Acetylon Presents Early Phase 1a/1b Results for Citarinostat (ACY-241) in Combination with Pomalyst® and Dexamethasone Showing Promising Treatment Responses in Relapsed or Relapsed-and-Refractory Multiple Myeloma

-- Data to be presented at the 58th Annual Meeting of the American Society of Hematology --

BOSTON – December 4, 2016 – Acetylon Pharmaceuticals, Inc., the leader in the development of selective histone deacetylase (HDAC) inhibitors for enhanced therapeutic outcomes, today announced that it will present initial clinical data from a Phase 1a/1b clinical trial evaluating the safety and preliminary anti-tumor activity of the selective HDAC6 inhibitor citarinostat (ACY-241), in combination with pomalidomide (Pom) (Pomalyst®, Celgene) and dexamethasone (Dex) for the treatment of relapsed or relapsed-and-refractory multiple myeloma (RRMM) at the 58th Annual Meeting of the American Society of Hematology (ASH) in San Diego, California.

"Early results of this study with citarinostat closely parallel recently published positive data for the Phase 2 trial of Acetylon's first selective HDAC6 inhibitor ricolinostat in combination with Pom and Dex in multiple myeloma, and compare favorably to historical controls. In early follow-up data for Celgene's MM-003 trial of Pom/Dex versus high dose Dex, there was a 17% overall response rate at 4.2 months. At a 4-month median follow up with citarinostat in combination with the same Pom/Dex regimen, we are seeing an impressive overall response rate of 46% as well as substantial improvement in progression free survival standing at 6.5 versus historical 4 months," said Robert Markelewicz, Senior Medical Director at Acetylon. "While these are still interim data, we are seeing that citarinostat combines favorably with Pom and Dex, and we will continue cohort expansion to explore selected biomarkers and confirm the dose and schedule for a planned pivotal trial."

Citarinostat (ACY-241) is an orally available selective HDAC6 inhibitor that is structurally similar to ricolinostat (ACY-1215), and administered in tablet form. The ACE-MM-200 study is a Phase 1a/1b clinical trial to determine the maximum tolerated dose, safety, and preliminary anti-tumor activity of citarinostat alone and in combination with pomalidomide and dexamethasone in patients with relapsed or relapsed-and-refractory multiple myeloma. Its sequential monotherapy/combination trial design allows patients access to combination therapy based on an established regimen starting in the second cycle of treatment, while establishing the safety, pharmacokinetics, and pharmacodynamics of citarinostat as both a monotherapy and in combination.

Initial results of the study suggest that citarinostat is well tolerated, with no maximum tolerated dose (MTD) observed at doses up to 480 mg once-a-day as a monotherapy and up to 360 mg once-a-day in combination with Pom/Dex. Tolerability in combination is similar to that reported for Pom/Dex alone. In 56 efficacy evaluable patients with a 4-month median follow-up, the confirmed overall response rate (ORR) was 46%, with a clinical benefit rate (CBR) of 59% and disease control rate (DCR) of 91%. The median duration of response (DOR) was 9.2 months and median progression-free survival (PFS) was 6.5 months.

Notably, similar response rates were seen across the refractory subsets, including patients who were previously refractory to pomalidomide and daratumumab. A dose of 360 mg once-a-day was selected as the recommended Phase 2 dose for citarinostat based on similarly low incremental toxicity, higher PK/PD exposure, and similar clinical efficacy when compared to the 180 mg dose.

Details of the presentation are as follows:

Date: Sunday, December 4, 2016

Time: 6:00pm – 8:00pm PST

Location: Hall GH (San Diego Convention Center)

Session: 653. Myeloma: Therapy, excluding Transplantation: Poster II

Abstract Number: 3307

Title: Selective HDAC6 Inhibitor ACY-241, an Oral Tablet, Combined with Pomalidomide and Dexamethasone: Safety and Efficacy of Escalation and Expansion Cohorts in Patients with Relapsed or Relapsed-and-Refractory Multiple Myeloma (ACE-MM-200 Study)

About HDAC6 Selective Inhibition

Citarinostat (ACY-241) and ricolinostat (ACY-1215) selectively inhibit the intracellular enzyme HDAC6, leading to an accumulation of excess protein and disrupting 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. HDAC6 inhibition also enhances immune responses to cancer cells, both singly and in synergistic combination with immunomodulatory drugs (IMiDs), immune checkpoint inhibitor antibodies, and/or cytotoxic antibodies. Currently available HDAC drugs non-selectively affect the expression of numerous other 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 anticipated to reduce or eliminate these often-severe side effects associated with non-selective HDAC inhibition and to enable the development of optimized treatment regimens, including maximally effective combination drug therapies.

About Acetylon

Acetylon Pharmaceuticals, Inc., based in Boston, Massachusetts, is the leader in the development of novel, selective 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 Class I and Class II histone deacetylase (HDAC) selective compounds for oral administration. Alteration of HDAC regulation through selective HDAC inhibition is thought to be applicable to a broad range of diseases including cancer, autoimmune and neurodegenerative diseases, neuropathy, and hemoglobinopathies including sickle cell disease and beta-thalassemia. www.acetylon.com

###

CONTACT:

Acetylon
Walter C. Ogier
President and Chief Executive Officer
(617) 245-1300

MEDIA:
MacDougall Biomedical Communications
Kari Watson or Casey R. Doucette, Ph.D.
(781) 235-3060
kwatson@macbiocom.com or cdoucette@macbiocom.com