# Aprea AB announces efficacy and safety data in high-grade serous ovarian cancer patients treated with investigational APR-246 presented at 2016 ASCO Annual Meeting

June 6, 2016

**June 6, 2016 —June 6, 2016, Stockholm, Sweden –** Aprea AB, a privately held, clinical-stage biopharmaceutical company developing novel anticancer therapies targeting the tumor suppressor protein p53, today presented clinical data from the Phase Ib part of the ongoing PiSARRO Phase Ib/II trial in collaboration with the European Network for Translational Research in Ovarian Cancer (EUTROC). Aprea's PiSARRO trial is investigating the safety and efficacy of APR-246 in combination with carboplatin and pegylated liposomal doxorubicin (PLD) in patients with relapsed high-grade serous ovarian cancer. Results presented at the Annual Meeting of the American Society of Clinical Oncology (ASCO) showed:

- APR-246 can be combined with standard chemotherapy at relevant doses, allowing the highest tested dose to be selected for continuing the study in a randomized Phase II trial.
- All 21 patients treated in the study that are evaluable according to RECIST criteria have stable disease or better. In addition, 15 out of 18 evaluable patients had a GCIG CA-125 (tumor antigen biomarker) response after three treatment cycles. Overall response rate (GCIG or RECIST) was 18/24 (75%).
- APR-246 showed linear pharmacokinetics with no accumulation and low intra patient variability and no indication of interaction between APR-246 and chemotherapy, supporting the combination of APR-246 with carboplatin and doxorubicin at relevant doses.
- The main related treatment-emergent Grade 3-4 adverse events have been neutropenia, anemia and vomiting. The most frequent treatment-emergent adverse events have been low-grade gastrointestinal (nausea/vomiting), central nervous system (dizziness) and hematological (neutropenia and thrombocytopenia) events. The hematological side effects can be attributed to the chemotherapy, although a contribution from the addition of APR-246 cannot be ruled out at this time. No new safety concerns have emerged in the study.

Prof. Charlie Gourley, Chair of Medical Oncology and Honorary Consultant in Medical Oncology at the University of Edinburgh, said of the results: "APR-246 is an extremely exciting new agent because it targets tumors with mutant forms of the p53 gene, which is the gene most frequently altered in human cancer. This study shows that APR-246 can be successfully combined with standard chemotherapy for ovarian cancer with minimal additional toxicity. The percentage of patients whose cancer responded to this treatment regime was encouraging and we look forward to validating these findings in a larger clinical trial."

Dr. Mikael von Euler, Chief Medical Officer of Aprea, said: "We are very pleased with the results of the Phase Ib trial and to be able to move this exciting drug forward into a randomized Phase II trial in the third quarter of this year. It is especially important that the patients who have more difficult-to-treat disease seem to get as much benefit as those with less aggressive disease. The current safety profile combined with the evidence of clinical activity suggests that APR-246 might become a very important drug for patients with ovarian cancer. Furthermore, the mechanism suggests that APR-246 might have relevance in other tumor types and we look forward to pursuing those opportunities."

**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, esophageal 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. In addition to pre-clinical testing, a Phase I clinical study has been completed, demonstrating a favorable safety profile and both biological and clinical responses in hematological tumors with mutations in the p53 gene. APR-246 is currently in a Phase Ib/II clinical trial in patients with high-grade serous ovarian cancer. The Phase Ib part has completed. In the Phase II clinical study, Aprea will enroll up to 400 ovarian cancer patients in Europe and the US. Patients will be randomized between carboplatin and pegylated liposomal doxorubicin with or without APR-246; the primary endpoint for the study is progression-free survival (PFS). The company is also expecting to begin additional clinical studies of APR-246 in other cancer indications.

**About Aprea**

Aprea AB is a Stockholm, Sweden and Boston, Massachusetts 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 Ib/II clinical development in ovarian cancer patients. In March 2016, Aprea completed a Euro 46 million Series B financing with an international syndicate co-led by Versant Ventures and 5AM Ventures, with additional participation by Sectoral Asset Management and HealthCap, acting as local lead investor. For more information, please visit www.aprea.com.

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