Verastem Announces Regulatory Strategy for Duvelisib New Drug Application Following Guidance from FDA

October 31, 2017

Company Plans to Submit a Single NDA Requesting Full Approval of Duvelisib for the Treatment of Patients with Relapsed or Refractory Chronic Lymphocytic Leukemia/Small Lymphocytic Lymphoma and Accelerated Approval for the Treatment of Patients with Relapsed or Refractory Follicular Lymphoma

Company Plans to Submit NDA for Duvelisib During First Quarter of 2018

BOSTON--(BUSINESS WIRE)--Oct. 31, 2017-- Verastem, Inc. (NASDAQ: VSTM), focused on discovering and developing drugs to improve the survival and quality of life of patients with cancer, today announced that a meeting was held with the U.S. Food and Drug Administration (FDA) regarding the regulatory path for duvelisib, the Company's first-in-class, oral, monotherapy, dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma, which is being developed for the treatment of patients with lymphoid malignancies. 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 chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL), and accelerated approval for the treatment of patients with relapsed or refractory follicular lymphoma (FL). The Company expects to submit the duvelisib NDA during the first quarter of 2018.

"With FDA guidance now in hand, we have a defined regulatory path forward for the duvelisib NDA," said Robert Forrester, President and Chief Executive Officer of Verastem. "Obtaining the FDA's guidance for this submission represents an important milestone for Verastem and for duvelisib as a potential new treatment for patients with relapsed or refractory CLL/SLL and patients with relapsed or refractory FL. Our near-term focus will be on preparing the NDA, which we expect to submit during the first quarter of next year."

In September 2017, Verastem reported that the Phase 3 DUO™ study met its primary endpoint with oral duvelisib monotherapy demonstrating superiority over ofatumumab for progression free survival (PFS) in patients with relapsed or refractory 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 (HR) of 0.52 ($p<0.0001$), representing a 48% reduction in the risk of progression or death. 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%.

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-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 extension 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 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 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 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 indolent non-Hodgkin lymphoma (iNHL) and a Phase 3 clinical trial in patients with chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (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

[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

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