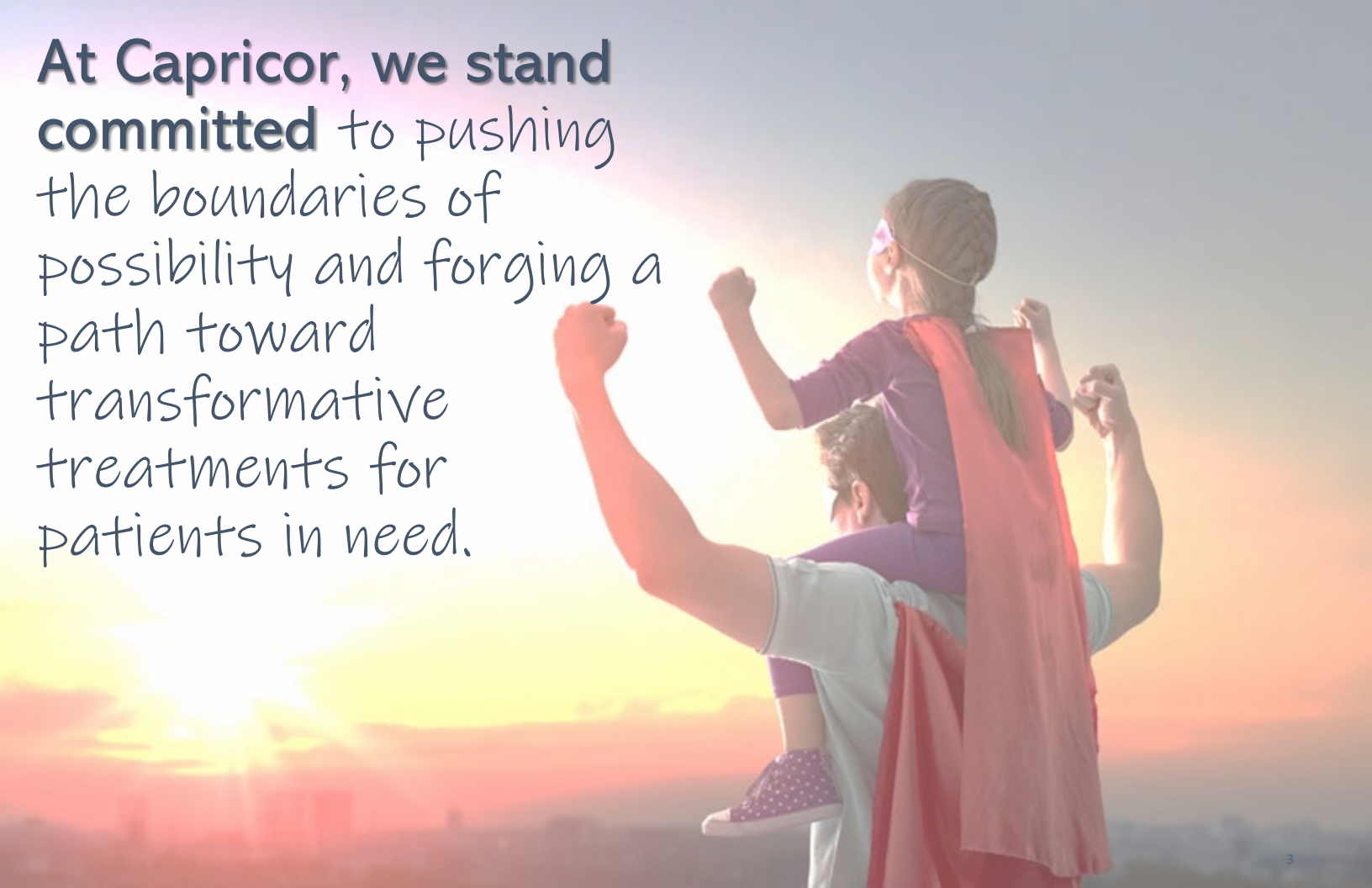

Capricor
Therapeutics™
Corporate Presentation
Capricor
Therapeutics™
Capricor Therapeutics, Inc.
Nasdaq: CAPR
November 2024

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 discovery efforts and clinical trials; the pace of enrollment of clinical trials; plans regarding regulatory filings, future research and clinical trials; regulatory developments involving products, including the ability to obtain regulatory approvals or otherwise bring products to market; manufacturing capabilities; dates for regulatory meetings; statements about our financial outlook; the ability to achieve product milestones and to receive milestone payments from commercial partners; plans regarding current and future collaborative activities and the ownership of commercial rights; potential future agreements; scope, duration, validity and enforceability of intellectual property rights; future reimbursement prices; future revenue streams and projections; expectations with respect to the expected use of proceeds from the recently completed offerings and the anticipated effects of the offerings; 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, 2023 as filed with the Securities and Exchange Commission on March 11, 2024 and in our Quarterly Report on Form 10-Q for the period ended September 30, 2024 as filed with the Securities and Exchange Commission on November 14, 2024. 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.

Deramiocel (CAP-1002) is an Investigational New Drug and is not approved for any indications. None of Capricor's exosome-based candidates have been approved for clinical investigation.

At Capricor, we stand committed to pushing the boundaries of possibility and forging a path toward transformative treatments for patients in need.



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Capricor's Evolution & History

2004: **Foundational** discovery of **CDCs and elucidation** of **mechanism** at Johns Hopkins University

Capricor **embarked** on a **10-year** journey to **define** the **MOA** of **cell** and **exosome biology**

2012: **Groundbreaking** publication in ***The Lancet*** showing CDCs **benefits**[1]

Duchenne muscular dystrophy (DMD)

Cardiovascular

2014: Capricor's uplisting to **NASDAQ**

2015: **Discovery** of the **exosomes** as the primary **MOA** of **CDCs**

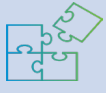
2018: **Stem Cell Reports** preclinical study in **DMD**

2019: **Positive Phase 1** results in **DMD** published in ***Journal of Neurology***

Today: **Global partnerships** established with **Nippon Shinyaku** for upwards of up to **~$1.5 billion**[2] in potential milestones and **revenue share**

2022: **Positive Phase 2** results in DMD **published** in ***The Lancet***

Today: Capricor has initiated its BLA submission for approval of deramiocel to treat DMD



Today: **StealthX™ exosome platform** focused on targeted therapeutics and vaccines

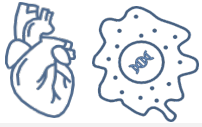
[1]Makkar, et al. *The Lancet*. 2012
[2]Subject to EU Agreement finalization

Groundbreaking Science to First-in-Class Product

Capricor Therapeutics

Pioneering Foundation
Cardiology and Cell Biology

Cardiosphere-derived cells (CDCs)



> **Initial technology:** developed at Johns Hopkins

> **Mechanism:** immunomodulatory, antifibrotic, pro-angiogenic and anti-inflammatory

> **Extensive IP portfolio:** ~125 patents & patent applications

> **Over 300 publications** by institutions worldwide[1]

Core Program
Deramiocel (CDCs)

Optimized allogeneic product being developed for commercialization

> **Lead indication:** Duchenne muscular dystrophy (DMD)

> **In-house GMP** manufacturing **facility** and expertise

> **Established commercial partnerships** in U.S., Japan and EU[2]

Research and Discovery
StealthX™ Exosome Platform

Opportunity for a new biological delivery platform

> **Targeted drug delivery** platform for RNAs, proteins and small molecules

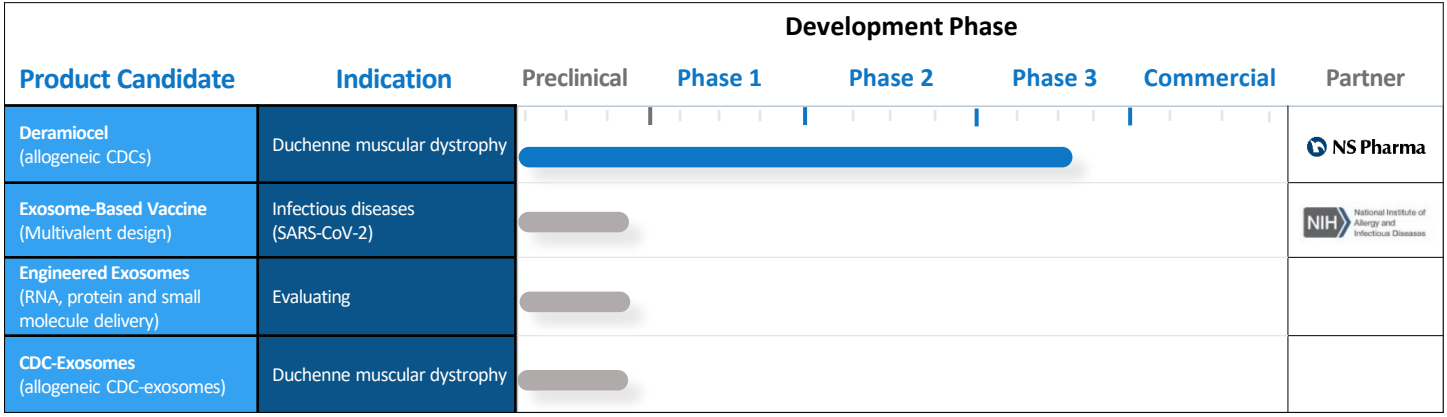
> Built on **prior** cell therapy **expertise**

> **Collaboration with NIH** for exosome-based multivalent vaccine

> **Aim to secure partnerships** to support platform advancement

[1]Based on PubMed search
[2]Subject to EU Agreement finalization

Capricor's Product Pipeline



Near-Term Value Driving Catalysts

Deramiocel for the Treatment of DMD

Key U.S. Regulatory Milestones

Pre-BLA meeting
August 2024 ✓

Initiate rolling BLA submission
October 2024 ✓

Aim to complete BLA submission
~Dec. 2024

Key U.S. Approval-Based Milestone

Potential PDUFA
~2H 2025

Potential Cash Milestones & PRV Sale

Partner milestones payable to Capricor through U.S. approval
$90 million

Partner sales-based milestones payable to Capricor post-approval U.S.
$605 million

Potential sale of PRV, if received
Est. $150 million[1]

[1]Last public sale of PRV = $158M

Proforma cash: $165 million[2]
Runway into 2027[3]
Outstanding common shares: 45.5 million

[2]Proforma cash includes 9/30/24 cash balance + Oct 2024 underwritten public offering
[3]Excludes potential milestones and PRV sale, if received

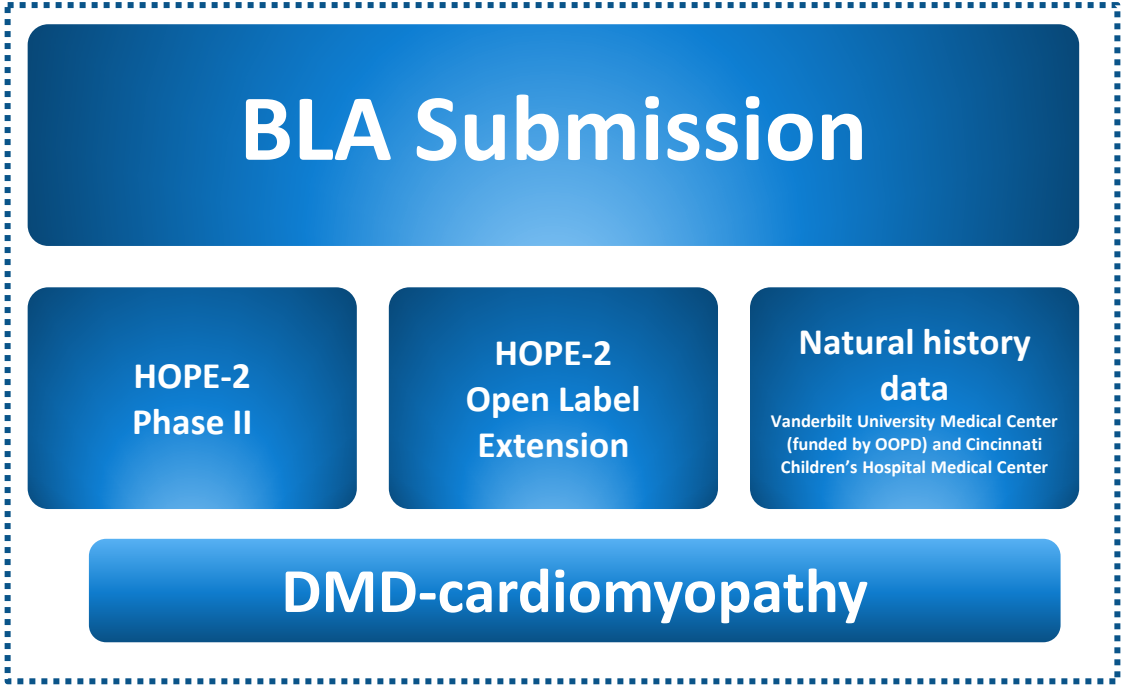
Deramiocel for the Treatment of DMD

Pathway to BLA

- Capricor intends to submit a BLA for **full approval**
- BLA filing supported with **Capricor's existing cardiac** and available **natural history data**
- Submission will seek **broad DMD-cardiomyopathy label**
- If approved, this would serve to address an **extensive population of DMD patients** (mutation agnostic)
- We believe this strategy **expedites** our path to market

Deramiocel BLA Supporting Data

To Support Potential Full Approval



Post-approval
label expansion

HOPE-3
Combined Cohorts A and B
n=~105

DMD-skeletal muscle
impairment

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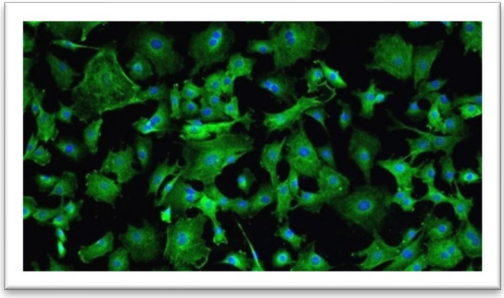
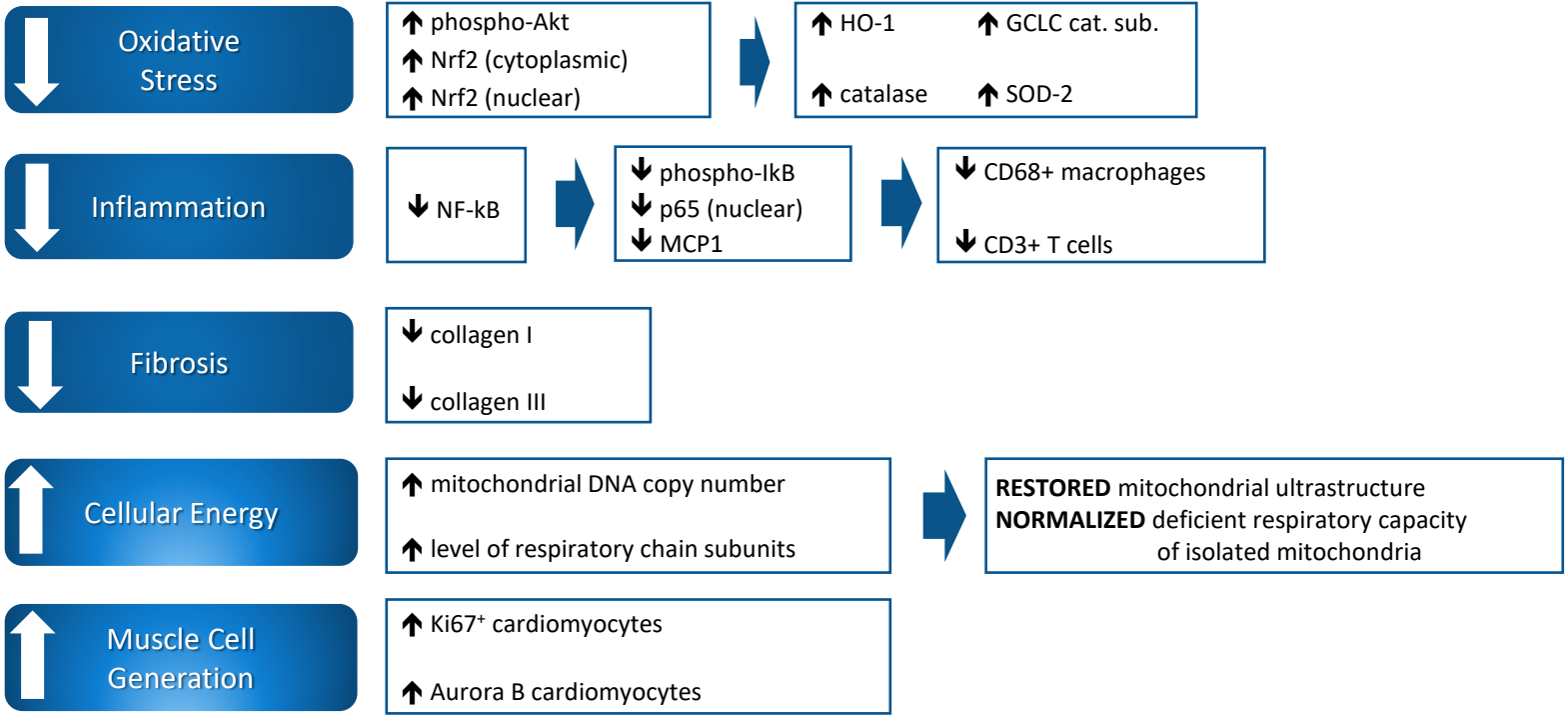
Deramiocel Overview

- **Deramiocel:** biologic consisting of allogeneic cardiosphere-derived cells (CDCs)
- **Mechanism of action:**
  - Immunomodulatory
  - Anti-inflammatory
  - Anti-fibrotic
  - Pro-angiogenic
- Investigated in over **200 patients**
- Potency assays accepted by FDA which support MOA
- **DMD regulatory designations**
  - ✓ Orphan Drug Designation (U.S. and EMA)
  - ✓ Regenerative Medicine Advanced Therapy (U.S.)
  - ✓ ATMP Designation (EMA)
  - ✓ Rare Pediatric Disease Designation (U.S.)
    - Capricor holds full rights to the PRV, if received



Deramiocel Mechanism of Action

Foundational Scientific Support



Aminzadeh, et al. *Stem Cell Reports*. 2018.

Deramiocel Manufacturing Method

Novel Process Enables a Multi-dose Allogeneic Product

Capricor receives **qualified human heart**

Explants derived from **cardiac tissue**

EDC expansion & **MCB** formulation

Cardiosphere Formation

CDC Expansion

RNA characterization

Deramiocel

Doses of **deramiocel** are **cryopreserved**

Doses of **deramiocel shipped** to infusion sites

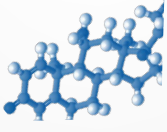
Deramiocel thawed and **administered i.v.**

150 million cells **4x per year**

Deramiocel has the Potential to Redefine the Standard of Care for DMD

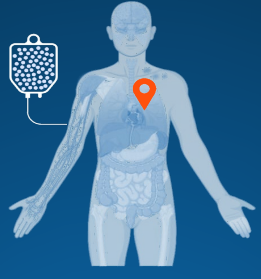
Deramiocel can be used in combination with existing therapeutics

GENE THERAPIES
Elevidys™



EXON SKIPPING THERAPIES
Viltepso® Exondys 51™, Amondys 45™, Vyondys 53™,

Deramiocel



First-in-class therapy for DMD-cardiomyopathy

CORTICOSTEROIDS
Emflaza®, Agamree®

OTHER THERAPEUTICS
Duvyzat™

DMD: Large Commercial Opportunity

Significant Unmet Need

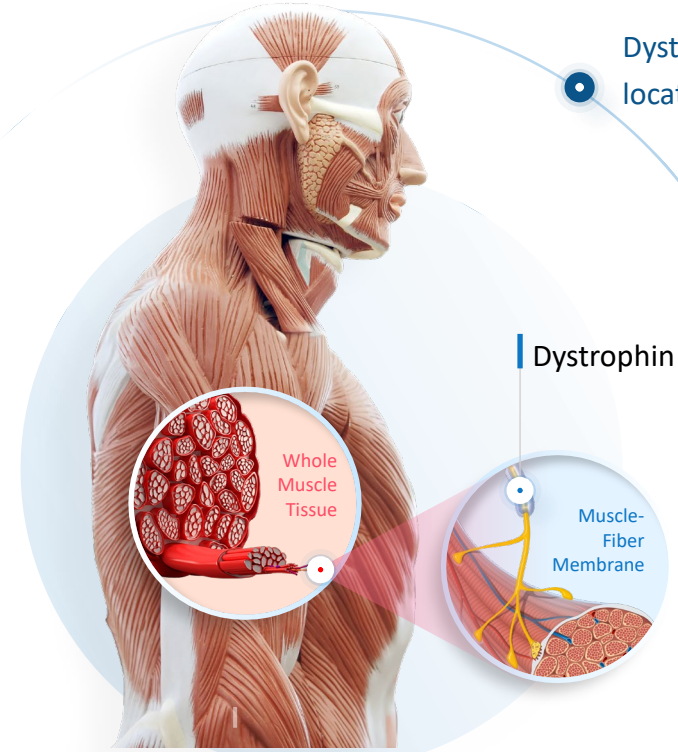
Deramiocel

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

Prevalence[1]	**~15,000-20,000** DMD patients in **United States**	**~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[2]	**$27B+** **Global market** size estimated by **2030**	
Commercial Opportunity	**Target reimbursement price** Aim to be similar or **higher** than approved **exon skipping** therapies	

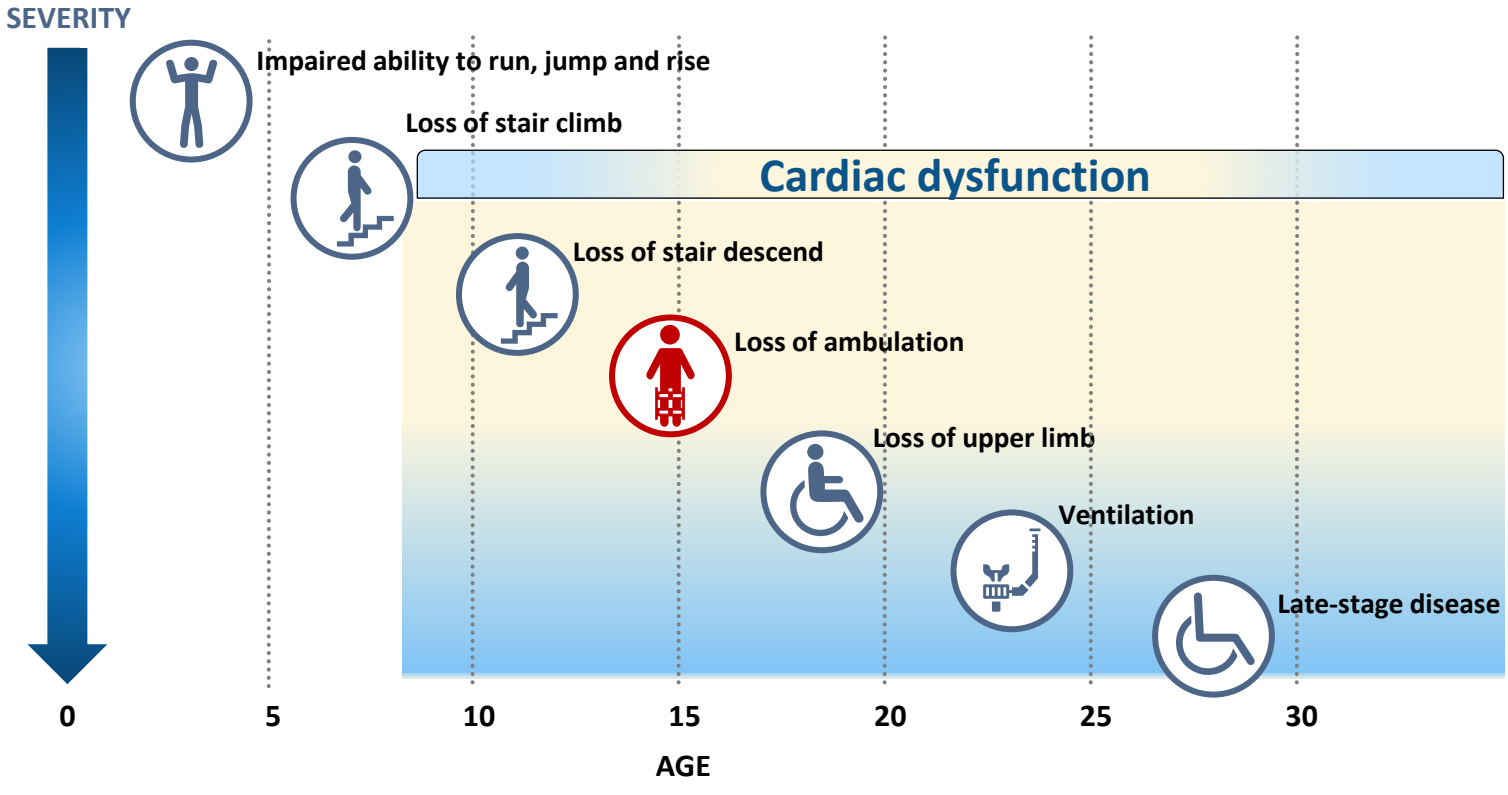
[1]DMD estimated prevalence
[2]Based on Research and Markets Report

DMD: Lack of Dystrophin Predisposes Muscle to Damage



- 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**

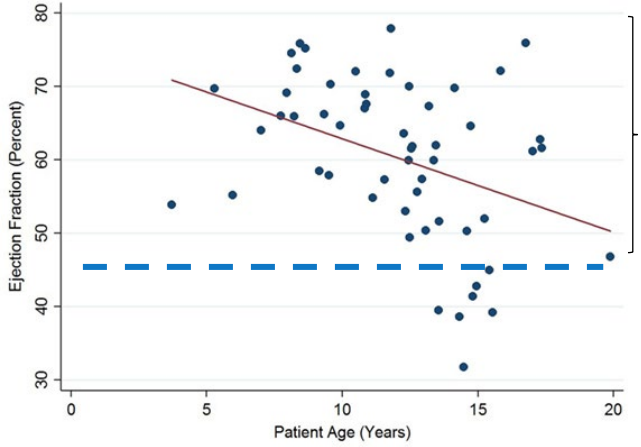
Duchenne Disease Trajectory



Cardiomyopathy in DMD

"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.



Steady course of decline in LVEF

Frontiers | Echocardiographic Image Quality Deteriorates with Age in Children and Young Adults with Duchenne Muscular Dystrophy (frontiersin.org)

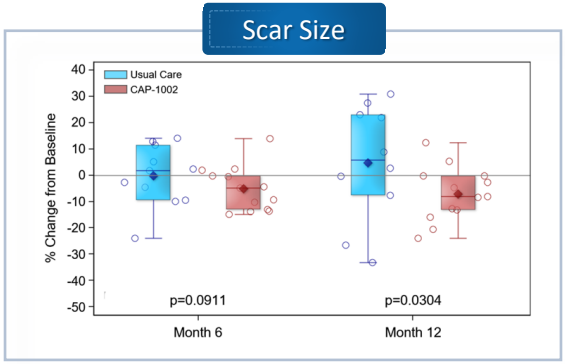
HOPE-Duchenne: Phase I/II Results

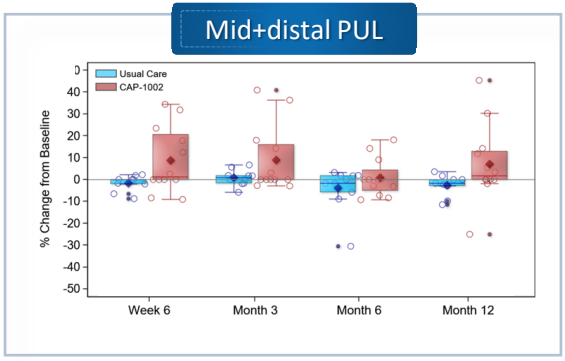
Overview: 25 patients, randomized and open-label

Dosing: 1-time, multi-vessel, intracoronary delivery of 75 million cells

RESULTS

- Reduction in cardiac scar at 6 and 12 months measured by MRI
- Improvements in cardiac function (systolic wall thickening) at 6 and 12 months
- Improvements in PUL (mid + distal)
  - Best improvement shown within the first 3 months
- Study published in the ***Journal of Neurology***





Study funded with the support of CIRM
Results published in *Neurology*, 2019.

HOPE-2: Phase 2 Overview

- **Design:** Phase 2, randomized, double-blind, placebo-controlled trial in DMD participants with reduced skeletal muscle function (9 USA sites)
- **Deramiocel dosing:** 150 million cells (i.v.) 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 Marbán, Suzanne Hendrix, Nathaniel Hogan, Rachel Ruckdeschel Smith, Michelle Eagle, Richard S Finkel, Cuixia Tian, Joanne Janas, Matthew M Harmelink, Arun S Varadhachary, Michael D Taylor, Kan N Hor, Oscar H Mayer, Erik K Henricson, Pat Furlong, Deborah D Ascheim, Siegfried Rogy, Paula Williams, Linda Marbán, 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 ($1·5\times10^8$ 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 PUL1.2 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: Phase 2 Results

Compelling Safety and Efficacy Published in *The Lancet*

Capricor Therapeutics™

Statistically and clinically **significant** changes in **PUL** v1.2
Δ2.6 points in deramiocel treated
p=0.01

Statistically and clinically **significant** improvements in **LVEF**
Δ4% in deramiocel treated
p=0.002

Favorable
safety and
tolerability profile[1]

71%

Slowing of **skeletal** muscle (PUL) progression[2]

1 Year

107%

Slowing of **cardiac** disease progression[2]

1 Year

3-Year

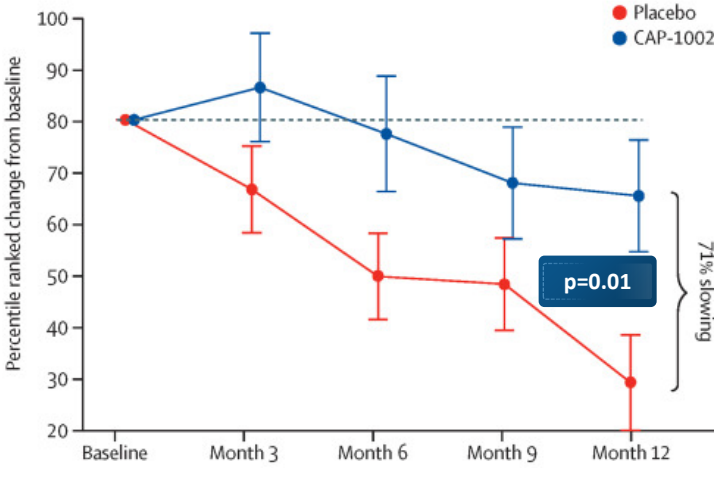
OLE data shows **sustained** effects of deramiocel treatment

1. Based on HOPE-2 and HOPE-2 OLE data
2. Based on percentile ranked change

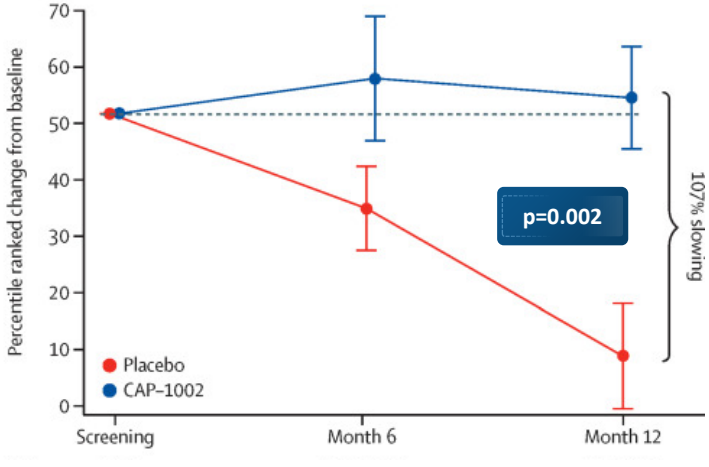
HOPE-2: Phase 2 Results

Statistically Significant Skeletal and Cardiac Improvements

Mid-level PUL v1.2



LV Ejection Fraction



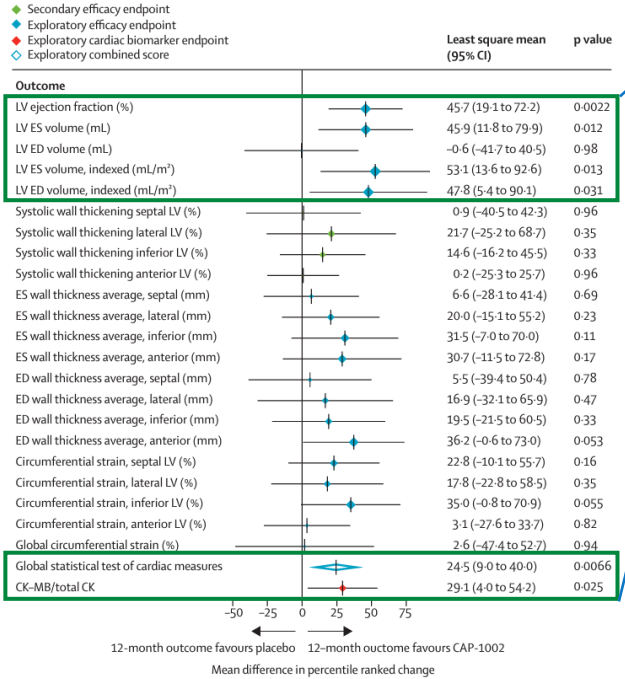
Δ=4% in LVEF by cardiac MRI

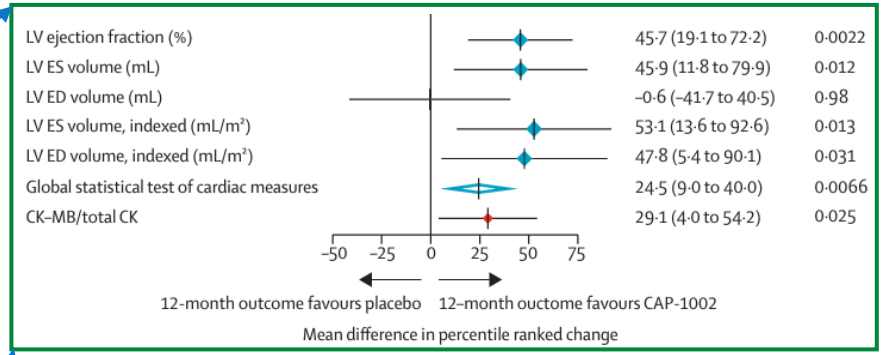
Percentile ranked change from baseline represents the percentage of all change scores smaller than the given value, where lower percentile ranked change indicates more disease progression.
Percent slowing means how much slower the disease progressed on treatment vs. placebo.
Mixed Model Repeated Measures 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.

Results published in *The Lancet*, March 2022.

HOPE-2: 21 of 22 Cardiac Measures

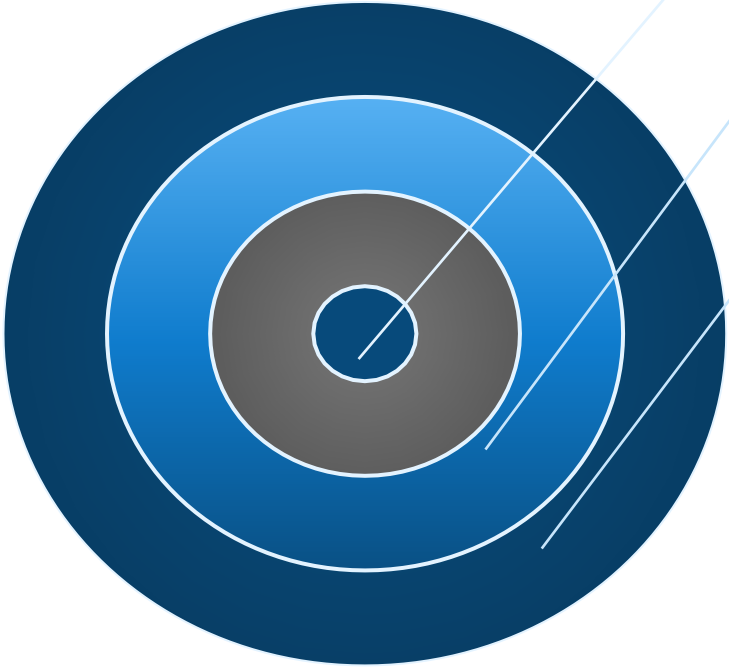
Favored Deramiocel Treatment over Placebo





Statistically significant treatment effect in critical measures of cardiac function

Potential Expansion of Deramiocel



Duchenne muscular dystrophy

Potential label expansion to include skeletal muscle myopathy (HOPE-3)

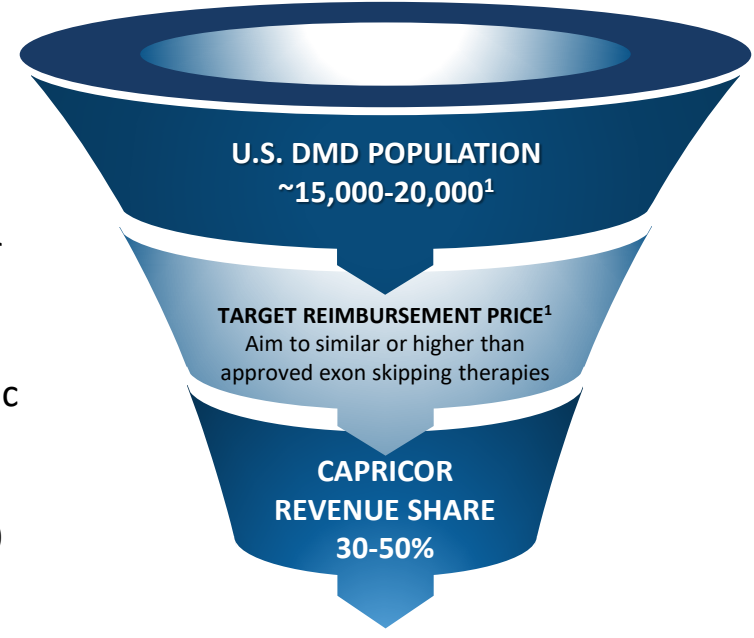
Becker muscular dystrophy

Becker cardiomyopathy develops with a similar progression to DMD-cardiomyopathy

Other orphan cardiomyopathies

Large Potential Revenue Opportunity

- **U.S. DMD prevalence[1]:** ~15,000-20,000 patients
- **Treatment regimen:** 4 doses per year
  - Potential for multi-year treatment
- **Target reimbursement price:** aim to be similar or higher than approved exon skipping therapies
- **Deramiocel opportunity:** first-in-class therapeutic option for DMD-cardiomyopathy
- **OLE commercial patients:** Potential to have ~100 patients transition to commercial product upon approval



[1]Based on internal/estimated projections and market research.
[2]Capricor revenue will less than product revenue due to revenue share under Distribution Agreement with Nippon Shinyaku

Partnership with Nippon Shinyaku

Commercialization in U.S., EU and Japan

Commercial rights: Deramiocel for the treatment of DMD

Potential milestones from agreements payable to Capricor total ~$1.5 billion[1]

U.S. deal ($40 million received to date)

- Up to $90 million in additional milestones up to and including BLA approval
- Up to $605 million in additional sales-based milestones
- Capricor revenue share (between 30-50%) which sum to be offset by amount paid for purchase of the product

Europe deal ($15 million received to date in equity investment)

- $20 million upfront payment due upon signing definitive agreement (target: Q4 2024)[1]
- Up to $715 million in additional development and sales-based milestones[1]
- Capricor to receive double-digit share of product revenue[1]

Japan deal ($12 million received to date)

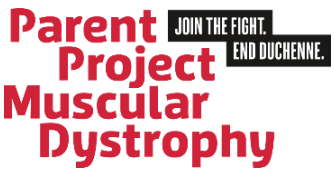
- Up to $89 million[2] in additional development and sales-based milestones
- Capricor to receive double-digit share of product revenue

Capricor responsible for product manufacturing and clinical activities necessary for potential approval

Nippon Shinyaku and NS Pharma have assembled teams to support a broad commercialization effort

[1]Subject to EU Agreement finalization
[2]Subject to foreign currency exchange rates

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)

Deramiocel Manufacturing Facility

San Diego, California

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Exosomes are Nature's Delivery Tool

Natural Drug Delivery

- ~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

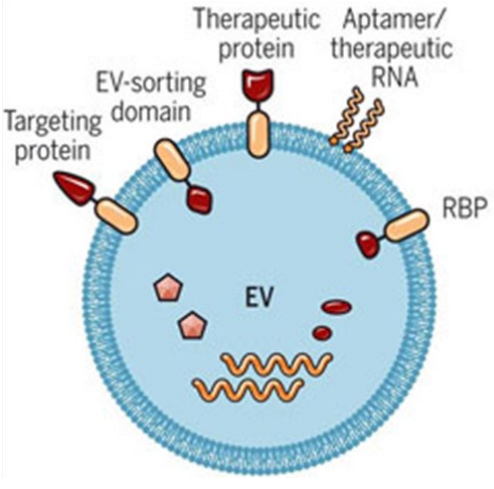
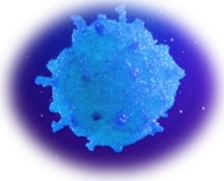
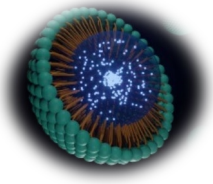


Image adapted from Wiklander, 2019

Extracellular vesicles (EV) - term for cell-derived vesicles, including exosomes

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™ Platform Overview

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

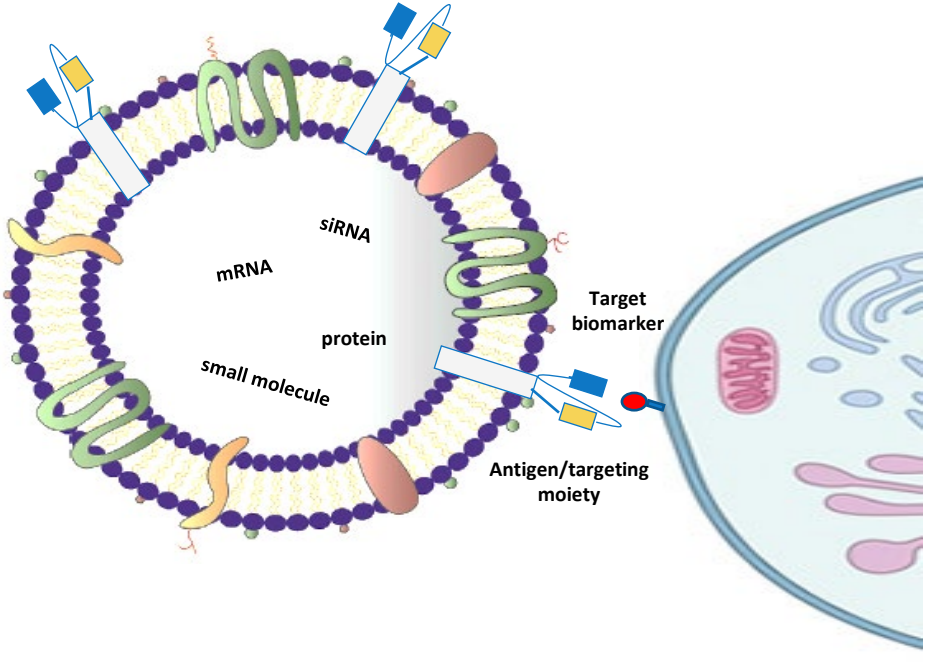
- ✓ Soluble proteins (ex. ScFvs)
- ✓ Transmembrane proteins (ex. Receptors)
- ✓ Viral antigens

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

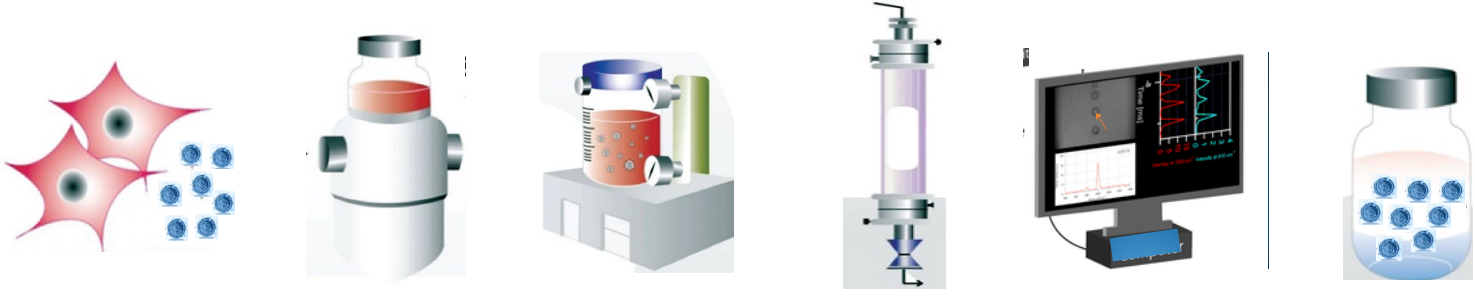
- ✓ siRNA
- ✓ miRNA
- ✓ ASOs
- ✓ Proteins
- ✓ Peptides
- ✓ Small molecules

Potential cell and tissue specific targets with targeting moieties

- ✓ Muscle
- ✓ Brain
- ✓ Lung



Exosomes: Scalable Production



Producer Cell Line → Cell Supernatant → Exosome Concentration → Exosome Purification → Exosome Characterization → Exosome Drug (DS/DP)

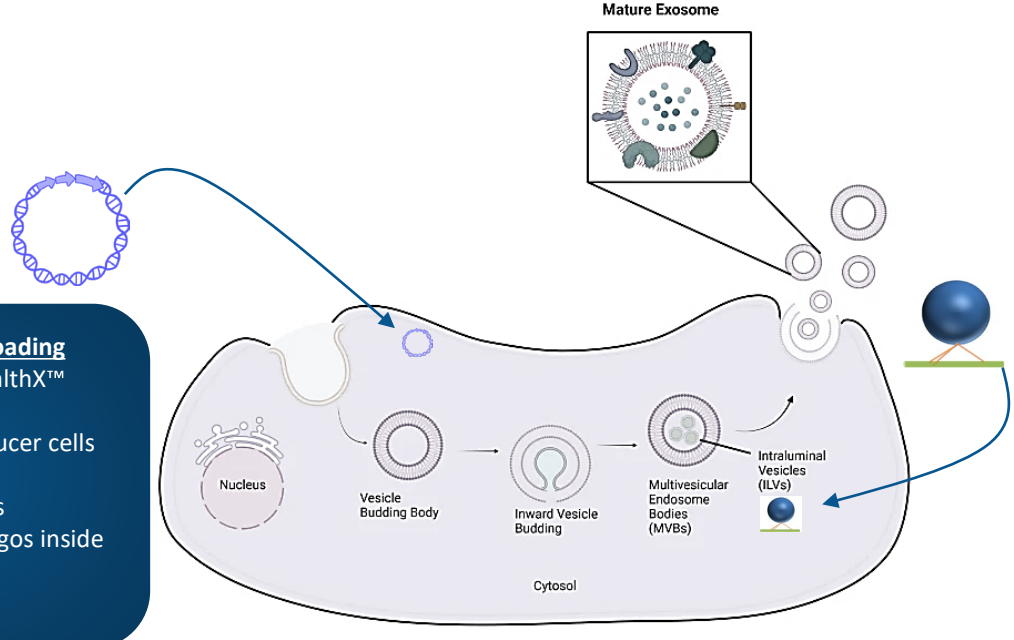
- **Capricor has developed a scalable, reproducible process for exosome purification**
  - Producer cell line is widely used for production in other applications
  - Exosome purification process developed using scalable processes
- **Capricor's exosomes have been extensively characterized using qualified assays**
  - >20 exosome DS and DP assays developed and qualified with guidance from FDA
  - Exosome yield, size, surface expression, DNA/RNA/lipid/protein 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

Image adapted from Dimik *et al.*, Drug Delivery and Translational Research (2022)

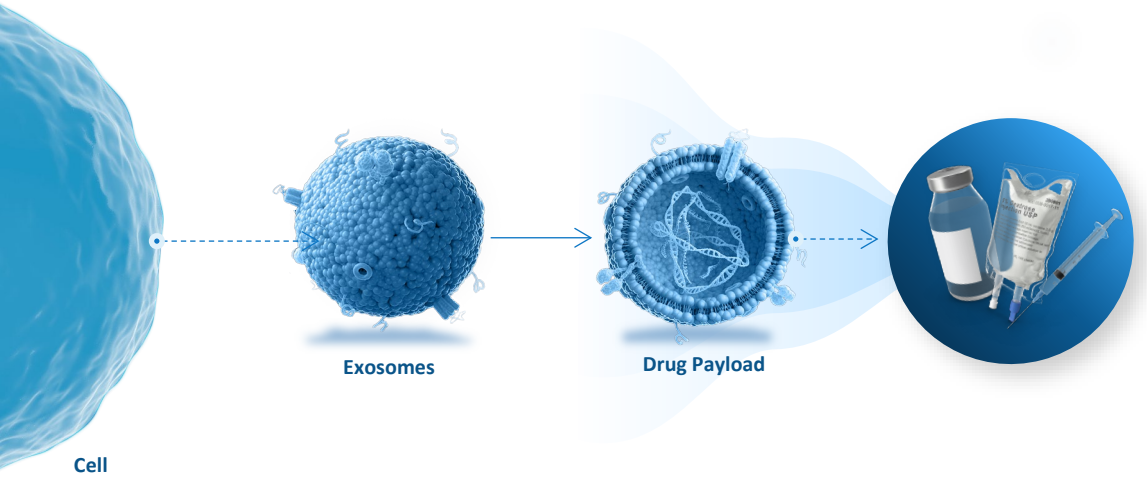
NIH Vaccine Collaboration

Exosome-Based Multivalent Vaccine

- Capricor's **StealthX™ multivalent vaccine** was **selected by Project NextGen**
  - Initiative's **aim** is to **advance** a pipeline of **innovative** vaccines to potentially provide broader and more durable protection against COVID-19
- The **National Institute of Allergy and Infectious Diseases** (NIAID) will **conduct** and **fully fund** a **Phase 1** clinical study, subject to regulatory approval
  - Capricor will **supply investigational product**
  - Anticipate study **initiation** in Q1 2025
  - Preliminary data expected in Q2 2025, subject to availability from NIAID
- 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 Goals

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

Broad Experience in Pharmaceutical & Life Sciences

Linda Marbán, Ph.D.
Chief Executive Officer
Prior experience: Excigen, Johns Hopkins University

AJ Bergmann, M.B.A
Chief Financial Officer
Prior experience: Gettleson, Witzer & O'Connor

Karen Krasney, J.D.
Executive Vice President and General Counsel
Prior experience: Biosensors International

Kristi Elliott, Ph.D.
Chief Science Officer
Prior experience: Exotech, Intrexon Corp

Jonathan Tayco
Vice President of Program Management and Business Operations
Prior experience: Celularity, Kite Pharma

Mark Awadalla
Vice President of Clinical Operations
Prior experience: Celularity, Mustang Bio, Celgene

Minghao Sun, Ph.D.
Vice President of Research & Product Development
Prior experience: Wuxi AppTec, Intrexon Corp

Yushi Feng, Ph.D.
Vice President of Regulatory Affairs
Prior experience: Codiak BioSciences, Wave Life Sciences, FDA

Table of Contents

1 Capricor Overview

2 Deramiocel DMD Program

3 StealthX™ Exosomes Platform

4 Appendix

DMD: Preclinical and Clinical Data

Trajectory of CDCs in DMD

Preclinical Data

HYPOTHESES

CDCs to treat cardiomyopathy

Left ventricular ejection fraction markedly improved vs. control

CDCs to improve exercise capacity

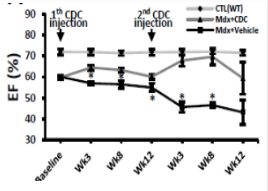
Exercise performance approximately doubled vs. control

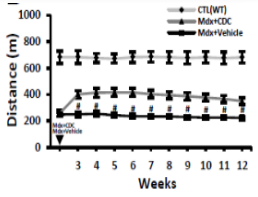
CDCs to improve skeletal muscle function

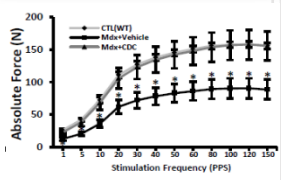
Twitch force, tetanic force, and fibrosis in soleus (slow-twitch) and extensor digitorum longus (fast-twitch) muscles significantly improved vs. control

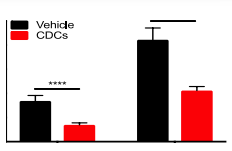
CDCs to treat diaphragm muscle

Fibrosis in the diaphragm markedly declined vs. control









Aminzadeh, et al. *Stem Cell Reports*. 2018.

HOPE-2 Open Label Extension

Study Overview

OLE Overview: 13 patients

- 6 original deramiocel patients; 7 original placebo patients; 1 patient withdrew

Objective: Continued evaluation of safety and efficacy of deramiocel

Demographics

- Current mean age: ~17 years
- All patients on stable regimen corticosteroids
- All patients were non-ambulant

Natural History Data Summary

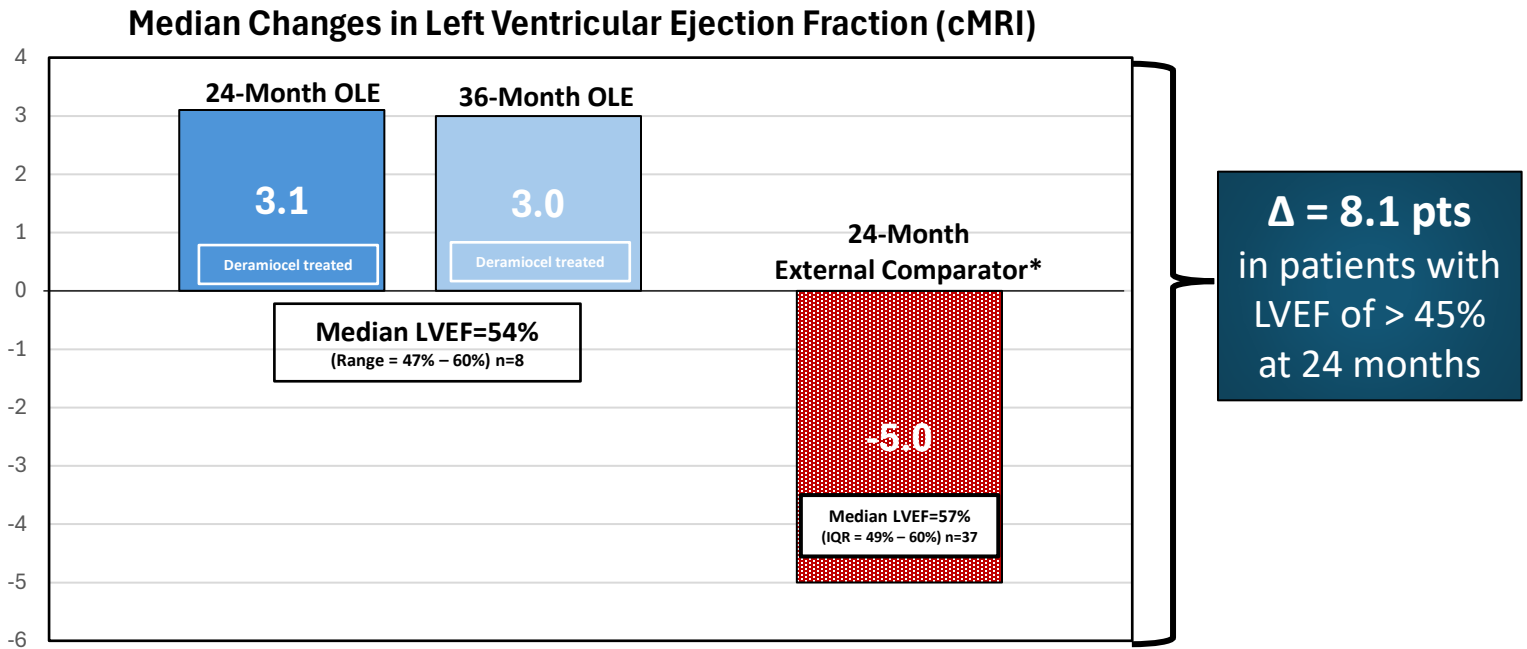
Source	Principal Investigator	Data	Status
Vanderbilt University Medical Center /FDA†	Jonathan Soslow, M.D.	Cardiac MRI	Summary Stats from publication

† "This study aims to focus on cardiomyopathy (heart muscle disease), which is the leading cause of death in Duchenne muscular dystrophy. The study will combine genetic differences with imaging and blood biomarkers to identify surrogate biomarkers that predict the risk of cardiac dysfunction in Duchenne muscular dystrophy and other related diseases. This information has the potential to improve future clinical trial efficiency in these diseases by decreasing their size and cost."

https://www.fda.gov/news-events/press-announcements/fda-awards-two-grants-natural-history-studies-rare-diseases

HOPE-2 OLE: 3-Year Cardiac Results

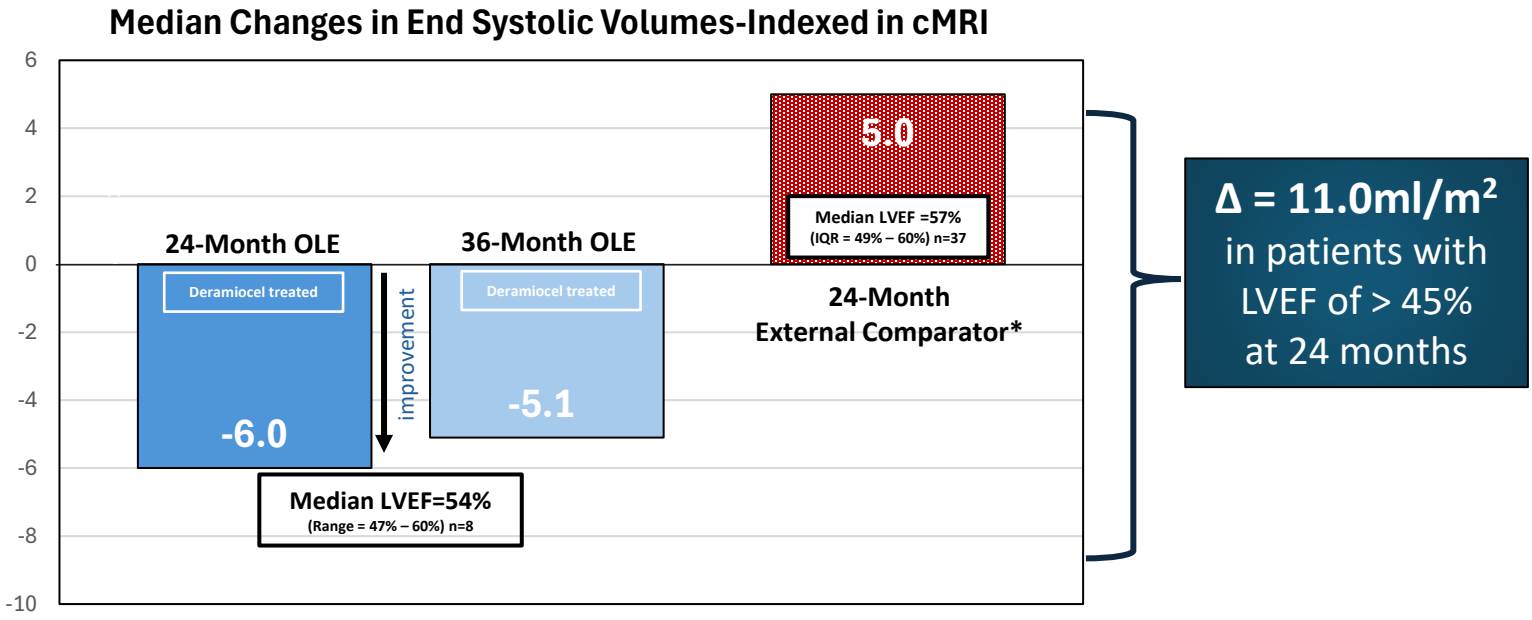
Ejection Fraction Compared to External Comparator



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

HOPE-2 OLE: 3-Year Cardiac Results

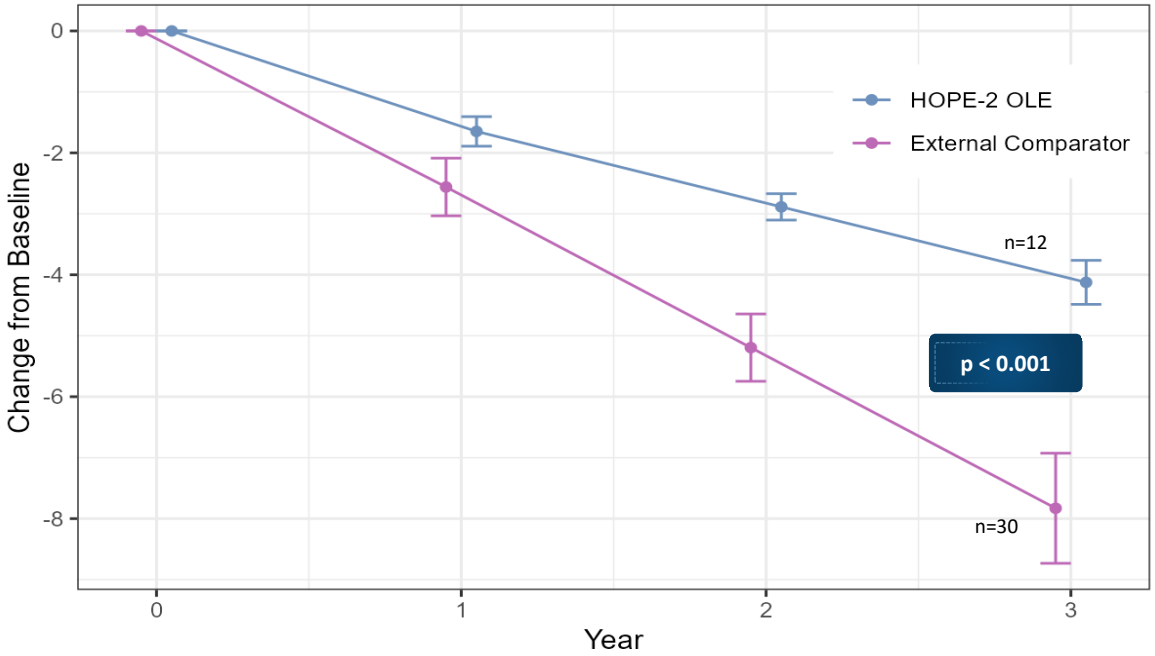
End Systolic Volumes-Indexed Compared to External Comparator



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

HOPE-2 OLE: 3-Year Skeletal Muscle Results

PUL v2.0 Total Score Compared to External Comparator



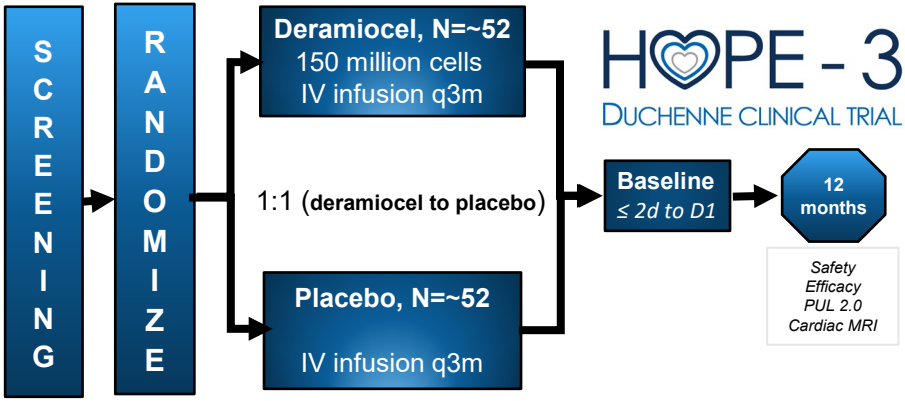
PUL v2.0 external comparator*

- Data matched by age, range of PUL v2.0 total score and entry item score
- All patients on standard of care
- Longitudinal data per patient between 2-3 years follow-up

*External DMD comparator from Cincinnati Children's Hospital

HOPE-3 Trial Overview

Current aim to support label expansion to skeletal muscle



Design & Endpoints

- Cohort A enrolled, n=61
- Cohort B enrolled, n=44; evaluating next steps
- Primary endpoint: PUL v2.0 at 12 months
- Secondary endpoints: LVEF, cardiac, QOL, etc.

Successful Interim Futility Analysis

- Completed in Q4 2023 on Cohort A

Outlook & Next Steps

- Plan to combine Cohorts A and B to serve as a post-approval study and support potential label expansion

NCT: 05126758

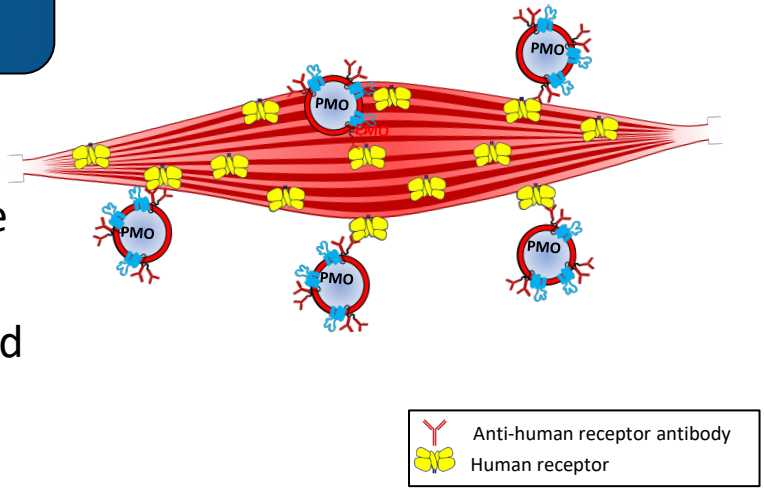
Exosomes as a Therapeutic Platform

Antisense Oligonucleotides (ASO)-Loaded Exosomes

StealthX™ Platform for DMD

StealthX-DMD (STX-DMD) engineered exosomes in development for dystrophin restoration

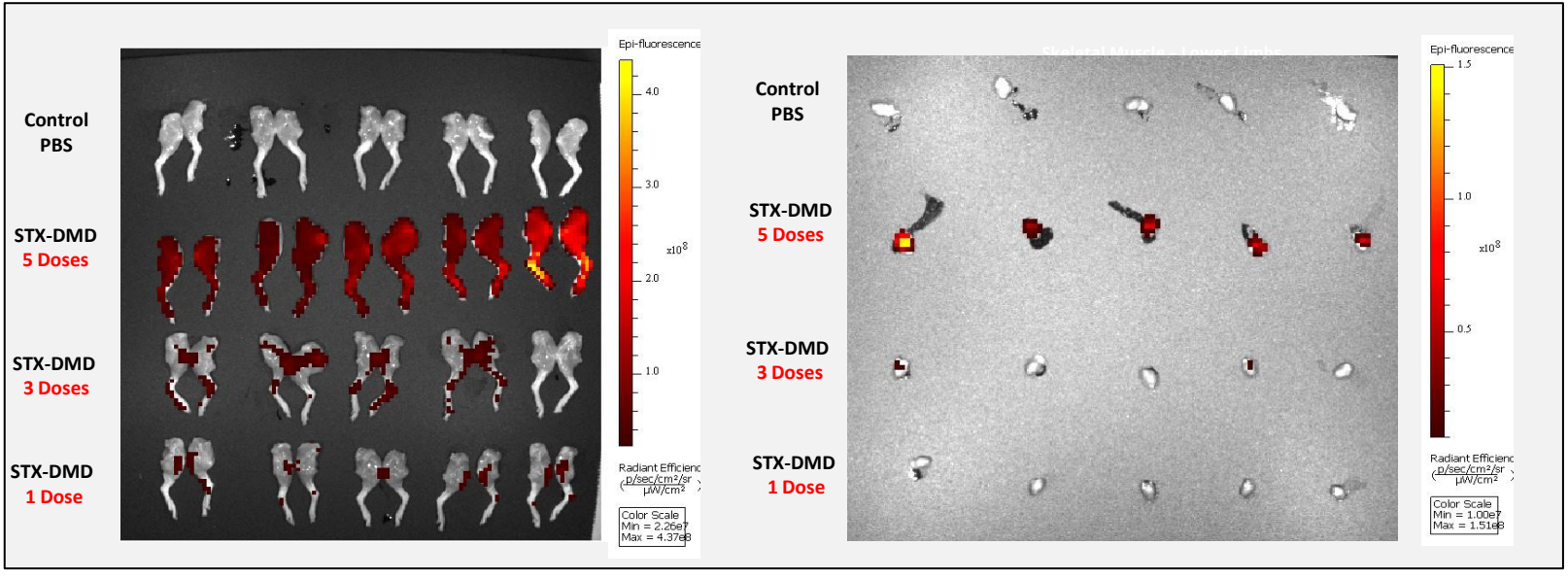
- Muscle-targeting antibodies expressed on the exosome surface
- PMOs loaded inside exosomes
- Aim: to deliver therapeutic payload to muscle and heart



*Unpublished data

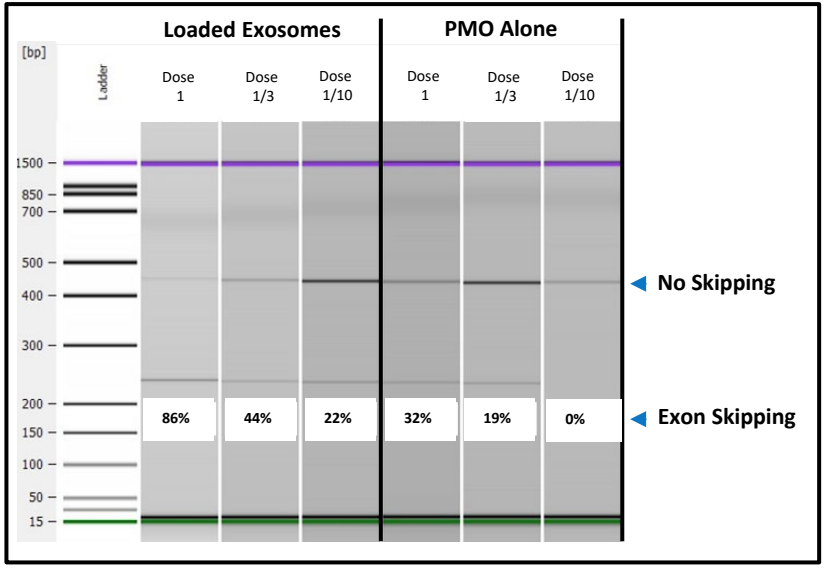
StealthX™ Targeted Delivery of Exosomes

Skeletal Muscle and Heart



- **STX-DMD engineered exosomes target skeletal muscle and heart**
- Significant STX-DMD accumulation observed after 5 doses

StealthX™ Efficient Exon Skipping



- Efficient, dose-dependent exon skipping using STX-DMD engineered exosomes
- **~2.5X greater exon skipping using PMO loaded exosomes vs. PMO alone**

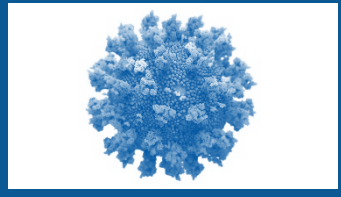
Exosomes as a Vaccine Platform

Proof of Concept Study Results

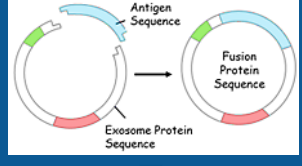
StealthX™ Rapid Vaccine Platform

Capricor Therapeutics™

Select Antigen of Interest



Create Producer Cell Line



Expand Vaccine Production

- **Rapid development cycle**
- **Versatile for multiplexing**
- **Native protein expression**
- **Potent, dose dependent response**
- **Elicits strong immunity (B and T cells)**
- **Potentially safer with no adjuvant or LNP**

StealthX™ STX-Spike & STX-Nucleocapsid

Exosome Characterisitics

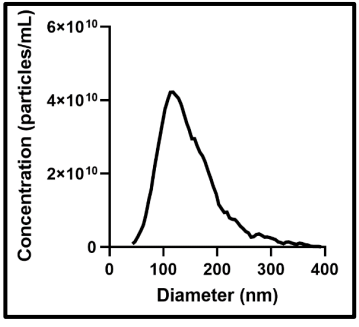
Capricor Therapeutics

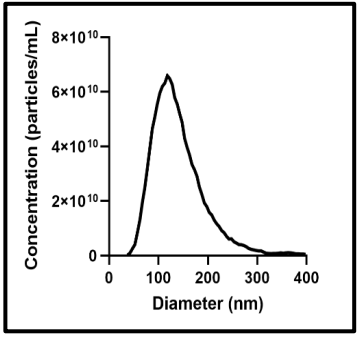
Spike Protein

STX-S (Spike) and STX-N (Nucleocapsid) are expressed at high level on the cells and exosomes

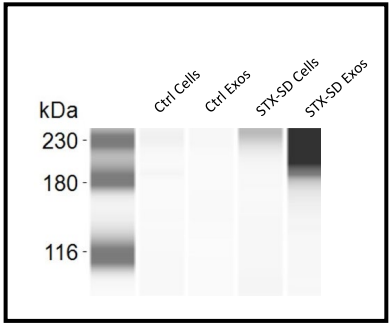
Nucleocapsid Protein

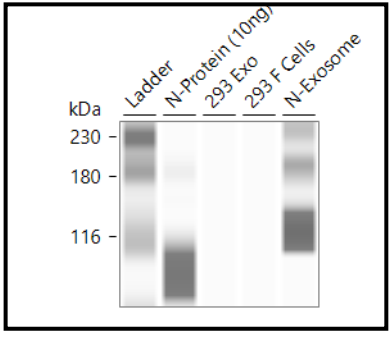
Expected Exosome Size Distribution



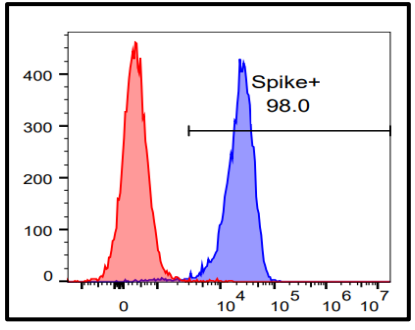


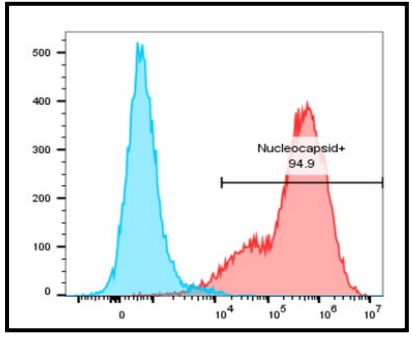
Exosome Protein Expression





Exosome Expression of S & N Proteins



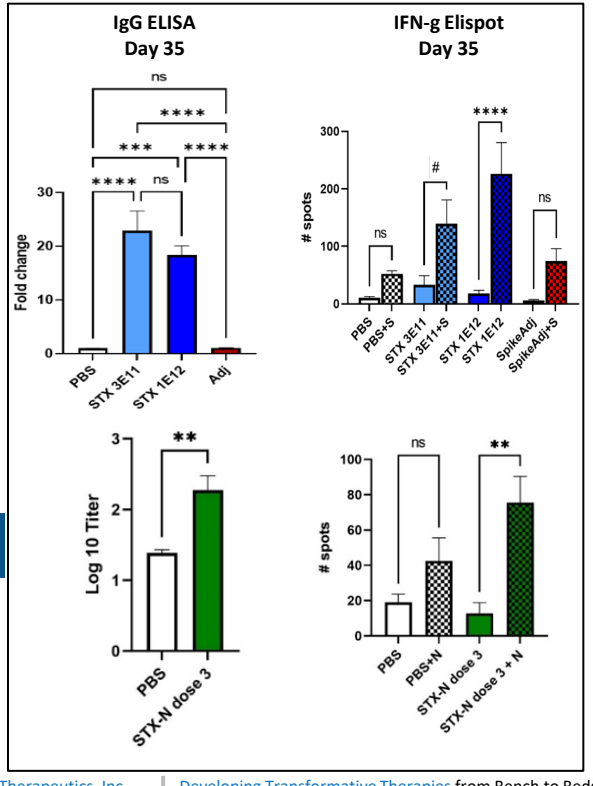


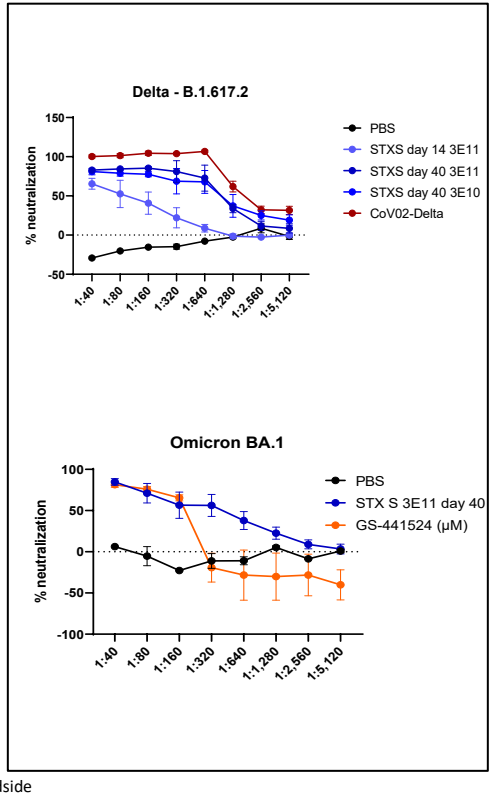
StealthX™ STX-Spike & Nucleocapsid

Antibody, T-Cell and Neutralization Responses

Spike Protein

Nucleocapsid Protein





- Potent, dose dependent antibody response induced by StealthX™ presented spike and nucleocapsid
- StealthX™ vaccine elicited a multi-functional T-cell response
- Neutralization shown in both Delta variants and omicron BA.1

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