ACADIA Pharmaceuticals Announces Encouraging Results from Initial Clinical Trials with ACP-104 in Patients with Schizophrenia

July 13, 2006

SAN DIEGO--(BUSINESS WIRE)--July 13, 2006--ACADIA Pharmaceuticals Inc. (Nasdaq:ACAD), a biopharmaceutical company utilizing innovative technology to fuel drug discovery and clinical development of novel treatments for central nervous system disorders, today announced results from three initial clinical studies of ACP-104 in patients with schizophrenia. The results of these studies demonstrated that ACP-104 is safe and well tolerated after repeated dosing of up to 600 mg per day, and that initial signals of antipsychotic effects were observed within the tolerated dose range of ACP-104. In addition, plasma levels of ACP-104 correlate with brain receptor occupancies indicating good penetration of ACP-104 into the brain. The three studies enrolled an aggregate of 74 patients with schizophrenia and were conducted in collaboration with Professor Carol Tamminga, M.D., from the University of Texas Southwestern Medical School in Dallas, Texas.

"The first three clinical trials of ACP-104 were successful in that they demonstrated a solid safety and tolerability profile for the compound and provided encouraging indications of antipsychotic activity in patients with schizophrenia," said Dr. Tamminga, Principal Investigator of the ACP-104 clinical studies. "Tolerability was better than anticipated and, in general, the patients in these studies positively evaluated their experience with ACP-104. These study results show the potential of ACP-104 as an innovative therapy for patients with schizophrenia and strongly support its further development."

Single Ascending-Dose Study

The first clinical trial was a randomized, double-blind, placebo-controlled, single ascending-dose study primarily designed to evaluate the safety, tolerability and pharmacokinetics of ACP-104 in patients with schizophrenia. A total of 24 patients were enrolled in the study and were assigned to one of five treatment cohorts. Within each cohort, each patient received placebo and two distinct doses of ACP-104 ranging from 25 mg to 250 mg on separate days.

Results of this study show that ACP-104 is safe and well tolerated at all doses tested. No dose-limiting toxicities or serious adverse events were observed in the study and a maximum tolerated dose was not reached. All adverse events were mild to moderate in severity, with the most frequent being sedation. No significant changes were observed in safety parameters such as electrocardiogram (ECG) measures (including QT/QTc interval), clinical chemistries and hematology. No extrapyramidal side effects were observed in the patients.

Multiple Ascending-Dose Study

The second clinical trial was a 14-day, steady-state, double-blind, placebo-controlled multiple ascending-dose study in patients with schizophrenia. This study was primarily designed to evaluate the safety, tolerability and pharmacokinetics of ACP-104, as well as to explore preliminary signals of antipsychotic effects. A total of 40 patients with schizophrenia were enrolled in the study in six dose cohorts. The patients were randomly assigned to ACP-104 or placebo in a treatment-to-placebo ratio of about 3:1. The patients were treated with escalating daily doses of ACP-104 ranging from 100 mg (50 mg twice-daily) to 800 mg (400 mg twice-daily) for 14 days. In the first four cohorts, patients received a fixed dose of ACP-104 or placebo for the first four days, and then the patients were escalated to a higher dose for the remaining ten days of the study. In the last two cohorts involving the 600 mg and 800 mg doses, there was a titration period followed by a period of fixed dosing.

The results of this study indicate that ACP-104 is safe and well tolerated at doses tested up to 600 mg per day, a dose considered to be the maximum tolerated dose in the study. Although this was a small study with a limited treatment duration, initial signals of antipsychotic effects, as indicated by reductions in Positive and Negative Syndrome Scale (PANSS) scores, were observed in patients given the two highest tolerated doses (400 mg and 600 mg) of ACP-104.

Adverse events were generally mild to moderate in severity. The most common adverse events included sleepiness, increased salivation, constipation, and tachycardia (an increased heart rate). No significant changes were observed in safety parameters such as ECG measures (including QT/QTc interval), and clinical chemistries. No extrapyramidal side effects were observed in the patients.

Six patients were discontinued in the study for adverse events, including three patients from the 800 mg cohort. Two of the adverse events were classified as serious adverse events, including one instance of a seizure deemed by the investigator as unrelated to the study drug, and one instance of a short-lasting fever of unknown origin. The fever was subsequently followed three days later by a transient and mild decrease in white blood cell counts referred to as mild leukopenia that was deemed most likely due to viral infection, but a possible relationship to the study drug could not be ruled out. The other four adverse events leading to discontinuation included instances of tachycardia and hypertension, which were deemed related to the study drug, and one instance of a fever, which was deemed unrelated to the study drug.

Positron Emission Tomography Study

The third study was an open label single-dose positron emission tomography (PET) study that was designed to determine the relationship between plasma levels of ACP-104 and occupancy of 5-HT(2A) receptors in the brain. A total of 10 patients with schizophrenia were enrolled in the study and received single oral doses of ACP-104 ranging from 25 mg to 150 mg. There was a relationship between plasma levels of ACP-104 and the degree of 5-HT(2A) receptor occupancy at all doses indicating good penetration of ACP-104 into the brain. Both the 100 mg and 150 mg doses of ACP-104 yielded significant 5-HT(2A) receptor occupancy comparable to that previously seen with clozapine at clinically effective doses.

"The demonstration that high doses of ACP-104 are well tolerated and provide signals of antipsychotic effects, coupled with the correlating plasma levels and brain receptor occupancies establishes a strong foundation for pursuing more advanced clinical studies with ACP-104, including a subsequent Phase IIb clinical trial," said Uli Hacksell, Ph.D., Chief Executive Officer of ACADIA.

About ACP-104

ACP-104, or N-desmethylclozapine, is the major metabolite of clozapine, and is being developed by ACADIA as a novel, stand-alone therapy for schizophrenia. It combines an atypical antipsychotic efficacy profile with the added potential benefit of enhanced cognition, thereby addressing one of the major challenges in treating schizophrenia today. ACP-104 combines M(1) muscarinic agonism, 5-HT(2A) inverse agonism, and D(2) and D(3) dopamine partial agonism in a single compound and, therefore, uniquely addresses what ACADIA believes are the three most promising target mechanisms for treating schizophrenia. ACADIA's development program for ACP-104 is supported in part by the Stanley Medical Research Institute (SMRI). SMRI is the largest private source of research funding in the United States for severe mental illness and is based in Bethesda, Maryland.

About Schizophrenia

Schizophrenia is a chronic disabling mental illness characterized by disturbances such as hallucinations and delusions as well as a range of cognitive disturbances and negative symptoms, including social withdrawal. Cognitive disturbances and negative symptoms are believed to be the major cause of patients' functional impairment and often prevent patients with schizophrenia from being fully contributing members of society. Despite the availability of current antipsychotic drugs with worldwide sales of approximately $14 billion in 2004, cognitive disturbances are poorly addressed by existing therapies and represent a large unmet medical need in the treatment of schizophrenia.

About ACADIA Pharmaceuticals

ACADIA is a biopharmaceutical company utilizing innovative technology to fuel drug discovery and clinical development of novel treatments for central nervous system disorders. ACADIA currently has five Phase II-stage clinical programs as well as a portfolio of preclinical and discovery assets directed at large unmet medical needs, including schizophrenia, Parkinson's disease, sleep maintenance insomnia, and neuropathic pain. All of the drug candidates in ACADIA's product pipeline emanate from discoveries made using its proprietary drug discovery platform. ACADIA's corporate headquarters is located in San Diego, California and it maintains research and development operations in both San Diego and Malmo, Sweden.

Forward-Looking Statements

Statements in this press release that are not strictly historical in nature are forward-looking statements. These statements include but are not limited to statements related to the potential for ACP-104 as a therapy for schizophrenia, any potential cognitive or other benefits of ACP-104, the safety, tolerability and efficacy of ACP-104, and future clinical trials of ACP-104. These statements are only predictions based on current information and expectations and involve a number of risks and uncertainties. Actual events or results may differ materially from those projected in any of such statements due to various factors, including the risks and uncertainties inherent in drug discovery, development and commercialization, collaborations with others and litigation. For a discussion of these and other factors, please refer to ACADIA's annual report on Form 10-K for the year ended December 31, 2005 filed with the United States Securities and Exchange Commission as well as other subsequent filings with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. This caution is made under the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. All forward-looking statements are qualified in their entirety by this cautionary statement and ACADIA undertakes no obligation to revise or update this press release to reflect events or circumstances after the date hereof.

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