



## Ziopharm Oncology to Present Data from Clinical Studies of Controlled IL-12 in Breast Cancer and Glioblastoma at American Society of Clinical Oncology (ASCO) Annual Meeting

May 16, 2018

- Data from biopsies shows Ad-RTS-hIL-12 plus veledimex recruits killer T cells into breast and brain cancers
- IL-12 continues to demonstrate activity as a single-agent therapy, prolongs survival in patients with recurrent glioblastoma
- Results further support development of Ad-RTS-hIL-12 plus veledimex in combination with checkpoint inhibitors
- Preclinical data published in *Cancer Gene Therapy* further support Ad-RTS-hIL-12 plus veledimex potential to treat gliomas

BOSTON, May 16, 2018 (GLOBE NEWSWIRE) -- [Ziopharm Oncology](#), Inc. (Nasdaq:ZIOP), a biotechnology company focused on development of next generation immunotherapies utilizing gene- and cell-based therapies to treat patients with cancer, today announced that clinical data showing the Company's Controlled IL-12 platform elicits a sustained increase in killer T cells with a good safety profile in patients with breast cancer and patients with brain cancer has been accepted for presentation at the [2018 American Society of Clinical Oncology \(ASCO\) Annual Meeting](#) in Chicago.

These data were generated from tumor biopsies from two open-label trials that evaluated Ad-RTS-hIL-12 plus veledimex, a gene therapy designed to induce and control expression of the powerful cytokine interleukin 12 (IL-12), in heavily pretreated patients with metastatic breast cancer or recurrent glioblastoma (rGBM).

In addition, the poster presentation will include an update on patient survival data from the Company's Phase 1 study of Ad-RTS-hIL-12 plus veledimex as monotherapy to treat patients with rGBM. The Company previously reported median overall survival (mOS) of 12.5 months for patients treated with Ad-RTS-hIL-12 plus 20 mg of veledimex (n=15) at a mean follow-up time of 11.1 months as of Oct. 18, 2017. This mOS compares favorably to the 5 to 8 months survival established in historical controls for patients with rGBM.

"Intratumoral injection of Ad-RTS-hIL-12 plus oral doses of veledimex clearly demonstrate ability to turn cold tumors hot and bring to tumors cancer-fighting T cells that were not there before," said Francois Lebel, M.D., Ziopharm's Chief Medical Officer and Executive Vice President for Research & Development. "These data support further development of our Controlled IL-12 platform in combination with immune checkpoint inhibitors, and we have one combination trial initiated in brain cancer and plans to advance a second tumor type later this year."

**Poster Title:** Demonstration of Anti-Tumor Immunity via Intratumoral Regulated Platform Ad-RTS-hIL-12 in Advanced Breast Cancer and Recurrent Glioblastoma Patients (Abstract #3038)

**Presenter:** Francois Lebel, M.D.

**Date:** Monday, June 4, 8 to 11:30 a.m. CT

**Location:** Hall A

**Session Title:** Developmental Therapeutics-Immunotherapy

### Paper Published in *Cancer Gene Therapy*

The Company today also announced a publication that highlights encouraging data from preclinical studies of regulated interleukin-12 under the control of the RheoSwitch Therapeutic System<sup>®</sup> (RTS<sup>®</sup>) in a syngeneic orthotopic model of glioma. These results were published in the journal *Cancer Gene Therapy*. In the paper, "[Regulated intratumoral expression of IL-12 using a RheoSwitch Therapeutic System<sup>®</sup> \(RTS<sup>®</sup>\) gene switch as gene therapy for treatment of glioma](#)," lead author John Barrett, Ph.D., Vice President of Research & Development, Translational Medicine at Ziopharm, concludes that controlled local expression of mouse IL-12 increases tumor infiltration of cytotoxic T cells and reduces regulatory T cells which results in prolonged survival in mouse glioma models.

### About Controlled IL-12

Ad-RTS-hIL-12 plus veledimex is a novel gene therapy candidate designed to express human interleukin-12 (hIL-12) under the control of an orally administered activator ligand, veledimex through a proprietary RheoSwitch Therapeutic System<sup>®</sup> (RTS<sup>®</sup>) gene switch. IL-12 is a powerful cytokine that has demonstrated a targeted, anti-tumor immune response with the ability to activate and recruit killer T cells to the tumor site. An ongoing Phase 1 trial is evaluating Ad-RTS-hIL-12 plus veledimex as a monotherapy to treat patients with rGBM, and a separate trial has been initiated to evaluate a single dose of Ad-RTS-hIL-12 plus veledimex in combination with OPDIVO<sup>®</sup> (nivolumab), an immune checkpoint inhibitor targeting programmed death-1 (PD-1). The Company also is enrolling pediatric patients in its Phase 1 trial of Ad-RTS-hIL-12 with veledimex for the treatment of brain tumors at multiple U.S. sites. The Company also is exploring combination therapies with Controlled IL-12 and checkpoint inhibitors in additional tumor types.

### About Ziopharm Oncology, Inc.

Ziopharm Oncology is a Boston-based biotechnology company focused on the development of next-generation immunotherapies utilizing gene- and cell-based therapies to treat patients with cancer. In partnership with Precigen Inc., a wholly-owned subsidiary of Intrexon Corporation (NYSE:XON), Ziopharm is focused on the development of two platform technologies designed to deliver safe, effective and scalable cell- and viral-based therapies for the treatment of multiple cancer types: Controlled IL-12 and *Sleeping Beauty* for genetically modifying T cells. The Company's lead asset, Ad-RTS-hIL-12 plus veledimex, has demonstrated in clinical trials the potential to control interleukin-12, leading to an infiltration of T cells that fight brain cancer. The Company also is advancing therapies using *Sleeping Beauty*, a non-viral approach to genetically modify chimeric antigen receptor (CAR<sup>+</sup>) and T-cell receptor (TCR<sup>+</sup>) T cells, which target specific antigens in blood cancers and neoantigens in solid tumors. *Sleeping Beauty* is designed using the Company's point-of-care technology, a shortened manufacturing process which potentially can be developed as a decentralized manufacturing process based in hospitals. These programs are being advanced in collaboration with Precigen and with MD Anderson Cancer Center, the National Cancer Institute and Merck KGaA, Darmstadt, Germany.

### Forward-Looking Disclaimer

This press release contains certain forward-looking statements within the meaning of 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 business and strategic plans, the availability of cash resources, the Company's ability to establish a commercially-viable manufacturing approach as well as the progress and timing of the development of the Company's research and development programs, including its potential initiation of a first in-human trial using its P-O-C manufacturing process and the timing for the initiation of its clinical trials. All 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: changes in the Company's financial condition and cash needs, funding or other strategic opportunities that become available to the Company, 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 Annual Report on Form 10-K for the fiscal year ended December 31, 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.

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