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AMBIT BIOSCIENCES CORPORATION PRESENTS PHASE I CLINICAL RESULTS OF AC220 IN PATIENTS WITH ACUTE MYELOID LEUKEMIA AT ASH CONFERENCE

San Diego, CA – Dec. 7, 2009 – Ambit Biosciences Corporation announced today that preliminary results from a clinical trial of its lead product candidate, AC220, in acute myeloid leukemia (AML) were presented at the 51st Annual Meeting of the American Society of Hematology (ASH) in New Orleans. AC220 is a novel, orally available, small molecule that was expressly optimized as a FMS-like tyrosine kinase-3 (FLT3) inhibitor for the treatment of AML.

The open-label, dose-escalation study, “AC220, a Potent, Selective, Second Generation FLT3 Receptor Tyrosine Kinase Inhibitor, in a First-in-Human Phase I AML Study,” was designed to evaluate the safety, tolerability, and pharmacokinetic profile of AC220 in AML patients with predominantly relapsed or refractory disease. AC220 was generally well-tolerated in AML patients, and importantly, there was no treatment-related mortality observed in this study. Clinical response and median survival were assessed as secondary endpoints and analyzed for the overall study population, in addition to patient subsets based on the presence or absence of the FLT3 genotype for the internal tandem duplication (ITD) mutation (FLT3 ITD+ and FLT3 ITD-, respectively). Following treatment with AC220, responses were observed in both FLT3 ITD+ and FLT3 ITD- patients, and responders in each group had at least a doubling in median survival versus non-responders.

“AML represents a challenging hematological malignancy to treat. A significant portion of AML patients have activating FLT3 mutations, and these patients have a particularly poor prognosis and are often refractory to current treatment options,” said Jorge Cortes MD, Internist and Professor, Deputy Chair, Department of Leukemia, Division of Cancer Medicine, The University of Texas M. D. Anderson Cancer Center, Houston, TX, and primary investigator for the Phase I Study. “These encouraging results with AC220 warrant further studies of this promising compound as a monotherapy and in combination with other treatments in patients with AML.”

Twenty-three (30%) of the 76 total patients responded, including 10 (56%) of the 18 FLT3 ITD+ patients, nine (20%) of the 45 FLT3 ITD- patients and four (31%) of the 13 patients whose FLT3 genotype was undetermined. Responders to AC220 included patients who had previously been treated with other investigational FLT3 inhibitors. The 10 patients (13% of the total patient population) with a complete response (CR) consisted of five FLT3 ITD+ patients, three FLT3 ITD- patients, and two patients whose FLT3 genotype was undetermined. The median duration of response across all responders was 12 weeks.

The overall study population had a median survival time of 14 weeks. Responders had a prolonged median survival versus non-responders, with 26 weeks versus nine weeks, respectively, in FLT3 ITD+ patients, and 19 versus nine weeks, respectively, for FLT3 ITD- patients.

AC220 plasma exposure was sustained between dose intervals and continued to increase in a dose-proportional manner for 12 to 450 mg/day, with a half-life of approximately 1.5 days. Adverse event (AE) data suggest that AC220 is safe and well-tolerated. The most common drug-related AEs in the study were gastrointestinal related (nausea, vomiting, dysgeusia, abdominal pain, anorexia, diarrhea), skin irritation, and peripheral edema. Each of these drug-related adverse events occurred in less than 15% of the study population, with most occurring in less than 10% of patients, and were primarily mild to moderate in severity. In addition, some patients experienced QTcF interval prolongation, which was asymptomatic and reversible. The maximum tolerated dose was determined to be 200mg continuous daily dosing.

Study Design

The trial enrolled and dosed 76 AML patients with a median age of 60 years who had received a median of four prior therapies. Patients were not selected for mutations at enrollment. In this trial, 18 (24%) patients were FLT3 ITD+, 45 (59%) patients were FLT3 ITD- and 13 (17%) were patients with an undetermined FLT3 genotype. Patients enrolled in the first 10 dosing cohorts received an intermittent dosing cycle of oral, once-daily AC220 (14 days on, 14 days off), escalating from 12 to 450 mg/day, with subsequent cohorts receiving continuous dosing of once-daily AC220 at 200 and 300 mg/day for 28 days.

About AC220

AC220, Ambit's lead product candidate, is a novel, potent, highly selective, orally bioavailable second-generation FLT3 inhibitor currently under evaluation as a monotherapy treatment in adult patients with relapsed/refractory acute myeloid leukemia (AML). AML is the most common type of blood cancer in adults, and the kinase FLT3 is mutated and constitutively activated in 25-40 percent of such patients. FLT3 ITD mutations predict poor prognosis and decreased response to existing treatments, including chemotherapy and hematopoietic stem cell transplant. Ambit leveraged KINOMEScan™, the company's proprietary, high-throughput method for screening small molecule compounds against a large number of human kinases, to advance AC220 from concept to lead candidate selection in only 18 months.

About Acute Myeloid Leukemia (AML)

Acute myeloid leukemia is a form of blood cancer. According to the American Cancer Society, approximately 13,000 new cases of AML will be diagnosed in the United States in 2008. The median age of a patient with AML is about 67 years. Standard treatment for patients 60 years or older with AML includes systemic combination chemotherapy. The median survival for patients receiving induction chemotherapy, which is associated with high mortality, is 6-11 months, with shorter survival for patients over the age of 60 years. The five-year survival rate for AML is less than 15 percent due to refractory and relapsed disease associated with standard treatments. According to a report from Decision Resources, the U.S. AML market is expected to more than double by 2015.

About Ambit Biosciences

Ambit Biosciences is a privately-held biopharmaceutical company engaged in the discovery and development of small molecule kinase inhibitors for the treatment of cancer, inflammatory disease, and other indications. Ambit employs a novel and proprietary kinase profiling technology, KINOMEScan™, to screen compounds against 442 human kinases.

Ambit's lead compound, AC220, is in clinical development for the treatment of AML and other indications. Ambit plans to commence in 2009 and 2010 several clinical studies with AC220, including a registration study in AML. Ambit's clinical pipeline also includes AC480, an oral pan-HER inhibitor that was in-licensed from BMS. Ambit is conducting Phase 2 studies with AC480 in patients with solid tumor cancers. Additionally, Ambit has an advancing pool of preclinical candidates targeting BRAF (in collaboration with Cephalon), JAK2, Aurora, and CSF1R. Through its KINOMEscan Division, Ambit markets its technology as a profiling service. For more information, visit www.ambitbio.com.

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