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Capricor Therapeutics to Meet with FDA to Discuss CAP-1002 to Treat Duchenne Muscular Dystrophy

Company Exploring Potential for Accelerated Approval under RMAT Designation, an Expedited Program for Regenerative Therapies

LOS ANGELES, Sept. 24, 2019 (GLOBE NEWSWIRE) -- Capricor Therapeutics (NASDAQ: CAPR), a clinical-stage biotechnology company focused on the development of first-in-class biological therapeutics for the treatment of Duchenne muscular dystrophy (DMD) and other rare disorders, announced today that it has been granted a Type B End-of-Phase 2 meeting with the FDA to discuss prespecified interim analysis of the Company's HOPE 2 clinical trial, which was reported in July of this year. The trial is a double-blind, placebo-controlled study of the company's therapy, CAP-1002, in steroid-treated boys and young men in the later stages of DMD, a fatal genetic disease with few treatment options. The pre-specified interim analysis was performed on the six-month follow-up.

Per FDA's interpretation of Section 506(g) of the federal Food, Drug and Cosmetic Act, which was added by the 21st Century Cures Act enacted in 2016, therapies with an RMAT designation may be eligible for accelerated approval based on previously agreed-upon intermediate endpoints that are reasonably likely to predict long-term clinical benefit.

"The data showed positive results across several independent measures, suggesting functional improvement in treated patients and providing the best evidence we've seen of clinical improvement in later stage Duchenne patients, with the exception of the use of steroids," said Linda Marbán, CEO of Capricor. "We will be discussing the significance of this data and the next steps in our CAP-1002 program with the FDA. Our goal is to identify pathways to get this important therapy to DMD patients as soon as we can and we believe the FDA's written guidance regarding accelerated approvals for therapies, like CAP-1002, that have the Regenerative Medicine Advanced Therapy (RMAT) designation provides the framework for these discussions."

The interim analysis of the six-month follow-up in HOPE-2 are consistent with the positive results seen in Capricor's HOPE-Duchenne Phase I/II clinical trial which was published in *Neurology*, the medical journal of the American Academy of Neurology, earlier this year.

The FDA has granted Capricor's CAP-1002 RMAT and Orphan Drug Designation, for which the FDA has also granted a Rare Pediatric Disease Designation. The Rare Pediatric Disease Designation, as well as the Orphan Drug Designation previously granted, covers the broad treatment of DMD. If Capricor were to receive market approval for CAP-1002 by the FDA, Capricor would be eligible to receive a Priority Review Voucher.

Capricor expects to announce further updates on its clinical development strategy for CAP-1002 in DMD later this year, including an update on guidance it receives from the FDA.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a devastating genetic disorder that causes muscle degeneration and leads to death, generally before the age of 30, most commonly from heart failure. It occurs in one in every 3,600 live male births across all races, cultures and countries. Duchenne muscular dystrophy afflicts approximately 200,000 boys and young men around the world. Treatment options are limited, and there is no cure.

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 immuno-modulatory 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 150 human subjects across several clinical trials.

About Capricor Therapeutics

Capricor Therapeutics, Inc. (NASDAQ: CAPR) is a clinical-stage biotechnology company focused on the discovery, development and commercialization of first-in-class biological therapeutics for the treatment of rare disorders. Capricor's lead candidate, CAP-1002, is an allogeneic cell therapy that is currently in clinical development for the treatment of DMD. Capricor is also exploring the potential of CAP-2003, a cell-free, exosome-based candidate, to treat a variety of disorders. For more information, visit www.capricor.com.

Keep up with Capricor on social media:www.facebook.com/capricortherapeutics, www.instagram.com/capricortherapeutics/ and https://twitter.com/capricor

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, 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, in its Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2019, as filed with the Securities and Exchange Commission on August 8, 2019, and in its Registration Statement on Form S-3 as filed with the Securities and Exchange Commission on October 24, 2018, and as amended by its Amendment No. 1 to Form S-3 filed with the Securities and Exchange Commission on July 17, 2019, together with prospectus supplements thereto. 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. CAP-2003 has not yet been approved for clinical investigation.

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