Aprea Therapeutics Presents Results From French Phase Ib/II Clinical Trial of APR-246 (Eprenetapopt) and Azacitidine in Patients with TP53 Mutant Myelodysplastic Syndromes and Acute Myeloid Leukemia at the 25th European Hematology Association Annual Meeti

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- 57% CR and 75% ORR rate by IWG criteria in all evaluable MDS patients
- 12.1 months median OS in intention-to-treat MDS patients with 9.7 months median duration of follow-up
- 13.7 months median OS in patients receiving 3 or more cycles of therapy with 9.7 months duration of follow-up

BOSTON, June 12, 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 oral presentation of updated data from its French Phase 1b/2 clinical trial at the 25th European Hematology Association Annual Meeting (EHA). The trial is evaluating the safety and efficacy of APR-246 (eprenetapopt) in combination with azacitidine (AZA) for the treatment of *TP53* mutant myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML). The clinical trial is sponsored by the Groupe Francophone des Myélodysplasies (GFM).

As of the April 1, 2020 data cutoff, the overall response rate (ORR) in 28 evaluable MDS patients was 75%, with a 57% complete remission (CR) rate, by International Working Group (IWG) criteria. With a median duration of follow-up of 9.7 months, the median overall survival (OS) for all enrolled patients (n=52) was 12.1 months and in MDS patients (n=34) was 12.1 months. For patients who remained on treatment for 3 or more cycles of treatment the median OS was higher at 13.7 months versus 2.8 months for patients who were on treatment for fewer than 3 cycles. Relative to baseline, mutant *TP53* variant allele frequency (VAF) was decreased in responding patients by 3 cycles of treatment, including 20 (51%) patients who achieved mutant *TP53* negativity by next-generation sequencing (NGS).

“The data from this ongoing trial of eprenetapopt with azacitidine continue to be very encouraging in these most difficult-to-treat *TP53* mutant MDS and AML patients, who not only have at least one *TP53* mutation but the majority of whom also have high risk cytogenetic abnormalities,” said Thomas Cluzeau, M.D., co-lead investigator for the GFM trial. “We continue to observe ORR and CR rates in these patients that are substantially higher than the GFM’s experience with azacitidine monotherapy. Furthermore, with increased duration of follow-up, we now also see the emergence of highly encouraging overall survival that appears to be better than azacitidine alone or in combination with others agents in this very high-risk molecular group of patients with a *TP53* mutation.”

Details of the on-demand oral presentation are as follows:

Title: APR-246 Combined with Azacitidine in *TP53* Mutated Myelodysplastic Syndromes (MDS) and Acute Myeloid Leukemia. A Phase 2 Study by the Groupe Francophone des Myélodysplasies (GFM)

Oral Abstract Session: Novel treatments for MDS I

Abstract: S181

About the Clinical Trial

Eligible patients in the Phase Ib/II clinical trial include hypomethylating agent (HMA) naïve, *TP53* mutated MDS and AML. All enrolled patients were to receive APR-246 as a 4,500 mg fixed dose IV daily for 4 days and AZA over 7 days in 28-day cycles. The primary endpoint of the trial is CR rate.

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 Myelodysplastic Syndromes

Myelodysplastic syndromes (MDS) represents 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.

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.

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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