Verastem Reports Third Quarter 2017 Financial Results

November 7, 2017

BOSTON--(BUSINESS WIRE)--Nov. 7, 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 third quarter ended September 30, 2017 and provided an overview of certain corporate developments and plans.

"The third quarter was a pivotal time for Verastem with the announcement of positive top-line data from the pivotal Phase 3 DUO™ study evaluating oral duvelisib monotherapy in patients with relapsed or refractory chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL)," said Robert Forrester, President and Chief Executive Officer of Verastem. "The DUO study met its primary endpoint, with progression-free survival (PFS) significantly favoring duvelisib monotherapy over ofatumumab, an approved standard of care treatment for patients with CLL/SLL. We recently met with the U.S. Food and Drug Administration (FDA), and based on the meeting and written feedback, we intend to submit, during the first quarter of 2018, 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). The NDA will be based on a comprehensive clinical data package, including the Phase 3 DUO and Phase 2 DYNAMO studies evaluating duvelisib in patients with advanced hematologic malignancies."

Mr. Forrester concluded, "At the upcoming American Society of Hematology 2017 Annual Meeting (ASH 2017), we will be presenting data from multiple studies, including the detailed positive results from the Phase 3 DUO study, which were selected for an oral presentation."

Third Quarter 2017 and Recent Highlights:

Duvelisib

- ***Announced Regulatory Strategy for Duvelisib NDA –*** Verastem recently announced that a meeting was held with the U.S. Food and Drug Administration (FDA) regarding the regulatory path for duvelisib. Based on the meeting with, and written feedback from the FDA, Verastem intends to submit 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 FL. Along with the clinical data from the DUO study, the duvelisib NDA submission will also contain the favorable results from the Phase 2 DYNAMO™ study in double-refractory indolent non-Hodgkin's lymphoma (iNHL), which also achieved its primary endpoint with an ORR of 46% ($p<0.0001$). In the subset of patients enrolled in the DYNAMO study with double-refractory FL (n=83), duvelisib demonstrated an ORR of 41%. The Company expects to submit the duvelisib NDA during the first quarter of 2018.
- ***Reported Positive Top-line Data from the Pivotal Phase 3 DUO Study in Relapsed or Refractory CLL/SLL –*** In September 2017, Verastem reported that the Phase 3 DUO study met its primary endpoint with oral duvelisib monotherapy demonstrating superiority over ofatumumab for PFS in patients with CLL/SLL. In this study, duvelisib achieved a statistically significant improvement in median PFS of 13.3 months, compared to 9.9 months for ofatumumab with a hazard ratio of 0.52 ($p<0.0001$), representing a 48% reduction in the risk of progression or death. Duvelisib monotherapy had 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.
- ***Expanded Duvelisib Program to Include Peripheral T-Cell Lymphoma –*** In September 2017, Verastem announced the expansion of its duvelisib development program to include targeting the treatment of patients with Peripheral T-Cell Lymphoma (PTCL). Duvelisib was granted Fast Track designation by the FDA for the treatment of patients with PTCL who have received at least one prior therapy. Development of duvelisib in PTCL is supported by encouraging Phase 1 clinical data which demonstrated a 50% investigator-assessed overall response rate in 16 heavily pre-treated patients with relapsed or refractory PTCL, including 3 (19%) complete responses and 5 (31%) partial responses. Verastem intends to initiate an open-label, multicenter, Phase 2 clinical trial evaluating the efficacy and safety of duvelisib in patients with relapsed or refractory PTCL by the end of 2017.
- ***Clinical Data from Pivotal Phase 3 DUO Study Selected for Oral Presentation at ASH 2017 –*** Verastem recently announced that an abstract highlighting clinical data from the Phase 3 DUO study was selected for oral presentation at 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," will be presented by principal investigator, Ian Flinn, M.D., Ph.D., Director of the Blood Cancer Research Program at Sarah Cannon Research Institute, on Sunday, December 10, 2017 at 4:30pm ET at the Georgia World Congress Center, in Building B, Level 5, Murphy BR 3-4.
- ***Additional Duvelisib Abstracts Selected for Presentation at ASH 2017 –*** Along with the Phase 3 DUO results, two additional duvelisib abstracts were selected for presentation 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," will be given as an oral presentation by Steven Horowitz, M.D., Memorial Sloan Kettering Cancer Center, on Monday, December 11, 2017 at 5:00pm ET at the Georgia World Congress Center, in Building A, Level 4, Marcus Auditorium. The abstract, 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," will be given as a poster presentation by Jonathan Pachter, Ph.D., Chief Scientific Officer of Verastem, on Saturday, December 9, 2017 from 5:30-7:30pm ET at the Georgia World Congress Center, in Building A, Level 1, Hall A2.

- ***Verastem to Host Key Opinion Leader Event at ASH 2017 –*** On Sunday, December 10, 2017, Verastem will host an investor and analyst reception, which will feature a moderated panel discussion/Q&A including Ian Flinn, MD, Director of the Blood Cancer Research Program at Sarah Cannon Cancer Center in Nashville, TN. The event will take place during the ASH 2017 annual meeting and interested parties can access a live webcast of the event beginning December 10, 2017 at 8pm ET by going to the "News and Press" section of the Verastem website at www.verastem.com.

Defactinib

- ***Published Scientific Data Highlighting Potential Role of Focal Adhesion Kinase (FAK) Inhibition in Pancreatic and Breast Cancer –*** In July 2017, Verastem announced the publication of two papers in the peer-reviewed journals, *PLoS One* and *Oncotarget*. The two published articles reported scientific findings from studies evaluating FAK inhibition in preclinical models of pancreatic and breast cancer and continue to validate the underlying thesis for ongoing clinical collaborations evaluating Verastem's lead FAK inhibitor, defactinib, in combination with chemotherapeutic and leading immunotherapeutic agents in several difficult to treat types of cancer. The *PLoS One* paper in pancreatic cancer is available here and the *Oncotarget* paper in breast cancer is available here.
- ***Defactinib Preclinical Abstract Selected for Presentation at ASH 2017 –*** An abstract describing preclinical data in combination with BCL-2 was selected for presentation at ASH 2017. The abstract, titled "Combinatorial Inhibition of Focal Adhesion Kinase and BCL-2 in AML," will be given as a poster presentation by Xiangmeng Wang, Ph.D., on Sunday, December 10, 2017 from 6:00-8:00pm ET at the Georgia World Congress Center, in Building A, Level 1, Hall A2.

Corporate and Financial

- ***Brian Stuglik, R.Ph. Appointed to the Board of Directors –*** In September 2017, Verastem announced the appointment of Mr. Stuglik to its Board. Mr. Stuglik, former Chief Marketing Officer for Lilly Oncology, brings to Verastem 35 years of experience in pharmaceutical and oncology commercialization in both the U.S. and international markets. He has successfully launched several multi-billion dollar brands over his career, including Gemzar®, Alimta® and Erbitux®.
- ***NgocDiep Le, M.D., Ph.D., 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 hematologic malignancies as well as IND and NDA submissions. Dr. Le joins Verastem from MedImmune (a 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. Prior to joining MedImmune, Dr. Le held roles of increasing responsibilities at Novartis and at GlaxoSmithKline where she led the MEK inhibitor, trametinib (Mekinist™), from the first-in-human studies to FDA approval. Dr. Le received a Bachelor in Science degree from the California Institute of Technology, and earned both MD and PhD degrees from Stanford University School of Medicine. Dr. Le will oversee the development strategy and activities for Verastem's duvelisib and defactinib.
- ***Julie B. Feder Appointed Chief Financial Officer –*** In July 2017, Verastem announced the appointment of Ms. Feder as its Chief Financial Officer. Ms. Feder is an accomplished financial professional with invaluable leadership experience in the healthcare industry. She joins Verastem from the Clinton Health Access Initiative, Inc. (CHAI), where she served as Chief Financial Officer. Prior to joining CHAI, Ms. Feder held finance roles of increasing responsibility at Genzyme Corporation including leading the internal audit function. Ms. Feder began her career at Deloitte & Touche LLP and she holds a Bachelor of Science in Accounting from Yeshiva University's Sy Syms School of Business.
- ***Achieved First Development Milestone Related to the Duvelisib License Agreement –*** In September 2017, upon achievement of positive top-line results from the Phase 3 DUO study, Verastem determined that the pre-specified criteria for the first milestone under the license agreement with Infinity Pharmaceuticals, Inc. (Infinity) had been met and recorded $6.0 million as research and development expense. Subsequently, in October 2017, Verastem made the milestone payment of $6.0 million to Infinity. The milestone was paid using funds drawn from Verastem's existing loan and security agreement with Hercules Capital, Inc.

Third Quarter 2017 Financial Results

Net loss for the three months ended September 30, 2017 (2017 Quarter) was $23.1 million, or $0.61 per share, as compared to a net loss of $7.9 million, or $0.21 per share, for the three months ended September 30, 2016 (2016 Quarter). Net loss includes non-cash stock-based compensation expense of $1.7 million and $1.3 million for the 2017 Quarter and 2016 Quarter, respectively. Verastem used $11.8 million for operating activities during the 2017 Quarter.

Research and development expense for the 2017 Quarter was $17.7 million compared to $4.2 million for the 2016 Quarter. The $13.5 million increase from the 2016 Quarter to the 2017 Quarter was primarily related to the achievement of a $6.0 million milestone pursuant to Verastem's license agreement with Infinity, an increase of $4.8 million in contract research organization (CRO) expense for outsourced biology, development and clinical services, which includes Verastem's clinical trial costs, an increase of approximately $2.0 million in consulting fees, an increase in stock-based compensation of approximately $423,000 and an increase in personnel related costs of approximately $153,000.

General and administrative expense for the 2017 Quarter was $5.4 million compared to $3.8 million for the 2016 Quarter. The increase of $1.6 million from the 2016 Quarter to the 2017 Quarter primarily resulted from increases in consulting and professional fees of $1.3 million and personnel costs of approximately $330,000.

As of September 30, 2017, Verastem had cash, cash equivalents and investments of $60.3 million compared to $80.9 million as of December 31, 2016.

The number of outstanding common shares as of September 30, 2017, was 39,945,028.

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 the second half of 2018.

About Duvelisib

Duvelisib is an 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-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 chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (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 is preparing to submit a New Drug Application to the U.S. Food and Drug Administration for the treatment of patients with relapsed or refractory CLL/SLL and patients with follicular lymphoma (FL) whose disease has progressed and are refractory to rituximab and to either chemotherapy or radioimmunotherapy. 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 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, 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 PI3K-delta and PI3K-gamma, which has successfully met its primary endpoint in a Phase 2 study in iNHL and a Phase 3 clinical trial in patients with CLL/SLL. 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, 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 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 the full data from the DUO study will not be consistent with the top-line results of the study; 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 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 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 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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[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, 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.
Condensed Consolidated Balance Sheets

(in thousands)

	September 30, 2017 (unaudited)	December 31, 2016
Cash, cash equivalents and investments	$ 60,264	$ 80,897
Prepaid expenses and other current assets	940	398
Property and equipment, net	989	1,417
Other assets	946	917
Total assets	**$ 63,139**	**$ 83,629**
Accounts payable and accrued expenses	$ 19,618	$ 10,991
Long-term debt	2,335	—
Other liabilities	201	341
Stockholders' equity	40,985	72,297
Total liabilities and stockholders' equity	**$ 63,139**	**$ 83,629**

Verastem, Inc.
Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2017	2016	2017	2016
Operating expenses:				
Research and development	$ 17,743	$ 4,216	$ 35,170	$ 12,887
General and administrative	5,394	3,843	14,582	12,315
Total operating expenses	23,137	8,059	49,752	25,202
Loss from operations	(23,137)	(8,059)	(49,752)	(25,202)
Interest income	121	137	416	417
Interest expense	(110)	—	(231)	—

Net loss	**$ (23,126)**	**$ (7,922)**	**$ (49,567)**	**$ (24,785)**
Net loss per share—basic and diluted	**$ (0.61)**	**$ (0.21)**	**$ (1.33)**	**$ (0.67)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**37,630**	**36,992**	**37,207**	**36,986**

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Verastem, Inc.
Brian Sullivan, 781-292-4214
Director, Corporate Development
bsullivan@verastem.com