Verastem Reports Year-End 2017 Financial Results

March 13, 2018

BOSTON--(BUSINESS WIRE)--Mar. 13, 2018-- Verastem, Inc. (NASDAQ:VSTM), focused on developing and commercializing drugs to improve the survival and quality of life of cancer patients, today reported financial results for the year ended December 31, 2017 and provided an overview of certain corporate developments and plans.

"The last year has been marked by significant achievement for Verastem with the reporting of positive data from the pivotal Phase 3 DUO™ study and culminating in the recent submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) seeking full approval for duvelisib for the treatment of relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) and accelerated approval for the treatment of relapsed or refractory follicular lymphoma (FL)," said Robert Forrester, President and Chief Executive Officer of Verastem. "As we await the potential acceptance and approval of the duvelisib NDA, we are diligently working to build our commercial infrastructure and preparing for our first potential product launch. I am delighted that Joe Lobacki has joined the team. Joe's formidable expertise in commercialising oncology drugs at Medivation, Micromet and Genzyme positions Verastem to successfully execute on our launch plan for duvelisib in the US."

Fourth Quarter 2017 and Recent Highlights:

Duvelisib

- ***Duvelisib NDA submitted to FDA*** – In early February 2018, Verastem submitted an NDA to the FDA seeking full approval for its lead product candidate duvelisib, a first-in-class oral dual inhibitor of phosphoinositide-3-kinase (PI3K)-delta and PI3K-gamma, for the treatment of relapsed or refractory CLL/SLL and accelerated approval for the treatment of relapsed or refractory FL. The NDA is supported by clinical data from the randomized Phase 3 DUO™ study, which met its primary endpoint by demonstrating statistically significant efficacy, along with a consistent and manageable safety profile, for duvelisib monotherapy in patients with relapsed or refractory CLL/SLL. The NDA is also supported by results from the Phase 2 DYNAMO™ study, which also met its primary endpoint by demonstrating a statistically significant improvement in overall response rate (ORR) compared to an historical control in patients with indolent non-Hodgkin's lymphoma that are double-refractory to both rituximab and chemotherapy or radioimmunotherapy.
- ***Clinical Data from Pivotal Phase 3 DUO Study Highlighted in an Oral Presentation at ASH 2017*** – Verastem presented results from the Phase 3 DUO study at the American Society of Hematology 2017 Annual Meeting (ASH 2017). The presentation, titled "Results from the Phase 3 DUO Trial: A Randomized Comparison of Duvelisib vs Ofatumumab in Patients with Relapsed/Refractory Chronic Lymphocytic Leukemia or Small Lymphocytic Lymphoma," was presented by principal investigator Ian Flinn, M.D., Ph.D., Director of the Blood Cancer Research Program at Sarah Cannon Research Institute. The DUO study met its primary endpoint with oral duvelisib monotherapy achieving a statistically significant improvement in progression free survival (PFS) compared to ofatumumab in patients with relapsed or refractory CLL/ SLL per a blinded independent review committee (IRC) using modified international workshop on CLL (iwCLL) and revised International Working Group (IWG) Response Criteria (median PFS=13.3 months versus 9.9 months, respectively; HR=0.52, p<0.0001), representing a 48% reduction in the risk of progression or death. Oral duvelisib monotherapy also achieved a statistically significant improvement in ORR compared to ofatumumab (74% vs 45%, respectively; p<0.0001), and reduced lymph node burden of less than 50% in most patients compared to ofatumumab (85% vs 16%, respectively). Duvelisib monotherapy demonstrated a manageable safety profile, with results from this study consistent with the well-characterized safety profile of duvelisib monotherapy in patients with advanced hematologic malignancies in previous studies. For duvelisib-treated patients, the median time on treatment was 50.3 weeks (range, 0.9 - 160.0) compared to 23.1 weeks (range, 0.1 - 26.1) for ofatumumab.
- ***Additional Duvelisib Abstracts Presented at ASH 2017*** – Along with the Phase 3 DUO results, two additional duvelisib abstracts were presented at ASH 2017. The abstract, titled "In Vitro, In Vivo, and Parallel Phase I Evidence Support the Safety and Activity of Duvelisib, a PI3K-δ,γ Inhibitor, in Combination with Romidepsin or Bortezomib in Relapsed/Refractory T-Cell Lymphoma," was given as an oral presentation by Alison Moskovitz, M.D., Memorial Sloan Kettering Cancer Center.
- ***Preclinical Data Highlighting the Synergistic Effects in Combination with Immunotherapy Presented at the American Society of Clinical Oncology Clinical Immuno-Oncology Symposium (ASCO-SITC)*** – In January 2018, Jonathan Pachter, Ph.D., Chief Scientific Officer of Verastem, presented preclinical data highlighting the potential synergistic effects of duvelisib in combination with immune checkpoint or co-stimulatory antibodies in B-cell lymphoma. This data, outlined in a poster titled "The Dual PI3K-δ,γ Inhibitor Duvelisib Stimulates Anti-Tumor Immunity and Enhances Efficacy of Immune Checkpoint and Co-Stimulatory Antibodies in a B-Cell Lymphoma Model," supports the further exploration of duvelisib in combination with anti-PD-1/PD-L1 or co-stimulatory antibodies in patients with B-cell malignancies.

Defactinib

- ***Defactinib Preclinical Abstract Presented at ASH 2017 –*** A poster describing preclinical data in combination with B-cell lymphoma 2 (BCL-2) was presented at ASH 2017. The abstract, titled “Combinatorial Inhibition of Focal Adhesion Kinase and BCL-2 in AML,” was presented by Xiangmeng Wang, Ph.D., MD Anderson Cancer Center.

Corporate and Financial

- ***Joseph Lobacki Appointed Chief Commercial Officer –*** In January 2018, Verastem announced the appointment of Joseph Lobacki as Executive Vice President and Chief Commercial Officer. Mr. Lobacki, formerly Chief Commercial Officer and Executive Council Member at Medivation, is responsible for overseeing the commercial strategy and execution for Verastem’s lead product candidate, duvelisib. Mr. Lobacki is a skilled leader in commercializing oncology drugs and his strong experience in hematologic oncology commercialization and marketing make him an invaluable addition to the Verastem team.
- ***Additional Financing Through Increasing Debt Facility to up to $50.0 Million and a $25.0 Million Public Offering –*** In January 2018, Verastem amended its loan and security agreement with Hercules Capital, Inc. (Hercules), increasing its existing borrowing limit under the loan facility from up to $25.0 million to up to $50.0 million in financing, subject to certain conditions of funding. In December 2017, the Company successfully completed an underwritten public offering of shares of common stock with gross proceeds totaling approximately $25.0 million.
- ***NgocDiep Le, MD, PhD, Appointed Chief Medical Officer –*** In October 2017, Verastem announced the appointment of Dr. Le as its Chief Medical Officer. A trained medical oncologist, Dr. Le is board certified in internal medicine and has 15 years of drug development experience across all phases in both solid and liquid tumors, with specialized expertise in clinical development. Dr. Le joins Verastem from MedImmune (a wholly owned subsidiary of AstraZeneca) where she served as Vice President, Immuno-Oncology Innovative Medicines and led the product development teams for multiple high-priority immuno-oncology assets. Dr. Le oversees the development strategy and activities for Verastem’s core assets, duvelisib and defactinib.
- ***Paid First Development Milestone to Infinity Pharmaceuticals*** – In October 2017, Verastem paid to Infinity Pharmaceuticals, Inc. (Infinity) a $6.0 million milestone payment, representing the first milestone under the duvelisib license agreement. This milestone is based on the achievement of positive top-line results from the Phase 3 DUO study evaluating the efficacy and safety of duvelisib in patients with relapsed or refractory CLL/SLL. The milestone was paid using funds drawn from Verastem’s existing loan and security agreement with Hercules.

Full Year 2017 Financial Results

Net loss for the year ended December 31, 2017 (2017 Period) was $67.8 million, or $1.76 per share, as compared to a net loss of $36.4 million, or $0.99 per share, for the year ended December 31, 2016 (2016 Period). Net loss includes non-cash stock-based compensation expense of $5.0 million and $6.2 million for the 2017 Period and 2016 Period, respectively. Verastem used $57.3 million of cash for operating activities during the 2017 Period.

Research and development expense for the 2017 Period was $46.4 million compared to $19.8 million for the 2016 Period. The $26.6 million increase from the 2016 Period to the 2017 Period was primarily related to an increase of $13.4 million in external clinical research organization expense for outsourced biology, chemistry, development and clinical services, which includes our clinical trial costs, the achievement of a $6.0 million milestone pursuant to our license agreement with Infinity, an increase of $5.1 million in consulting fees, and an increase in personnel related costs of $1.9 million.

General and administrative expense for the 2017 Period was $21.4 million compared to $17.2 million for the 2016 Period. The increase of $4.2 million from the 2016 Period to the 2017 Period primarily resulted from increases in consulting and professional fees of $4.4 million, including $2.5 million related to commercial launch preparation, and an increase in personnel costs of $1.0 million. These increases were partially offset by a decrease in stock-based compensation expense of $1.5 million.

As of December 31, 2017, Verastem had cash, cash equivalents and investments of $86.7 million compared to $80.9 million as of December 31, 2016.

The number of outstanding common shares as of December 31, 2017, was 50,800,908.

Financial Guidance

Based on our current operating plans, we expect to have sufficient cash, cash equivalents and investments to fund our current operating plan and capital expenditure requirements into the second half of 2018.

About Duvelisib

Duvelisib is a first-in-class investigational, dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma, two enzymes 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- and T-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.[1,2,3] Duvelisib was evaluated in late- and mid-stage extension trials, including DUO™, a randomized, Phase 3 monotherapy study in patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL),[4] and DYNAMO™, a single-arm, Phase 2 monotherapy study in patients with refractory indolent non-Hodgkin lymphoma (iNHL).[5] Both DUO and DYNAMO achieved their primary endpoints and Verastem has submitted a new drug application (NDA) requesting the full approval of duvelisib for the treatment of patients with relapsed or refractory CLL/SLL, and accelerated approval for the treatment of patients with relapsed or refractory follicular lymphoma (FL). Duvelisib is also being developed by Verastem for the treatment of peripheral

T-cell lymphoma (PTCL), and is being investigated in combination with other agents through investigator-sponsored studies.[6] Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

About Defactinib

Defactinib is an investigational inhibitor of focal adhesion kinase (FAK), a non-receptor tyrosine kinase 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 and enhancement of anti-tumor immunity.[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 (NSCLC), 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 developing and commercializing drugs to improve the survival and quality of life of cancer patients. 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 indolent Non-Hodgkin Lymphoma (iNHL) and a Phase 3 clinical trial in patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL). Verastem has submitted a New Drug Application (NDA) requesting the full approval of duvelisib for the treatment of patients with relapsed or refractory CLL/SLL, and accelerated approval for the treatment of patients with relapsed or refractory follicular lymphoma (FL). 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 (NSCLC), and mesothelioma. Verastem's product candidates seek to treat cancer by modulating the local tumor microenvironment and enhancing anti-tumor immunity. 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, and Verastem's PI3K and FAK programs generally, the structure of our planned and pending clinical trials, Verastem's financial guidance and the timeline and indications for clinical development and regulatory submissions. 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 acceptance or approval of the NDA will not occur on the expected timeframes or at all; that even if data from clinical trials is positive, regulatory authorities may require additional studies for approval and the product may not prove to be safe and effective; 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 the full data from the DUO study will not be consistent with the previously presented results of the study; that data may not be available when expected, including for the Phase 3 DUO™ study; that the degree of market acceptance of product candidates, if approved, may be lower than expected; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that there may be competitive developments affecting our product candidates; that data may not be available when expected; 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/SLL 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, 2017 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 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 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 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, NCT02158091

[7] Schaller M.D. and Parsons J.T. 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 F.J. 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.

Selected Consolidated Balance Sheets

(in thousands)

	December 31, 2017	December 31, 2016
Cash, cash equivalents and investments	$ 86,672	$ 80,897
Prepaid expenses and other current assets	1,115	398
Property and equipment, net	861	1,417
Other assets	1,143	917
Total assets	**$ 89,791**	**$ 83,629**
Accounts payable and accrued expenses	$ 17,128	$ 10,991
Long-term debt	14,828	—
Other liabilities	151	341
Stockholders' equity	57,684	72,297
Total liabilities and stockholders' equity	**$ 89,791**	**$ 83,629**

Verastem, Inc.

Consolidated Statements of Operations

(in thousands, except per share amounts)

	Year ended December 31, 2017	2016
Operating expenses:		
Research and development	$ 46,423	$ 19,779
General and administrative	21,381	17,223
Total operating expenses	67,804	37,002
Loss from operations	(67,804)	(37,002)
Interest income	561	562
Interest expense	(559)	—
Net loss	**$ (67,802)**	**$ (36,440)**
Net loss per share—basic and diluted	**$ (1.76)**	**$ (0.99)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**38,422**	**36,988**

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