



**HOPE-2 Data Update
Conference Call
July 15, 2019**

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; plans regarding current and future collaborative activities and the ownership of commercial rights; scope, duration, validity and enforceability of intellectual property rights; future royalty streams, 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, 2018 as filed with the Securities and Exchange Commission on March 29, 2019, and as amended by its Amendment No. 1 to Annual Report on Form 10-K/A filed with the Securities and Exchange Commission on April 1, 2019 and in its Quarterly Report on Form 10-Q as filed with the Securities and Exchange Commission on May 14, 2019. 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.

CAP-1002 is an Investigational New Drug and is not approved for any indications. Capricor's exosomes technology, including CAP-2003, has not yet been approved for clinical investigation.

Call Participants

- **Linda Marban, Ph.D.** – Chief Executive Officer, Capricor Therapeutics, Inc.
- **Craig McDonald, M.D.**, is professor and chair of the Department of Physical Medicine and Rehabilitation and Director of the Neuromuscular Disease Clinics at the University of California, Davis. Dr. McDonald is an internationally recognized expert in the clinical management and rehabilitation of neuromuscular diseases including DMD. He is the national PI of the Capricor HOPE-2 Trial.
- **Richard G. Holcomb, Ph.D.**, is a consulting biostatistician for Capricor located in Minneapolis, Minnesota and has worked on clinical trials of FDA regulated products for over 150 device and drug companies in the past 40 years.
- **AJ Bergmann**, Chief Financial Officer, Capricor Therapeutics, Inc.

Our Mission

Capricor is focused on the discovery, development and commercialization of innovative cell and exosome-based therapies for patients with immune-inflammatory rare diseases with a focus on Duchenne muscular dystrophy.



Capricor's Product Pipeline

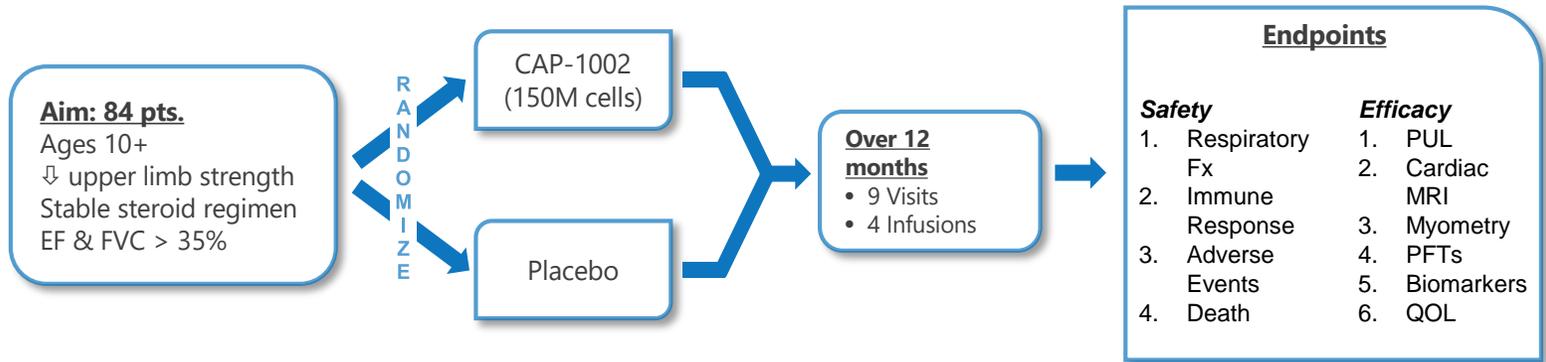
Candidate	Indication	Development Phase				Status
		Preclinical	Phase I	Phase II	Phase III	
CAP-1002 (allogeneic CDCs)	Duchenne Muscular Dystrophy					<ul style="list-style-type: none"> HOPE-2 trial Improvement in skeletal and cardiac muscle function seen in randomized clinical trial in advanced DMD (HOPE-Duchenne) Orphan Drug, Rare Pediatric Disease and RMAT Designations
CAP-2003 (CDC-exosomes)	Inflammatory / Fibrotic Disorders					<ul style="list-style-type: none"> Exploring potential indications

CAP-1002 is an Investigational New Drug and is not approved for any indications.
 CAP-2003, Capricor's exosomes technology, has not yet been approved for clinical investigation.

CDCs = cardiosphere-derived cells

CAP-1002: Duchenne Muscular Dystrophy Program

HOPE-2 Clinical Trial Design

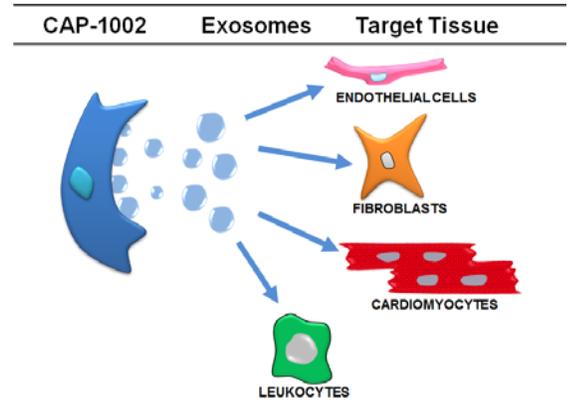


- **Design:** Phase II, randomized, double-blind, placebo-controlled trial
- **Objective:** Evaluate safety and efficacy of intravenous (IV) CAP-1002 administered every three months in participants with DMD and reduced muscle function
- **Sites:** approximately 9 sites (USA)

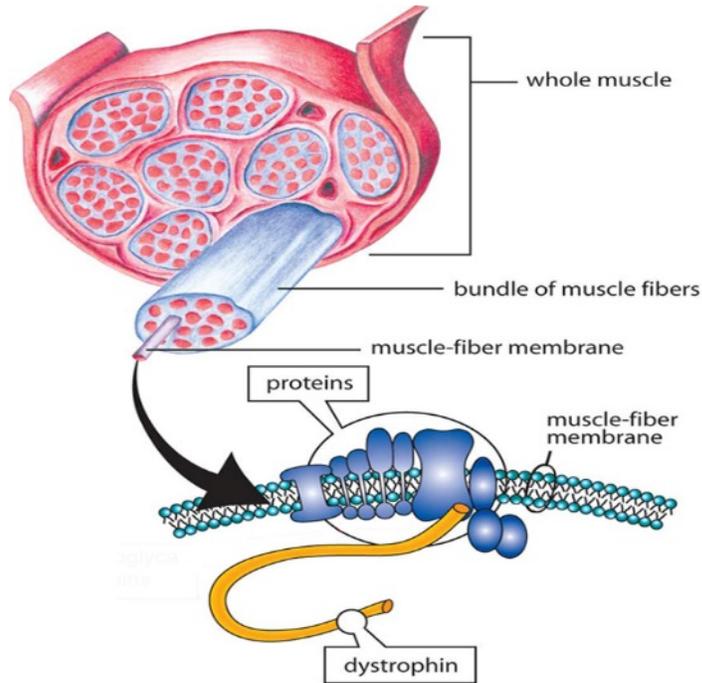
Capricor's CAP-1002 Technology

– CAP-1002 is a biologic consisting of allogeneic cardiosphere-derived cells (CDCs)

- Manufactured from donated heart muscle
- **Does not act by “stemness” – the cells do not engraft into host tissue**
- MOA: cells secrete exosomes
 - Contain non-coding RNAs and proteins
 - Internalized by target cells
 - Stimulate diverse and lasting changes in cellular behavior
- CAP-1002 has been investigated in several clinical trials and more than 150 human subjects

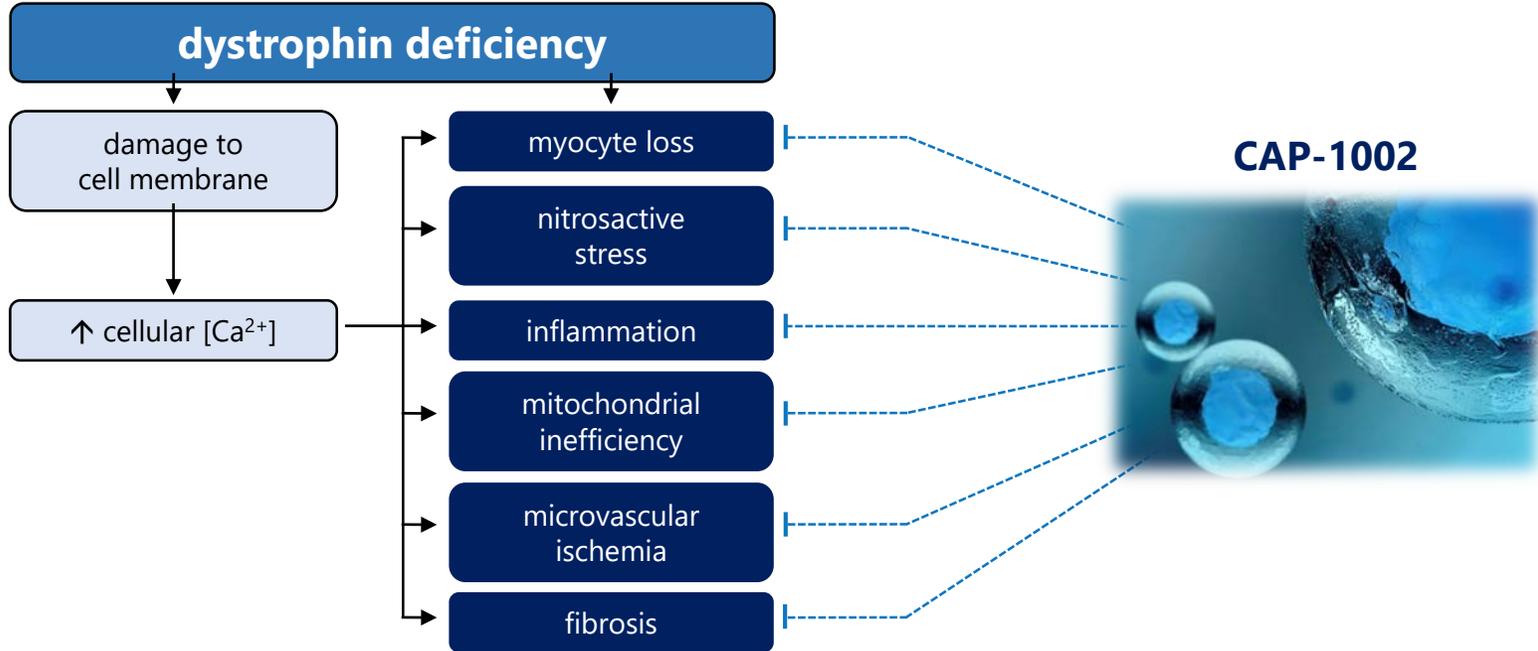


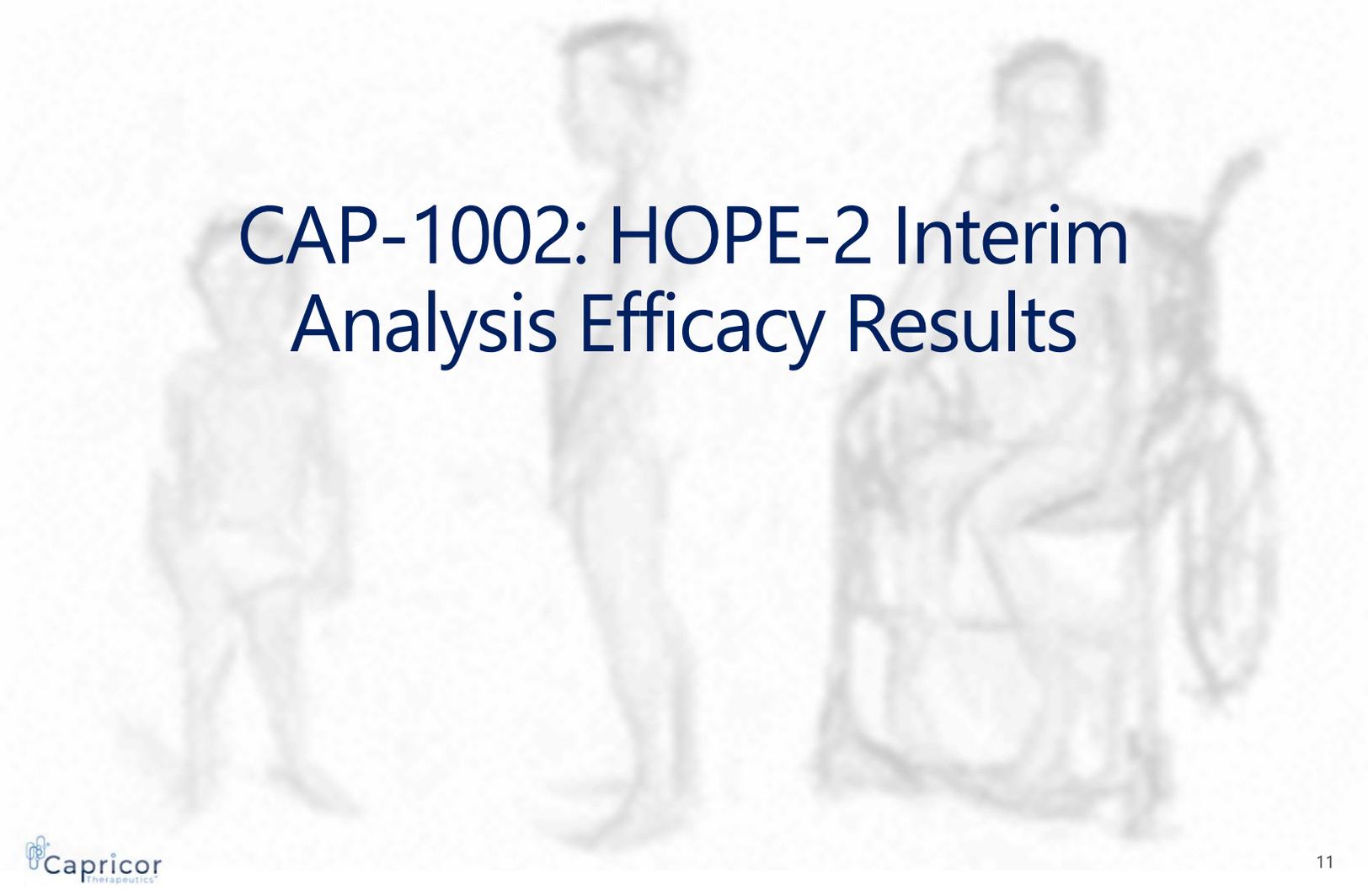
Lack of Dystrophin Predisposes Muscle to Damage



- Dystrophin is a structural protein in muscle
- Acts both as a cushion and a kind of glue
- Without dystrophin, muscles 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.**

CAP-1002 Targets Multiple Disease Processes in DMD





CAP-1002: HOPE-2 Interim Analysis Efficacy Results

HOPE-2 Interim Analysis Breakdown

- Intent-to-Treat population = 20 subjects
- Safety population = 20 subjects
- Per Protocol population = 17 subjects
 - 3 subjects were excluded due to missed infusions
- Safety population data available for interim analysis by visit:

Visit	Placebo	CAP-1002	Total
Day 1	12	8	20
Month 3	10	7	17
Month 6	7	6	13
Month 9	4	2	6
Month 12	2	1	3

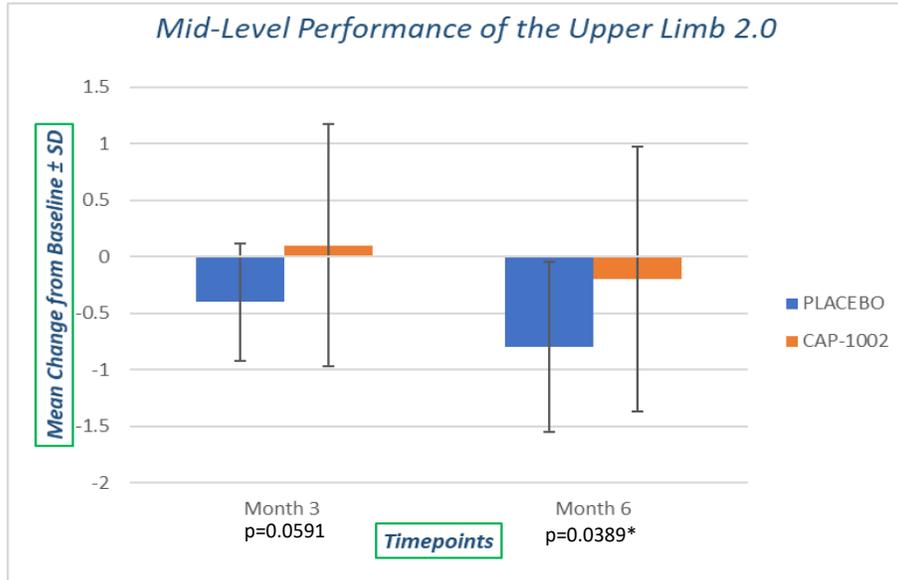
HOPE-2 Patient Demographics

- **CAP-1002 and Placebo groups had similar demographics and baseline characteristics**
 - Mean (SD) age = 14.3 (3.11 years)
 - Mean (SD) BMI = 22.6 (4.88) kg/m²
 - PUL entry scores were either in the 2-3 range or the 4-5 range (stratified)
- **DMD medical history and disease progression similar between the groups**
 - All patients were on steroids (stable regimen)
 - 80% were non-ambulant
 - No history of spinal surgery or symptomatic heart failure

HOPE-2 Efficacy Endpoints – Interim Analysis

- **Skeletal**
 - PUL 2.0 and PUL 1.2
 - Grip Strength
 - Tip to tip pinch strength
 - Additional skeletal measures
- **Pulmonary**
 - Peak Expiratory Flow
 - Inspiratory Flow Reserve
 - Forced Vital Capacity
- **Cardiac**
 - Myocardium mass
 - Systolic wall thickening
 - Additional cardiac measures
- **Quality of life assessments**

Mid-Level Performance of the Upper Limb (PUL 2.0)



Legend:

Colored boxes heights either positive or negative represent mean change from baseline

Bars represent \pm one standard deviation from the mean

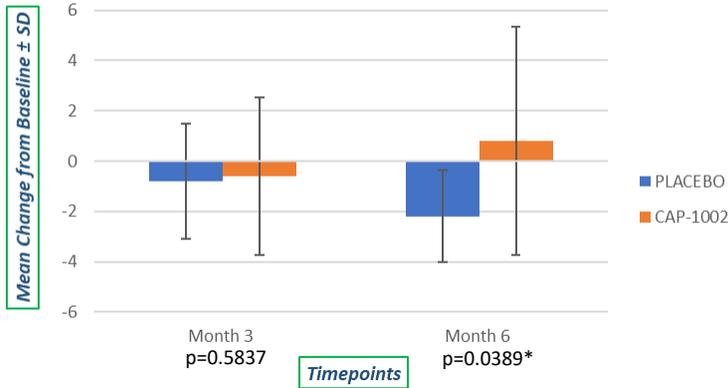
Analysis done in Per Protocol Population

* Statistically significant

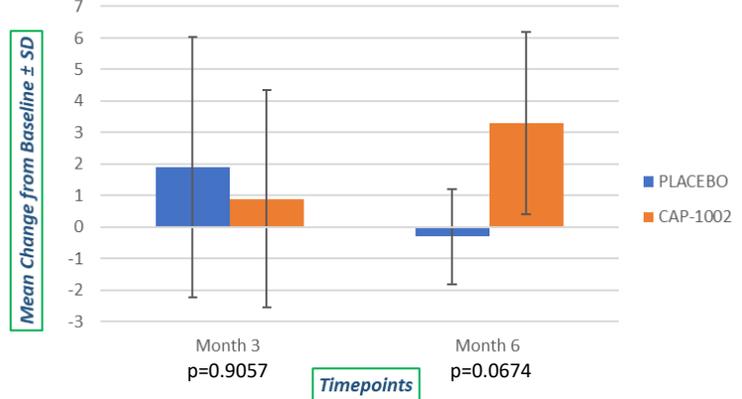
- A significant improvement in PUL 2.0 was observed at 6 months in subjects treated with CAP-1002 when compared with placebo treated subjects

Independent Skeletal Muscle: Grip Strength and Tip to Tip Pinch Strength

Grip Strength



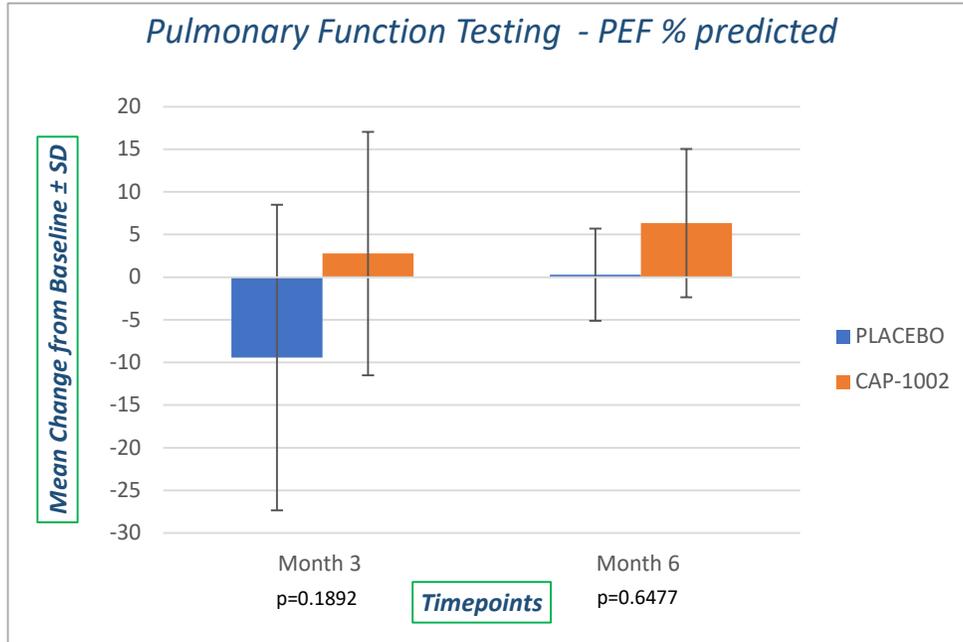
Tip to Tip Pinch Strength



* Statistically significant

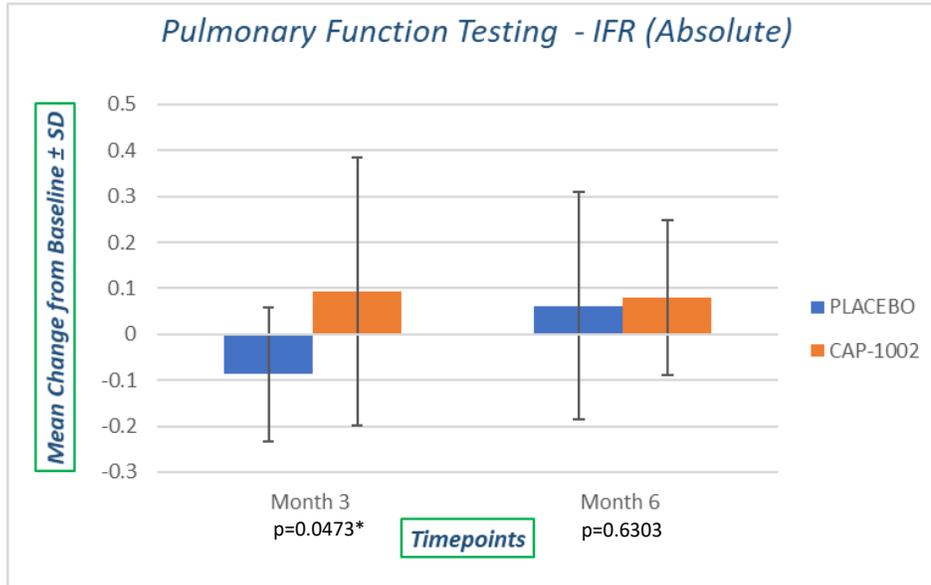
- An improvement in Grip Strength (statistically significant) and in Tip to Tip Pinch Strength was observed at 6 months in CAP-1002 group when compared with placebo group

PFTs: Peak Expiratory Flow (PEF) - % Predicted



- An improvement in Peak Expiratory Flow was observed at 3 and 6 months in CAP-1002 group when compared with placebo group

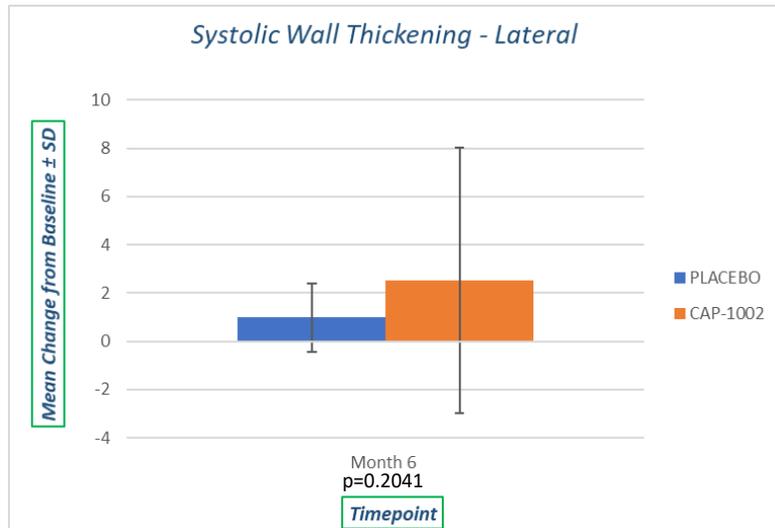
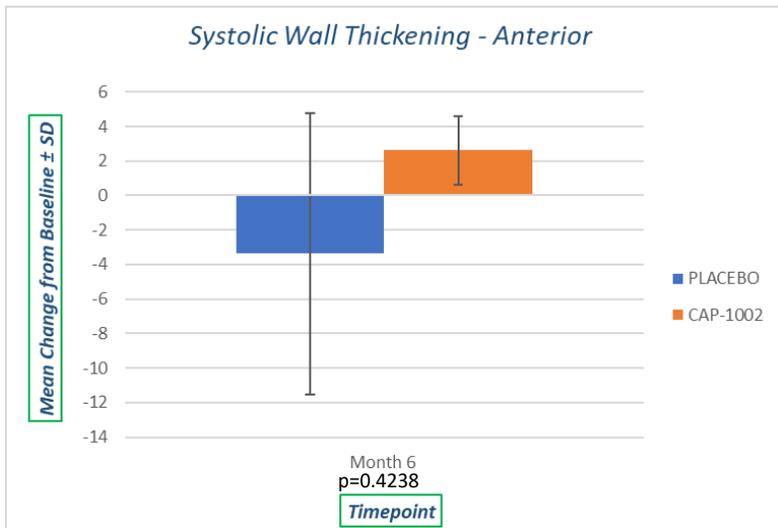
PFTs: Inspiratory Flow Reserve (IFR) - Absolute



* Statistically significant

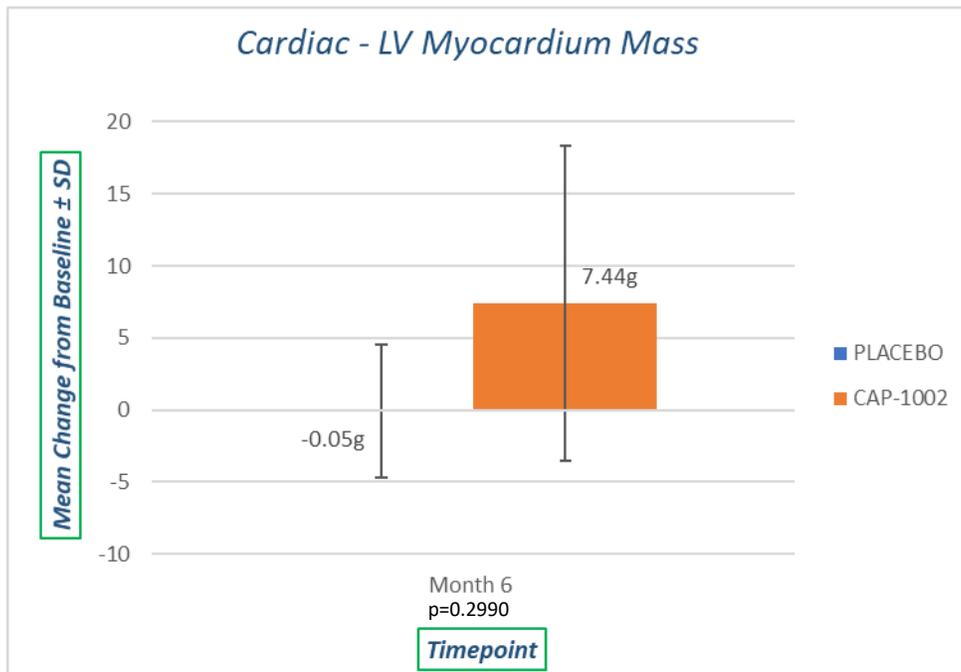
- **A significant improvement in Inspiratory Flow Reserve was observed at 3 months in CAP-1002 group when compared with placebo group**

Cardiac: Systolic Wall Thickening – Anterior & Lateral



- **An improvement in anterior and lateral systolic wall thickening was observed at 6 months in CAP-1002 group when compared with placebo group**

Cardiac: LV Myocardium Mass



- An increase in left ventricle myocardium mass was observed at 6 months in CAP-1002 group when compared with placebo group

HOPE-2 Interim Analysis Safety Results

- A total of 56 infusions were performed in HOPE-2 to date
 - With the exception of two serious adverse events¹ in the form of immediate immune reactions, no safety signals were identified
- To reduce the risk of future adverse events, Capricor initiated a commonly used pre-medication regimen including intravenous steroids and antihistamines
- Since initiation of the pre-treatment regimen, 30 infusions of CAP-1002 or placebo have been administered with only one serious adverse event reported that required an overnight observation of the patient.

HOPE-2 Interim Analysis Data Summary

- **Statistical Significance** in PUL 2.0 at 6 months ($p=0.0389$) and strong signal at 3 months ($p=0.0591$)
- **Statistical Significance** in grip strength (independent skeletal measure) at 6 months ($p=0.0389$) and a strong trend in tip to tip pinch strength at 6 months.
- **Statistical Significance** in IFR (pulmonary) at 3 months ($p=0.0473$)
- **Positive trends in pulmonary measures**
 - Peak Expiratory Flow
- **Additional positive trends in cardiac measures**
 - Anterior and lateral wall thickening (similar to positive changes seen in HOPE-Duchenne)
 - LV myocardium mass
- **Conclusion:** the interim data seen in HOPE-2 is consistent with FDA's guidance on requirements for potential registration (PUL + skeletal) and supportive pulmonary and/or cardiac measures
- Capricor will continue its ongoing discussions with the FDA about its DMD program



Comments by Craig McDonald, M.D.



Thank you

Questions and Answer