Aprea Therapeutics Receives OK from FDA to Initiate Phase 1 Clinical Studies for Next-Generation Mutant p53 Reactivator, APR-548

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BOSTON, Oct. 07, 2020 (GLOBE NEWSWIRE) -- Aprea Therapeutics Inc., (NASDAQ: APRE), a clinical-stage biotechnology company focused on developing and commercializing novel cancer therapeutics that reactivate mutant p53 tumor suppressor protein, today announced that the U.S. Food and Drug Administration (FDA) has accepted the Company's Investigational New Drug (IND) application for APR-548 to treat *TP53* mutant myelodysplastic syndromes (MDS). APR-548 is a next-generation small molecule reactivator of mutant p53 that is being developed for oral administration.

"The IND acceptance by FDA is an important milestone for APR-548 and Aprea's development of next-generation reactivators of mutant p53," said Christian S. Schade, Chairman and Chief Executive Officer of Aprea Therapeutics. "We look forward to initiating the Phase 1 clinical trial of APR-548 in MDS and to expanding our leadership in the development of needed therapeutic options for patients with p53 mutated cancers."

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 eprenetapopt (APR-246), a small molecule in clinical development for hematologic malignancies, including myelodysplastic syndromes (MDS) and acute myeloid leukemia (AML). Eprenetapopt 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. 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 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 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 is ongoing. Eprenetapopt has received Breakthrough Therapy, Orphan Drug and Fast Track designations from the U.S. Food and Drug Administration for MDS, and Orphan Drug designation from the European Medicines Agency for MDS, AML and ovarian cancer.

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. A Phase 1 clinical trial of APR-548 in *TP53* mutant MDS is planned.

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 in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2020. 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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