# Ziopharm Oncology Announces Clearance of Taiwan's First IND of Non-viral CAR-T for the Treatment of Relapsed CD19+ Leukemias and Lymphomas

December 21, 2020

*– Advances Eden BioCell's clinical program to validate Rapid Personalized Manufacturing (RPM) –*

*– Clinical trial to study autologous CD19-specific CAR-T using RPM technology designed to reduce cost and simplify production for infusion the day after gene transfer –*

BOSTON, Dec. 21, 2020 (GLOBE NEWSWIRE) -- Ziopharm Oncology, Inc. ("Ziopharm" or "the Company") (Nasdaq: ZIOP), today announced that the Taiwan Food and Drug Administration has cleared an investigational new drug application (IND) from Eden BioCell, a joint venture between Ziopharm and cell therapy company TriArm Therapeutics, for its phase 1 clinical trial to evaluate patient-derived CD19-specific CAR-T, using Ziopharm's Rapid Personalized Manufacturing (RPM) technology. This is an investigational treatment for patients with relapsed $CD19^+$ leukemias and lymphomas and the first clinical study of autologous non-viral CD19-specific CAR-T in Taiwan.

This trial will utilize Ziopharm's non-viral *Sleeping Beauty* cell engineering technology to infuse autologous CAR-T the day after T cells have been genetically modified. 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. The trial is being conducted at National Taiwan University Hospital.

"This study is a testament to the relationship Ziopharm has quickly established with Eden BioCell and TriArm and the progress using patients' T cells under RPM to target malignancies," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. "The results will help us understand the benefit of engineering T cells with membrane bound IL-15 which could benefit not only CAR-T, but also the engineering of T cells to express T-cell receptors."

Jay Zhang, Co-Founder and Chief Executive Officer of TriArm, added, "We are very excited to receive clearance of our IND in Taiwan. The learnings from this study will build upon the encouraging early data we are seeing with patients treated with RPM CAR-T targeting CD19 malignancies under compassionate use. We believe our approach has the potential to transform CAR-T therapy by dramatically decreasing the amount of time needed for manufacturing engineered T cells, thereby increasing efficacy and decreasing cost."

"CAR-T therapy has proved an effective therapy for B-cell cancers," noted Dr. Shang-Ju Wu, Division of Hematology, Department of Internal Medicine, National Taiwan University Hospital and Principal Investigator for the study. "Further optimization by shortening the manufacturing time would be of great importance to make this therapy more available to patients. We are honored to be involved in the clinical development of this non-viral CAR-T therapy produced using RPM. We hope the data derived from this current trial will advance CAR-T therapy to benefit our patients."

Up to 24 patients with relapsed $CD19^+$ leukemias and lymphomas will be enrolled in this phase 1 trial, with the goal of infusing 16 subjects (Taiwan FDA #1096030182). The primary endpoint of the study is to evaluate the safety and tolerability of autologous CD19-specific T cells manufactured using the RPM process.

**About Eden BioCell**
In December 2018, Ziopharm and TriArm Therapeutics announced the launch of Eden BioCell to lead clinical development and commercialization of *Sleeping Beauty*-generated CAR-T therapies in Greater China. Ziopharm licensed the rights to *Sleeping Beauty*-generated CAR-T therapies targeting the CD19 antigen using Ziopharm's RPM technology in Greater China to Eden BioCell. TriArm has committed up to $35 million to this joint venture, and Eden BioCell is owned 50-50 by Ziopharm and TriArm.

**About TriArm Therapeutics**
TriArm Therapeutics is a cell therapy company formed by Panacea Venture with R&D operations in Germany, United States and Greater China region. The company is dedicated to the treatment of cancer and autoimmune diseases.

**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 cancer each year. With its multiplatform approach, Ziopharm is at the forefront of immuno-oncology. 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 potential benefits of the Company's CAR-T therapy and the Company's expectations regarding the number of patients expected in this phase 1 clinical trial. 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 Eden BioCell's operating plans that may impact its 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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