Verastem Reports Third Quarter 2016 Financial Results

November 7, 2016

Company Adds Late-Stage, Complementary Oncology Product Candidate Duvelisib to Pipeline

BOSTON--(BUSINESS WIRE)--Nov. 7, 2016-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to treat cancer, today reported financial results for the third quarter ended September 30, 2016, and also provided an overview of certain corporate developments.

“Last week, we announced the inlicensing of the late-stage oncology product candidate duvelisib from Infinity Pharmaceuticals. This transaction brings a complementary development program on attractive financial terms,” said Robert Forrester, President and Chief Executive Officer of Verastem. “Duvelisib has a proven mechanism of action and has clinically demonstrated anti-cancer activity, along with a manageable safety profile, in several lymphoid malignancies. We believe duvelisib has the potential to help patients with lymphoma.”

Mr. Forrester continued, “Since the beginning of 2016, we have made significant progress with our immuno-oncology focused clinical development program aimed at advancing our FAK inhibitors in combination with immunotherapies and other current and emerging standard of care treatments. We recently announced a new clinical collaboration with Cancer Research UK and MSD to evaluate defactinib in combination with MSD’s anti-PD-1 immunotherapy pembrolizumab (Keytruda®) MSD’s PD-1 immunotherapy in patients with mesothelioma, non-small cell lung and pancreatic cancer. This is our third clinical collaboration to be announced this year and we are delighted to be working with such prestigious organizations to clinically elucidate the potential of defactinib in combination with immunotherapeutics across several different cancer types.”

Third Quarter 2016 and Recent Highlights:

Duvelisib

- ***Inlicensed Late-stage, Complementary Oncology Product Candidate Duvelisib*** – Last week, Verastem and Infinity Pharmaceuticals, Inc. (Infinity) announced the signing of an agreement under which Verastem licensed exclusive worldwide rights to develop and commercialize Infinity’s duvelisib, an investigational product candidate currently in development for hematologic malignancies. In consideration for duvelisib, Verastem will pay to Infinity no upfront payment, and up to $28 million in milestones. Positive data from DUO®, a Phase 3, randomized monotherapy study of duvelisib in patients with relapsed or refractory CLL, triggers the first milestone payment. Verastem will also pay royalties on worldwide net sales. Duvelisib is well aligned with Verastem's strategic focus of developing novel anti-cancer therapeutics that modulate the tumor microenvironment.
- ***Phase 2 DYNAMO Data to be Reported at ASH 2016*** – Phase 2 clinical data for duvelisib will be presented at the 58th American Society of Hematology (ASH) Annual Meeting, which is being held December 3-6, 2016 in San Diego. In an oral presentation, titled “A phase 2 study demonstrating the clinical activity of duvelisib in patients with relapsed refractory indolent non-Hodgkin lymphoma,” (Publication ID: 1218) Ian Flinn, MD, PhD, Director, Hematologic Malignancies Program, Sarah Cannon Research Institute, will describe the results from DYNAMO®, a Phase 2 study evaluating the efficacy and safety of duvelisib in relapsed/refractory iNHL. The oral presentation will take place on Monday, December 5, 2016, at 7:30 PM PT at the San Diego Convention Center, Ballroom 20BC.

Defactinib (VS-6063)

- ***New Clinical Collaboration with Cancer Research UK and MSD to Evaluate Defactinib in Combination with Immunotherapy in Mesothelioma, Non-small Cell Lung and Pancreatic Cancer*** – In September 2016, the companies announced a new clinical trial collaboration agreement to evaluate the combination of Verastem’s defactinib and MSD’s PD-1 immunotherapy pembrolizumab (Keytruda®). This clinical collaboration is based on discoveries by scientists at the Edinburgh Cancer Research UK Centre at the University of Edinburgh who showed that inhibiting FAK increases the effectiveness of anti-PD-1 agents. The trial is expected to enroll up to 60 patients and will commence in early 2017.
- ***Published Preclinical Research in Nature Medicine*** – In July 2016, Verastem announced the publication of preclinical research conducted by its scientific collaborator, David G. DeNardo, PhD, Assistant Professor of Medicine, Division of Oncology, Department of Immunology, Washington University School of Medicine in St. Louis. In the published study, Dr. DeNardo demonstrates that FAK inhibition decreases fibrosis and immunosuppressive cell populations in pancreatic ductal adenocarcinoma, rendering previously unresponsive tumors sensitive to chemo- and immunotherapy. These findings provide important support and rationale for the ongoing Phase 1 dose-escalation clinical studies evaluating Verastem’s FAK inhibitors in combination with pembrolizumab and gemcitabine, and, gemcitabine and Abraxane® in patients with pancreatic cancer.

Third Quarter 2016 Financial Results

Net loss for the third quarter ended September 30, 2016 (2016 Quarter) was $7.9 million, or $0.21 per share, as compared to a net loss of $15.4

million, or $0.42 per share, for the third quarter ended September 30, 2015 (2015 Quarter). Net loss includes non-cash stock-based compensation expense of $1.3 million and $2.1 million for the 2016 Quarter and 2015 Quarter, respectively.

Research and development expense for the 2016 Quarter was $4.2 million compared to $11.3 million for the 2015 Quarter. The $7.1 million decrease from the 2015 Quarter to the 2016 Quarter was primarily related to a decrease of $5.3 million in contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, a decrease in personnel related costs of approximately $653,000 due to lower headcount as a result of our reduction in force in Q4 2015, a decrease of approximately $491,000 in consulting fees, a decrease in lab supplies of approximately $228,000, and a decrease in stock-based compensation and other expenses of approximately $376,000.

General and administrative expense for the 2016 Quarter was $3.8 million compared to $4.2 million for the 2015 Quarter. The decrease of approximately $387,000 from the 2015 Quarter to the 2016 Quarter primarily resulted from decreases in stock-based compensation expense of approximately $665,000 and personnel costs of approximately $108,000. These decreases were partially offset by increases in professional fees of approximately $235,000 and consulting and other costs of approximately $151,000.

As of September 30, 2016, Verastem had cash, cash equivalents and investments of $86.9 million compared to $110.3 million as of December 31, 2015. Verastem used $6.0 million for operating activities during 2016 Quarter.

The number of outstanding common shares as of September 30, 2016, was 36,992,418.

Financial Guidance

Based on current operating plans, we expect to have sufficient cash, cash equivalents and short-term investments to fund our research and development programs and operations into 2018.

About the Tumor Microenvironment

The tumor microenvironment encompasses various cellular populations and extracellular matrices within the tumor or cancer niche that support cancer cell survival. This includes immunosuppressive cell populations such as regulatory T cells, myeloid-derived suppressor cells, M2 tumor-associated macrophages, as well as tumor-associated fibroblasts and extracellular matrix proteins which can hamper the entry and therapeutic benefit of cytotoxic immune cells and anti-cancer drugs. In addition to targeting the proliferative and survival signaling of cancer cells, Verastem's compounds duvelisib, defactinib, VS-4718 and VS-5584 also target the tumor microenvironment as a mechanism of action to potentially improve a patients response to therapy.

About Duvelisib

Duvelisib is an investigational, dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma, two enzymes that are known to help support the growth and survival of malignant B cells and T cells. PI3K signaling may lead to the proliferation of malignant B-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.[1,2,3] Duvelisib is currently being evaluated in late- and mid-stage clinical trials, including DUO®, a randomized, Phase 3 monotherapy study in patients with relapsed/refractory chronic lymphocytic leukemia (CLL)[4], and DYNAMO®, a single-arm, Phase 2 monotherapy study in patients with refractory indolent non-Hodgkin lymphoma (iNHL) that achieved its primary endpoint of overall response rate upon topline analysis of efficacy data[5]. Duvelisib is also being evaluated for the treatment of hematologic malignancies through investigator-sponsored studies, including T cell lymphoma. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.[6]

About Defactinib

Defactinib (VS-6063) is an investigational inhibitor of Focal Adhesion Kinase (FAK), a non-receptor tyrosine kinase encoded by the PTK-2 gene that mediates oncogenic signaling in response to cellular adhesion and growth factors.[7] Based on the multi-faceted roles of FAK, defactinib is used to treat cancer through modulation of the tumor microenvironment, enhancement of anti-tumor immunity, and reduction of cancer stem cells.[8,9] Defactinib is currently being evaluated in three separate clinical collaborations in combination with immunotherapeutic agents for the treatment of several different cancer types including pancreatic, ovarian, non-small cell lung cancer, and mesothelioma. These studies are combination clinical trials with pembrolizumab and avelumab from Merck & Co. and Pfizer/Merck KGaA, respectively.[10,11,12] Information about these and additional clinical trials evaluating the safety and efficacy of defactinib can be found on www.clinicaltrials.gov.

About Verastem, Inc.

Verastem, Inc. (NASDAQ:VSTM) is a biopharmaceutical company focused on discovering and developing drugs to improve outcomes for patients with cancer. Verastem is currently developing duvelisib, a dual inhibitor of phosphoinositide-3-kinase (PI3K)-delta and PI3K-gamma, which has successfully met its primary endpoint in a Phase 2 study and is currently being evaluated in a Phase 3 clinical trial in patients with chronic lymphocytic leukemia (CLL). Other clinical product candidates include focal adhesion kinase (FAK) inhibitors defactinib (VS-6063) and VS-4718, and dual PI3K/mTOR inhibitor VS-5584. Defactinib is currently being evaluated in three separate clinical collaborations in combination with immunotherapeutic agents for the treatment of several different cancer types, including pancreatic, ovarian and non-small cell lung cancer, and mesothelioma. Verastem's product candidates seek to treat cancer by modulating the local tumor microenvironment, enhancing anti-tumor immunity and reducing cancer stem cells. For more information, please visit www.verastem.com.

Verastem forward-looking statements notice:

This press release includes forward-looking statements about Verastem's strategy, future plans and prospects, including statements regarding the development and activity of Verastem's product candidates, duvelisib, defactinib (VS-6063), VS-4718 and VS-5584, and Verastem's FAK, PI3K/mTOR programs generally, the structure of our planned and pending clinical trials and the timeline and indications for clinical development, including reporting top-line data, and regulatory submissions, our rights to develop or commercialize our product candidates and our ability to finance contemplated development activities and fund operations for a specified period. The words "anticipate," "appear," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Each forward-looking statement is subject to risks and

uncertainties that could cause actual results to differ materially from those expressed or implied in such statement. Applicable risks and uncertainties include the risks that the preclinical testing of Verastem's product candidates and preliminary or interim data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that data may not be available when expected, including for the Phase 3 DUO study; that enrollment of clinical trials may take longer than expected; that our product candidates will cause unexpected safety events or result in an unmanageable safety profile as compared to their level of efficacy; that duvelisib will be ineffective at treating patients with lymphoid malignancies; that Verastem will be unable to successfully initiate or complete the clinical development of its product candidates; that the development of Verastem's product candidates will take longer or cost more than planned; that Verastem may not have sufficient cash to fund its contemplated operations; that the cost of the transaction to Verastem will not provide the intended positive financial results; that Verastem or Infinity will fail to fully perform under the license agreement; that the transition of the duvelisib program from Infinity will not be completed; that Verastem will not pursue or submit regulatory filings for its product candidates, including for duvelisib in patients with CLL or iNHL; and that Verastem's product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients. Other risks and uncertainties include those identified under the heading "Risk Factors" in Verastem's Annual Report on Form 10-K for the year ended December 31, 2015 and in any subsequent SEC filings. The forward-looking statements contained in this press release reflect Verastem's current views with respect to future events, and Verastem does not undertake and specifically disclaims any obligation to update any forward-looking statements.

References

[1]Winkler D.G., Faia K.L., DiNitto J.P. et al. PI3K-delta and PI3K-gamma inhibition by IPI-145 abrogates immune responses and suppresses activity in autoimmune and inflammatory disease models. Chem Biol 2013; 20:1-11.

[2]Reif K et al.Cutting Edge: Differential Roles for Phosphoinositide 3 kinases, p110-gamma and p110-delta, in lymphocyte chemotaxis and homing. J Immunol 2004:173:2236-2240.

[3]Schmid M et al. Receptor Tyrosine Kinases and TLR/IL1Rs Unexpectedly activate myeloid cell PI3K, a single convergent point promoting tumor inflammation and progression. Cancer Cell 2011;19:715-727.

[4]www.clinicaltrials.gov, NCT02004522

[5]www.clinicaltrials.gov, NCT01882803

[6]www.clinicaltrials.gov, NCT02783625, NCT02783625, NCT02158091

[7]Schaller MD and Parsons JT. Focal adhesion kinase: an integrin-linked protein tyrosine kinase. Trends Cell Biol. 1993 3: 258-62.

[8]Jiang H et al. Targeting focal adhesion kinase renders pancreatic cancers responsive to checkpoint immunotherapy. Nat Med 2016: Aug 22(8) 851-60.

[9]Sulzmaier FJ et al. FAK in cancer: mechanistic findings and clinical applications. Nature Rev Cancer. 2014 14: 598-610.

[10]www.clinicaltrials.gov, NCT02546531

[11]www.clinicaltrials.gov, NCT02943317

[12]www.clinicaltrials.gov, NCT02758587

Verastem, Inc.
Unaudited Selected Consolidated Balance Sheets

(in thousands)

	September 30, 2016	December 31, 2015
Cash, cash equivalents and investments	$ 86,882	$ 110,258
Prepaid expenses and other current assets	431	585
Property and equipment, net	1,569	2,048
Other assets	162	203
Total assets	**$ 89,044**	**$ 113,094**
Accounts payable and accrued expenses	$ 6,220	$ 10,040

Other liabilities	388	585
Stockholders' equity	82,436	102,469
Total liabilities and stockholders' equity	**$ 89,044**	**$ 113,094**

Verastem, Inc.
Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2016	**2015**	**2016**	**2015**
Operating expenses:				
Research and development	$ 4,216	$ 11,304	$ 12,887	$ 32,877
General and administrative	3,843	4,230	12,315	13,361
Total operating expenses	8,059	15,534	25,202	46,238
Loss from operations	(8,059)	(15,534)	(25,202)	(46,238)
Interest income	137	89	417	236
Net loss	**$ (7,922)**	**$ (15,445)**	**$ (24,785)**	**$ (46,002)**
Net loss per share—basic and diluted	**$ (0.21)**	**$ (0.42)**	**$ (0.67)**	**$ (1.29)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**36,992**	**36,898**	**36,986**	**35,594**

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