



Alexion Reports Results from First Clinical Trial of Novel Anti-Cancer Antibody Samalizumab at ASH Annual Meeting

CHESHIRE, Conn.--(BUSINESS WIRE)-- [Alexion Pharmaceuticals, Inc.](#) (Nasdaq: ALXN) today announced the presentation of interim results from the first Phase I/II trial of samalizumab, the company's investigational, first-in-class humanized monoclonal antibody. The ongoing trial is evaluating the safety, maximum tolerated dose, pharmacokinetics, and pharmacodynamics of samalizumab in adult patients with advanced stage B-cell chronic lymphocytic leukemia (B-CLL) or multiple myeloma (MM). Data presented today at the 52nd American Society of Hematology (ASH) Annual Meeting and Exposition in Orlando indicate that samalizumab was well tolerated at all doses studied, exhibited a dose-dependent biological and pharmacokinetic response, and exhibited initial evidence of anti-tumor activity. (1)

"Samalizumab is designed to target the CD200 molecule, which is overexpressed in certain types of cancer. The early-stage clinical findings presented today are consistent with the predicted mechanism of action of samalizumab and support further study of this novel antibody," said Stephen Squinto, Ph.D., Executive Vice President and Head of Research and Development at Alexion. "We look forward to completing this trial, analyzing the final data set, and further investigating samalizumab as an innovative treatment for patients with other cancers."

Twenty-six patients with advanced CLL or MM were enrolled in the sequential, dose-escalation Phase I/II study. Patients received a single IV dose of samalizumab and were eligible to receive additional cycles of a single IV infusion every 28 days if the first dose was tolerated and patients exhibited at least stable disease. Most patients (77%; 20/26) received multiple samalizumab cycles in dose cohorts ranging from 50 mg/m² to 600 mg/m².

Samalizumab appeared to be well tolerated. Adverse events to date were mostly mild or moderate, and were considered manageable. The most common adverse events included fatigue (50%), headache (20%), fever (20%), and rash (20%). Grade 3 to 5 events deemed possibly, probably, or definitely related to study drug included anemia (8%), neutropenia (8%), thrombocytopenia (4%), reduced visual acuity (4%), respiratory syncytial virus infection (4%), muscular weakness (4%), and rash (4%). Samalizumab dosing was associated with no severe or dose-limiting adverse cytokine reactions. The maximum tolerated dose was not reached in this trial.

In patients with sufficient peripheral immune cells to evaluate biological activity, 95% (19/20) showed 81% to 98% reductions in peripheral CD200+ CD4+ T cells. In addition, 67% (14/21) of patients demonstrated 64% to 75% CD200 loss on B-CLL cells following the first dose of samalizumab, which may represent CD200 down-modulation or CD200+ cell loss. These findings are consistent with the predicted immunomodulatory mechanism of action for samalizumab, which is designed to inhibit CD200-dependent immune suppression, enabling a more efficient immune response against CD200+ tumor cells.

Overall, 36% (8/22) of evaluable patients experienced at least a 10% reduction in bulky disease. Notably, one patient, who received 13 cycles of samalizumab (400mg/m²), achieved a confirmed partial response as defined in the protocol, with a maximum 71% reduction in bulky disease by CAT scan together with >50% reduction in absolute lymphocyte count while maintaining neutrophil count $\geq 1.5 \times 10^9/L$.

About Samalizumab

The CD200 molecule is over-expressed on the surface of certain tumors including CLL, multiple myeloma, non-Hodgkins lymphoma, melanoma, ovarian cancer and neuroblastoma. CD200 may inhibit the body's immune response to that tumor, allowing for the growth and survival of tumors. Laboratory data have shown that samalizumab, previously known as ALXN6000, blocks binding of CD200 to the CD200 receptor, expressed on cells of the monocyte/macrophage lineage and on T lymphocytes, which has been shown to enhance the immune response to tumors and reduce tumor growth. (2) Non-clinical anti-tumor activity of samalizumab was reported in the January 2008 issue of the *Journal of Immunology*. (3)

About Chronic Lymphocytic Leukemia

CLL is the second most common type of leukemia in adults. (4) CLL, which involves production of abnormal lymphocytes (white blood cells), starts in the bone marrow and can spread through the blood to the lymph nodes, spleen, liver and other parts of the body. (5) B-cell CLL is characterized by clonal expansion of abnormal B lymphocytes. Patients with CLL may develop enlargement of the spleen and lymph nodes, in addition to pancytopenia (depletion of red and white blood cells and platelets), which ultimately results in hemorrhage, infection and death. (6)

About Multiple Myeloma

Multiple myeloma (MM) is a cancer of the plasma cells in the bone marrow. (7) In people with MM, the uncontrolled growth of myeloma cells can lead to bone destruction, anemia, kidney dysfunction, and other organ dysfunction. (7) Patient prognosis is determined by both the number and specific properties of myeloma cells. (7) CD200 is overexpressed on the majority of myeloma cells, and is an important and independent factor in MM prognosis. (8)

About Alexion

Alexion Pharmaceuticals, Inc. is a biopharmaceutical company working to develop and deliver life-changing drug therapies for patients with serious and life-threatening medical conditions. Alexion is engaged in the discovery, development and commercialization of therapeutic products aimed at treating patients with a wide array of severe disease states, including hematologic and kidney diseases, transplant, other inflammatory disorders, and cancer. Soliris is Alexion's first marketed product. Alexion is evaluating other potential indications for Soliris as well as other formulations of eculizumab for additional clinical indications, and is pursuing development of other antibody product candidates in early stages of development. This press release and further information about Alexion Pharmaceuticals, Inc. can be found at: www.alexionpharma.com.

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Safe Harbor Statement

This news release contains forward-looking statements, including statements related to anticipated clinical development milestones and potential health and medical benefits of samalizumab for the potential treatment of patients with cancer. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, decisions of regulatory authorities regarding marketing approvals, the possibility that results of published reports or clinical trials are not predictive of safety and efficacy of samalizumab in broader patient populations, the risk that clinical trials may not be completed successfully, the risk that third parties won't agree to license any necessary intellectual property to Alexion on reasonable terms or at all, the risk that third party payors will not reimburse for the use of any product at acceptable rates or at all, and a variety of other risks set forth from time to time in Alexion's filings with the Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended September 30, 2010, and in Alexion's other filings with the Securities and Exchange Commission. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

References

- (1) Abstract 2465 entitled "First-in-Human Phase I Dose Escalation Study of a Humanized Anti-CD200 Antibody (Samalizumab) in Patients with Advanced Stage B Cell Chronic Lymphocytic Leukemia (B-CLL) or Multiple Myeloma (MM)," presented in a poster session at the 52nd American Society of Hematology (ASH) Annual Meeting and Exposition, December 5, 2010, at 6:00 p.m. by Dr. Daruka Mahadevan.
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- (3) Kretz-Rommel A, Qin F, Dakappagari N et al. *J Immunol.* 2008; 180 (2):699-705.
- (4) National Cancer Institute. General Information About Chronic Lymphocytic Leukemia. <http://www.cancer.gov/cancertopics/pdq/treatment/CLL/Patient/page1/print>. Accessed on August 5, 2008.
- (5) American Cancer Society. Overview: Leukemia - Chronic Lymphocytic (CLL). http://www.cancer.org/docroot/CRI/CRI_2_1x.asp?nav=criov&dt=62. Accessed on August 4, 2008.
- (6) Catovsky D. Definition and diagnosis of sporadic and familial chronic lymphocytic leukemia. *Hematol Oncol Clin North Am.* 2004; 18(4):783-94.
- (7) Durie BGM. International Myeloma Foundation. Concise Review of the Disease and Treatment Options. 2008/2009 edition. Available at http://myeloma.org/pdfs/cr08-eng_f1web.pdf. Accessed November 18, 2010.
- (8) Moreaux J, et al. CD200 is a new prognostic factor in multiple myeloma. *Blood.* 2006;108(13):4194-7. Available at <http://www.ncbi.nlm.nih.gov/pubmed/16946299>. Accessed November 18, 2010.

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