



July 15, 2016

## **ZIOPHARM Issues Statement Regarding Phase I Study of Gene Therapy Candidate Ad-RTS-hIL-12 in Brain Cancer**

BOSTON, July 15, 2016 (GLOBE NEWSWIRE) -- ZIOPHARM Oncology, Inc. (Nasdaq:ZIOP) today issued the following statement regarding the Company's ongoing multicenter Phase 1 study of Ad-RTS-hIL-12 + orally administered veledimex in recurrent or progressive glioblastoma (GBM) or grade III malignant glioma:

"This Phase I study is being conducted in late-stage, recurrent GBM, so these patients are all, unfortunately, medically fragile. The first two patient deaths, which occurred 6.7 months and 3.9 months after treatment, were unrelated to study drug. A third death has just been reported to us and we are collecting and analyzing information in order to properly and timely report it to the FDA. The cause of death is intracranial hemorrhage, which occurred some time after the patient had been discharged from the treating center. This is an isolated case, and there have been no reported related instances of brain hemorrhage in any previous cohort or prior studies with Ad-RTS-hIL-12 + veledimex. Enrollment remains open in the study, and we will be discussing with our Safety Review Committee the appropriate course of action. For patients who have experienced multiple recurrences, as these patients have, prognoses are particularly poor. Median follow up in the first dose cohort from our study is now 8 months, in a population with an expected overall survival of 3 to 5 months for patients that have failed temozolomide and bevacizumab, or equivalent salvage chemotherapy. For the patients that remain in follow up in this Phase I study, we believe that preliminary overall survival remains encouraging. The Company expects to provide an update once a course of action has been determined."

### **About Glioblastoma**

Glioblastoma is an aggressive primary brain tumor affecting approximately 74,000 people worldwide each year.<sup>i, ii</sup> Recurrent glioblastoma is an aggressive cancer with one of the lowest 3-year survival rates, at 3%, among all cancers.<sup>iii</sup> For patients who have experienced multiple recurrences the prognosis is particularly poor, with a median overall survival (OS) of 6-7 months, while OS in patients that have failed temozolomide and bevacizumab, or equivalent salvage chemotherapy, is approximately 3-5 months.<sup>iv, v</sup>

### **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. 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 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: 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 pre-clinical 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 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, 2015, and our Quarterly Report for the quarter ended March 31, 2016. 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.

**Trademarks:**

RheoSwitch Therapeutic System<sup>®</sup> (RTS<sup>®</sup>) technology is a registered trademark of Intrexon Corporation.

- 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. International Agency for Research on Cancer. *World Cancer Report*. 2003. <http://www.iarc.fr/en/publications/pdfs-online/wcr/2003/WorldCancerReport.pdf>.
- iv. Omuro, A. Glioblastoma and Other Malignant Gliomas. A Clinical Review *JAMA*. 2013 Nov 6;310(17):1842-50.
- v. Iwamoto et al. Patterns of relapse and prognosis after bevacizumab failure in recurrent glioblastoma. *Neurology* 2009; 73 (15):1200-1206

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