Aprea Therapeutics Receives FDA Fast Track Designation and Orphan Drug Designation for APR-246 for the Treatment of Myelodysplastic Syndromes (MDS)

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April 16, 2019 —BOSTON, MA. and STOCKHOLM, SWEDEN, April 16, 2019 – Aprea Therapeutics, a privately held, clinical stage biopharmaceutical company developing novel anticancer therapies targeting the tumor suppressor protein p53, today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to APR-246 for the treatment of patients with MDS having a *TP53* mutation. In addition, FDA has also granted Orphan Drug Designation to APR-246 for treatment of MDS.

"The granting of Fast Track designation and Orphan Drug Designation by FDA for APR-246 in *TP53* mutated MDS underscores the significant unmet medical need in this disease," said Christian S. Schade, President and Chief Executive Officer of Aprea. "With our Phase 3 clinical study in MDS underway, we look forward to continuing our productive dialogue with FDA and bringing APR-246 to patients as soon as possible."

The FDA's Fast Track program facilitates the development of drugs intended to treat serious conditions and that have the potential to address unmet medical needs. A drug program with Fast Track status is afforded greater access to the FDA for the purpose of expediting the drug's development, review and potential approval. In addition, the Fast Track program allows for eligibility for Accelerated Approval and Priority Review, if relevant criteria are met, as well as for Rolling Review, which means that a drug company can submit completed sections of its New Drug Application (NDA) for review by FDA, rather than waiting until every section of the NDA is completed before the entire application can be submitted for review.

Orphan Drug Designation is granted by the FDA Office of Orphan Products Development to advance the evaluation and development of safe and effective therapies for the treatment of rare diseases or conditions affecting fewer than 200,000 people in the U.S. The designation can provide development and commercial incentives for designated compounds and medicines, including eligibility for a seven-year period of market exclusivity in the U.S. after product approval, FDA assistance in clinical trial design, tax credits related to clinical trial expenses, and an exemption from FDA user fees.

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

APR-246 has been shown to reactivate mutant and inactivated p53 protein – by restoring wild-type p53 conformation and function – and thereby induce programmed cell death in human cancer cells. APR-246 has demonstrated pre-clinical anti-tumor activity in a wide variety of solid and hematological (blood) tumors, 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 I/II clinical program with APR-246 has been completed, demonstrating a favorable safety profile, biological activity and clinical responses in hematological malignancies and solid tumors with mutations in the *TP53* gene.

About Myelodysplastic Syndromes

Myelodysplastic syndromes (MDS) represents a spectrum of hematopoietic stem cell malignancies in which bone marrow fails to produce sufficient healthy blood cells. Approximately 30-40% of MDS patients progress to acute myeloid leukemia (AML) and mutation of the p53 tumor suppressor protein is thought to contribute to disease progression. Mutations in p53 are found in

approximately 20% of MDS and AML patients and are associated with poor overall prognosis.

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 that reactivate the tumor suppressor protein, p53. The Company's lead drug candidate is APR-246, a first-in-class small molecule in clinical development for myelodysplastic syndromes (MDS), acute myeloid leukemia (AML), as well as additional hematologic and solid tumor malignancies. Aprea has commenced a Phase 3 clinical study in p53 mutated MDS and completed enrollment in a Phase 1b/2 clinical trial in p53 mutated high-risk MDS and oligoblastic AML with APR-246 and azacitidine. Additional Phase 1b/2 studies for APR-246 in MDS and AML are also underway and in planning together with other approved anti-cancer agents. Aprea is also developing second generation p53 reactivators that have best-in-class potential. The Company recently completed a Series C financing raising a total of approximately US$62 million. The financing round was led by the Redmile Group, with participation by Rock Springs Capital and Janus Henderson Investors, and included existing investors: 5AM Ventures, Versant Ventures, HealthCap, Sectoral Asset Management and Karolinska Development AB (Nasdaq Stockholm: KDEV). For more information, please visit www.aprea.com.

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