Synta Announces Advancement of Ganetespib into Phase 3 Extension of AML LI-1 Study for Patients with AML and High-Risk MDS

July 21, 2014

- Follows Positive Interim Analysis of Phase 2 Results -

- First of Three Randomized Studies Sponsored by Cardiff University and Supported by the Leukemia & Lymphoma Research Fund and Cancer Research UK to Include Ganetespib -

LEXINGTON, Mass.--(BUSINESS WIRE)--Jul. 21, 2014-- Synta Pharmaceuticals Corp. (NASDAQ: SNTA) today announced the advancement of ganetespib into the Phase 3 extension of the AML LI-1 (less intensive) trial. AML LI-1 is a multicenter, randomized Phase 2/3 clinical study evaluating several novel treatment regimens, including the combination of ganetespib with low dose cytarabine (Ara-C), in newly diagnosed elderly patients with acute myeloid leukemia (AML) or high-risk myelodysplastic syndrome (MDS) who are not eligible for intensive chemotherapy. Ganetespib is a next-generation inhibitor of the chaperone protein Hsp90, which is critical for the activation and stability of numerous proteins that drive cancer growth and proliferation. Ganetespib has been studied in over 1000 patients to date.

Advancement into the Phase 3 extension follows an interim analysis of results from 50 patients who received the ganetespib-cytarabine combination in the Phase 2 portion of the trial. The primary efficacy outcome in Phase 2 was rate of complete response. Per protocol, the Phase 3 extension will include an interim futility analysis and enroll approximately 200 patients in the ganetespib-cytarabine and the cytarabine alone arms, for a total of approximately 400 patients. The primary efficacy endpoint for the Phase 3 extension will include overall survival. The Company is currently in discussion with study investigators, and anticipates providing additional details, including the timing of study milestones, as they become formalized.

The AML LI-1 trial is the first of three multicenter, randomized studies supported by the Leukemia & Lymphoma Research Fund and Cancer Research UK to include a ganetespib treatment arm. AML LI-1 is being conducted under the auspices of the UK's National Cancer Research Institute (NCRI) Haematological Oncology Study Group, with investigators in Denmark, France, New Zealand, and the UK, and under the sponsorship of Cardiff University, UK. The other two studies, to be initiated later this year, are the AML-18 trial, evaluating ganetespib with standard DA (daunorubin and Ara-C) in patients over 60 years old who can tolerate intensive chemotherapy, and the AML-19 trial, evaluating ganetespib in combination with conventional chemotherapy in younger patients with AML.

"AML LI-1's 'pick-the-winner' trial design is intended to rapidly assess new therapeutics with the potential to improve outcomes in very difficult to treat patients with AML and MDS," said Professor Alan K. Burnett, Head of Haematology at Cardiff University and Chief Investigator of the AML LI-1 trial. "We look forward to continuing the ganetespib-cytarabine combination study in this population, and to further evaluating ganetespib's full potential in AML through the comprehensive research program developed by the UK NCRI Group, which includes the AML-18 and AML-19 trials."

"Ganetespib's progress in the AML LI-1 study underscores the potential of Hsp90 inhibition across a broad range of cancers," said Dr. Vojo Vukovic, Chief Medical Officer, Synta. "We look forward to elucidating this potential through large, randomized studies, including the ongoing Company-sponsored GALAXY-2 Phase 3 trial in advanced non-small cell lung adenocarcinoma and investigator-sponsored trials in AML/MDS, breast cancer and ovarian cancer."

About AML and MDS

AML is a rapidly progressing hematologic cancer characterized by uncontrolled proliferation of immature blast cells in the bone marrow. The American Cancer Society estimates there will be approximately 18,860 new cases of AML and approximately 10,460 deaths in the U.S. in 2014. AML patients with relapsed or refractory disease and newly diagnosed AML patients over 60 years of age with poor prognostic risk factors typically die within one year, resulting in an acute need for new treatment options for these patients.

MDS is a hematopoietic stem cell neoplasm characterized by disordered and ineffective hematopoiesis which results in irreversible decline in the number and quality of blood-forming cells. Patients often develop severe anemia requiring frequent blood transfusions. In most cases progressive bone marrow failure results in neutropenia and thrombocytopenia, and in about one third of patients the disease progresses into AML, usually within a few years.

About Ganetespib

Ganetespib, an investigational drug candidate, is a selective inhibitor of heat shock protein 90 (Hsp90), a molecular chaperone which controls the folding and activation of a number of client proteins that drive tumor development and progression. Many solid and hematologic tumors are dependent on Hsp90 client proteins including proteins involved in "oncogene addiction" (ALK, HER2, mutant BRAF and EGFR, androgen receptor, estrogen receptor, and JAK2); proteins involved in resistance to chemotherapy and

radiation therapy (ATR, BCL2, BRCA1/2, CDK1/4, CHK1, survivin, and WEE1); proteins involved in angiogenesis (HIF-1alpha, VEGFR, PDFGR, and VEGF); and proteins involved in metastasis (MET, RAF, AKT, MMPs, HIF-1alpha, and IGF-1R). In preclinical models, inhibition of Hsp90 by ganetespib results in the inactivation, destabilization, and eventual degradation of these cancer-promoting proteins. Ganetespib is being evaluated in trials in lung cancer, breast cancer, and other tumor types. The most common adverse event seen to date has been transient, mild or moderate diarrhea, which has been manageable with standard supportive care. Information on these trials can be found at www.clinicaltrials.gov. Ganetespib has received Fast Track designation from FDA for second-line treatment of non-small cell lung adenocarcinoma in combination with docetaxel.

About Synta Pharmaceuticals

Synta Pharmaceuticals Corp. is a biopharmaceutical company focused on discovering, developing, and commercializing small molecule drugs to extend and enhance the lives of patients with severe medical conditions, including cancer and chronic inflammatory diseases. Synta has a unique chemical compound library, an integrated discovery engine, and a diverse pipeline of clinical- and preclinical-stage drug candidates with distinct mechanisms of action and novel chemical structures. All Synta drug candidates were invented by Synta scientists using our compound library and discovery capabilities. For more information, please visit www.syntapharma.com.

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