Verastem Oncology Receives Orphan Drug Designation from FDA for COPIKTRA for the Treatment of T-Cell Lymphoma

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BOSTON--(BUSINESS WIRE)--Oct. 3, 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 duvelisib (COPIKTRA™) has received orphan drug designation from the U.S. Food and Drug Administration for use in the treatment of T-Cell lymphoma. The designation was created to encourage the development of drugs that may provide significant benefit to patients suffering from rare diseases.

“Receiving orphan drug designation for T-Cell Lymphoma, in addition to the previously-granted Fast Track status, for Peripheral T-Cell lymphoma, marks another important regulatory milestone to bring COPIKTRA to patients who are faced with this aggressive type of disease with limited therapeutic options,” said Brian Stuglik, Chief Executive Officer of Verastem Oncology. “We look forward to sharing the results of our Phase 2 PRIMO study and efficiently advancing our development program in this indication.”

COPIKTRA is approved in the United States for the treatment of chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least 2 prior therapies and accelerated approval in follicular lymphoma (FL) after at least 2 prior systemic therapies. COPIKTRA is not currently approved for the treatment of T-cell lymphoma. The Company’s ongoing Phase 2 PRIMO study will provide guidance on a duvelisib monotherapy dosing regimen in patients with relapsed or refractory peripheral T-cell lymphoma (PTCL) and further characterize its efficacy and tolerability in this population.

In the U.S., under the Orphan Drug Act, the FDA’s Office of Orphan Products Development (OOPD) grants orphan drug status to a drug or biologic intended for the safe and effective treatment, diagnosis or prevention of rare diseases/disorders, which is generally a disease that affects fewer than 200,000 individuals in the U.S. and that are expected to provide a significant therapeutic advantage over existing treatments. Orphan designation qualifies a company for benefits that apply across all stages of drug development, including an accelerated approval process, seven years of market exclusivity following marketing approval, tax credits on U.S. clinical trials, eligibility for orphan drug grants, and a waiver of certain administrative fees.

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]

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% (4% fatal) 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% (<1% fatal) of COPIKTRA-treated patients. Monitor for the development of severe diarrhea or colitis. Withhold COPIKTRA.
- Fatal and/or serious cutaneous reactions occurred in 5% (<1% fatal) of COPIKTRA-treated patients. Withhold COPIKTRA.
- Fatal and/or serious pneumonitis occurred in 5% (<1% fatal) 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 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.[3,4,5] 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.[6] 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 within the meaning of the Private Securities Litigation Reform Act of 1995 that are subject to risks, uncertainties and other factors, 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; that enrollment of clinical trials may take longer than expected; 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;; and 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. 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] 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

[3] 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.

[4] 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.

[5] 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.

[6] www.clinicaltrials.gov, NCT03372057.

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