Aprea Therapeutics Announces Expansion of Clinical Trial Evaluating Eprenetapopt for the Front-Line Treatment of TP53 Mutant Acute Myeloid Leukemia (AML)

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- Commenced expansion cohort in combination with venetoclax and azacitidine
- Added cohort to be activated in combination with azacitidine

BOSTON, July 16, 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 the expansion of patient enrollment in its Phase 1 clinical trial evaluating eprenetapopt in *TP53* mutant AML. Following the completion of the safety lead-in portion of the clinical trial, the first expansion cohort will evaluate the combination of eprenetapopt with venetoclax and azacitidine in frontline *TP53* mutant AML. The Company also plans to activate a cohort in the trial that will evaluate eprenetapopt with azacitidine in frontline *TP53* mutant AML, expanding upon results for *TP53* mutant AML patients recently presented from two independent Phase 1b/2 clinical trials.

The lead-in portion of the Phase 1 AML Trial evaluated the tolerability of eprenetapopt with venetoclax, with or without azacitidine, and no dose-limiting toxicities were observed in patients receiving either regimen. The expansion part of the clinical trial will treat approximately 30 front-line *TP53* mutant AML patients with the triplet therapy of eprenetapopt with venetoclax and azacitidine. The Company will also evaluate front-line treatment with the doublet therapy of eprenetapopt and azacitidine in approximately 30 additional *TP53* mutant AML patients. Safety and efficacy will be evaluated in both patient cohorts.

"We are encouraged by the tolerability of the eprenetapopt regimens observed to-date in the trial and look forward to continued evaluation of the potential efficacy of eprenetapopt with venetoclax and azacitidine for the frontline treatment of *TP53* mutant AML," said Dr. Eyal Attar, Senior Vice President and Chief Medical Officer of Aprea. "In addition, we plan to also enroll a cohort of patients with *TP53* mutant AML that will receive eprenetapopt with azacitidine to expand on the promising data generated in the two Phase 1b/2 MDS/AML trials, where the results in AML patients compare favorably to recently presented data for the venetoclax and azacitidine doublet regimen in *TP53* mutant AML."

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.

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.

About AML

AML is the most common form of adult leukemia, with the highest incidence in patients aged 60 years and older. AML is characterized by proliferation of abnormal immature white blood cells which impairs production of normal blood cells. AML can develop de novo or may arise secondary to progression of other hematologic disorders or from chemotherapy or radiation treatment for a different, prior malignancy. Mutations in p53 are found in up to 20% of AML patients and are associated with poor overall prognosis. There are no currently approved therapies specifically for *TP53* mutant 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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