

October 21, 2019



# Capricor Therapeutics to Host Key Opinion Leader Call on the Role of CAP-1002 for the Treatment of Duchenne Muscular Dystrophy (DMD)

*Call Scheduled for Thursday, October 24<sup>th</sup> at 10:30am Eastern Time*

LOS ANGELES, Oct. 21, 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 will host a Key Opinion Leader (KOL) call on the role of CAP-1002 in the of treatment of Duchenne Muscular Dystrophy (DMD) on Thursday, October 24<sup>th</sup> at 10:30am Eastern Time.

The call will feature a presentation by KOL Craig M. McDonald, MD, UC Davis Health, and principal investigator of Capricor's HOPE-2 trial who will provide an overview of Duchenne Muscular Dystrophy (DMD), its progression, current treatment options, and new treatments in development for the disease. Dr. McDonald will be available to answer questions at the conclusion of the call.

Capricor's management team will also provide an overview of the Company's lead candidate, CAP-1002, a cell therapy that is currently in clinical development for the treatment of Duchenne muscular dystrophy (DMD). CAP-1002 consists of allogeneic cardiosphere-derived cells, or CDCs, a unique population of cells that contains cardiac progenitor cells. CAP-1002 has been shown to exert potent immunomodulatory activity and alter the immune system's activity to encourage cellular regeneration. Data from the pre-specified interim analysis demonstrated that teens and young men in the advanced stages of DMD saw improvements in skeletal, pulmonary, and cardiac measurements after receiving multiple doses of CAP-1002. Specifically, patients showed improvements in the Performance of the Upper Limb (PUL), a tool specifically designed for assessing high (shoulder), mid (elbow) and distal (wrist & hand) function, with a conceptual framework reflecting the progression of weakness in ambulant and non-ambulant patients.

The FDA has granted Capricor's CAP-1002 (Regenerative Medicine Advanced Therapy Designation) [RMAT](#) and [Orphan Drug Designation](#), for which the FDA has also granted a Rare Pediatric Disease Designation.

Craig M. McDonald, MD is currently a Professor of Medicine and the Chair of the Department of Physical Medicine & Rehabilitation at UC Davis Health. He received his M.D. and M.R.M. from the University of Washington School of Medicine as well as his A.B. in Human Biology from Stanford University. Dr. McDonald is also board-certified in

neuromuscular medicine and pediatric rehabilitation medicine. He is an internationally recognized expert in clinical management, rehabilitation, and precision therapeutics for children and adults with neuromuscular diseases. Dr. McDonald has been a pioneer in the development of novel outcome measures for clinical trials focused on disabled populations. He is widely known for his expertise in the treatment and evaluation of children and young adults with Duchenne muscular dystrophy and other neuromuscular diseases. Dr. McDonald serves as director and principal investigator of UC Davis' successfully renewed NINDS-funded site in the NeuroNEXT Neurosciences Clinical Trials National Consortium (one of two NeuroNEXT sites on the West Coast). Dr. McDonald is also the director of rehabilitation services at Shriners Hospital for Children - Northern California.

**Conference Call – Thursday, October 24<sup>th</sup> at 10:30amET/7:30amPT**

Domestic: 877-407-0792

International: 201-689-8263

Conference ID: 13695624

Webcast: [https://viaavid.webcasts.com/starthere.jsp?ei=1266685&tp\\_key=bc6b2e6ed4](https://viaavid.webcasts.com/starthere.jsp?ei=1266685&tp_key=bc6b2e6ed4)

**About HOPE-2**

HOPE-2 is a randomized, double-blind, placebo-controlled, Phase II clinical trial of the company's lead investigational therapy, CAP-1002 in steroid-treated boys and young men who are in advanced stages of DMD. The study protocol called for treatment via intravenous delivery with either CAP-1002 (150 million cells per infusion) or placebo every 3 months.

**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 proprietary population of cells that contains cardiac progenitor cells 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 approximately 150 human subjects across several clinical trials.

**About Capricor Therapeutics**

Capricor Therapeutics, Inc. (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 Duchenne muscular dystrophy. Capricor is also exploring the potential of CAP-2003, a cell-free, exosome-based candidate, to treat a variety of disorders. HOPE-Duchenne, Capricor's Phase I/II trial was funded in part by the California Institute for Regenerative Medicine. For more information, visit [www.capricor.com](http://www.capricor.com).

Keep up with Capricor on social media: [www.facebook.com/capricortherapeutics](https://www.facebook.com/capricortherapeutics), [www.instagram.com/capricortherapeutics/](https://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.

For more information, please contact:

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Source: Capricor Therapeutics, Inc.