Aprea Therapeutics Announces Positive Results from Phase 2 Trial of Eprenetapopt + Azacitidine for Post-Transplant Maintenance Therapy in TP53 Mutant MDS and AML

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- 58% relapse free survival at 1 year post-transplant
- 79% overall survival at 1 year post-transplant

BOSTON, July 21, 2021 (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 positive results from its Phase 2 trial evaluating eprenetapopt with azacitidine for post-transplant maintenance therapy in patients with *TP53* mutant MDS and AML.

In 33 patients enrolled in the trial, the relapse free survival (RFS) at 1 year post-transplant was 58% and the median RFS was 12.1 months. The overall survival (OS) at 1 year post-transplant was 79%, with a median OS of 19.3 months. Prior clinical trials evaluating post-transplant outcomes in *TP53* mutant MDS and AML patients have reported a 1-year post-transplant RFS of ~30% and a median OS of ~5-8 months. In addition, the post- transplant regimen of eprenetapopt and azacitidine was well tolerated among patients in the clinical trial. The Company plans to discuss the data from this Phase 2 clinical trial with the U.S. Food and Drug Agency (FDA) in the second half of 2021 and expects to present data at a future scientific or medical conference.

"The post-transplant RFS and OS data with eprenetapopt and azacitidine maintenance therapy in these very difficult-to-treat *TP53* mutant MDS and AML patients are incredibly exciting," said trial principal investigator Asmita Mishra, M.D., of the H. Lee Moffitt Cancer Center and Research Institute. "Although transplant is currently the only potentially curative treatment for patients with *TP53* mutant MDS and AML, the risk of relapse with current standard of care remains unacceptably high and the median OS post-transplant is very limited at 8 months or less. Post-transplant maintenance therapy with eprenetapopt and azacitidine could, if approved, represent a new treatment paradigm that meaningfully improves outcomes for these patients with limited treatment options."

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 and solid tumors. Eprenetapopt has received Breakthrough Therapy, Orphan Drug and Fast Track designations from the FDA for myelodysplastic syndromes (MDS), Orphan Drug and Fast Track designations from the FDA for acute myeloid leukemia (AML), and Orphan Drug designation from the European Commission for MDS and AML. 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 has been completed and failed to meet the primary statistical endpoint of complete remission. A Phase 1/2 clinical trial of eprenetapopt with venetoclax

and azacitidine for the frontline treatment of *TP53* mutant AML met the primary efficacy endpoint of complete remission. Additional clinical trials in hematologic malignancies and solid tumors are ongoing. Eprenetapopt has received Breakthrough Therapy, Orphan Drug and Fast Track designations from the FDA for MDS, Orphan Drug and Fast Track designations from the FDA for AML, and Orphan Drug designation from the European Medicines Agency for MDS and AML.

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.

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.

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 that 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; secondary AML carries a worse prognosis than de novo AML. Mutations in *TP53*, which are associated with poor overall prognosis, occur in approximately 20% of patients with newly diagnosed AML, more than 30% of patients with therapy-related AML and approximately 70-80% of patients with complex karyotype.

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 study analyses, clinical trials, regulatory submissions, and projected cash position. We may, in some cases use terms such as “future,” “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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