



## **Aprea Therapeutics Receives FDA Fast Track Designation for Eprenetapopt in the Treatment of TP53 Mutant Acute Myeloid Leukemia (AML)**

November 30, 2020

BOSTON, Nov. 30, 2020 (GLOBE NEWSWIRE) -- Aprea Therapeutics, Inc. (Nasdaq: APRE), a biopharmaceutical company focused on developing and commercializing novel cancer therapeutics that reactivate the mutant tumor suppressor protein, p53, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for eprenetapopt in the treatment of patients with *TP53* mutant acute myeloid leukemia (AML). The Company previously received Breakthrough Therapy, Orphan Drug and Fast Track designations for eprenetapopt in the treatment of patients with *TP53* mutant myelodysplastic syndromes (MDS).

The FDA's Fast Track designation is intended to facilitate the development and review of drug candidates that treat serious conditions and address an unmet medical need. A drug candidate that receives Fast Track designation may be eligible for more frequent interaction with the FDA to discuss the drug candidate's development plan as well as eligibility for accelerated approval and priority review.

"We are pleased to have received Fast Track designation for eprenetapopt in the treatment of *TP53* mutant AML, a cancer for which outcomes are poor and there are no current therapeutic options specifically for these patients," said Eyal C. Attar, M.D., Chief Medical Officer of Aprea. "Emerging data from our AML trials evaluating eprenetapopt with azacitidine, and with eprenetapopt, azacitidine and venetoclax, are promising and we continue to enroll patients to identify the best treatment regimen. As these data mature in 2021, we look forward to continued interaction with FDA as we map out opportunities for an accelerated pathway to potential approval."

### **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, 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, Fast Track designation from the FDA for AML, 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](http://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 is ongoing. Eprenetapopt has received Breakthrough Therapy, Orphan Drug and Fast Track designations from the FDA for MDS, Fast Track designation from the FDA for AML, 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. Enrollment in a Phase 1 clinical trial of APR-548 is anticipated to begin in the first quarter of 2021

### **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.

Corporate Contacts:

Scott M. Coiante  
Sr. Vice President and Chief Financial Officer  
617-463-9385

Gregory A. Korb  
Vice President of Business Development  
617-463-9385



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