

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 forwardlooking 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.

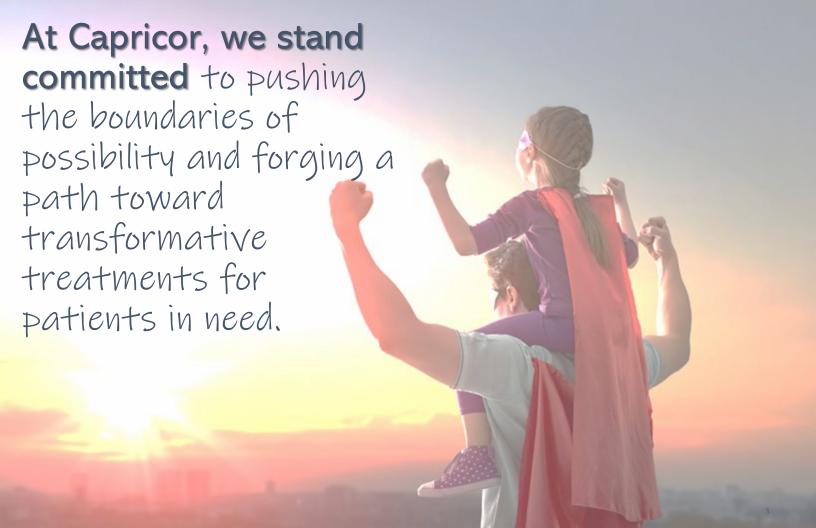


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Capricor's History & Evolution



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 benefits¹

2018: Stem Cell Reports preclinical study in DMD

Today: Global partnerships with Nippon Shinyaku for upwards of up to ~\$1.5 billion² in potential milestones and revenue share Today: Capricor's BLA accepted for review seeking approval of deramiocel to treat DMD



Duchenne muscular dystrophy (DMD)

Cardiovascular

2014: Capricor's uplisting to NASDAQ

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

2019: Positive Phase 1 results in DMD published in

Journal of
Neurology

2022: Positive Phase 2 results in DMD published in The Lancet Today: StealthX[™] exosome platform focused on targeted therapeutics and vaccines

Groundbreaking Science to First-in-Class Product Capricor



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 worldwide1

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. and Japan

Research and Discovery StealthXTM Exosome Platform

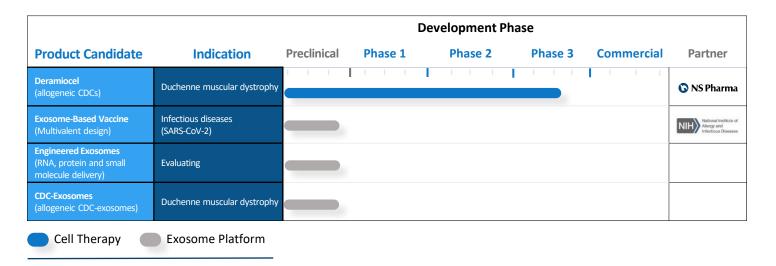
Opportunity for a new biological delivery platform



- > Targeted drug delivery platform for RNAs, proteins and small molecules
- > Built on prior cell therapy expertise
- > Collaboration with NIH for exosomebased multivalent vaccine
- > Aim to secure partnerships to support platform advancement

Capricor's Product Pipeline





Near-Term Value Driving Catalysts



Deramiocel for the Treatment of DMD









Proforma cash: \$165 million²
Runway into 2027³
Outstanding common shares: ~45.5 million

Deramiocel for the Treatment of DMD Capricon



Pathway to Approval

- ✓ FDA has accepted Capricor's BLA seeking <u>full approval</u> for deramiocel in the U.S.
- ✓ BLA supported with Capricor's existing cardiac and natural history data
- ✓ Submission seeking **broad DMD-cardiomyopathy label**
- ✓ If approved, this would serve to address an extensive population of DMD patients (mutation agnostic)

Timeline to Potential Launch



FDA Designations

- RMAT designation
- Orphan drug designation
- Rare pediatric designation

BLA filing

- BLA accepted for review (March 2025)
- BLA submitted for full approval
- Application granted priority review

Target PDUFA

- PDUFA action
 - August 31, 2025

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2 Deramiocel DMD Program

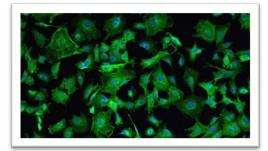
3 StealthXTM Exosomes Platform

4 Appendix

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 Europe)
 - Regenerative Medicine Advanced Therapy (U.S.)
 - ✓ ATMP Designation (Europe)
 - ✓ Rare Pediatric Disease Designation (U.S.)
 - Capricor holds full rights to the PRV, if received

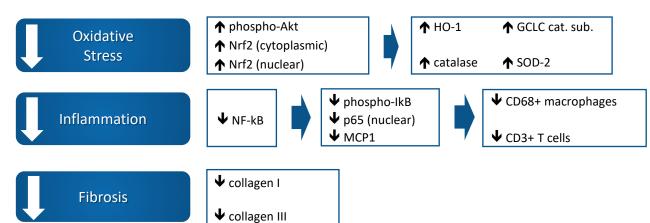




Deramiocel Mechanism of Action



Foundational Scientific Support





- ↑ mitochondrial DNA copy number
- ↑ level of respiratory chain subunits



RESTORED mitochondrial ultrastructure **NORMALIZED** deficient respiratory capacity

of isolated mitochondria

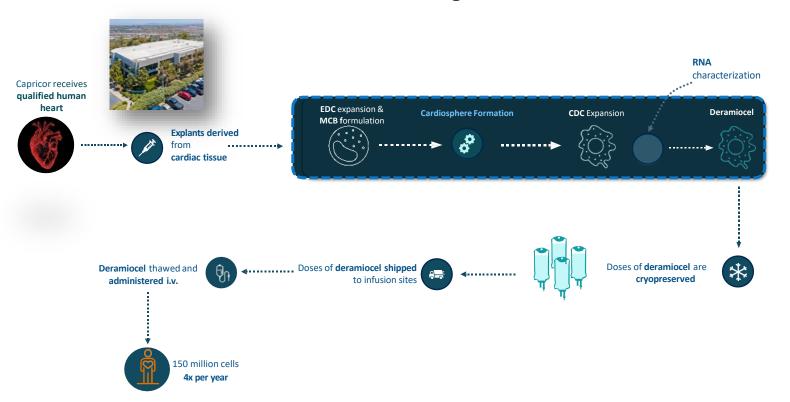


- ↑ Ki67⁺ cardiomyocytes
- ↑ Aurora B cardiomyocytes

Deramiocel Manufacturing



Novel Process Enables a Multi-dose Allogeneic Product



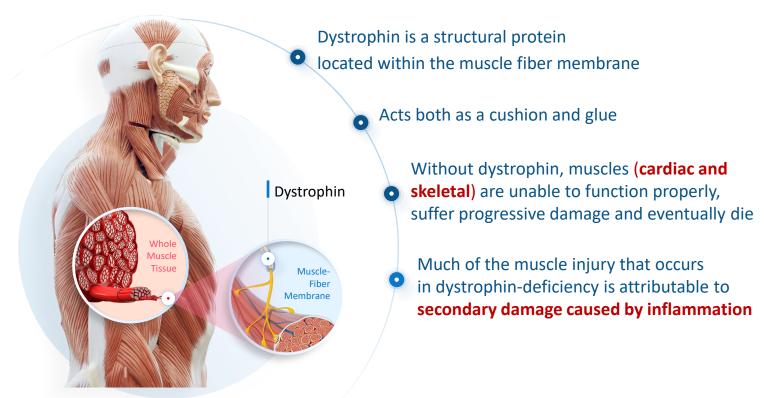
Deramiocel Manufacturing Facility

San Diego, California



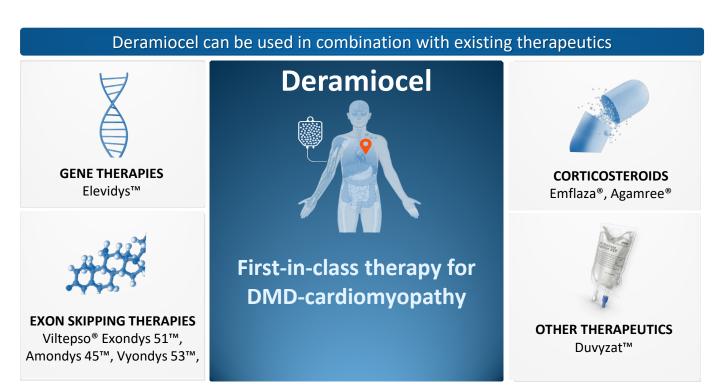
DMD: Lack of Dystrophin Predisposes

Muscle to Damage



Deramiocel has the Potential to Redefine Capricor

the Standard of Care for DMD



DMD: Large Commercial Opportunity Capricor

Deramiocel

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

Prevalence¹

~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 Size² \$27B+

Global market size estimated by 2030

Commercial **Opportunity**

Target reimbursement price

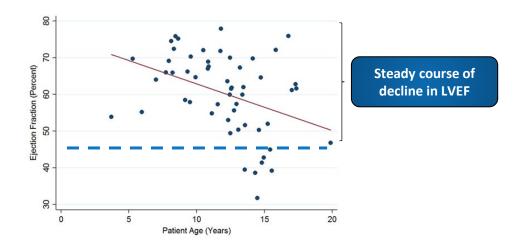
Aim to be similar or **higher** than approved **exon skipping** therapies

Cardiomyopathy in DMD



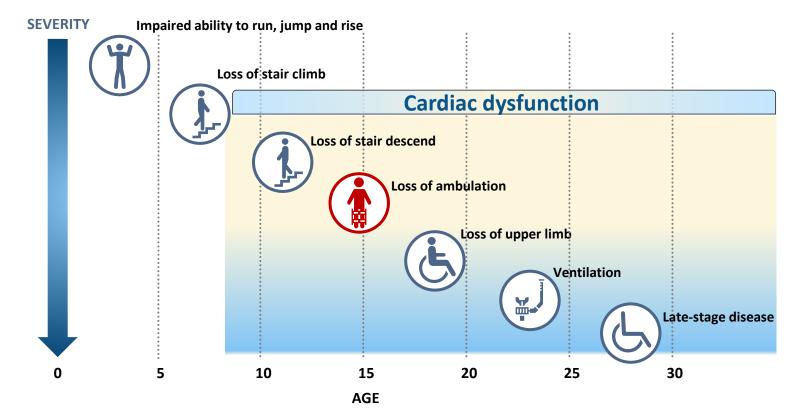
"Cardiopulmonary failure is the leading cause of mortality in DMD in the current era...Unfortunately, standard heart failure therapies are not DMD-specific and have limited efficacy....For maximal efficacy, most therapies should begin early in the disease process..."

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



Duchenne Disease Trajectory





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, placebocontrolled, phase 2 trial

Craig M McDonald, Eduardo Marbán, Suzanne Hendrix, Nathoniel Hogan, Rochel Ruckdeschel Smith, Meichelle Eagle, Richard S Finkel, Cuixia Tian, Joanne Janas, Matthew M Harmelink, Arun S Varadhachary, Michael D Taylor, Kan N Hor, Oxcar H Moyer, Erik K Henricson, Pat Furlang, Deborah D Aschim, Selfripk Roap, valud 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 (1-5×10* 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 mild-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, gow, NCT93406780.

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 placebog 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-mount change from baseline in mid-level elbow PUL.1.2 favoured CAP-1002 over placebo (percentile difference 36-2, 95% CI 12.7-57-7; 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*



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 $\Delta 4\%$ in deramiocel treated p=0.002



Favorable safety and tolerability profile¹

71%

Slowing of skeletal muscle (PUL) progression² 1 Year 107%

Slowing of cardiac disease progression²

1 Year

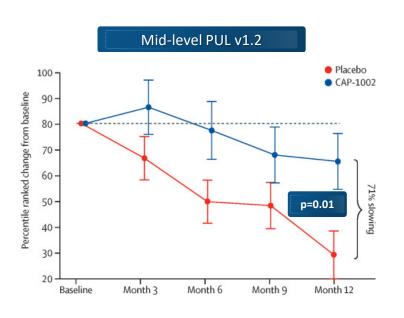
3-Year

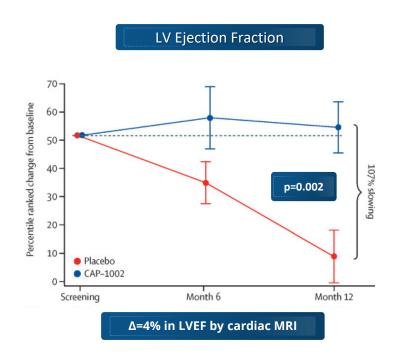
OLE data shows **sustained** effects of deramiocel treatment

HOPE-2: Phase 2 Results



Statistically Significant Skeletal and Cardiac Improvements





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.

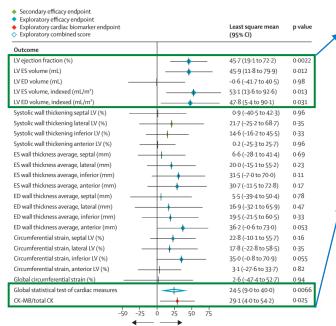
Percent slowing means how much slower the disease progressed on treatment vs. placebo.

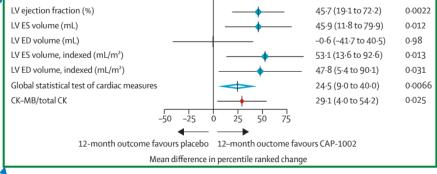
Mixed Model Repeated Measures analysis on percentile ranked change from baseline, adjusting for baseline score, treatment, visit, treatment-by-visit, PUL entry-item score at randomization, and site. Least-squares means are graphed.

HOPE-2: 21 of 22 Cardiac Measures



Favored Deramiocel Treatment over Placebo





Statistically significant treatment effect in critical measures of cardiac function

12-month outcome favours placebo 12-month outcome favours CAP-1002

Mean difference in percentile ranked change

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

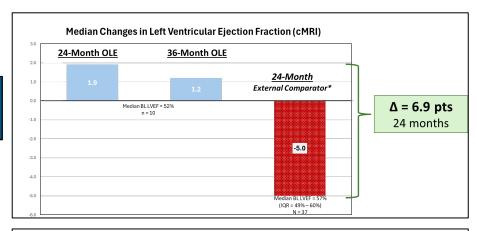
† "This study aims to focus on cardiomyopathy (heart muscle disease), which is the leading cause of death in Duchenne muscular dystrophy. The study will combine genetic differences with imaging and blood biomarkers to identify surrogate biomarkers that predict the risk of cardiac dysfunction in Duchenne 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

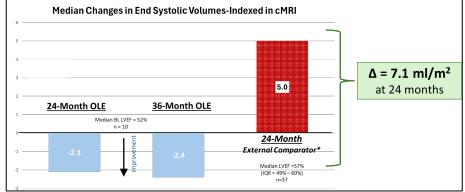
HOPE-2 OLE: 3-Year Cardiac Results Capricor



Ejection fraction compared to external comparator



End systolic volumesindexed compared to external comparator



Large Potential Revenue Opportunity Capricor

- U.S. DMD prevalence¹: ~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



¹Based on internal/estimated projections and market research.

Partnership with Nippon Shinyaku



NS Pharma



Partnership with Nippon Shinyaku



Commercial rights: Deramiocel for the treatment of DMD

- Potential milestones from agreements payable to Capricor total ~\$1.5 billion¹
- Capricor responsible for product manufacturing and clinical activities necessary for potential approvals in territories
- Nippon Shinyaku and NS Pharma have assembled teams to support broad commercialization efforts



United States Distribution

- Up to \$695M in potential milestones (\$10M received in Q1 2025)
- Capricor to receive (between 30-50%) of product revenue, offset by amount paid for purchase of the product



Japan Distribution

- Up to \$89M² in potential milestones
- Capricor to receive double-digit share of product revenue

Europe Distribution (based on term sheet)¹

- Up to \$735M in potential milestones¹
- Capricor to receive double-digit share of product revenue¹

30

Key Duchenne Advocacy Relationships





Parent CONTRESIONS.
Project Muscular
Dystrophy







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.

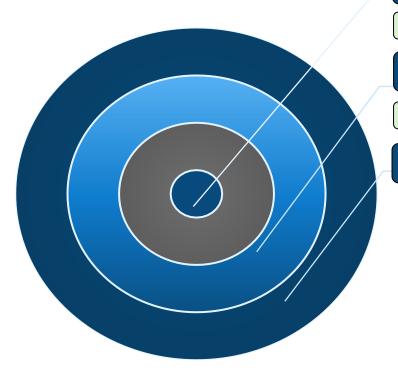
Texas Children's Hospital (USA)

Chet Villa, M.D.

Cincinnati Children's Hospital Medical Center (USA)

Potential Expansion of Deramiocel





Duchenne muscular dystrophy

Potential label expansion to include skeletal muscle myopathy

Becker muscular dystrophy

Becker cardiomyopathy has similar progression to DMD-cardiomyopathy

Other orphan cardiomyopathies

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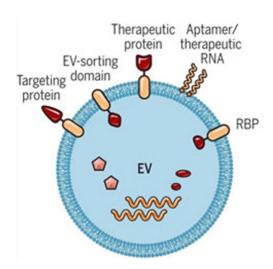
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Exosomes are Nature's Delivery Tool

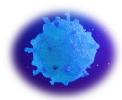


Natural Drug Delivery

- ~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



Potential Benefits: Exosomes vs. LNPs Capricor





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

StealthXTM Platform Overview



StealthXTM technology allows Capricor to present diversified proteins *outside* of exosomes

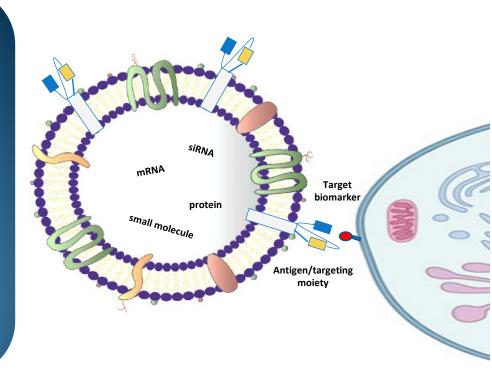
- ✓ Soluble proteins (ex. ScFvs)
- ✓ Transmembrane proteins (ex. Receptors)
- ✓ Viral antigens

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



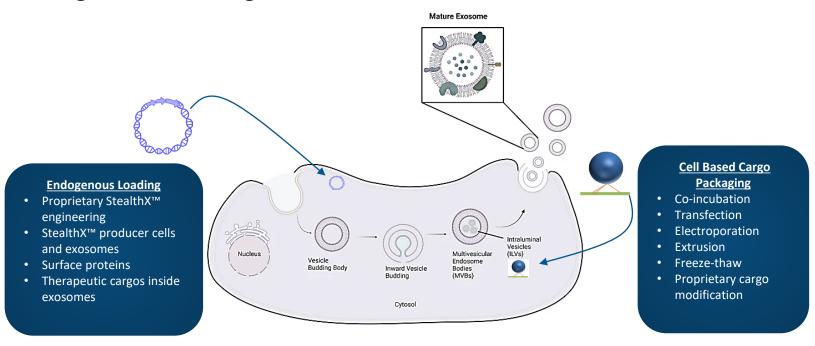


- Capricor has developed a scalable, reproducible process for exosome purification
 - Producer cell line is widely used for production in other applications
 - Exosome purification process developed using scalable processes
- Capricor's exosomes have been extensively characterized using qualified assays
 - >20 exosome 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



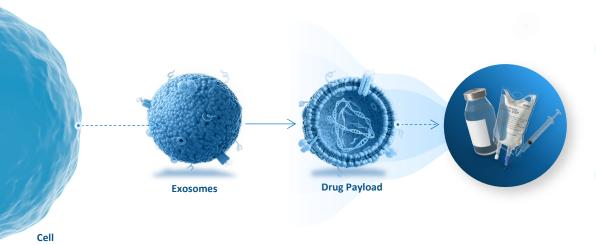
Endogenous and Exogenous Methods



StealthXTM 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



Experienced Leadership Team

Capricor

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: Biosepsors International



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



Mark Awadalla
Chief Development Officer
Prior experience: Celularity, Mustang Bio, Celgene



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

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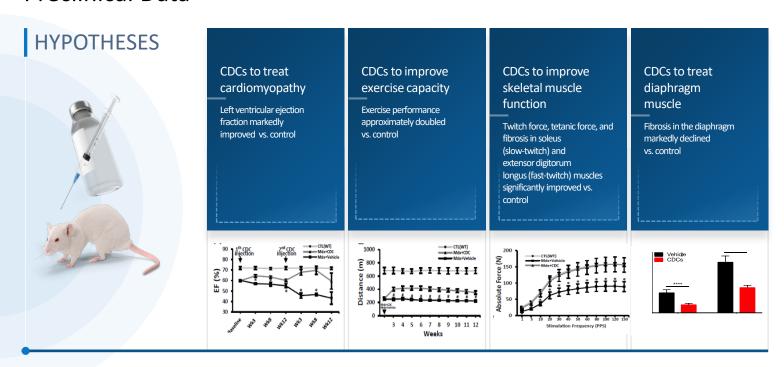


DMD: Preclinical and Clinical Data

Trajectory of CDCs in DMD



Preclinical Data



HOPE-Duchenne: Phase I/II Results

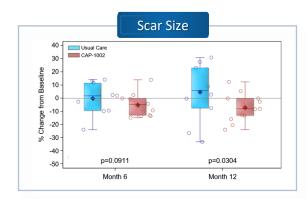


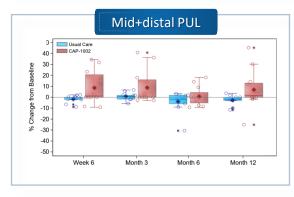
Overview: 25 patients, randomized and open-label

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

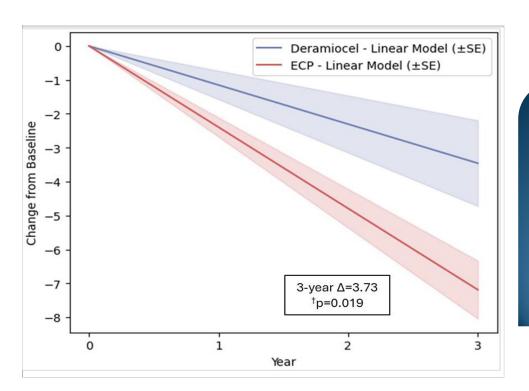




HOPE-2 OLE: 3-Year Skeletal Muscle Results



PUL v2.0 Total Score Compared to External Comparator



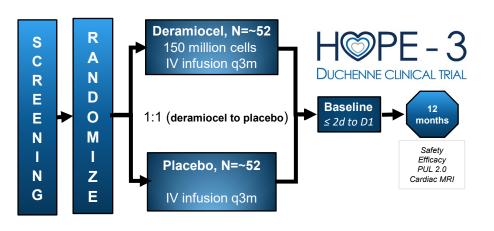
PUL v2.0 external comparator*

- 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

HOPE-3 Trial Overview



Current aim to support label expansion to skeletal muscle



Design & Endpoints

- Cohorts A and B: n=~105
- Evaluating next steps for potential global expansion
- Primary endpoint: PUL v2.0 at 12 months
- Secondary endpoints: LVEF, cardiac, QOL, etc.

Successful 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



Exosomes as a Therapeutic Platform

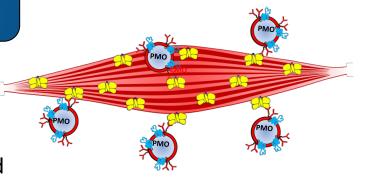
Antisense Oligonucleotides (ASO)-Loaded Exosomes

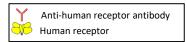
StealthXTM Platform for DMD



StealthXTM-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

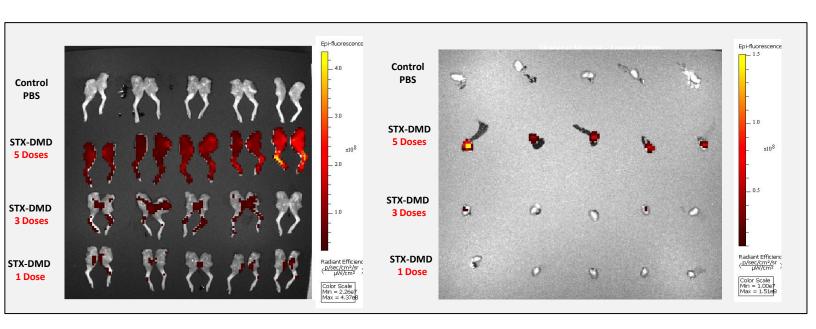




StealthXTM Targeted Delivery of Exosomes

Skeletal Muscle and Heart

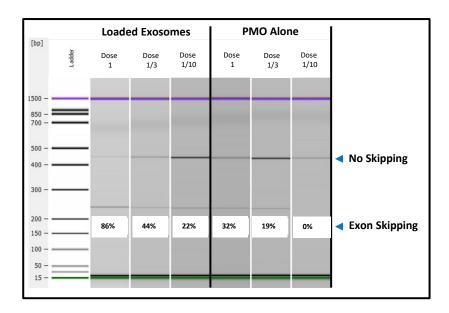




- STX-DMD engineered exosomes target skeletal muscle and heart
- Significant STX-DMD accumulation observed after 5 doses

StealthXTM 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

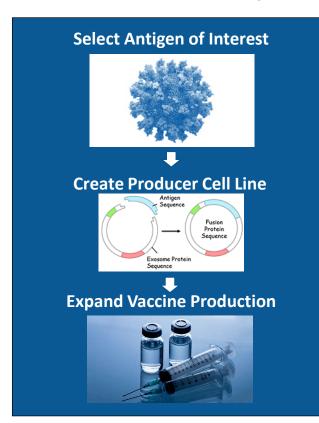


Exosomes as a Vaccine Platform

Proof of Concept Study Results

StealthXTM Rapid Vaccine Platform



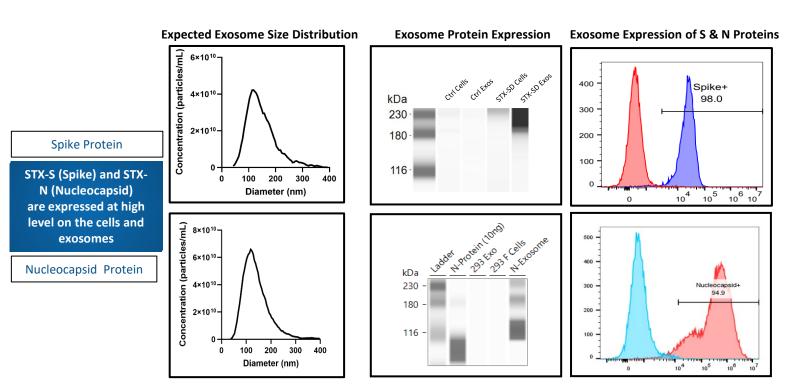


- Rapid development cycle
- Versatile for multiplexing
- Native protein expression
- Potent, dose dependent response
- Elicits strong immunity (B and T cells)
- Potentially safer with no adjuvant or LNP

StealthXTM STX-Spike & STX-Nucleocapsid

Exosome Characterisitics

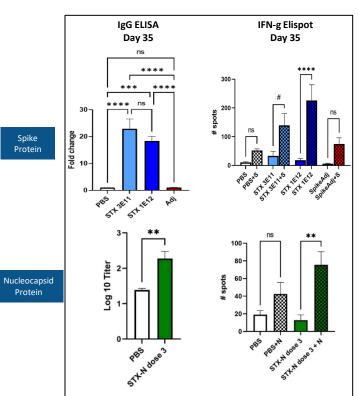


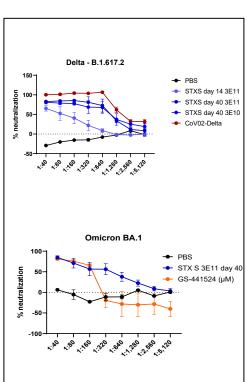


StealthXTM STX-Spike & Nucleocapsid _{Capricor}



Antibody, T-Cell and Neutralization Responses





- 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

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
 - Study initiation activities underway
- If NIAID finds our vaccine meets its criteria for safety and efficacy, they may consider our program for a funded Phase 2

