Verastem Oncology Announces Dosing of First Patient in Yakult Honsha Co., Ltd.'s Japanese Bridging Study Evaluating COPIKTRA® (Duvelisib) in Patients with Relapsed or Refractory Chronic Lymphocytic Leukemia/Small Lymphocytic Lymphoma

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BOSTON--(BUSINESS WIRE)--Oct. 7, 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 its partner Yakult Honsha Co., Ltd. (Yakult) has dosed the first patient in a Phase 1b Japanese bridging study evaluating COPIKTRA® (duvelisib) in patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) following at least one prior therapy. COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma in the United States.

"The start of patient dosing in Yakult's first clinical study of COPIKTRA in Japan is another important step forward in Verastem Oncology's strategy to expand the global potential of COPIKTRA for hematological malignancies across the globe," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "The results of this study are expected to form the basis of a regulatory submission for COPIKTRA for the treatment of relapsed or refractory CLL/SLL in Japan, where therapies are extremely limited. We look forward to supporting Yakult through the clinical development of COPIKTRA in Japan to help rapidly advance this oral, novel therapy for patients living with CLL/SLL."

Verastem and Yakult entered into an exclusive licensing agreement in June 2018 for Yakult to develop and commercialize COPIKTRA for the treatment, prevention or diagnosis of all oncology indications in Japan. Yakult's Phase 1b, multicenter, open-label study is expected to enroll approximately 10 patients with relapsed or refractory CLL/SLL after at least one prior therapy. The primary endpoint of the study is objective response rate. Secondary endpoints of the study include overall survival, progression free survival and safety. This Phase 1b study is expected to serve as a bridging study based on the efficacy and safety observed in Verastem Oncology's Phase 3 DUO study. The results of the Phase 1b bridging study are expected to form the basis of a regulatory submission for COPIKTRA for the treatment of relapsed or refractory CLL/SLL in Japan.

COPIKTRA was approved in September 2018 by the U.S. Food and Drug Administration (FDA) for the treatment of adult patients with relapsed or refractory CLL/SLL after at least two prior therapies. In addition, COPIKTRA has been granted accelerated approval by the FDA for the treatment of adult patients with relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. Accelerated approval in FL was based on overall response rate and continued approval may be contingent upon verification and description of clinical benefit in confirmatory trials.

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.

SELECT IMPORTANT SAFETY INFORMATION

This does not include all information needed to use COPIKTRA™ (duvelisib) safety and effectivelySee 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.**

INDICATIONS AND USAGE

COPIKTRA is a kinase inhibitor indicated for the treatment of adult patients with:

- Relapsed or refractory chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL) after at least two prior therapies.
- Relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. Accelerated approval based on overall response rate and continued approval may be contingent upon confirmatory trials

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.

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 within the meaning of the Private Securities Litigation Reform Act of 1995 that are subject to risks, uncertainties and other factors, including the possibility of unfavorable results from clinical trials evaluating COPIKTRA (duvelisib) for the treatment CLL/SLL. Verastem and/or Yakult may also be unable to enroll patients in future studies in Japan and may need to modify or delay these studies or perform additional studies. Ultimately, Verastem and/or Yakult may fail to secure regulatory approval for COPIKTRA (duvelisib) in that region. These risks, uncertainties and other factors could cause actual results to differ materially from those referred to in the forward-looking statements. The reader is cautioned not to rely on these forward-looking statements.

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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