



FOR IMMEDIATE RELEASE

## **Acetylon Announces Publication of Preclinical Safety and Efficacy Results for Lead Drug Candidate ACY-1215 in Multiple Myeloma**

-- Results from *In Vivo* and *In Vitro* Studies Presented  
at the 52<sup>nd</sup> Annual Meeting of the American Society of Hematology --

**BOSTON, Mass., December 6, 2010** – [Acetylon Pharmaceuticals](#) today announced the publication of favorable results of preclinical safety and efficacy testing of its oral selective HDAC6 inhibitor drug candidate, ACY-1215, in multiple myeloma. 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 first-in-class proteasome inhibitor drug bortezomib (Velcade<sup>®</sup>, Takeda Millennium Pharmaceuticals), demonstrated effectiveness in two *in vivo* disease models of multiple myeloma as well as against drug-resistant multiple myeloma patient cells. Inhibition of HDAC6 results in inhibition of a critical intracellular mechanism for degradation of misfolded proteins called the “aggresome” pathway, which was demonstrated to result in potent killing of myeloma cells via apoptosis (programmed cell death) while being well tolerated and non-toxic to normal cells. The results have been published in a special on-line edition of the scientific journal of the American Society of Hematology, *Blood*, (116(21), 2997, Nov. 19, 2010) and were presented yesterday at the Society’s 52<sup>nd</sup> Annual Meeting in Orlando, Florida.

“These preclinical efficacy and safety results provide the rationale for the initiation of human clinical trials of ACY-1215 in multiple myeloma,” stated Noopur Raje, M.D., Associate Professor of Medicine at Harvard Medical School and Director of the Center for Multiple Myeloma at the Massachusetts General Hospital Cancer Center. “We are particularly encouraged by the effectiveness we have observed in several different disease models of this challenging cancer, including overcoming bortezomib resistance in multiple myeloma patient cells by synergistic combination of this selective HDAC6 inhibitor.”

Acetylon is currently focused on the development of potential drug candidates based on next-generation Class II-selective 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.

“Based on these new data, we are encouraged that the selective HDAC6 inhibitor, ACY-1215, in addition to overcoming drug resistance in multiple myeloma, should also overcome the substantial side effect profile that is associated with current first-generation, non-selective HDAC inhibitors,” commented Walter Ogier, President and Chief Executive Officer of Acetylon. “We are currently



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focusing our efforts on advancing ACY-1215 and other promising selective HDAC inhibitors into human clinical trials.”

The *Blood* publication, titled “Selective Inhibition of HDAC 6 With a New Prototype Inhibitor (ACY1215) Overcomes Bortezomib Resistance in Multiple Myeloma,” is authored by Loredana Santo, M.D., and Noopur Raje, M.D., of the Massachusetts General Hospital Cancer Center, Division of Hematology and Oncology along with collaborators at the Dana-Farber Cancer Institute, Division of Hematologic Neoplasia; the Brigham and Women’s Hospital, Department of Pathology; and Acetylon Pharmaceuticals Inc. (all based 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 a) to produce normal proteins, through disruption of the HSP-90 protein chaperone system, and b) to dispose of damaged misfolded proteins through modification of microtubules and disruption of the aggresome protein disposal pathway. Since metabolically active cancer and autoimmune cells produce large amounts of misfolded proteins, inhibition of HDAC6 results in further increased generation and accumulation of protein “trash”, triggering self-destruction of diseased cells via programmed cell death and leading to regression of disease.

### **About Acetylon**

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 based on technology initially developed at the Dana-Farber Cancer Institute and at Harvard University. Acetylon’s technologies were initially discovered and developed by its scientific founders: Kenneth C. Anderson, MD, Kraft Family Professor of Medicine at Harvard Medical School and the Dana-Farber Cancer Institute; James E. Bradner, MD, Assistant Professor of Medicine at Harvard Medical School and the Dana-Farber Cancer Institute; and Ralph Mazitschek, Ph.D., Instructor at Harvard Medical School and the Center for Systems Biology at Massachusetts General Hospital, in conjunction with their academic collaborators.

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