



March 1, 2018

Ziopharm Oncology Reports Fourth Quarter and Full Year 2017 Financial Results and Provides Corporate Update

Company to host conference call today at 4:30 p.m. ET

BOSTON, March 01, 2018 (GLOBE NEWSWIRE) -- [Ziopharm Oncology](#), Inc. (Nasdaq:ZIOP), a biotechnology company focused on development of next generation immunotherapies utilizing gene- and cell-based therapies to treat patients with cancer, today announced its financial results for the fourth quarter and year ended December 31, 2017, and provided an update on the Company's recent activities.

"This is an exciting year for Ziopharm and the clinical development of our two platform technologies, Controlled IL-12 and *Sleeping Beauty*," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. "With *Sleeping Beauty*, we achieved major milestones in 2017 and are looking forward to what is ahead in 2018. We plan to move our point-of-care technology into the clinic and, for the first time ever, we intend to infuse patients with T cells genetically modified and manufactured without virus using the *Sleeping Beauty* platform within two days from harvesting the T cells from patients. Also, *Sleeping-Beauty*-modified T-cell receptors targeting neoantigens — the very mutations that cause solid tumors — will enter the clinic with Dr. Steven Rosenberg's team at the National Cancer Institute."

Dr. Cooper continued, "With Controlled IL-12, in addition to investigating this cytokine as a monotherapy, we look forward to seeing data from our first combination of IL-12 and an immune checkpoint inhibitor, and exploring its impact on the care options for the treatment of patients with brain cancer. We believe Controlled IL-12 is a powerful platform that can turn cold tumors hot and has the potential for broad applicability across oncology."

Program Updates

Controlled IL-12

Ziopharm is advancing Ad-RTS-hIL-12 plus vedolimex as a gene therapy product candidate 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 conditionally 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[®] (RTS[®]) by the small molecule vedolimex, an activator ligand which crosses the blood-brain barrier.

Combination Trial with Checkpoint Inhibitor in rGBM Initiated. As announced earlier this year in January, Ziopharm has initiated a trial of adult patients with rGBM to evaluate a single dose of Ad-RTS-hIL-12 plus vedolimex in combination with OPDIVO[®] (nivolumab), an immune checkpoint inhibitor targeting programmed death-1 (PD-1).

Plans to Initiate Pivotal Trial in rGBM in Second Half of 2018. Ziopharm plans to initiate a pivotal trial for Ad-RTS-hIL-12 plus vedolimex for the treatment of patients with rGBM in the second half of 2018. As previously disclosed, Ziopharm is currently in the process of completing Chemistry Manufacturing and Control technical requirements for a planned Phase 3 clinical trial, subject to regulatory approval.

Phase 1 Trial for Pediatric Brain Tumors Ongoing. Ziopharm is enrolling pediatric patients in its Phase 1 trial of Ad-RTS-hIL-12 with vedolimex for the treatment of brain tumors.

Fourth Quarter Highlights

In November 2017, Ziopharm presented updated data from its Phase 1 trial of Ad-RTS-hIL-12 plus vedolimex to treat patients with rGBM that supports a survival benefit and the underlying immune system mechanism at the 22nd Annual Meeting and Education Day of the Society for Neuro-Oncology.

The data showed median overall survival (mOS) of 12.5 months sustained for patients treated with Ad-RTS-hIL-12 plus 20 mg of vedolimex (n=15) at a mean follow-up time of 11.1 months as of October 18, 2017. This mOS compares favorably to

the 5 to 8 months survival established in historical controls for patients with rGBM. Additional highlights observed included:

- | An anti-tumor effect evident with centralized review of magnetic resonance imaging showing decreasing size of brain tumor lesions in patients;
- | Immunohistochemistry analyses from three of three patient biopsies greater than four months after completion of veledimex demonstrated that IL-12 results in an extensive infiltration of CD8⁺ T cells within the tumor;
- | These same biopsies showed sustained production of interferon-gamma, a cytokine crucial to arming an immune response in the tumor microenvironment;
- | Interferon-gamma was undetectable in the peripheral blood at the time of biopsies providing further evidence of an on-target T-cell response;
- | These same biopsies demonstrated evidence of an anti-tumor response;
- | These same biopsies showed upregulation of both PD-1 and PD-L1, which suggests added potential efficacy for combining Ad-RTS-hIL-12 plus veledimex with an immune checkpoint inhibitor;
- | Ratio of circulating killer CD8⁺ T cells to suppressor FOXP3⁺ T cells correlates with overall survival;
- | Patients in the trial who received low-dose systemic corticosteroids for peri-operative management have a much better survival rate than those who received higher doses of corticosteroids, as the latter presumably interferes with immune activation;
- | Ad-RTS-hIL-12 plus veledimex continues to be well tolerated, as adverse events (AE) in the trial were predictable and reversible, neurologic AEs were relatively mild and transient, and there were no drug-related deaths.

Adoptive Cell Therapies

Using Ziopharm's non-viral approach leveraging *Sleeping Beauty* to genetically modify cells, the Company is developing chimeric antigen receptor (CAR) T-cell (CAR⁺ T) and T-cell receptor (TCR) T-cell (TCR⁺ T) therapies. These programs are being advanced in collaboration with Precigen Inc., a wholly-owned subsidiary of Intrexon Corporation, and with MD Anderson Cancer Center, the National Cancer Institute and Merck KGaA, Darmstadt, Germany. This non-viral approach to genetically modifying T cells has the potential to reduce the costs of and expand access to this immunotherapy based on very rapid production and thus avoiding the need for centralized manufacturing.

Initiation of First Point-of-Care Clinical Trial Expected in 2018. Ziopharm is advancing the *Sleeping Beauty* platform towards point-of-care manufacturing for the very rapid manufacturing of genetically modified CAR⁺ T cells, with Ziopharm's first clinical trial utilizing this approach expected to begin in the second half of 2018. Ziopharm's third-generation point-of-care trial intends to use the *Sleeping Beauty* platform to manufacture CAR⁺ T cells co-expressing membrane-bound interleukin-15, or mbIL15, within two days after harvesting T cells from the patient.

Phase 1 Trial of *Sleeping Beauty*-Modified TCRs to Treat Solid Tumors to Initiate in Second Half of 2018. The NCI anticipates initiation of a Phase 1 trial in the second half of 2018 to evaluate adoptive cell transfer (ACT)-based immunotherapies genetically modified using the *Sleeping Beauty* transposon/transposase system to express TCRs for the treatment of solid tumors. Ziopharm, Intrexon, and the NCI last year entered into a Cooperative Research and Development Agreement to develop and evaluate ACT for patients with advanced cancers using autologous peripheral blood lymphocytes genetically modified using the *Sleeping Beauty* system to express TCRs that recognize specific immunogenic mutations, or neoantigens, expressed within a patient's cancer.

Phase 1 Trial of CD33-specific CAR⁺ T Therapy for Acute Myeloid Leukemia (AML). Enrollment is underway at MD Anderson Cancer Center in the Phase 1 adoptive cellular therapy clinical trial of CAR⁺ T-cell therapy in patients with refractory/recurrent AML that express CD33. This trial infuses autologous T cells genetically modified with lentivirus to express a CD33-specific CAR and a cetuximab-activated kill switch for elimination of genetically modified cells. Data from this trial are expected to serve as the basis for evaluating CD33 as a potential target for further development using non-viral manufacturing of T cells with Ziopharm's point-of-care technology.

Fourth Quarter Update

Data supporting the third-generation point-of-care technology were presented at the 59th American Society of Hematology (ASH) Annual Meeting in December 2017, where first- and second-generation *Sleeping Beauty* clinical trial data demonstrated tolerability, disease response including long-term survival, and sustained persistence of infused CD19-specific CAR⁺ T cells. Ziopharm also presented at ASH preclinical data demonstrating that *Sleeping Beauty* can manufacture CAR⁺ T cells co-expressing mbIL15 in less than two days. In addition, preclinical data were also presented last month at the 2018 [Keystone Symposia Emerging Cellular Therapies: T Cells and Beyond](#). These data further demonstrated that T cells expressing CD19-specific CAR with mbIL15 could be generated with the *Sleeping Beauty* system in less than two days and did not require *ex vivo* activation or propagation. Ziopharm observed in these trials that T cells designed to express mbIL15 showed greater persistence and more potent antitumor activity than comparator T cells without mbIL15.

Graft-versus-Host Disease (GvHD) Update

Following an in-depth review of Ziopharm's research and development portfolio, management made the strategic decision to focus resources on developing Controlled IL-12 and *Sleeping Beauty* platforms for oncology indications and to stop development of engineered cell therapies for the treatment of GvHD. Ziopharm reverted its rights to the GvHD program to Precigen and is winding down its final clinical activities.

Fourth Quarter 2017 Financial Results

- | Net loss applicable to the common stockholders for the fourth quarter of 2017 was \$18.3 million, or \$(0.13) per share, compared to a net loss of \$14.8 million, or \$(0.11) per share, for the fourth quarter of 2016. The increase is primarily due to an increase in operating expenses of \$2.3 million and an increase of \$1.5 million related to the value of preferred stock dividends.
- | Research and development expenses were \$11.2 million for the fourth quarter of 2017, compared to \$9.4 million for the fourth quarter of 2016. The increase in research and development expenses for the three months ended December 31, 2017 is primarily due to expanded development in Ziopharm's gene and cell therapy programs.
- | General and administrative expenses were \$3.9 million for the fourth quarter of 2017, compared to \$3.3 million for the fourth quarter of 2016.
- | Ziopharm ended the quarter with unrestricted cash resources of approximately \$70.9 million.
- | As part of Ziopharm's strategic co-development activities at MD Anderson Cancer Center, a prepayment of approximately \$31.9 million remains available for programs to be conducted by the Company at MD Anderson Cancer Center under the current Research and Development Agreement.
- | Ziopharm believes its current resources will be sufficient to fund its currently planned operations into the fourth quarter of 2018.

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

	Three Months Ended December 31, (unaudited)		Year Ended December 31, (audited)	
	2017	2016	2017	2016
Collaboration revenue	\$ 1,597	\$ 1,597	\$ 6,389	\$ 6,861
Operating expenses:				
Research and development	11,181	9,389	45,084	157,791
General and administrative	3,852	3,319	14,798	14,377
Total operating expenses	15,033	12,708	59,882	172,168
Loss from operations	(13,436)	(11,111)	(53,493)	(165,307)
Other income (expense), net	166	32	465	134
Change in fair value of derivative liabilities	(3)	(145)	(1,295)	(124)
Net loss	(13,273)	(11,224)	(54,323)	(165,297)
Preferred stock dividends	(4,999)	(3,532)	(18,938)	(7,123)
Net loss applicable to common stockholders	\$ (18,272)	\$ (14,756)	\$ (73,261)	\$ (172,420)
Basic and diluted net loss per share	\$ (0.13)	\$ (0.11)	\$ (0.53)	\$ (1.32)
Weighted average common shares outstanding used to compute basic and diluted net loss per share	140,644,238	130,524,204	136,938,264	130,391,463

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

(audited)

	December 31, 2017	December 31, 2016
Cash and cash equivalents	70,946	81,053
Working capital	69,927	89,075
Total assets	105,606	106,348
Total stockholders' (deficit)	(96,806)	(77,298)

Conference Call and Slide Webcast

Ziopharm will host a conference call and webcast slide presentation today, March 1, 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 3782628. 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. The webcast will be recorded and available for replay on Ziopharm's website for two weeks.

About Ziopharm Oncology, Inc.

Ziopharm Oncology is a Boston-based biotechnology company focused on development of next-generation immunotherapies utilizing gene- and cell-based therapies to treat patients with cancer. In partnership with Precigen Inc., a wholly-owned subsidiary of Intrexon Corporation (NYSE:XON), Ziopharm is focused on the development of two platform technologies designed to deliver safe, effective and scalable cell- and viral-based therapies for the treatment of multiple cancer types: Controlled IL-12 and *Sleeping Beauty* for genetically modifying T cells. The Company's lead gene therapy product candidate, Ad-RTS-hIL-12 plus veledimex, has demonstrated in clinical trials the potential to control interleukin-12, leading to an infiltration of T cells that fight brain cancer. The Company also is advancing therapies using *Sleeping Beauty*, a non-viral approach to genetically modify chimeric antigen receptor (CAR⁺) and T-cell receptor (TCR⁺) T cells, which target specific antigens in blood cancers and neoantigens solid tumors. *Sleeping Beauty* is designed using the Company's point-of-care technology, a shortened manufacturing process which potentially can be developed as a decentralized manufacturing process based in hospitals. These programs are being advanced in collaboration with Precigen and with MD Anderson Cancer Center, the National Cancer Institute and Merck KGaA, Darmstadt, Germany.

Forward-Looking Disclaimer

This press release contains certain forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Forward-looking statements are statements that are not historical facts. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "would," "could," "potential," "possible," "hope" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These statements include, but are not limited to, statements regarding the statements regarding the Company's financial condition and results of operations; the advancement of, and anticipated development and regulatory milestones and plans related to the Company's product candidates and clinical studies including the Company's research, clinical development and manufacturing plans for its Ad-RTS-hIL-12 plus veledimex gene therapy product candidate; the application of the Company's platforms for the treatment of oncology and the progress and timing of the development of its research and development programs; plans related to the Company's collaborations, particularly with Precigen and National Cancer Institute; and the Company's ability to establish a commercially-viable manufacturing approach and its expected outcomes. All 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: changes in the Company's financial condition and cash needs, funding or other strategic opportunities that become available to the Company, the Company's ability to finance its 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 other product candidates will advance further in the preclinical research or clinical trial 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 the Company's other therapeutic products it develops will be successfully marketed if approved; the strength and enforceability of the Company's intellectual property rights; competition from other pharmaceutical and biotechnology companies; as well as other risk factors contained in the Company's periodic and interim reports filed from time to time with the Securities and Exchange Commission, including but not limited to, the risks and uncertainties set forth in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the fiscal quarter ended December 31, 2017 and subsequent reports that the Company may file with the Securities and Exchange Commission. Readers are cautioned not to place undue reliance on these forward-looking statements that

speak only as of the date hereof, and the Company does 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.

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