May 2, 2017

ZIOPHARM Oncology Announces FDA Acceptance of IND for CD33-Specific CAR-T Cell Therapy Targeting Relapsed/Refractory Acute Myeloid Leukemia

Phase 1 Study Expected to Initiate in Third Quarter 2017

BOSTON, May 02, 2017 (GLOBE NEWSWIRE) -- ZIOPHARM Oncology, Inc. (Nasdaq:ZIOP), a biopharmaceutical company focused on new immunotherapies, today announced that an investigator-initiated Investigational New Drug (IND) application to the U.S. Food and Drug Administration (FDA) for a Phase 1 trial infusing the Company's CD33-specific CAR$^+$ T therapy for relapsed or refractory acute myeloid leukemia (AML) is now active, with the first patient to be enrolled in the study expected to begin treatment in the third quarter of 2017. The CD33-specific CAR$^+$ T cells incorporate a kill switch designed to eliminate the modified T cells under potential adverse safety conditions.

"Relapsed AML is an aggressive disease with very poor outcomes," said William G. Wierda, M.D., Ph.D., Professor and Center Medical Director, Department of Leukemia, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center and principal investigator for the CD33 study. "*In vivo* preclinical animal studies have demonstrated that these CAR-T cells targeting CD33 exhibit specific killing of AML cells, eliminating disease burden, and significantly enhancing survival compared to controls, and I look forward to evaluating the safety and effectiveness of this gene therapy for patients with AML."

Francois Lebel, M.D., Executive Vice President, Research and Development, Chief Medical Officer at ZIOPHARM added, "CAR-T cells expressing CD33 have shown promise in preclinical studies, but to-date, there has been limited experience in humans, representing a significant white space for us in treating AML. We look forward to seeing the positive preclinical results with our CD33-specific CAR-T cells translate to the clinic for relapsed/refractory AML patients who have far too few treatment options. In parallel with this Phase 1 study, we have also begun preclinical studies to evaluate rapid non-viral manufacturing of CAR+ T CD33-specific therapy for treatment of AML under point-of-care."

AML is a rapidly progressing cancer of the blood and bone marrow characterized by uncontrolled proliferation of immature blast cells with multiple associated gene mutations. The American Cancer Society estimates that there were approximately 20,000 new cases of AML and over 10,000 patient deaths from AML in the United States in 2016. A majority of AML patients relapse or present with refractory disease and have overall poor prognosis.

This will be the second CAR target for genetically modified T cells to be studied at The University of Texas MD Anderson Cancer Center under the research and development agreement among ZIOPHARM, Intrexon Corporation (NYSE:XON), and MD Anderson.

About ZIOPHARM Oncology, Inc.
ZIOPHARM Oncology is a Boston, Massachusetts-based biotechnology company employing novel gene expression, control and cell technologies to deliver safe, effective and scalable cell- and viral-based therapies for the treatment of cancer and graft-versus-host-disease. The Company's immuno-oncology programs, in collaboration with Intrexon Corporation (NYSE:XON) and the MD Anderson Cancer Center, include chimeric antigen receptor T cell (CAR-T) and other adoptive cell-based approaches that use non-viral gene transfer methods for broad scalability. The Company is advancing programs in multiple stages of development together with Intrexon Corporation's RheoSwitch Therapeutic System® technology, a switch to turn on and off, and precisely modulate, gene expression in order to improve therapeutic index. The Company's pipeline includes a number of cell-based therapeutics in both clinical and preclinical testing which are focused on hematologic and solid tumor malignancies.

Forward-Looking Safe-Harbor Statement
This press release contains certain forward-looking information about ZIOPHARM Oncology, Inc. that is intended to be covered by the safe harbor for "forward-looking statements" provided by 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 plans and expectations regarding its securities offerings, fundraising activities and financial strategy, the progress, timing and results of preclinical and clinical trials involving the Company's drug candidates, and the progress of the Company's research and development programs. All of such

statements are subject to certain risks and uncertainties, many of which are difficult to predict and generally beyond the control of the Company, that could cause actual results to differ materially from those expressed in, or implied by, the forward-looking statements. These risks and uncertainties include, but are not limited to: our ability to finance our operations and business initiatives and obtain funding for such activities, whether chimeric antigen receptor T cell (CAR-T) approaches, Ad-RTS-hIL-12, TCR and NK cell-based therapies, or any of our other therapeutic candidates will advance further in the preclinical or clinical trials process 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 indications; whether chimeric antigen receptor T cell (CAR-T) approaches, Ad-RTS-hIL-12, TCR and NK cell-based therapies, and our other therapeutic products will be successfully marketed if approved; the strength and enforceability of our intellectual property rights; competition from other pharmaceutical and biotechnology companies; and the other risk factors contained in our periodic and interim reports filed from time to time with the Securities and Exchange Commission, including but not limited to, our Annual Report on Form 10-K for the fiscal year ended December 31, 2016 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2017. Readers are cautioned not to place undue reliance on these forward-looking statements that speak only as of the date hereof, and we do not undertake any obligation to revise and disseminate forward-looking statements to reflect events or circumstances after the date hereof, or to reflect the occurrence of or non-occurrence of any events.

Trademarks

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