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

November 5, 2020

BOSTON, Nov. 05, 2020 (GLOBE NEWSWIRE) -- Aprea Therapeutics, Inc. (Nasdaq: APRE), a biopharmaceutical company focused on developing and commercializing novel cancer therapeutics that reactivate the mutant tumor suppressor protein, p53, today reported financial results for the three and nine months ended September 30, 2020 and provided a business update.

"We made solid progress during the third quarter across our development pipeline as we approach top-line data from the Phase 3 clinical trial evaluating eprenetapopt with azacitidine for the treatment of front-line *TP53* mutant myelodysplastic syndromes (MDS) patients by year-end 2020" said Christian S. Schade, Chairman and Chief Executive Officer of Aprea. "We continue to execute on our goal of expanding the clinical opportunities for our p53 reactivator programs, including expansion of our front-line AML clinical trials, enrollment of the first patients in our solid tumor trial, initiation of our lymphoma trial and clearance from FDA to proceed with the Phase 1 trial of our next-generation p53 reactivator, APR-548."

Business Operations Update:

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

- **Pivotal Phase 3 MDS Trial—**The Company has completed the full enrollment of 154 patients in its pivotal Phase 3 randomized, controlled trial evaluating eprenetapopt with azacitidine as frontline therapy in HMA-naïve *TP53* mutant myelodysplastic syndromes (MDS) patients with a primary endpoint of complete remission (CR) rate. The Company remains confident it will have top-line data available by year-end 2020.

- **Phase 2 MDS/AML Post-Transplant Trial—**The Company has completed the target enrollment of 31 patients in its single-arm, open-label Phase 2 trial evaluating eprenetapopt 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.

- **Phase 1/2 AML Trial—**The Company is currently enrolling its Phase 1/2 trial evaluating the safety, tolerability, and preliminary efficacy of eprenetapopt therapy in *TP53* mutant AML patients. The lead-in portion of the trial evaluated the tolerability of eprenetapopt with venetoclax, with or without azacitidine, and no dose-limiting toxicities were observed in 12 patients receiving either regimen. Based on these results, the Company has expanded the trial to treat approximately 30 additional frontline *TP53* mutant AML patients with the combination of eprenetapopt, venetoclax and azacitidine. The Company also plans to activate a separate cohort in the trial to evaluate the combination of eprenetapopt with azacitidine in approximately 30 frontline *TP53* mutant AML patients.

- **Phase 1 NHL Trial—**The Company has designed and plans to conduct a Phase 1 clinical trial in relapsed/refractory *TP53* mutant chronic lymphoid leukemia (CLL) assessing eprenetapopt with venetoclax and rituximab, and eprenetapopt with ibrutinib in order to further assess eprenetapopt in hematological malignancies. A poster describing the clinical trial has been accepted for presentation at the 62nd American Society of Hematology (ASH) Annual Meeting (abstract # 1311) on December 5, 2020 from 10:00 am to 6:30 pm eastern time. The Company is targeting the first patient to be enrolled in the fourth quarter of 2020.

- **Phase 1/2 Solid Tumor Trial—**Based on *in vivo* data suggesting synergistic activity between eprenetapopt and immuno-therapy agents including anti-PD-1 antibody, the Company has designed and is conducting Phase 1/2 clinical trials in relapsed/refractory gastric, bladder and non-small cell lung cancers assessing eprenetapopt with anti-PD-1 therapy. Five patients have been enrolled in the safety review cohort of this trial.

- **APR-548 --** The Company's second product candidate, APR-548, is a pre-clinical, next-generation p53 reactivator with the

potential for oral administration. APR-548 exhibits high oral bioavailability in preclinical testing and is being developed in an oral dosage form. The Company completed Investigational New Drug, or IND, enabling preclinical studies of APR-548 and filed an IND with the FDA. The Company received clearance from the FDA in October 2020 to initiate Phase 1 clinical trials for APR-548. The Company anticipates enrollment in the Phase 1 clinical trial to begin in the first quarter of 2021.

Third Quarter Financial Results

- **Cash and cash equivalents:** As of September 30, 2020, the Company had $101.1 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 the full year 2020 to be between $35.0 million $40.0 million. The Company believes its cash and cash equivalents as of September 30, 2020 will be sufficient to meet its current projected operating requirements into 2023.

- **Research and Development (R&D) expenses:** R&D expenses were $8.8 million for the quarter ended September 30, 2020, compared to $4.9 million for the comparable period in 2019. The increase in R&D expenses was primarily related to the continued development of the Company's lead product candidate, eprenetapopt in the following ongoing clinical trials; our pivotal Phase 3 clinical trial of eprenetapopt with azacitidine for frontline treatment of *TP53* mutant MDS, our Phase 1/2 clinical trials in relapsed/refractory gastric, bladder and non-small cell lung cancers assessing eprenetapopt with anti-PD-1 therapy, our Phase 1 clinical trial in relapsed/refractory *TP53* mutant chronic lymphoid leukemia (CLL) assessing eprenetapopt with venetoclax and rituximab, and eprenetapopt with ibrutinib and our Phase 2 post-transplant MDS/AML clinical trial.

- **General and Administrative (G&A) expenses:** G&A expenses were $3.5 million for the quarter ended September 30, 2020, compared to $2.3 million for the comparable period in 2019. The increase in G&A expenses was primarily due to increased non-cash stock-based compensation, increased insurance expense and increased commercial development expense.

- **Net loss:** Net loss was $12.3 million, or $0.58 per share for the quarter ended September 30, 2020, compared to a net loss of $6.2 million, or $5.29 per share for the quarter ended September 30, 2019. The Company had 21,186,827 shares of common stock outstanding as of September 30, 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 eprenetapopt (APR-246), a small molecule in clinical development for hematologic malignancies, including myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML). Eprenetapopt 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. APR-548, a next generation small molecule reactivator of mutant p53, is being developed for oral administration. 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, eprenetapopt and APR-548

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.

Eprenetapopt (APR-246) 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 eprenetapopt 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 eprenetapopt 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.

A pivotal Phase 3 clinical trial of eprenetapopt and azacitidine for frontline treatment of *TP53* mutant MDS is ongoing. Eprenetapopt has received Breakthrough Therapy, Orphan Drug and Fast Track designations from the FDA for MDS, and Orphan Drug designation from the European Medicines Agency for MDS, AML and ovarian cancer.

APR-548 is a next-generation small molecule p53 reactivator. APR-548 has demonstrated high oral bioavailability, enhanced potency relative to eprenetapopt in *TP53* mutant cancer cell lines and has demonstrated *in vivo* tumor growth inhibition following oral dosing of tumor-bearing mice. A Phase 1 clinical trial of APR-548 in *TP53* MDS is planned.

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)

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 101,146,633	$ 130,088,869
Prepaid expenses and other current assets	1,257,077	2,955,878
Total current assets	102,403,710	133,044,747
Property and equipment, net	0,696	41,639
Right of use lease and other noncurrent assets	389,982	521,499
Total assets	$ 102,834,388	$ 133,607,885
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 4,349,977	$ 2,176,852
Accrued expenses	9,084,389	6,642,553
Lease liability—current	244,310	242,329
Total current liabilities	13,678,676	9,061,734
Lease liability—noncurrent	132,773	302,621
Total liabilities	13,811,449	9,364,355
Commitments and contingencies		
Stockholders' equity:		
Common stock, par value $0.001; 21,186,827 and 21,022,752, shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	21,187	21,023
Additional paid-in capital	229,986,911	226,284,548
Accumulated other comprehensive loss	(12,370,630)	(11,533,778)
Accumulated deficit	(128,614,529)	(90,528,263)
Total stockholders' equity.	89,022,939	124,243,530
Total liabilities and stockholders' equity	$ 102,834,388	$ 133,607,885

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 8,761,095	$ 4,910,409	$ 28,551,246	$ 12,908,679
General and administrative	3,473,210	2,307,946	10,036,564	4,655,861
Total operating expenses	12,234,305	7,218,355	38,587,810	17,564,540
Other income (expense):				

Interest income (expense)	(9,212)	(6,098)	217,908	(13,537)
Foreign currency (loss) gain	(74,565)	975,034	283,636	2,591,008
Total other income (expense)	(83,777)	968,936	501,544	2,577,471
Net loss	$ (12,318,082)	$ (6,249,419)	$ (38,086,266)	$ (14,987,069)
Other comprehensive income (loss):				
Foreign currency translation	(168,982)	(2,940,174)	(836,852)	(4,926,841)
Total comprehensive loss	(12,487,064)	(9,189,593)	(38,923,118)	(19,913,910)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.58)	$ (5.29)	$ (1.80)	$ (12.72)
Weighted-average common shares outstanding, basic and diluted	21,186,827	1,181,726	21,115,797	1,178,206

Source: Aprea Therapeutics