Verastem Reports First Quarter 2017 Financial Results

May 10, 2017

BOSTON--(BUSINESS WIRE)--May 10, 2017-- Verastem, Inc. (NASDAQ: VSTM), focused on discovering and developing drugs to improve the survival and quality of life of cancer patients, today reported financial results for the first quarter ended March 31, 2017 and provided an overview of certain corporate developments.

“Following the presentation of positive data from the DYNAMO™ study of duvelisib in indolent non-Hodgkins Lymphoma (iNHL) at theAmerican Society of Hematology conference in December 2016, we are focused on executing against the important milestones that lie ahead, beginning with reporting top-line duvelisib data from the Phase 3 DUO™ study in chronic lymphocytic leukemia (CLL), which is expected mid-year 2017,” said Robert Forrester, President and Chief Executive Officer of Verastem. “We continue to believe duvelisib has significant potential as a convenient, oral monotherapy for patients with relapsed CLL and possibly other lymphomas, where there remains an unmet medical need.”

Mr. Forrester continued, “For defactinib, the program continues to advance across three ongoing clinical collaborations evaluating focal adhesion kinase (FAK) inhibition in combination with immuno-oncology agents.”

First Quarter 2017 and Recent Highlights:

Duvelisib

- ***Long Term Follow Up Data from the DYNAMO Study Selected for Oral Presentation at the 14th International Conference on Malignant Lymphoma (ICML)*** – In early May, Verastem announced that an abstract highlighting long term follow up data from the ongoing Phase 2 DYNAMO study was selected for oral presentation at ICML 2017 in Lugano, Switzerland. The presentation, titled “DYNAMO: A Phase 2 Study Demonstrating the Clinical Activity of Duvelisib in Patients with Double-Refractory Indolent Non-Hodgkin Lymphoma,” will be presented by Pier Luigi Zinzani, M.D., Ph.D., of the University of Bologna Institute of Hematology, on Thursday, June 15, 2017 at 15:40 CET in Room A, Cinema Corso and Aula Magna (Lugano University).
- ***Ongoing Phase 3 DUO Study in Relapsed or Refractory CLL*** – The efficacy and safety 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 trial was fully enrolled in November 2015. 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.
- ***Published Scientific Research Demonstrating the Potential of Duvelisib in Combination with Venetoclax*** – A recent publication[1] in *Leukemia* by Patel and colleagues provides scientific rationale for the combination of duvelisib with the BCL2 inhibitor venetoclax for the treatment of CLL. Using samples from duvelisib-treated CLL patients, this group at the University of Texas MD Anderson Cancer Center found that duvelisib-treatment increased expression of several pro-apoptotic proteins such that the CLL cells were poised for apoptosis. They went on to show that CLL cells from patients after duvelisib treatment were killed more effectively by venetoclax than CLL cells taken from the same patients before duvelisib treatment.

Defactinib (VS-6063)

- ***Presented Defactinib Data at the 2017 American Association for Cancer Research Annual Meeting*** – In an oral presentation titled, “Reprogramming the tumor microenvironment to improve responses to therapy,” Verastem scientific collaborator David G. DeNardo, Ph.D., Assistant Professor of Medicine, Division of Oncology, Department of Immunology, Washington University School of Medicine in St. Louis, described data demonstrating that FAK inhibition can enable efficacy of PD-1 inhibition in preclinical models of pancreatic cancer that, like the clinical disease, are otherwise refractory to checkpoint inhibition. Verastem’s FAK inhibitor, defactinib, is currently being evaluated in combination with Merck’s PD-1 inhibitor, pembrolizumab, and gemcitabine in patients with advanced pancreatic ductal adenocarcinoma (PDAC). Initial analysis of immune biomarkers from matched pairs of metastatic biopsies, taken either pre- or post-treatment, from patients with PDAC showed an increase in activated proliferating cytotoxic T-cells together with a reduction in tumor-associated macrophages (TAMs).
- ***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 defactinib 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.

- ***Updated Data from the Window of Opportunity Study in Mesothelioma Selected for Poster Presentation at the American Society of Clinical Oncology (ASCO) 2017 Annual Meeting*** – An abstract highlighting updated data from the ongoing Phase 2 Window of Opportunity study was selected for a poster presentation at ASCO 2017 in Chicago. The presentation, titled "Effect of FAK inhibitor defactinib on tumor immune changes and tumor reductions in a phase II window of opportunity study in malignant pleural mesothelioma (MPM)," will be presented by Raphael Bueno, M.D., of the Brigham and Women's Hospital and Harvard Medical School, on Saturday, June 3, 2017 from 8:00-11:30am CT in Hall A at McCormick Place.

Corporate and Financial

- ***Eric K. Rowinsky Appointed to the Board of Directors*** – Verastem announced the appointment of Eric K. Rowinsky, M.D., to its Board of Directors. Dr. Rowinsky brings to Verastem nearly 30 years of experience in the development of cancer treatments, such as cetuximab (Erbitux®) when he was Chief Medical Officer of ImClone Systems, as well as Cyramza®, Portrazza®, Taxol®, Taxotere®, Hycamtin®, Tarceva®, Camptosar®, Tykerb®, and cixutumumab, among others. Dr. Rowinsky is a member of the board of directors of Biogen, Navidea, and Fortress Biotech, all public life sciences companies, and has served on the board of directors of BIND Therapeutics, a life-science company acquired by Pfizer. Dr. Rowinsky is replacing Paul A. Friedman, M.D. who is transitioning from his role as Director to become a member of Verastem's Clinical and Scientific Advisory Board.
- ***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 its hematology-oncology clinical programs. Dr. Youssoufian brings over 25 years of product development and commercialization experience to Verastem, having served as Chief Medical Officer at BIND Therapeutics, Ziopharm Oncology and Imclone Systems, and other senior roles at Progenics, Sanofi Aventis and Bristol-Myers Squibb where he was involved in the development of Sprycel®, Taxotere® Erbitux®, Cyramza®, Portrazza® and Lartruvo®.
- ***Additional Key Personnel Appointments*** – Michael Ferraresso joined Verastem as Vice President, Commercial Operations, and Verastem also appointed several highly experienced individuals to its Clinical and Scientific Advisory Board, including Lori Kunkel, M.D., former Chief Medical Officer at Pharmacyclics, Edmund J. Pezalla, M.D., MPH, Former Vice President, Pharmaceutical Policy and Strategy at Aetna, Greg Berk, M.D., former Chief Medical Officer at Verastem, Inc., Cheryl Cohen, former Chief Commercial Officer at Medivation, Inc., and Brian Stuglik, R.Ph, former Vice President and Chief Marketing Officer, Oncology Global Marketing at 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.

First Quarter 2017 Financial Results

Net loss for the three months ended March 31, 2017 (2017 Quarter) was $13.0 million, or $0.35 per share, as compared to a net loss of $8.3 million, or $0.22 per share, for the three months ended March 31, 2016 (2016 Quarter). Net loss includes non-cash stock-based compensation expense of $1.2 million and $1.6 million for the 2017 Quarter and 2016 Quarter, respectively.

Research and development expense for the 2017 Quarter was $8.4 million compared to $4.2 million for the 2016 Quarter. The $4.2 million increase from the 2016 Quarter to the 2017 Quarter was primarily related to an increase of $2.8 million in contract research organization expense for outsourced biology, chemistry, development and clinical services, which includes clinical trial costs, an increase in personnel related costs of approximately $965,000, and an increase of approximately $554,000 in consulting fees. These increases were offset by a decrease in stock-based compensation and other expenses of approximately $86,000.

General and administrative expense for the 2017 Quarter was $4.8 million compared to $4.3 million for the 2016 Quarter. The increase of approximately $508,000 from the 2016 Quarter to the 2017 Quarter primarily resulted from an increase in consulting and professional fees of approximately $922,000, partially offset by a decrease in stock-based compensation expense of approximately $397,000.

As of March 31, 2017, Verastem had cash, cash equivalents and investments of $72.6 million compared to $80.9 million as of December 31, 2016. Verastem used $10.7 million for operating activities during the 2017 Quarter.

The number of outstanding common shares as of March 31, 2017, 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.

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.[2,3,4] 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, 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[6]. Duvelisib is also being evaluated for the treatment of hematologic malignancies through investigator-sponsored studies, including T-cell lymphoma.[7] 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.[8] 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.[9,10] 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.[11,12,13] 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, 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, Inc. 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 Pharmaceuticals, Inc. (Infinity) will fail to fully perform under the duvelisib license agreement; 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

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Verastem, Inc.
Unaudited Condensed Consolidated Balance Sheets

(in thousands)

	March 31, 2017	December 31, 2016
Cash, cash equivalents and investments	$ 72,571	$ 80,897
Prepaid expenses and other current assets	1,434	398
Property and equipment, net	1,271	1,417
Other assets	973	917
Total assets	**$ 76,249**	**$ 83,629**
Accounts payable and accrued expenses	$ 13,233	$ 10,991
Long-term debt	2,249	—
Other liabilities	295	341
Stockholders' equity	60,472	72,297
Total liabilities and stockholders' equity	**$ 76,249**	**$ 83,629**

Verastem, Inc.
Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended March 31, 2017	2016
Operating expenses:		
Research and development	$ 8,385	$ 4,179
General and administrative	4,763	4,255
Total operating expenses	13,148	8,434
Loss from operations	(13,148)	(8,434)
Interest income	155	140
Interest expense	(12)	—
Net loss	**$ (13,005)**	**$ (8,294)**
Net loss per share—basic and diluted	**$ (0.35)**	**$ (0.22)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**36,992**	**36,975**

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