Ziopharm Oncology Announces Initiation of Phase 1 Trial Evaluating Rapid Personalized Manufacturing CAR-T Technology in Patients with Relapsed CD19+ Leukemias and Lymphomas

July 9, 2020

- RPM leverages the Sleeping Beauty system to reduce cost and simplify production for CD19-specific CAR-T infusion the day after gene transfer -

BOSTON, July 09, 2020 (GLOBE NEWSWIRE) -- Ziopharm Oncology, Inc. ("Ziopharm" or "the Company") (Nasdaq:ZIOP), today announced the initiation of a phase 1 clinical trial to evaluate CD19-specific CAR-T, using its Rapid Personalized Manufacturing (RPM) technology, as an investigational treatment for patients with relapsed $CD19^+$ leukemias and lymphomas. The trial is now open for enrollment at The University of Texas MD Anderson Cancer Center.

In this trial, the Company utilizes its non-viral *Sleeping Beauty* genetic engineering technology to infuse CAR-T the day after electroporation. Ziopharm's RPM CD19-specific CAR-T therapy results from the stable, non-viral insertion of DNA into the genome of resting T cells to co-express the chimeric antigen receptor (CAR), membrane-bound IL-15 (mbIL15) and a safety switch.

"We are pleased to expand the scope of our clinical development with MD Anderson, as we seek to evaluate our RPM technology using CD19-specific CAR-T cells," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. "RPM is a promising manufacturing solution, as T cells from the bloodstream are genetically reprogramed with DNA plasmids from the *Sleeping Beauty* system and then simply administered the next day.

"Our CAR-T therapy can be administered at low cell doses, which may control cytokine release syndrome and is appealing for the treatment of patients including those with CD19-expressing malignancies that have relapsed after allogeneic bone marrow transplantation (BMT). There are limited effective treatment options for such patients as evidenced by the low rate of remission and poor long-term survival," Dr. Cooper added.

Up to 24 patients with advanced $CD19^+$ leukemias and lymphomas who have relapsed after allogeneic BMT will be enrolled in this investigator-initiated trial (NCT03579888). The primary endpoint of the study is to determine the safety and maximum tolerated dose of donor-derived genetically modified CD19-specific T cells manufactured using the RPM process. An additional study is planned through Ziopharm's joint venture with Eden BioCell to evaluate the RPM technology using patient-derived (autologous) CD19-specific CAR-T in Greater China.

Research reveals three-year survival for adults with $CD19^+$ acute lymphoblastic leukemia after allogeneic BMT ranges from 30% to 65%.[1] For patients with other $CD19^+$ cancers, allogeneic BMT can provide three-year survival rates between 30% to 75%.[1] Few patients experience a durable remission following allogeneic BMT, regardless of the treatment modality, with some having a median survival of only 2 to 3 months.[2]

About Ziopharm Oncology, Inc.
Ziopharm is developing non-viral and cytokine-driven cell and gene therapies that weaponize the body's immune system to treat the millions of people globally diagnosed with a solid tumor each year. With its multiplatform approach, Ziopharm is at the forefront of immuno-oncology with a goal to treat any type of solid tumor. Ziopharm's pipeline is built for commercially scalable, cost effective T-cell receptor T-cell therapies based on its non-viral *Sleeping Beauty* gene transfer platform, a precisely controlled IL-12 gene therapy, and rapidly manufactured *Sleeping Beauty*-enabled CD19-specific CAR-T program. The Company has clinical and strategic collaborations with the National Cancer Institute, The University of Texas MD Anderson Cancer Center and Regeneron Pharmaceuticals. For more information, please visit www.ziopharm.com.

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 progress, design and timing of the Company's research and development programs, the potential benefits of the Company's therapies, and the Company's expectations regarding the number of patients in 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 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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[1] D'Souza A, Fretham C. Current Uses and Outcomes of Hematopoietic Cell Transplantation (HCT): CIBMTR Summary Slides, 2018. Available at https://www.cibmtr.org

[2] Keil F, Prinz E, Kalhs P, *et al*. Treatment of leukemic relapse after allogeneic stem cell transplantation with cytotoreductive chemotherapy and/or immunotherapy or second transplants. Leukemia 2001; 15:355-361.

Source: ZIOPHARM Oncology Inc