



Avila Therapeutics Presents Data on AVL-292, a Selective Bruton's Tyrosine Kinase Inhibitor, for the Treatment of B Cell Cancers at the 2011 ASH Annual Meeting

*Clinical Data Show Promising Effects of AVL-292 in Patients with Chronic Lymphocytic Leukemia
Preclinical Data Demonstrate Potential of AVL-292 in the Treatment of Multiple Myeloma*

BEDFORD, MA - December 12, 2011 - Avila Therapeutics, Inc., announced today the presentation of clinical and preclinical data for AVL-292, a highly selective Bruton's tyrosine kinase (Btk) inhibitor, at the 2011 American Society of Hematology (ASH) Annual Meeting and Exposition taking place in San Diego, CA. Avila presented data on the safety and pharmacology of AVL-292 from the first two patient cohorts from the ongoing Phase 1b clinical study of AVL-292 in B cell cancers.

AVL-292 demonstrated biological activity in patients suffering from chronic lymphocytic leukemia (CLL), a blood cancer in which B lymphocytes are overproduced. In addition, Avila presented preclinical data demonstrating the therapeutic potential of AVL-292 in the prevention of bone destruction caused by multiple myeloma and multiple myeloma-related bone disease.

"The early clinical activity observed with AVL-292 in patients with B-cell malignancies is very encouraging," said Daruka Mahadevan, MD, PhD, Director of Drug Development and Translational Research at the University of Arizona Cancer Center. "We look forward to further evaluating the potential of AVL-292 in this clinical setting, as there is a critical need for new treatment options for patients with chronic lymphocytic leukemia and B-cell non-Hodgkin lymphomas."

"We were very pleased with this data as our ongoing clinical trial with AVL-292 sets the foundation for its advancement into Phase 2 development next year. In addition these data show the emerging potential of AVL-292 to treat a range of cancer types and further establish Btk as an important target for these patients," said Katrine Bosley, Chief Executive Officer of Avila. "AVL-292 is a prime example of Avila's ability to design and develop targeted covalent drugs. We believe that this approach can address clinical challenges and create medicines for a broad range of diseases."

In a poster presentation entitled "Clinical Development of AVL-292; A Potent, Selective Covalent Btk Inhibitor for the Treatment of B Cell Malignancies," Avila presented clinical data establishing that:

- AVL-292 was generally safe and well-tolerated across the first two dose levels of 125 mg and 250 mg. At each dose level, AVL-292 was administered orally once per day on a continuous basis.
- Five of the six patients treated at these doses have stable disease and continue to receive AVL-292. All subjects in the first dose cohort (125 mg) have been treated for >100 days to date.
- AVL-292 achieved complete and sustained occupancy of Btk (a biomarker of target inhibition) as measured in patient blood samples collected at the 250 mg dose level.



- In all four of the subjects diagnosed with chronic lymphocytic leukemia, absolute lymphocyte counts increased (lymphocytosis) within four weeks of treatment initiation. This treatment effect has also been observed with other B cell receptor pathway inhibitors currently in clinical development.

The ongoing Phase 1b clinical trial of AVL-292 is being conducted in patients with B cell malignancies including B-cell non-Hodgkin lymphoma (B-NHL), chronic lymphocytic leukemia (CLL), and Waldenstrom's macroglobulinemia.

In a second poster presentation entitled "Targeting Bruton's Tyrosine Kinase as a Novel Approach to Inhibit Osteoclast Function in Multiple Myeloma," Avila's collaborators at the Massachusetts General Hospital's Cancer Center presented preclinical results establishing that AVL-292:

- Inhibited bone degradation activity by osteoclasts derived *ex vivo* from multiple myeloma patient monocytes as determined in a bone pit formation assay;
- Inhibited osteoclast function through inhibition of Ca²⁺ mobilization that is stimulated by RANK ligand (RANKL) signaling;
- Reduced osteoclast-stimulated proliferation of multiple myeloma cells in co-culture.

About AVL-292 and Bruton's Tyrosine Kinase (Btk)

AVL-292 is a novel, orally available, covalent drug that inhibits Bruton's tyrosine kinase (Btk). Inhibition of Btk is a promising new approach to treatment of diseases that are driven by B cells, including certain hematologic cancers such as non-Hodgkin lymphoma and B cell chronic lymphocytic leukemia and autoimmune diseases such as rheumatoid arthritis.

AVL-292 selectively and covalently bonds to Btk to inactivate and silence its activity. This mechanism of action confers greater target selectivity and a longer duration of action than is typical of conventional small molecule drugs. In preclinical studies, AVL-292 was efficacious in a variety of animal disease models. AVL-292 is in clinical development and has successfully completed two Phase 1a clinical studies to date.

Development of AVL-292 is supported in part by The Leukemia & Lymphoma Society.

About Avila Therapeutics™, Inc.

Avila Therapeutics is a clinical-stage biotechnology company focused on the design and development of targeted covalent drugs to achieve best-in class outcomes. The company's product pipeline has been built using its proprietary Avilomics™ platform and is currently focused on cancer, viral infection and autoimmune disease. Avila's most advanced product candidate, AVL-292, a potential treatment for cancer and autoimmune diseases, is currently in Phase 1 clinical testing. Avila is funded by leading



venture capital firms: Abingworth, Advent Venture Partners, Atlas Venture, Novartis Option Fund, and Polaris Venture Partners. For additional information, please visit <http://www.avilatx.com>.

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