Aprea Therapeutics Reports First Quarter 2020 Financial Results and Provides Update on Business Operations

May 15, 2020

BOSTON, May 15, 2020 (GLOBE NEWSWIRE) -- Aprea Therapeutics, Inc. (Nasdaq: APRE), a biopharmaceutical company focused on developing and commercializing novel cancer therapeutics that reactivate mutant tumor suppressor protein, p53, today reported financial results for the three months ended March 31, 2020 and provided a business update.

“Despite the challenges caused by the emerging COVID-19 pandemic, we continued to make significant progress to advance the development of our lead compound, eprenetapopt. In January 2020 we were granted Breakthrough Therapy Designation by the FDA to support our Phase 3 development program of eprenetapopt in combination with azacitidine,” said Christian S. Schade, President and Chief Executive Officer of Aprea. “We are proud of the Aprea team, for their efforts to navigate through these uncharted times to support and advance our aggressive development of both eprenetapopt and our next generation p53 reactivator, APR-548, with minimal interruption.”

Business Operations Update:

The Company is conducting, supporting and planning multiple clinical trials of *eprenetapopt* or APR-246:

- **Pivotal Phase 3 MDS Trial—**The Company is currently enrolling a pivotal Phase 3 randomized, controlled trial evaluating APR-246 with azacitidine as frontline therapy in HMA-naïve *TP53* mutant myelodysplastic syndromes (MDS) patients with a primary endpoint of CR rate. Though the Company had initially observed a decrease in both patient screening and patient enrollment as a result of the coronavirus (*COVID-19*) pandemic, the Company has recently observed increased patient screening activity and has currently enrolled 140 patients in the trial with a number of additional patients now scheduled for screening. The Company currently plans to close enrollment of this trial in the second quarter of 2020 and remains confident it will have top-line data available by year-end 2020.

- **Phase 2 MDS/AML Post-Transplant Trial—**The Company is currently enrolling its single-arm, open-label Phase 2 trial evaluating APR-246 with azacitidine as post-transplant maintenance therapy in *TP53* mutant MDS and acute myeloid leukemia (AML) patients who have received an allogeneic stem cell transplant. Though the Company had initially observed a decrease in both patient screening and patient enrollment as a result of the *COVID-19* pandemic, the Company has recently observed increased patient screening activity and has currently enrolled 16 out of 31 patients in this trial with a number of additional patients scheduled for screening. The Company believes that it will complete enrollment in this trial in the third quarter of 2020.

- **Phase 1 AML Trial—**Based on *in vitro* data evidencing synergistic activity between APR-246 and venetoclax, the Company is conducting a Phase 1 clinical trial in frontline and relapsed/refractory *TP53* mutant AML assessing APR-246 with venetoclax with or without azacitidine. The goals of the study include determining the safety, tolerability and preliminary efficacy of the combinations. The first patient was enrolled in 1Q 2020 and the Company completed enrollment of the first two safety cohorts of three patients each. Together with its investigators and clinical sites, the Company continues to assess the impact of the *COVID-19* pandemic on the enrollment and the ability to maintain patients enrolled in this trial.

- **Phase 1 NHL Trial—**As further assessment of APR-246 in hematological malignancies, the Company has designed and plans to conduct a Phase 1 clinical trial in relapsed/refractory *TP53* mutant chronic lymphoid leukemia (CLL) and mantle cell lymphoma (MCL) assessing APR-246 with venetoclax and rituximab, and APR-246 with ibrutinib. The Company is targeting the first patient to be enrolled in the second half of 2020.

- **Phase 1/2 Solid Tumor Trial—**Based on *in vivo* data evidencing synergistic activity between APR-246 and immuno-

therapy agents including anti-PD-1 antibody, the Company has designed and plans to conduct Phase 1/2 clinical trials in relapsed/refractory gastric, bladder and non-small cell lung cancers assessing APR-246 with anti-PD-1 therapy. The Company is targeting the first patient to be enrolled in the second half of 2020.

- **APR-548 --** The Company's second product candidate, APR-548, is a next-generation p53 reactivator with the potential for oral administration. APR-548 is a unique analog of APR-246 and therefore a pro-drug of MQ. APR-548 exhibits high oral bioavailability in preclinical testing and is being developed in an oral dosage form. The Company has completed Investigational New Drug, or IND, enabling preclinical studies of APR-548 and is targeting the submission of an IND in the first half of 2020.

First Quarter Financial Results

- **Cash and cash equivalents:** As of March 31, 2020, the Company had $122.5 million of cash and cash equivalents compared to $130.1 million of cash and cash equivalents as of December 31, 2019. The Company expects cash burn for 2020 to be between $35.0 million $40.0 million. The Company believes its cash and cash equivalents as of March 31, 2020 will be sufficient to meet its current projected operating requirements into 2023.

- **Research and Development (R&D) expenses:** R&D expenses were $9.1 million for the quarter ended March 31, 2020, compared to $3.7 million for the comparable period in 2019. The increase in R&D expenses was primarily related to the advancement of the Company's lead product candidate, APR-246. In Q1 2019 the Company commenced a pivotal Phase 3 clinical trial of APR-246 with azacitidine for frontline treatment of *TP53* mutant MDS which is supported by two ongoing Phase 1b/2 investigator initiated trials, one in the U.S. and one in France, testing APR-246 with azacitidine as frontline treatment in *TP53* mutant MDS and AML patients. In addition, in Q1 2020, the Company began enrolling patients in a Phase 1 clinical trial in frontline and relapsed/refractory *TP53* mutant AML assessing APR-246 with venetoclax with or without azacitidine.

- **General and Administrative (G&A) expenses:** G&A expenses were $2.8 million for the quarter ended March 31, 2020, compared to $0.7 million for the comparable period in 2019. The increase in G&A expenses was primarily due to increased insurance and professional fees associated with operating as a public company, as well as increased personnel costs.

- **Net loss:** Net loss was $9.4 million, or $0.45 per share for the quarter ended March 31, 2020, compared to a net loss of $3.5 million, or $2.97 per share for the quarter ended March 31, 2019. The Company had 21,054,842 shares of common stock outstanding as of March 31, 2020.

About Aprea Therapeutics, Inc.

Aprea Therapeutics, Inc. is a biopharmaceutical company headquartered in Boston, Massachusetts with research facilities in Stockholm, Sweden, focused on developing and commercializing novel cancer therapeutics that reactivate mutant tumor suppressor protein, p53. The Company's lead product candidate is APR-246 (*eprenetapopt)*, a small molecule in clinical development for hematologic malignancies, including myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML). APR-246 has received Breakthrough Therapy, Orphan Drug and Fast Track designations from the FDA for MDS, and Orphan Drug designation from the European Commission for MDS, AML and ovarian cancer. For more information, please visit the company website at www.aprea.com.

The Company may use, and intends to use, its investor relations website at https://ir.aprea.com/ as a means of disclosing material nonpublic information and for complying with its disclosure obligations under Regulation FD.

About p53 and APR-246 (*eprenetapopt)*

The p53 tumor suppressor gene is the most frequently mutated gene in human cancer, occurring in approximately 50% of all human tumors. These mutations are often associated with resistance to anti-cancer drugs and poor overall survival, representing a major unmet medical need in the treatment of cancer.

APR-246 (*eprenetapopt)* is a small molecule that has demonstrated reactivation of mutant and inactivated p53 protein – by restoring wild-type p53 conformation and function – thereby inducing programmed cell death in human cancer cells. Pre-clinical anti-tumor activity has been observed with APR-246 in a wide variety of solid and hematological cancers, including MDS, AML, and ovarian cancer, among others. Additionally, strong synergy has been seen with both traditional anti-cancer agents, such as chemotherapy, as well as newer mechanism-based anti-cancer drugs and immuno-oncology checkpoint inhibitors. In addition to pre-clinical testing, a Phase 1/2 clinical program with APR-246 has been completed, demonstrating a favorable safety profile and both biological and confirmed clinical responses in hematological malignancies and solid tumors with mutations in the *TP53* gene.

Forward-Looking Statement

Certain information contained in this press release includes "forward-looking statements", within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended, related to our clinical trials, regulatory submissions and projected cash position. We may, in some cases use terms such as "predicts," "believes," "potential," "continue," "anticipates," "estimates," "expects," "plans," "intends," "targeting," "confidence," "may," "could," "might," "likely," "will," "should" or other words that convey uncertainty of the future events or outcomes to identify these forward-looking statements. Our forward-looking statements are based on current beliefs and expectations of our

management team that involve risks, potential changes in circumstances, assumptions, and uncertainties. Any or all of the forward-looking statements may turn out to be wrong or be affected by inaccurate assumptions we might make or by known or unknown risks and uncertainties. These forward looking statements are subject to risks and uncertainties including risks related to the success and timing of our clinical trials or other studies, risks associated with the coronavirus pandemic and the other risks set forth in our filings with the U.S. Securities and Exchange Commission. For all these reasons, actual results and developments could be materially different from those expressed in or implied by our forward-looking statements. You are cautioned not to place undue reliance on these forward-looking statements, which are made only as of the date of this press release. We undertake no obligation to publicly update such forward-looking statements to reflect subsequent events or circumstances.

Source: Aprea Therapeutics, Inc.

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Aprea Therapeutics, Inc.
Condensed Consolidated Balance Sheets
(Unaudited)

	March 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$122,513,357	$130,088,869
Prepaid expenses and other current assets	2,090,941	2,955,878
Total current assets	124,604,298	133,044,747
Property and equipment, net	45,934	41,639
Right of use lease and other noncurrent assets	474,898	521,499
Total assets	$125,125,130	$133,607,885
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$3,006,688	$2,176,852
Accrued expenses	8,298,769	6,642,553
Lease liability—current	238,771	242,329
Total current liabilities	11,544,228	9,061,734
Lease liability—noncurrent	227,288	302,621
Total liabilities	11,771,516	9,364,355
Commitments and contingencies		
Stockholders' equity:		
Common stock, par value $0.001; 21,054,842 and 21,022,752, shares issued and outstanding at March 31, 2020 and December 31, 2019, respectively.	21,055	21,023
Additional paid-in capital	227,219,510	226,284,548
Accumulated other comprehensive loss	(13,958,431)	(11,533,778)
Accumulated deficit	(99,928,520)	(90,528,263)
Total stockholders' equity	113,353,614	124,243,530
Total liabilities and stockholders' equity	$125,125,130	$133,607,885

Aprea Therapeutics, Inc.
Condensed Consolidated Statements of Operations and Comprehensive Loss
(Unaudited)

	Three Months Ended March 31,	
	2020	**2019**
Operating expenses:		
Research and development	$9,096,122	$3,678,444
General and administrative	2,776,468	729,326
Total operating expenses	11,872,590	4,407,770
Other income (expense):		
Interest income (expense)	224,442	(3,348)
Foreign currency gain	2,247,891	935,916
Total other income	2,472,333	932,568
Net loss	$(9,400,257)	$(3,475,202)

Other comprehensive loss:				
Foreign currency translation	(2,424,653	)	(2,031,175	)
Total comprehensive loss	(11,824,910	)	(5,506,377	)
Net loss per share attributable to common stockholders, basic and diluted	$(0.45	)	$(2.97	)
Weighted average basic and diluted shares of common stock outstanding	21,052,726		1,171,193	

Source: Aprea Therapeutics