



ZIOPHARM Oncology, Inc.

May 16, 2013

ZIOPHARM Announces Presentation of Promising Nonclinical and Phase I Study Results of Ad-RTS-IL-12 in Advanced Melanoma at ASGCT 16th Annual Meeting

BOSTON, May 16, 2013 (GLOBE NEWSWIRE) -- ZIOPHARM Oncology, Inc. (Nasdaq:ZIO) today announced promising results from nonclinical and Phase 1 studies in advanced melanoma using Ad-RTS-IL-12, a novel DNA-based therapeutic candidate.

Findings were presented in an oral session at the 16th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT), held May 15-18 in Salt Lake City, Utah. A presentation of the study, titled "Nonclinical and Phase I Clinical Studies with a Regulated Adenoviral Gene Delivery of IL-12 Show Promising Clinical Activity in Unresectable Stage III/IV Melanoma," was delivered by John Nemunaitis, M.D., Executive Medical Director of Mary Crowley Medical Research Center.

For the studies, Ad-RTS-IL-12, utilizing RheoSwitch Therapeutic System® (RTS®) technology, was injected intratumorally. Gene expression and protein production were controlled through the administration of an oral activator ligand (INXN-1001). Nonclinical studies demonstrated a dose-dependent, immune mediated reduction in tumor volume in several different syngeneic tumor models, which led to a Phase 1 dose-escalation study in subjects with unresectable stage III/IV melanoma.

Study subjects (n=14) were treated in four dose cohorts of the activator ligand. Compelling clinical activity was observed in five of seven (71 percent) patients dosed at the two highest dose levels. These five patients experienced prominent inflammatory responses in injected and non-injected lesions and transient increases then decreases in the size of injected and non-injected lesions. Clinical activity in these higher dose cohorts coincided with the highest serum levels of IL-12 and IFN- γ , including a four-fold median increase from baseline at peak levels compared with lower dose cohorts. Flow cytometric analyses of peripheral blood mononuclear cell (PBMC) samples also revealed seven-fold and four-fold median increases from baseline at peak levels in absolute CD3+ and CD8+ T cell values, respectively, compared with lower dose cohorts. The most common related adverse events (AEs) included fever, nausea, vomiting, fatigue and arthralgia. One death occurred in the study, unrelated to treatment, due to septicemia secondary to cellulitis and colitis.

"Ad-RTS-IL-12 is clearly a potent immune stimulator with a demonstrated immune mediated effect in melanoma and other cancers," said Dr. Nemunaitis. "While immune therapy in melanoma has led to advances in treatment, there remains a significant unmet medical need in many patients with this disease. The systemic delivery of IL-12 protein has been explored as a potential treatment of different cancers, but has, thus far, been limited by severe toxicity. In the current study, the controlled expression of IL-12, through a regulatable gene therapy strategy was found to limit systemic toxicity while inducing biological and clinical activity in a dose-dependent fashion. We look forward to seeing how these promising data continue to translate in larger outcome studies, including the ongoing Phase 2 study."

ZIOPHARM is currently conducting a Phase 2 multi-center, single-arm, open-label expansion study in up to 15 patients with unresectable Stage III or IV melanoma.

About ZIOPHARM Oncology, Inc.:

ZIOPHARM Oncology is a biopharmaceutical company focused on the development and commercialization of new cancer therapies. The Company's clinical programs include:

Ad-RTS-IL-12 is currently being tested in two Phase 2 studies, the first for the treatment of advanced melanoma, and the second in combination with palifosfamide for the treatment of non-resectable recurrent or metastatic breast cancer. Ad-RTS-IL-12 uses synthetic biology to enable controlled delivery of therapeutic interleukin-12 (IL-12), a protein important for enhancing the development of an immune response to cancer. In partnership with Intrexon Corporation, ZIOPHARM's DNA synthetic biology platform employs an inducible gene-delivery system that enables controlled delivery of genes that produce therapeutic proteins to treat cancer. This controlled delivery is achieved by producing IL-12 under the control of Intrexon's proprietary biological "switch" (the RheoSwitch Therapeutic System® or RTS® platform) to turn on/off the therapeutic protein expression at the tumor site.

Palifosfamide (ZIO-201) is a potent, bi-functional DNA alkylating agent that has activity in multiple tumors by evading typical resistance pathways. Palifosfamide is in the same class as bendamustine, cyclophosphamide, and ifosfamide. It is currently being studied in an adaptive Phase 3 study in small cell lung cancer. Enrollment in this study was suspended with 188 subjects randomized, and being followed for overall survival. Data is expected in the first half of 2014.

Indibulin (ZIO-301) is a novel, tubulin binding agent that is expected to have several potential benefits, including oral dosing,

application in multi-drug resistant tumors, no neuropathy and a tolerable toxicity profile. It is currently being studied in a Phase 1/2 trial in metastatic breast cancer.

Darinaparsin (ZIO-101) is a novel mitochondrial-and hedgehog-targeted agent (organic arsenic) currently in ongoing studies with Solasia Pharma K.K.

ZIOPHARM's operations are located in Boston, MA. Further information about ZIOPHARM may be found at www.ziopharm.com.

Forward-Looking Safe Harbor Statement:

This press release contains certain forward-looking information about ZIOPHARM Oncology, Inc. that is intended to be covered by the safe harbor for "forward-looking statements" provided by the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. Words such as "expect(s)," "feel(s)," "believe(s)," "will," "may," "anticipate(s)" and similar expressions are intended to identify forward-looking statements. These statements include, but are not limited to, statements regarding our ability to successfully develop and commercialize our therapeutic products; our ability to expand our long-term business opportunities; financial projections and estimates and their underlying assumptions; and future performance. All of such statements are subject to certain risks and uncertainties, many of which are difficult to predict and generally beyond the control of the Company, that could cause actual results to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include, but are not limited to: whether palifosfamide, Ad-RTS-IL-12, darinaparsin, indibulin, or any of our other therapeutic products will advance further in the clinical trials process and whether and when, if at all, they will receive final approval from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies and for which indications; whether palifosfamide, Ad-RTS-IL-12, darinaparsin, indibulin, and our other therapeutic products will be successfully marketed if approved; whether any of our other therapeutic product discovery and development efforts will be successful; our ability to achieve the results contemplated by our collaboration agreements; the strength and enforceability of our intellectual property rights; competition from other pharmaceutical and biotechnology companies; the development of, and our ability to take advantage of, the market for our therapeutic products; our ability to raise additional capital to fund our operations on terms acceptable to us; general economic conditions; and the other risk factors contained in our periodic and interim SEC reports filed from time to time with the Securities and Exchange Commission, including but not limited to, our Annual Report on Form 10-K for the fiscal year ended December 31, 2012, and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013. Readers are cautioned not to place undue reliance on these forward-looking statements that speak only as of the date hereof, and we do not undertake any obligation to revise and disseminate forward-looking statements to reflect events or circumstances after the date hereof, or to reflect the occurrence of or non-occurrence of any events.

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