Verastem Oncology Announces Collaboration with The Leukemia & Lymphoma Society to Accelerate Development of Duvelisib for the Treatment of Peripheral T-Cell Lymphoma

November 7, 2018

BOSTON, Mass.--(BUSINESS WIRE)--Nov. 7, 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 a collaboration with The Leukemia & Lymphoma Society® (LLS), to accelerate the development of duvelisib for the treatment of patients with peripheral T-cell lymphoma (PTCL), an aggressive type of non-Hodgkin lymphoma (NHL). Verastem Oncology's duvelisib was selected for the LLS's Therapy Acceleration Program® (TAP) which provides additional resources to support the development of therapies for patients with blood cancers. The Company plans to use the funds to conduct certain translational and clinical activities relating to the development of duvelisib for the treatment of PTCL. LLS and Verastem Oncology will share the cost of the development program, portions of which will be conducted in collaboration with Memorial Sloan Kettering Cancer Center, The Dana-Farber Cancer Institute, The Washington University in St. Louis and Stanford University.

"The selection of duvelisib for a TAP collaboration with the LLS underscores the potential of this innovative, oral monotherapy for patients with relapsed or refractory PTCL," said Robert Forrester, President and Chief Executive Officer of Verastem Oncology. "This collaboration provides important funding to accelerate the advancement of duvelisib as a potential new treatment for patients battling PTCL, either as a monotherapy or in combination with other anti-cancer agents. We look forward to collaborating with the LLS team on the exciting work ahead in order to advance duvelisib through the clinic and ultimately to the patients and families suffering from this devastating cancer."

Duvelisib is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, that has demonstrated clinical activity in a Phase 1 clinical trial in 16 heavily pre-treated patients with relapsed or refractory PTCL (Horwitz, et al. *Blood.* Feb 2018). Results from the study showed duvelisib demonstrated a 50% overall response rate, including 19% complete responses.

Verastem Oncology is currently conducting an open-label, multicenter, Phase 2 clinical trial (the PRIMO study) 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, funds from the TAP will be used to support the expansion of an investigator-sponsored study being conducted by Steven Horwitz, MD, Medical Oncologist, Memorial Sloan Kettering Cancer Center and NYC Health + Hospitals/Bellevue. This study is evaluating the combination of duvelisib with romidepsin, an HDAC inhibitor, with a goal to create deeper and more durable responses for patients with relapsed or refractory PTCL. Initial data on the combination as presented at the American Society of Hematology (ASH) 2017 Annual Meeting, demonstrated a 64% overall response rate including 34% complete responses.

"The mission of our TAP program is to identify and fund the most promising investigational therapies that have the potential to change the standard of care for patients with blood cancers, with a particular focus on areas of the highest unmet medical need, such as relapsed or refractory PTCL," Lee Greenberger, PhD, Chief Scientific Officer of LLS, commented. "Our partnership with Verastem Oncology is an excellent fit with our key initiative to accelerate development of new and cutting edge therapies like duvelisib, which specifically inhibits only certain PI3K family members, that have shown promising early clinical results in PTCL."

** Duvelisib's use in patients suffering from PTCL is solely investigational in nature; its use has not been deemed safe and effective by the U.S. Food and Drug Administration.*

About the Therapy Acceleration Program®

The Leukemia & Lymphoma Society's Therapy Acceleration Program® (TAP) identifies and funds innovative projects related to therapies, supportive care or diagnostics that have the potential to change the standard of care for patients with blood cancer, especially in areas of high unmet medical need. TAP funding assists both clinical investigators and companies in gaining critical clinical proof of concept data that better enables them to obtain the resources they need or a partner to complete the testing, registration and marketing of new treatments, supportive care and diagnostics for leukemia, lymphoma and myeloma. TAP funding is different from the traditional grant at LLS. The TAP review process is separate from the grant process and each approved project is closely monitored by TAP staff. To learn more about how TAP works, please click here.

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.

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

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 COPIKTRA, and Verastem Oncology's PI3K and FAK programs generally, its intent to commercialize COPIKTRA, the potential commercial success of COPIKTRA, the anticipated adoption of COPIKTRA 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 launch timing and commercial success of COPIKTRA in the United States; uncertainties regarding physician and patient adoption of COPIKTRA, including those related to the safety and efficacy of COPIKTRA; the uncertainties inherent in research and development of COPIKTRA, such as negative or unexpected results of clinical trials; whether and when any applications for COPIKTRA 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 COPIKTRA, 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 COPIKTRA will be commercially successful in such jurisdictions; Verastem Oncology's ability to obtain, maintain and enforce patent and other intellectual property protection for COPIKTRA 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 COPIKTRA; 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 COPIKTRA; 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 COPIKTRA 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 COPIKTRA 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 June 30, 2018 as filed with the Securities and Exchange Commission (SEC) on August 8, 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.

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