



Verastem Pays Milestone Payment to Infinity Pharmaceuticals

October 17, 2017

BOSTON, Mass.--(BUSINESS WIRE)--Oct. 17, 2017-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to improve the survival and quality of life of cancer patients, today announced payment of a \$6 million milestone to Infinity Pharmaceuticals, Inc., representing the first milestone under the duvelisib license agreement between Verastem and Infinity. 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 chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL).

In addition, Verastem drew an additional advance of \$7.5 million from its existing \$25 million loan and security agreement, dated March 21 2017, with Hercules Capital, Inc. (the Term Loan Facility). The proceeds will be used to pay the \$6 million milestone payment to Infinity, for ongoing research and development programs, and for general corporate purposes. Verastem has drawn a total of \$10 million under the Term Loan Facility, leaving \$15 million in available additional advances, subject to certain conditions of funding.

"Payment of this milestone to Infinity reflects the attainment of a critical milestone for the duvelisib development program, positive data from the Phase 3 DUO study in CLL/SLL," said Julie B. Feder, Chief Financial Officer of Verastem. "We have elected to employ the non-dilutive option of drawing a second tranche of funding under our Term Loan Facility. We believe this approach is a prudent use of the strategic financial tools that we have at hand as we advance the program towards a potential NDA filing in H1 2018."

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 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. Verastem plans to share these clinical data with the U.S. Food and Drug Administration (FDA) during Q4 2017 with the goal of filing a New Drug Application (NDA) with the FDA during the first half of 2018. The duvelisib NDA submission will also be supported by favorable results from the Phase 2 DYNAMO™ study in indolent non-Hodgkin's lymphoma (iNHL), which also achieved its primary endpoint with an ORR of 46% ($p < 0.0001$).

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 CLL/SLL⁴ and DYNAMO™, a single-arm, Phase 2 monotherapy study in patients with refractory iNHL that achieved its primary endpoint of ORR.⁵ Duvelisib is also being evaluated for the treatment of other hematologic malignancies, including T-cell lymphoma, 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 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. 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 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. will fail to fully

perform under the duvelisib license agreement; that Verastem will not pursue or submit regulatory filings for its product candidates; 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, NCT02783625, NCT02158091

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