April 24, 2024

Capricor Therapeutics Announces Positive Type-B Meeting with FDA for CAP-1002 Program for Duchenne Muscular Dystrophy

-Company Aligned with FDA on Demonstration of Non-Clinical Comparability; Allowing for Immediate Use of San Diego Manufacturing Facility-

-FDA Feedback Supports Requests for a Pre-BLA Meeting and Subsequent Rolling BLA Submission Following Upcoming Q2 Type-B FDA Meeting-

-Company Granted Subsequent Type-B Clinical Meeting with FDA in Second Quarter to Continue to Discuss Pathway to BLA-

-Capricor Management to Host Virtual Investor Webcast to Discuss Latest Program Updates on Monday, April 29 at 8:30 a.m. ET-

SAN DIEGO, April 24, 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, today announced an update from the Company's recent Type-B Chemistry, Manufacturing and Controls ("CMC") meeting with the U.S. Food and Drug Administration ("FDA") on next steps for the Biologics License Application ("BLA") submission with its lead asset CAP-1002 in treating Duchenne muscular dystrophy ("DMD").

The FDA has affirmed alignment with Capricor on the following topics:

Pre-BLA Meeting and Rolling BLA Submission

- The FDA advised Capricor to include discussion for a pre-BLA meeting and rolling BLA schedule in the upcoming Type-B meeting.
    - Based on this feedback, Capricor has already been granted a subsequent Type-B meeting to be held in the second quarter of 2024 to discuss these topics, with the results of those discussions to potentially lead to an accelerated BLA filing.
    - Capricor plans to share with FDA its HOPE-2 open label extension ("OLE") 3-year safety and efficacy data which is expected to be available in the second quarter of 2024 as part of Capricor's ISS and ISE strategy.

Establishment of Non-Clinical Comparability

- The FDA agreed that comparability between drug product manufactured at our two different facilities (Los Angeles and San Diego) has been demonstrated using the

provided analytical comparability data.
    - This allows for the use of CAP-1002 drug product manufactured at our San Diego manufacturing facility upon potential product approval.
    - Data from Cohort B of the HOPE-3 clinical trial will not be necessary for FDA approval of the product.

"I am extremely pleased with our recent FDA interactions as we continue to work collaboratively with the agency to align on the most expeditious path towards registration of CAP-1002 for the treatment of DMD," said Linda Marbán, Ph.D., Capricor's chief executive officer. "Capricor has generated extensive safety and efficacy data in multiple clinical trials and we are very encouraged by the FDA's agreement that we have successfully demonstrated product comparability which allows for a seamless transition to our San Diego manufacturing facility without the need for additional clinical data. Contingent upon our upcoming meeting, FDA is supportive of our plan to submit a rolling BLA, which may expedite our path to potential approval. In addition, establishment of non-clinical comparability allows us to be able to conserve resources and focus on preparing our facility in San Diego for potential launch. Furthermore, we continue to work diligently with our partner, Nippon Shinyaku (U.S. subsidiary: NS Pharma, Inc.) as we prepare for the potential launch of CAP-1002. Looking ahead, later this quarter, we remain on track to report the 3-year HOPE-2 OLE results as well as reporting the outcome of our next FDA meeting."

CAP-1002 for the treatment of DMD has received Orphan Drug Designation and the regulatory pathway for CAP-1002 is supported RMAT (Regenerative Medicine Advanced Therapy Designation). In addition, if Capricor were to receive FDA marketing approval for CAP-1002 for the treatment of DMD, Capricor would be eligible to receive a Priority Review Voucher ("PRV") based on its previous receipt of a rare pediatric disease designation. Capricor retains full rights to the PRV, if received.

Virtual Investor Webcast and Conference Call

Capricor management will host a virtual investor webcast and conference call with slides on Monday, April 29, 2024, at 8:30 a.m. ET. To participate in the conference call, please dial 888-886-7786 (domestic/toll-free) or 416-764-8658 (international) and reference the conference ID: 34112256. Participants can use guest dial-in numbers above to be answered by an operator or click the Call me™ link for instant telephone access. To participate via webcast, please click here to view the slides. 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 in preclinical development

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, 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.

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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Source: Capricor Therapeutics