February 29, 2024

Capricor Therapeutics Reports Fourth Quarter and Full Year 2023 Financial Results and Provides Corporate Update

-Enrollment Complete in Cohort A of Phase 3, Pivotal, HOPE-3 Trial of CAP-1002 in Duchenne Muscular Dystrophy; Announced Successful Interim Futility Analysis; On Track to Report Top-Line Data in Q4 2024-

-Upcoming Type-B Meeting with FDA in Q1 2024 to Discuss Commercial Manufacturing Planning with an Aim to Expedite BLA Pathway for CAP-1002 in Duchenne Muscular Dystrophy-

-CAP-1002 Scale-Up Expansion Underway at New San Diego Manufacturing Facility in Preparation for Commercial Launch-

-Plan to Report 3-Year Data from HOPE-2 Open-Label Extension (OLE) Trial in Q2 2024-

-Announced Selection by Project NextGen for a Clinical Trial of Our Novel Exosome-Based Multivalent Vaccine in Collaboration with the National Institutes of Health-

-Conference Call and Webcast Today at 4:30 p.m. ET-

SAN DIEGO, Feb. 29, 2024 (GLOBE NEWSWIRE) -- Capricor Therapeutics (NASDAQ: CAPR), a biotechnology company developing transformative cell and exosome-based therapeutics for the treatment and prevention of rare diseases, announced today its financial results for the fourth quarter and full year ended December 31, 2023 and provided a corporate update.

“2023 was a pivotal year for Capricor marked by major advances in our CAP-1002 cell therapy program for the treatment of Duchenne muscular dystrophy (DMD),” said Linda Marbán, Ph.D., Capricor’s chief executive officer. “Over the course of the year, we completed enrollment in Cohort A of our Phase 3, HOPE-3 pivotal trial, reported the positive outcome of our interim futility analysis, aligned with the U.S. Food and Drug Administration (FDA) on key deliverables necessary for the filing of a Biologics License Application (BLA), completed construction on our new San Diego manufacturing facility and began clinical dosing of Cohort B of the HOPE-3 trial from our San Diego facility. Each of these achievements are major milestones on our path towards potential approval of CAP-1002 for the treatment of DMD. While there are limited treatment options currently available for patients with DMD, we believe in the potential of CAP-1002’s ability to significantly slow down disease progression. Observed robust and consistent efficacy combined with a favorable safety/tolerability profile, positions CAP-1002 as a potential anchor therapy for patients. In 2024, we plan to meet with FDA to continue to discuss options for potential

expedited approval pathways and Capricor is well-positioned to execute on several important milestones including reporting top-line data from our pivotal trial in the fourth quarter of 2024."

Dr. Marbán, continued, "We continue to progress our proprietary StealthX™ exosome platform technology as part of our strategy to leverage exosomes for therapeutic development. We recently announced a collaboration for our exosome-based multivalent vaccine for the prevention of SARS-CoV-2 with the National Institute of Allergy and Infectious Diseases (NIAID) where they will conduct and fully fund a Phase 1 clinical trial, subject to regulatory approval. In addition, we are engaged in several discussions with potential partners focused on the development of an engineered exosome therapeutic, which would support our goal of building a pipeline of targeted exosome-based therapeutics."

Fourth Quarter 2023 and Recent Operational Highlights

CAP-1002 Duchenne Muscular Dystrophy Program: CAP-1002 is an investigational cell therapy in Phase 3 development for the treatment of DMD. CAP-1002 aims to slow disease progression through immunomodulatory, anti-inflammatory, and anti-fibrotic actions, with the goal of potentially improving skeletal and cardiac muscle function in patients with DMD. HOPE-3, our Phase 3 study, is a multi-center, randomized, double-blind, placebo-controlled clinical trial comprised of two cohorts evaluating the safety and efficacy of CAP-1002 in participants with DMD and impaired skeletal muscle function. The trial is being conducted in the United States. Approximately 102 eligible study subjects will participate in this dual-cohort study (Cohort A and B). CAP-1002 for the treatment of DMD has received Orphan Drug Designation and RMAT (Regenerative Medicine Advanced Therapy Designation). In addition, if Capricor receives FDA marketing approval for CAP-1002 for the treatment of DMD, Capricor would be eligible to receive a Priority Review Voucher (PRV) based on our previous receipt of a rare pediatric disease designation.

- Enrollment has been completed for Cohort A in our Phase 3 trial which enrolled 61 subjects randomized to either CAP-1002 or placebo in a 1:1 ratio.
- Reported a positive outcome from the interim futility analysis for Cohort A which triggered the first milestone payment of $10.0 million under our U.S. Distribution and Commercialization Agreement with Nippon Shinyaku. There is an additional $90.0 million in potential milestone payments up to the time of approval which are triggered upon certain regulatory-based achievements. Following potential approval, there is an additional $605.0 million in potential milestones payments which may be payable to Capricor based on various sales-based targets being met. Further, Capricor will receive a meaningful mid-range double-digit revenue share of product revenue under the terms of this Agreement.
- Next steps for Cohort A: plan to report top-line data in the fourth quarter of 2024.
- Enrollment is underway for Cohort B designed to enroll approximately 44 subjects randomized to either CAP-1002 or placebo in a 1:1 ratio.
- Next steps for Cohort B: expect to complete enrollment in the second quarter of 2024.
- Announced the scale-up to expand manufacturing capacity of CAP-1002 to our new San Diego facility, intended for commercial use, subject to regulatory approval. This facility was designed to be a versatile and cost-effective way to bring CAP-1002 to market efficiently and it is expected that our enhanced manufacturing capacity will

increase our supply capabilities and improve our margins on ultimate product sales, if any. We are currently producing CAP-1002 doses at our San Diego facility for use in Cohort B.

- Announced a positive outcome from a Type-B meeting held with FDA in the third quarter of 2023. In the meeting, the FDA affirmed alignment on the current HOPE-3 clinical trial design comprised of two cohorts and our plan to submit a BLA supported by results from Cohort A which uses product manufactured from our Los Angeles manufacturing facility.
- We plan to meet with FDA in the first quarter of 2024 to continue discussing our pathway to BLA. In the upcoming Type-B meeting, we intend to discuss our further CMC plans for commercial launch, if approved, with an aim to expedite the approval pathway to our BLA filing. Our ultimate goal is to transition to our San Diego manufacturing facility for commercial manufacturing as quickly as possible.
- Hosted a webinar in conjunction with Parent Project Muscular Dystrophy (PPMD) where key updates on our DMD program were outlined.
- Presented a late-breaking poster at the 28th International Annual Congress of the World Muscle Society (WMS). Highlights from the poster included data from the HOPE-2 OLE trial measured by the Performance of the Upper Limb (PUL 2.0) showing a delta change=4.9 points, p=0.021 after 24-months of treatment, compared with the placebo patient group.

Exosome Program: Exosomes are membrane-bound extracellular vesicles which are secreted by most cells and contain characteristic lipids, proteins and nucleic acids such as mRNA and microRNAs. Exosomes act as messengers to regulate the functions of neighboring or distant cells and have been shown to regulate functions such as cell survival, proliferation, inflammation and tissue regeneration. We are developing our exosome technology, using our proprietary StealthX™ platform which is focused on the areas of vaccinology, targeted delivery of oligonucleotides, proteins and small molecule therapeutics to potentially treat and prevent a diverse array of diseases.

- Announced that our proprietary StealthX™ exosome-based multivalent vaccine (StealthX™ vaccine) for the prevention of SARS-CoV-2 was selected to be part of Project NextGen, an initiative by the U.S. Department of Health and Human Services to advance a pipeline of new, innovative vaccines providing broader and more durable protection for COVID-19. As part of Project NextGen, NIAID, part of the National Institutes of Health, will conduct and fund a Phase 1 clinical trial with our StealthX™ vaccine, subject to regulatory approval. Under the terms of the collaboration, Capricor will supply the investigational product and NIAID's Division of Microbiology and Infectious Diseases (DMID) will oversee the trial.
- Next steps for this project: NIAID plans to initiate the Phase 1 clinical trial in late 2024, subject to regulatory approval. If NIAID finds that our StealthX™ vaccine meets its criteria for safety and efficacy, they may consider our program for a funded Phase 2.
- Currently, in collaboration with an undisclosed pharmaceutical company, we are also investigating the therapeutic application of our StealthX™ exosome platform.
- Presented a late-breaking poster at the WMS on the application of our StealthX™ exosome platform for the delivery of antisense oligonucleotides (ASO). Highlights from the poster included data showing the presence of exosomes loaded with a labeled ASO in the lower limbs of mice 24 hours post-intravenous (IV) injection. Notably, the

exosomes carrying the muscle-targeting moiety were not detected in any other tissues except for the expected clearance pathways (kidney and liver) with a single dose.

Corporate Updates

- Announced receipt of our first milestone payment of $10.0 million under our U.S. Distribution and Commercialization Agreement with Nippon Shinyaku.
- Announced completion of a registered direct offering with participation from Nippon Shinyaku for gross proceeds of approximately $23.0 million.

Anticipated Upcoming Milestones

CAP-1002 Duchenne Muscular Dystrophy Program

- Plan to have a Type-B meeting with FDA in the first quarter of 2024 to discuss commercial manufacturing planning with an aim to expedite the BLA pathway.
- Plan to report 3-year HOPE-2 OLE data in the second quarter of 2024.
- Plan to complete HOPE-3 (Cohort B) enrollment in the second quarter of 2024.
- Plan to report topline data from HOPE-3 (Cohort A) in the fourth quarter of 2024.
- Continue to explore opportunities for additional partnerships outside of the U.S. and Japan to support the potential commercialization of CAP-1002 in DMD.

Exosome Program

- Subject to regulatory approval, plan to announce IND clearance for our StealthX™ vaccine for the prevention of SARS-CoV-2.
- Plan to provide updates on our NIAID collaboration for our StealthX™ vaccine as they become available.
- Continue to explore opportunities for partnerships and non-dilutive sources of funding to support advancement of our StealthX™ exosome platform technology.

Fourth Quarter and Full Year 2023 Financial Results

Cash position: The Company's cash, cash equivalents and marketable securities totaled approximately $39.5 million as of December 31, 2023 compared to approximately $41.4 million on December 31, 2022. In the fourth quarter of 2023, Capricor raised approximately $1.5 million in net proceeds through issuances of common stock at an average price of approximately $4.47 per share under its at-the-market offering program. Additionally in January 2024, the Company received $10.0 million from the first milestone payment under our U.S. Distribution and Commercialization Agreement with Nippon Shinyaku.

Revenues: Capricor's primary source of revenue was from the ratable recognition of the $40.0 million (upfront and milestone payments) in accordance with its U.S. Commercialization and Distribution Agreement with Nippon Shinyaku. Revenues for the fourth quarter of 2023 were approximately $12.1 million compared with approximately $1.0 million for the fourth quarter of 2022.

Operating expenses: Total operating expenses for the fourth quarter of 2023 were approximately $13.2 million compared with approximately $9.0 million for the fourth quarter of 2022.

Net loss: The Company reported a net loss of approximately $0.8 million, or $0.02 per share, for the fourth quarter of 2023, compared to a net loss of approximately $7.7 million, or $0.31 per share, for the fourth quarter of 2022. For the year ended December 31, 2023, the Company reported a net loss of approximately $22.3 million, or $0.83 per share, compared to a net loss of approximately $29.0 million, or $1.18 per share, for the year ended December 31, 2022.

Financial Outlook

Capricor believes that based on the current operating plan and financial resources, its available cash, cash equivalents and marketable securities will be sufficient to cover anticipated expenses and capital requirements into the first quarter of 2025. This expectation excludes any additional potential milestone payments under its exclusive commercialization and distribution agreements with Nippon Shinyaku, as well as any strategic use of capital not currently in the Company's base-case planning assumptions.

Conference Call and Webcast

To participate in the conference call, please dial 1-888-886-7786 (Domestic/Toll-Free) or 1-416-764-8658 (International) and reference the conference ID: 83986877. Participants can use guest dial-in numbers above and be answered by an operator or click the Call me™ link for instant telephone access. To participate via a webcast, please click here. A replay of the webcast will be available following the conclusion of the live broadcast and will be accessible on the Company's website.

About Capricor Therapeutics

Capricor Therapeutics, Inc. (NASDAQ: CAPR) is a biotechnology company dedicated to advancing transformative cell and exosome-based therapeutics to redefine the treatment landscape for rare diseases. At the forefront of our innovation is our lead product candidate, CAP-1002 — an allogeneic cardiac-derived cell therapy. Extensive preclinical and clinical studies have shown CAP-1002 to demonstrate immunomodulatory, antifibrotic, and regenerative actions specifically tailored for dystrophinopathies and heart disease. CAP-1002 is currently advancing through Phase 3 clinical development for the treatment of Duchenne muscular dystrophy (DMD). Capricor is also harnessing the power of our exosome technology, using our proprietary StealthX™ platform which is focused on the areas of vaccinology, targeted delivery of oligonucleotides, proteins and small molecule therapeutics to potentially treat and prevent a diverse array of diseases. At Capricor, we stand committed to pushing the boundaries of possibility and forging a path toward transformative treatments for those in need. For more information, visit capricor.com, and follow Capricor 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; 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 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, 2022, as filed with the Securities and Exchange Commission on March 17, 2023 and in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2023, as filed with the Securities and Exchange Commission on November 14, 2023. 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.

Capricor has entered into a partnership for the exclusive commercialization and distribution of CAP-1002 for DMD in the United States and Japan with Nippon Shinyaku Co., Ltd. (U.S. subsidiary: NS Pharma, Inc.), subject to regulatory approval. 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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CAPRICOR THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(UNAUDITED)

	Three months ended December 31,		Year ended December 31,	
	2023	**2022**	**2023**	**2022**
REVENUE				
Revenue	$12,088,089	$ 959,903	$ 25,178,066	$ 2,551,469

TOTAL REVENUE	12,088,089	959,903	25,178,066	2,551,469
OPERATING EXPENSES				
Research and development	9,940,167	6,231,806	36,448,039	21,816,949
General and administrative	3,210,947	2,794,442	12,589,619	10,431,903
TOTAL OPERATING EXPENSES	13,151,114	9,026,248	49,037,658	32,248,852
LOSS FROM OPERATIONS	(1,063,025)	(8,066,345)	(23,859,592)	(29,697,383)
OTHER INCOME (EXPENSE)				
Other income	67,657	-	67,657	190,582
Investment income	233,932	379,699	1,510,434	521,535
Loss on disposal of fixed assets	(653)	(34,266)	(6,041)	(34,266)
TOTAL OTHER INCOME (EXPENSE)	300,936	345,433	1,572,050	677,851
NET LOSS	(762,089)	(7,720,912)	(22,287,542)	(29,019,532)
OTHER COMPREHENSIVE INCOME (LOSS)				
Net unrealized gain (loss) on marketable securities	122,605	(17,223)	130,569	105,244
COMPREHENSIVE LOSS	$ (639,484)	$ (7,738,135)	$(22,156,973)	$(28,914,288)
Net loss per share, basic and diluted	$ (0.02)	$ (0.31)	$ (0.83)	$ (1.18)
Weighted average number of shares, basic and diluted	30,664,100	25,163,711	26,778,360	24,552,688

CAPRICOR THERAPEUTICS, INC.
SUMMARY BALANCE SHEETS

	December 31, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$39,487,703	$41,421,262
Total assets	$58,734,327	$50,094,910

Total liabilities	$36,132,860	$38,308,816
Total stockholders' equity - 31,148,320 and 25,241,402 common shares issued and outstanding at December 31, 2023 and December 31, 2022, respectively	22,601,467	11,786,094
Total liabilities and stockholders' equity	$58,734,327	$50,094,910

Source: Capricor Therapeutics