



AMBIT BIOSCIENCES INITIATES FIRST PHASE 2 CLINICAL TRIAL OF AC220 IN ACUTE MYELOID LEUKEMIA

Potential to Support U.S. Regulatory Filing

San Diego, CA – Dec. 11, 2009 – Ambit Biosciences Corporation announced today the enrollment and dosing at the University of Texas M. D. Anderson Cancer Center of the first patient in the ACE (**AC220** Monotherapy **Efficacy**) Phase 2 pivotal trial in patients with relapsed or refractory acute myeloid leukemia (AML). AC220 is a novel, orally available, potent and highly selective small molecule that was specifically designed as a FMS-like tyrosine kinase-3 (FLT3) inhibitor. The ACE study is designed to be a pivotal trial that could potentially support a United States regulatory filing for AC220 to address a targeted AML patient population with the internal tandem duplication (ITD) activating mutation for FLT3.

"Based on the promising results of our first-in-human clinical trial presented at the American Society of Hematology (ASH), we are pleased to advance AC220 into this important Phase 2 study in AML. We are deeply grateful to our investigators, their staff, and their patients, as well as our entire team at Ambit, for their vital contributions in achieving this significant milestone," said Wendell Wierenga, EVP of Research and Development at Ambit. "If the ACE study is successful, AC220 could emerge as the first once-a-day, orally available targeted therapy for AML patients. Over the next several months we plan to initiate additional clinical trials with AC220 to support the potential registration in AML, and to explore its utility in certain types of solid tumors."

"AML is a devastating disease for which few treatment options exist," 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, and principal investigator for the ACE Study. "There are no treatments currently approved by the FDA that specifically target the activating ITD mutations of the FLT3 kinase, present in 25 to 40 percent of AML patients, and a key prognostic factor of clinical outcome. Not only has AC220 demonstrated clinical activity in patients with FLT3 ITD mutations, but the drug has also been well tolerated. We look forward to continuing to evaluate the safety and efficacy of AC220 as a single agent in relapsed and refractory AML patients."

Phase 2 ACE Study Design

The ACE study is a single-arm, Phase 2 multinational clinical trial that will evaluate a 200 mg dose of AC220, taken orally once daily as a monotherapy treatment in FLT3 ITD-positive AML patients who have relapsed or are refractory after first-line AML therapy, age 60 years or older, or after second-line AML therapy or stem cell transplant, age 18 years or older. The co-primary endpoints of the study are overall composite complete remission rate and complete remission rate. Secondary endpoints include duration of remission, progression-free survival, overall survival, safety and tolerability. The ACE study is scheduled to enroll 180 patients across an estimated 100 sites in the United States, Canada, France, Germany, Italy, the Netherlands, Poland, Spain, and the United Kingdom. Screening at several sites in

the United States has already commenced, and study initiation at additional sites in the United States, Canada and Europe is expected to commence in early 2010.

The study is based on data from a first-in-human Phase 1 clinical trial of AC220 presented earlier this month at the ASH meeting in New Orleans. The preliminary results from that trial in heavily pre-treated AML patients (median of four prior courses of therapy) with predominantly relapsed or refractory AML showed that AC220 was generally well tolerated up to and including once daily continuous dosing of 200 mg, and no treatment-related mortality was observed in the study. Responses were seen in both FLT3 ITD-positive and FLT3 ITD-negative AML patients, with an overall response rate of 56 and 20 percent, respectively. Responders in both the FLT3 ITD-positive and FLT3 ITD-negative groups had at least a doubling of median overall survival compared to non-responders (26 vs. nine weeks for FLT3 ITD-positive, and 19 vs. nine weeks for FLT3 ITD-negative). The most common drug-related AEs in the study were gastrointestinal-related (nausea, vomiting, dysgeusia, abdominal pain, anorexia, diarrhea), skin irritation, and peripheral edema. All 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 200 mg continuous once-daily dosing.

For more information about the ACE Study, please visit <http://www.clinicaltrials.gov/ct2/show/NCT00989261?term=ac220&rank=1>, or call 858-334-2136 in the United States, or email to ACEstudy@ambitbio.com.

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 and elderly patients with relapsed or 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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