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ZIOPHARM Announces Publication in Scientific Reports Describing Genetic Editing of Human Leukocyte Antigen in Cell Therapies to Broaden Their Human Application

Goal to Genetically Match Universal Biological Products Between One Donor and Multiple Recipients

BOSTON, Feb. 23, 2016 (GLOBE NEWSWIRE) -- ZIOPHARM Oncology, Inc. (Nasdaq:ZIOP), a biopharmaceutical company focused on new cancer immunotherapies, today announced the publication of a study in *Scientific Reports*, a journal of the Nature Publishing Group, describing the genetic editing of human leukocyte antigen (HLA) in hematopoietic stem cells as a means of broadening the human application of these and other cell therapies. The article, titled "Genetic editing of HLA expression in hematopoietic stem cells to broaden their human application", is available online first at http://www.nature.com/srep/.

Laurence Cooper, M.D., Ph.D., Chief Executive Officer of ZIOPHARM and senior author of the journal article, noted: "Genetic editing of HLA expression is a step towards generating universal biological products, where one donor's cells may become suitable for sustained engraftment in multiple unrelated recipients. Unlocking the method by which HLA repertoire can be modified is one key to achieving this goal and fully harnessing the potential for off-the-shelf (OTS) therapies in immuno-oncology applications. Together with our partners at Intrexon and MD Anderson, we are bringing to bear multiple technologies to advance the findings of these studies and achieve this objective, with the goal of deploying it across our T-cell and natural killer (NK)-cell therapy platforms."

Transplantation of allogeneic, or donor-derived, hematopoietic stem cells (HSCs) into recipients with hematologic disorders is used to restore bone marrow function, termed hematopoiesis. Finding a suitable donor can be challenging due to the need to match the constellation of HLA with the recipient. For the study, researchers at The University of Texas MD Anderson Cancer Center eliminated expression of one set of HLA molecules, termed HLA-A, on donor HSC using artificial zinc finger nucleases. Other HLA molecules, such as HLA B and C remained expressed to help prevent elimination by resident NK cells. Following genetic editing, the HSCs maintained their ability to engraft and reconstitute hematopoiesis. This paper reveals that genetically altered HSC harvested from a small pool of donors will then match with a large number of unrelated recipients, which has two implications. First, it broadens the number of recipients who might benefit from bone marrow transplantation, which has particular appeal for racial minorities underserved by the current genetic makeup of unrelated donors. Second, it paves the way for generating OTS cells that are HLA matched with multiple recipients, even though they were obtained from one donor.

ZIOPHARM is developing various cell-based immuno-oncology programs, including CAR-T, TCR and NK adoptive cell-based therapies, in collaboration with its partner 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. The Company's synthetic 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 more 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 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: whether chimeric antigen receptor T cell (CAR T) approaches, Ad-RTS-IL-12, TCR and NK cell-based therapies, or any of our other therapeutic candidates will advance further in the pre-clinical 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 CAR T approaches, Ad-RTS-IL-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 SEC 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, 2014, and our Quarterly Reports on Form 10Q for the quarters ended March 31, 2015, June 30, 2015 and September 30, 2015. 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

RheoSwitch Therapeutic System® (RTS®) technology is a registered trademark of Intrexon Corporation.

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