



## Corporate Presentation

Capricor Therapeutics, Inc.

Nasdaq: CAPR

May 2026

# 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, revenue and reimbursement estimates, projected terms of definitive agreements, our financial position, our possible uses of existing cash and investment resources, and statements regarding our litigation with Nippon Shinyaku Co., Ltd. and NS Pharma, Inc., including the nature of the dispute, our expectations regarding any legal proceedings, and our ability to commercialize Deramiocel independent of our existing distribution agreement 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, 2025, as filed with the Securities and Exchange Commission on March 17, 2026 and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, as filed with the Securities and Exchange Commission on May 13, 2026. All forward-looking statements in this presentation are based on information available to Capricor as of the date hereof, and Capricor assumes no obligation to update these forward-looking statements.

Deramiocel and the StealthX™ vaccine are investigational candidates and have not been approved for commercial use in any indication.

At Capricor, we are committed to advancing transformative treatments and delivering meaningful outcomes for patients in need.



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Capricor Therapeutics (CAPR) Overview

Deramiocele DMD Program Overview

StealthX™ Exosomes Platform Overview

# Capricor's History & Evolution



From Discovery to Late-Stage Clinical Validation and Platform Expansion

**2012:** Initial publication demonstrating early clinical benefits of CDCs<sup>1</sup>

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



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

**2015:** Discovery of exosomes as the primary mode of action of CDCs



**2021:** Relocated Capricor's HQ to San Diego, California

**2022:** Published positive Phase 2 HOPE-2 results in [The Lancet](#)

**Present:** BLA accepted for review: PDUFA action Aug. 22, 2026

**Q4 2025:** Announced positive topline results from HOPE-3 Phase 3 study in DMD



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

**2014:** Uplisted to NASDAQ Capital Market (CAPR)

**2019:** Published positive clinical results from HOPE-Duchenne Phase 1 study in [Journal of Neurology](#)<sup>2</sup>



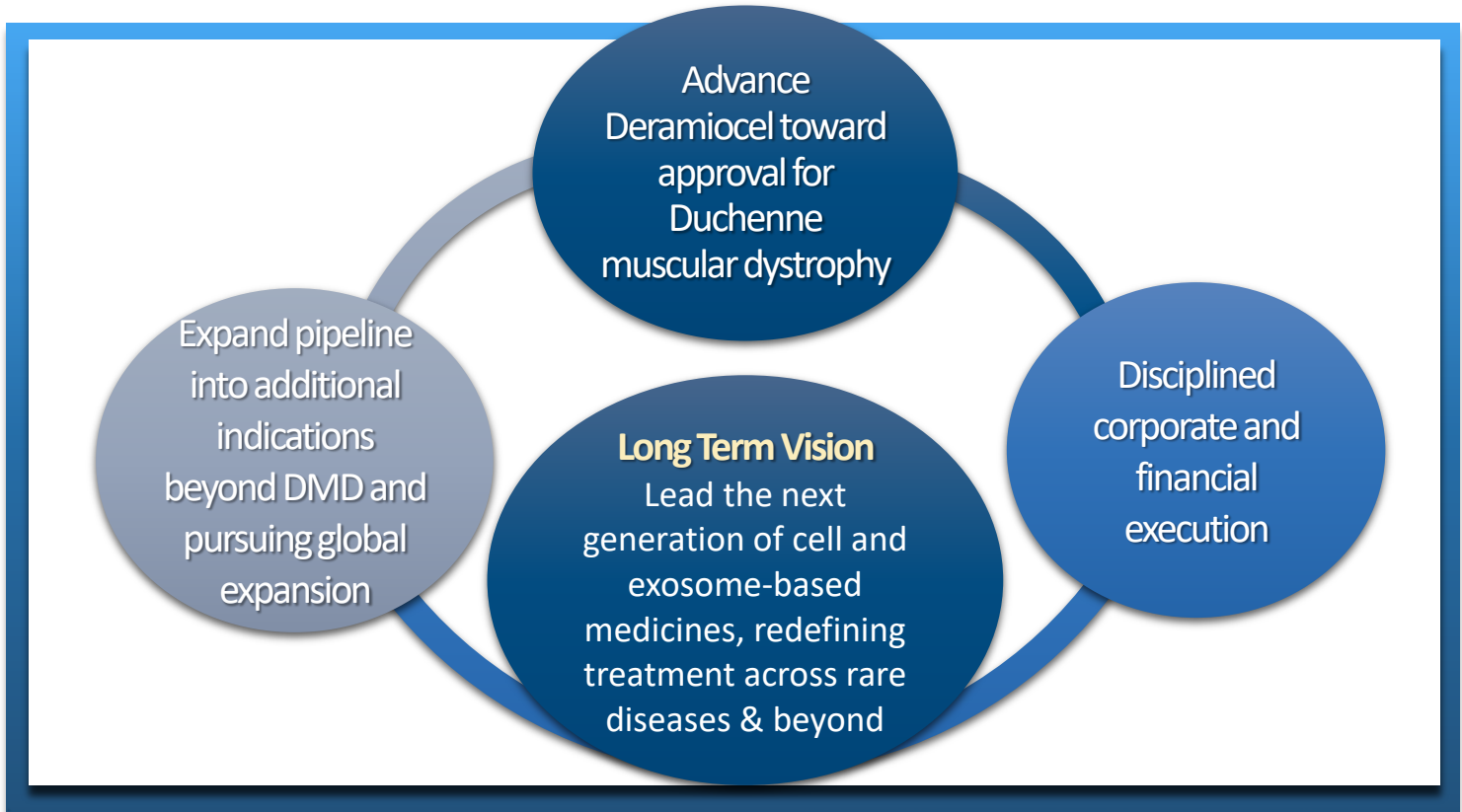
**2025:** Initiated Phase 1 StealthX™ exosome-based vaccine study in collaboration with the NIH

**Platform expansion** Leveraging exosome-biology beyond DMD into new indications

<sup>1</sup>Makkar, et al. [The Lancet](#), 2012

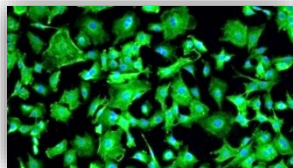
<sup>2</sup>Study funded with the support from the California Institute for Regenerative Medicine (CIRM)

# Capricor's Strategic Priorities



# Groundbreaking Science

## Developing First-in-Class Therapeutics



### Scientific Foundation Cardiology and Cell Biology

- **Initial technology:** developed at Johns Hopkins University
- **Lead product:** cellular therapy comprised of cardiosphere-derived cells (CDCs); endogenous human heart stromal cell population
- **Extensive IP portfolio:** ~150 patents & patent applications

### Lead Program in Rare Disease Duchenne Muscular Dystrophy



- **Lead indication:** DMD: rare, genetic disease afflicting 15,000 boys and young men in U.S.<sup>1</sup>
- **Positive safety and efficacy** results demonstrated across Phase 1, 2 and 3
- **In-house GMP manufacturing**
- **BLA:** currently under U.S. FDA review
- **PDUFA action date:** Aug. 22, 2026

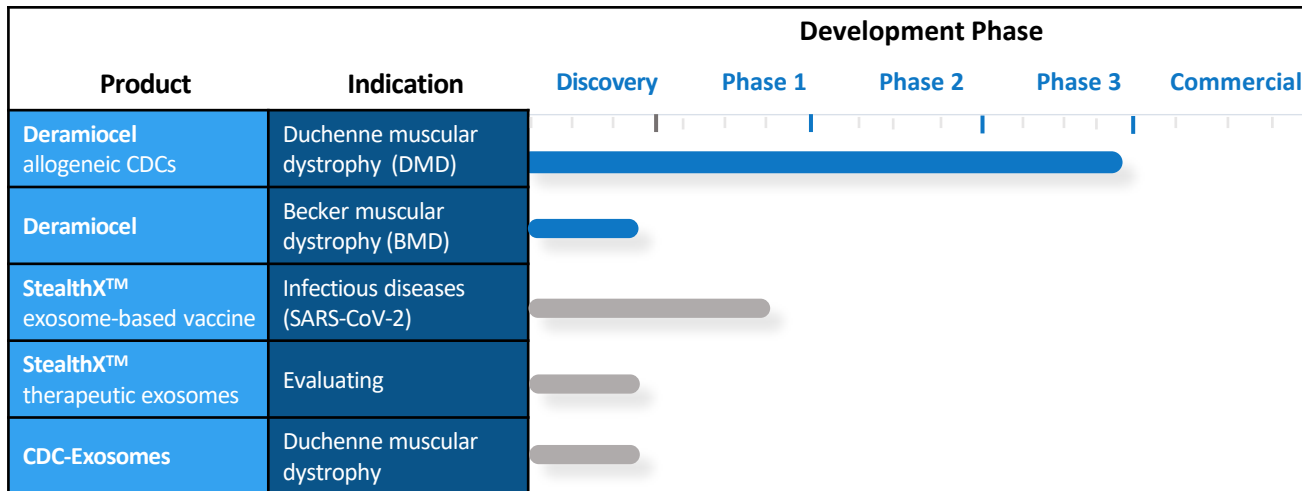


### Pipeline Development StealthX™ Exosome Platform

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

# Capricor's Product Pipeline

Advancing Transformative Therapies for Rare Diseases & Beyond



 Cell Therapy     Exosome Platform

# Deramiocele Duchenne Program

**PDUFA Target Action Date: August 22, 2026**

- ❖ **HOPE-3 Phase 3 trial** (n=106; randomized, double-blind, placebo-controlled) met its primary endpoint (PUL v2.0; p=0.03), key cardiac secondary endpoint (LVEF; p=0.04), and all Type I error-controlled secondary endpoints
- ❖ **Deramiocele BLA under active FDA review**; labeling discussions expected to commence soon
- ❖ If approved, Deramiocele would serve to be mutation agnostic, with the potential to address a broad DMD patient population
- ❖ **GMP manufacturing facility operational**; second-floor expansion underway
- ❖ **Building commercial capabilities to support a potential U.S. launch**

# Capricor: Financial Overview

## Runway and Potential Cash Infusions



<b>Cash balance</b>	<b>\$279 million<sup>1</sup></b>	<b>Employees: ~300 FTEs</b>
<b>Current runway</b>	<b>Through 2027*</b> *Excludes any potential revenue from product sales and the potential monetization of a PRV	
<b>Outstanding shares</b>	<b>57.9 million<sup>1</sup></b>	
<b>Eligible for priority review voucher (PRV)</b>	<b>Potential sale of PRV, if received</b> <b>~\$150-200 million<sup>2</sup></b>	

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Deramioce<sup>l</sup> DMD Program Overview

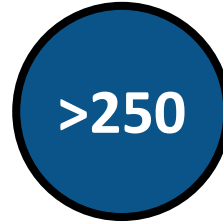
StealthX<sup>TM</sup> Exosomes Platform Overview

# Deramiocele: Cellular Therapy

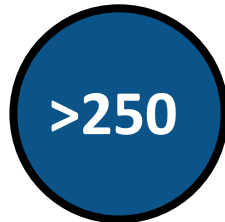
Comprised of Human Allogeneic Cardiosphere-Derived Cells (CDCs)



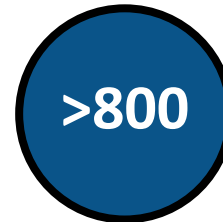
CDCs are derived from cells of transplant qualified human hearts; **they are not stem cells**



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

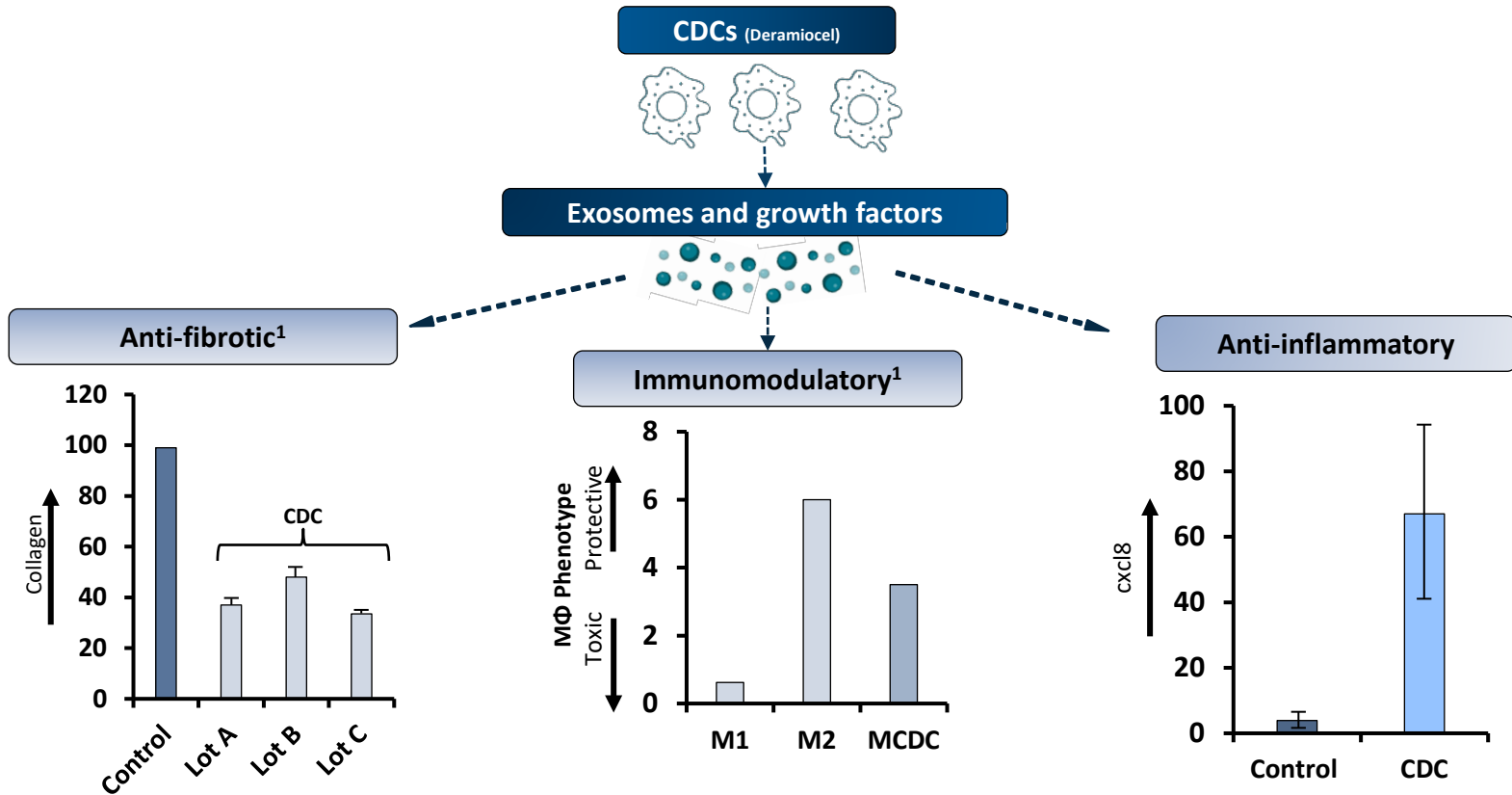


Patients administered CDCs across multiple clinical trials



Doses of intravenous Deramiocele administered to patients with DMD

# Deramiocel's Multi-Modal Mechanism



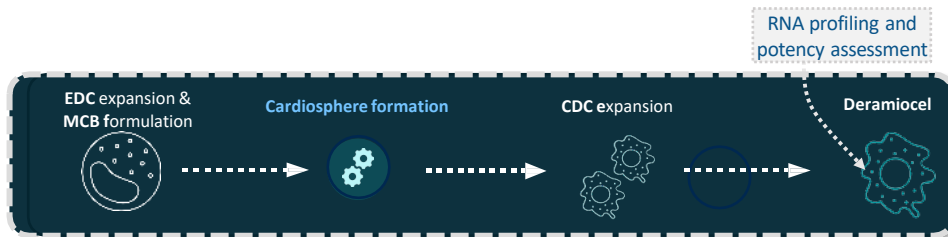
# Deramiocel Manufacturing

## Novel Process Enables a Multi-Dose Allogeneic Product

Capricor receives  
transplant qualified  
human heart



Explants derived  
from cardiac  
tissue



RNA profiling and  
potency assessment

EDC expansion &  
MCB formulation

Cardiosphere formation

CDC expansion

Deramiocel



Deramiocel doses shipped  
to infusion centers



Deramiocel doses are  
cryopreserved



Deramiocel  
administered IV



150 million cells  
4x per year

# Capricor's GMP Manufacturing Facility

**San Diego, California**

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



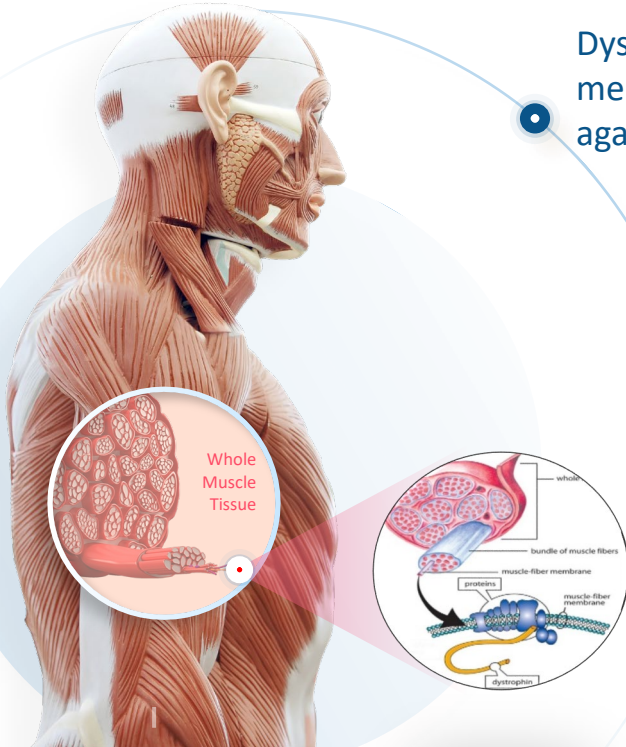
# DMD: A Devastating Rare Disease

## High Unmet Needs Across the Entire Disease Trajectory

Dystrophin is a key structural protein at the muscle cell membrane that maintains muscle integrity and protects against damage; acts both as a cushion and glue

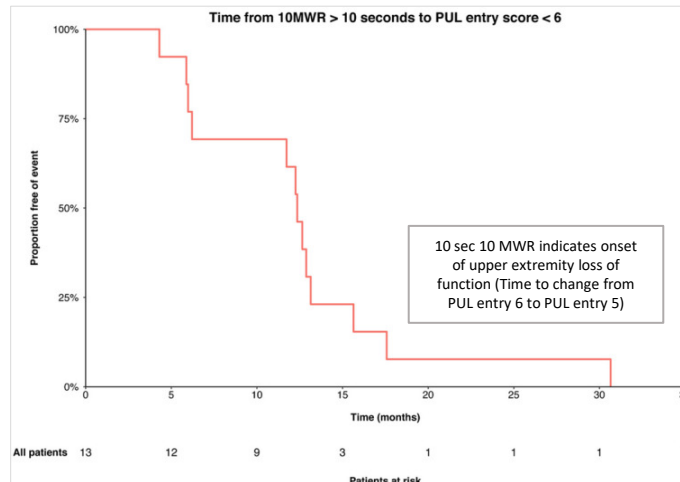
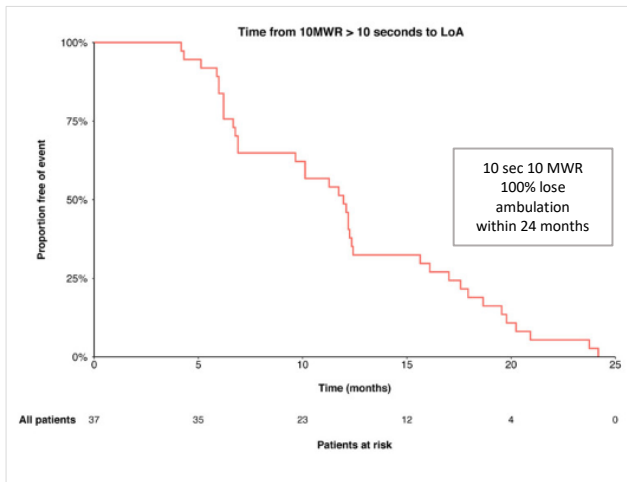
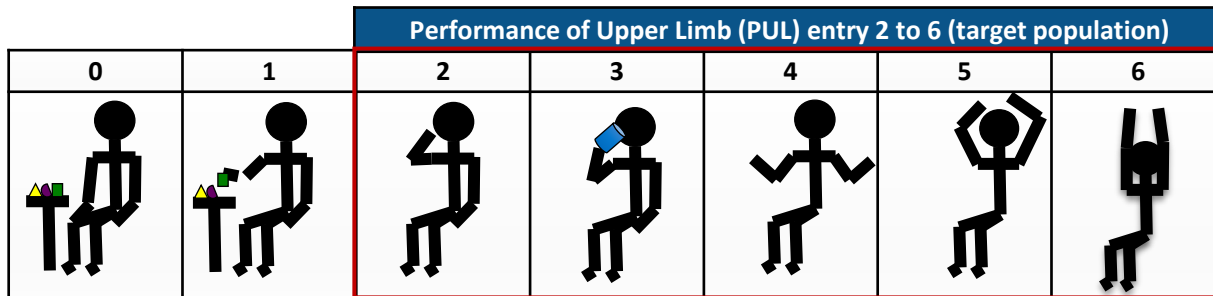
Much of the muscle injury that occurs in DMD is attributable to **secondary damage caused by inflammation**

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



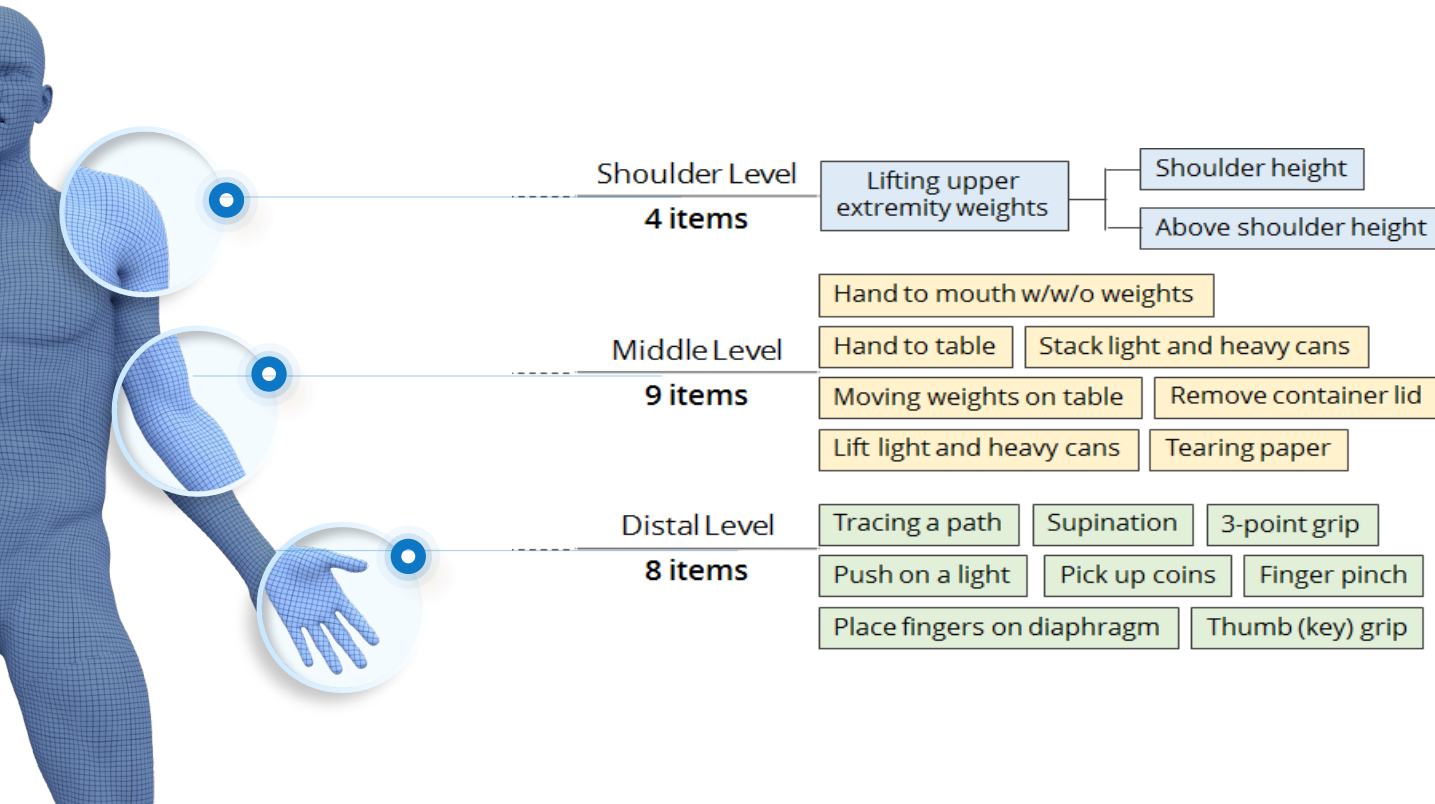
# Approaching Loss of Ambulation

## Late-Ambulatory Patients with DMD



# Performance of Upper Limb (PUL)

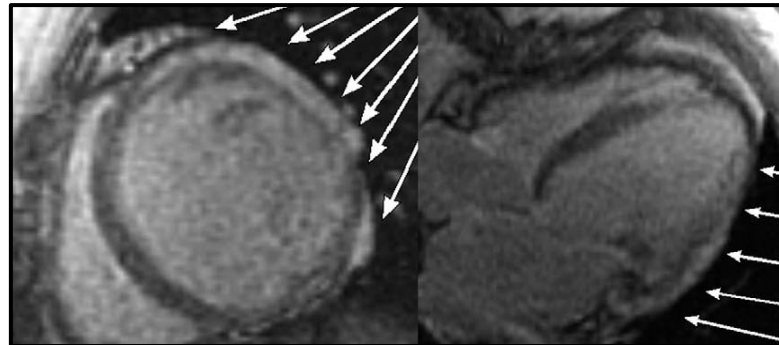
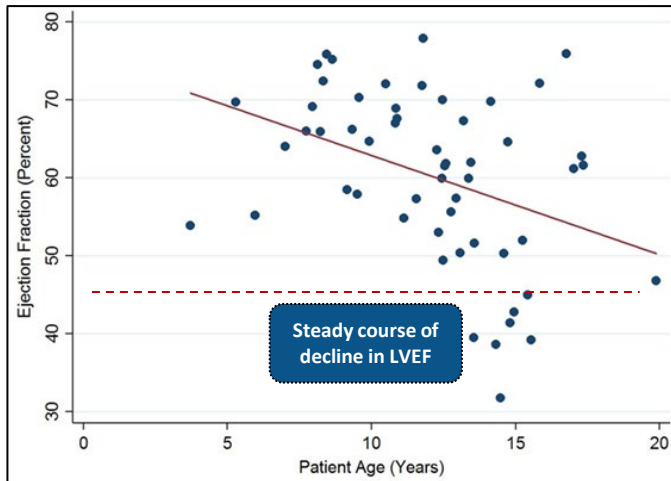
## Validated Tool to Assess Skeletal Muscle Function



# Duchenne Cardiomyopathy

**“Cardiopulmonary failure is the leading cause of mortality in DMD in the current era...Unfortunately, standard heart failure therapies are not DMD-specific and have limited efficacy...For maximal efficacy, most therapies should begin early in the disease process...”**

*Circulation: Heart Failure, (2023) , Soslow J.H., M.D., et al.*



**Cardiac MRI in DMD patient**

*Delayed enhancement indicative of myocardial fibrosis*

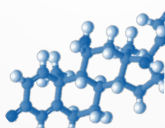
# Deramiocele has the Potential to Redefine the Standard of Care for Duchenne



Deramiocele can be used in combination with existing therapeutics



**GENE THERAPIES**



**EXON SKIPPING THERAPIES**

## Deramiocele



First-in-class potential therapy for Duchenne muscular dystrophy  
**CARDIAC AND SKELETAL MUSCLE**

- ❖ Orphan Drug Designation (FDA and EMA)
- ❖ Regenerative Medicine Advanced Therapy Designation (FDA)
- ❖ Rare Pediatric Disease Designation (FDA)
- ❖ Advanced Therapy Medicinal Product Designation (EMA)



**CORTICOSTEROIDS**



**STANDARD CARDIAC MEDICATIONS**

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

## A Decade of Development in Duchenne

### HOPE-DUCHENNE<sup>1</sup> Phase 1

**N = 25**  
**IC infusion**

- Improved skeletal and cardiac and function
- Reduced cardiac scarring
- Informed dosing and administration

### HOPE-DUCHENNE<sup>1</sup> Open label extension (OLE)

**N = 8**  
**IV infusion**

- First study with IV and multiple dosing
- Generally safe to increase number of cells and frequency of dosing

### HOPE-2 Phase 2

**N = 20**  
**IV infusion**

- Significant improvements in skeletal muscle function
- Significant preservation across multiple cardiac endpoints
- Generally well-tolerated

### HOPE-2 OLE

**N = 13**  
**IV infusion**

- Confirms HOPE-2 results
- Sustained efficacy shown over 4 years
- Favorable long-term safety profile
- Matched external comparator data

### HOPE-3 Phase 3

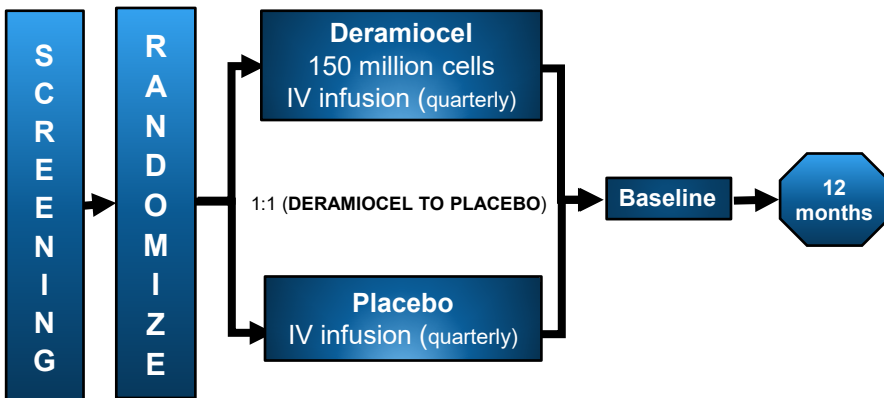
**N = 106**  
**IV infusion**

- Positive topline results announced in Dec. 2025
- Primary and key secondary endpoints met
- Generally well-tolerated

# HOPE-3 Pivotal Phase 3 Trial

## Overview

## HOPE - 3 DUCHENNE CLINICAL TRIAL



## Design & Endpoints

- ❖ Phase 3: randomized (1:1), double-blind, placebo-controlled study
- ❖ **N = 106 subjects randomized**
- ❖ Conducted in the United States: 20 clinical sites
- ❖ **Primary efficacy endpoint<sup>1</sup>:** PUL v2.0 *skeletal muscle assessment*
- ❖ **Key secondary endpoint<sup>1</sup>:** left ventricular fraction (LVEF) *cardiac assessment*
- ❖ **Other secondary endpoints<sup>1</sup>:** mid-level PUL v.2.0, GST and LGE
- ❖ **Announced positive topline results: Dec. 2025**

# HOPE-3: Study Demographics

Baseline Demographics	Placebo (n=52)	Deramioce <sup>l</sup> (n=54)	Overall (n=106) <sup>1</sup>
<b>Age (years)</b>			
N	52	54	106
Mean (SD)	14.6 (2.95)	15.4 (3.10)	15.0 (3.04)
Median	14	15	15
Min, Max	10, 22	10, 22	10, 22
<b>PUL v2.0 entry item score</b>			
2,3	23 (44.2)	25 (46.3)	48 (45.3)
4,5,6	29 (55.8)	29 (53.7)	58 (54.7)
<b>Diagnosed cardiomyopathy<sup>2</sup></b>			
No	14 (26.9)	13 (24.1)	27 (25.5)
Yes	38 (73.1)	41 (75.9)	79 (74.5)
<b>Baseline LVEF%</b>			
n	46	45	91
Mean (SD)	59.303 (6.108)	55.345 (7.743)	57.346 (7.206)
Median	59.309	55.892	57.532
Min, Max	47.395, 73.981	36.537, 71.112	36.537, 73.981
<b>Ambulatory status</b>			
Non-ambulatory	44 (84.6)	46 (85.2)	90 (84.9)
Ambulatory	8 (15.4)	8 (14.8)	16 (15.1)

<sup>1</sup>One subject enrolled but dropped out prior to baseline assessment (n=105)

<sup>2</sup>Updated as of Feb. 2026; subgroup: 64 of 79 patients with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months

# HOPE-3: Safety Profile Results

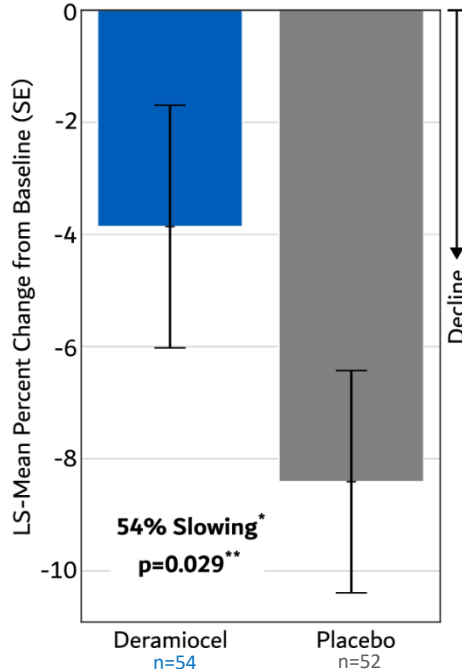
<b>Overview</b>	<b>Placebo (n=52), n (%)</b>	<b>Deramiocel (n=53), n (%)</b>	<b>Overall (n=105<sup>1</sup>), n (%)</b>
Any TEAEs	43 (82.7)	50 (94.3)	93 (88.6)
TEAEs related to IP or administration procedure	19 (36.5)	44 (83.0)	63 (60.0)
TEAEs related to IP	16 (30.8)	44 (83.0)	60 (57.1)
TEAEs related to administration procedure	9 (17.3)	23 (43.4)	32 (30.5)
TEAEs related to IP or administration procedure by maximum severity			
Mild (grade 1)	15 (28.8)	19 (35.8)	34 (32.4)
Moderate (grade 2)	3 (5.8)	25 (47.2)	28 (26.7)
Severe (grade 3)	0	0	0
Life-threatening (grade 4)	1 (1.9)	0	1 (1.0)
Fatal (grade 5)	0	0	0
TEAEs leading to death	0	0	0
Any serious TEAEs	5 (9.6)	1 (1.9)	6 (5.7)
Serious TEAEs related to IP or administration procedure	1 (1.9)	1 (1.9)	2 (1.9)

# HOPE-3: Topline Efficacy Results

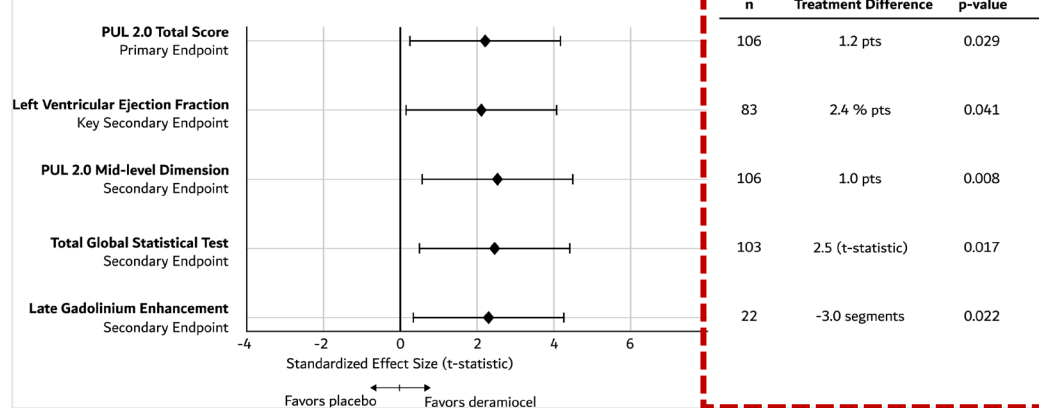
Primary Endpoint Met with Statistical Significance Achieved in All Type-1 Error Controlled Secondary Endpoints

## PUL 2.0 Total Score - Month 12

Primary Endpoint



## HOPE-3 Type-1 Error Controlled Endpoints - Deramiocel vs. Placebo



\*LS-Mean difference = 4.55 percentage point (1.2 -point difference on the PUL scale)

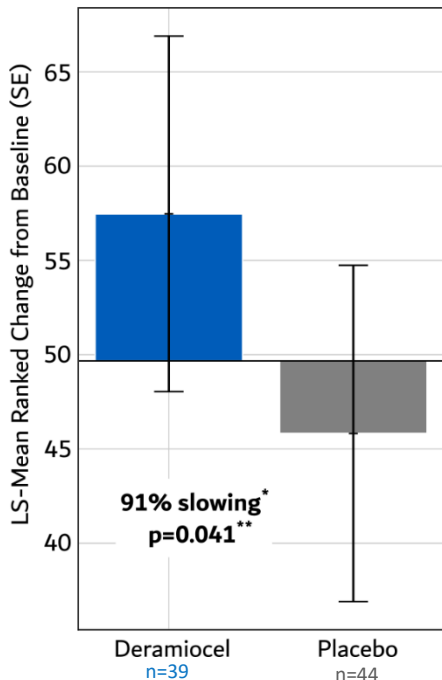
\*\* Based on prespecified repeated measures model using percent change from baseline

# HOPE-3: Topline Cardiac Efficacy Results

## Left Ventricular Ejection Fraction

### Left Ventricular Ejection Fraction - Month 12

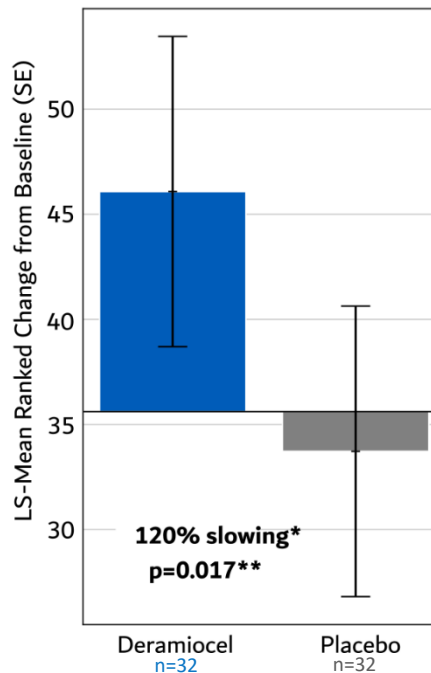
Key Secondary - ITT Population



\* LS-mean difference = 11.65 ranks (2.4 percentage point difference in LVEF)  
\*\* Based on prespecified rank ANCOVA model  
LVEF: n reflects the number of patients in the ITT population with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months (n=83)

### Left Ventricular Ejection Fraction - Month 12

Secondary Endpoint - Cardiomyopathy Population



\* LS-mean difference = 12.36 ranks (3.3 percentage point difference in LVEF)  
\*\* Based on prespecified rank ANCOVA model  
LVEF: n reflects the number of patients in the subgroup population with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months (n=64)

# DMD: Large Commercial Opportunity

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

Prevalence<sup>1</sup>

**15,000**

DMD patients in **United States**

**200,000**

DMD patients **worldwide**

Life  
Expectancy

**~25-30 years**

Disease  
Burden

**High unmet clinical need**

Patients experience **high symptom burden** including muscle weakness, **loss of ambulation**, loss of independence to transfer, feed or turn, **respiratory** 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

### First-in-Class Therapy for DMD



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

### Slows Disease Progression



Data shows **slowing of DMD skeletal and cardiac disease progression** in **multiple** clinical trials

### Sustained Benefit



**Long-term data** continue to support **safety and potential disease attenuation**

### Safety Profile



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

## Significant Potential Commercial Reach

### Commercial Infrastructure



Capricor is **building in-house commercial capabilities** to support the potential commercialization of **Deramiocele in the U.S.**

### Patient Support



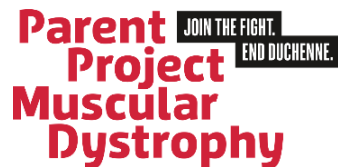
**Patient support** leveraging **Capricor's deep understanding of patients and physicians**

### Large Reimbursement Potential



Pricing estimates **similar to approved** exon skipping drugs

# Key Duchenne Advocacy Relationships



# World-Class Duchenne Advisory Board



**Craig McDonald, M.D. (National PI)**  
University of California, Davis (USA)

A headshot of Craig McDonald, a man with short grey hair and glasses, wearing a dark suit, white shirt, and patterned tie, smiling against a blue background.

**Jonathan Soslow, M.D. MSCI**  
Vanderbilt University Medical Center (USA)

**Chet Villa, M.D.**  
Cincinnati Children's Hospital Medical Center (USA)

**Timothy Franson, M.D., FACP, FIDSA**  
Faegre Drinker Biddle & Reath LLP (USA)

**Michelle Eagle, Ph.D., M.Sc., MCSP**  
Atom International Ltd. (UK)

**Eugenio Mercuri, M.D., Ph.D.**  
Catholic University of the Sacred Heart (Italy)

**Kan Hor, M.D.**  
Nationwide Children's Hospital (USA)

**Pat Furlong**  
Parent Project Muscular Dystrophy, PPMD (USA)

**Michael Taylor, M.D., Ph.D.**  
Texas Children's Hospital (USA)

# Potential Indication Expansion of Deramiocele

A diagram consisting of four concentric circles. The innermost circle is dark blue. The next ring is a lighter blue. The third ring is a medium blue. The outermost ring is the darkest blue. Three white lines originate from the innermost circle and extend outwards, pointing to the three text boxes on the right.

**DUCHENNE MUSCULAR DYSTROPHY**

**BECKER MUSCULAR DYSTROPHY**

Becker cardiomyopathy has similar progression to DMD-cardiomyopathy

**OTHER POTENTIAL DISEASES STATES**

- Cardiomyopathies
- Dystrophinopathies
- Muscular dystrophies

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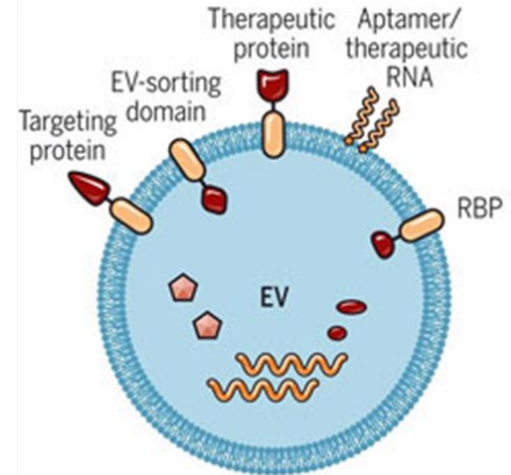
Deramiocele DMD Program Overview

StealthX™ Exosomes Platform Overview

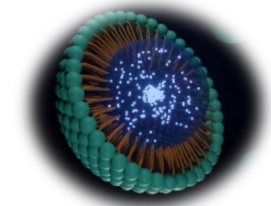
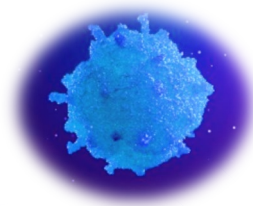
# Exosomes are Nature's Delivery Tool

## 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



	<i>Natural Exosomes</i>	<i>Synthetic LNPs</i>
Commercial manufacturing	+	+++
Therapeutic loading	++	++
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

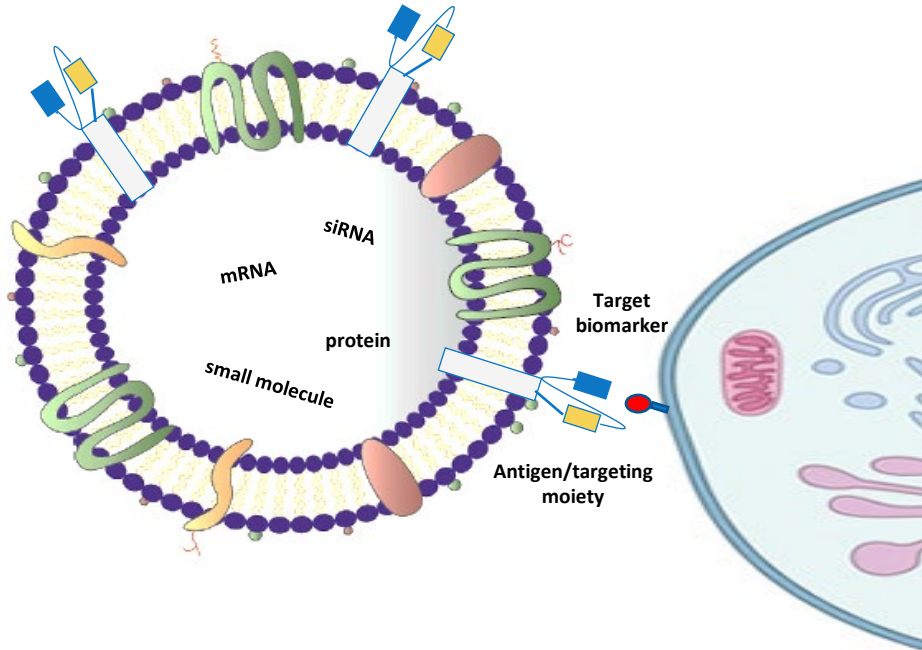
- ✓ Soluble proteins (ex. ScFvs)
- ✓ Transmembrane proteins (ex. Receptors)
- ✓ Viral antigens

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

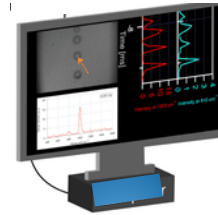
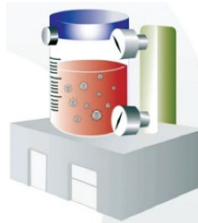
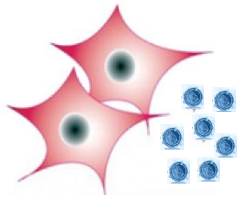
- ✓ RNA (siRNA, etc.)
- ✓ ASOs
- ✓ Proteins
- ✓ Peptides
- ✓ Small molecules

Potential cell and tissue specific targets with targeting moieties

- ✓ Muscle
- ✓ Brain
- ✓ Lung



# Exosomes: Scalable Production



Producer Cell Line

Cell Supernatant

Exosome Concentration

Exosome Purification

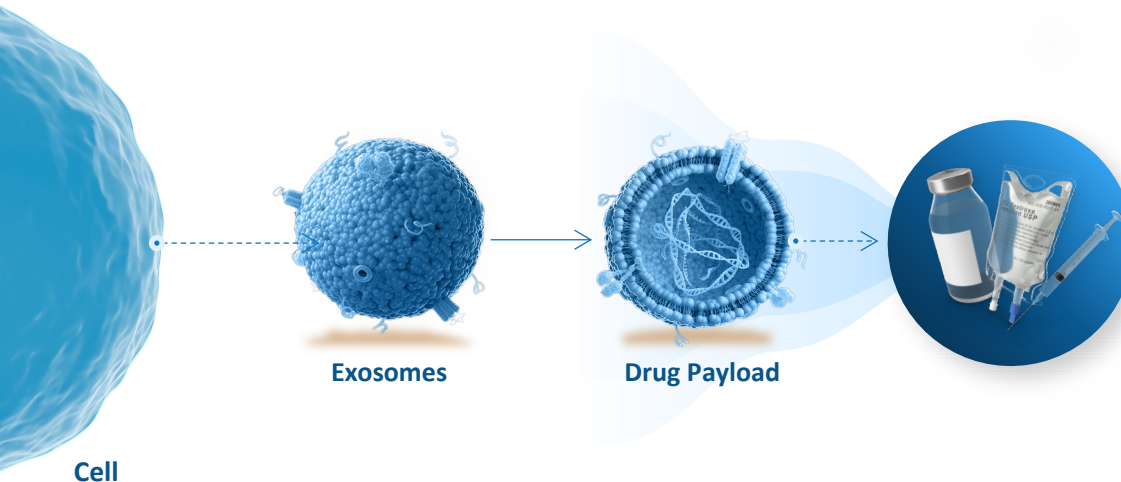
Exosome Characterization

Exosome Drug Product

- ❖ **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**
  - Exosome assays developed and qualified with guidance from FDA
  - Exosome yield, size, surface expression, payload content, loading and potency

# StealthX™ Exosome Platform

Building a New Potential 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

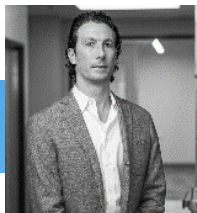
# Experienced Leadership Team

Extensive Scientific and Operational Experience Across Pharma & Biotech



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

*Prior experience: Excigen, 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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