Aprea Therapeutics Receives FDA Breakthrough Therapy Designation for APR-246 in Combination with Azacitidine for the Treatment of Myelodysplastic Syndromes (MDS) with a TP53 Mutation

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BOSTON, Jan. 30, 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 the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for APR-246 in combination with azacitidine for the treatment of myelodysplastic syndromes (MDS) with a susceptible *TP53* mutation.

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 directly contribute to disease progression and a poor overall prognosis.

“Breakthrough Therapy Designation further supports our development program for APR-246 in combination with azacitidine in MDS patients with a *TP53* mutation,” said Christian S. Schade, Chief Executive Officer of Aprea. “Outcomes for MDS patients with a *TP53* mutation are poor and there are no current therapeutic options specifically for these patients. We look forward to continued interaction with FDA regarding our ongoing Phase 3 clinical study and our clinical development program to advance APR-246.”

The FDA’s Breakthrough Therapy Designation is intended to expedite the development and review of a drug candidate that is planned to treat a serious or life-threatening disease or condition when preliminary clinical evidence indicates that the drug may demonstrate substantial improvement over available therapies on one or more clinically significant endpoints.

About p53 and APR-246

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 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 APR-246 and azacitidine for frontline treatment of *TP53* mutant MDS is ongoing. APR-246 has received 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 Aprea Therapeutics

Aprea Therapeutics Inc., (NASDAQ: APRE) is a biopharmaceutical company headquartered in Boston, Massachusetts with research facilities in Stockholm, Sweden, focused on developing and commercializing novel cancer therapeutics that reactivate the mutant tumor suppressor protein p53. The Company’s lead product candidate is APR-246, a small molecule in clinical development for hematologic malignancies, including myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML). 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 www.ir.aprea.com as a means of disclosing material nonpublic information and for complying with its disclosure obligations under Regulation FD.

Forward-Looking Statements

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 and regulatory submissions. We may, in some cases use terms such as “predicts,” “believes,” “potential,” “continue,” “anticipates,” “estimates,” “expects,” “plans,” “intends,” “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 and the other risks set forth in our filings with the U.S. Securities and Exchange Commission, including our Quarterly Report on Form 10-Q. 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.

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