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Ziopharm Oncology Announces Updated Findings from Phase 1 Study of Ad-RTS-hIL-12 + Veledimex in Recurrent Glioblastoma Presented at American Academy of Neurological Surgery Annual Meeting

BOSTON, Sept. 18, 2017 (GLOBE NEWSWIRE) -- [Ziopharm Oncology, Inc.](http://www.ziopharm.com) (Nasdaq:ZiOP), a biopharmaceutical company focused on new immunotherapies, today announced that updated data from a subset of patients in its Phase 1 multicenter study of Ad-RTS-hIL-12 + veledimex in patients with recurrent or progressive glioblastoma (GBM) was presented at the 79th Annual Meeting of the American Academy of Neurological Surgery (AANS) in Santa Barbara, CA. Ad-RTS-hIL-12 + veledimex is Ziopharm's adenoviral (Ad) gene therapy candidate for the controlled expression of human interleukin-12 (hIL-12), a critical protein for stimulating an anti-cancer immune response.

"We are seeing additional evidence suggestive of inflammatory pseudo-progression rather than tumor progression in patients with recurrent glioblastoma who were biopsied after administration of Ad-RTS-hIL-12 + veledimex," said E. Antonio Chiocca, M.D., Ph.D., Harvey W. Cushing Professor of Neurosurgery, Department of Surgery, Harvard Medical School, Surgical Director, Center for Neuro-oncology, Dana-Farber Cancer Institute, Chairman, Neurosurgery, Brigham and Women's Hospital and Co-Director, Institute for the Neurosciences, Brigham and Women's Hospital. "These updated immunohistochemistry data on top of promising survival underscore the possible immunomodulatory benefits of localized and controlled IL-12 and support the advancement of this therapy candidate into a larger outcome study."

Dr. Chiocca's oral presentation, "A Phase I Clinical Trial of Regulated Interleukin-12 Immunogene Therapy for Recurrent Glioblastoma," included detail on three biopsied patients with evidence of documented pseudo-progression rather than tumor progression. Pseudo-progression may be seen in post-treatment imaging studies of cancers where the tumor appears larger compared to baseline, but these changes are due to infiltration of immune cells, as evidenced by subsequent biopsies. Some GBM lesions on repeated MRI scans show evidence of progression followed by regression which is consistent with immune-mediated anti-tumor effects.

Dr. Chiocca also provided an overview of trial data previously presented at the 2017 American Society of Clinical Oncology Annual Meeting from patients receiving intratumoral Ad-RTS-hIL-12 with 20 mg of orally-administered veledimex (n = 15) following craniotomy including:

- 1 A median overall survival (mOS) of 12.5 months for patients with recurrent glioblastoma (GBM) comparing favorably to historical controls;
- 1 The observed ratio of CD8⁺/FOXP3⁺ (effector/suppressor) T cells suggesting overall survival appears correlated with IL-12-mediated cellular immune activation; and
- 1 A superior survival rate in the 20mg cohort has been observed in those patients that received low dose steroids.

A copy of the slides from AANS will be available following presentation in the [Presentations and Publications](#) section of the Company's website, <http://www.ziopharm.com>.

About Ziopharm Oncology, Inc.

Ziopharm Oncology is a Boston, Massachusetts-based biotechnology company employing innovative 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[®] (RTS[®]) 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 June 30, 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.

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