

November 8, 2017



# **Capricor Therapeutics Reports Third Quarter 2017 Financial Results and Provides Update on Duchenne Muscular Dystrophy Development Program**

**12-Month Results from HOPE Clinical Trial to be Presented Next Week at AHA**

**Potential Registration Trial Planned to Initiate in the First Quarter of 2018**

**Company to Host Conference Call and Webcast Today at 4:30 p.m. ET**

LOS ANGELES, Nov. 8, 2017 /PRNewswire/ --[Capricor Therapeutics, Inc.](#) (NASDAQ: CAPR) today announced its financial results for the third quarter ended September 30, 2017 and provided an update on its development of CAP-1002 for the treatment of Duchenne muscular dystrophy (DMD).

"Our team has been busy with preparations to launch the HOPE-2 clinical trial of CAP-1002, for which we recently submitted an Investigational New Drug application (IND) to the FDA. We are actively building our organization in key functional areas as we continue to advance this candidate toward potential registration," said Linda Marbán, Ph.D., president and chief executive officer. "In the near-term, we look forward to the 12-month results from our HOPE clinical trial to be presented at the upcoming AHA meeting."

## **Third Quarter 2017 and Recent Highlights**

- Recently submitted an IND to the U.S. Food and Drug Administration (FDA) for the planned randomized, double-blind, placebo-controlled HOPE-2 clinical trial to evaluate CAP-1002 in patients with DMD.
- Announced that Craig M. McDonald, M.D., an internationally-recognized leader in the clinical management of neuromuscular diseases, has been named as national principal investigator of the planned HOPE-2 clinical trial.
- Presented data supporting the intravenous delivery of CAP-1002, which demonstrated increased exercise capacity and diaphragm function, as well as decreased cardiac scar, in an animal model of DMD, at the Cell and Gene Meeting on the Mesa in La Jolla, California.
- Presented positive six-month data from the randomized Phase I/II HOPE clinical trial of CAP-1002 in boys and young men with DMD in a late-breaking session of the 22<sup>nd</sup> International Congress of the World Muscle Society in Saint-Malo, France, which showed that patients treated with intracoronary CAP-1002 demonstrated improvement

in skeletal muscle function according to the Performance of the Upper Limb (PUL) test as well as improvement in cardiac muscle function.

- Announced the U.S. Food and Drug Administration's willingness to accept the PUL as the basis for the primary efficacy endpoint for clinical studies intended to support the potential registration of CAP-1002.
- Announced that CAP-1002 had been granted Rare Pediatric Disease Designation by the FDA.

### **Anticipated Events and Milestones**

- 12-month results from the Phase I/II HOPE clinical trial of CAP-1002 in DMD to be presented at a late-breaking session of the American Heart Association (AHA) Scientific Sessions 2017 in Anaheim, California on Nov. 15. Capricor will hold a conference call and webcast to review these results with physician investigators on Nov. 15 at 1:30 p.m. PT / 4:30 p.m. ET.
- To present the poster "Cardiosphere-Derived Cell and Mesenchymal Stem Cell Extracellular Vesicles Contain Distinct RNA Cargo" at the AHA Scientific Sessions 2017.
- To present the poster "CDCs Show a Strong Immunomodulatory Activity and Improve Muscle Physiology When Systemically Delivered" at the ActionDuchenne International Conference to be held in Birmingham, UK.
- Results from the Phase II ALLSTAR clinical trial of CAP-1002 in post-myocardial infarction to be presented at a late-breaking session of the AHA Scientific Sessions 2017 on Nov. 15.
- Plan to initiate the randomized, double-blind, placebo-controlled HOPE-2 clinical trial of intravenous, repeat-dose CAP-1002 in DMD in the first quarter of 2018, subject to regulatory approval.
- Plan to submit an Investigational New Drug application (IND) for CAP-2003 (cardiosphere-derived cell exosomes) for the treatment of hypoplastic left heart syndrome in 2018.

### **Third Quarter Financial Results**

The Company reported a net loss of approximately \$2.7 million, or \$0.12 per share, for the third quarter of 2017, compared to a net loss of approximately \$5.3 million, or \$0.29 per share, for the third quarter of 2016.

As of September 30, 2017, the Company's cash, cash equivalents and marketable securities totaled approximately \$13.9 million, compared to approximately \$16.2 million on December 31, 2016. Capricor believes that its current financial resources should be sufficient to fund its operations and meet its financial obligations through the third quarter of 2018 based on the Company's current projections.

### **Conference Call and Webcast**

The Company will host a conference call at 4:30 p.m. ET today. To participate, please dial (866) 939-3921 (domestic) or (678) 302-3550 (international) and reference the access code 45894700.

Access to the live webcast as well as the link to the replay of the call can be found at

<http://capricor.com/news/events/>. The webcast will be archived for approximately 30 days.

## 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 "off-the-shelf" cell therapy that is currently in clinical development for the treatment of Duchenne muscular dystrophy. Capricor has also established itself as one of the leading companies investigating the field of extracellular vesicles and is 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](http://www.capricor.com).

The Phase I/II HOPE and Phase II ALLSTAR clinical trials were funded in part by the California Institute for Regenerative Medicine.

## 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, 2016 as filed with the Securities and Exchange Commission on March 16, 2017, in its Registration Statement on Form S-3, as filed with the Securities and Exchange Commission on September 28, 2015, together with the prospectus included therein and prospectus supplements thereto, and in its Quarterly Report on Form 10-Q for the quarter ended June 30, 2017, as filed with the Securities and Exchange Commission on August 14, 2017. 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. Capricor's exosomes technology, including CAP-2003, has not yet been approved for clinical investigation.*

(unaudited)

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
INCOME				
Collaboration income	\$ -	\$ 683,595	\$ 1,367,186	\$ 2,506,511
Grant income	260,771	63,186	770,855	585,177
Other income	52,500	-	52,500	-
TOTAL INCOME	313,271	746,781	2,190,541	3,091,688
OPERATING EXPENSES				
Research and development	1,862,369	4,727,111	8,247,700	13,376,178
General and administrative	1,088,635	1,259,744	3,524,815	3,778,699
TOTAL OPERATING EXPENSES	2,951,004	5,986,855	11,772,515	17,154,877
LOSS FROM OPERATIONS	(2,637,733)	(5,240,074)	(9,581,974)	(14,063,189)
OTHER INCOME (EXPENSE)				
Investment income	10,393	5,410	26,726	16,347
Interest expense	(107,653)	(98,749)	(318,500)	(241,760)
TOTAL OTHER INCOME (EXPENSE)	(97,260)	(93,339)	(291,774)	(225,413)
NET LOSS	(2,734,993)	(5,333,413)	(9,873,748)	(14,288,602)
OTHER COMPREHENSIVE GAIN (LOSS)				
Net unrealized gain (loss) on marketable securities	1,276	(3,855)	5,069	(8,462)
COMPREHENSIVE LOSS	\$ (2,733,717)	\$ (5,337,268)	\$ (9,868,679)	\$ (14,297,064)
Net loss per share, basic and diluted	\$ (0.12)	\$ (0.29)	\$ (0.44)	\$ (0.81)
Weighted average number of shares, basic and diluted	23,378,141	18,286,816	22,311,369	17,594,749

**CAPRICOR THERAPEUTICS, INC.  
SUMMARY BALANCE SHEETS**

	September 30, 2017 (unaudited)	December 31, 2016
Cash, cash equivalents and marketable securities	\$ 13,894,937	\$ 16,194,888
Total assets	\$ 15,793,539	\$ 18,747,355
Total deferred revenue	-	1,367,186
Total liabilities	\$ 20,064,313	\$ 22,750,509

Total stockholders' equity (deficit) - 25,100,388 and 21,399,019 common shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively

Total liabilities and stockholders' equity

---

---

(4,270,774)

\$ 15,793,539

---

---

(4,003,154)

\$ 18,747,355

For more information, please contact:

AJ Bergmann, Vice President of Finance

+1-310-358-3200

[abergmann@capricor.com](mailto:abergmann@capricor.com)



View original content with multimedia: <http://www.prnewswire.com/news-releases/capricor-therapeutics-reports-third-quarter-2017-financial-results-and-provides-update-on-duchenne-muscular-dystrophy-development-program-300552207.html>

SOURCE Capricor Therapeutics, Inc.