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ZIOPHARM Announces Presentation of Data From CD19-Specific CAR⁺ T-Cell Therapy Programs at ASH Annual Meeting

– Results demonstrate survival benefit in long-term follow-up infusing autologous genetically modified T cells after HSCT–

– Results demonstrate survival benefit in follow-up infusing donor-derived genetically modified T cells after allogeneic HSCT, including haploidentical HSCT –

– Next-generation Sleeping Beauty CD19-specific CAR⁺ T-cell trial initiated –

BOSTON, Dec. 7, 2015 (GLOBE NEWSWIRE) -- ZIOPHARM Oncology, Inc. (Nasdaq:[ZIO](#)), a biopharmaceutical company focused on new cancer immunotherapies, today announced that results from its CD19-specific CAR T-cell therapy programs were presented at the 57th American Society of Hematology (ASH) Annual Meeting in Orlando, Florida. The presentation (Abstract 862), titled "Pre-Emptive Donor Lymphocyte Infusion with CD19-Directed, CAR-Modified T Cells Infused after Allogeneic Hematopoietic Cell Transplantation for Patients with Advanced CD19+ Malignancies" was presented by lead author Partow Kebriaei, M.D., Associate Professor, Department of Stem Cell Transplantation and Cellular Therapy, Division of Cancer Medicine, The University of Texas MD Anderson Cancer Center, in an oral session, and is available at www.ziopharm.com. The investigational therapies infusing T cells genetically modified to express a CD19-specific chimeric antigen receptor (CAR), described in the presentation, were exclusively licensed to ZIOPHARM and its collaboration partner, Intrexon Corporation (NYSE:[KON](#)), through an agreement with MD Anderson.

"This study, which was initially designed to test the safety and tolerability of the *Sleeping Beauty* platform, a system for the non-viral genetic modification of cells, appears to show a survival benefit with a doubling of overall survival compared to historical controls," said Dr. Richard Champlin, Chair, Department of Stem Cell Transplantation and Cellular Therapy at MD Anderson. "This is the measure of clinical success for a post-transplant therapy, where the contribution of either treatment is difficult to discern in early follow-up. Also promising was the absence of GvHD among HLA-mismatched CAR⁺ T-cell recipients, even at large doses, which supports infusing third-party T cells."

In the two separate trials described in Dr. Kebriaei's presentation, patient-derived (autologous) or donor-derived (allogeneic) CAR-modified T cells targeting CD19 were administered to recipients with advanced CD19⁺ malignancies after hematopoietic stem-cell transplantation (HSCT). The studies employed the *Sleeping Beauty* system, a cost-effective non-viral two plasmid electroporation method to stably express CAR in T cells.

Seven patients with advanced non-Hodgkin lymphoma (NHL) were treated with autologous cells, all of whom remained alive and six of whom remained in complete remission at a median 25.5 months of follow-up translating to a three-year progression free survival (PFS) of 83% and a three-year overall survival (OS) of 100%.

Nineteen patients with advanced acute lymphoblastic leukemia and NHL were treated with allogeneic T cells. One-year PFS among these patients was 53% and one-year OS was 63%. Among eight patients who received donor-derived T cells after haploidentical HSCT, all remained alive and six remained in complete remission after a median of 5.2 months of follow up, translating to a one-year PFS of 75% and OS of 100%.

No infusion related or late toxicity was observed in any recipients. A mild elevation in cytokines was observed, without cytokine storm. Autologous and allogeneic cells survived an average of 201 and 51 days, respectively.

A graft-versus-host disease (GvHD) rate of 11% was observed among recipients receiving donor-derived CAR⁺ T cells which did not differ from controls. Because of this low rate of GvHD, a complication associated with allogeneic HSCT, administration of up to 10⁸/m² genetically modified haploidentical T cells was possible. This lays the groundwork for infusing HLA-mismatched CAR⁺ T cells as an off-the-shelf broadly-accessible therapeutic.

"*Sleeping Beauty*, which is among the fastest technologies to go from bench to bedside, was a critical innovation of this trial,"

said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of ZIOPHARM. "In addition to demonstrating safety and durable responses, it provides us a pathway for testing new CARs, co-expressing CAR with other molecules, and to do so at relatively low cost in the setting of both patient-derived and third-party derived products. We look forward to the new trial initiated at MD Anderson using *Sleeping Beauty*-modified T cells expressing a next-generation CD19-specific CAR in active CD19⁺ lymphoid malignancies."

Results from the haploidentical population were also presented separately in an oral presentation, titled "Donor-derived CD19-specific CAR⁺ T-cell therapy after haploidentical hematopoietic stem-cell transplantation," by Dr. Cooper, at the Haplo2015 Symposium, December 3, 2015, in Orlando, Florida. The presentation is available at www.ziopharm.com.

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:[KON](#)) 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 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 chimeric antigen receptor T cell (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.

Lori Ann Occhiogrosso
ZIOPHARM Oncology, Inc.
617-259-1987

David Pitts
Argot Partners
212-600-1902