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)

April 29, 2024

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.)**

10865 Road to the Cure, Suite 150, San Diego, California	**92121**
(Address of principal executive offices)	**(Zip Code)**

(858) 727-1755
(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.

Attached hereto as Exhibit 99.1 to this Current Report on Form 8-K is a slide presentation that the Company reviewed in conjunction with its CAP-1002 Duchenne muscular dystrophy ("DMD") program update call and webcast on April 29, 2024.

The information under Item 7.01 of this Current Report on Form 8-K and Exhibit 99.1 attached hereto is being furnished and shall not be deemed to be filed for 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, nor shall it be incorporated by reference into any of the Company's filings under the Exchange Act, unless expressly set forth as being incorporated by reference into such filing.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits

99.1	Capricor Therapeutics, Inc. slide presentation, dated April 29, 2024.
104	Cover Page Interactive Data File (formatted as inline XBRL).

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: April 29, 2024

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

Exhibit 99.1


Capricor
Therapeutics™
CAP-1002 DMD Program
Update Call
April 29, 2024
Nasdaq: CAPR

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; scope, duration, validity and enforceability of intellectual property rights; future reimbursement prices; future royalty streams and 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, 2023 as filed with the Securities and Exchange Commission on March 11, 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.

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 Attendees

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

Kristi Elliott, Ph.D., **Chief Science Officer**

Mark Awadalla, **Vice President of Clinical Operations**

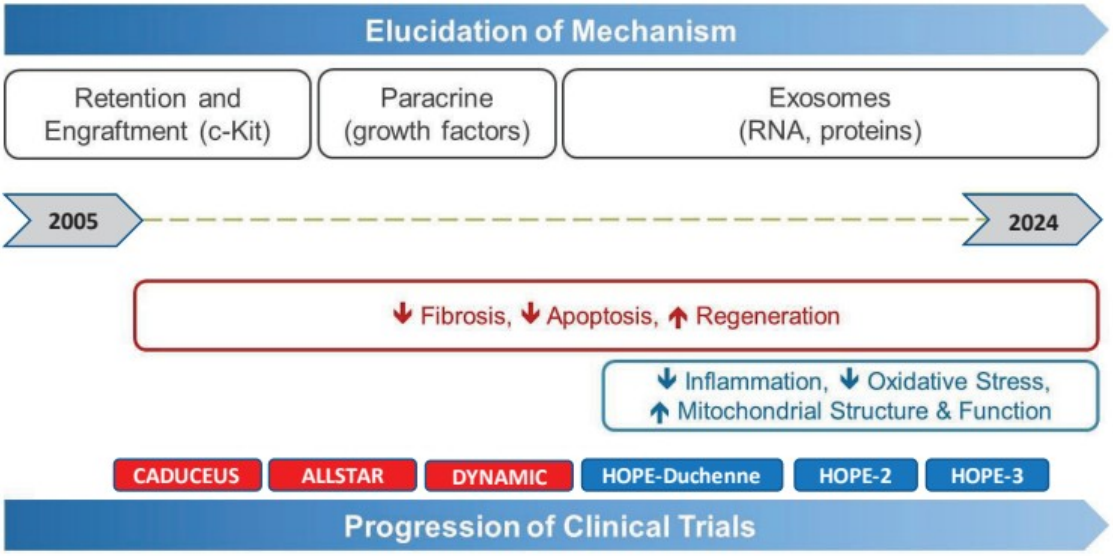
AJ Bergmann, M.B.A., **Chief Financial Officer**

Capricor Call Agenda

1. Capricor Introduction & DMD Program Overview
2. Recent FDA Regulatory Overview & Updates
3. CAP-1002 Cell Therapy Mechanism & Potency Data
4. Commercial Planning for Potential Launch
5. Conclusions & Q&A

Capricor's Evolution

Scientific and Clinicial Developments over 19 Years



CAP-1002 DMD Clinical Overview

Study, Phase, Status, Number of patients (N)	Population	Design	Efficacy Outcomes/Status
HOPE-Duchenne, Phase 1/2 2016-2017, **Completed** **N=25**	Males (>12 years) with cardiomyopathy secondary to DMD	Randomized 1:1 CAP-1002 versus usual care	• Improvements in **PUL v1.2** (mid+distal) • Reduction in cardiac scar at 6 and 12 months • **Published in *Journal of Neurology***
HOPE-Duchenne OLE 2017-2018 **Completed** **N=8**	Males (>12 years) randomized to usual care group in HOPE-Duchenne and completed the 12-month follow-up	Open-label extension	• Assessed for safety
HOPE-2, Phase 2 2018-2020, **Completed** **N=20**	Males (>10 years) with DMD and evidence of skeletal muscle impairment	Double-blind, randomized 1:1 CAP-1002 versus placebo	• Statistically significant results in multiple parameters • Primary endpoint: **mid-level PUL v1.2 (p=0.01)** • Secondary endpoints: **full PUL v2.0 (p=0.04)** • **LV ejection fraction (p=0.002)** • **Published in *The Lancet***
HOPE-2 OLE 2020-present, **Ongoing** N=12	Males (>10 years) with DMD who participated in HOPE-2 and completed the 12-month follow-up	Open-label extension	• Study ongoing • Positive 2-year results, **full PUL v2.0 (p=0.021)** • **~66% patients improved** in LV ejection fraction
HOPE-3 Cohort A, Phase 3 2022-present, **Ongoing** N=61	Males (>~10 years) with DMD	Double-blind, randomized 1:1 CAP-1002 versus placebo	• Study ongoing • Enrollment complete • **Successful interim futility analysis (Q4 2023)**
HOPE-3 Cohort B, Phase 3 2023-present, **Ongoing** N=~44			• Study ongoing, evaluating options
HOPE-3 OLE 2023-present, **Ongoing** N=~26	Males (>10 years) with DMD who participated in HOPE-3 and completed the 12-month follow-up	Open-label extension	• Study ongoing

Capricor's Regulatory Designations

CAP-1002 for DMD

GOAL OF FDA'S RMAT DESIGNATION

To facilitate efficient development and expedite review of a drug

Products may also be eligible for accelerated approval

- RMAT provides benefits that include more frequent meetings with FDA to discuss the development plan for the product candidate
- Eligibility for rolling review and priority review

Similar to breakthrough therapy designation:

- On the basis of a surrogate or intermediate endpoint reasonably likely to predict long-term clinical benefit
- Reliance upon data obtained from a meaningful number of sites

RMAT Designation

Rare Pediatric Disease Designation

Orphan Drug Designation

Performance of Upper Limb (PUL Test)

To Assess Skeletal Muscle Function

Shoulder Level
4 items

- Lifting upper extremity weights
  - Shoulder height
  - Above shoulder height

Middle Level
9 items

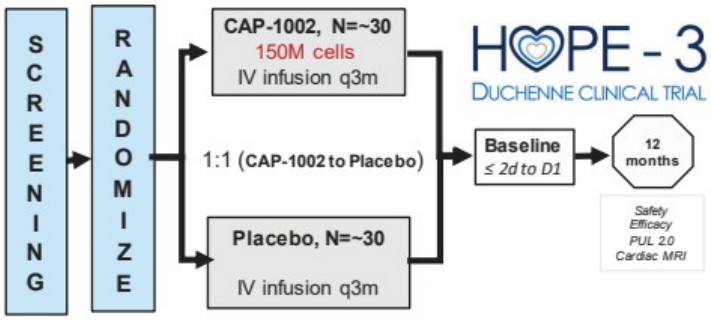
- Hand to mouth w/w/o weights
- Hand to table
- Stack light and heavy cans
- Moving weights on table
- Remove container lid
- Lift light and heavy cans
- Tearing paper

Distal Level
8 items

- Tracing a path
- Supination
- 3-point grip
- Push on a light
- Pick up coins
- Finger pinch
- Place fingers on diaphragm
- Thumb (key) grip

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

HOPE-3: Phase 3 Pivotal Trial Overview



Design

- Cohort A : **61 patients enrolled**

Endpoints

- **Primary endpoint**: change in PUL v2.0 at 12 months
- Various secondary endpoints: cardiac, QOL, etc.

Successful Interim Analysis

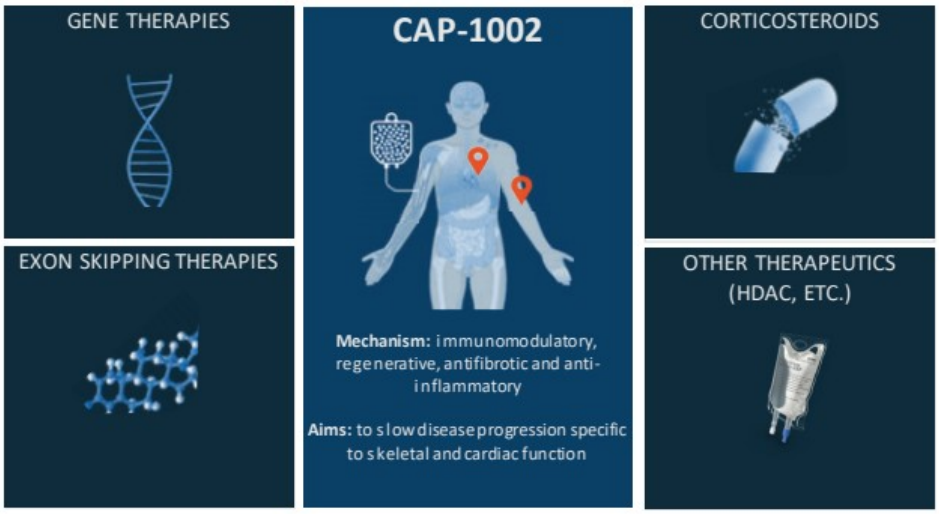
- Completed in Dec. 2023
- Primarily for futility
- Trial continued as planned

Outlook & Next Steps

- **Cohort A enrollment complete**
- Topline 12-month data expected in Q4 2024
- Cohort A to support BLA submission

CAP-1002 has the Potential

to Redefine the Standard of Care for DMD



Capricor Call Agenda

1. Capricor Introduction & DMD Program Overview
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Summary of Regulatory Achievements

Date	Regulatory Interaction	Outcome/Purpose
Q3 2021	Type-B End-of-Phase-2 meeting	• Clearance of Phase 3 HOPE-3 protocol
Q1 2023	Type B CMC meeting	• Feedback on further development of CAP-1002 potency assays
Q3 2023	Type B clinical meeting	• Discussed BLA submission requirements • Alignment on HOPE-3 Cohort B design for use of drug product from San Diego site
Q1 2024	Type B CMC meeting	• Alignment on non-clinical comparability from Los Angeles and San Diego sites • Alignment on HOPE-3 Cohort A sufficient to support BLA • Feedback supporting requests for pre-BLA meeting and rolling BLA submission timelines
Q2 2024 *upcoming*	Type B clinical meeting	• Obtain FDA feedback on clinical data analysis strategies and CMC control strategy • Formally request pre-BLA meeting and rolling BLA submission
2H 2024 *target*	Pre-BLA meeting	• Initiation of rolling BLA – *pending*

FDA Alignment on Path to BLA

Type-B CMC Meeting Outcome (March 2024)

CAP-1002 Commercial Manufacturing

- Demonstrated non-clinical comparability with analytical data and potency assay
- Use of San Diego facility upon potential product approval
- HOPE-3 Cohort B not necessary for FDA approval

Pre-BLA Meeting and Rolling BLA

- Feedback supports requests for a Pre-BLA meeting and rolling BLA submission timelines following upcoming Type-B meeting

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CAP-1002 Cell Therapy Overview

- **CAP-1002:** biologic consisting of allogeneic cardiosphere-derived cells (CDCs)
  - Endogenous population of stromal cells obtained from donated healthy human hearts
- **Multiple-modalities**:
  - Stimulating muscle tissue growth
  - Retaining muscle function
  - Decreasing inflammation
  - Preventing scarring
- Investigated in over **200 patients**
- **FDA designations in DMD:**
  - ✓ Orphan Drug Designation
  - ✓ Regenerative Medicine Advanced Therapy (RMAT) designation
  - ✓ Rare Pediatric Disease Designation, **Capricor holds full rights to the Priority Review Voucher,** if received

Novel Potency Assay of CAP-1002

Potency assays are foundational for product approval

Clinically efficacious CAP-1002 lots used to develop **two** potency assays

RNAseq assay

- innovative, novel approach showing CAP-1002 lots have the same "**fingerprint**" as clinically beneficial lots

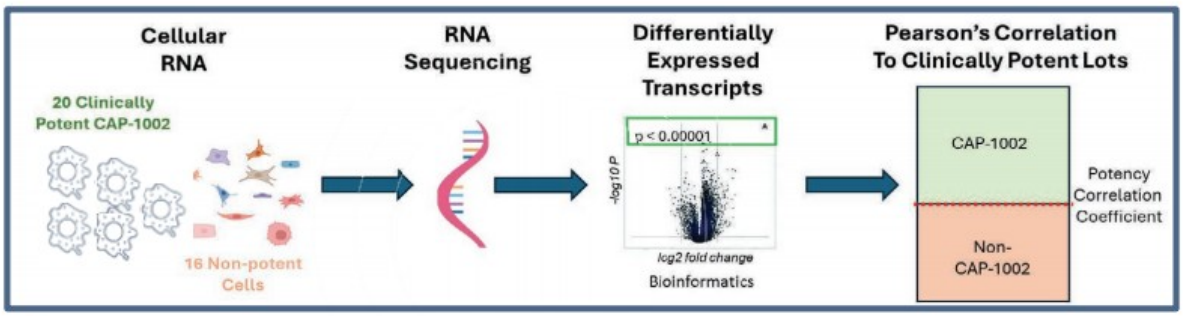
Anti-fibrosis assay

- traditional, cell-based assay demonstrating CAP-1002 mechanism of action, specifically requested by FDA

- Assays are **confirmatory** of potency
- Statistical analysis used to demonstrate equivalence using both potency assays

CAP-1002 Clinical Correlation

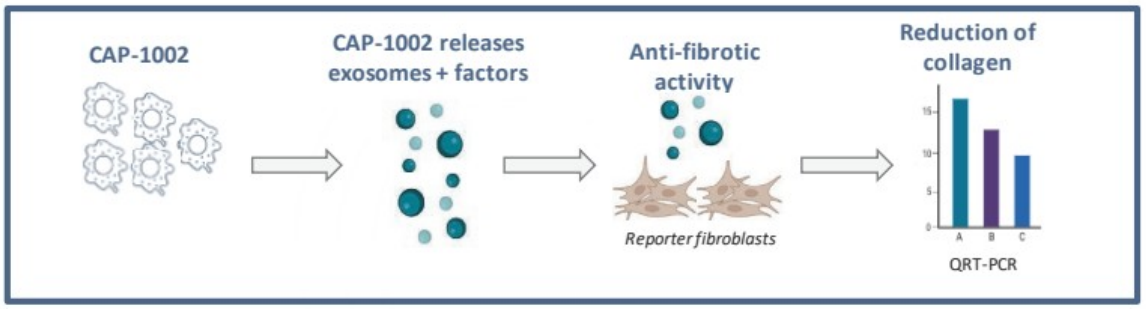
RNAseq+Bioinformatics



- **RNA sequencing** used to create a cell profile or unique "**fingerprint**" of CAP-1002
  - **Different from other cells:** by using profiles of non-CAP-1002 cells
  - **Correlates to clinical potency**: by using the profile of CAP-1002 clinically beneficial lots
- **Stringent and statistically significant (<0.00001)** bioinformatics model

Anti-Fibrosis Cell-Based

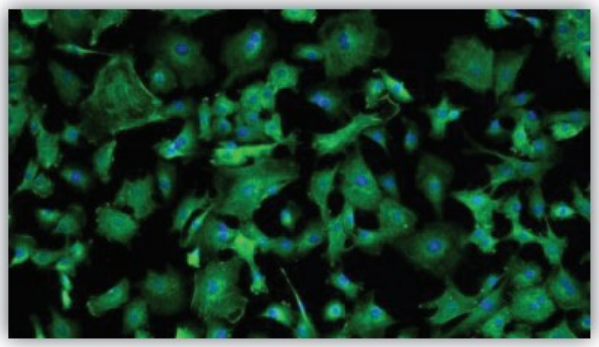
Potency Assay



- **Key mechanism of action of CAP-1002 is anti-fibrosis**
  - Fibrosis: known pathological feature in DMD
  - Tissue hardening with scar formation from increased deposition of proteins (e.g., **collagen**)
- Cell-based assay shows anti-fibrotic activity of released exosomes and factors from CAP-1002
- **Potency demonstrated using an anti-fibrosis assay, indicative of mechanism of action**

CAP-1002 Potency Summary

- **Two first-in-class distinct highly specific potency assays designed to be confirmatory**
- Data supports San Diego and Los Angeles CAP-1002 lots are ***potent***
- San Diego and Los Angeles CAP-1002 drug product are ***statistically equivalent***
- FDA agreed assays are suitable to **demonstrate** comparability



CAP-1002 GMP Manufacturing Facility

San Diego, California

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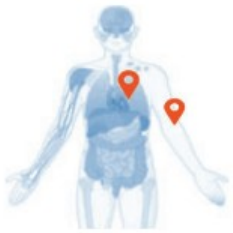
Significant Potential Revenue Opportunity

U.S. DMD Prevalence[1]

- 15,000-20,000 cases
- Non-ambulant population: **~50%**
- Total addressable patients: ~7,500-10,000



- **Treatment regimen:** 4 doses per year
- Target Reimbursement Price* *Aim to be similar or higher than approved exon skipping therapies*
- Potential for multi-year treatment
- Small market penetration: annual product revenue estimates could exceed **~$1.5B**[2]

- **CAP-1002 – first-in-class therapeutic option for patients with limited therapeutic options**
- **Potential to have ~100 patients on OLE transition to commercial product upon approval**

[1]Based on internal/estimated projections and market research. CAP-1002 is not an approved product
[2]Capricor revenue will less than product revenue due to revenue share under Distribution Agreement with Nippon Shinyaku

Commercial Preparations

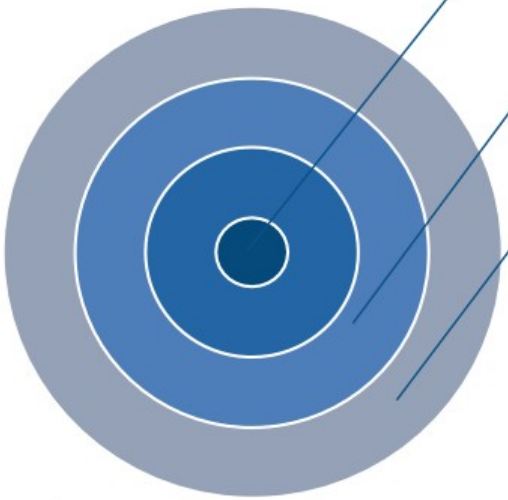
In collaboration with NS Pharma

CAP-1002: Profile & Commercial Reach

[1]Based on HOPE-2 and HOPE-2 OLE data

[2]Capricor revenue will less than product revenue due to revenue share under Distribution Agreement with Nippon Shinyaku

Potential Expansion of CAP-1002



Duchenne muscular dystrophy (late-stage)
~7,500-10,000 patients in U.S.*

Duchenne muscular dystrophy (early-stage)
~7,500-10,000 patients in U.S.*
Expansion to younger patients

Becker muscular dystrophy
~ 5,000 patients in U.S.*

*Becker cardiomyopathy develops at a similar age to DMD cardiomyopathy (~14 years old)

*Based on internal/estimated projections and market research.

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Key Takeaways

- Non-clinical comparability demonstrated using 2 distinct potency assays
- San Diego facility to be used upon potential approval
- Upcoming Type-B meeting to discuss Pre-BLA meeting and rolling BLA schedule
- Phase 3 HOPE-3 Cohort A topline data expected in late Q4 2024
- Presentation of HOPE-2 OLE 3-year data to FDA at upcoming meeting
- Commercial planning for launch underway


Thank you
Q&A
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