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ZIOPHARM Reports Positive Interim Results in Patients With Advanced Melanoma From Ongoing Phase 1/2 Study of Ad-RTS-IL-12

Potent biological activity observed in injected and non-injected lesions

Findings presented at Melanoma Bridge 2013 Conference

Conference Call Scheduled for 8:00 am EST on Monday, December 9

BOSTON, Dec. 7, 2013 (GLOBE NEWSWIRE) -- ZIOPHARM Oncology, Inc. (Nasdaq:ZIOP) today announced positive interim results from its ongoing Phase 1/2 study of Ad-RTS-IL-12, a novel DNA-based therapeutic candidate that is being evaluated with the oral activator, veledimex, in patients with advanced melanoma. The results from this multicenter study were presented at Melanoma Bridge 2013 Conference at the session "Best Abstracts on News in Immunotherapy", an international conference co-sponsored by Istituto Nazionale Tumori Fondazione, Sidra Medical and Research Center, and the Society for ImmunoTherapy of Cancer that is being held in Naples, Italy.

In this study, 21 patients with unresectable, recurrent stage III/IV melanoma have been treated with intratumoral injections of Ad-RTS-IL-12 and the oral activator veledimex. The purpose of the study is to evaluate the safety and tolerability of the Ad-RTS-IL-12 and veledimex therapy, determine tumor and immune response, and select the optimal dose and schedule of veledimex for future study. To date, expression of IL-12 mRNA in study subjects' tumors was determined to be very high and tightly controlled by veledimex dose with expression ranging from a median increase of approximately 1,000 times with an oral dose of 100 mg to approximately 100,000 times with an oral dose of 160 mg. In addition, upon stopping veledimex dosing, expression of the IL-12 mRNA returned to baseline levels, demonstrating the "on" and "off" control of Intrexon Corporation's (NYSE:XON) RheoSwitch Therapeutic System® platform. In this dose range, results to date demonstrate that Ad-RTS-IL-12 + veledimex has potent biologic activity, as measured by on-mechanism and on-target toxicity and response in injected and non-injected lesions. In addition, increased tumor infiltrating lymphocytes were observed in the tumor microenvironment at these doses, suggesting multiple favorable biologic effects of IL-12 expression. Following treatment, 11 of 16 evaluable patients have demonstrated a response of stable disease or better on a per lesion basis.

The most common severe adverse events (SAEs) were pyrexia, hypotension, mental status changes, and cytokine release syndrome. Four of seven patients with SAEs had veledimex dosing stopped during cycle 1. Three had SAEs during subsequent cycles, and stopped veledimex dosing at that time. Importantly, all SAEs were reversed after veledimex dosing was stopped, demonstrating the "on" and "off" control of veledimex on gene expression.

"Immunotherapy is a powerful and promising approach to the treatment of many cancers, including advanced melanoma," said Dr. John Nemunaitis, MD, Executive Medical Director, Mary Crowley Medical Research Center, and lead investigator of the study. "A major limitation to turning on the immune system in cancer is the many checks and balances that mask the tumor from the immune system. Delivery of interleukin-12 using the regulated gene expression system Ad-RTS-IL-12 provides us with the ability to turn on the immune system and finely control this potent anti-cancer immune response to derive clinical effect and manage tolerability. To date, this study has demonstrated immune response and encouraging activity, along with a tolerability profile that is well controlled by dose of the activator ligand veledimex. This should combine well with other emerging treatments to create a new treatment option for advanced melanoma and other cancers."

"The ability to precisely modulate gene expression at the site of the cancer represents a potential paradigm shift in cancer treatment," said Francois Lebel, M.D., Senior Vice President, Clinical Development and Medical Operations at ZIOPHARM. "We believe the evidence of safety, efficacy and control of gene expression in this study is encouraging and warrants the completion of this trial and the pursuit of a larger Phase 2 clinical trial in patients with advanced melanoma in 2014. We look forward to reporting the full results of this study at a future medical meeting and to initiating later-stage clinical testing in a larger patient population."

ZIOPHARM is developing Ad-RTS-IL-12 using Intrexon Corporation's RheoSwitch Therapeutic System® platform to control the expression of interleukin-12 and enable its safe and effective delivery as an anti-tumor agent. The company is advancing the Ad-RTS-IL-12 platform in melanoma, breast cancer and glioblastoma.

Conference Call Information:

ZIOPHARM management, joined by Larry Norton, M.D., Deputy Physician-in-Chief for Breast Cancer Programs, Memorial Sloan-Kettering Cancer Center, Medical Director, Breast Cancer Programs, will host a conference call and live audio webcast on Monday, December 9, 2013, at 8:00 AM ET. The call can be accessed by dialing (877) 402-8188 (U.S. and Canada) or (330) 871-4581 (international). The passcode for the conference call is 'ZIOPHARM.' To access the live audio webcast, or the subsequent archived recording, visit the "Investors - Events & Presentations" section of the ZIOPHARM website at www.ziopharm.com. The webcast will be recorded and available for replay on the Company's website for two (2) weeks.

About ZIOPHARM Oncology, Inc.:

ZIOPHARM Oncology is a Boston, Massachusetts-based biotechnology company employing novel gene expression and control technology to deliver DNA for the treatment of cancer. ZIOPHARM's technology employs Intrexon Corporation's RheoSwitch Therapeutic System® platform to turn on and off, and precisely modulate, gene expression at the cancer site in order to improve the therapeutic index. This technology is currently being evaluated in Phase 2 clinical studies of the immune system cytokine interleukin-12 for the treatment of breast cancer and advanced melanoma. Multiple new Investigational New Drug applications for new targets using synthetic biology technology with monogenic and multigenic approaches are expected in 2014 and 2015. ZIOPHARM is also developing novel small molecules as potential cancer therapeutics.

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 Ad-RTS-IL-12, DC-RTS-IL-12, palifosfamide, 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 Ad-RTS-IL-12, DC-RTS-IL-12, palifosfamide, 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 September 30, 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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