October 16, 2017

ZIOPHARM Oncology Announces First Patient Dosed in New Phase 1 Study of Ad-RTS-hIL-12 plus Veledimex for the Treatment of Pediatric Brain Tumors

BOSTON, Oct. 16, 2017 (GLOBE NEWSWIRE) -- ZIOPHARM Oncology, Inc. (Nasdaq:ZIOP), a biopharmaceutical company developing new gene and cell-based immunotherapies for cancer, today announced that the first patient has been dosed in a new Phase 1 study of Ad-RTS-hIL-12 with veledimex for the treatment of pediatric brain tumors.

This open label study will assess the safety and tolerability of a single intratumoral injection of Ad-RTS-hIL-12, a gene therapy designed to control the expression of human interleukin 12 (hIL-12), a critical protein for stimulating a localized anti-cancer immune response. The study is conducted in two groups: the first is comprised of pediatric patients with recurrent or progressive brain tumors in the cortex, while the second is comprised of pediatric patients with diffuse intrinsic pontine glioma (DIPG).

"Studies in adults with recurrent glioblastoma have shown that Ad-RTS-hIL-12 with veledimex is not only well tolerated, but also have shown growing evidence that this treatment elicits a targeted immune response against brain tumor cells that gives rise to improvement in overall survival," said Francois Lebel, M.D., Executive Vice President, Research and Development, Chief Medical Officer at ZIOPHARM. "We look forward to advancing our studies in pediatric patients with brain tumors as these patients have limited-to-no therapeutic options."

This Phase 1 study is being conducted at leading pediatric cancer centers across the United States, including Ann & Robert H. Lurie Children's Hospital in Chicago, Dana-Farber Cancer Institute in Boston and the University of California, San Francisco. The first pediatric patient to receive Ad-RTS-hIL-12 plus veledimex is receiving care at Lurie Children's.

"Pediatric gliomas are a devastating diagnosis for children and families, and DIPG, specifically, while rare, is extremely aggressive and always a fatal disease with no viable treatment options," said Stewart Goldman, M.D., Division Head Hematology-Oncology, Neuro-Oncology & Stem Cell Transplantation at Lurie Children's. "We look forward to evaluating the potential of Ad-RTS-hIL-12 plus veledimex as a treatment option for children with brain tumors."

About Glioma

Glioblastoma (GBM) is a fast-growing, aggressive type of central nervous system tumor, with an estimated 12,390 new adult cases predicted in 2017 according to the American Brain Tumor Association. Recurrence rates for this type of cancer are near 90 percent, and prognosis for adult patients is poor with treatment often combining multiple approaches including surgery, radiation and chemotherapy [i]. In children, the incidence of brain cancer is approximately 4.84 per 100,000, according to the National Cancer Institute. Glioma in the cortex (cerebrum) of children is unusual and is treated along the same lines as in adults with occurrence common and survival poor. Glioma in the pontine region of the brain, or DIPG, accounts for approximately 15 percent of all cases of pediatric brain tumors, with a median survival time of less than one year[ii]. Because of where these tumors are situated, DIPG is inaccessible to surgery and there are no curative options.

About Ad-RTS-hIL-12 plus Veledimex

ZIOPHARM is advancing Ad-RTS-hIL-12 plus veledimex as a gene therapy for recurrent GBM (rGBM). Ad-RTS-hIL-12 is an adenoviral vector administered via a single injection into the tumor and engineered to express hIL-12, a powerful cytokine that has demonstrated the potential to stimulate a targeted, anti-tumor immune response. The expression of hIL-12 is controlled and modulated with the RheoSwitch Therapeutic System® (RTS®) by the small molecule veledimex, an activator ligand which has been shown to cross the blood brain barrier. The Company has recently reported that biopsies from three patients treated with Ad-RTS-hIL-12 plus veledimex provided evidence of documented pseudo-progression rather than tumor progression. Pseudo-progression may be seen in serial 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. ZIOPHARM's Phase 1 stereotactic study of Ad-RTS-hIL-12 with veledimex for the treatment of patients with brain tumors is underway. The Company also plans to initiate enrollment of adult patients with rGBM who will receive a single dose of Ad-RTS-hIL-12 plus veledimex in combination with a checkpoint inhibitor targeting programmed cell death protein 1 (PD-1) by the end of the year.

About ZIOPHARM Oncology, Inc.

ZIOPHARM Oncology is a Boston-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 progress and timing of the development 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: the Company's ability to finance its 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 other product candidates will advance further in the preclinical research or clinical trial 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 the Company's other therapeutic products it develops will be successfully marketed if approved; the strength and enforceability of the Company's intellectual property rights; competition from other pharmaceutical and biotechnology companies; as well as other risk factors contained in the Company's periodic and interim reports filed from time to time with the Securities and Exchange Commission, including but not limited to, the risks and uncertainties set forth in the "Risk Factors" section of the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2017 and subsequent reports that the Company may file with the Securities and Exchange Commission. Readers are cautioned not to place undue reliance on these forward-looking statements that speak only as of the date hereof, and the Company does 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/

[ii] Hargrave D, Bartels U, Bouffet E. Diffuse brainstem glioma in children: critical review of clinical trials. Lancet Oncol. 2006 Mar;7(3):241-8.

Trademarks

RheoSwitch Therapeutic System® and RTS® are registered trademarks of Intrexon Corporation.

Contact:
David Connolly
ZIOPHARM Oncology
617-502-1881
dconnolly@ziopharm.com

or
David Pitts
Argot Partners
212-600-1902
david@argotpartners.com