June 28, 2024

Capricor Therapeutics Announces Long-Term Benefit of Deramiocel (CAP-1002) in Both Skeletal Muscle and Cardiac Function in the HOPE-2 OLE Study in Duchenne Muscular Dystrophy

-Results in Performance of the Upper Limb (PUL v2.0) Continue to Show Slowing of Disease Progression in Later-Stage DMD Patients-

-Improvements Seen in Multiple Cardiac Endpoints Demonstrating Stabilization of Cardiac Function Over 3 Years of Treatment-

-Pre-BLA Meeting with FDA Scheduled for Q3 2024 to Discuss Options to Expedite BLA Filing-

-Results to be Presented at PPMD Annual Conference on June 29 in Session titled "Approaches to Alter Progression"-

SAN DIEGO, June 28, 2024 (GLOBE NEWSWIRE) -- Capricor Therapeutics (NASDAQ: CAPR), a biotechnology company developing transformative cell and exosome-based therapeutics for the treatment of rare diseases, today announced additional positive 3-year safety and efficacy results from its ongoing HOPE-2 open label extension (OLE) study with its lead asset, deramiocel (CAP-1002), for the treatment of Duchenne muscular dystrophy (DMD).

Data from the HOPE-2 OLE study demonstrated improvements in multiple cardiac measures, including left ventricular ejection fraction (LVEF), as well as indexed volumes (left ventricular end systolic volume (LVESV) and left ventricular end diastolic volume (LVEDV)). These are measures of cardiac function and are considered highly relevant in terms of predicting long-term outcomes. In addition, greater improvements in cardiac function were observed in those patients that had higher ejection fractions (> 45%) at the beginning of the HOPE-2 randomized trial. Published data supports the need for early intervention in order to maintain function and potentially slow the progression of the cardiomyopathy, one of the leading causes of death in patients with DMD. Currently, there is no approved treatment specifically for DMD cardiomyopathy, which underscores the need for additional therapies to treat DMD. Additionally, as previously reported, patients showed a statistically significant benefit (+3.7 points, $p< 0.001$) in the PUL v2.0 total score when compared to an external comparator dataset of similar DMD patients. The HOPE-2 OLE study continues to show a favorable safety profile for long-term treatment of deramiocel. These data will be shared at this year's Parent Project Muscular Dystrophy (PPMD) 30th Annual Conference being held in

Orlando, Florida on June 29, 2024.

3-Year HOPE-2 OLE Study Results

Primary endpoint Skeletal muscle (upper-limb function)	**3-year timepoint Change from baseline***		**Delta change p-value**
Performance of upper limb (PUL v2.0)	**Deramiocel (n=12)**	**External comparator (n=32)**	
	-4.1 points	-7.8 points	+3.7 points p< 0.001

External comparator PUL data provided by Cincinnati Children's Hospital Medical Center (CCHMC)
*Baseline is referred to as start of HOPE-2 OLE study, changes in means are shown

Secondary endpoints Cardiac function	**3-year timepoint Change from baseline***	
	Deramiocel (n=10)**	**Deramiocel (n=8)** *Patients with >45% LVEF at end of HOPE-2*
Left ventricular ejection fraction (LVEF %) *Positive value indicates improvement*	+1.2%	+3.0%
Indexed volumes *Negative value indicates improvement*		
LV end systolic volume, indexed (mL/m^2)	-2.4	-5.1
LV end diastolic volume, indexed (mL/m^2)	-5.7	-10.0

*Baseline is referred to as start of HOPE-2 study, changes in medians are shown; 3-year timepoint on HOPE-2 OLE corresponds to 5 years post HOPE-2 Baseline
All cardiac outcomes were measured using magnetic resonance imaging (cMRI)
**10 of 12 participants were able to receive MRI

"The results of the open label study are tremendously important for DMD patients, as they showed sustained skeletal and cardiac benefits after 3 years of continuous treatment with deramiocel, which underscores the potential long-term benefits this therapy can offer patients with DMD," said Linda Marbán, Ph.D., Capricor's chief executive officer. "Based on our HOPE-2 OLE data and prior clinical results, we plan to discuss with the U.S. Food and Drug Administration (FDA) options to expedite our Biologics License Application (BLA) filing. We continue to work closely with FDA with the goal of bringing deramiocel to patients as quickly as possible and look forward to sharing further updates as they become available. We thank the patients, their families and the broader Duchenne community for continuing to work with us throughout the development of this promising therapy."

The PPMD Annual Conference is the largest annual, international event focused on the latest research, clinical trials and care initiatives for DMD. Now in its 30th year, the meeting is attended by researchers, caregivers and patients looking to interact and drive change in the fight to end DMD. For more information, or to register, please click here. Capricor plans to make its presentation available on the publications section of the Company's website following the formal conference presentation.

About HOPE-2 Open Label Extension (OLE) Study

HOPE-2 was a randomized, double-blind, placebo-controlled, Phase 2 clinical study of Capricor's lead investigational therapy, deramiocel, in boys and young men who have DMD. Study patients were treated via intravenous delivery with either deramiocel (150 million cells per infusion) or placebo every 3 months. Data from a total of 20 patients was analyzed (12 placebo and 8 treated) at the 12-month time-point and the results were published in The Lancet. After the completion of the HOPE-2 study, all patients stopped treatment for approximately 392 days (mean, range [239, 567]), which is referred to as the gap phase. Then all eligible patients who wished to remain on treatment entered the HOPE-2-OLE study where they receive deramiocel (150 million cells per infusion) every three months. The HOPE-2-OLE study previously met its primary endpoint at the one-year timepoint on the PUL v2.0 (p=0.02). The HOPE-2-OLE study remains ongoing and into its fourth year and participants continue to be monitored for safety, cardiac and functional performance.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a devastating genetic disorder characterized by progressive weakness and chronic inflammation of the skeletal, heart and respiratory muscles with mortality at a median age of approximately 30 years. It is estimated that DMD occurs in approximately one in every 3,500 male births and that the patient population is estimated to be approximately 15,000-20,000 in the United States. DMD pathophysiology is driven by the impaired production of functional dystrophin, which normally functions as a structural protein in muscle. The reduction of functional dystrophin in muscle cells leads to significant cell damage and ultimately causes muscle cell death and fibrotic replacement. Treatment options are limited and there is no cure.

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, deramiocel (CAP-1002), an allogeneic cardiac-derived cell therapy. Extensive preclinical and clinical studies have shown deramiocel to demonstrate immunomodulatory, antifibrotic, and regenerative actions specifically tailored for dystrophinopathies and heart disease. Deramiocel 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 revenue streams and 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, and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2024, as filed with the Securities and Exchange Commission on May 14, 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 an agreement for the exclusive commercialization and distribution of deramiocel (*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. Deramiocel 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:

Capricor Company Contact:
AJ Bergmann, Chief Financial Officer
abergmann@capricor.com
858.727.1755

Source: Capricor Therapeutics