Acerta Pharma Announces Study Published in New England Journal of Medicine Demonstrates Acalabrutinib (ACP-196) Shows Marked Activity in Relapsed Chronic Lymphocytic Leukemia

Clinical Trial Reports a 95 Percent Response Rate in Relapsed CLL, Most Prevalent Form of Adult Leukemia

Clinical Data to be Presented at ASH 2015 Annual Meeting on December 7, 2015

REDWOOD CITY, Calif. and OSS, the Netherlands, Dec. 7, 2015 -- Acerta Pharma B.V. (Acerta), a clinical-stage biopharmaceutical company, announced today the New England Journal of Medicine (NEJM) has published Phase 1-2 clinical data on the investigational drug acalabrutinib, a novel, second-generation, selective and potent inhibitor of Bruton's tyrosine kinase (Btk). The study showed a 95 percent response rate in patients with relapsed chronic lymphocytic leukemia (CLL), the most prevalent form of adult leukemia. The NEJM manuscript for acalabrutinib can be found at www.NEJM.org.

John C. Byrd, MD, of The Ohio State University Comprehensive Cancer Center, will present the clinical data for acalabrutinib at the 57th Annual Meeting of the American Society of Hematology (ASH) in Orlando, Florida today. The data highlights the results of 61 patients with relapsed CLL who were sequentially enrolled and treated at six sites across the United States and United Kingdom. The median age of the patients was 62 years, and patients had received a median of three previous therapies for CLL; 31 percent had chromosome 17p13.1 deletion, and 75 percent had unmutated immunoglobulin heavy-chain variable genes. Investigators reported an overall response rate of 95 percent (85 percent partial response, 10 percent partial response with lymphocytosis) at a median follow-up of 14.3 months. The most common adverse events were headache, diarrhea, and increased weight. Most adverse events were grade 1-2, self-limiting and resolved over time. No patient experienced major hemorrhage, atrial fibrillation, or Richter's transformation (when CLL transforms into an aggressive form of lymphoma), and only one patient's cancer progressed.

"We are thrilled to share clinical data for acalabrutinib for the first time. The results published in the NEJM and the presentation by Dr. Byrd at the 2015 ASH meeting are milestones advancing Acerta closer to our goal of becoming a leader in oncology drug development," said David Johnson, Chief Executive Officer of Acerta. "This is the first of what we believe will be many successful developments emerging from Acerta. The acalabrutinib results communicated today represent a potential step forward in the development of treatments to improve the outcomes of patients living with CLL. Acerta would like to recognize and thank the patients, investigators, and collaborators who have made this work possible."

"The data is very exciting. What is particularly remarkable is how well patients are tolerating this therapy," said Dr. Byrd, corresponding author and principal investigator of the study. "Btk inhibitors are transforming CLL from an incurable to a chronic disease, especially considering that standard CLL therapies typically produce a 35-40 percent response rate in this disease setting." Dr. Byrd has no financial interests in Acerta.

Acalabrutinib is in active clinical development for CLL and other malignancies, including a global phase 3 study of acalabrutinib compared with ibrutinib, a marketed first-generation Btk inhibitor. Acalabrutinib is an investigational agent and is not approved for marketing by any regulatory authority.

About Acalabrutinib (ACP-196)

Acalabrutinib, also known as ACP-196, is a novel, orally bioavailable, second-generation, irreversible inhibitor of Bruton's tyrosine kinase (Btk) that was rationally designed to be more potent and selective than existing Btk inhibitors. Acerta is currently developing acalabrutinib in multiple hematologic malignancies and solid tumors, as well as rheumatoid arthritis. Acalabrutinib is an investigational agent and is not currently approved by any regulatory authority. A total of 21 clinical trials are currently open and evaluating acalabrutinib alone and in combination with multiple other agents including pembrolizumab, obinutuzumab, and ACP-319. For a list of available trials, visit www.clinicaltrials.gov.

About Acerta Pharma

Acerta is a leader in the field of covalent binding technology and is applying this technology to create novel selective therapies intended for the treatment cancer and autoimmune diseases. Acerta's lead molecule, acalabrutinib (ACP-196), is a selective and potent inhibitor of Btk. Acerta is also developing ACP-319, a novel selective inhibitor of PI3k delta. The company has operations in Oss, the Netherlands and multiple U.S. sites. The U.S. headquarters is in Redwood City, CA.