July 15, 2019

Capricor Therapeutics Announces Positive Results from its Interim Analysis in the HOPE-2 Trial to Treat Patients with Duchenne Muscular Dystrophy

--Interim Analysis Showed Statistically Significant Improvements in the Performance of the Upper Limb, Grip Strength and Inspiratory Flow Reserve in the Randomized Double-Blind Placebo Controlled Study --

--Company to Hold Conference Call Today at 5:00 AM PT / 8:00 AM ET--

LOS ANGELES, July 15, 2019 (GLOBE NEWSWIRE) -- Capricor Therapeutics, Inc. (NASDAQ: CAPR), a clinical-stage biotechnology company, today announced that a pre-specified interim analysis performed on 6-month data from the HOPE-2 trial showed statistically significant results across several independent clinical measures.

“I am incredibly pleased with the outcome of the interim analysis as it has demonstrated the biologic activity of CAP-1002 that has resulted in changes of clinically relevant outcomes including the upper limb, the hand and diaphragmatic function,” said Craig McDonald, M.D., the national principal investigator for the HOPE-2 clinical trial and UC Davis professor and chair of its Department of Physical Medicine and Rehabilitation. “For these older boys who have no further therapeutic options, these data support the hope that CAP-1002 may one day become an important therapeutic option and possibly slow the advancement of the disease.

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 Duchenne muscular dystrophy (DMD), a debilitating genetic disorder. DMD is characterized by progressive weakness and chronic inflammation of the skeletal, heart, and respiratory muscles. Study patients were treated via intravenous delivery with either CAP-1002 (150 million cells per infusion) or placebo every 3 months.

In the interim analysis, top-line data from a total of 17 patients was analyzed in the per protocol population (10 placebo and 7 treated) at the 3 month time-point and 12 patients (6 placebo and 6 treated) were analyzed at the 6 month time-point. Approximately 80% of the patients were non-ambulant. Demographic and baseline characteristics were similar between the two treatment groups.

Skeletal Assessments

To assess skeletal muscle function, investigators used the mid-level dimension of the Performance of the Upper Limb (PUL) 1.2 and 2.0 tools. The PUL evaluates manual tasks that relate to activities of daily living that are very important for quality of life. The U.S. Food and Drug Administration (FDA) has suggested the use of the updated PUL 2.0 version as the primary efficacy endpoint in support of a Biologics License Application (BLA). Positive results were seen in the PUL 1.2 version which is consistent with the positive results seen in Capricor's HOPE-Duchenne Phase I/II clinical trial published in *Neurology*, the medical journal of the American Academy of Neurology. Additional independent tests assessing grip strength showed statistically significant results at 6 months and tests assessing tip to tip pinch strength showed positive results.

Time-point	**3 months**			**6 months**		
Treatment	**CAP-1002**	**Placebo**	**p-value**	**CAP-1002**	**Placebo**	**p-value**
Mid-level Performance of the Upper Limb PUL 2.0	0.1 (1.07)	-0.4 (0.52)	0.0591	-0.2 (1.17)	-0.8 (0.75)	0.0389
Tip to Tip Pinch Strength	0.9 (3.44)	1.9 (4.12)	0.9057	3.3 (2.88)	-0.3 (1.51)	0.0674
Grip Strength	-0.6 (3.15)	-0.8 (2.30)	0.5897	0.8 (4.54)	-2.2 (1.83)	0.0389

Table: Skeletal Assessments at 3- and 6-month time-points.
Mean Change from baseline (standard deviation) showed.

Pulmonary Assessments

To assess pulmonary function, investigators measured several clinically relevant parameters. At 3 months, inspiratory flow reserve (absolute), a reflection of diaphragmatic strength, showed a statistically significant improvement (p=0.0473). Additionally, positive trends were seen at 3 months in peak expiratory flow (% predicted), another measure of diaphragmatic strength.

Cardiac Assessments

Magnetic resonance imaging (MRI) was used to assess cardiac structure and function at 6 months. Positive trends were found in cardiac muscle function including systolic wall thickening and cardiac mass among those treated with CAP-1002 compared to placebo. Duchenne hearts atrophy progressively and have impaired systolic function. Improved mass and wall thickening suggest possible cardiac regeneration and functional improvement. Although these trends did not reach statistical significance, they were consistent with the cardiac findings seen in the previously published HOPE-Duchenne study.

Safety

In late December 2018, Capricor put a voluntary hold on dosing after two patients in the HOPE trials had a serious adverse event in the form of an immediate immune reaction. The investigation suggested the patient may have developed hypersensitivity to something contained in the investigational product, including an excipient or inactive ingredient in the formulation. To reduce the risk of future adverse events, Capricor initiated a commonly used

pre-medication strategy including intravenous steroids and antihistamines to prevent or mitigate potential immune reactions during the administration. Since the initiation of the pre-treatment regimen, 30 infusions of investigational drug (CAP-1002 or placebo) have been administered to HOPE-2 patients with only one serious adverse event reported that required an overnight observation of the patient.

Summary

In summary, a statistically significant outcome relative to placebo controls was shown in PUL 2.0 at 6 months, with supportive, positive treatment effects also seen in some independent skeletal and pulmonary assessments. Positive trends, although not statistically significant, were observed in other skeletal, pulmonary and cardiac measures.

Although we have collected data from 2 treated patients at the 9-month timepoint, 1 of which at the 12-month time-point, Capricor is not able to draw any conclusions at this time with respect to this data.

“We are extremely pleased and it is truly extraordinary that even in such as small sample size, we achieved statistically significant improvements in several clinically relevant parameters. In these older patients, functional improvement in the upper limb is highly meaningful for their quality of life. To our knowledge, this is the first randomized double-blind, placebo-controlled study in DMD that has shown statistically significant functional improvement in steroid treated boys.” said Linda Marbán, president and CEO of Capricor.

Pat Furlong, Founding President and CEO of Parent Project Muscular Dystrophy, a nonprofit organization leading the fight to end Duchenne said, "I am encouraged by the prospects that this data has for people with Duchenne, especially our non-ambulatory community who have limited therapeutic options. Additionally, CAP-1002 potentially provides cardiac benefits in this patient population where heart failure continues to be the leading cause of mortality."

The FDA has granted Capricor RMAT and Orphan Drug Designation. Capricor met with the FDA in December 2018 as part of the expedited review afforded under the RMAT designation which the FDA granted to CAP-1002 in February 2018. Additionally, the FDA has granted a Rare Pediatric Disease Designation to CAP-1002. The Rare Pediatric Disease Designation, as well as the Orphan Drug Designation previously granted, covers the broad treatment of DMD. Upon receiving market approval for CAP-1002 by the FDA, Capricor would be eligible to receive a Priority Review Voucher.

Capricor will continue its ongoing discussions with the FDA about the DMD program and future plans.

Conference Call and Webcast

Capricor will host a conference call and webcast with slides today, July 15, 2019, at 8:00 a.m. ET to discuss the top-line interim results of the HOPE-2 clinical trial.

To participate in the conference call, please dial 866-717-4562 (domestic) or 210-874-7812 (international) and reference the access code: 1081257

To participate via a webcast, please visit: https://edge.media-server.com/mmc/p/y9iygowj to view the slides. The webcast will be archived for approximately 30 days and will be available

at http://capricor.com/news/events/.

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 approximately 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 Duchenne muscular dystrophy. Capricor is also exploring the potential of CAP-2003, a cell-free, exosome-based candidate, to treat a variety of disorders. The HOPE-Duchenne trial was funded in part by the California Institute for Regenerative Medicine. 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, 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 and in its Quarterly Report on Form 10-Q as filed with the Securities and Exchange Commission on May 14, 2019. 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:

AJ Bergmann, Chief Financial Officer
+1-310-358-3200
abergmann@capricor.com

Source: Capricor Therapeutics, Inc.