Aprea Therapeutics Reports Fourth Quarter and Full Year 2020 Financial Results and Provides Update on Business Operations

March 16, 2021

BOSTON, March 16, 2021 (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 months and year ended December 31, 2020 and provided a business update.

"Though disappointed the topline complete remission rate from the Phase 3 clinical trial narrowly missed its primary endpoint, we continue to analyze the totality of the data from the study to understand those differences from our prior Phase 2 experience in frontline MDS patients and expect to present these findings in the second quarter of 2021," said Christian S. Schade, Chairman and Chief Executive Officer of Aprea. "Our dedicated team remains committed to the clinical development of eprenetapopt and our next generation, oral p53 reactivator, APR-548, in hematological and solid tumor malignancies. In 2021, we look forward to sharing data from our current clinical studies as well as our plans to expand the clinical pipeline to include new indications."

Business Operations Update:

The Company is conducting, supporting, and planning multiple clinical trials of *eprenetapopt* (APR-246) and APR-548:

- **Pivotal Phase 3 MDS Trial—**In December 2020, the Company announced its pivotal Phase 3 randomized, controlled trial evaluating eprenetapopt with azacitidine as frontline therapy in HMA-naïve *TP53* mutant myelodysplastic syndromes (MDS) patients failed to meet its predefined primary endpoint of complete remission (CR) rate. Analysis of the primary endpoint at this data cut demonstrated a 53% higher number of patients achieving a CR in the experimental arm receiving eprenetapopt with azacitidine versus the control arm receiving azacitidine alone but did not reach statistical significance. The Company is completing analysis from this Phase 3 clinical trial and expects to present additional information in the second quarter of 2021.

- **Phase 2 MDS/AML Post-Transplant Trial** – The Company has completed enrollment of 33 patients in a single-arm, open-label Phase 2 clinical trial evaluating eprenetapopt with azacitidine as post-transplant maintenance therapy in *TP53* mutant MDS and AML patients who have received an allogeneic stem cell transplant. The Company anticipates initial results from the primary endpoint of relapse-free survival at 12 months in the second quarter of 2021.

- **Phase 1/2 AML Trial** – The Company is currently enrolling a Phase 1/2 clinical 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 33 additional frontline *TP53* mutant AML patients with the combination of eprenetapopt, venetoclax and azacitidine. In the 19 frontline AML patients who are evaluable for efficacy with the triplet regimen, the Company has observed a 63% CR + CRi composite response rate and a 31% CR rate. The Company anticipates completion of enrollment in the triplet regimen expansion cohort during the second quarter of 2021 with availability of preliminary response rate data from the cohort also in the second quarter of 2021.

- **Phase 1 NHL Trial** – The Company is currently enrolling 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. The first patient was enrolled in the first quarter of 2021. The Company is also planning to evaluate the combination of eprenetapopt with venetoclax in relapsed/refractory mantle cell lymphoma.

- **Phase 1/2 Solid Tumor Trial** – The Company is currently enrolling a Phase 1/2 clinical trial in relapsed/refractory gastric, bladder and non-small cell lung cancers assessing eprenetapopt with anti-PD-1 therapy. The dose-escalation phase of the trial enrolled 6 patients with advanced solid tumors and no dose-limiting toxicities were observed. Based on these results, the Company is enrolling expansion cohorts for patients with advanced gastric, bladder and non-small cell lung cancers and has currently enrolled 8 patients across these expansion arms.

- **APR-548 --** The Company's second product candidate, APR-548, is a next-generation p53 reactivator that is being developed in an oral dosage form. The Company has planned a Phase 1 dose-escalation clinical trial evaluating the safety, tolerability, and preliminary efficacy of APR-548 with azacitidine in frontline and relapsed/refractory MDS patients. The Company anticipates the first patient to be enrolled early in the second quarter of 2021.

Fourth Quarter Financial Results

- **Cash and cash equivalents:** As of December 31, 2020, the Company had $89.0 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 2021 to be between $30.0 million $35.0 million. The Company believes its cash and cash equivalents as of December 31, 2020 will be sufficient to meet its current projected operating requirements into 2023.
- **Research and Development (R&D) expenses:** R&D expenses were $9.3 million for the quarter ended December 31, 2020, compared to $8.0 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; its pivotal Phase 3 clinical trial of eprenetapopt with azacitidine for frontline treatment of *TP53* mutant MDS, its Phase 1/2 clinical trial for the treatment of *TP53* mutant AML with venetoclax and azacitidine, its Phase 1/2 clinical trial in relapsed/refractory gastric, bladder and non-small cell lung cancers assessing eprenetapopt with anti-PD-1 therapy, its Phase 1 clinical trial in relapsed/refractory *TP53* mutant chronic lymphoid leukemia (CLL) assessing eprenetapopt with venetoclax and rituximab, and eprenetapopt with ibrutinib and its Phase 2 post-transplant MDS/AML clinical trial.
- **General and Administrative (G&A) expenses:** G&A expenses were $4.9 million for the quarter ended December 31, 2020, compared to $3.9 million for the comparable period in 2019. The increase in G&A expenses was primarily due to increases in non-cash stock-based compensation, insurance expense and commercial development expense.
- **Net loss:** Net loss was $15.4 million, or $0.73 per share for the quarter ended December 31, 2020, compared to a net loss of $13.1 million, or $0.64 per share for the quarter ended December 31, 2019. The Company had 21,186,827 shares of common stock outstanding as of December 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 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, Fast Track designation from the FDA for AML, 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 but at the primary data cut, it failed to meet its predefined primary endpoint of complete remission rate. 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

	December 31, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$89,017,686	$130,088,869
Prepaid expenses and other current assets	3,399,019	2,955,878
Total current assets	92,416,705	133,044,747
Property and equipment, net	38,515	41,639
Right of use lease and other noncurrent assets	349,999	521,499
Total assets	$92,805,219	$133,607,885
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$4,503,619	$2,176,852
Accrued expenses	10,571,237	6,642,553
Lease liability—current	256,309	242,329
Total current liabilities	15,331,165	9,061,734
Lease liability—noncurrent	78,847	302,621
Total liabilities	15,410,012	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 December 31, 2020 and 2019, respectively.	21,187	21,023
Additional paid-in capital	231,418,356	226,284,548
Accumulated other comprehensive loss	(10,037,261)	(11,533,778)
Accumulated deficit	(144,007,075)	(90,528,263)
Total stockholders' equity	77,395,207	124,243,530
Total liabilities and stockholders' equity	$92,805,219	$133,607,885

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

	Three Months Ended December 31,		Year Ended December 31,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 9,328,079	$ 8,041,993	$ 37,879,325	$ 20,950,672
General and administrative	4,895,323	3,937,765	14,931,887	8,593,626
Total operating expenses	14,223,402	11,979,758	52,811,212	29,544,298

Other income (expense):				
Interest income	4,744	169,888	222,652	156,351
Foreign currency (loss) gain	(1,173,888)	(1,262,868)	(890,252)	1,328,140
Total other income (expense)	(1,169,144)	(1,092,980)	(667,600)	1,484,491
Net loss	$ (15,392,546)	$ (13,072,738)	$ (53,478,812)	$ (28,059,807)
Other comprehensive income (loss):				
Foreign currency translation	2,333,369	2,154,388	1,496,517	(2,772,453)
Total comprehensive loss	(13,059,177)	(10,918,350)	(51,982,295)	(30,832,260)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.73)	$ (0.64)	$ (2.53)	$ (4.67)
Weighted-average common shares outstanding, basic and diluted	21,186,827	20,318,040	21,133,651	6,002,486

Source: Aprea Therapeutics