Aprea Therapeutics Completes Full Enrollment of Phase 3 Clinical Trial in TP53 Mutant Myelodysplastic Syndromes (MDS)

June 3, 2020

- *Topline data expected by year-end 2020*
- *Applications for US and EU regulatory approval planned for 2021*

BOSTON, June 03, 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 announced that patient enrollment in its Phase 3 clinical trial evaluating eprenetapopt with azacitidine for the treatment of front-line *TP53* mutant myelodysplastic syndromes (MDS) has been completed. Topline results are expected by year-end 2020. Aprea plans to include the results of the trial in a New Drug Application (NDA) to the U.S. FDA and a Marketing Authorization Application (MAA) to the EMA in 2021.

“Completion of enrollment in this Phase 3 trial is an important milestone for Aprea and our first-in-class p53 reactivator, eprenetapopt,” said Christian S. Schade, President and Chief Executive Officer of Aprea. “We continue to advance the development of eprenetapopt with the goal of providing an urgently needed therapeutic option to patients with p53 mutated MDS.”

The randomized, controlled pivotal Phase 3 trial is designed to evaluate eprenetapopt with azacitidine compared with azacitidine alone as front-line therapy in intermediate, high, and very high risk *TP53* mutant MDS patients. The multi-center trial enrolled 154 patients, randomized 1:1 to the two arms with a primary endpoint of CR rate. The trial has 90% power with P-value < 0.05 to detect a difference in CR rates of 50% in the eprenetapopt-containing arm versus 25% in the azacitidine-only control arm.

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 – and thereby induce 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.

About MDS

Myelodysplastic syndromes (MDS) represent a spectrum of hematopoietic stem cell malignancies in which bone marrow fails to produce sufficient numbers of healthy blood cells. Approximately 30-40% of MDS patients progress to acute myeloid leukemia (AML) and mutation of the p53 tumor suppressor protein is thought to contribute to disease progression. Mutations in p53 are found in up to 20% of MDS and AML patients and are associated with poor overall prognosis. There are no currently approved therapies specifically for *TP53* mutant MDS or AML patients.

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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