

March 11, 2021



Capricor Therapeutics Reports Fourth Quarter and Full Year 2020 Financial Results and Provides Corporate Update

Exosomes Platform Technology

- Pre-IND Meeting with FDA this Quarter to Discuss Multivalent Exosome-mRNA Vaccine-
- Pipeline Expansion Underway Using Engineered Exosomes-

Cell Therapy Program

- In Discussions with FDA on Pathway to Approval for Duchenne Muscular Dystrophy-
- Phase II INSPIRE Top-Line Data Expected in Third Quarter-
- To Host Conference Call and Webcast Today at 4:30 p.m. ET-

LOS ANGELES, March 11, 2021 (GLOBE NEWSWIRE) -- [Capricor Therapeutics](#) (NASDAQ: CAPR), a biotechnology company focused on the development of transformative cell- and exosome-based therapeutics for the treatment and prevention of a broad spectrum of diseases, announced today its financial results for the fourth quarter and full year 2020 and provided a corporate update.

"2020 was a tremendously important and meaningful year for Capricor marked by advances in our engineered exosome platform technology and continued progress in our clinical-stage cell therapy programs for Duchenne muscular dystrophy and COVID-19," said Linda Marbán, Ph.D., Capricor's president and chief executive officer. "Pivotal to the plan to initiate our engineered exosome platform technology was the appointment of Dr. Stephen Gould as executive consultant to guide the development of this platform. In order to validate the technology, we developed a multi-antigen vaccine for SARS-CoV-2 which led to several publications describing that our vaccine candidate can induce long-lasting immune responses to multiple SARS-CoV-2 proteins, and potentially elicit a broad-based, cellular and humoral immunity. We are now planning on moving this vaccine into the clinic in 2021, subject to regulatory approval."

"One of the reasons the exosomes are potentially so useful and transformative is their ability to speak the language of a cell. We believe that exosomes loaded with biologic payloads will be important in the development of new therapeutics and vaccines, added Dr. Marbán. "Capricor, in 2021, is focused on becoming a leading exosome-RNA company and all of our work in the development of a vaccine for SARS-CoV-2 is simply a prelude to the future development of exosome-based vaccines and therapeutics, with the ultimate goal of generating formulations of engineered exosomes and synthetic mRNAs to potentially prevent and treat human diseases. The opportunities for pipeline expansion are very exciting."

Dr. Marbán continued, “we have now conducted two positive clinical trials in Duchenne muscular dystrophy. Our HOPE-2 trial demonstrated statistically significant results in upper limb skeletal muscle and cardiac function. We are currently discussing this program with FDA to determine the appropriate path forward and are actively discussing partnership opportunities for this asset. We believe these initiatives position us well as we look forward to a productive year in our therapeutics development programs.”

Fourth Quarter & Full Year 2020 Highlights and Recent Operational Developments

Pipeline Development

- **Exosomes Platform**

- Announced New Advances in Exosome-Mediated mRNA Delivery Platform (*March 2021*)
- Announced Positive Preclinical Data for Multivalent Exosome-mRNA Vaccine For COVID-19 (*November 2020*)
- Announced strategic plan for exosomes platform technology product expansion (*March 2020*)
- Announced appointment of Stephen Gould, Ph.D. as Executive Consultant to oversee our exosomes program (*March 2020*)

- **Cell Therapy (CAP-1002) for Duchenne Muscular Dystrophy**

- Announced Manufacturing Collaboration with Lonza for the Development of CAP-1002 for the Treatment of Duchenne Muscular Dystrophy and Other Indications (*January 2021*)
- Announced Positive Results from HOPE-2 Study in Patients with DMD Treated with CAP-1002 at World Muscle Society Congress 2020 (*November 2020*)
- Announced Positive Top-Line Results from HOPE-2 Study in Patients with DMD Treated with CAP-1002 (*May 2020*)
 - One-Year Results from Randomized, Double-Blind, Placebo-Controlled Study Demonstrated Improved Performance of Upper Limb (PUL) 2.0 ($p=0.05$)
 - First Ever Study in DMD that Correlates Stabilization in Cardiac Function with a Reduction in a Biomarker of Cell Damage

- **CAP-1002 for COVID-19**

- Announced DSMB Recommendation to Continue Phase II INSPIRE Trial for Severe COVID-19 Patients (*December 2020*)
- Initiated Dosing of Patients in Phase II INSPIRE Trial (*November 2020*)
- Announced Compassionate Use Data in COVID-19 Patients Treated with CAP-1002 (*April 2020*)
- Initiated Compassionate Use Program for Severe COVID-19 Patients using CAP-1002 (*April 2020*)

Publications & Key Opinion Leader Support

- Announced Publication Demonstrating Advances in our Exosome-Mediated mRNA Delivery Platform (*March 2021*)
- Announced Publication on the Development of Serology Test to Confirm Activity of SARS-CoV-2 Vaccines (*February 2021*)

- Announced Publication Demonstrating Methods for Enhanced Potency of Cardiosphere-Derived Exosomes (*January 2021*)
- Announced Publication Demonstrating Positive Preclinical Data for Multivalent Exosome-mRNA Vaccine For COVID-19 (*November 2020*)
- Hosted Key Opinion Leader Call on Cardiac Complications of DMD with a presentation by Michael Taylor, M.D., Ph.D., Director of Advanced Imaging Innovation (Cincinnati Children's Hospital) (*April 2020*)
- Hosted Key Opinion Leader Call on Why Exosomes are Uniquely Suited for Vaccine Development with a presentation by Stephen J. Gould, Ph.D., from Johns Hopkins University (*March 2020*)

Anticipated Events and Targeted Milestones in 2021

- Plan for pre-IND meeting with FDA to discuss next steps in exosome-mRNA vaccine clinical strategy
- Plan to file an IND for exosome-mRNA vaccine product candidate for SARS-CoV-2, subject to regulatory approval
- Plan to publish additional preclinical data on exosomes platform
- Plan to announce pipeline expansion with engineered exosomes platform
- Plan to announce further updates on DMD program
- Plan to announce top-line data from INSPIRE study

Fourth Quarter and Full Year Financial Results

The Company reported a net loss of approximately \$4.2 million, or \$0.21 per share, for the fourth quarter of 2020, compared to a net loss of approximately \$1.5 million, or \$0.34 per share, for the fourth quarter of 2019.

For the year ended December 31, 2020, the Company reported a net loss of approximately \$13.7 million, or \$0.88 per share, compared to a net loss of approximately \$7.6 million, or \$2.06 per share, for the year ended December 31, 2019.

As of December 31, 2020, the Company's cash, cash equivalents and marketable securities totaled approximately \$32.7 million, compared to approximately \$9.9 million on December 31, 2019. Additionally, in 2020, Capricor raised approximately \$22.9 million in net proceeds at an average price of approximately \$6.34 per share under its at-the-market offering program.

Financial Outlook

Capricor believes that based on the current operating plan and financial resources, the Company expects that the cash, cash equivalents and marketable securities will be sufficient to cover expenses and capital requirements through at least the second quarter of 2023.

Conference Call and Webcast

To participate in the conference call, please dial 877-451-6152 (Domestic/Toll-Free) or 201-389-0879 (International) and reference the conference ID: 13717080

To participate via a webcast and view the slides, please visit:

<http://public.viavid.com/index.php?id=143779>

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

About Capricor Therapeutics

Capricor Therapeutics, Inc. (NASDAQ: CAPR) is a biotechnology company focused on the development of transformative cell- and exosome-based therapeutics and vaccines for the treatment and prevention of a broad spectrum of diseases. Capricor's lead candidate, CAP-1002, is an allogeneic cardiac-derived cell therapy that is currently in clinical development for the treatment of Duchenne muscular dystrophy and the cytokine storm associated with COVID-19. Capricor is also developing our exosomes platform technology as a next-generation therapeutic platform. Our current focus is on the development of exosomes loaded with nucleic acids, including mRNA, to treat or prevent a variety of diseases. For more information, visit www.capricor.com and follow the Company on [Facebook](#), [Instagram](#) and [Twitter](#).

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.

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, 2019 as filed with the Securities and Exchange Commission on March 27, 2020 and in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2020 as filed with the Securities and Exchange Commission on November 13, 2020. 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. None of Capricor's exosome-based candidates have been approved for clinical investigation.

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**CAPRICOR THERAPEUTICS, INC.
CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(UNAUDITED)**

	Three months ended December 31,		Years ended December 31,	
	2020	2019	2020	2019
REVENUE				
Revenue	\$ 57,830	\$ 222,100	\$ 310,250	\$ 1,005,028
TOTAL REVENUE	57,830	222,100	310,250	1,005,028
OPERATING EXPENSES				
Research and development	2,745,104	828,749	8,457,000	5,141,805
General and administrative	1,493,266	876,720	5,543,221	3,597,111
TOTAL OPERATING EXPENSES	4,238,370	1,705,469	14,000,221	8,738,916
LOSS FROM OPERATIONS	(4,180,540)	(1,483,369)	(13,689,971)	(7,733,888)
OTHER INCOME (EXPENSE)				

Investment income	2,608	13,951	32,943	94,791
Loss on disposal of fixed asset	<u>-</u>	<u>-</u>	<u>-</u>	<u>(2,720)</u>
TOTAL OTHER INCOME (EXPENSE)	<u>2,608</u>	<u>13,951</u>	<u>32,943</u>	<u>92,071</u>
NET LOSS	<u>(4,177,932)</u>	<u>(1,469,418)</u>	<u>(13,657,028)</u>	<u>(7,641,817)</u>
OTHER COMPREHENSIVE INCOME (LOSS)				
Net unrealized gain (loss) on marketable securities	<u>-</u>	<u>(757)</u>	<u>757</u>	<u>(13,150)</u>
COMPREHENSIVE LOSS	\$ (4,177,932)	\$(1,470,175)	\$(13,656,271)	\$(7,654,967)
Net loss per share, basic and diluted	\$ (0.21)	\$ (0.34)	\$ (0.88)	\$ (2.06)
Weighted average number of shares, basic and diluted	20,373,647	4,338,434	15,571,056	3,711,333

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

	December 31, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	<u>\$ 32,665,874</u>	<u>\$ 9,885,378</u>
Total assets	<u>\$ 34,618,796</u>	<u>\$ 11,113,637</u>
 Total liabilities	 <u>\$ 6,419,012</u>	 <u>\$ 4,274,251</u>
 Total stockholders' equity - 20,577,123 and 5,227,398 common shares issued and outstanding at December 31, 2020 and December 31, 2019, respectively	 <u>28,199,784</u>	 <u>6,839,386</u>
Total liabilities and stockholders' equity	<u>\$ 34,618,796</u>	<u>\$ 11,113,637</u>

