Verastem Reports Year-End 2016 Financial Results

March 23, 2017

BOSTON--(BUSINESS WIRE)--Mar. 23, 2017-- Verastem, Inc. (NASDAQ: VSTM), focused on discovering and developing drugs to treat cancer, today reported financial results for the year ended December 31, 2016, and also provided an overview of certain corporate developments.

“2016 was a year of significant achievement for Verastem with the in-licensing of duvelisib, a late-stage, clinical product candidate with broad potential across B-cell and T-cell lymphoid malignancies, and the advancement of defactinib into clinical development in combination with immuno-oncology agents,” said Robert Forrester, President and Chief Executive Officer of Verastem. “As we enter 2017, we are laser-focused on several important milestones, beginning with reporting top-line duvelisib data from the Phase 3 DUO™ study in chronic lymphocytic leukemia (CLL) expected mid-year 2017. There remains an unmet medical need for patients with relapsed CLL. We believe duvelisib has potential as a convenient, oral monotherapy with an expected and manageable safety profile for patients with relapsed CLL. For defactinib, we look forward to advancing our ongoing combination trials into important expansion cohorts across several high unmet need indications.”

Mr. Forrester continued, “On the financial front, we ended 2016 with $80.9 million in cash, cash equivalents and investments, which we believe is sufficient to support our research and development programs and operations into 2018. In March 2017, we entered into a loan facility with Hercules Capital, Inc. for up to $25.0 million, subject to certain conditions including positive DUO data, which would provide us with additional financial flexibility to advance duvelisib.”

Fourth Quarter 2016 and Recent Highlights:

Duvelisib

- ***In-licensed Late-stage, Complementary Oncology Product Candidate Duvelisib*** – Verastem and Infinity Pharmaceuticals, Inc. (Infinity) announced the signing of an agreement under which Verastem licensed exclusive worldwide rights to develop and commercialize duvelisib, an investigational product candidate currently in development for hematologic malignancies. Duvelisib is well aligned with Verastem's strategic focus of developing novel anti-cancer therapeutics that modulate the tumor microenvironment. The transaction provides a new oncology product candidate with demonstrated activity in lymphoid malignancies.
- ***Ongoing Phase 3 DUO Study in Relapsed or Refractory CLL*** – The safety and efficacy of duvelisib is currently being evaluated in the randomized Phase 3 DUO study in patients with relapsed or refractory CLL. In the DUO study, approximately 300 patients were randomized 1:1 to receive duvelisib (25mg BID) or ofatumumab (8 weekly infusions, starting with an initial intravenous dose of 300mg on day 1 followed by 7 weekly doses of 2,000mg, then 2,000mg monthly for 4 cycles). The primary endpoint of this study is progression free survival (PFS). Key secondary endpoints include overall response rate (ORR), overall survival, duration of response (DOR) and safety. Verastem expects to report top-line data from the DUO study in mid-year 2017.
- ***Positive Phase 2 DYNAMO ™Data Reported at ASH 2016*** – Positive Phase 2 clinical data from the DYNAMO study demonstrating the clinical activity of duvelisib in patients with relapsed refractory indolent non-Hodgkin lymphoma (iNHL) were presented at the 58th American Society of Hematology (ASH) Annual Meeting in December 2016. 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, M.D., Ph.D. (Director, Hematologic Malignancies Program, Sarah Cannon Research Institute), described results from 129 patients with double refractory iNHL (median 3 prior anti-cancer regimens, range 1-18). The study met its primary endpoint, achieving an ORR of 46% as determined by an independent review committee (IRC) (p=0.0001; 95% CI 0.37-0.55). Among disease subgroups, the ORR was 41% in follicular lymphoma (n=83), 68% in small lymphocytic lymphoma (n=28), and 33% in marginal zone lymphoma (n=18). The median DOR among all patients was 9.9 months. Notably, 83% of patients had reductions in the size of their target lymph nodes per the IRC. Duvelisib was generally well tolerated, with an expected and manageable safety profile with appropriate risk mitigation. The DYNAMO study showed that duvelisib monotherapy has a favorable benefit-risk profile in refractory iNHL patients and may represent an important treatment option in this population.

Defactinib (VS-6063)

- ***Dosed the First Patient in Combination Trial of Defactinib and Avelumab in Patients with Ovarian Cancer*** – As announced in January 2017, the first patient was dosed in a new clinical trial evaluating avelumab, an investigational fully human anti-PD-L1 IgG1 monoclonal antibody, in combination with Verastem’s defactinib, an investigational focal adhesion kinase (FAK) inhibitor, in patients with advanced ovarian cancer. This multicenter, open-label, dose-escalation and dose-expansion Phase 1/2 clinical trial is designed to assess the safety, pharmacokinetics, pharmacodynamics, and initial observations of clinical activity of the avelumab/defactinib combination in patients with recurrent or refractory stage III-IV

ovarian cancer. The study is being conducted in collaboration with the alliance between Merck KGaA, Darmstadt, Germany, which in the U.S. and Canada operates as EMD Serono, and Pfizer, and is expected to enroll approximately 100 patients at up to 15 sites across the U.S.

Corporate and Financial

- ***Hagop Youssoufian, MSc, M.D., Named Head of Hematology and Oncology Development*** – In January 2017, Dr. Youssoufian assumed this leadership role at Verastem to oversee the clinical and regulatory development of Verastem's pipeline, including duvelisib, and provide overall strategic and tactical leadership to our hematology-oncology clinical programs. Dr. Youssoufian brings over 25 years of product development and commercialization experience to Verastem, having served in senior leadership roles at several oncology-focused companies, including BIND Therapeutics, Progenics Pharmaceuticals, Ziopharm Oncology, Imclone Systems, Sanofi Aventis and Bristol-Myers Squibb where he was involved in the development of Sprycel®, Taxotere® and Erbitux®.
- ***Additional Key Personnel Appointments*** – Recently, Michael Ferraresso joined Verastem as Vice President, Commercial Operations, and Verastem also appointed several highly experienced individuals to the Clinical and Scientific Advisory Board including:
    - Lori Kunkel, M.D., Former Chief Medical Officer, Pharmacyclics
    - Edmund J. Pezalla, M.D., MPH, Former VP, Pharmaceutical Policy and Strategy at Aetna
    - Greg Berk, M.D., Former Chief Medical Officer, Verastem
    - Cheryl Cohen, Former Chief Commercial Officer, Medivation
    - Brian Stuglik, PharmD., Former VP and Chief Marketing Officer, Oncology Global Marketing, Eli Lilly
- ***Secured $25 Million Loan Facility*** – In March 2017, Verastem entered into a Loan and Security Agreement with Hercules Capital, Inc. for up to $25.0 million in financing. Verastem received the first $2.5 million of financing under the Loan and Security Agreement when the transaction closed. The proceeds will be used for Verastem's ongoing research and development programs and for general corporate purposes. Additional tranches of up to $22.5 million in aggregate will be available subject to certain conditions, including positive data from the Phase 3 DUO clinical trial evaluating duvelisib in patients with relapsed or refractory CLL.

Full Year 2016 Financial Results

Net loss for the year ended December 31, 2016 (2016 Period) was $36.4 million, or $0.99 per share, as compared to a net loss of $57.9 million, or $1.61 per share, for the year ended December 31, 2015 (2015 Period). Net loss includes non-cash stock-based compensation expense of $6.2 million and $9.7 million for the 2016 Period and 2015 Period, respectively.

Research and development expense for the 2016 Period was $19.8 million compared to $40.6 million for the 2015 Period. The $20.8 million decrease from the 2015 Period to the 2016 Period was primarily related to a decrease of $15.6 million in external contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, a $3.4 million decrease in personnel related costs, primarily due to the reduction in workforce in October 2015, a decrease of $1.3 million in stock-based compensation expense and a decrease of $1.5 million in lab supplies, travel and other research and development expense. These decreases were partially offset by an increase of approximately $947,000 in consulting and professional fees.

General and administrative expense for the 2016 Period was $17.2 million compared to $17.6 million for the 2015 Period. The approximate $411,000 decrease from the 2015 Period to the 2016 Period primarily resulted from a decrease of $2.1 million in stock-based compensation expense. This decrease was partially offset by increases of $1.1 million in consulting and professional fees, approximately $280,000 in personnel costs, and a net increase of approximately $306,000 of other general and administrative costs.

As of December 31, 2016, Verastem had cash, cash equivalents and investments of $80.9 million compared to $110.3 million as of December 31, 2015. Verastem used $29.5 million for operating activities during the 2016 Period.

The number of outstanding common shares as of December 31, 2016, was 36,992,418.

Financial Guidance

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

Conference Call Information

The Verastem management team will host a conference call today, Thursday, March 23, 2017, at 4:30 PM (ET). The call can be accessed by dialing 1-877-341-5660 or 1-315-625-3226 five minutes prior to the start of the call and providing the passcode 89196444.

The live, listen-only webcast of the conference call can be accessed by visiting the investors section of the Company's website at www.verastem.com. A replay of the webcast will be archived on the Company's website for 90 days following the call.

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 product

candidates, including duvelisib and defactinib, also target the tumor microenvironment as a mechanism of action to potentially improve a patient's 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 or refractory CLL[4], and DYNAMO™, a single-arm, Phase 2 monotherapy study in patients with refractory iNHL that achieved its primary endpoint of ORR upon top-line analysis of efficacy data[5]. Duvelisib is also being evaluated for the treatment of hematologic malignancies through investigator-sponsored studies, including T-cell lymphoma.[6] Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

About Defactinib

Defactinib is an investigational inhibitor of 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 cancer, ovarian cancer, 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 PI3K-delta and PI3K-gamma, which has successfully met its primary endpoint in a Phase 2 study in iNHL and is currently being evaluated in a Phase 3 clinical trial in patients with CLL. In addition, Verastem is developing the FAK inhibitor defactinib, which is currently being evaluated in three separate clinical collaborations in combination with immunotherapeutic agents for the treatment of several different cancer types, including pancreatic cancer, ovarian cancer 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 investigational product candidates, including duvelisib and defactinib (VS-6063), and Verastem's PI3K and FAK 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," "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 Verastem or Infinity will fail to fully perform under the duvelisib license agreement; that the transition of the duvelisib program from Infinity will not be completed; that Verastem may be unable to make additional draws under its debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; 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, 2016 and in any subsequent filings with the U.S. Securities and Exchange Commission. The forward-looking statements contained in this press release reflect Verastem's views as of the date of this release, 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

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[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)

	December 31, 2016	December 31, 2015
Cash, cash equivalents and investments	$ 80,897	$ 110,258
Prepaid expenses and other current assets	398	585
Property and equipment, net	1,417	2,048
Other assets	917	203
Total assets	**$ 83,629**	**$ 113,094**
Accounts payable and accrued expenses	$ 10,991	$ 10,040
Other liabilities	341	585
Stockholders' equity	72,297	102,469
Total liabilities and stockholders' equity	**$ 83,629**	**$ 113,094**

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Year ended December 31, 2016	2015
Operating expenses:		
Research and development	$ 19,779	$ 40,565
General and administrative	17,223	17,634
Total operating expenses	37,002	58,199
Loss from operations	(37,002)	(58,199)
Interest income	562	334
Net loss	**$ (36,440)**	**$ (57,865)**
Net loss per share—basic and diluted	**$ (0.99)**	**$ (1.61)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**36,988**	**35,932**

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