Verastem to Present Long-Term Follow-Up Data from the DYNAMO™ Study at the 14th International Conference on Malignant Lymphoma

June 7, 2017

Duvelisib Monotherapy Treatment Demonstrates 47% ORR in Patients with Double-Refractory Indolent Non-Hodgkin Lymphoma

88% of Patients had a Reduction in the Size of Target Lymph Nodes

Duvelisib Remains Well-Tolerated in Long-Term Follow Up

BOSTON--(BUSINESS WIRE)--Jun. 7, 2017-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to improve the survival and quality of life of cancer patients, today announced long-term follow-up data from the DYNAMO™ study, which met its primary endpoint of Overall Response Rate (ORR; p=0.0001) at the final analysis, will be presented at the 14th International Conference on Malignant Lymphoma (ICML), being held June 14-17, 2017 in Lugano, Switzerland. DYNAMO is a Phase 2 clinical study evaluating the safety and efficacy of duvelisib monotherapy in patients with indolent non-Hodgkin lymphoma (iNHL) who were refractory to both rituximab and chemotherapy or radioimmunotherapy. Duvelisib is an investigational dual inhibitor of phosphoinositide-3-kinase (PI3K)-delta and PI3K-gamma that has demonstrated clinical activity as an oral monotherapy in multiple hematologic cancers, including chronic lymphocytic leukemia (CLL), iNHL, and T-cell lymphoma.

The long-term follow-up results from the study will be highlighted in an oral presentation titled, “DYNAMO: A Phase 2 Study Demonstrating the Clinical Activity of Duvelisib in Patients with Double-Refractory Indolent Non-Hodgkin Lymphoma,” given by Pier Luigi Zinzani, MD, PhD, University of Bologna Institute of Hematology, on Thursday, June 15, 2017 at 15:40 CET (9:40 ET) in Room A, Cinema Corso and Aula Magna (Lugano University).

“The data we are presenting at ICML help fill in the clinical picture for those patients who receive duvelisib over a longer period,” noted Dr. Zinzani. “Not only did duvelisib monotherapy continue to show robust and durable responses in double-refractory iNHL, but longer-term exposure to duvelisib did not reveal any unexpected safety findings. These results suggest duvelisib has favorable benefit-risk in double-refractory iNHL, and may provide an important new treatment option for a population in need of new targeted therapies.”

Patients enrolled in DYNAMO all had double-refractory iNHL and a median of 3 prior anticancer regimens. Of the 129 patients enrolled, 61 responded (1 complete response [CR], 60 partial responses [PR]), for an overall response rate (ORR) of 47%, as determined by an independent review committee. The ORR in each of the three disease subgroups included: 43% in follicular lymphoma (n=83); 68% in small lymphocytic lymphoma (n=28); and 33% in marginal zone lymphoma (n=18). Responses generally occurred shortly after the start of treatment (median 2 months). Notably, 88% of all patients treated with duvelisib had a reduction in the size of their target lymph nodes. Overall, median duration of response was 10 months, median progression-free survival was 9 months, and median overall survival was 27.8 months.

With additional follow-up (median 18 months), the safety profile of duvelisib monotherapy remains consistent with what has been previously reported in iNHL and other hematologic malignancies. The most common Grade ≥ 3 adverse events were hematologic in nature (neutropenia 30%, thrombocytopenia 15%, anemia 14%). Diarrhea was the most frequently reported nonhematologic adverse event (47%; 15% Grade ≥ 3). As expected in a heavily pretreated and refractory patient population, infections of all types and grades were observed (56%). Pneumonitis and colitis, events previously described with duvelisib, remained relatively uncommon (9% and 5%, respectively). Treatment discontinuations attributed to the most common adverse events were infrequent, suggesting that these events were generally manageable.

“The clinical activity and durability of responses observed in the DYNAMO study seen across a range of highly-refractory disease subtypes, together with the well-characterized and manageable safety profile, highlight the potential of this drug in lymphoid malignancies,” said Hagop Youssoufian, MSc, MD, Head of Hematology and Oncology Development at Verastem. “What I find really encouraging, is that we saw these results in patients refractory to both rituximab and chemotherapy, a specific population with unmet medical need.”

Following conclusion of Dr. Zinzani's presentation, a copy of the presentation will be available here.

More About the Phase 2 DYNAMO ™Study

DYNAMO™ is a Phase 2, single-arm study, which evaluated the efficacy and safety of duvelisib 25 mg twice daily as monotherapy in 129 iNHL patients, including follicular lymphoma (n=83), small lymphocytic lymphoma (n=28), and marginal zone lymphoma (n=18) whose disease has progressed and are refractory to rituximab and to either chemotherapy or radioimmunotherapy. The primary endpoint of the study was ORR as assessed by an independent review committee.

About the Tumor Microenvironment

The tumor microenvironment encompasses multiple tumor and non-tumor cell populations and an extracellular matrix that support cancer cell survival. This includes immunosuppressive regulatory T-cells, myeloid-derived suppressor cells, tumor-associated macrophages, cancer-associated fibroblasts, and extracellular matrix proteins that can hamper the entry and therapeutic benefit of cytotoxic T-cells and anti-cancer drugs. In addition to targeting the proliferative and survival signaling of cancer cells, Verastem’s product candidates, including duvelisib and defactinib, also target the tumor microenvironment to potentially improve response to therapy.

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[4] 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 hematologic malignancies through investigator-sponsored studies, including T-cell lymphoma.[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 is currently being evaluated in a Phase 3 clinical trial in patients with CLL. 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 our rights to develop or commercialize our product candidates. 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 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; 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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