Verastem Oncology Reports Second Quarter 2018 Financial Results

August 8, 2018

BOSTON--(BUSINESS WIRE)--Aug. 8, 2018-- Verastem, Inc. (Nasdaq: VSTM) (Verastem Oncology or the Company), focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients, today reported financial results for the quarter ended June 30, 2018 and provided an overview of certain corporate developments.

"During the second quarter of 2018, we've been actively preparing for the commercialization of duvelisib, our first-in-class, oral dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma for the treatment of patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) or follicular lymphoma (FL)," said Robert Forrester, President and Chief Executive Officer of Verastem Oncology. "In advance of our target action date of October 5, 2018, we have been building our U.S. sales force and commercial capabilities in preparation for a potential product launch of duvelisib in the U.S. in 2018. On the financial front, we have significantly strengthened our balance sheet, ending June 30, 2018 with $168.7 million in cash and cash equivalents."

Second Quarter 2018 and Recent Highlights:

Corporate and Financial

- ***Duvelisib NDA accepted by FDA with priority review*** – In April 2018, Verastem Oncology announced that the U.S. Food and Drug Administration (FDA) accepted the duvelisib New Drug Application (NDA) for filing with Priority Review, with a target action date of October 5, 2018. In the accepted NDA, the Company is seeking full approval for duvelisib, its first-in-class investigational oral dual inhibitor of 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 duvelisib NDA is supported by clinical data from the randomized Phase 3 DUO™ study evaluating duvelisib as a monotherapy in patients with relapsed or refractory CLL/SLL, as well as the Phase 2 DYNAMO™ study evaluating patients with iNHL that are double-refractory to both rituximab and chemotherapy or radioimmunotherapy. Both DUO and DYNAMO achieved their primary endpoints.
- ***Hosted Analyst and Investor Day highlighting commercial potential of duvelisib –*** In early May 2018, Verastem Oncology hosted an Analyst and Investor Day in New York City titled, "Duvelisib: Harnessing the Power of Dual PI3K Inhibition." Key opinion leaders in the hematologic oncology field including Dr. Lori Kunkel, Former Chief Medical Officer, Pharmacyclics, Dr. Jennifer Brown, Dana-Farber Cancer Institute, Dr. Ian Flinn, Sarah Cannon Research Institute, Dr. Steven Horwitz, Memorial Sloan Kettering Cancer Center as well as Dr. Brian Koffman, Founder & Medical Director of the Chronic Lymphocytic Leukemia (CLL) Society, and a CLL patient, joined the Verastem Oncology executive leadership team for an in-depth discussion regarding the unmet need among CLL/SLL and FL patients, where PI3K-delta and PI3K-gamma inhibitors fit into the treatment paradigm, and the growing opportunity for duvelisib in CLL/SLL and FL, and beyond. The Company also provided an overview of its duvelisib commercial strategy and initiatives. The webcast is available within the "Media" section of the Company's website at www.verastem.com.
- ***Signed exclusive license agreement with Yakult Honsha Co., Ltd. (Yakult) to develop and commercialize duvelisib in Japan –*** In June 2018, Verastem Oncology announced its entry into an exclusive license and collaboration agreement with Yakult to develop and commercialize duvelisib for the treatment, prevention or diagnosis of all oncology indications in Japan. The transaction, which carries a total deal value of up to $100.0 million, includes a one-time upfront payment of $10.0 million and up to an additional $90.0 million if certain future pre-specified development, regulatory and commercial milestones are successfully achieved by Yakult. In addition, Verastem Oncology is also eligible to receive double-digit royalties based on future net sales of duvelisib in Japan. Pursuant to the agreement, Yakult has the right to develop and commercialize duvelisib in Japan at its own cost and expense. In addition, Yakult may fund certain global development costs on a pro-rata basis. Verastem Oncology retains all rights to duvelisib outside of Japan.
- ***Strengthened the balance sheet through the sale of equity for net proceeds of approximately $105 Million*** – In May 2018, Verastem Oncology completed an underwritten registered offering of 8,944,444 shares of its common stock at a price to the public of $4.50 per share. The net proceeds to Verastem from the offering were approximately $38.3 million. In June 2018, Verastem Oncology completed a registered offering of 7,166,666 shares of its common stock at a price of $6.00 per share to funds managed by Consonance Capital. The net proceeds to Verastem Oncology from this offering were approximately $42.8 million. The Company also sold 6,314,410 shares of common stock under its at-the-market equity offering program for net proceeds of approximately $23.7 million.
- ***Joined the Russell 3000® Index –*** In June 2018, the Company joined the broad-market Russell 3000® Index as part of the Russell US Indexes annual reconstitution.

Scientific Presentations at Major Medical Meetings

Duvelisib

- ***The efficacy of duvelisib monotherapy following disease progression on ofatumumab monotherapy in patients with relapsed/refractory CLL or SLL in the DUO™ crossover extension study*** – In June 2018, at both the American Society of Clinical Oncology 2018 Annual Meeting (ASCO 2018) and the European Hematology Association 2018 Annual Meeting (EHA 2018), Dr. Byrone Kuss, Flinders Medical Centre, and Dr. Peter Hillman, St. James University Hospital, respectively, presented additional data from the open-label, DUO crossover extension study where patients with radiologically confirmed progressive disease (PD) following treatment with ofatumumab in DUO were given the option to receive treatment with duvelisib. Among the 89 evaluable patients (median 3 prior therapies; range 2-8), duvelisib as a monotherapy achieved a 73% overall response rate (ORR) per investigators assessment in the extension study (95% confidence interval CI: 64,82); 5% complete response with incomplete marrow recovery (CRi), and 68% partial response (PR). The median progression-free survival (mPFS) for duvelisib in the DUO crossover extension study was 15 months (95% CI: 10,17). Notably, 83% of patients in the duvelisib arm post-crossover had >50% reductions in the size of their target nodal lesions. The safety profile of duvelisib as a monotherapy was manageable and consistent with what was observed in the Phase 3 DUO™ study. These data build upon the previously reported positive DUO results and further support duvelisib as an effective oral monotherapy treatment option for patients with relapsed or refractory CLL/SLL.
- ***A Phase IB/II study of duvelisib in combination with Fludarabine (F), Cyclophosphamide (C), and Rituximab (R) (dFCR) for frontline therapy of younger CLL patients*** – At EHA 2018, Dr. Matthew Davids, Dana-Farber Cancer Institute, presented data on the 31 patients evaluable for post-dFCR response. The ORR was 94%, with 26% (n=8) of patients achieving a complete response (CR) or CRi, and 68% achieving a PR. The best rate of minimum residual disease (MRD) negativity in the bone marrow (BM) in patients with at least one evaluation was 76% (22 of 29 patients). All patients who achieved CR/CRi at the primary endpoint also had BM-MRD negativity (26%). Among survivors, the median follow-up is 24.5 months (range 6.9-46 months). The two-year progression-free survival and overall survival rates for patients in the study were both 97%. Eight patients have now completed two years of duvelisib maintenance therapy. Based on these results the recommended Phase 2 dose of duvelisib in combination with FCR was 25mg twice daily. The most common all grade non-hematologic adverse events (AEs) were nausea (72%, all Grade 1/2), fatigue (69%, 3% Grade 3), fever (53%, all Grade 1/2), diarrhea (47%, 3% Grade 3), transaminitis (34%, 28% Grade 3/4), anorexia (34%, all Grade 1/2), vomiting (28%, all Grade 1/2), pruritus (16%, 3% Grade 3), arthritis (9%, all Grade 2) and Cytomegalovirus (CMV) reactivation (6%, both Grade 2). The most common all grade hematologic adverse events were thrombocytopenia (65%; 34% Grade 3-4), neutropenia (59%; 50% Grade 3-4), and anemia (38%, 16% Grade 3). Serious AEs included transaminitis (Grade ≥3), febrile neutropenia (n=6, all Grade 3), pneumonia (n=6, including 3 cases of PJP despite planned prophylaxis), and colitis (n=1 Grade 2, n=1 Grade 3). These results suggest that duvelisib in combination with FCR is an effective regimen for the initial therapy of younger, fit CLL patients and results in a high rate of BM-MRD negativity (76%), significantly higher than historical data with FCR.
- ***The effect of duvelisib, a dual inhibitor of PI3K-δ,γ, on components of the tumor microenvironment in previously untreated follicular lymphoma patients*** – At both ASCO 2018 and EHA 2018, Dr. Carla Casulo, University of Rochester, Wilmot Cancer Center, presented data from blood samples from healthy volunteers and FL patients treated in the CONTEMPO study. Samples collected both pre- and post-duvelisib treatment were analyzed. Ex vivo and in vitro PI3K-delta assays and PI3K-gamma assays, with PI3K-gamma-selective (idelalisib, TGR-1202, IPI-3063) and PI3K-delta-selective (IPI-549) inhibitors were compared. Collectively, the results of this analysis support the thesis that duvelisib disrupts PI3K-delta and PI3K-gamma function in FL patients, inhibiting the tumor microenvironment (TME) cancer-supportive macrophages and T-cells.
- ***Duvelisib inhibition of chemokines in patients with CLL (DUO™) and iNHL (DYNAMO™)*** – At both ASCO 2018 and EHA 2018, Dr. David Weaver, Verastem Oncology's Vice President, Translational Medicine, presented data showing that PI3K-delta inhibition directly targets proliferation and survival of malignant leukemia and lymphoma cells, while PI3K-gamma inhibition modulates the TME through key support cells, including tumor-associated macrophages, nurse-like stroma and T-cells, and via soluble factors stimulating tumor growth, survival and migration. Serum samples from patients in the Phase 3 DUO study in relapsed/refractory CLL/SLL and the Phase 2 DYNAMO study in relapsed/refractory indolent non-Hodgkin lymphoma (iNHL) were collected at baseline and at infusion cycle date C2D1 and used for correlative studies of 24 chemokines, cytokines and serum factors. These data support the hypothesis that treatment with duvelisib results in significant reduction of chemokines potentially derived from the tumor cells and TME and that further investigation of the effects of duvelisib on TME pharmacodynamic markers is warranted.
- ***Presented scientific data supporting immuno-oncology applications of duvelisib at the 3rd annual Advances in Immuno-Oncology Congress*** – In May 2018, Jonathan Pachter, Ph.D., Verastem Oncology's Chief Scientific Officer, gave an oral presentation highlighting the unique potential of duvelisib, as a dual inhibitor of PI3K-delta and PI3K-gamma, to enhance the efficacy of immune checkpoint and co-stimulatory antibodies in preclinical models of both hematological malignancies and solid tumors. Dr. Pachter also moderated a round table discussion regarding novel checkpoint pathways and emerging strategies for combined modality treatment.

Defactinib

- ***Presented preliminary Phase 1 results from combination trial with defactinib, pembrolizumab and gemcitabine in advanced cancer –*** At ASCO 2018, Dr. Andrea Wang-Gillam presented a poster describing results from the ongoing Phase 1 study evaluating defactinib in combination with pembrolizumab and gemcitabine in patients with advanced cancer, including pancreatic cancer. The combination treatment appears to be well tolerated, the recommended Phase 2 dose was established, and the expansion phase of the study is now ongoing. Encouraging signs of clinical activity were observed in three pancreatic ductal adenocarcinoma (PDAC) patients treated beyond 250 days, including one patient with confirmed PR and two patients with stable disease. Meaningful reductions (57-96%) in the pancreatic cancer marker CA19-9 were also observed in all three patients. In addition, analysis of paired biopsies showed that the combination treatment induced desirable biomarker changes including increased proliferating CD8+ T-cells and reduced immunosuppressive Tregs and macrophages.
- ***Presented scientific data supporting immuno-oncology applications of defactinib at the 3rd Annual Advances in Immuno-Oncology Congress –*** During Dr. Pachter's oral presentation, he also provided an update on the scientific rationale and clinical progress of Verastem Oncology's lead focal adhesion kinase (FAK) inhibitor, defactinib, in combination with PD-1 and PD-L1 inhibitors in solid tumors.

All posters and presentations are available within the "Media" section of the Company's website at www.verastem.com.

Second Quarter 2018 Financial Results

Net loss for the three months ended June 30, 2018 (2018 Quarter) was $18.4 million, or $0.30 per share, as compared to a net loss of $13.4 million, or $0.36 per share, for the three months ended June 30, 2017 (2017 Quarter). Net loss for the 2018 Quarter includes license revenue of $10.0 million, related to the upfront payment received in connection with the license and collaboration agreement with Yakult in June 2018. Cash used in operating activities, excluding the upfront payment from Yakult, was $20.3 million for the 2018 Quarter.

Research and development expense for the 2018 Quarter was $12.4 million compared to $9.0 million for the 2017 Quarter. The $3.4 million increase from the 2017 Quarter to the 2018 Quarter was primarily related to an increase of $1.6 million in contract research organization expense for outsourced biology, development and clinical services, which includes the Company's clinical trial costs, an increase of $1.0 million in personnel related costs, and an increase of $0.4 million in stock-based compensation expense.

General and administrative expense for the 2018 Quarter was $15.8 million compared to $4.4 million for the 2017 Quarter. The increase of $11.4 million from the 2017 Quarter to the 2018 Quarter primarily resulted from increases in consulting and professional fees of $5.2 million, including $3.6 million related to commercial launch preparation activities, and an increase in personnel related costs of $4.4 million.

As of June 30, 2018, Verastem Oncology had cash and cash equivalents of $168.7 million compared to $86.7 million of cash, cash equivalents and investments as of December 31, 2017.

The number of outstanding common shares as of June 30, 2018 was 73,579,699.

Financial Guidance

Based on the Company's current operating plans, assuming a favorable regulatory decision and estimated revenue, it expects to have sufficient cash and cash equivalents to fund operations into 2020.

About Duvelisib

Duvelisib is a first-in-class investigational oral, 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. Verastem Oncology's 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) was accepted for filing by the U.S. Food and Drug Administration (FDA), granted Priority Review and assigned a target action date of October 5, 2018. Duvelisib is also being developed by Verastem Oncology 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 Oncology

Verastem, Inc. (Nasdaq:VSTM), operating as Verastem Oncology, is a biopharmaceutical company focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients. Verastem Oncology 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 and a Phase 3 clinical trial in patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL). Verastem Oncology's 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) was accepted for filing by the U.S. Food and Drug Administration, granted Priority Review and assigned a target action date of October 5, 2018. In addition, Verastem Oncology is developing the focal adhesion kinase (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 Oncology'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.

Forward-looking statements notice:

This press release includes forward-looking statements about Verastem Oncology's strategy, future plans and prospects, including statements regarding Verastem Oncology's future financial position, objectives of management, the development and activity of Verastem Oncology's investigational product candidates, including duvelisib and defactinib, and Verastem Oncology's PI3K and FAK programs generally, the structure of its planned and pending clinical trials, Verastem Oncology's financial guidance and the timeline and indications for clinical development, regulatory submissions and commercialization activities. 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 approval of Verastem Oncology's New Drug Application for duvelisib will not occur on the expected timeframe or at all, including by the U.S. Food and Drug Administration's target action date; 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 Oncology'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 a filing of a European Marketing Authorization Application may not be achieved; 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 Verastem Oncology's product candidates is uncertain; that there may be competitive developments affecting its product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that Verastem Oncology's 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 Oncology will be unable to successfully initiate or complete the clinical development and eventual commercialization of its product candidates; that the development and commercialization of Verastem Oncology's product candidates will take longer or cost more than planned; that Verastem Oncology may not have sufficient cash to fund its contemplated operations; that Verastem Oncology or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreement; that Verastem Oncology 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 Oncology will not pursue or submit regulatory filings for its product candidates, including for duvelisib in patients with CLL/SLL or iNHL; and that Verastem Oncology'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 the Company's Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2018, its Annual Report on Form 10-K for the year ended December 31, 2017 as filed with the Securities and Exchange Commission (SEC) on March 13, 2018 and in any subsequent filings with the SEC. The forward-looking statements contained in this press release reflect Verastem Oncology's views as of the date hereof, and the Company does not assume and specifically disclaims any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by law.

References

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[4] www.clinicaltrials.gov, NCT02004522

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

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[10] www.clinicaltrials.gov, NCT02546531

[11] www.clinicaltrials.gov, NCT02943317

[12] www.clinicaltrials.gov, NCT02758587

Verastem, Inc.

Condensed Consolidated Balance Sheets

(in thousands)

	June 30, 2018 (unaudited)	December 31, 2017
Cash, cash equivalents and investments	$ 168,692	$ 86,672
Prepaid expenses and other current assets	1,745	1,115
Property and equipment, net	1,270	861
Other assets	1,211	1,143
Total assets	**$ 172,918**	**$ 89,791**
Accounts payable, accrued expenses and other current liabilities	$ 22,132	$ 17,128
Long-term debt	23,520	14,828
Other liabilities	399	151
Stockholders' equity	126,867	57,684
Total liabilities and stockholders' equity	**$ 172,918**	**$ 89,791**

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended June 30,		Six months ended June 30,	
	2018	2017	2018	2017
Revenue:				
License revenue	$ 10,000	$ —	$ 10,000	$ —
Total revenue	10,000	—	10,000	—
Operating expenses:				
Research and development	12,381	9,042	23,315	17,427
General and administrative	15,813	4,425	25,640	9,188
Total operating expenses	28,194	13,467	48,955	26,615
Loss from operations	(18,194)	(13,467)	(38,955)	(26,615)
Interest income	343	140	534	295
Interest expense	(516)	(109)	(996)	(121)
Net loss	**$ (18,367)**	**$ (13,436)**	**$ (39,417)**	**$ (26,441)**
Net loss per share—basic and diluted	**$ (0.30)**	**$ (0.36)**	**$ (0.70)**	**$ (0.71)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**61,256**	**36,992**	**56,074**	**36,992**

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