



**FOR IMMEDIATE RELEASE**

**Acetylon Pharmaceuticals Announces Initiation of Phase 1-2a Clinical Trial of ACY-1215  
for Multiple Myeloma**

**BOSTON, Mass. – September 22, 2011** – Acetylon Pharmaceuticals today announced the initiation of patient treatments in a three-part Phase 1-2a clinical trial of ACY-1215, an oral Class II histone deacetylase (HDAC) inhibitor, in adults with relapsed and relapsed/refractory multiple myeloma. The Phase 1 dose-ranging study will evaluate ACY-1215 alone and in combination with bortezomib (Velcade®) and dexamethasone, a current standard of care for treatment of myeloma. Following identification of optimal combination dosing in Phase 1, the Phase 2a portion of the study will evaluate rate and duration of cancer response to ACY-1215 in combination with standard of care treatment to achieve proof-of-concept. Selective inhibition of the intracellular enzyme HDAC6 may provide enhanced anti-cancer effectiveness through potentially reduced side effects and improved tolerability compared to current non-selective inhibitors, which are active against both Class I and Class II HDACs. This clinical trial is being conducted by Acetylon with alliance support from The Leukemia & Lymphoma Society (LLS).

“Acetylon’s efficient methodology to design specific small molecule orally bioavailable HDAC inhibitors has allowed us to quickly advance our first drug candidate, ACY-1215, into clinical trials within two and a half years after program inception,” said Walter C. Ogier, President and Chief Executive Officer and co-founder of Acetylon. “Multiple myeloma is a challenging disease for physicians and patients alike because, in spite of remarkable advances in patient care over the past two decades, current treatments provide only short-lived benefit for many patients, and they often have debilitating side-effects which necessitates early termination of potentially effective care. Eventually, physicians run out of treatment alternatives for patients. Acetylon is dedicated to finding new, safe and efficacious therapies for this disease that surpass current treatment options.”

“Selective inhibition of HDAC6 by ACY-1215 targets a key protein degradation pathway known to be upregulated in certain cancers, including multiple myeloma. ACY-1215 is thus designed to specifically target cancer cells to achieve disease response accompanied by a potentially more favorable side effect profile compared to currently available HDAC drugs, which can cause dysregulation of normal cells due to comparative lack of selectivity,” commented Sagar Lonial, MD, Professor and Vice Chair of Clinical Affairs in the Department of Hematology and Medical Oncology at the Winship Cancer Institute of Emory University and lead investigator in the clinical trial. “This trial is the first clinical step towards development of optimized HDAC6 inhibitor treatment regimens for multiple myeloma, including maximally effective combination drug therapies.”

The Phase 1a and 1b portions of the trial will enroll patients with relapsed or relapsed/refractory multiple myeloma in a 21-day treatment cycle to determine the maximum tolerated dose of ACY-1215 as monotherapy or in combination with bortezomib and dexamethasone, followed by up to five additional sequential cycles of treatment. The Phase 2a portion is designed to determine the objective response rate to ACY-1215 in combination with bortezomib and dexamethasone in patients with



relapsed or relapsed/refractory multiple myeloma over up to six 21-day cycles. The trial is being conducted in several major cancer centers across the United States, including the Massachusetts General Hospital Cancer Center, the University of Wisconsin Carbone Cancer Center, the Winship Cancer Institute of Emory University, and others still pending initiation.

#### **About ACY-1215**

Blood cancers such as multiple myeloma are characterized by successive genetic mutations resulting in rapid cell proliferation and excess production of intracellular proteins. ACY-1215 selectively inhibits the intracellular enzyme HDAC6, leading to inactivation of the “aggresome” pathway for degradation of damaged proteins. The resultant accumulation of excess waste protein in malignant cells triggers programmed cell death, called “apoptosis,” with little or no effect on normal cells. Currently available HDAC drugs non-selectively target multiple HDAC enzymes including those of Class I, resulting in dysregulated expression of numerous genes in normal cells as well as cancer cells. Side effects commonly associated with non-selective HDAC drugs include gastrointestinal dysfunction, lowered blood platelet levels and risk of hemorrhage, and profound fatigue as well as potential for severe cardiac complications. 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 Pharmaceuticals, Inc.**

Acetylon Pharmaceuticals, Inc. is applying its unique capabilities to discover and develop next-generation, highly selective small molecule drugs to realize the therapeutic potential of HDAC inhibition to treat cancer, inflammatory and other diseases, while reducing the side effects common to this class of drugs. The Company is located in Boston and is based on technology initially developed at the Dana-Farber Cancer Institute and at Harvard University. [www.acetylon.com](http://www.acetylon.com)

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