# UNITED STATES
# SECURITIES AND EXCHANGE COMMISSION

**Washington, D.C. 20549**

# FORM 8-K

**CURRENT REPORT**
**Pursuant to Section 13 or 15(d) of**
**The Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported)

**September 24, 2021**

# CAPRICOR THERAPEUTICS, INC.

**(Exact name of Registrant as Specified in its Charter)**

| **Delaware** | **001-34058** | **88-0363465** |
|---|---|---|
| **(State or other jurisdiction of incorporation)** | **(Commission File Number)** | **(I.R.S. Employer Identification No.)** |

| **8840 Wilshire Blvd., 2nd Floor, Beverly Hills, CA** | **90211** |
|---|---|
| **(Address of principal executive offices)** | **(Zip Code)** |

**(310) 358-3200**
**(Registrant's telephone number, including area code)**

**Not Applicable**
**(Former name or former address, if changed since last report)**

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (17 CFR §230.405) or Rule 12b-2 of the Securities Exchange Act of 1934 (17 CFR §240.12b-2).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Securities registered pursuant to Section 12(b) of the Act:

| **Title of Each Class** | **Trading Symbol(s)** | **Name of Each Exchange on Which Registered** |
|---|---|---|
| Common Stock, par value $0.001 per share | CAPR | The Nasdaq Capital Market |

**Item 7.01 Regulation FD Disclosure.**

On September 24, 2021, the Company, provided an update on the recently announced final 1-year results from the HOPE-2 clinical trial, in the form of a slide presentation. The slide presentation is located on the "Investors" section of the Company's website at www.capricor.com. A copy of the slide presentation is also attached hereto as Exhibit 99.2 and is incorporated by reference into this Item 7.01 of this Current Report on Form 8-K.

The information contained in this Form 8-K (including Exhibit 99.1 attached hereto) is being furnished and shall not be deemed to be "filed" for the purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section and shall not be incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as shall be expressly set forth by specific reference in such filing.

**Item 8.01 Other Events.**

On September 24, 2021, Capricor Therapeutics, Inc. (the "Company") announced positive final 1-year results from the HOPE-2 clinical trial. HOPE-2 was a randomized, double-blind, placebo-controlled, Phase II clinical trial of CAP-1002 (allogeneic cardiosphere-derived cells) in boys and young men who are in advanced stages of Duchenne muscular dystrophy. A copy of the press release is attached hereto as Exhibit 99.1 and is incorporated by reference into this Item 8.01 of this Current Report on Form 8-K.

**Item 9.01 Financial Statements and Exhibits.**

**(d) Exhibits**

| | |
|---|---|
| 99.1 | Press Release, titled "Capricor Therapeutics Announces Positive Final Data From its Phase 2 HOPE-2 Trial in Patients with Duchenne Muscular Dystrophy Treated with CAP-1002", dated September 24, 2021. |
| 99.2 | Capricor Therapeutics, Inc. slide presentation dated September 24, 2021. |
| 104 | Cover Page Interactive Data File (formatted as inline XBRL and contained in Exhibit 101). |

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**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned, hereunto duly authorized.

**CAPRICOR THERAPEUTICS, INC.**

Date: September 24, 2021

By: /s/ Linda Marbán, Ph.D.
Linda Marbán, Ph.D.
Chief Executive Officer

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**Capricor Therapeutics Announces Positive Final Data From its Phase 2 HOPE-2 Trial in Patients with Duchenne Muscular Dystrophy Treated with CAP-1002**

*–Trial Met its Primary Efficacy Endpoint of Mid-level Performance of Upper Limb (PUL) v1.2 (p=0.01)–*

*–Additional Positive Endpoints of Full PUL v2.0 (p=0.04) and Cardiac Endpoint of Ejection Fraction (p=0.002)–*

*–One-Year Results Demonstrated CAP-1002 Slowed Decline by 71% (Mid-level PUL v1.2)–*

*–CAP-1002 Significantly Improved Cardiac Function in Patients–*

*–Results Presented Today at World Muscle Society Annual Meeting in Late Breaking Oral Presentation–*

*–Principal Investigator Dr. Craig McDonald and Capricor Management Team will Host a Conference Call and Webcast Today at 8:30 a.m. ET–*

**LOS ANGELES, Calif.,** Sept. 24, 2021 – Capricor Therapeutics (NASDAQ: CAPR) ("Capricor" or "the Company"), a biotechnology company focused on the development of transformative cell and exosome-based therapeutics for the treatment and prevention of a broad spectrum of diseases, announced today positive final data from the HOPE-2 clinical trial using CAP-1002 to treat patients in advanced stages of Duchenne muscular dystrophy (DMD). The HOPE-2 clinical trial met its primary efficacy endpoint of mid-PUL v1.2 as well as various skeletal and cardiac endpoints suggesting clinically relevant slowing of disease progression. CAP-1002 is Capricor's cell-based therapeutic candidate whose mechanism of action is immunomodulatory, anti-fibrotic and has been shown to regenerate skeletal and cardiac muscle cells. This final data will be presented today at this year's World Muscle Society Virtual Congress (WMS).

Dr. Craig McDonald, the national principal investigator for the HOPE-2 clinical trial and UC Davis professor and chair of the Department of Physical Medicine and Rehabilitation commented, "This groundbreaking study is extremely exciting as we saw statistically significant changes of CAP-1002 in both skeletal and cardiac function. For these older boys who have limited therapeutic options, these data support the belief that CAP-1002 may become an important therapeutic option and possibly slow the progression of DMD."

HOPE-2 was a randomized, double-blind, placebo-controlled, Phase 2 clinical trial of the Company's lead investigational therapy, CAP-1002, in boys and young men who have DMD and are non-ambulant, the later stage of the disease process. The trial was conducted at nine sites across the United States. Study patients were treated via intravenous delivery with either CAP-1002 (150 million cells per infusion) or placebo every 3 months. Data from a total of 20 patients was analyzed (12 placebo and 8 treated) at the 12-month time-point in the intent to treat (ITT) population. Approximately 80% of the patients were non-ambulant and all patients were on a stable regimen of steroids. Demographic and baseline characteristics were similar between the two treatment groups. Final data analysis demonstrated that young men in the advanced stages of DMD experienced improvements in skeletal and cardiac measurements after receiving four doses of CAP-1002 over the course of 1 year.

Subjects in the trial were evaluated using the Performance of the Upper Limb (PUL), a validated tool specifically designed for assessing high (shoulder), mid (elbow) and distal (wrist & hand) function, with a conceptual framework reflecting the progression of weakness in upper limb function.

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**Final Efficacy Results**

| **12-month Difference in Change from Baseline†** | | |
|---|---|---|
| | **Δ, CAP-1002 vs. Placebo (n=8, n=12)** | **p-value** |
| **Skeletal Muscle (Upper Limb Function)** | | |
| Mid-level PUL (version 1.2) | 2.6 | 0.01 |
| Shoulder + Mid + Distal PUL (version 1.2) | 3.2 | 0.02 |
| Shoulder + Mid + Distal PUL (version 2.0) | 1.8 | 0.04 |
| **Cardiac Function** | | |
| LV Ejection Fraction % | 4.0 | 0.002 |
| LV End Diastolic Volume, Indexed mL/m$^2$ | -12.4‡ | 0.03 |
| LV End Systolic Volume, Indexed mL/m$^2$ | -4.2‡ | 0.01 |
| Creatine Kinase-MB (% of total CK) | -2.2‡ | 0.02 |

†Non-parametric mixed model repeated measures analysis with percentile ranked baseline, treatment, visit, visit-by-treatment interaction, PUL entry-item score at stratification, and site as model effects. Percentile ranked change from baseline converted back to original scale.

‡Negative value favors CAP-1002.

ITT (intent-to-treat) population shown.

CAP-1002 was generally safe and well tolerated throughout the study. With the exception of two hypersensitivity reactions early in the clinical trial, which were mitigated with a common pre-medication regimen, there were no serious safety signals identified by the HOPE-2 Data and Safety Monitoring Board (DSMB).

“The significance of this data is vitally important to patients and the DMD community. The data suggests that CAP-1002 slowed the decline of DMD in patients for whom few options currently exist,” said Dr. Linda Marbán, Ph.D., Chief Executive Officer of Capricor. “Now that we have clarity from the FDA and based on the strength of this data set, we are poised to embark on the HOPE-3 pivotal trial once we have secured an appropriate partner that can help drive CAP-1002 forward towards commercialization. Most importantly, we are thankful to the patients and families who participated in this study so that we can demonstrate the impact of CAP-1002 in treating DMD.”

These data were recently accepted at this year’s World Muscle Society Virtual Congress as a late-breaking oral presentation, due to its high-impact research findings that are of great interest to congress participants. The late-breaking results will be presented today, September 24, 2021.

This is the second clinical trial investigating CAP-1002 showing similar results in the treatment of DMD patients. Capricor completed the HOPE-Duchenne (Phase 1/2) trial in 2019, the results of which were published in *Neurology*, the medical journal of the American Academy of Neurology. The Company has initiated a technology transfer with Lonza, a leading global CMO to prepare for commercial manufacturing of CAP-1002.

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Dr. McDonald is a professor of pediatrics, professor and chair of the Department of Physical Medicine and Rehabilitation, director of Rehabilitation Services and director of the Neuromuscular Disease Clinics at UC Davis Health. He has served as a principal investigator for more than 30 industry-sponsored trials for DMD and is the study chair for the Duchenne Natural History Study of the Cooperative International Neuromuscular Research Group, a consortium of medical and scientific investigators from academic and research centers who share the common goal of aiming to positively impact the lives of neuromuscular disease patients and their families by conducting well-controlled clinical studies.

**Conference Call and Webcast Details**

Capricor will host a conference call and webcast with slides today, September 24, 2021, at 8:30 a.m. ET
to discuss the final data of the HOPE-2 study. To participate in the conference call, please dial 877-451-6152 (domestic) or 201-389-0879 (international) and reference the access code: 13723043

To participate via a webcast, please visit: http://public.viavid.com/index.php?id=146508 to view the slides. The webcast will be archived for approximately 30 days and will be available at
http://capricor.com/news/events/.

**About CAP-1002**

CAP-1002 consists of allogeneic cardiosphere-derived cells, or CDCs, a type of progenitor cell that has been shown in pre-clinical and clinical studies to exert potent immunomodulatory activity and is being investigated for its potential to modify the immune system’s activity to encourage cellular regeneration. CDCs have been the subject of over 100 peer-reviewed scientific publications and have been administered to over 200 human subjects across several clinical trials.

**About Duchenne Muscular Dystrophy**

Duchenne muscular dystrophy is a devastating genetic disorder characterized by progressive weakness and chronic inflammation of the skeletal, heart and respiratory muscles Patients suffering from DMD typically lose their ability to walk in their teenage years and generally die of cardiac or respiratory complications by age 30. It occurs in one in every 3,600 live male births across all races, cultures and countries. DMD afflicts approximately 200,000 boys and young men around the world. Treatment options are limited, and there is no cure.

**About Capricor Therapeutics**

Capricor Therapeutics, Inc. (NASDAQ: CAPR) is a biotechnology company focused on developing transformative cell and exosome-based therapeutics and vaccines for treating and preventing a broad spectrum of diseases. Capricor's lead candidate, CAP-1002, is an allogeneic cardiac-derived cell therapy that is currently in clinical development for treating Duchenne muscular dystrophy and the cytokine storm associated with COVID-19. Capricor is also developing its exosome technology as a next-generation therapeutic platform. The Company’s current focus is on developing exosomes loaded with nucleic acids, including mRNA, to treat or prevent a variety of diseases. For more information, visit www.capricor.com, and follow the Company on Facebook, Instagram and Twitter.

**Cautionary Note Regarding Forward-Looking Statements**

Statements in this press release 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, revenue 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, 2020, as filed with the Securities and Exchange Commission on March 15, 2021, and in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021 as filed with the Securities and Exchange Commission on August 13, 2021. 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.

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*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.*

For more information, please contact:

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310.358.3200

# HOPE-2 Multi-center Randomised Clinical Trial of Intravenous Human Cardiosphere-Derived Cells for Late-Stage Duchenne Muscular Dystrophy

## Data Presentation
## 1 Year Final Results

Craig McDonald, M.D., University of California, Davis Health

Eduardo Marbán, MD, PhD, Suzanne Hendrix, PhD, Nathaniel Hogan, PhD, Rachel Ruckdeschel Smith, PhD, Michelle Eagle, PhD, Richard S. Finkel, MD, Cuixia Tian, MD, Joanne Janas, MD, Matthew M. Harmelink, MD, Arun S. Varadhachary, MD, PhD, Michael D. Taylor, MD, Kan N. Hor, MD, Oscar H. Mayer, MD, Erik K. Henricson, PhD, Pat Furlong, Deborah A. Ascheim, MD, Siegfried Rogy, Paula Williams, Linda Marbán, PhD

Capricor Therapeutics™

WMS World Muscle Society



# 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, revenue 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, 2020 as filed with the Securities and Exchange Commission on March 15, 2021 and in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2021 as filed with the Securities and Exchange Commission on August 13, 2021. 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. None of Capricor's exosome-based candidates have 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.

- **AJ Bergmann**, Chief Financial Officer, Capricor Therapeutics, Inc.



# Capricor Therapeutics

## Company Highlights

**Capricor is a cell and exosome-based platform therapeutics company**

**Publicly Traded (NASDAQ: CAPR)**

- Research Headquarters: San Diego, CA
- Employees: 30
- Founded: 2006, public in 2013

**Promising Therapeutic and Vaccine Candidates**

- Cell Therapy (Duchenne muscular dystrophy and COVID-19)
- Exosome-mRNA multivalent vaccine (COVID-19)
- Exosome-RNA therapeutics (undisclosed)

**Over 100 publications from multiple institutions worldwide on both platforms with extensive in-vivo and clinical data**

**External Collaborations**

- US Army
- US Department of Defense
- Stephen Gould, Ph.D. Laboratory (Johns Hopkins University)
- Cedars-Sinai Medical Center

## Data Presentation: Dr. Craig McDonald

### 1 Year Final Results



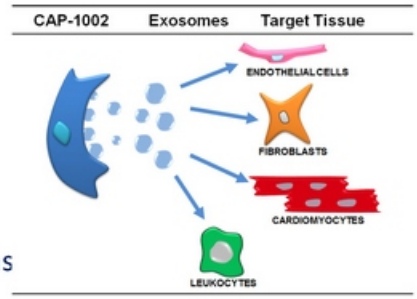
# Disclosure: Dr. McDonald

**I have the following conflict(s) of interest to declare:**

- Served as a consultant and advisory board member for Capricor Therapeutics on issues related to DMD drug development
- Academic Institution has received research funding from Capricor Therapeutics for the conduct of DMD clinical trials

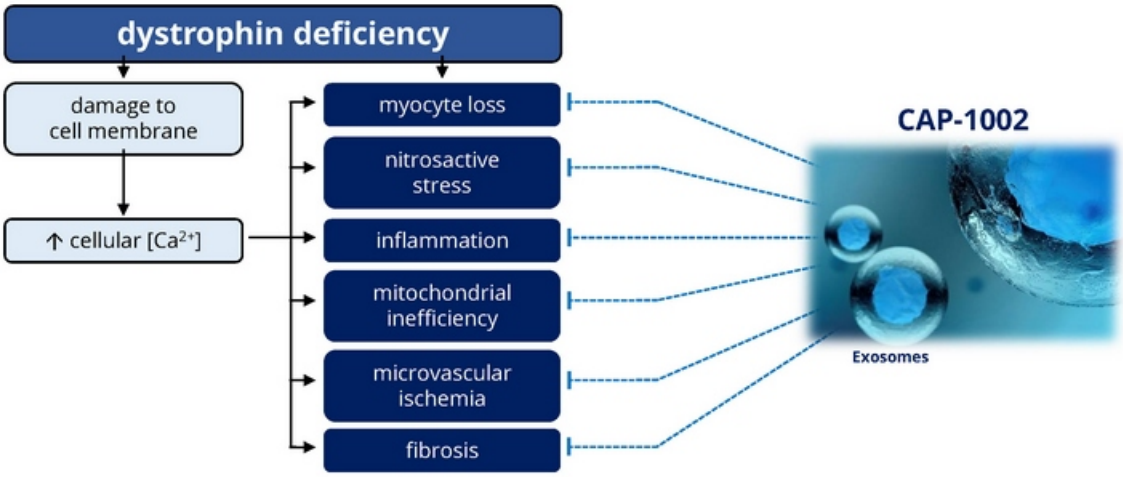
# Capricor's CAP-1002 Technology

- **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
  - Mechanism - cells secrete exosomes:
    - ➢Contain miRNA, non-coding RNAs and proteins
    - ➢Internalized by target cells
    - ➢Stimulate diverse and lasting changes in cellular behavior
    - ➢3 known miRNAs drive CAP-1002 potency
  - CAP-1002 has been investigated in multiple independent clinical trials and in more than 200 human subjects

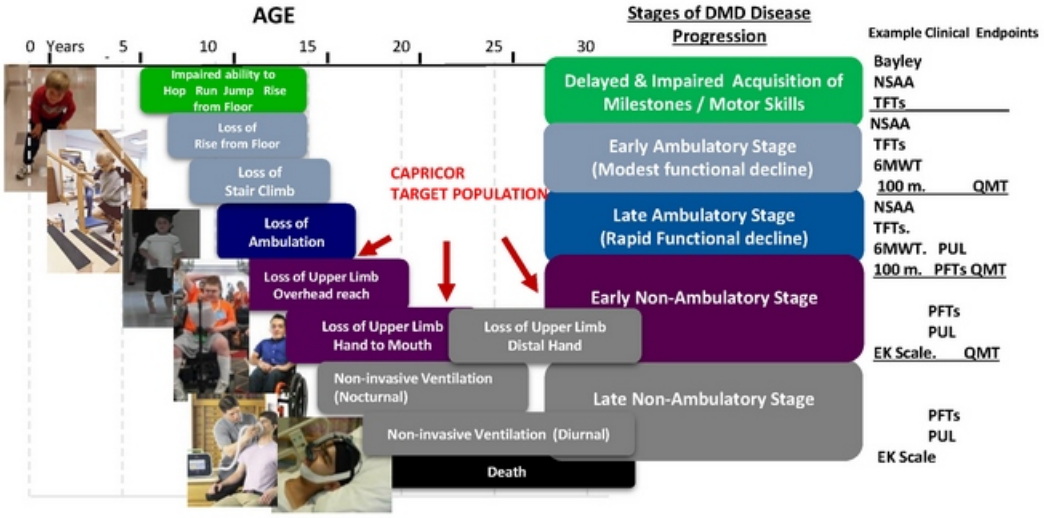





# CAP-1002 Targets Multiple Disease Processes in DMD



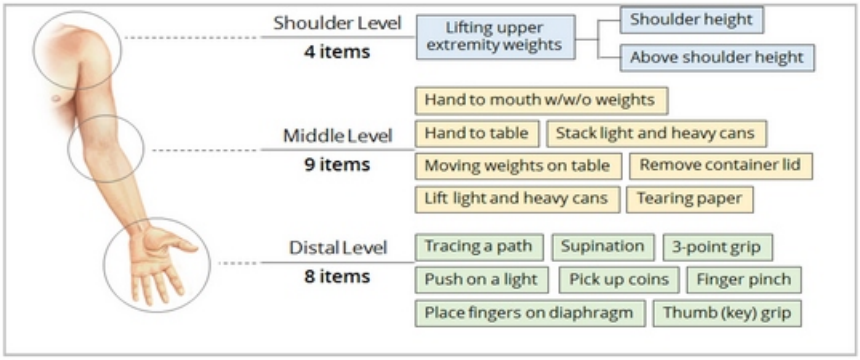
# DMD Progression is Sequential, Non-Linear and Irreversible






# Primary Efficacy Endpoint

## Mid-Level PUL v.1.2

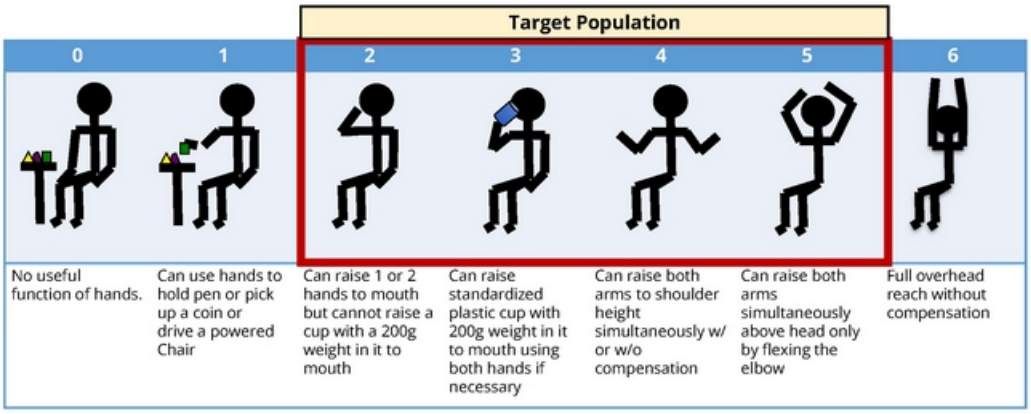



**PUL v.2.0:**

- 3-point response scale - more robust and reproducible than v1.2
- Compensatory strategies allowed to achieve tasks (not allowed in v1.2)
- v2.0: better able to detect change at 12 months at all levels of ability*

*Mayhew et al, 2019; Pane et al, 2018.

# Performance of Upper Limb (Entry Items)






# HOPE-2 Clinical Trial Design

- **Design:** Phase II, randomized, double-blind, placebo-controlled trial in participants with DMD and reduced skeletal muscle function
- **Objective:** Evaluate safety and efficacy of CAP-1002
- **Dosing Regimen:** 150M cells delivered intravenously every 3 months
- **Sites:** 9 sites (USA)
- **Data:** ITT population - 20 subjects

**Demographics**

- Mean age: 14.3 years
- All patients were on corticosteroids
- ~ 80% of patients were non-ambulant

https://www.clinicaltrials.gov/ct2/show/study/NCT03406780.

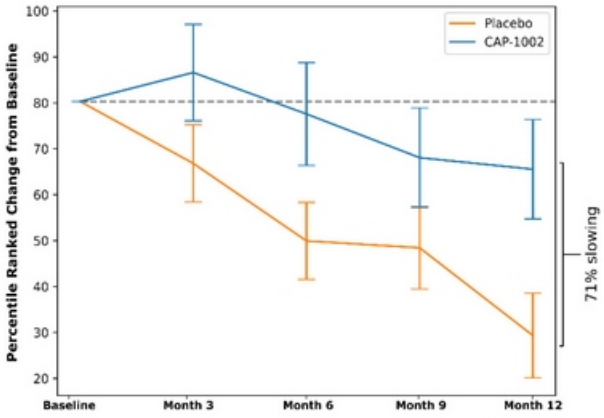
# HOPE-2 Final Data Safety Results

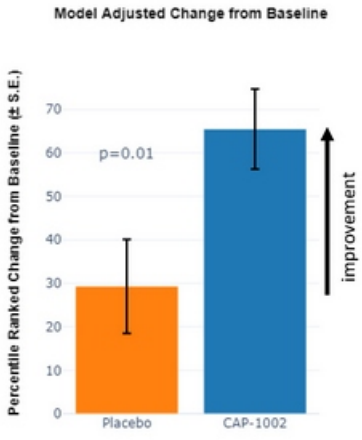
- **A total of 69 infusions (CAP-1002 or placebo) were performed in HOPE-2**
  - Generally safe and well tolerated throughout the study
  - With the exception of two hypersensitivity reactions, no safety signals were identified



# HOPE-2 Final Data Efficacy Analysis

**Primary Efficacy Endpoint: PUL 1.2 Mid Level**



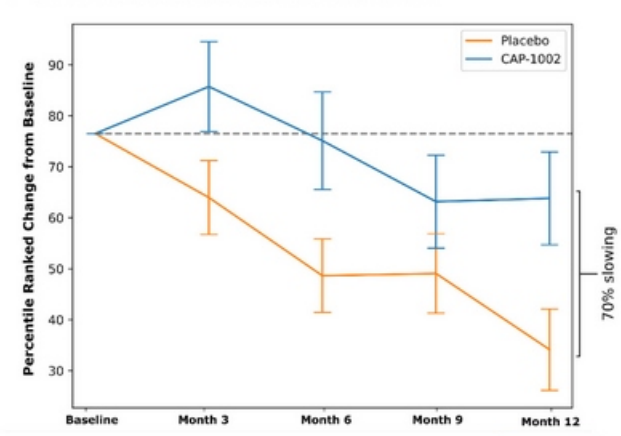

**p=0.01**

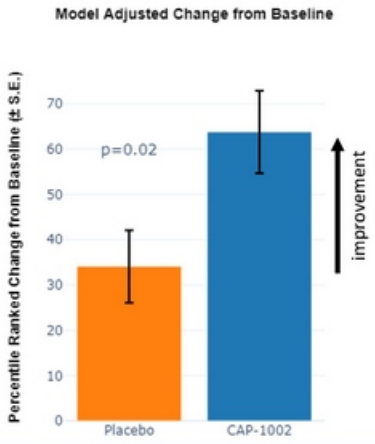
**Δ=2.6 points in CAP-1002 vs. placebo at 12-months**

Mixed Model for Repeated Measures (MMRM) analysis was performed using percentile ranked change from baseline as dependent variable and percentile ranked baseline score, treatment, visit, treatment-by-visit interaction, PUL entry-item score at randomization, and site as model effects. Adjusted model outcomes are report as least-squares means (LS-Mean).

# HOPE-2 Final Data Efficacy Analysis

**PUL 1.2 Combined Total Dimension**




**p=0.02**

**Δ=3.2 points in CAP-1002 vs. placebo at 12-months**

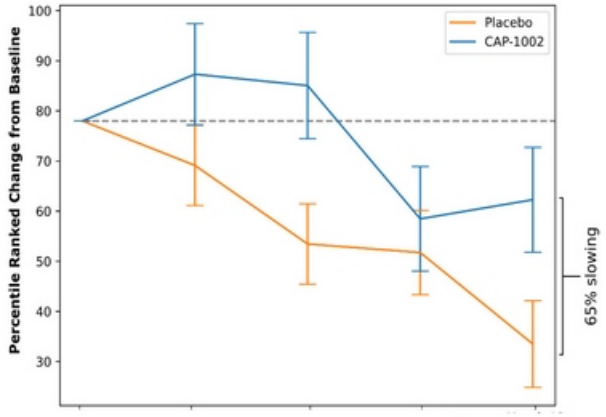
**Clinical meaningfulness assessed as 1 point change**

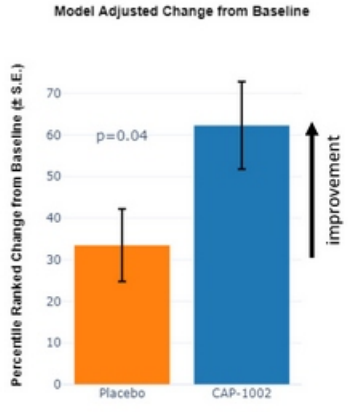
Mixed Model for Repeated Measures (MMRM) analysis was performed using percentile ranked change from baseline as dependent variable and percentile ranked baseline score, treatment, visit, treatment-by-visit interaction, PUL entry-item score at randomization, and site as model effects. Adjusted model outcomes are report as least-squares means (LS-Mean).



# HOPE-2 Final Data Efficacy Analysis

**PUL 2.0 Combined Total Dimension**



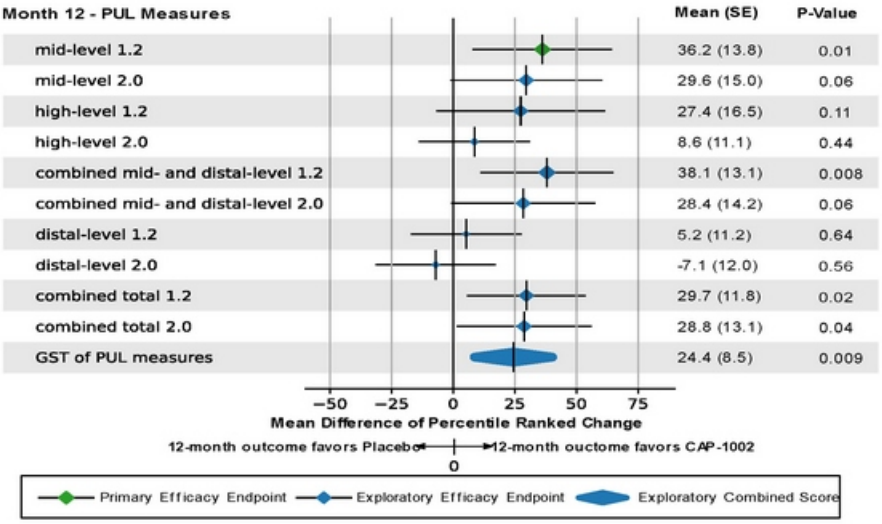

**p=0.04**

**Δ=1.8 points in CAP-1002 vs. placebo at 12-months**

Mixed Model for Repeated Measures (MMRM) analysis was performed using percentile ranked change from baseline as dependent variable and percentile ranked baseline score, treatment, visit, treatment-by-visit interaction, PUL entry-item score at randomization, and site as model effects. Adjusted model outcomes are report as least-squares means (LS-Mean).

# HOPE-2 Final Data Efficacy Analysis

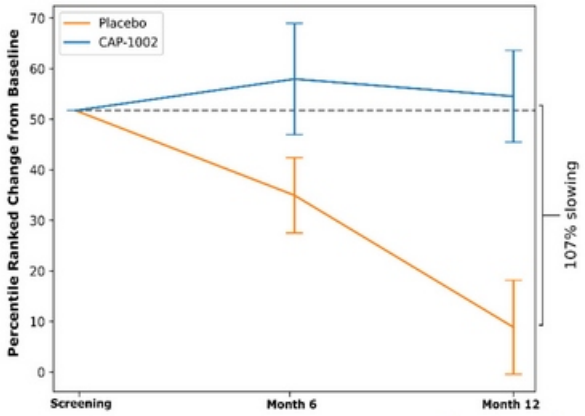
**All Dimensions of PUL**



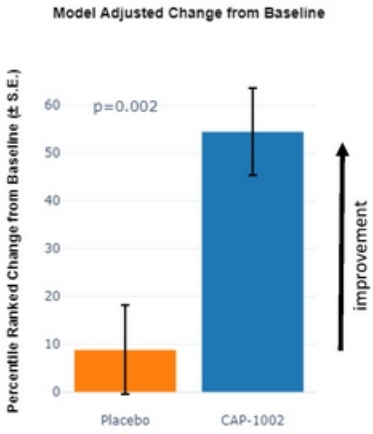



# HOPE-2 Final Data Efficacy Analysis

**Left Ventricular Ejection Fraction**




p=0.002

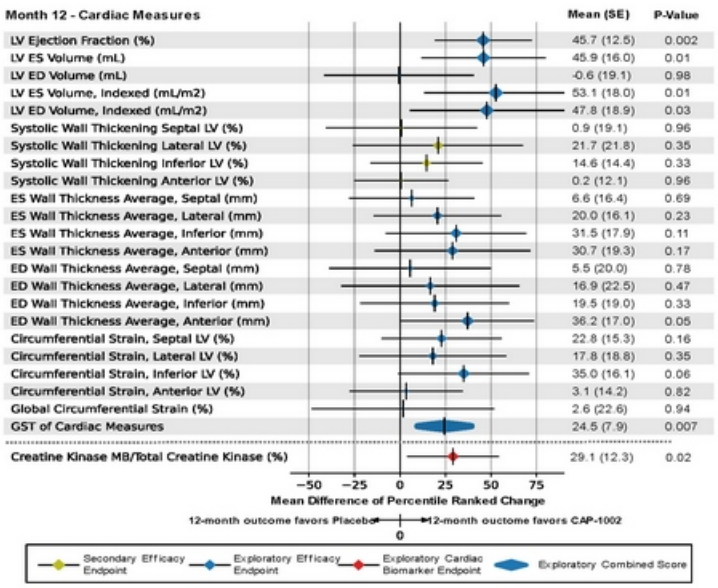



Δ=4% in LVEF by cardiac MRI

Mixed Model for Repeated Measures (MMRM) analysis was performed using percentile ranked change from baseline as dependent variable and percentile ranked baseline score, treatment, visit, treatment-by-visit interaction, PUL entry-item score at randomization, and site as model effects. Adjusted model outcomes are report as least-squares means (LS-Mean).

# HOPE-2 Final Data Efficacy Analysis

**All Cardiac MRI Measures**



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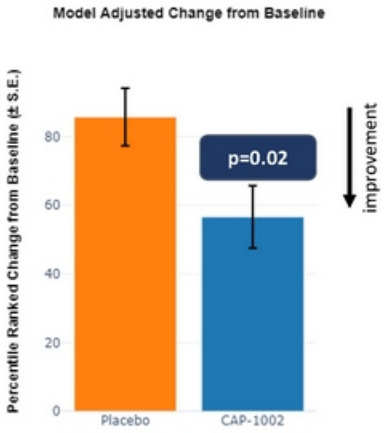
**Creatine Kinase MB / Total Creatine Kinase %**




Mixed Model for Repeated Measures (MMRM) analysis was performed using percentile ranked change from baseline as dependent variable and percentile ranked baseline score, treatment, visit, treatment-by-visit interaction, PUL entry-item score at randomization, and site as model effects. Adjusted model outcomes are report as least-squares means (LS-Mean).

# Conclusions

- CAP-1002 cell therapy appears to be safe and effective in attenuating deterioration of upper limb and cardiac function in late-stage DMD.
- A therapy that stabilizes or reverses cardiac deterioration while improving upper limb function would be unique in its ability to address, synergistically, the tremendous burden of disease seen in non-ambulatory DMD patients
- Currently ongoing extension studies may confirm therapeutic durability and safety of CAP-1002 beyond 12 months in non-ambulant DMD patients.
- HOPE-3, the Phase 3 pivotal study of CAP-1002 in DMD, currently in planning stages
- Additional placebo-controlled trials will be needed to assess the benefit and risks of CAP-1002 in younger ambulatory patients



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## Program Update: Dr. Linda Marban



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# DMD Program Moving Forward

- **HOPE-3**: Phase III, randomized, double-blind, placebo-controlled trial
- **Enrollment:** 65-75 patients
- **Number of sites:** Est. 20
- **Targeting:** Non-Ambulant DMD patients
- **Primary Endpoint:** PUL 2.0 at 12 months
- **Secondary Endpoints:** Cardiac, QOL, clinical status

- Discussions with potential partners underway
- Technology Transfer of CAP-1002 in process with Lonza for potential commercial launch



# Acknowledgments


- **All patients and their families who participated in the HOPE-2 Study**
- **HOPE-2 Investigators**
  - Craig McDonald, MD (UC Davis)
  - Cuixia Tian, MD (CCHMC)
  - Russell Butterfield, MD (University of Utah)
  - Richard Finkel, MD (Nemours Children's Hospital)
  - Joanne Janas, MD (Children's Hospital of Colorado)
  - Matthew Harmelink, MD (Children's Hospital of Wisconsin)
  - Arun Varadhachary, MD (Washington University, Saint Louis Children's Hospital)
  - Brenda Wong, MD (University of Massachusetts)
  - Katherine Mathews, MD (University of Iowa, Children's Hospital)

Thank you
Question and Answer