



May 17, 2017

ZIOPHARM Oncology Announces Results of Ad-RTS-hIL-12 + Veledimex in Recurrent Glioblastoma to be Presented at the 2017 ASCO Annual Meeting

— Updated Survival Through Late May to be Presented on June 5, 2017 —

BOSTON, May 17, 2017 (GLOBE NEWSWIRE) -- [Ziopharm Oncology, Inc.](#) (Nasdaq:ZIO), a biopharmaceutical company focused on new immunotherapies, today announced that it will present updated results from its Phase 1, multicenter, dose-escalation study and 20-mg expansion cohort of Ad-RTS-hIL-12 + orally administered veledimex in patients with recurrent or progressive glioblastoma (GBM) at the 2017 American Society of Clinical Oncology (ASCO) Annual Meeting to be held June 2-6 in Chicago. Ad-RTS-hIL-12 + veledimex is a novel, viral gene therapy candidate for the controlled expression of interleukin-12 (IL-12), a pro-inflammatory cytokine critical for stimulating anti-cancer immune responses.

As noted in the abstract released today, subjects with recurrent or progressive grade III or IV glioma undergoing resection were intratumorally injected with 2×10^{11} adenovirus (Ad) particles and received veledimex for 15 daily doses, beginning just prior to surgery. The primary endpoint of the study was safety and tolerability of Ad-RTS-hIL-12 + veledimex, with secondary endpoints including overall survival. Updated results will be presented at the meeting on June 5.

Glioblastoma is an aggressive brain tumor affecting approximately 74,000 people worldwide annually.^{i,ii} Patients with recurrent GBM typically have a median overall survival (mOS) of 6-7 months, and overall survival in patients who have failed temozolomide, bevacizumab or equivalent salvage chemotherapy, is approximately 3-5 months.^{iii,iv}

"Recurrent glioblastoma is a rapidly progressing disease with no standard of care and extremely poor prognoses, and thus there is an urgent need for new treatment options," said Francois Lebel, M.D., Executive Vice President, Research and Development, Chief Medical Officer at ZIOPHARM. "We look forward to updating results from this study at ASCO and to soon advancing Ad-RTS-hIL-12 + veledimex into additional treatment settings, including a stereotactic arm, a pediatric trial in diffuse intrinsic pontine glioma patients and a combination study with an immune checkpoint inhibitor. We also continue to assess several paths for the initiation of a pivotal trial and look forward to providing updates on this strategy following completion of discussions with regulators and clinical advisors."

Details for the poster presentation at ASCO 2017:

Title: Expanded phase I study of intratumoral Ad-RTS-hIL-12 plus oral veledimex: Tolerability and survival in recurrent glioblastoma

Abstract Number: [2044](#)

Session: Central Nervous System Tumors

Date and Time: Monday, June 5, 2017, 1:15 — 4:45 p.m. CT

About ZIOPHARM Oncology, Inc.:

ZIOPHARM Oncology is a Boston, Massachusetts-based biotechnology company employing novel gene expression, control and cell technologies to deliver safe, effective and scalable cell- and viral-based therapies for the treatment of cancer and graft-versus-host-disease. The Company's immuno-oncology programs, in collaboration with Intrexon Corporation (NYSE:XON) and the MD Anderson Cancer Center, include chimeric antigen receptor T cell (CAR-T) and other adoptive cell-based approaches that use non-viral gene transfer methods for broad scalability. The Company is advancing programs in multiple stages of development together with Intrexon Corporation's RheoSwitch Therapeutic System[®] technology, a switch to turn on and off, and precisely modulate, gene expression in order to improve therapeutic index. The Company's pipeline includes a number of cell-based therapeutics in both clinical and preclinical testing which are focused on hematologic and solid tumor malignancies.

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, and in some cases can be identified by terms such as "may," "will," "could," "expects," "plans," "anticipates," and "believes." These statements include, but are not limited to, statements regarding the Company's plans and expectations regarding its securities offerings, fundraising activities and financial strategy, the progress, timing and results of preclinical and clinical trials involving the Company's drug candidates, and the progress of the Company's research and development programs. 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 by, the forward-looking statements. These risks and uncertainties include, but are not limited to: our ability to finance our operations and business initiatives and obtain funding for such activities, whether chimeric antigen receptor T cell (CAR-T) approaches, Ad-RTS-hIL-12, TCR and NK cell-based therapies, or any of our other therapeutic candidates will advance further in the preclinical or 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 chimeric antigen receptor T cell (CAR-T) approaches, Ad-RTS-hIL-12, TCR and NK cell-based therapies, and our other therapeutic products will be successfully marketed if approved; the strength and enforceability of our intellectual property rights; competition from other pharmaceutical and biotechnology companies; and the other risk factors contained in our periodic and interim 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, 2016 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2017. 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.

ⁱ Mrugala MM. Advances and challenges in the treatment of glioblastoma: a clinician's perspective. *Discov Med.* 2013;15:221-230. <http://www.discoverymedicine.com/Maciej-M-Mrugala/2013/04/25/advances-and-challenges-in-the-treatment-of-glioblastoma-a-clinicians-perspective/>. Accessed March 24, 2015.

ⁱⁱ McCubrey JA, LaHair MM, Franklin RA. OSU—0312 in the treatment of glioblastoma. *Mol Pharmacol.* 2006;70:437-439.

ⁱⁱⁱ Omuro, A. Glioblastoma and Other Malignant Gliomas. A Clinical Review *JAMA.* 2013 Nov 6;310(17):1842-50.

^{iv} Iwamoto et al. Patterns of relapse and prognosis after bevacizumab failure in recurrent glioblastoma. *Neurology* 2009; 73 (15):1200-1206

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