



## Corporate Presentation

Capricor Therapeutics, Inc.

Nasdaq: CAPR

October 2025

# Forward Looking Statements



Statements in this presentation regarding the efficacy, safety, and intended utilization of Capricor's product candidates; the initiation, conduct, size, timing and results of clinical trials; the pace of enrollment of clinical trials; plans regarding regulatory filings, future research and clinical trials; regulatory developments involving products, including future interactions with regulatory authorities and the ability to obtain regulatory approvals or otherwise bring products to market; manufacturing capabilities; dates for regulatory meetings; the potential that required regulatory inspections may be delayed or not be successful which would delay or prevent product approval; the ability to achieve product milestones and to receive milestone payments from commercial partners; and any other statements about Capricor's management team's future expectations, beliefs, goals, plans or prospects constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Any statements that are not statements of historical fact (including statements containing the words "believes," "plans," "could," "anticipates," "expects," "estimates," "should," "target," "will," "would" and similar expressions) should also be considered to be forward-looking statements. There are a number of important factors that could cause actual results or events to differ materially from those indicated by such forward-looking statements. More information about these and other risks that may impact Capricor's business is set forth in Capricor's Annual Report on Form 10-K for the year ended December 31, 2024, as filed with the Securities and Exchange Commission on March 26, 2025, and in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2025, as filed with the Securities and Exchange Commission on August 11, 2025. All forward-looking statements in this press release are based on information available to Capricor as of the date hereof, and Capricor assumes no obligation to update these forward-looking statements.

Capricor has entered into an agreement for the exclusive commercialization and distribution of Deramioce<sup>l</sup> for DMD in the United States and Japan with Nippon Shinyaku Co., Ltd. (U.S. subsidiary: NS Pharma, Inc.), subject to regulatory approval. Deramioce<sup>l</sup> and the StealthX<sup>™</sup> vaccine are investigational candidates and have not been approved for commercial use in any indication.

**At Capricor, we  
stand committed**

to pushing the  
boundaries of  
possibility and forging  
a path toward  
transformative  
treatments for  
patients in need.



# Table of Contents

## Capricor Overview

### Deramiocele DMD Program Overview

### StealthX™ Exosomes Platform Overview

# Capricor's History & Evolution



**2004:** Foundational discovery of **Cardiosphere-derived cells (CDCs)** at Johns Hopkins University



**2012:** Groundbreaking publication in *The Lancet* showing CDCs clinical **benefits**<sup>1</sup>

**2015:** Discovery of **exosomes** as the primary **mechanism** of action (**MOA**) of CDCs



**2018:** Published preclinical **Duchenne muscular dystrophy (DMD)** study in *Stem Cell Reports*

**2022-2023:** Established commercial partnerships with **Nippon Shinyaku** for exclusive distribution in the **U.S. and Japan**

**2024:** Capricor **filed BLA** seeking approval of **Deramiscoel** to treat DMD

**Q4-2025:** **Topline** data from HOPE-3 **Phase 3** clinical trial expected



**2005:** Capricor was founded and **embarked** on a ~**20-year** journey to **elucidate** the **mechanism** of **cell-based** biology for **therapeutic** development

**2014:** Capricor's **uplisting** to **NASDAQ** Capital Market (**CAPR**)

**2019:** Published **positive** results from HOPE-Duchenne **Phase 1 study** in the *Journal of Neurology*<sup>2</sup>

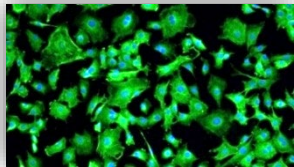
**2022:** Published **positive** results from HOPE-2 **Phase 2 study** in *The Lancet*



**2025:** **StealthX™** exosome **vaccine** Phase 1 study **initiated** in collaboration with **NIAID**

# Groundbreaking Science

First-in-Class Products in Development



## Scientific Foundation Cardiology and Cell Biology

- **Initial Technology:** developed at Johns Hopkins University
- **Core Product:** cellular therapy comprised of cardiosphere-derived cells; endogenous human heart stromal cell population
- **Extensive IP portfolio:** ~150 patents & patent applications

## Lead Program in Rare Disease Duchenne Muscular Dystrophy



- ❖ **Indication:** DMD, rare, x-linked fatal disorder afflicting ~15,000 boys and young men in U.S.
- ❖ **Positive safety and efficacy results** shown in multiple clinical studies (Phase 1, 2 & open-label extension)
- ❖ **In-house GMP manufacturing**
- ❖ **Established commercial partnerships** in U.S. and Japan

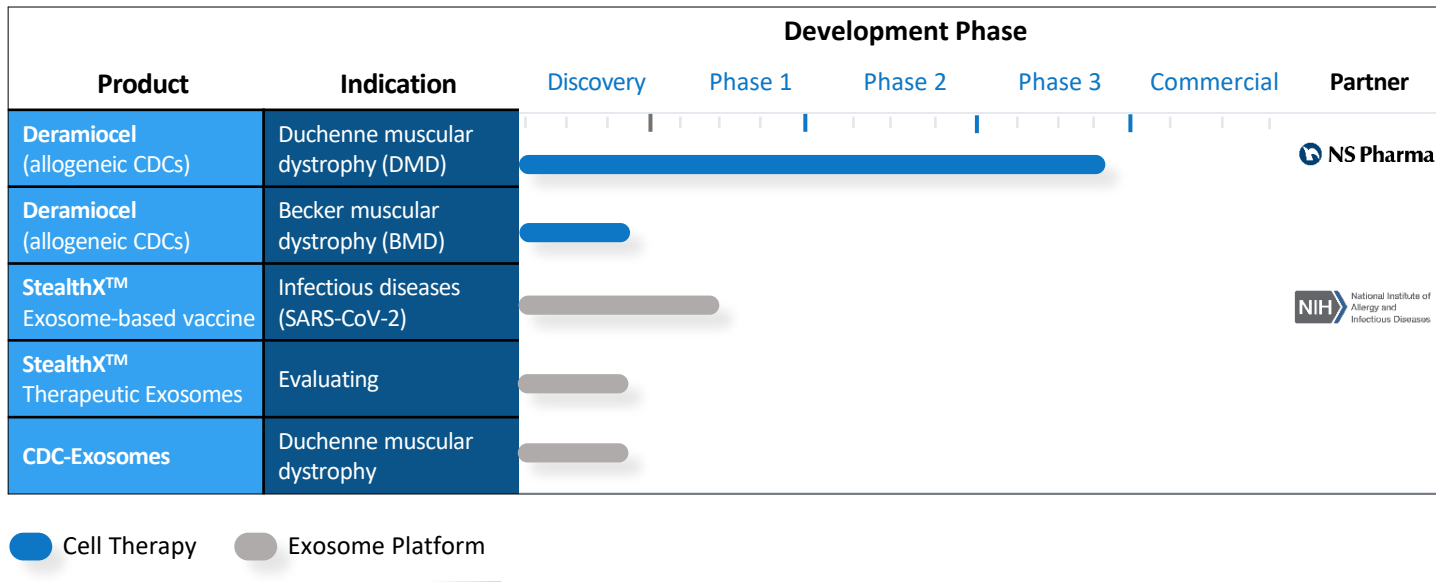


## Pipeline Development StealthX™ Exosome Platform

- **Targeted** natural drug delivery platform
- **Aim to secure partnerships** for platform advancement
- **Phase 1 study underway** in collaboration with NIH with exosome-based vaccine

# Capricor's Product Pipeline

Advancing Transformative Therapies for Rare Diseases & Beyond



# Deramiocele: BLA Status & Next Steps



## BLA Submission

- BLA filed in Dec. 2024; accepted and granted priority review
- Successful mid-cycle meeting and PLI inspection, responded to over 50 information requests
- CRL received in July 2025; cited BLA did not meet statutory requirement for substantial evidence of effectiveness and need for additional clinical data

## FDA Alignment

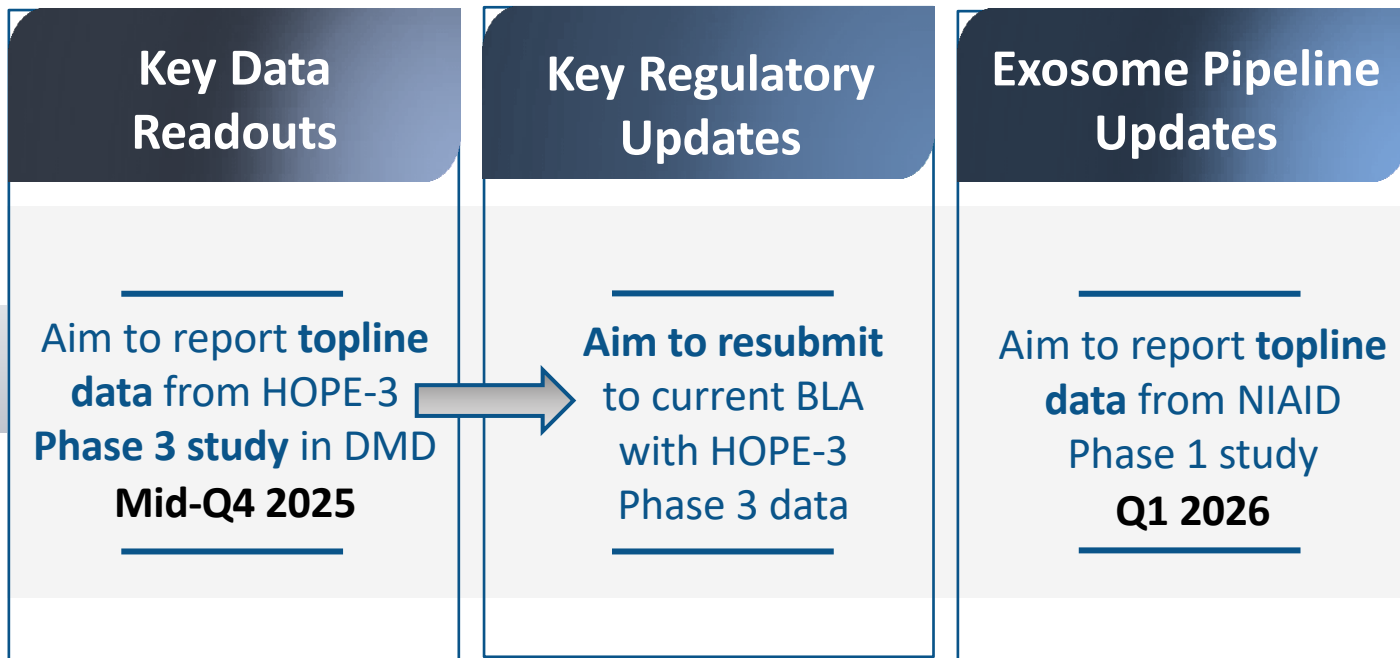
- ✓ Recent Type A meeting; key meeting outcomes were:
- ✓ HOPE-3 Phase 3 trial should serve as additional study requested in CRL and alignment on key endpoints
- ✓ HOPE-3 data can be submitted within the current BLA

## Next Steps

- Announce HOPE-3 topline data
- Plan to submit HOPE-3 results with complete response to the CRL
- Aim to potentially secure label encompassing both cardiac and skeletal muscle function in DMD



# Key Upcoming Value Driving Catalysts



# Capricor Financial Snapshot



## Overview & Potential Cash Infusions

**Cash Balance**

**\$123 million<sup>1</sup>**

As of June 30, 2025

**Current Runway**

**Into ~Q4 2026**

Based on current operating plan<sup>2</sup>

**Outstanding  
Shares**

**45.7 million<sup>1</sup>**

**Milestone Payments  
to Capricor**

(NS Pharma: U.S. Distribution Agreement)

**U.S. approval:** \$80 million

**Sales-based milestones:** up to \$605 million

**Eligible for Priority  
Review Voucher**

**Potential sale of PRV, if received**  
**~\$150 million<sup>3</sup>**

<sup>1</sup>Based on Q2 10Q

<sup>2</sup>This expectation excludes any additional potential milestone payments under the Agreements with Nippon Shinyaku, as well as any strategic use of capital not currently in our base case planning assumptions.

<sup>3</sup>Estimate based on recent public sale of PRV = ~\$158M

# Table of Contents



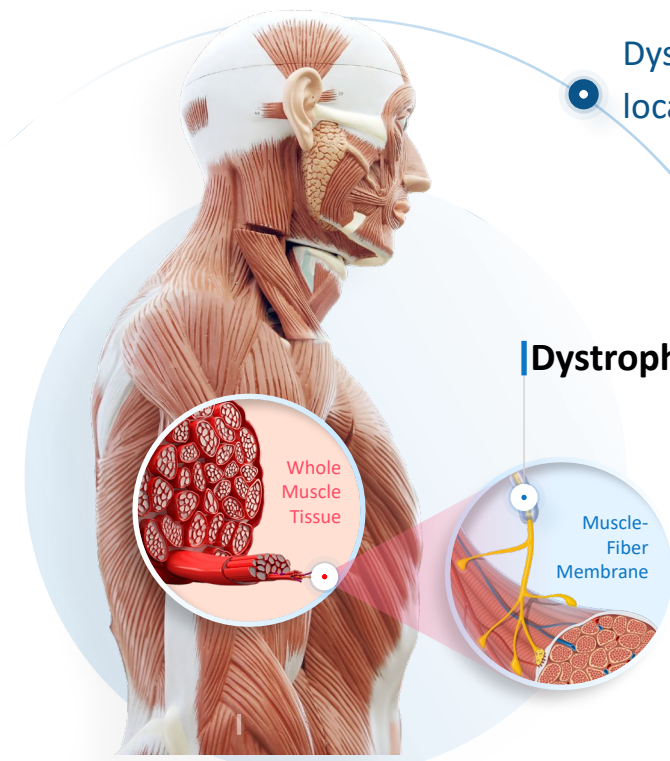
Capricor Overview

Deramiocele DMD Program Overview

StealthX™ Exosomes Platform Overview

# DMD: A Devastating Rare Disease

High Unmet Needs Across the Entire Disease Trajectory



Dystrophin is a structural protein  
located within the muscle fiber membrane

Acts both as a cushion and glue

Without dystrophin, muscles (**cardiac and skeletal**) are unable to function properly, suffer progressive damage and eventually die

Much of the muscle injury that occurs in dystrophin-deficiency is attributable to **secondary damage caused by inflammation**

## Dystrophin

Whole  
Muscle  
Tissue

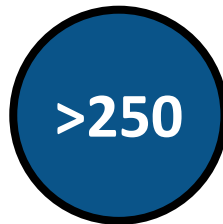
Muscle-  
Fiber  
Membrane

# Deramiocele: Cellular Therapy

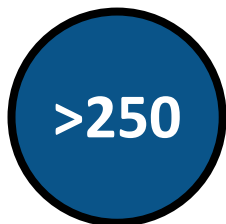
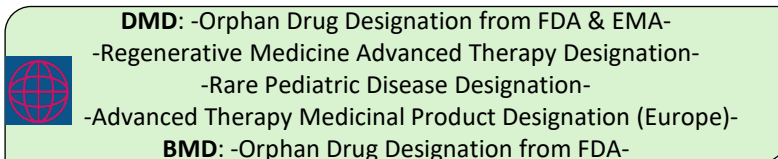
Comprised of Human Allogeneic Cardiosphere-Derived Cells (CDCs)



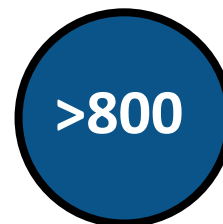
**CDCs are derived from cells of healthy human hearts; they are not stem cells**



**Peer-reviewed scientific publications<sup>1</sup>**

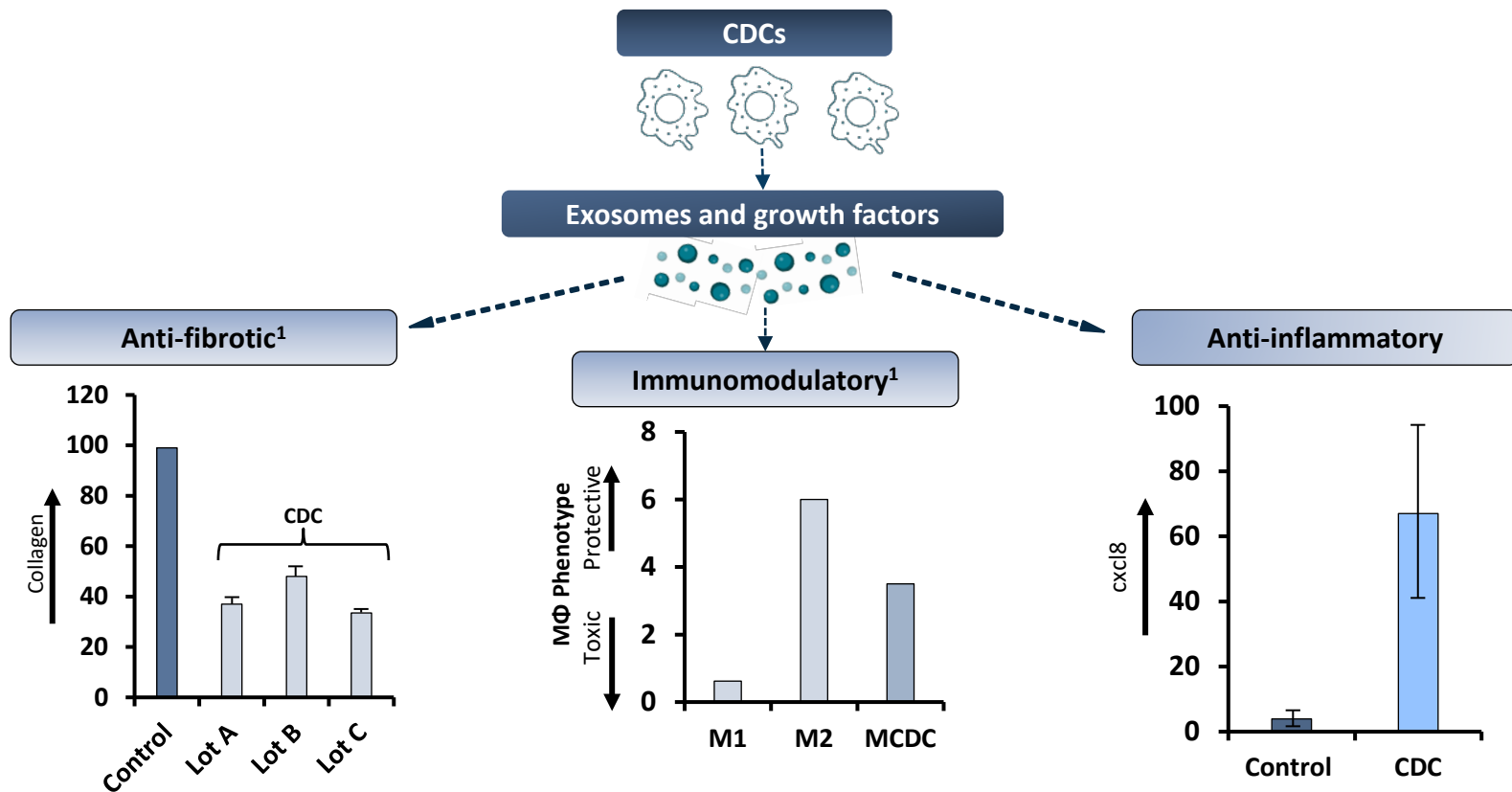


**Patients administered CDCs across multiple clinical trials**



**Doses of intravenous (IV) Deramiocele administered to treat patients with DMD**

# Deramiocele's Multi-Modal Mechanism



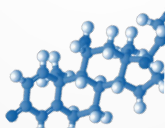
# Deramiciol has the Potential to Redefine the Standard of Care for Duchenne



Deramiciol can be used in combination with existing therapeutics



**GENE THERAPIES**



**EXON SKIPPING THERAPIES**

## Deramiciol



**First-in-class potential therapy for  
DMD  
CARDIAC AND SKELETAL MUSCLE**



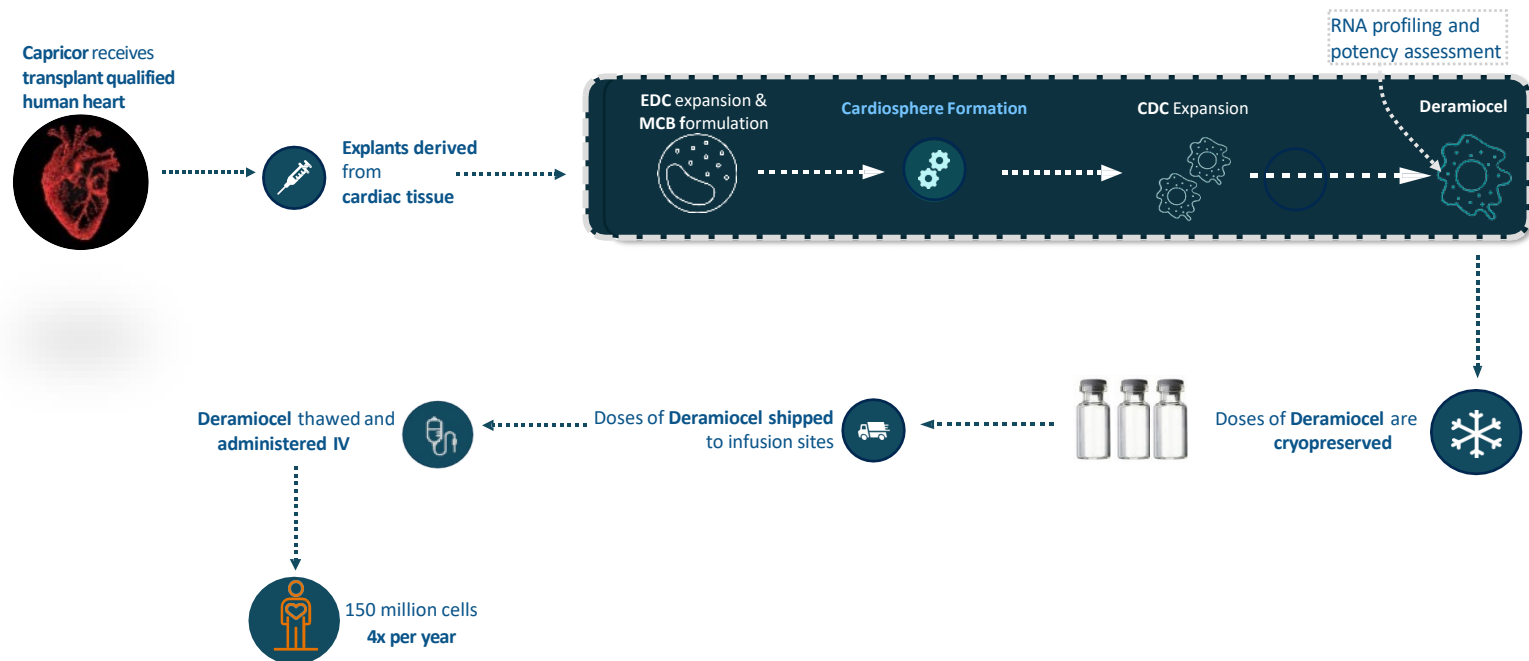
**CORTICOSTEROIDS**



**STANDARD CARDIAC  
MEDICATIONS**

# Deramiocele Manufacturing

## Novel Process Enables a Multi-dose Allogeneic Product





# Capricor's GMP Manufacturing Facility for Deramioce

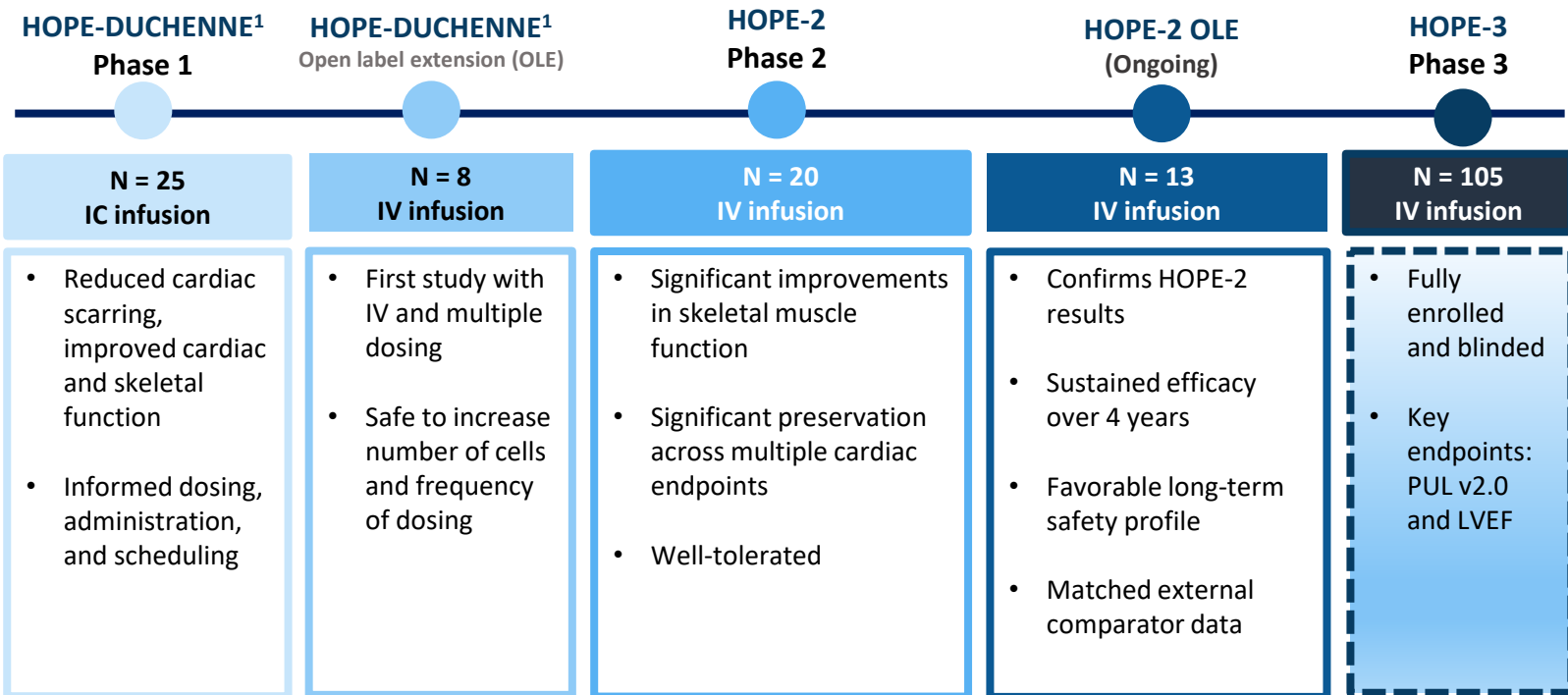
**La Jolla, California**

FDA successfully completed its  
Pre-License Inspection (PLI) in  
June 2025



# Deramioce<sup>l</sup>'s Clinical Development

10 Years of Development in DMD



# HOPE-2: Phase 2 Overview

- **Design:** Phase 2, randomized, double-blind, placebo-controlled trial in DMD patients with reduced skeletal muscle function (9 USA sites)
- **Deramiocecel dosing:** 150 million cells (IV delivery) every 3 months over 1 year
- **Data:** 20 subjects (12 placebo, 8 treated)
- **Primary endpoint:** mid-level PUL v1.2
- **Secondary endpoints:** LVEF, PUL v2.0, cardiac, etc.
- **Results:** published in *The Lancet* 2022

## ➤ Demographics

- Mean age: ~14 years
- All patients on corticosteroids
- ~90% of patients non-ambulant

### Repeated intravenous cardiosphere-derived cell therapy in late-stage Duchenne muscular dystrophy (HOPE-2): a multicentre, randomised, double-blind, placebo-controlled, phase 2 trial

Craig M McDonald, Eduardo Marbín, Suzanne Hendrix, Nathaniel Hogan, Rachel Ruckdeschel Smith, Michelle Eagle, Richard S Finkel, Cuixia Tian, Joanne Janas, Matthew M Hammelink, Arun S Varadachary, Michael D Taylor, Kan N Hor, Oscar H Mayer, Erik K Hennricson, Pat Furlong, Deborah D Ascheim, Siegfried Rogy, Paula Williams, Linda Marbín, with the HOPE-2 Study Group\*

#### Summary

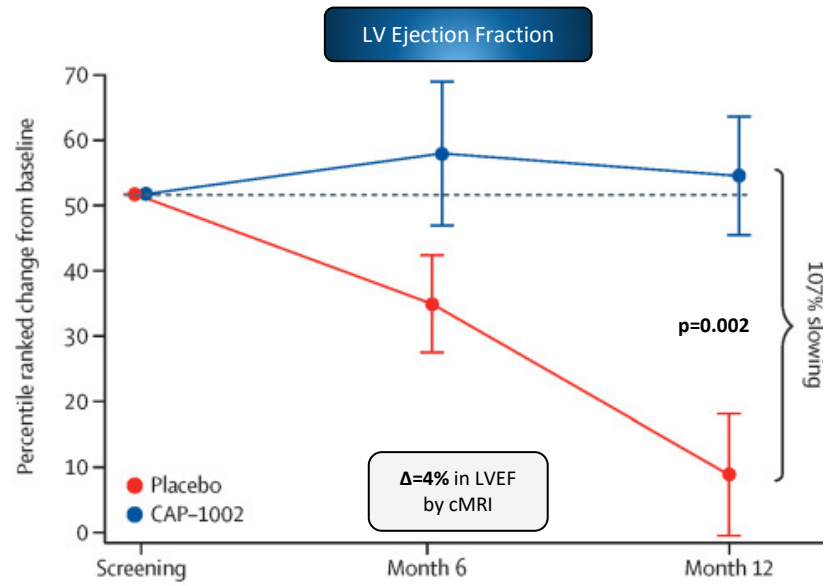
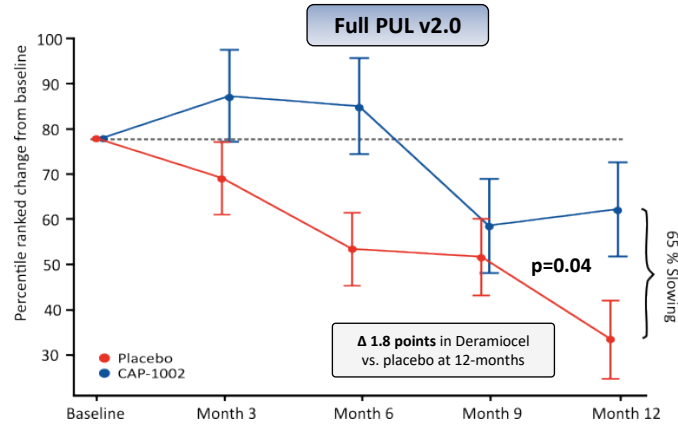
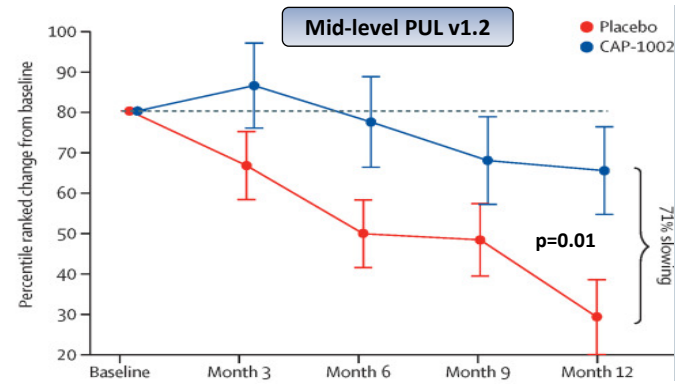
**Background** Cardiosphere-derived cells (CDCs) ameliorate skeletal and cardiac muscle deterioration in experimental models of Duchenne muscular dystrophy. The HOPE-2 trial examined the safety and efficacy of sequential intravenous infusions of human allogeneic CDCs in late-stage Duchenne muscular dystrophy.

**Methods** In this multicentre, randomised, double-blind, placebo-controlled, phase 2 trial, patients with Duchenne muscular dystrophy, aged 10 years or older with moderate upper limb impairment, were enrolled at seven centres in the USA. Patients were randomly assigned (1:1) using stratified permuted blocks to receive CAP-1002 ( $1.5 \times 10^8$  CDCs) or placebo intravenously every 3 months for a total of four infusions. Clinicians, caregivers, patients, and clinical operations personnel were fully masked to treatment groups. The primary outcome was the change in mid-level elbow Performance of Upper Limb version 1.2 (PUL 1.2) score at 12 months, assessed in the intention-to-treat population. Safety was assessed in all individuals who received an investigational product. This trial is registered with ClinicalTrials.gov, NCT03406780.

**Findings** Between March 1, 2018, and March 31, 2020, 26 male patients with Duchenne muscular dystrophy were enrolled, of whom eight were randomly assigned to the CAP-1002 group and 12 to the placebo group (six were not randomised due to screening failure). In patients who had a post-treatment PUL 1.2 assessment (eight in the CAP-1002 group and 11 in the placebo group), the mean 12-month change from baseline in mid-level elbow PUL 1.2 favoured CAP-1002 over placebo (percentile difference 36.2, 95% CI 12.7–59.7; difference of 2.6 points;  $p=0.014$ ). Infusion-related hypersensitivity reactions without long-term sequelae were observed in three patients, with one patient discontinuing therapy due to a severe allergic reaction. No other major adverse reactions were noted, and no deaths occurred.

# HOPE-2: Breakthrough Data Slowing DMD Progression

## Statistically Significant Skeletal and Cardiac Results



Results published in *The Lancet* March 2022.  
Percentile ranked change from baseline represents the percentage of all change scores smaller than the given value, where lower percentile ranked change indicates more disease progression.  
Percent slowing means how much slower the disease progressed on treatment vs. placebo.  
Mixed Model Repeated Measures analysis on percentile ranked change from baseline, adjusting for baseline score, treatment, visit, treatment-by-visit, PUL entry-item score at randomization, and site. Least squares means are graphed.

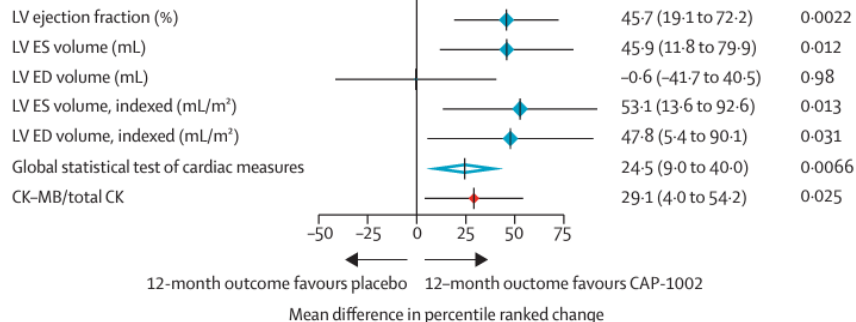
# HOPE-2: 21 of 22 Cardiac Measures

## Favored Deramiocecel Treatment over Placebo

- ◆ Secondary efficacy endpoint
- ◆ Exploratory efficacy endpoint
- ◆ Exploratory cardiac biomarker endpoint
- ◆ Exploratory combined score

Outcome	Least square mean (95% CI)	p value
LV ejection fraction (%)	45.7 (19.1 to 72.2)	0.0022
LV ES volume (mL)	45.9 (11.8 to 79.9)	0.012
LV ED volume (mL)	-0.6 (-41.7 to 40.5)	0.98
LV ES volume, indexed (mL/m <sup>2</sup> )	53.1 (13.6 to 92.6)	0.013
LV ED volume, indexed (mL/m <sup>2</sup> )	47.8 (5.4 to 90.1)	0.031
Systolic wall thickening septal LV (%)	0.9 (-40.5 to 42.3)	0.96
Systolic wall thickening lateral LV (%)	21.7 (-25.2 to 68.7)	0.35
Systolic wall thickening inferior LV (%)	14.6 (-16.2 to 45.5)	0.33
Systolic wall thickening anterior LV (%)	0.2 (-25.3 to 25.7)	0.96
ES wall thickness average, septal (mm)	6.6 (-28.1 to 41.4)	0.69
ES wall thickness average, lateral (mm)	20.0 (-15.1 to 55.2)	0.23
ES wall thickness average, inferior (mm)	31.5 (-7.0 to 70.0)	0.11
ES wall thickness average, anterior (mm)	30.7 (-11.5 to 72.8)	0.17
ED wall thickness average, septal (mm)	5.5 (-39.4 to 50.4)	0.78
ED wall thickness average, lateral (mm)	16.9 (-32.1 to 65.9)	0.47
ED wall thickness average, inferior (mm)	19.5 (-21.5 to 60.5)	0.33
ED wall thickness average, anterior (mm)	36.2 (-0.6 to 73.0)	0.053
Circumferential strain, septal LV (%)	22.8 (-10.1 to 55.7)	0.16
Circumferential strain, lateral LV (%)	17.8 (-22.8 to 58.5)	0.35
Circumferential strain, inferior LV (%)	35.0 (-0.8 to 70.9)	0.055
Circumferential strain, anterior LV (%)	3.1 (-27.6 to 33.7)	0.82
Global circumferential strain (%)	2.6 (-47.4 to 52.7)	0.94
Global statistical test of cardiac measures	24.5 (9.0 to 40.0)	0.0066
CK-MB/total CK	29.1 (4.0 to 54.2)	0.025

12-month outcome favours placebo 12-month outcome favours CAP-1002  
Mean difference in percentile ranked change

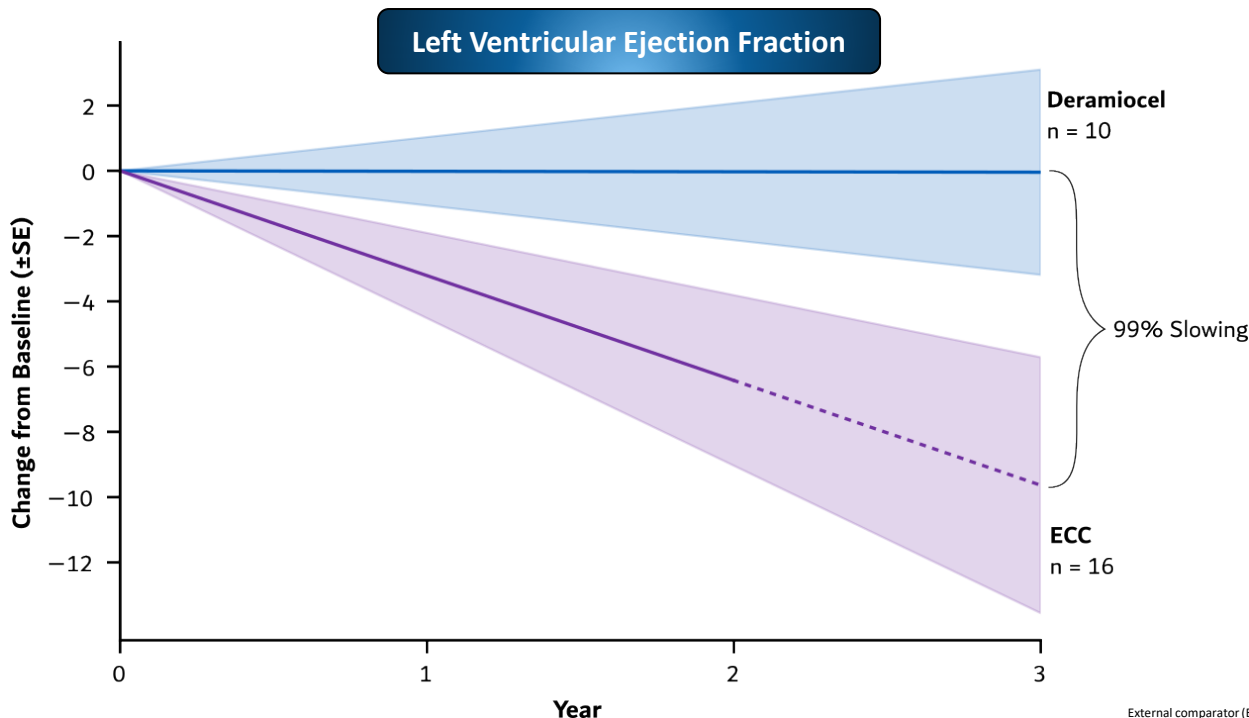


**Statistically significant treatment effect  
in critical measures of cardiac function**



# HOPE-2 OLE: 3-Year Cardiac Results

99% Slowing of Disease Progression



Yearly  $\Delta = 3.2$   
2-year  $\Delta = 6.4$   
 $p=0.008$

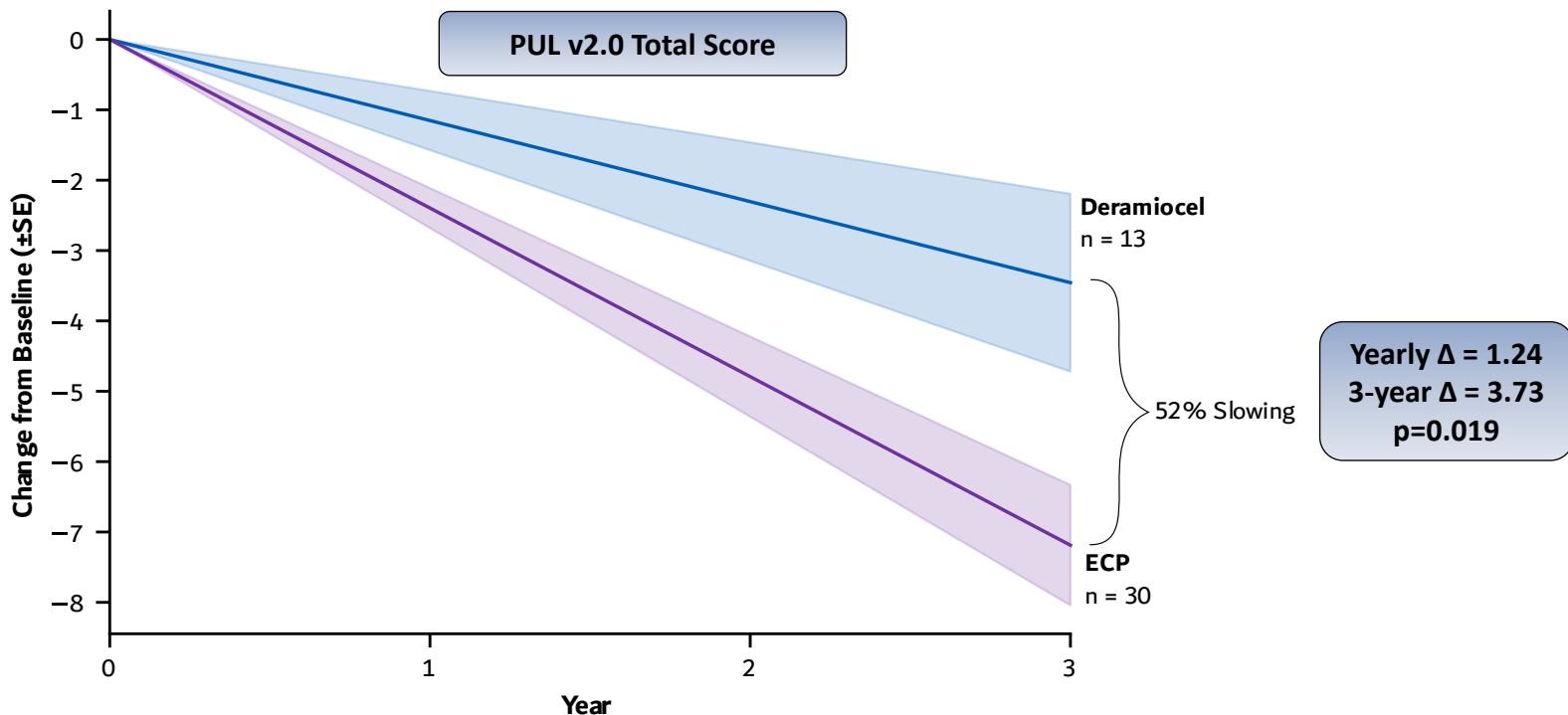
\*Soslow, et al. Cardiovascular Measures of All-Cause Mortality in DMD. *Circ Heart Fail*. 2023

Capricor Therapeutics, Inc. | Developing Transformative Therapies from Bench to Bedside

External comparator (ECC) from Vanderbilt University Medical Center  
Propensity-score matched using age, baseline LVEF and their interaction  
All patients on standard of care cardiac medication  
Deramioceol patients with 2- and 3-year cardiac MRI  
External controls with at least 2 years of follow-up

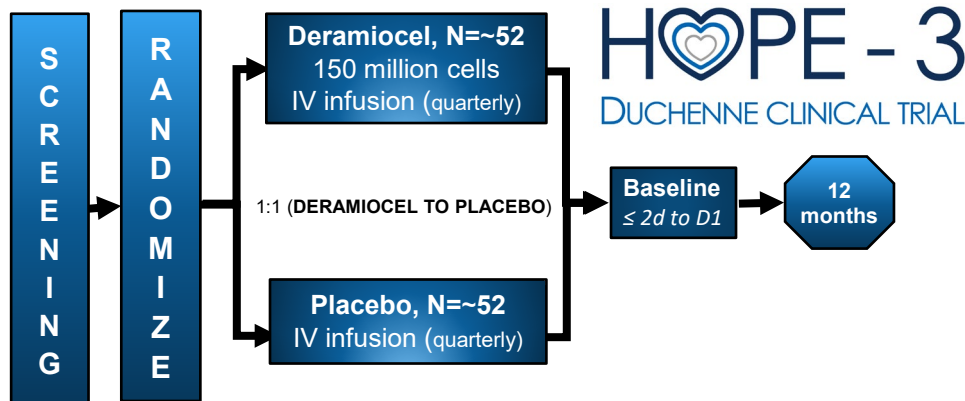
# HOPE-2 OLE: 3-Year Skeletal Results

52% Slowing of Disease Progression



# HOPE-3 Pivotal Phase 3 Trial

Topline Data Expected in Fourth Quarter of 2025



## Design & Endpoints

- Fully enrolled: 105 patients
- Randomized, 1:1, double-blind, placebo-controlled
- Key endpoints: PUL v2.0 (primary) and LVEF

## Successful Futility Analysis

- Completed in 2023 (Cohort A)

## Next Steps

- Plan to resubmit to current BLA with HOPE-3 results following receipt of data



# DMD: Large Commercial Opportunity

## Deramioce<sup>l</sup>

Potential to be  
the first-in-class  
cellular therapy  
for DMD  
patients

### Prevalence<sup>1</sup>

**~15,000**

DMD patients in **United States**

**~150,000-200,000**

DMD patients **worldwide**

### Life Expectancy

**25-30 years**

### Disease Burden

**High unmet clinical need**

Patients experience highly **burdensome** symptoms, including **progressive muscle damage, loss of ambulation**, respiratory issues and **cardiomyopathy**

### Market Size<sup>2</sup>

**~\$27 Billion**

**Global market** size estimated by **2030**

### Commercial Opportunity

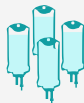
**Target reimbursement price**

Aim to be similar or **higher** than approved **exon skipping** therapies

# Deramiocele: Strong Commercial Profile

## Strong Clinical Profile<sup>1</sup>

### Multi-Functional Treatment



Deramiocele has immunomodulatory, anti-fibrotic and anti-inflammatory properties

### Slows DMD Progression



Data has shown to **slow DMD cardiac and skeletal disease progression**

### Sustained Benefit



**Long-term data** continue to suggest potential **disease attenuation** out to **4 years**

### Safety Profile



Over **800 IV infusions** of Deramiocele to date with **favorable safety profile**

## Significant Potential Commercial Reach

### Commercial Partnership



**Alliance** with an industry leader in **Nippon Shinyaku** for **U.S. and Japanese** markets

### Patient Support



**Patient support** leveraging **Capricor's deep understanding of patients and physicians**

### Large Reimbursement Potential



With small market penetration, **annual revenue** estimates could **exceed 1.5B<sup>2</sup>**; pricing estimates **similar to approved** exon skipping drugs

# Partnership with Nippon Shinyaku

## Commercial Distribution of Deramiciol for DMD



- Capricor responsible for product manufacturing and clinical activities necessary for potential approvals in select territories
- Nippon Shinyaku and NS Pharma teams to support broad commercialization efforts



**日本新薬**  
NIPPON SHINYAKU CO., LTD.



**NS Pharma**

### United States Partnership

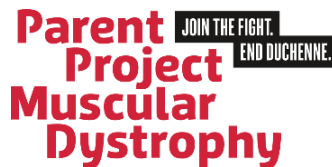
- Capricor to receive \$80M milestone payment at approval and up to \$605M in potential sales-based milestones
- Capricor to receive between 30-50% of product revenue, offset by amount paid for purchase of the product

### Japan Partnership

- Capricor to receive up to \$89M<sup>1</sup> in potential milestones and double-digit share of product revenue

**Europe Territory** - discussions ongoing

# Key Duchenne Advocacy Relationships



# World-Class DMD Advisory Board



## Pat Furlong

Parent Project Muscular Dystrophy (USA)

## Kan Hor, M.D.

Nationwide Children's Hospital (USA)

## Timothy Franson, M.D., FACP, FIDSA

Faegre Drinker Biddle & Reath LLP (USA)

## Michelle Eagle, Ph.D., M.Sc., MCSP

Atom International Ltd. (UK)

## Oscar Henry Mayer, M.D.

Children's Hospital of Philadelphia (USA)

## Eugenio Mercuri, M.D., Ph.D.

Catholic University of the Sacred Heart (Italy)

## Suzanne Hendrix, Ph.D.

Pentara Corporation (USA)

## Francesco Muntoni, M.D.

University College London (UK)

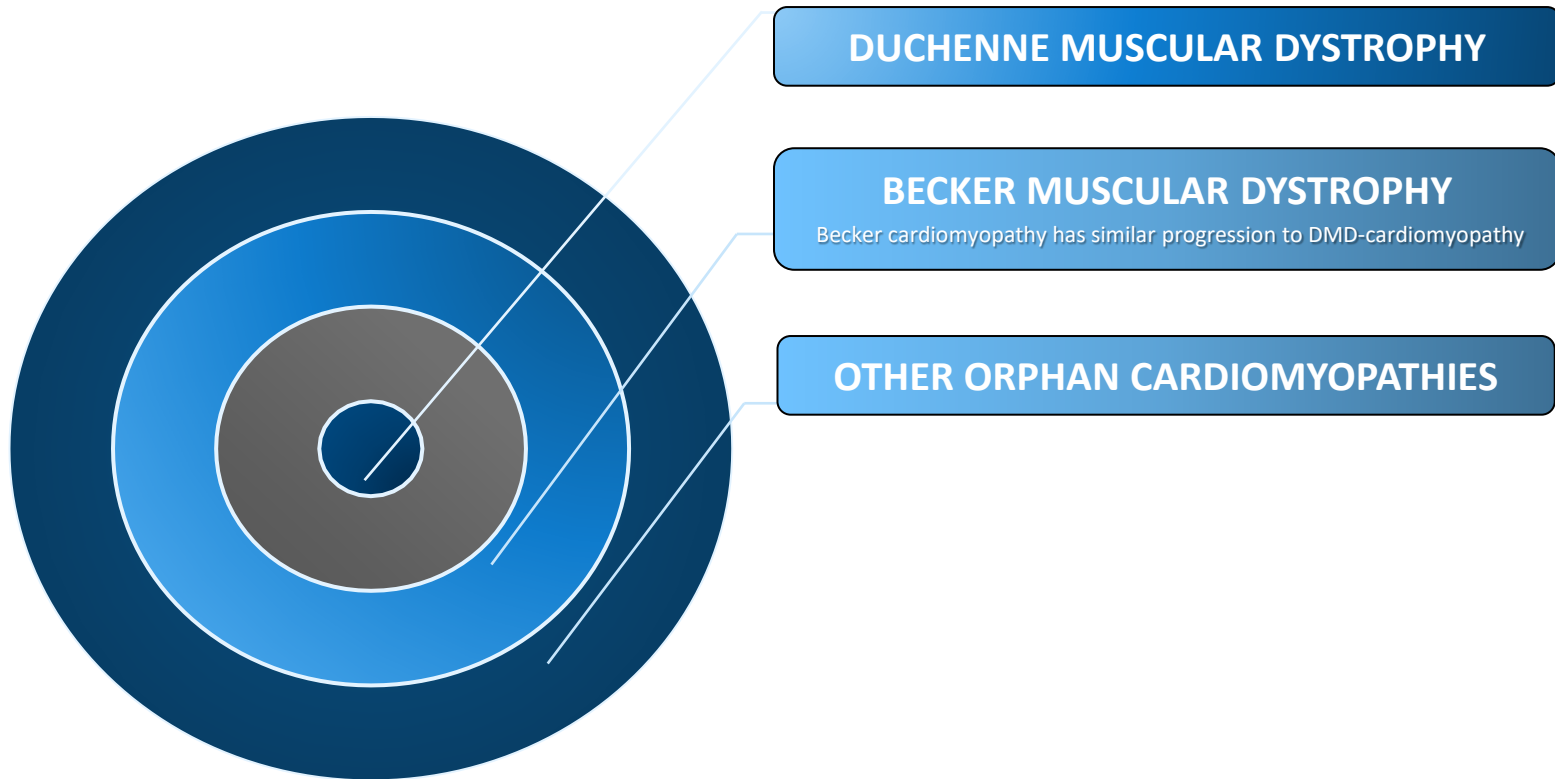
## Michael Taylor, M.D., Ph.D.

Texas Children's Hospital (USA)

## Chet Villa, M.D.

Cincinnati Children's Hospital Medical  
Center (USA)

# Potential Indication Expansion of Deramiocele



# Table of Contents

Capricor Overview

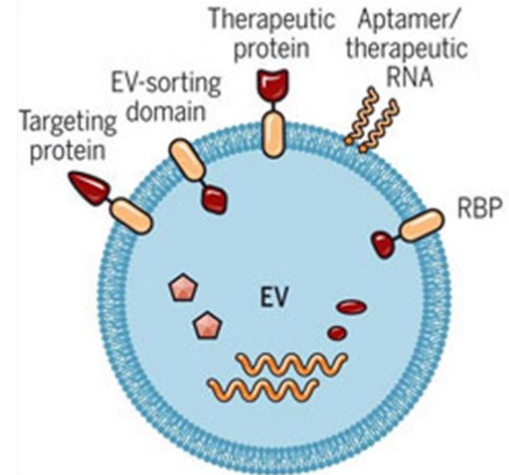
Deramiocele DMD Program Overview

StealthX™ Exosomes Platform Overview

# Exosomes are Nature's Delivery Tool

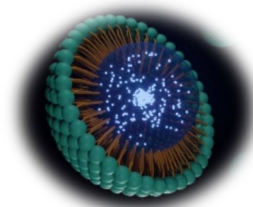
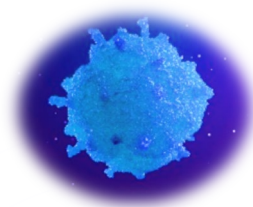
## Natural Drug Delivery Platform

- ~100 nanometer vesicles
- Made by nearly all cells
- Abundant in blood and biofluids
- Transfers signals and molecules to other cells
- Decades of transfusion and transplantation medicine indicates safety
- Can be used to deliver RNAs, DNA, proteins and small molecules





# Potential Benefits: Exosomes vs. LNPs



	Natural Exosomes	Synthetic LNPs
Commercial Manufacturing	+	+++
Drug/Therapeutic Loading	++	++
Drug/Therapeutic Release	+++	+
Cellular Uptake	+++	+
Targeting	+++	+
Low Immunogenicity	+++	+
Safety (expected)	(+++)	+
Clinical trials	+	+++

# StealthX™ Exosome Platform

StealthX™ technology allows Capricor to present diversified proteins outside of exosomes

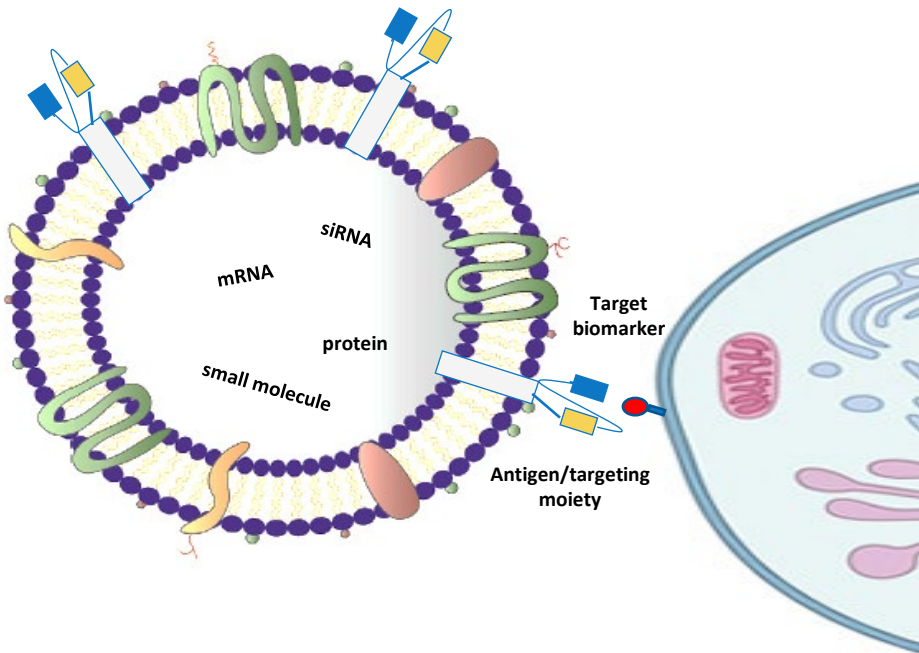
- ✓ Soluble proteins (ex. ScFvs)
- ✓ Transmembrane proteins (ex. Receptors)
- ✓ Viral antigens

StealthX™ technology allows Capricor to load diversified payloads inside of exosomes

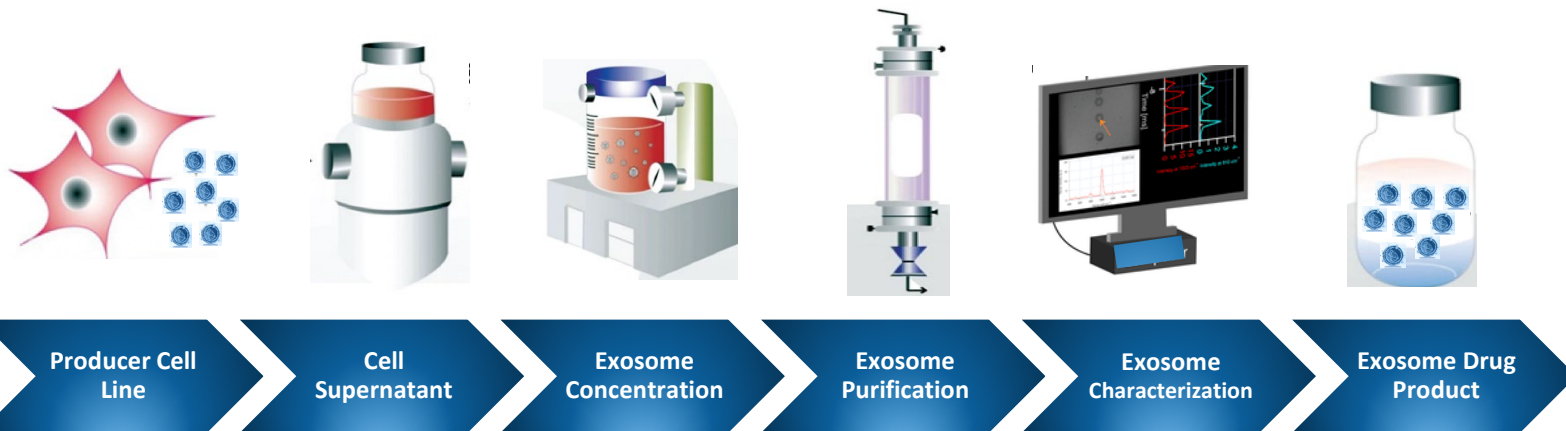
- ✓ siRNA
- ✓ miRNA
- ✓ ASOs
- ✓ Proteins
- ✓ Peptides
- ✓ Small molecules

Potential cell and tissue specific targets with targeting moieties

- ✓ Muscle
- ✓ Brain
- ✓ Lung



# Exosomes: Scalable Production



❖ **Capricor has developed a scalable, reproducible process for exosome purification**

- Producer cell line is widely used for production in other applications
- Exosome purification process developed using scalable processes

❖ **Capricor's exosomes have been extensively characterized using qualified assays**

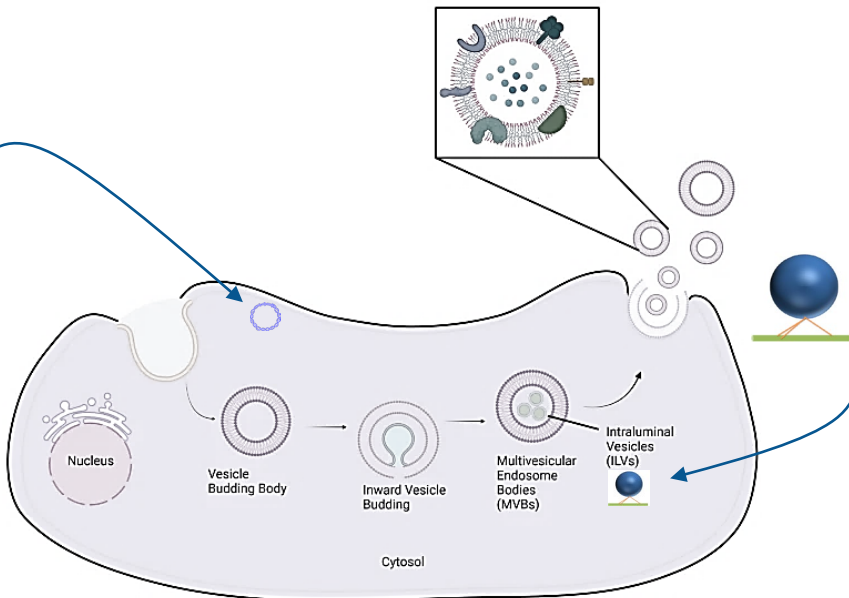
- >20 exosome assays developed and qualified with guidance from FDA
- Exosome yield, size, surface expression, payload content, loading and potency

# Exosome Loading of Drug Payloads

## Endogenous and Exogenous Methods

### Endogenous Loading

- Proprietary StealthX™ engineering
- StealthX™ producer cells and exosomes
- Surface proteins
- Therapeutic cargos inside exosomes



### Cell Based Cargo Packaging

- Co-incubation
- Transfection
- Electroporation
- Extrusion
- Freeze-thaw
- Proprietary cargo modification

# Active NIH Collaboration Underway



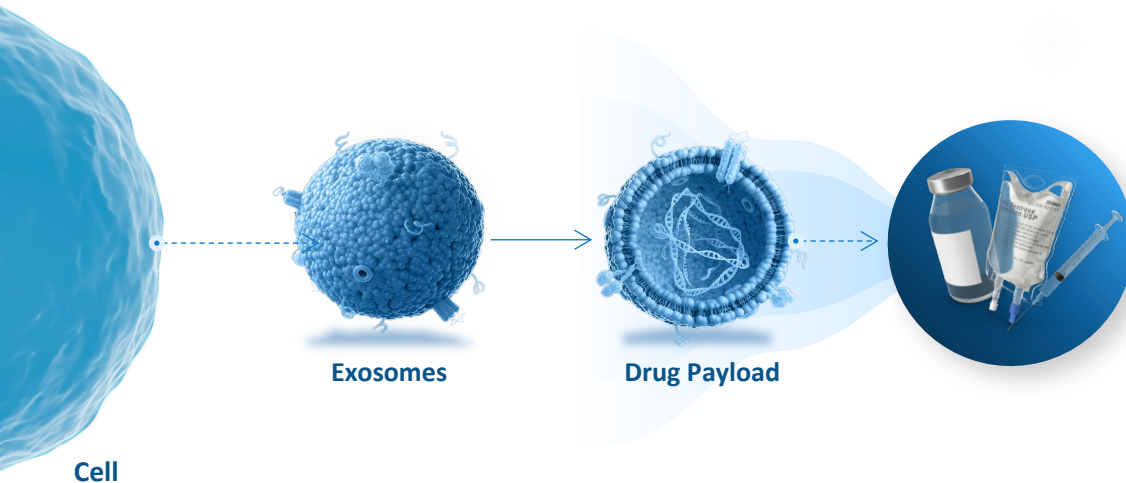
## Exosome-Based Multivalent Vaccine

- Capricor's **StealthX™ vaccine** was **selected by Project NextGen**
  - Aim is to advance a pipeline of innovative vaccines which may provide broader and more durable protection against COVID-19 and other potential infectious threats
- The **National Institute of Allergy and Infectious Diseases (NIAID)** is conducting and funding the **Phase 1 clinical trial**
  - Study is underway with **topline data** expected in **~Q1 2026**
  - Capricor is supplying investigational product
- If NIAID finds our vaccine **meets** its criteria for **safety** and **efficacy**, they may consider our program for a **funded Phase 2**



# StealthX™ Exosome Platform

Building a New Class of Medicines



- **Monogenic Diseases**

RNA, protein and small molecule therapeutics

- **Infectious Diseases**

Vaccines

- **Oncology**

Vaccines and targeted delivery therapeutics

## ✓ Goals

↗ Scale and partner

👤 Drive research through collaborations

⚡ Expand and exploit platform and IP through partnerships

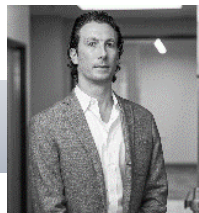
# Experienced Leadership Team

Extensive Scientific and Operational Experience Across Pharma, Biotech & Clinical Development



**Linda Marbán, Ph.D.**  
Chief Executive Officer

*Prior experience: Excligen, Johns Hopkins University*



**AJ Bergmann, M.B.A**  
Chief Financial Officer

*Prior experience: Gettleston, Witzer & O'Connor*



**Michael Binks, M.D.**  
Chief Medical Officer

*Prior experience: Pfizer, GlaxoSmithKline*



**Kristi Elliott, Ph.D.**  
Chief Operating & Science Officer

*Prior experience: Exotech, Intrexon Corp*




**Mark Awadalla**  
Chief Development Officer

*Prior experience: Celularity, Mustang Bio, Celgene*



**Karen Krasney, J.D.**  
Executive VP and General Counsel

*Prior experience: Biosensors International*



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