The Leukemia & Lymphoma Society and Acetylon Pharmaceuticals Partner to Advance the Clinical Development of Acetylon's ACY-1215 Drug Candidate for Multiple Myeloma

- Funding Will Support the First Clinical Trial of a Class II-Selective Next Generation HDAC Inhibitor -

WHITE PLAINS, New York and BOSTON, Massachusetts, May 11, 2011 – The Leukemia & Lymphoma Society (LLS) and Acetylon Pharmaceuticals today announced a newly formed business alliance whereby Acetylon and LLS will jointly support a Phase I/II clinical trial of Acetylon's oral selective HDAC6 inhibitor drug candidate, ACY-1215, in multiple myeloma. LLS has committed to provide up to $4.85 million in milestone-tranched funding to Acetylon, and a joint clinical oversight committee will be formed. Acetylon is focused on the discovery and development of potential drug candidates based on next-generation Class II-selective HDAC inhibitors.

"We are very excited about the rapid bench-to-bedside translation of HDAC6-selective inhibitors in multiple myeloma, both alone and in combination with proteasome inhibitors," said Ken Anderson, MD, the Kraft Family Professor of Medicine at the Dana-Farber Cancer Institute and Harvard Medical School and co-founder of Acetylon. "Based on our preclinical studies, we expect ACY-1215 to demonstrate very potent antitumor activity and a very favorable side effect profile. We are highly honored and most grateful to partner with The Leukemia & Lymphoma Society in bringing this first-in-class selective HDAC6 inhibitor to patients."

"Selective inhibition of HDAC proteins has the potential to dramatically improve the efficacy and tolerability of this promising class of targeted therapies. The successful development of ACY-1215, the first pharmaceutical HDAC6 selective inhibitor, represents a major advance. Acetylon's partnership with The Leukemia & Lymphoma Society reflects a shared interest in realizing an improved standard of care for patients with blood cancers, and in particular multiple myeloma," said James (Jay) E. Bradner, MD, Assistant Professor of Medicine at the Dana-Farber Cancer Institute and Harvard Medical School, who is also a co-founder of Acetylon.

"Multiple myeloma is one of the most common and therapeutically challenging hematologic malignancies, and development of a therapeutic agent that reduces side effects while providing greater efficacy is a key goal for LLS," said Richard Winneker, Senior Vice President, Research at LLS. "Our work with Acetylon represents LLS' commitment to finding cures in multiple myeloma and all other blood cancers and explores the clinical potential for HDAC6-selective inhibition, which has demonstrated exciting results in preclinical studies at the Dana-Farber Cancer Institute and Massachusetts General Hospital."

"The Leukemia & Lymphoma Society has been a driving force behind many breakthroughs in the treatment of multiple myeloma, as well as other blood cancers," said Walter C. Ogier, President and Chief Executive Officer and co-founder of Acetylon Pharmaceuticals, Inc. "We

are excited to partner with them and grateful that they have agreed to provide major support for the clinical development of Acetylon's lead drug candidate, ACY-1215."

Under the agreement, Acetylon will conduct a three-part Phase I/II clinical trial of ACY-1215 in adults with relapsed and relapsed/refractory multiple myeloma to achieve human proof-of-concept for selective HDAC6 inhibition. LLS will provide $4.85 million in non-dilutive, milestone-based, conditionally repayable funding, representing half of the projected costs of the clinical trial. The Phase Ia portion of the trial involving patients with relapsed and relapsed/ refractory multiple myeloma is expected to begin in the next several months.

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," in stressed cancer cells, 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 The Leukemia & Lymphoma Society

The Leukemia & Lymphoma Society® (LLS) is the world's largest voluntary health agency dedicated to blood cancer. The LLS mission: Cure leukemia, lymphoma, Hodgkin's disease and multiple myeloma, and improve the quality of life of patients and their families. LLS funds lifesaving blood cancer research around the world and provides free information and support services. Founded in 1949 and headquartered in White Plains, NY, LLS has chapters throughout the United States and Canada. To learn more, visit www.LLS.org or contact the Information Resource Center at (800) 955-4572. www.lls.org.

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, autoimmune 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. Drs. Anderson and Bradner are scientific founders and shareholders of Acetylon Pharmaceuticals. www.acetylon.com

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