Recap and Update of Positive Data from Three Presentations in Patients with TP53 Mutant Myelodysplastic Syndromes (MDS) and Acute Myeloid Leukemia (AML) Presented at the 2021 American Society of Hematology (ASH) Annual Meeting

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BOSTON, Dec. 14, 2021 (GLOBE NEWSWIRE) -- Lead investigators from clinical trials evaluating *eprenetapopt* in patients with *TP53* mutant MDS or AML presented positive, updated data at the 2021 ASH Annual Meeting. All studies evaluated the tolerability and efficacy of Aprea Therapeutics, Inc. (Nasdaq: APRE) lead product candidate, *eprenetapopt*.

Data summaries of the presentations are as follows:

Title: Phase II Trial of Eprenetapopt (APR-246) in Combination with Azacitidine (AZA) As Maintenance Therapy for *TP53* Mutated AML or MDS Following Allogeneic Stem Cell Transplantation (SCT)
Presenter: Asmita Mishra, M.D., H. Lee Moffitt Cancer Center and Research Institute Tampa, Florida
Oral Abstract Session: 723. Allogeneic Transplantation: Long-term Follow-up and Disease Recurrence

> **Data Summary**: In 33 patients enrolled in the trial, the relapse free survival (RFS) at 1 year post-transplant was 60% and the median RFS was 12.5 months. The overall survival (OS) at 1 year post-transplant was 79%, with a median OS of 20.6 months. The post- transplant regimen of eprenetapopt and azacitidine was well tolerated among patients in the clinical trial.

Title: Long-Term Follow-up and Combined Phase 2 Results of Eprenetapopt (APR-246) and Azacitidine (AZA) in Patients with *TP53* Mutant Myelodysplastic Syndromes (MDS) and Oligoblastic Acute Myeloid Leukemia (AML)
Presenter: David Sallman, M.D., H. Lee Moffitt Cancer Center and Research Institute, Tampa, Florida
Oral Abstract Session: 637. Myelodysplastic Syndromes—Clinical and Epidemiological: Treatment of High Risk Myelodysplastic Syndrome

> **Data Summary:** By ITT analysis (n=100), ORR was 69% and CR was 43% by International Working Group criteria; Biallelic *TP53* mutation or complex karyotype was significantly associated with higher CR rate (49% versus 8%; P=0.01); combination therapy was well tolerated in treated patients.

Title: Phase I and Expansion Study of Eprenetapopt (APR-246) in Combination with Venetoclax (VEN) and Azacitidine (AZA) in *TP53*-Mutant Acute Myeloid Leukemia (AML)
Presenter: Guillermo Garcia-Manero, M.D., The University of Texas MD Anderson Cancer Center, Houston, Texas
Poster Abstract Session: 616. Acute Myeloid Leukemias: Investigational Therapies, Excluding Transplantation and Cellular Immunotherapies: Poster III

> **Data Summary:** In 39 efficacy evaluable patients ORR was 64%, CR was 39%, and CR + CRi and the CR + CRh rates were each 56%. The triplet combination of eprenetapopt, venetoclax and azacitidine, was tolerable as an outpatient regimen.

Presentations of these data can be accessed from “Presentations” in the News and Events section of the Company’s website at Link.

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. 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. Eprenetapopt is currently on clinical hold in myeloid malignancies. Eprenetapopt has received 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.

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.

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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