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

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BOSTON, Aug. 11, 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 and six months ended June 30, 2020 and provided a business update.

"The full enrollment in June of our Phase 3 clinical trial evaluating eprenetapopt (APR-246) with azacitidine for the treatment of front-line *TP53* mutant myelodysplastic syndromes (MDS) was a major milestone for Aprea and we look forward to top-line data by year-end 2020," said Christian S. Schade, President and Chief Executive Officer of Aprea. "In addition, we continue to advance the clinical development of eprenetapopt in different clinical settings and have recently enrolled the first patient in our solid tumor clinical trial program."

Business Operations Update:

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

- **Pivotal Phase 3 MDS Trial—**During the second quarter of 2020, the Company 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 is currently enrolling 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. Though the Company had initially observed a decrease in both patient screening and patient enrollment as a result of the *COVID-19* pandemic, patient screening activity has returned to expected levels and the trial has currently enrolled 24 out of 31 patients with a number of additional patients in screening. The Company anticipates completing enrollment of this trial in the third quarter of 2020.

- **Phase 1 AML Trial—**The Company is currently enrolling its Phase 1 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 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—**To further assess eprenetapopt 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 eprenetapopt with venetoclax and rituximab, and eprenetapopt 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 suggesting synergistic activity between eprenetapopt 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 eprenetapopt with anti-PD-1 therapy. The Company enrolled its first patient in August 2020.

- **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. However, based on feedback from the FDA, the Company will not be able to initiate human clinical trials of APR-548 until it is able to provide additional information necessary to address questions raised by the FDA.

Second Quarter Financial Results

- **Cash and cash equivalents:** As of June 30, 2020, the Company had $112.9 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 June 30, 2020 will be sufficient to meet its current projected operating requirements into 2023.

- **Research and Development (R&D) expenses:** R&D expenses were $10.7 million for the quarter ended June 30, 2020, compared to $4.3 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, eprenetapopt. In Q1 2019 the Company commenced a pivotal Phase 3 clinical trial of eprenetapopt with azacitidine for frontline treatment of *TP53* mutant MDS which completed enrollment in Q2 2020 and is supported by two ongoing Phase 1b/2 investigator initiated trials, one in the U.S. and one in France, testing eprenetapopt with azacitidine as frontline treatment in *TP53* mutant MDS and AML patients. In addition, in Q1 2020, the Company continued enrolling patients in a Phase 2 post-transplant MDS/AML clinical trial began enrolling patients in a Phase 1 clinical trial in frontline and relapsed/refractory *TP53* mutant AML assessing eprenetapopt with venetoclax with or without azacitidine.

- **General and Administrative (G&A) expenses:** G&A expenses were $3.8 million for the quarter ended June 30, 2020, compared to $1.7 million for the comparable period in 2019. The increase in G&A expenses was primarily due to increased insurance expense, non-cash stock-based compensation, personnel related costs and commercial development expense.

- **Net loss:** Net loss was $16.4 million, or $0.78 per share for the quarter ended June 30, 2020, compared to a net loss of $5.3 million, or $4.45 per share for the quarter ended June 30, 2019. The Company had 21,186,827 shares of common stock outstanding as of June 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 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.

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 EMA for MDS, AML and ovarian cancer.

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)

	June 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 112,861,504	$ 130,088,869
Prepaid expenses and other current assets	1,475,223	2,955,878
Total current assets .	114,336,727	133,044,747
Property and equipment, net	43,906	41,639
Right of use lease and other noncurrent assets	438,162	521,499
Total assets .	$ 114,818,795	$ 133,607,885
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 5,443,126	$ 2,176,852
Accrued expenses .	8,827,860	6,642,553
Lease liability—current .	241,527	242,329
Total current liabilities	11,512,513	9,061,734
Lease liability—noncurrent .	185,926	302,621
Total liabilities	14,698,439	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 June 30, 2020 and December 31, 2019, respectively	21,187	21,023
Additional paid-in capital	228,597,264	226,284,548
Accumulated other comprehensive loss	(12,201,648)	(11,533,778)
Accumulated deficit	(116,296,447)	(90,528,263)
Total stockholders' equity	100,120,356	124,243,530
Total liabilities and stockholders' equity .	$ 114,818,795	$ 133,607,885

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 10,694,029	$ 4,319,826	$ 19,790,151	$ 7,998,270
General and administrative	3,786,886	1,618,589	6,563,354	2,347,915
Total operating expenses	14,480,915	5,938,415	26,353,505	10,346,185
Other income (expense):				
Interest income (expense)	2,678	(4,091)	227,120	(7,439)
Foreign currency (loss) gain	(1,889,690)	680,058	358,201	1,615,974
Total other income (expense)	(1,887,012)	675,967	585,321	1,608,535
Net loss	$ (16,367,927)	$ (5,262,448)	$ (25,768,184)	$ (8,737,650)

Other comprehensive income (loss):				
Foreign currency translation	1,756,783	44,508	(667,870)	(1,986,667)
Total comprehensive loss	(14,611,144)	(5,217,940)	(26,436,054)	(10,724,317)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.78)	$ (4.45)	$ (1.22)	$ (7.43)
Weighted-average common shares outstanding, basic and diluted	21,107,056	1,181,583	21,079,891	1,176,417

Source: Aprea Therapeutics