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Acetylon Pharmaceuticals Reports Ricolinostat (ACY-1215) is Active and Well-tolerated in Ongoing Phase 1b Studies in Patients with Refractory Multiple Myeloma at the 19th Congress of the EHA

BOSTON – June 13, 2014 – Acetylon Pharmaceuticals, Inc., the leader in the development of selective histone deacetylase (HDAC) inhibitors for enhanced therapeutic outcomes, today presented an update from ongoing Phase 1b studies in which ricolinostat (ACY-1215) has continued to demonstrate promising activity and tolerability in combination with Revlimid® (lenalidomide) or Velcade® (bortezomib) for the treatment of relapsed or refractory multiple myeloma. Data from the studies were presented at the 19th Congress of the European Hematology Association (EHA) in Milan, Italy.

All 22 evaluable patients receiving ricolinostat in combination with Revlimid demonstrated stable disease or better, with 64% having partial response or better, including 1 complete response, 5 very good partial responses, and 8 partial responses (including 1 PRu) as evaluated by the International Myeloma Working Group (IMWG) standards. 3 patients also achieved minor responses. 10 of the evaluable patients were previously refractory to either full dose or maintenance Revlimid. Ricolinostat in combination with Velcade demonstrated an overall response rate (ORR), defined as partial response or better, of 53% in 19 evaluable patients, or 36% of the intention-to-treat population. 10 patients were previously refractory to Velcade.

“In these early studies, the selective HDAC6 inhibitor, ricolinostat, appears to yield signs of clinical activity, including responses in some patients previously refractory to lenalidomide or bortezomib, when it is dosed in combination regimens with each respective agent,” commented Noopur Raje, MD, Associate Professor, Department of Medicine, Harvard Medical School, Director, Multiple Myeloma Program, Medical Oncology, Massachusetts General Hospital, and investigator in both clinical studies of ricolinostat. “In addition, ricolinostat has demonstrated a safety profile that has allowed for extended cycles of treatment with these standard-of-care agents.”

Ricolinostat has been well-tolerated in both studies to-date. In combination with Revlimid, treatment emergent adverse events (AEs) have been mostly low grade and considered not related to ricolinostat and included diarrhea, fatigue, headache, muscle spasms, neutropenia, rash and upper respiratory infection. Two grade 3 neutropenia events and one grade 3 syncope event were considered possibly related to ricolinostat. In combination with Velcade, treatment emergent AEs have been mostly grade 1-2 and not attributed to study drug. AEs greater than or equal to grade 3 have included hematologic abnormalities (11), elevated amylase (2), other asymptomatic laboratory abnormalities (3), and one patient with stomach cramps, diarrhea, and fatigue. One patient with cardiac history had a fatal pulmonary embolism after 3 cycles of treatment. No maximum tolerated dose has been established in either trial. Pharmacodynamic assessments in peripheral blood cells showed preferential increases in acetylated tubulin, a marker of HDAC6 inhibition, compared to acetylated histone, a Class 1 HDAC marker, in all patients receiving doses of 80 mg or greater.

"We are encouraged by the responses we have seen with ricolinostat in combination with Revlimid and Velcade thus far, and we look forward to continued results from these studies," commented Catherine A. Wheeler, MD, Vice President, Clinical Development of Acetylon.

About Ricolinostat

Blood cancers such as multiple myeloma and lymphoma are characterized by successive genetic mutations resulting in uncontrolled cell proliferation and dysfunctional production of intracellular proteins. Ricolinostat (ACY-1215) selectively inhibits the intracellular enzyme HDAC6, which leads to an accumulation of excess protein and in addition may disrupt critical proliferative signals in malignant cells. Disruption of these molecular processes in cancer cells triggers programmed cell death, called "apoptosis," with little or no effect on normal cells. Currently available HDAC drugs affect the expression of numerous genes in normal cells as well as cancer cells, which can result in side effects such as gastrointestinal dysfunction, lowered blood platelet levels and risk of hemorrhage and profound fatigue as well as potential for significant cardiac toxicity. Selective inhibition of HDAC6 is expected to reduce or eliminate these often-severe side effects associated with non-selective HDAC inhibition and may enable the development of optimized treatment regimens, including maximally effective combination drug therapies.

About Acetylon

Acetylon Pharmaceuticals, Inc., based in Boston, Massachusetts, is a leader in the development of novel small molecule drugs targeting epigenetic mechanisms for the enhancement of therapeutic outcomes in cancer and other critical human diseases. The Company's epigenetic drug discovery platform has yielded a proprietary portfolio of optimized, orally-administered Class I and Class II histone deacetylase (HDAC) selective compounds. Alteration of HDAC regulation through selective HDAC inhibition is thought to be applicable to a broad range of diseases including cancer, sickle cell disease and beta-thalassemia, and autoimmune and neurodegenerative diseases. Acetylon's lead drug candidate, ricolinostat (ACY-1215), is a selective HDAC6 inhibitor currently in Phase 1b clinical development for the treatment of multiple myeloma. The Company recently announced a strategic collaboration agreement with Celgene Corporation, which includes an exclusive option for the future acquisition of Acetylon by Celgene. Acetylon's scientific founders are affiliated with Harvard University, the Dana-Farber Cancer Institute, the Massachusetts General Hospital, and Harvard Medical School. www.acetylon.com

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