Ziopharm Oncology Completes Enrollment of Controlled IL-12 Monotherapy Expansion Substudy in Phase 1 Brain Cancer Trial

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-- Substudy promptly accrued 36 patients in less than six months, with majority of newly enrolled patients receiving low-dose steroids --

BOSTON, Feb. 11, 2019 (GLOBE NEWSWIRE) -- Ziopharm Oncology, Inc. (Nasdaq:ZIOP) today announced that it rapidly completed enrollment and treated a total of 36 patients in less than six months in a substudy (Clinicaltrials.gov NCT03679754) to expand a Phase 1 trial evaluating its Controlled IL-12 platform as a monotherapy for the treatment of recurrent glioblastoma (rGBM). The trial was over enrolled with eleven more patients than the target goal of 25, which the Company attributes to enthusiasm stemming from the trial's encouraging survival and tumor biopsy data.

Ziopharm is developing its Controlled IL-12 platform, or Ad-RTS-hIL-12 plus veledimex, as a drug to control the production of interleukin 12 (IL-12). In the setting for the treatment of rGBM, previously reported Phase 1 data demonstrated an increased survival benefit when using 20mg of veledimex, which is extended further when patients also received low-dose steroids versus higher doses. A majority of the newly enrolled patients in the substudy were treated with low-dose steroids.

"Glioblastoma is a difficult-to-treat brain cancer, with very few therapeutic options at recurrence and there is a significant need to develop new treatment options," said Rimas Lukas, M.D., associate professor of Neurology at Feinberg School of Medicine, Northwestern University. "This investigational therapy designed to stimulate and control expression of interleukin 12, a very powerful immuno-stimulant, has shown promise in treating brain cancer, especially when minimizing the use of immune-suppressive steroids. This substudy accrued quite rapidly, and we look forward to the results from treating these additional patients with rGBM."

The Company is now evaluating data from 51 patients with rGBM treated with 20mg veledimex dose and assessing the impact of systemic dosing of steroids. The safety profile in the substudy is consistent with the main, Phase 1, dose-escalation study (Clinicaltrials.gov NCT02026271) in which patients received varied systemic dosing of steroids, with all adverse reactions being manageable and reversible. Ziopharm anticipates reporting on preliminary data from this substudy at medical meetings this year.

About Ad-RTS-hIL-12 plus veledimex

At the 2018 annual meeting of the Society for Neuro-Oncology, Ziopharm presented data from its Phase 1 dose-escalation trial showing that Controlled IL-12 (Ad-RTS-hIL-12 plus veledimex) had a positive survival benefit, with 15 patients who received 20mg veledimex reaching 12.7 months median overall survival (mOS) at a mean follow up of 13.1 months. A subset of these patients (n=6) who received low-dose steroids (20mg or less of dexamethasone cumulatively over 15 days while receiving veledimex) had mOS of 17.8 months compared to 6.4 months mOS for patients (n=9) who received more than 20mg of dexamethasone during the same period. The survival data from patients who received the preferred dosing regimen of Controlled IL-12 with 20mg veledimex and low-dose steroids compare favorably to a benchmark mOS of 5 to 8 months for patients with rGBM that continues to serve as historical control.

The Company has treated more than 100 patients, including more than 75 patients with rGBM, with Ad-RTS-hIL-12 plus veledimex and administered more than 1,300 doses of veledimex across three types of solid tumors, building a significant safety profile, mechanistic dataset and evidence of anti-tumor effect. Biopsy data demonstrated that Controlled IL-12 turns immunologically-cold tumors hot based on sustained infiltration of killer T cells which is likely responsible for the preliminary improved survival observed with use of Ad-RTS-hIL-12 plus veledimex as monotherapy in patients with rGBM. Biopsy data also revealed upregulation of immune checkpoints providing a compelling rationale for combining Controlled IL-12 with PD-1 inhibitors.

Learn more about Controlled IL-12 online at https://ziopharm.com/controlled-il-12/.

About Ad-RTS-hIL-12 plus veledimex in combination with PD-1 inhibitors

The Company also is advancing Controlled IL-12 as a combination therapy with PD-1 inhibitors. Enrollment in a substudy of the ongoing Phase 1 trial to evaluate Controlled IL-12 in combination with the PD1 inhibitor OPDIVO® (nivolumab) is on track to be completed in the second quarter of this year (Clinicaltrials.gov NCT03636477). The Company is expected to begin a Phase 2 trial to evaluate Ad-RTS-hIL-12 plus veledimex in combination with Regeneron Pharmaceuticals' PD-1 antibody Libtayo ® (cemiplimab-rwlc) in the second quarter of 2019.

About Ziopharm Oncology, Inc.

Ziopharm Oncology is an immuno-oncology company focused on developing end-to-end cost-effective solutions using its non-viral *Sleeping Beauty* platform for TCR and CAR T-cell therapies and immune-stimulating gene therapy with Controlled Interleukin 12 (IL-12). The *Sleeping Beauty* platform genetically modifies T cells with DNA plasmids, instead of virus, to express T-cell receptors (TCRs) to target specific antigens in solid tumors, and chimeric antigen receptors (CARs) to target CD19 in blood cancers with membrane-bound interleukin 15 (mbIL15) and a kill switch for under 2-day

manufacturing. The *Sleeping Beauty* platform is being advanced in collaboration with the National Cancer Institute, The University of Texas MD Anderson Cancer Center and Eden BioCell. The Company also is developing its Controlled IL-12 platform or Ad-RTS-hIL-12 plus veledimex as monotherapy and in combination with immune checkpoint inhibitors to treat brain cancer, including a collaboration with Regeneron Pharmaceuticals.

Forward-Looking Statements Disclaimer
This press release contains forward-looking statements as defined in 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 progress and timing of the development of Ziopharm's research and development programs, including the timing for the initiation and completion of its clinical trials. Although Ziopharm's management team believes that the expectations reflected in such forward-looking statements are reasonable, investors are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Ziopharm, that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include among other things, changes in our operating plans that may impact our cash expenditures, the uncertainties inherent in research and development, future clinical data and analysis, including whether any of Ziopharm's product candidates will advance further in the preclinical research or clinical trial process, including receiving clearance from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies to conduct clinical trials and whether and when, if at all, they will receive final approval from the U.S. FDA or equivalent foreign regulatory agencies and for which indication; the strength and enforceability of Ziopharm's intellectual property rights; competition from other pharmaceutical and biotechnology companies as well as risk factors discussed or identified in the public filings with the Securities and Exchange Commission made by Ziopharm, including those risks and uncertainties listed in Ziopharm's quarterly report on Form 10-Q for the quarter ended September 30, 2018 filed by Ziopharm with the Securities and Exchange Commission. We are providing this information as of the date of this press release, and Ziopharm does not undertake any obligation to update or revise the information contained in this press release whether as a result of new information, future events or any other reason.

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