



November 6, 2017

## ZiOPHARM Oncology Reports Third Quarter 2017 Financial Results and Provides Update on Recent Activities

- | ***Company to host conference call today at 4:30 p.m. ET***
- | ***Pivotal, randomized control trial in rGBM to initiate by end of 2017***
- | ***Combination trial with checkpoint inhibitor in rGBM to initiate by end of 2017***
- | ***Point-of-care trial with CAR<sup>+</sup> T cells co-expressing membrane-bound interleukin (mbIL15) to initiate in 2018***

BOSTON, Nov. 06, 2017 (GLOBE NEWSWIRE) -- [ZiOPHARM Oncology, Inc.](http://www.ziopharm.com) (Nasdaq:ZiOP), a biopharmaceutical company developing new gene and cell-based immunotherapies for cancer, today announced its financial results for the third quarter ended Sept. 30, 2017, and provided an update on plans to initiate a pivotal trial in brain cancer, progress with its chimeric antigen receptor (CAR) T cell Phase 1 trials and expectations for a Phase 1 trial in solid tumors to be conducted at the National Cancer Institute (NCI).

"At ZiOPHARM, we remain focused on fully realizing the potential of immunotherapy by focusing on cost, complexity, and control to overcome the barriers of price and manufacturing," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of ZiOPHARM Oncology.

Dr. Cooper continued, "With the Sleeping Beauty system, we are genetically modifying T cells with chimeric antigen receptors and T-cell receptors to target hematologic cancers and solid tumors with rapid and cost-efficient manufacturing in a matter of days, not weeks. Based on preclinical and clinical data we have generated over the last two years, which we are excited to share at ASH 2017, we will undertake clinical trials in the coming year manufacturing T-cell therapies in less than two days under point-of-care."

He added, "We also are excited about initiating pivotal and combination trials as we develop Ad-RTS-hIL-12 plus veledimex to treat brain cancer. Our demonstrated ability to safely control interleukin-12 to stimulate an immune anti-tumor response in the brain under control of the RheoSwitch<sup>®</sup> has shown a survival benefit compared to historical controls in patients who have failed multiple lines of therapy, all of which energizes us to bring this much needed therapeutic option to patients as soon as possible."

### Program Updates

#### *Ad-RTS-hIL-12 plus veledimex for gliomas*

ZiOPHARM is advancing Ad-RTS-hIL-12 plus veledimex as a gene therapy to treat patients with recurrent glioblastoma (rGBM). Ad-RTS-hIL-12 is an adenoviral vector administered via a single injection into the tumor and engineered to express human IL-12, a powerful cytokine that has demonstrated a targeted, anti-tumor immune response. The expression of hIL-12 is controlled and modulated with the RheoSwitch Therapeutic System<sup>®</sup> (RTS<sup>®</sup>) by the small molecule veledimex, an activator ligand which crosses the blood-brain barrier.

**Initiation of Pivotal Trial in rGBM to Begin by End of 2017** - ZiOPHARM will initiate a pivotal trial for Ad-RTS-hIL-12 plus veledimex for the treatment of rGBM by the end of 2017. Based on guidance from U.S. and European regulatory authorities relative to a registration trial, as well as feedback from key opinion leaders and other stakeholders, the Company has decided to move forward with a randomized control trial. The Company remains in active discussions with potential partners in further development of this asset.

**Initiation of Combination Trial with Checkpoint Inhibitor in rGBM to Begin by End of 2017** - ZiOPHARM will initiate a trial of adult patients with rGBM who will receive a single dose of Ad-RTS-hIL-12 plus veledimex in combination with a checkpoint inhibitor targeting programmed cell death protein 1 (PD-1) by the end of 2017.

**Announced Initiation of Phase 1 for Pediatric Tumors** — Subsequent to the initiation of a Phase 1 study to evaluate the stereotactic administration of Ad-RTS-hIL-12 plus veledimex in adult patients with rGBM during the second quarter, ZiOPHARM recently began a new Phase 1 study of Ad-RTS-hIL-12 with veledimex for the treatment of pediatric brain tumors and is recruiting patients.

**Announced Four Presentation Abstracts at the 2017 Annual Meeting of the Society for Neuro-Oncology** - The Company will provide an update to survival of patients and associated correlative studies from the Phase 1 trial during the 22nd Annual Meeting and Education Day of the Society for Neuro-Oncology (SNO). The meeting will be held Nov. 16 - 19 in San Francisco.

At the 2017 American Society of Clinical Oncology Annual Meeting in June, the Company reported encouraging results from its multi-center Phase 1 trial evaluating Ad-RTS-hIL-12 plus veledimex following craniotomy. The median overall survival (mOS) for patients receiving 20 mg of veledimex was 12.5 months, which compared favorably to historical controls. Additional data from this trial also shows three lines of evidence supporting potential efficacy:

- | Patients who received low-dose systemic corticosteroids for post-operative management have a much better survival rate than those who received higher doses of corticosteroids, as the latter presumably interferes with immune activation; and
- | mOS appears directly correlated to an increased ratio of CD8<sup>+</sup>/FOXP3<sup>+</sup> (effector/suppressor) T cells measured in peripheral blood which is consistent with IL-12-mediated cellular immune activation; and
- | Biopsies of three patients many weeks after completion of veledimex showed infiltration of CD8<sup>+</sup> T cells which is attributed to the effects of IL-12, as well as evidence of the imaging phenomenon known as pseudo-progression.

#### Advancing CAR T Therapies to Point-of-Care

ZIOPHARM is developing novel adoptive cell-based therapies, including chimeric antigen receptor (CAR) T-cell therapies, with a unique focus on developing scalable, efficient technologies to overcome the high costs that come with manufacturing using virus to genetically program T cells.

**Announced Abstracts Accepted for Presentation at the 2017 American Society of Hematology Annual Meeting** - In investigator-led studies conducted at MD Anderson Cancer Center, ZIOPHARM has used DNA plasmids from first- and second-generation Sleeping Beauty (SB) system to express CAR in clinical trials to render T cells specific for CD19. The first-generation trials yielded safety and efficacy data from treating patients with non-Hodgkin lymphoma (NHL) and acute lymphoblastic leukemia (ALL). The ongoing second-generation trial, which was designed to refine the CAR and manufacturing processes, infuses CD19-specific CAR<sup>+</sup> T cells in patients with advanced lymphoid malignancies after lymphodepleting chemotherapy. With this second-generation trial, the Company is testing the revised CAR design, shortening the manufacturing process to two weeks, and establishing continued safety of SB-modified T cells in patients.

These two trials support the Company's plans to undertake the very rapid production of T cells under technology referred to as "point-of-care" (P-O-C). The Company expects to stop enrollment in the second-generation study in 2018 when it initiates the third-generation study to leverage SB to manufacture CAR<sup>+</sup> T cells co-expressing a membrane-bound version of the cytokine interleukin (IL)-15, or mblL15, in less than two days.

Posters to be presented at the 2017 American Society of Hematology Annual Meeting (ASH) include:

- | Updates on patients enrolled in the first- and second-generation trials featuring response, survival data, and persistence of infused CAR<sup>+</sup> T cells;
- | Further preclinical *in vitro* and *in vivo* data for the P-O-C technology to generate clinical-grade CD19-specific T cells will be presented at ASH.
- | Update on the Company's ongoing Phase 1 study of CD33-specific CAR<sup>+</sup> T-cell therapy for treatment of relapsed or refractory acute myeloid leukemia, or AML.
- | Update on the genetic engineering of regulatory T cells for the treatment of graft-versus-host-disease.

#### T-Cell Receptors Targeting Neoantigens

The Company is developing genetically modified T-cell receptors (TCRs) using SB to treat solid tumor targets under a Cooperative Research and Development Agreement (CRADA), with the National Cancer Institute (NCI). Preparations are underway at the NCI for a clinical trial to evaluate adoptive cell transfer using the Company's non-viral system to express tumor-specific TCRs that recognize specific immunogenic mutations, or neoantigens.

**Anticipate Filing of IND under CRADA with NCI in First Quarter of 2018** - Research conducted under the CRADA is under the direction of Steven A. Rosenberg, M.D., Ph.D., Chief of the Surgery Branch at the NCI. The Company expects that once ongoing validation of the manufacturing process at NCI is complete, an Investigational New Drug application to open a Phase 1 trial will be filed with the U.S. Food and Drug Administration (FDA) in the first quarter of 2018.

## Corporate Update

Today, the Company also announced that effective as of November 6, 2017, Dr. David Mauney will serve as the Company's Chief Operating Officer on an interim basis, in addition to his current role as the Company's Executive Vice President and Chief Business Officer. Effective as of the same date, Caesar J. Belbel will no longer serve as the Company's Chief Operating Officer. He will continue to serve as the Company's Executive Vice President, Chief Legal Officer and Secretary.

## Third-Quarter 2017 Financial Results

- | Net loss applicable to the common shareholders for the third quarter of 2017 was \$17.6 million, or \$(0.13) per share, compared to a net loss of \$14.4 million, or \$(0.11) per share, for the third quarter of 2016. The increase is primarily due to an increase in operating expenses of \$2.2 million and an increase of \$1.3 million related to the value of preferred stock dividends.
- | Research and development expenses were \$11.1 million for the third quarter of 2017, compared to \$9.0 million for the third quarter of 2016. The increase in research and development expenses for the three months ended September 30, 2017 is primarily due to expanded development in our gene and cell therapy programs.
- | General and administrative expenses were \$3.6 million for the third quarter of 2017, compared to \$3.5 million for the third quarter of 2016.
- | The Company ended the quarter with unrestricted cash resources of approximately \$84.4 million.
- | As part of our strategic co-development activities at MD Anderson Cancer Center, a prepayment of approximately \$29.4 million remains for programs to be conducted by the Company at MD Anderson Cancer Center under the current Research and Development Agreement.
- | The Company believes its current resources will be sufficient to fund its currently planned operations into the fourth quarter of 2018.

## Conference Call and Slide Webcast

ZIOPHARM will host a conference call and webcast slide presentation today, Nov. 6, at 4:30 p.m. ET. The call can be accessed by dialing 1-844-309-0618 (U.S. and Canada) or 1-661-378-9465 (international). The passcode for the conference call is 8769629. To access the slides and live audio webcast, or the subsequent archived recording, visit the "Investors & Media" section of the ZIOPHARM website at [www.ziopharm.com](http://www.ziopharm.com). The webcast will be recorded and available for replay on the Company's website for two weeks.

## About ZIOPHARM Oncology, Inc.

ZIOPHARM Oncology is a Boston, Massachusetts-based biotechnology company employing innovative 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<sup>®</sup> (RTS<sup>®</sup>) 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 September 30, 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

RheoSwitch Therapeutic System<sup>®</sup> and RTS<sup>®</sup> are registered trademarks of Intrexon Corporation.

(Tables follow)

**ZIOPHARM Oncology, Inc.**  
**Statements of Operations**  
(in thousands except share and per share data)  
(unaudited)

	<b>Three Months Ended September 30,</b>	
	<b>2017</b>	<b>2016</b>
Collaboration revenue	\$ 1,598	\$ 1,598
Operating expenses:		
Research and development, including cost of contracts	11,105	8,975
General and administrative	3,571	3,537
Total operating expenses	14,676	12,512
Loss from operations	(13,078)	(10,914)
Other income (expense), net	175	39
Change in derivative liabilities	202	21
Net loss	(12,701)	(10,854)
Preferred stock dividends	(4,903)	(3,591)
Net loss applicable to common stockholders	\$ (17,604)	\$ (14,445)
Basic and diluted net loss per share	\$ (0.13)	\$ (0.11)
Weighted average common shares outstanding used to compute basic and diluted net loss per share	140,632,297	130,496,035

**ZIOPHARM Oncology, Inc.**  
**Balance Sheet Data**  
(in thousands)  
(unaudited)

<b>September 30, 2017</b>	<b>December 31, 2016</b>
-------------------------------	------------------------------

Cash and cash equivalents	84,406	81,053
Working capital	96,699	89,075
Total assets	116,559	106,348
Total stockholders' (deficit)	(79,795)	(77,298)

Contacts:

David Connolly  
ZIOPHARM Oncology  
617-502-1881  
[dconnolly@ziopharm.com](mailto:dconnolly@ziopharm.com)

David Pitts  
Argot Partners  
212-600-1902  
[david@argotpartners.com](mailto:david@argotpartners.com)