



## Ziopharm Oncology Reports First Quarter 2018 Financial Results and Provides Corporate Update

May 10, 2018

- Expanding Controlled IL-12 platform to explore additional tumor types
- Cash runway extended to 2Q 2019 with changes to Controlled IL-12 development plans
- ASCO poster to detail IL-12 data in breast cancer, glioblastoma, highlight potential for combination with checkpoint inhibitors
  - Sleeping Beauty CAR-T cells manufactured in two days entering clinic in 2H
  - Company to host conference call today at 4:30 p.m. ET

BOSTON, May 10, 2018 (GLOBE NEWSWIRE) -- [Ziopharm Oncology](#), Inc. (Nasdaq:ZIOP), today announced financial results for the first quarter ended March 31, 2018, and provided an update on the Company's recent activities.

"The landscapes of CAR-T therapies and the use of cytokines are playing out as anticipated," said Laurence Cooper, M.D., Ph.D., Chief Executive Officer of Ziopharm. "Regarding CAR-T, we find the dominating concern among patients, physicians and payers is the high costs and lengthy manufacturing time. We are addressing these issues head on with *Sleeping Beauty* CAR-T cells manufactured in two days with our technology called 'point-of-care,' which we expect to enter the clinic this year."

Dr. Cooper continued, "Immune-stimulatory cytokines have gained significant momentum as potential combination therapies with immune checkpoint inhibitors. We are excited to build on our Controlled IL-12 platform and its ability to turn cold tumors hot as we explore more tumor types that can benefit from our ability to regulate IL-12."

### Program Updates

#### *Controlled IL-12*

Ziopharm is advancing Ad-RTS-hIL-12 plus veledimex as a gene therapy to treat patients with recurrent glioblastoma (rGBM) as a monotherapy and in combination with an immune checkpoint inhibitor. Ad-RTS-hIL-12 plus veledimex is an adenoviral vector (Ad) administered via a single injection into the tumor and engineered to express human IL-12 (hIL-12), a powerful cytokine that has demonstrated a targeted, anti-tumor immune response with the ability to activate and recruit killer T cells to the tumor site. 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 is taken by mouth and crosses the blood-brain barrier.

The Company today announced that it is focusing its resources on studies of Controlled IL-12 in additional tumor types to demonstrate the value of this platform technology and, as a result, the Company is pausing plans for the pivotal trial in rGBM.

**Combination Trial with OPDIVO<sup>®</sup> (nivolumab) in rGBM Initiated.** Ziopharm has initiated a trial of adult patients with rGBM to evaluate a single dose of Ad-RTS-hIL-12 plus daily veledimex for 15 days in combination with OPDIVO<sup>®</sup> (nivolumab), an immune checkpoint inhibitor targeting programmed death-1 (PD-1). In January, the protocol for this first-ever dosing combination was finalized with the U.S. Food and Drug Administration (FDA), and since then the Company has been working with clinical trial sites. Ziopharm expects the first patient to be dosed in this trial in the second quarter.

**Additional Oncology Indications to be Explored Including in Combination with Immune Checkpoint Inhibitors.** The Company previously has presented compelling data demonstrating that Ad-RTS-hIL-12 plus veledimex can turn cold tumors hot by recruiting and activating a sustained immune response that favorably changes the tumor microenvironment in different tumor types. Therefore, Ziopharm plans to explore the potential of the Controlled IL-12 platform by combining it with immune checkpoint inhibitors in multiple tumor types in addition to rGBM later this year.

**Pivotal Trial in rGBM Paused.** Ziopharm is pausing plans for its pivotal randomized control trial for Ad-RTS-hIL-12 plus 20mg dose of veledimex for the treatment of rGBM. The Company is progressing in resolving previously disclosed technical requirements related to Chemistry and Manufacturing Control (CMC), making this asset phase-3 ready for rGBM.

**Expansion Substudy Added to Phase 1 Trial in rGBM.** Ziopharm is initiating an expansion substudy in its Phase 1 trial to evaluate Ad-RTS-hIL-12 plus veledimex as a monotherapy to treat patients with rGBM. The Company is adding patients who have not received steroids for four weeks prior and have not been treated with bevacizumab for their disease to the cohort receiving a 20mg dose of veledimex. Previously, the Company has shown improvement in survival benefit in patients who received Ad-RTS-hIL-12 plus 20mg of veledimex with no steroids or low-dose steroids. The data from these patients will help further understand the benefits of IL-12 as a single-agent.

**Phase 1 for Pediatric Tumors Ongoing.** Ziopharm is enrolling pediatric patients in its Phase 1 trial of Ad-RTS-hIL-12 with veledimex for the treatment of brain tumors at multiple U.S. sites.

**ASCO 2018 Poster Title Announced.** The company today announced that a research poster entitled, "Demonstration of Anti-Tumor Immunity via Intratumoral Regulated Platform Ad-RTS-hIL-12 in Advanced Breast Cancer and Recurrent Glioblastoma Patients," has been accepted for presentation during the 2018 American Society of Clinical Oncology (ASCO) annual meeting. The presentation is scheduled for the Developmental Therapeutics-Immunotherapy poster session on Monday, June 4, 8 to 11:30 a.m. CT in Hall A. Abstract details will be made public on May 16, per ASCO embargo rules.

*Sleeping Beauty and Adoptive Cell Therapies*

Using Ziopharm's non-viral approach leveraging *Sleeping Beauty* platform 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 Second Half of 2018.** Ziopharm is advancing the *Sleeping Beauty* platform towards point-of-care manufacturing for the very rapid manufacturing of genetically modified CAR<sup>+</sup> 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<sup>+</sup> T cells co-expressing membrane-bound interleukin-15, or mbl15, within two days after genetically modifying T cells from the patient. The Company today announced that the investigational new drug (IND) application has been submitted to the FDA by MD Anderson to initiate a Phase 1 trial to evaluate the point-of-care technology for the investigational treatment of CD19<sup>+</sup> leukemia and lymphoma.

**Phase 1 Trial of *Sleeping Beauty*-Modified TCRs to Treat Solid Tumors Expected to Initiate in Second Half of 2018.** The NCI expects to initiate a Phase 1 trial in the second half of 2018 to evaluate adoptive cell transfer-based immunotherapies genetically modified using the *Sleeping Beauty* platform 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 adoptive cell therapy 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 solid tumor.

**Phase 1 Trial of CD33-specific CAR<sup>+</sup> T Therapy for Acute Myeloid Leukemia (AML).** Enrollment is underway at MD Anderson in the Phase 1 adoptive cellular therapy clinical trial of CAR<sup>+</sup> 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 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.

#### Corporate Update

Ziopharm appointed Robert Hadfield, who has financial, biotech and legal experience, to serve as general counsel. He joined the company from the Longwood Fund, a health care venture capital firm in Boston, and Flex Pharma before that, serving as general counsel in both. Mr. Hadfield received his J.D. from Georgetown University.

#### First-Quarter 2018 Financial Results

- Net loss applicable to the common shareholders for the first quarter of 2018 was \$21.1 million, or \$(0.15) per share, compared to a net loss of \$19.7 million, or \$(0.15) per share, for the first quarter of 2017. The increased net loss is primarily due to a decrease in revenue recognized related to the implementation of ASU 2014-09 and an increase in operating expenses.
- Research and development expenses were \$10.2 million for the first quarter of 2018, compared to \$12.0 million for the first quarter of 2017. The decrease in research and development expenses for the three months ended March 31, 2018 is primarily due to decreased manufacturing and preclinical activity related to our cell and gene therapy programs, as our trials prepare to be moved forward in the clinic.
- General and administrative expenses were \$6.2 million for the first quarter of 2018, compared to \$3.6 million for the first quarter of 2017. The increase in general and administrative expenses for the three months ended March 31, 2018 is primarily due to severance and non-cash stock compensation expense.
- The Company ended the quarter with unrestricted cash resources of approximately \$51.1 million.
- In addition, a prepayment of approximately \$32.4 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 second quarter of 2019.

#### Conference Call and Slide Webcast

Ziopharm will host a webcast and conference call today, May 10, 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 4857096. To access the slides and live 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-based biotechnology company focused on the 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 asset, 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<sup>+</sup>) and T-cell receptor (TCR<sup>+</sup>) T cells, which target specific antigens in blood cancers and neoantigens in 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, 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 business and strategic plans, the availability of cash resources, the Company's ability to establish a commercially-viable manufacturing approach as well as the progress and timing of the development of the Company's research and development programs, including its potential initiation of a first in-human trial using its P-O-C manufacturing process and the timing for the initiation of its clinical trials. 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 year 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.

### Ziopharm Oncology, Inc.

#### Statements of Operations

(in \$ thousands except share and per share data)

Unaudited

	Three Months Ended March 31		
	2018	2017	
Collaboration revenue	\$146	\$1,597	
Operating expenses:			
Research & Development	\$10,183	\$11,967	
General and Administrative	\$6,159	\$3,595	
Total operating expenses	\$16,342	\$15,562	
Loss from operations		(\$16,196 )	(\$13,965 )
Other income (expense), net	\$148	\$38	
Change in fair value of derivative liabilities	\$28		(\$1,560 )
Net loss		(\$16,020 )	(\$15,487 )
Preferred stock dividends		(\$5,120 )	(\$4,171 )
Net loss applicable to common stockholders		(\$21,140 )	(\$19,658 )
Basic and diluted net loss per share		(\$0.15 )	(\$0.15 )
Weighted average common shares outstanding used to compute basic and diluted net loss per share	140,853,120	130,696,400	

### Ziopharm Oncology, Inc.

#### Balance Sheet Data

(in thousands)

(Unaudited)

	March 31, 2018	December 31, 2018
Cash and cash equivalents	\$51,108	\$70,946
Working capital	\$62,053	\$69,927
Total assets	\$87,461	\$105,606
Total stockholders' (deficit)	(\$123,149 )	(\$96,806 )

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