Ziopharm Oncology Announces Recent Publications in Peer-Reviewed Scientific Journals

January 30, 2020

– Encouraging long-term outcome data after infusion of *Sleeping Beauty*-modified CAR-T published in *Blood* –

– TCRs targeting mutations in TP53 obtained from T cells in peripheral blood to overcome need for surgical resection published in *Clinical Cancer Research* –

BOSTON, Jan. 30, 2020 (GLOBE NEWSWIRE) -- Ziopharm Oncology, Inc. (Nasdaq:ZIOP), today announced two publications in peer-reviewed scientific journals supporting the Company's ongoing clinical programs by reinforcing two of the foundational technologies underlying the Company's approach to the genetic modification of T cells to target cancer. A paper in the journal *Blood* enhances the clinical data set surrounding the *Sleeping Beauty* platform by indicating encouraging long-term survival of patients that received CAR-T. The *Clinical Cancer Research* publication demonstrates that T-cell receptors (TCRs) targeting genetic changes within TP53 can be obtained from T cells in the peripheral blood, overcoming the need to harvest a patient's tumor. The Company plans to expand its existing library of TCRs as part of its commitment to advance clinical development for the treatment of patients whose solid tumors have driver mutations, including in TP53, using *Sleeping Beauty*-modified T cells.

"These articles authored by our partners at MD Anderson Cancer Center and the National Cancer Institute highlight two of the technologies we are developing," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. "The *Blood* publication describes the multi-year survival of patients with non-Hodgkin lymphoma after CD19-specific CAR-T and long-term persistence of the T cells manufactured with the *Sleeping Beauty* system. The *Clinical Cancer Research* publication describes how TCRs with specificity to mutations within TP53 present in tumor cells can be obtained from circulating T cells, which overcomes the need to obtain tumor-infiltrating lymphocytes through surgical resection. Taken together, we believe these reports reinforce the potential of our *Sleeping Beauty* system as an ideal solution for the non-viral gene transfer of T cells and facilitate the manufacture of TCR-T by obtaining neoantigen-specific TCRs from peripheral blood."

A letter published in *Blood,*[1] the journal of the American Society of Hematology, discussed long-term outcomes of seven patients with relapsed or refractory B-cell lymphoid malignancies, all of whom had received CD19-specific CAR-T cells infused two days following autologous hematopoietic stem-cell transplantation (NCT00968760). Four patients demonstrated sustained persistence of CAR-T (median time of persistence duration was 4.5 years, range 2-5 years). Five-year progression-free survival and overall survival were 71% and 86%, respectively.

Data published online in *Clinical Cancer Research*[2] showed that TP53 mutation-reactive T cells circulating in peripheral blood are a source of neoantigen-specific TCRs for adoptive cell therapy. Last year, Ziopharm announced an exclusive license with the National Cancer Institute to access a library of TCRs against cancer-specific neoantigens in hotspots targeting TP53, KRAS and EGFR, for use with the *Sleeping Beauty* platform. Ziopharm's TCR-T cell therapy program is led by Drew Deniger, Ph.D., who joined the company from NCI and is the last author on the article published in *Clinical Cancer Research.* Additional information regarding this publication, including commentary from Dr. Steven Rosenberg, chief of the Surgery Branch at NCI, is available on the website of the American Association for Cancer Research.[3]

1 https://ashpublications.org/blood/article-abstract/doi/10.1182/blood.2019002920/431274/Long-term-outcomes-of-Sleeping-Beauty-generated?redirectedFrom=fulltext

2 https://clincancerres.aacrjournals.org/content/clincanres/early/2020/01/22/1078-0432.CCR-19-1874.full.pdf

3 https://www.aacr.org/Newsroom/Pages/News-Release-Detail.aspx?ItemID=1382

Forward-Looking Statements

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 use of the *Sleeping Beauty* system for gene therapy and ability to genetically modify T cells to target cancer. 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. Food and Drug Administration 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.

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 T-cell receptor (TCR) and chimeric antigen receptor (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 TCRs to target neoantigens inside and outside hotspots for solid tumors and CAR to target CD19 for blood cancers using the Company's "Rapid Personalized Manufacture" to produce and release CAR-T as soon as the day after gene transfer. 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 is also 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.

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