Verastem Oncology Announces Presentation of Updated Duvelisib Combination Clinical Data in Peripheral T-Cell Lymphoma at the American Society of Hematology 2018 Annual Meeting

December 3, 2018

Combination of Duvelisib and Romidepsin Resulting in a 59% Overall Response Rate in Relapsed or Refractory Peripheral T-Cell Lymphoma

BOSTON--(BUSINESS WIRE)--Dec. 3, 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 announced an oral presentation highlighting new clinical data from an investigator sponsored trial, led by Steven Horwitz, MD, Memorial Sloan Kettering Cancer Center (MSK), evaluating duvelisib in combination with romidepsin in patients with relapsed or refractory T-cell lymphomas, including peripheral T-cell lymphoma (PTCL) and cutaneous T-cell lymphoma (CTCL) at the American Society of Hematology (ASH) 2018 Annual Meeting, taking place December 1-4, 2018, in San Diego. Duvelisib is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma.

“The data demonstrate that the combination of oral duvelisib and romidepsin has an acceptable safety profile and the presence of early signals of anti-lymphoma activity in patients with PTCL,” said Steven Horwitz, MD, Memorial Sloan Kettering Cancer Center (MSK), co-principal investigator of the Phase 1 study, and lead author of the oral presentation. “The response rate observed to date from the combination is compelling considering that PTCL is an aggressive type of non-Hodgkin lymphoma for which new therapies are desperately needed. We look forward to further elucidating the potential of this novel combination regimen through completion of this expansion cohort.”

Phase 1 Results with Duvelisib and Romidepsin in Relapsed or Refractory PTCL

In this multicenter, dose-expansion portion of the Phase 1 trial, oral duvelisib was dosed at 75mg twice-daily (BID) on days 1-28. Romidepsin 10mg/m^2 was dosed on Days 1, 8, and 15 on a 28-day cycle. Of the 38 patients evaluable for efficacy (PTCL, n=27; CTCL, n=11), 21 responded (9 complete responses (CRs) and 12 partial responses (PRs)) for an overall response rate (ORR) of 55%. Sixteen of the 27 patients with PTCL responded (9 CRs and 7 PRs) for an ORR of 59%. Five of the 11 patients with CTCL responded (all PRs) for an ORR of 45%. Median progression-free survival (PFS) for patients with PTCL was 6.72 months and 5.41 months for patients with CTCL. Among the 39 patients evaluable for safety, the most common Grade ≥3 adverse events were neutropenia (33%), diarrhea (15%) and increased alanine aminotransferase (13%).

A PDF copy of the oral presentation will be available here following the conclusion of the session.

Verastem Oncology is currently conducting an open-label, multicenter, Phase 2 clinical trial (the PRIMO study; NCT03372057) evaluating the efficacy and safety of duvelisib monotherapy in adult patients with histologically confirmed relapsed or refractory PTCL. This study is expected to enroll approximately 120 patients.

In addition, Verastem Oncology’s PTCL program was recently selected to participate in The Leukemia and Lymphoma Society’s® Therapy Acceleration Program® which provides additional resources to support the development of therapies for patients with blood cancers. The program is supporting work on translational biomarkers of response, patient enrollment acceleration in PRIMO and an increase in the total patient enrollment in the combination study of duvelisib and romidepsin being presented at ASH.

Details for presentation are as follows:

Oral Presentation

Title: The combination of Duvelisib, a PI3K-δ,γ Inhibitor, and Romidepsin is highly active in relapsed/refractory peripheral T-cell lymphoma with low rates of transaminitis: Results of a multicenter, multi-arm phase 1 study with expansion cohorts
Presenter: Steven Horwitz, Memorial Sloan Kettering Cancer Center and NYC Health + Hospitals/Bellevue
Abstract Number/Publication ID: 683
Session: 624. Hodgkin Lymphoma and T/NK Cell Lymphoma—Clinical Studies: Immunotherapy and Targeted Strategies

About Peripheral T-Cell Lymphoma

Peripheral T-cell lymphoma (PTCL) is a rare, aggressive type of non-Hodgkin lymphoma (NHL) that develops in mature white blood cells called “T cells” and “natural killer (NK) cells” [1] which circulate with the lymphatic system.[2] PTCL accounts for between 10-15% of all non-Hodgkin lymphomas (NHLs) and generally affects people aged 60 years and older.[1] Although there are many different subtypes of peripheral T-cell lymphoma, they often present in a similar way, with widespread, enlarged, painless lymph nodes in the neck, armpit or groin.[2] There is currently no established standard of care for patients with relapsed or refractory disease.[1]

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a commercial biopharmaceutical company committed to the development and commercialization of medicines to improve the lives of patients diagnosed with cancer. We are driven by the strength, tenacity and courage of those battling cancer – single-minded in our resolve to deliver new therapies that not only keep cancer at bay, but improve the lives of patients diagnosed with cancer. Because for us, it’s personal.

Our first FDA approved product is now available for the treatment of patients with certain types of indolent non-Hodgkin’s lymphoma (iNHL). Our pipeline comprises product candidates that seek to treat cancer by modulating the local tumor microenvironment. 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 the development and activity of Verastem Oncology's lead product duvelisib, and Verastem Oncology's PI3K and FAK programs generally, its intent to commercialize duvelisib, the potential commercial success of duvelisib, the anticipated adoption of duvelisib by patients and physicians, the structure of its planned and pending clinical trials 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, among other things, uncertainties regarding the commercial success of duvelisib in the United States; uncertainties regarding physician and patient adoption of duvelisib, including those related to the safety and efficacy of duvelisib; the uncertainties inherent in research and development of duvelisib, such as negative or unexpected results of clinical trials; whether and when any applications for duvelisib may be filed with regulatory authorities in any other jurisdictions; whether and when regulatory authorities in any other jurisdictions may approve any such other applications that may be filed for duvelisib, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted and, if approved, whether duvelisib will be commercially successful in such jurisdictions; Verastem Oncology's ability to obtain, maintain and enforce patent and other intellectual property protection for duvelisib and its other product candidates; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of duvelisib; that regulatory authorities in the U.S. or other jurisdictions, if approved, could withdraw approval; whether preclinical testing of Verastem Oncology's product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for Verastem Oncology's product candidates is uncertain; the risk that third party payors (including government agencies) will not reimburse for duvelisib; 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 duvelisib or Verastem Oncology's other product candidates will cause unexpected safety events, experience manufacturing or supply interruptions or failures, or result in unmanageable safety profiles as compared to their levels 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 FL in other jurisdictions; 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 September 30, 2018 as filed with the Securities and Exchange Commission (SEC) on November 7, 2018, its Annual Report on Form 10-K for the year ended December 31, 2017 as filed with the 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

[1] The Leukemia & Lymphoma Society. Peripheral T-Cell Lymphoma Facts. July 2014.

[2] Leukemia Foundation. http://www.leukaemia.org.au/blood-cancers/lymphomas/non-hodgkin-lymphoma-nhl/peripheral-t-cell-lymphoma

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