FOR IMMEDIATE RELEASE

Acetylon Announces Publication of Preclinical Pharmacokinetic, Pharmacodynamic and Bone Disease Results for ACY-1215 in Multiple Myeloma

-- Results from *In Vivo* and *In Vitro* Studies were Presented at the 53rd Annual Meeting of the American Society of Hematology --

BOSTON, Mass., December 12, 2011 – Acetylon Pharmaceuticals today announced the publication of preclinical safety and effectiveness results for its oral selective HDAC6 inhibitor drug candidate, ACY-1215, which has recently entered Phase 1-2a clinical trials in North America. The results include favorable pharmacokinetic and pharmacodynamic properties that confirm HDAC6 selectivity and potent anti-cancer activity for the small molecule drug candidate as well as cellular evidence for the potential to limit painful and disabling bone disease in multiple myeloma. The results have been published in a special on-line edition of the scientific journal of the American Society of Hematology (ASH), *Blood*, 118(21), Abstracts 2908 and 2912, Nov. 17, 2011) and were be presented on Sunday, December 11th, at the 53rd ASH Annual Meeting and Exposition in San Diego, California.

In studies conducted at the Massachusetts General Hospital and at the Dana-Farber Cancer Institute, ACY-1215, when administered either as a single agent or in synergistic combination with the proteasome inhibitor bortezomib (Velcade®, Takeda Millennium Pharmaceuticals), demonstrated potent and selective hyperacetylation of cellular tubulin, a marker of HDAC6 inhibition, versus histone acetylation in tumors. Tubulin hyperacetylation was correlated to effectiveness both *in vitro* and *in vivo* in disease models of multiple myeloma, as well as against drug-resistant multiple myeloma patient cells. ACY-1215, when administered *in vitro* either as a single agent or with bortezomib, enhanced bone-building cellular osteoblast formation and calcium deposition, while inhibiting harmful osteoclast differentiation and bone resorption which are otherwise promoted in multiple myeloma.

"These latest preclinical results provide further support of the clinical investigation of ACY-1215 in multiple myeloma, including the Phase 1a/1b/2a clinical trial of ACY-1215 that is now underway at our own institution and at several other major US hospitals," stated Noopur Raje, MD, Director of the Center for Multiple Myeloma at the Massachusetts General Hospital Cancer Center and Associate Professor of Medicine at Harvard Medical School. "Based on the favorable impact on bone disease in our preclinical models, we are further encouraged that HDAC6 inhibition may provide substantial benefit for multiple myeloma patients."

"The reversal of bone damage in multiple myeloma is a known clinical benefit of proteasome inhibition, and we believe this is the first preclinical evidence that HDAC6 inhibition may provide added, synergistic patient benefit and enhanced quality of life regarding this terribly painful and debilitating consequence of multiple myeloma," commented Simon Jones, PhD, Vice President of Biology and Preclinical Development at Acetylon. "We will closely monitor this potential, added measure of patient benefit in our further clinical trials of ACY-1215."

"These preclinical data clearly demonstrate the potential advantage of HDAC6-selective inhibitors such as ACY-1215 versus currently available pan-HDAC inhibitors such as Zolinza, also called vorinostat or SAHA. The results also demonstrate the optimal pharmacokinetics, pharmacodynamic properties and powerful anti-myeloma effectiveness of ACY-1215 in preclinical disease models," commented John H. van Duzer, PhD, Vice President of Chemistry and Manufacturing at Acetylon. "Our expectation is that the HDAC6 selectivity profile of ACY-1215 has the potential to provide enhanced benefits to patients since it is ideally suited for synergistic, combination with other oral myeloma drugs including Velcade and the next generation of proteasome inhibitors and with dexamethasone."

Acetylon is currently focused on the development of potential drug candidates based on next-generation Class II-selective histone deacetylase (HDAC) inhibitors. The Class IIB enzyme, HDAC6, has emerged as an important target in inflammatory disease, neurologic disease and broadly in cancer. Acetylon Pharmaceuticals believes that its next-generation, selective HDAC inhibitor compounds may accomplish enhanced clinical utility by reducing or eliminating the debilitating and sometimes life-threatening side effects associated with the current first-generation of non-selective HDAC inhibitors and providing enhanced disease response and patient outcomes.

The two *Blood* publications, titled "Pharmacodynamic and Pharmacokinetic properties of a novel and selective HDAC6 inhibitor, ACY-1215, in Combination with Bortezomib in Multiple Myeloma," and "Selective HDAC6 inhibition via ACY-1215, either alone or in combination with bortezomib, restores osteoblast function and suppresses osteoclast differentiation in multiple myeloma," are authored by Loredana Santo, MD, Noopur Raje, MD and colleagues of the Massachusetts General Hospital Cancer Center, Division of Hematology and Oncology, as well as scientific collaborators at the Dana-Farber Cancer Institute, Division of Hematologic Neoplasia and the Lurie Family Imaging Center and Department of Pediatric Oncology; the Massachusetts General Hospital, Center for Systems Biology; the Brigham and Women's Hospital, Department of Pathology; Harvard Medical School; and Acetylon Pharmaceuticals Inc. (all located in Boston, MA).

About HDAC6 Inhibition

Acetylon's lead HDAC6 inhibitor program is focused on enhancing drug potency and reducing or eliminating side effects common to HDAC inhibition through highly selective targeting of the HDAC6 enzyme. Inhibition of HDAC6 versus other isoforms uniquely preserves normal gene expression in cells, thereby minimizing patient toxicity. At the same time, HDAC6 inhibition severely disrupts diseased cells' ability to produce normal proteins, through disruption of the

HSP-90 protein chaperone system and to dispose of damaged misfolded proteins through modification of microtubules and disruption of the aggresome protein disposal pathway. Metabolically active cancer and autoimmune cells produce large amounts of misfolded proteins and inhibition of HDAC6 further increases the generation and accumulation of protein "trash", triggering self-destruction of diseased cells via programmed cell death and leading to regression of disease.

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. www.acetylon.com

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