Aprea Therapeutics Announces First Patients Enrolled in Phase Ib/II Clinical Study of APR-246 for the Treatment of Myelodysplastic Syndrome

May 24, 2017

May 24, 2017 —BOSTON, MA., and STOCKHOLM, SWEDEN, May 23, 2017 – Aprea Therapeutics, a privately held, clinical stage biopharmaceutical company developing novel anticancer therapies targeting the tumor suppressor protein p53, announced today that the first patient was enrolled in the Phase Ib/II clinical study of APR-246 in myelodysplastic syndrome (MDS). The study, sponsored by the Moffitt Cancer Center with financial support from the Evans MDS Clinical Research Consortium and the MDS foundation, will evaluate APR-246 in combination with azacitadine for the treatment of TP53 mutant MDS.

"The initiation of this clinical trial in MDS is the next step in Aprea's clinical development strategy for APR-246 and we are pleased to collaborate with the Moffitt Cancer Center on this important study," said Christian S. Schade, President and Chief Executive Officer of Aprea Therapeutics. "We are committed to advancing clinical evaluation of APR-246 in both solid tumors and hematological malignancies. Together with our ongoing Phase II clinical study in ovarian cancer and upcoming clinical studies in other tumor types, this Phase Ib/II study in MDS affirms Aprea's leadership in developing next-generation anticancer treatments targeting p53."

"My hope is that the combination of APR-246 with azacitidine in MDS patients with TP53 mutation will be a promising therapeutic option, leading to TP53 clonal eradication and improved survival," said David Sallman MD, who is the lead principal investigator of this study.

About Aprea Therapeutics

Aprea Therapeutics is a Boston, Massachusetts- and Stockholm, Sweden-based biopharmaceutical company focused on the discovery and development of novel anticancer compounds reactivating the tumor suppressor protein, p53. The Company's lead program, APR-246, a first-in-class small molecule drug candidate, is in Phase II clinical development in ovarian cancer patients, and additional clinical studies with APR-246 in other cancer indications are planned. In March 2016, Aprea completed a EUR 46 million Series B financing with an international syndicate co-led by Versant Ventures and 5AM Ventures, with additional participation by Sectoral Asset Management, HealthCap, acting as local lead investor, and existing investor, Karolinska Development. For more information, please visit www.apreatherapeutics.com.

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 anticancer drugs and poor overall survival, representing a major unmet medical need in the treatment of cancer.

APR-246 has been shown to reactivate mutant p53 protein – by reconverting mutant p53 into wild-type p53 conformation and function – and thereby induce programmed cell death in human cancer cells. APR-246 has demonstrated compelling pre-clinical antitumor activity in a wide variety of solid and hematological (blood) tumors, including ovarian cancer, small cell lung cancer, oesophageal cancer and AML (acute myeloid leukemia), among others. Additionally, strong synergy has been seen with both traditional anticancer agents, such as chemotherapy, as well as newer mechanism-based anticancer drugs and immuno-oncology checkpoint inhibitors. In addition to pre-clinical testing, a Phase I/Ib 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 p53 gene. The Company has commenced a Phase II study on ovarian cancer and is expecting to initiate additional clinical studies of APR-246 in other cancer indications.

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