Verastem Oncology Announces COPIKTRA™ (Duvelisib) Presentations at the 18th Annual International Workshop on Chronic Lymphocytic Leukemia

September 20, 2019

BOSTON--(BUSINESS WIRE)--Sep. 20, 2019-- Verastem, Inc. (Nasdaq:VSTM) (Verastem Oncology or the Company), a biopharmaceutical company focused on developing and commercializing medicines seeking to improve the survival and quality of life of cancer patients, today announced that three posters highlighting clinical data for COPIKTRA™ (duvelisib) will be presented at the 18th Annual International Workshop on Chronic Lymphocytic Leukemia taking place September 20-23, 2019, in Edinburgh, UK. The presented abstracts focus on clinical data from the Phase 3 DUO study including evaluation of COPIKTRA efficacy and safety in heavily pre-treated patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL), dose modification data and results from a post-hoc analysis evaluating the effect of COPIKTRA on lymphocytosis in patients, including those with high-risk factors.

“Many patients with CLL or SLL eventually relapse and are in need of additional therapy options to help control their disease,” said Marco Montillo, M.D., Head, Department of Hematology and Oncology, Niguarda Cancer Center, Niguarda Hospital, Milan, Italy. “In the DUO study, duvelisib monotherapy demonstrated significantly improved clinical outcomes in patients with relapsed refractory disease who had received two or more prior therapies. In the study, the safety profile of duvelisib was generally manageable through dose modifications or interruptions.”

“The data presented at iwCLL this year highlight findings from the Phase 3 DUO study in the labeled indication of relapsed or refractory CLL/SLL after at least 2 prior therapies,” commented Hagop Youssoufian, MSc, M.D., Head of Medical Strategy at Verastem Oncology. “Of note, response rates appeared to be preserved in COPIKTRA-treated patients whose adverse events, occurring on therapy, were effectively managed through dose interruptions or dose reductions. These findings from the study are an important factor in helping physicians navigate the management of adverse events in the clinical setting with the goal of not compromising efficacy.”

COPIKTRA, a targeted oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, received approval as monotherapy from the U.S. Food and Drug Administration (FDA) in September 2018 for the treatment of patients with relapsed or refractory CLL/SLL after at least two prior therapies. COPIKTRA also received accelerated approval for the treatment of adult patients with relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. Continued approval in FL may be contingent upon verification and description of clinical benefit in confirmatory trials.

Details for the iwCLL 2019 poster presentations are as follows:

Title: An improved benefit-risk profile of duvelisib in patients with chronic lymphocytic leukemia or small lymphocytic lymphoma who received ≥2 prior therapies

Lead author: Marco Montillo, Niguarda Cancer Center

Presentation ID: 1914

Date and time: Sunday, September 22, 2019; all day

Title: Effect of dose modifications on response to duvelisib in patients with relapsed or refractory CLL/SLL in the DUO trial

Lead author: Paolo Ghia, Università Vita-Salute San Raffaele and IRCCS Ospedale San Raffaele

Presentation ID: 2034

Date and time: Sunday, September 22, 2019; all day

Title: Patterns of duvelisib-induced lymphocytosis in patients with relapsed/refractory chronic lymphocytic leukemia/small lymphocytic lymphoma, including those with high-risk factors treated in the DUO trial

Lead author: Jacqueline Barrientos, Zucker School of Medicine at Hofstra/Northwell

Presentation ID: 2033

Date and time: Sunday, September 22, 2019; all day

PDF copies of these poster presentations will be available here after the meeting.

SELECT IMPORTANT SAFETY INFORMATION

This does not include all information needed to use COPIKTRA™ (duvelisib) safely and effectively. See full Prescribing Information.

WARNING: FATAL AND SERIOUS TOXICITIES: INFECTIONS, DIARRHEA OR COLITIS, CUTANEOUS REACTIONS, and PNEUMONITIS

See full Prescribing Information for complete boxed warning

- **Fatal and/or serious infections occurred in 31% of COPIKTRA-treated patients. Monitor for signs and symptoms of infection. Withhold COPIKTRA if infection is suspected.**
- **Fatal and/or serious diarrhea or colitis occurred in 18% of COPIKTRA-treated patients. Monitor for the development of severe diarrhea or colitis. Withhold COPIKTRA.**
- **Fatal and/or serious cutaneous reactions occurred in 5% of COPIKTRA-treated patients. Withhold COPIKTRA.**
- **Fatal and/or serious pneumonitis occurred in 5% of COPIKTRA-treated patients. Monitor for pulmonary symptoms and interstitial infiltrates. Withhold COPIKTRA.**

WARNINGS AND PRECAUTIONS

- Hepatotoxicity: Monitor hepatic function.
- Neutropenia: Monitor blood counts.
- Embryo-Fetal toxicity: COPIKTRA can cause fetal harm. Advise patients of potential risk to a fetus and to use effective contraception.

ADVERSE REACTIONS

The most common adverse reactions (≥20%) are diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain, and anemia.

To report Adverse Reactions, contact FDA at 1-800-FDA-1088 (1-800-332-1088) or www.fda.gov/medwatch and Verastem Oncology at 1-877-7RXVSTM (1-877-779-8786).

DRUG INTERACTIONS

- CYP3A inducers: Avoid co-administration with strong CYP3A inducers.
- CYP3A inhibitors: Monitor for COPIKTRA toxicities when co-administered with strong or moderate CYP3A inhibitors. Reduce COPIKTRA dose to 15 mg twice daily when co-administered with strong CYP3A4 inhibitors.
- CYP3A substrates: Monitor for signs of toxicities when co-administering COPIKTRA with sensitive CYP3A substrates.

USE IN SPECIFIC POPULATIONS

Lactation: Advise women not to breastfeed.

Please see accompanying full Prescribing Information, including Boxed Warning.

About Chronic Lymphocytic Leukemia/Small Lymphocytic Lymphoma

Chronic lymphocytic leukemia (CLL) and small lymphocytic lymphoma (SLL) are cancers that affect lymphocytes and are essentially the same disease, with the only difference being the location where the cancer primarily occurs. When most of the cancer cells are located in the bloodstream and the bone marrow, the disease is referred to as CLL, although the lymph nodes and spleen are often involved. When the cancer cells are located mostly in the lymph nodes, the disease is called SLL. The symptoms of CLL/SLL include a tender, swollen abdomen and feeling full even after eating only a small amount. Other symptoms can include fatigue, shortness of breath, anemia, bruising easily, night sweats, weight loss, and frequent infections. However, many patients with CLL/SLL will live for years without symptoms. There are approximately 200,000 patients in the US affected by CLL/SLL with nearly 20,000 new diagnoses this year alone. While there are therapies currently available, real-world data reveals that a significant number of patients either relapse following treatment, become refractory to current agents, or are unable to tolerate treatment, representing a

significant medical need. The potential of additional oral agents, particularly as a monotherapy that can be used in the general community physician's armamentarium, may hold significant value in the treatment of patients with CLL/SLL.

About COPIKTRA™ (duvelisib)

COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-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] COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. COPIKTRA is also being developed by Verastem Oncology for the treatment of peripheral T-cell lymphoma (PTCL), for which it has received Fast Track status, and is being investigated in combination with other agents through investigator-sponsored studies.[4] For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

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, and financial results. 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 and uncertainties, among other things, regarding: the commercial success of COPIKTRA™ inthe United States; 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; our ability to obtain, maintain and enforce patent and other intellectual property protection for COPIKTRA and our 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; the fact that regulatory authorities in the U.S. or other jurisdictions, if approved, could withdraw approval; whether preclinical testing of our 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 our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse for COPIKTRA; 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 COPIKTRA or our 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 we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we may not have sufficient cash to fund our contemplated operations; that we, CSPC Pharmaceutical Group, Yakult Honsha Co., Ltd., Sanofi or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreements; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will not pursue or submit regulatory filings for our product candidates, including for duvelisib in patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) or indolent non-Hodgkin lymphoma (iNHL) in other jurisdictions; and that our 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, 2019, as filed with the Securities and Exchange Commission (SEC) on August 1, 2019, its Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the SEC on March 12, 2019 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 Winkler D.G., Faia K.L., DiNitto J.P. 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 K 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 M 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, NCT03372057.

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