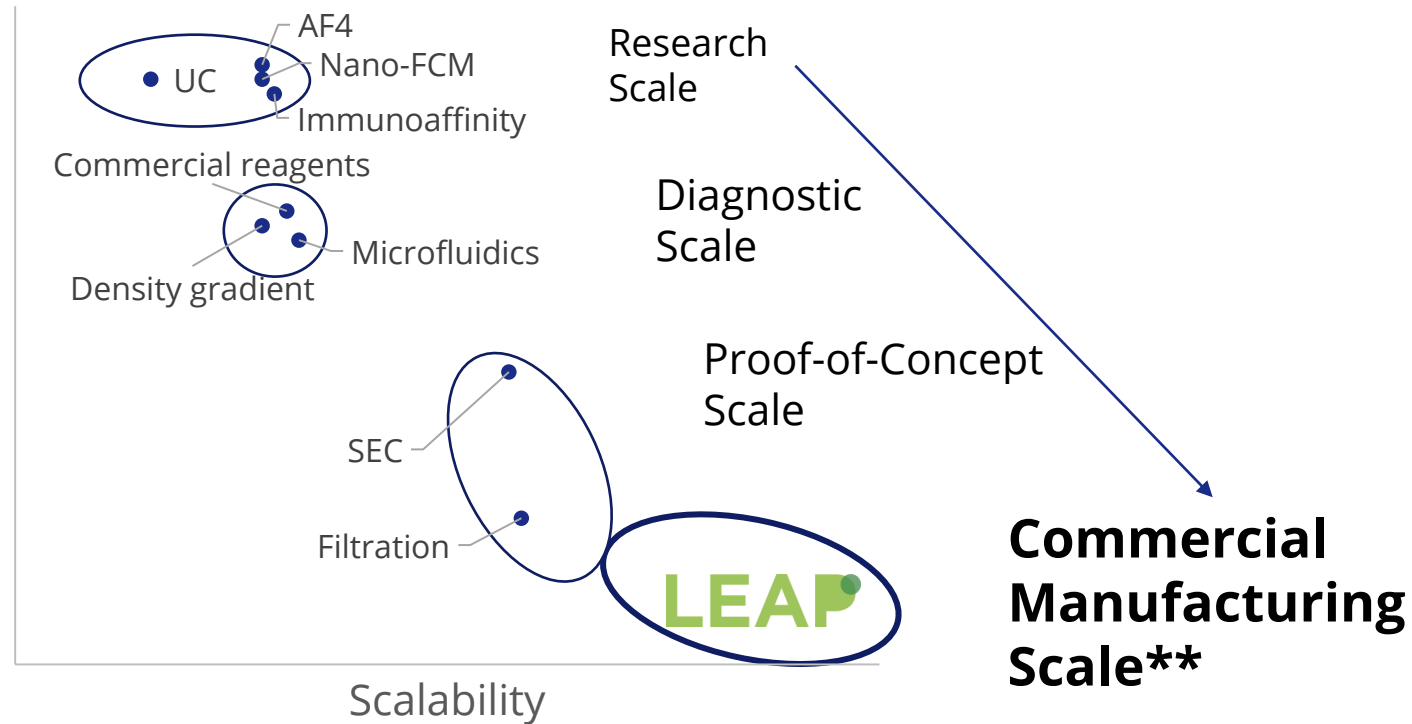
☒ = Achieved in CY 2020

☒ = Targeted for CY 2020

# LEAP vs Other EV Purification Techniques



State of the Art, EV Purification as of June 2020\*



**Unlike all other alternatives, LEAP technology**

- (i) is readily scaled up to over 1,000L scale,
- (ii) uses industry-standard equipment and processes
- (iii) uses low-cost, reusable consumables (from validated sterilization process)

\* Adapted from <https://doi.org/10.1016/j.tibtech.2020.05.012>  
Technologies and Standardization in Research on Extracellular Vesicles

Srujan Gandham,<sup>1,4</sup> Xianyi Su,<sup>2,4</sup> Jacqueline Wood,<sup>2,4</sup> Angela L. Nocera,<sup>1</sup> Sarath Chandra Alli,<sup>2,3</sup> Lara Milane,<sup>1</sup> Alan Zimmerman,<sup>2</sup> Mansoor Amiji,<sup>1</sup> and Alexander R. Ivanov,<sup>2,\*</sup>



\*\* LEAP assessment from Exopharm, based on industrial use to date; LEAP Patents processing through National phases at present

# Milestones Toward Revenue





For personal use only  
Therapeutic Areas  
Technology

## Naïve EVs (NEVs)

- ☒ Autologous Phase I
- ☒ **Allogeneic Phase I** 
- ☐ Allogeneic Phase I 

## Engineered EVs (EEVs)

- ☒ Assembly of IP
- ☒ **Demonstrate Proof of Concept**
- ☐ First Partnering Deal 

- ☒ Demonstration via Clinical Trial
- ☒ **Expansion of EVs Know-how**
- ☐ Bioprocessing Partnering Deal 

- ☒ = Achieved in CY 2020
- ☒ = Targeted for CY 2020

# Our Team



For personal use only





# Exopharm Ltd.

*Refreshing Medicine, Today*

**Dr Chris Baldwin**

Chief Commercial Officer  
chris.baldwin@exopharm.com  
+61 450 290 280

[www.exopharm.com](http://www.exopharm.com)

We believe ...

- EV Medicines will be a major modality across therapeutic areas
- Exopharm has the technology and the team to play a major role in EV Medicine
- Exopharm is on track to achieve commercial success with both Naïve EVs and Engineered EVs