Ziopharm Oncology Receives Positive Opinion for Orphan Drug Designation from the European Medicines Agency for Ad-RTS-hIL-12 plus Veledimex for the Treatment of Glioma

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BOSTON, Aug. 08, 2019 (GLOBE NEWSWIRE) -- Ziopharm Oncology, Inc. (“Ziopharm” or “the Company”) (Nasdaq: ZIOP), today announced the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) adopted a positive opinion recommending Ad-RTS-hIL-12 plus veledimex (Controlled IL-12) for designation as an orphan medicinal product for the treatment of glioma.

“Malignant glioma is an aggressive and life-threatening cancer with few treatment options,” said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. “We are pleased to receive a positive opinion from EMA COMP for orphan drug designation, as this represents another important milestone for our clinical program to treat recurrent glioblastoma multiforme.”

Orphan Medicinal Product designation by the European Commission provides certain regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union and has the potential to be of significant benefit. In addition to a 10-year period of marketing exclusivity in the EU after product approval, this designation could allow for several incentives, including protocol assistance, access to the centralized authorization procedure and reduced regulatory fees.

About Ad-RTS-hIL-12 plus veledimex (Controlled IL-12 platform)
Ziopharm’s Controlled IL-12 platform is an investigational gene therapy designed to induce and control the production of human interleukin 12 (hIL-12) a master-regulator of the immune system. The Company has treated more than 100 patients, including more than 75 patients with recurrent glioblastoma multiforme (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 effects.

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 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 6 to 9 months for patients with rGBM that serves as historical control.

The Company announced earlier this year the completion of the enrollment in an “Expansion Substudy” (Clinicaltrials.gov NCT03679754) that enlarged the phase 1 trial by an additional 36 patients with Ad-RTS-hIL-12 plus 20mg/day veledimex for up to 14 days. In that cohort, 75% of patients (27/36) received low-dose steroids.

FDA Fast Track and Orphan Designation
In April 2019, Ziopharm announced that the U.S. Food and Drug Administration (FDA) had granted Fast Track designation for the Company’s Controlled IL-12 program for the treatment of rGBM in adults. Ziopharm also previously received Orphan Drug designation from the FDA’s Office of Orphan Products for the treatment of patients with malignant glioma.

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

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 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 the Company’s very rapid T-cell manufacturing process. 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 in collaboration with Regeneron Pharmaceuticals.

Note Regarding Forward-Looking Statements
This news 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 potential clinical benefits of its Controlled IL-12 program in treating patients and the potential benefits of receiving Orphan Medicinal Product designation. 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, whether the European Commission will designate Controlled IL-12 as an Orphan Medicinal Product, 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 (FDA) or equivalent foreign regulatory agencies to conduct clinical trials and whether and when, if at all, they will receive final approval from the 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 most recent Quarterly Report on Form 10-Q 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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