



Verastem Oncology Receives FDA Approval of COPIKTRA™ (duvelisib) Capsules

September 24, 2018

Company to host conference call today at 4:30pm ET

BOSTON--(BUSINESS WIRE)--Sep. 24, 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 that the U.S. Food and Drug Administration (FDA) has approved COPIKTRA™, an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma. COPIKTRA is approved for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (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. The indication in FL is approved under accelerated approval based on overall response rate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

"With today's FDA approval of COPIKTRA, Verastem Oncology is delivering upon our commitment to patients with cancer by bringing a new oral medicine to market," said Robert Forrester, President and Chief Executive Officer of Verastem Oncology. "We are pleased to be able to introduce COPIKTRA during National Blood Cancer Awareness Month. At Verastem Oncology, we are driven by the strength and courage of those battling cancer, and we are committed to advancing therapies such as COPIKTRA with the potential to make a significant impact for patients, their caregivers and physicians. We are immensely grateful to the many patients who participated in the duvelisib clinical trial program over the years leading to this pivotal moment."

"COPIKTRA is an important addition to the evolving treatment paradigm for patients with CLL/SLL and FL," said Ian Flinn, MD, PhD, Director of the Lymphoma Research Program at Sarah Cannon Research Institute and lead investigator of the DYNAMO™ and DUO™ studies. "The approval of COPIKTRA for the treatment of relapsed or refractory CLL/SLL after at least two prior therapies, or relapsed or refractory FL after at least two prior systemic therapies, is based on clinical trial data gathered from the treatment of over 400 adult patients. COPIKTRA is a significant addition to physicians' treatment armamentarium that I believe will address an unmet need for patients who have limited options once they have progressed after two prior therapies."

Use of COPIKTRA is associated with a **BOXED WARNING** for four fatal and/or serious toxicities: infections, diarrhea or colitis, cutaneous reactions, and pneumonitis. Verastem Oncology is implementing an informational Risk Evaluation and Mitigation Strategy to provide appropriate dosing and safety information to better support physicians in managing their patients on COPIKTRA.

Additionally, use of COPIKTRA is also associated with adverse reactions which may require dose reduction, treatment delay or discontinuation of COPIKTRA. WARNINGS AND PRECAUTIONS are provided for infections, diarrhea or colitis, cutaneous reactions, pneumonitis, hepatotoxicity, neutropenia, and embryo-fetal toxicity. The most common ADVERSE REACTIONS (reported in ≥ 20% of patients) were diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain, and anemia.

Please see www.COPIKTRAHCP.com/prescribinginformation for full Prescribing Information including **BOXED WARNING** and Medication Guide in addition to the full Important Safety Information provided below.

Lymphoma is the most common blood cancer, and CLL/SLL and FL are common types of indolent non-Hodgkin lymphomas (iNHL). There are an estimated 681,000 people living with non-Hodgkin lymphoma in the US alone, including nearly 350,000 cases of CLL/SLL or FL.¹ Many of these patients will eventually relapse or develop refractory disease.

"Each lymphoma patient's experience is unique, and today's approval offers patients with CLL/SLL and FL a new treatment option and a new opportunity for hope," said Meghan Gutierrez, Chief Executive Officer of the Lymphoma Research Foundation. "We applaud the FDA, Verastem and the patients who participated in the clinical trials for their roles in advancing treatment options for people living with CLL/SLL and FL."

The New Drug Application for COPIKTRA received Priority Review. Priority Review is reserved for medicines that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications. The FDA had previously granted COPIKTRA Fast Track Designation in CLL and FL as well as Orphan Drug Designation for CLL/SLL and FL. For the indication of FL, COPIKTRA was approved under FDA regulations for accelerated approval.

"We are excited to offer a new treatment that can allow patients to manage their disease with an oral monotherapy," said Joseph Lobjack, Executive Vice President and Chief Commercial Officer of Verastem Oncology. "We continue to hear from physicians and patients that there is a great need for additional treatment options to fight chronic cancers such as CLL/SLL and FL. In preparation for this approval, we have assembled an experienced oncology commercial team, established our distribution network, and we are ready to make COPIKTRA commercially available to patients."

COPIKTRA will be available in the U.S. market immediately. Verastem Oncology is committed to helping patients with CLL/SLL and FL access COPIKTRA through our Verastem Cares™ program. Verastem Cares is a comprehensive, personalized program designed to provide information and assistance to patients who have been prescribed COPIKTRA.

Patients, physicians, pharmacists, or other healthcare professionals with questions about COPIKTRA should contact 1-833-570-2273 (CARE) or visit www.COPIKTRA.com.

Conference Call Information

The Verastem Oncology management team will host a conference call today, Monday, September 24, 2018, at 4:30 PM (ET). The call can be accessed by dialing 1-877-341-5660 (toll-free) or 1-315-625-3226 (international) five minutes prior to the start of the call and providing the passcode 4496677.

The live, listen-only webcast of the conference call can be accessed by visiting the investors section of the Company's website at www.verastem.com. A replay of the webcast will be archived on the Company's website for 90 days following the call.

Indications and Usage

Chronic Lymphocytic Leukemia (CLL)/Small Lymphocytic Lymphoma (SLL)

COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory CLL or SLL after at least two prior therapies.

Follicular Lymphoma (FL)*

COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory FL after at least two prior systemic therapies.

**This indication is approved under accelerated approval based on overall response rate (ORR). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.*

COPIKTRA Clinical Trials

Efficacy in Relapsed or Refractory CLL/SLL

A randomized, multicenter, open-label trial (DUO™; NCT02004522) compared COPIKTRA versus ofatumumab in 319 adult patients with CLL (N = 312) or SLL (N = 7) after at least one prior therapy. The study randomized patients with a 1:1 ratio to receive either COPIKTRA 25mg BID until disease progression or unacceptable toxicity, or ofatumumab for 7 cycles.

The approval of COPIKTRA was based on efficacy and safety analysis of patients with at least 2 prior lines of therapy, where the benefit:risk appeared greater in this more heavily pretreated population compared to the overall trial population.

In this subset (95 randomized to COPIKTRA, 101 to ofatumumab), the median patient age was 69 years (range: 40 to 90 years), 59% were male, and 88% had an ECOG performance status of 0 or 1. Forty-six percent received 2 prior lines of therapy, and 54% received 3 or more prior lines. At baseline, 52% of patients had at least one tumor \geq 5 cm, and 22% of patients had a documented 17p deletion.

During randomized treatment, the median duration of exposure to COPIKTRA was 13 months (range: 0.2 to 37), with 80% of patients receiving at least 6 months and 52% receiving at least 12 months of COPIKTRA. The median duration of exposure to ofatumumab was 5 months (range: < 0.1 to 6).

Efficacy was based on progression-free survival (PFS) as assessed by an Independent Review Committee (IRC). Other efficacy measures included overall response rate (ORR). Efficacy of COPIKTRA compared to ofatumumab specifically in patients treated with at least two prior therapies is below.

Outcome per IRC	COPIKTRA	Ofatumumab
	N = 95	N = 101
PFS		
Number of events, n (%)	55 (58)	70 (69)
Progressive disease	44	62
Death	11	8
Median PFS (SE), months ^a	16.4 (2.1)	9.1 (0.5)
Hazard Ratio (SE), ^b	0.40 (0.2)	
COPIKTRA/ofatumumab		
Response rate		
ORR, n (%) ^c	74 (78)	39 (39)
CR	0 (0)	0 (0)
PR	74 (78)	39 (39)
Difference in ORR, % (SE)	39 (6.4)	

Abbreviations: CR = complete response; IRC = Independent Review Committee; PFS = progression-free survival; PR = partial response; SE = standard error

^a Kaplan-Meier estimate

^b Standard Error of ln(hazard ratio) = 0.2

^c IWCLL or revised IWG response criteria, with modification for treatment-related lymphocytosis

Efficacy in Relapsed or Refractory FL

Efficacy of COPIKTRA in patients with previously treated FL is based on a single-arm, multicenter trial (DYNAMO™; NCT01882803).

In DYNAMO, COPIKTRA 25 mg BID was administered in patients with FL (N = 83) who were refractory to rituximab and to either chemotherapy or radioimmunotherapy. Refractory disease was defined as less than a partial remission or relapse within 6 months after the last dose. The trial excluded patients with Grade 3b FL, large cell transformation, prior allogeneic transplant, and prior exposure to a PI3K inhibitor or to a Bruton's tyrosine kinase

inhibitor.

The median age was 64 years (range: 30 to 82 years), 68% were male, and 37% had bulky disease assessed at baseline (target lesion \geq 5 cm). Patients had a median of 3 prior lines of therapy (range: 1 to 10), with 94% being refractory to their last therapy and 81% being refractory to 2 or more prior lines of therapy. Most patients (93%) had an ECOG performance status of 0 or 1.

The median duration of exposure to COPIKTRA was 5 months (range: 0.4 to 24), with 41% of patients receiving at least 6 months and 10% receiving at least 12 months of COPIKTRA.

Efficacy was based on overall response rate and duration of response as assessed by an IRC, as shown below.

Endpoint	FL N = 83
ORR, n (%) ^a	35 (42)
95% CI	(31, 54)
CR, n (%)	1 (1)
PR, n (%)	34 (41)
Duration of response	
Range, months	0.0 ⁺ to 41.9 ⁺
Patients maintaining response at 6 months, n/N (%)	15/35 (43)
Patients maintaining response at 12 months, n/N (%)	6/35 (17)

Abbreviations: CI = confidence interval; CR = complete response; IRC = Independent Review Committee; ORR = overall response rate; PR = partial response

^a Per IRC according to Revised International Working Group criteria

⁺ Denotes censored observation

Important Safety 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

Infections: Serious, including fatal (18/442; 4%), infections occurred in 31% of patients receiving COPIKTRA 25 mg BID (N=442). The most common serious infections were pneumonia, sepsis, and lower respiratory infections. Median time to onset of any grade infection was 3 months (range: 1 day to 32 months), with 75% of cases occurring within 6 months. Treat infections prior to initiation of COPIKTRA. Advise patients to report new or worsening signs and symptoms of infection. For grade 3 or higher infection, withhold COPIKTRA until infection has resolved. Resume COPIKTRA at the same or reduced dose.

Serious, including fatal, *Pneumocystis jirovecii* pneumonia (PJP) occurred in 1% of patients taking COPIKTRA. Provide prophylaxis for PJP during treatment with COPIKTRA and following completion of treatment with COPIKTRA until the absolute CD4⁺ T cell count is greater than 200 cells/ μ L. Withhold COPIKTRA in patients with suspected PJP of any grade, and permanently discontinue if PJP is confirmed.

Cytomegalovirus (CMV) reactivation/infection occurred in 1% of patients taking COPIKTRA. Consider prophylactic antivirals during COPIKTRA treatment to prevent CMV infection including CMV reactivation. For clinical CMV infection or viremia, withhold COPIKTRA until infection or viremia resolves. If COPIKTRA is resumed, administer the same or reduced dose and monitor patients for CMV reactivation by PCR or antigen test at least monthly.

Diarrhea or Colitis: Serious, including fatal (1/442; <1%), diarrhea or colitis occurred in 18% of patients receiving COPIKTRA 25 mg BID (N=442). Median time to onset of any grade diarrhea or colitis was 4 months (range: 1 day to 33 months), with 75% of cases occurring by 8 months. The median event duration was 0.5 months (range: 1 day to 29 months; 75th percentile: 1 month).

Advise patients to report any new or worsening diarrhea. For patients presenting with mild or moderate diarrhea (Grade 1-2) (i.e., up to 6 stools per day over baseline) or asymptomatic (Grade 1) colitis, initiate supportive care with antidiarrheal agents, continue COPIKTRA at the current dose, and monitor the patient at least weekly until the event resolves. If the diarrhea is unresponsive to antidiarrheal therapy, withhold COPIKTRA and initiate supportive therapy with enteric acting steroids (e.g., budesonide). Monitor the patient at least weekly. Upon resolution of the diarrhea, consider restarting COPIKTRA at a reduced dose.

For patients presenting with abdominal pain, stool with mucus or blood, change in bowel habits, peritoneal signs, or with severe diarrhea (Grade 3)

(i.e., > 6 stools per day over baseline), withhold COPIKTRA and initiate supportive therapy with enteric acting steroids (e.g., budesonide) or systemic steroids. A diagnostic work-up to determine etiology, including colonoscopy, should be performed. Monitor at least weekly. Upon resolution of the diarrhea or colitis, restart COPIKTRA at a reduced dose. For recurrent Grade 3 diarrhea or recurrent colitis of any grade, discontinue COPIKTRA. Discontinue COPIKTRA for life-threatening diarrhea or colitis.

Cutaneous Reactions: Serious, including fatal (2/442; <1%), cutaneous reactions occurred in 5% of patients receiving COPIKTRA 25 mg BID (N=442). Fatal cases included drug reaction with eosinophilia and systemic symptoms (DRESS) and toxic epidermal necrolysis (TEN). Median time to onset of any grade cutaneous reaction was 3 months (range: 1 day to 29 months, 75th percentile: 6 months) with a median event duration of 1 month (range: 1 day to 37 months, 75th percentile: 2 months).

Presenting features for the serious events were primarily described as pruritic, erythematous, or maculo-papular. Less common presenting features include exanthem, desquamation, erythroderma, skin exfoliation, keratinocyte necrosis, and papular rash. Advise patients to report new or worsening cutaneous reactions. Review all concomitant medications and discontinue any medications potentially contributing to the event. For patients presenting with mild or moderate (Grade 1-2) cutaneous reactions, continue COPIKTRA at the current dose, initiate supportive care with emollients, antihistamines (for pruritus), or topical steroids, and monitor the patient closely. Withhold COPIKTRA for severe (Grade 3) cutaneous reaction until resolution. Initiate supportive care with steroids (topical or systemic) or antihistamines (for pruritus). Monitor at least weekly until resolved. Upon resolution of the event, restart COPIKTRA at a reduced dose. Discontinue COPIKTRA if severe cutaneous reaction does not improve, worsens, or recurs. For life-threatening cutaneous reactions, discontinue COPIKTRA. In patients with SJS, TEN, or DRESS of any grade, discontinue COPIKTRA.

Pneumonitis: Serious, including fatal (1/442; <1%), pneumonitis without an apparent infectious cause occurred in 5% of patients receiving COPIKTRA 25 mg BID (N=442). Median time to onset of any grade pneumonitis was 4 months (range: 9 days to 27 months), with 75% of cases occurring within 9 months. The median event duration was 1 month, with 75% of cases resolving by 2 months.

Withhold COPIKTRA in patients with new or progressive pulmonary signs and symptoms such as cough, dyspnea, hypoxia, interstitial infiltrates on a radiologic exam, or a decline by more than 5% in oxygen saturation, and evaluate for etiology. If the pneumonitis is infectious, patients may be restarted on COPIKTRA at the previous dose once the infection, pulmonary signs and symptoms resolve. For moderate non-infectious pneumonitis (Grade 2), treat with systemic corticosteroids and resume COPIKTRA at a reduced dose upon resolution. If non-infectious pneumonitis recurs or does not respond to steroid therapy, discontinue COPIKTRA. For severe or life-threatening non-infectious pneumonitis, discontinue COPIKTRA and treat with systemic steroids.

Hepatotoxicity: Grade 3 and 4 ALT and/or AST elevation developed in 8% and 2%, respectively, of patients receiving COPIKTRA 25 mg BID (N=442). Two percent of patients had both an ALT or AST > 3 X ULN and total bilirubin > 2 X ULN. Median time to onset of any grade transaminase elevation was 2 months (range: 3 days to 26 months), with a median event duration of 1 month (range: 1 day to 16 months).

Monitor hepatic function during treatment with COPIKTRA. For Grade 2 ALT/AST elevation (> 3 to 5 X ULN), maintain COPIKTRA dose and monitor at least weekly until return to < 3 X ULN. For Grade 3 ALT/AST elevation (> 5 to 20 X ULN), withhold COPIKTRA and monitor at least weekly until return to < 3 X ULN. Resume COPIKTRA at the same dose (first occurrence) or at a reduced dose for subsequent occurrences. For grade 4 ALT/AST elevation (> 20 X ULN), discontinue COPIKTRA.

Neutropenia: Grade 3 or 4 neutropenia occurred in 42% of patients receiving COPIKTRA 25 mg BID (N=442), with Grade 4 neutropenia occurring in 24% of all patients. Median time to onset of grade ≥ 3 neutropenia was 2 months (range: 3 days to 31 months), with 75% of cases occurring within 4 months.

Monitor neutrophil counts at least every 2 weeks for the first 2 months of COPIKTRA therapy, and at least weekly in patients with neutrophil counts < 1.0 Gi/L (Grade 3-4). Withhold COPIKTRA in patients presenting with neutrophil counts < 0.5 Gi/L (Grade 4). Monitor until ANC is > 0.5 Gi/L, then resume COPIKTRA at same dose for the first occurrence or at a reduced dose for subsequent occurrences.

Embryo-Fetal Toxicity: Based on findings in animals and its mechanism of action, COPIKTRA can cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to a fetus. Conduct pregnancy testing before initiating COPIKTRA treatment. Advise females of reproductive potential and males with female partners of reproductive potential to use effective contraception during treatment and for at least 1 month after the last dose.

ADVERSE REACTIONS

B-cell Malignancies Summary

Fatal adverse reactions within 30 days of the last dose occurred in 8% (36/442) of patients treated with COPIKTRA 25 mg BID. Serious adverse reactions were reported in 289 patients (65%). The most frequent serious adverse reactions that occurred were infection (31%), diarrhea or colitis (18%), pneumonia (17%), rash (5%), and pneumonitis (5%).

Adverse reactions resulted in treatment discontinuation in 156 patients (35%) most often due to diarrhea or colitis, infection, and rash. COPIKTRA was dose reduced in 104 patients (24%) due to adverse reactions, most often due to diarrhea or colitis and transaminase elevation. The most common adverse reactions (reported in $\geq 20\%$ of patients) were diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain and anemia.

CLL/SLL: Fatal adverse reactions within 30 days of the last dose occurred in 12% (19/158) of patients treated with COPIKTRA and in 4% (7/155) of patients treated with ofatumumab. Serious adverse reactions were reported in 73% (115/158) of patients treated with COPIKTRA and most often involved infection (38%; 60/158) and diarrhea or colitis (23%; 36/158). COPIKTRA was discontinued in 57 patients (36%), most often due to diarrhea or colitis, infection, and rash. COPIKTRA was dose reduced in 46 patients (29%) due to adverse reactions, most often due to diarrhea or colitis and rash. The most common adverse reactions with COPIKTRA (reported in $\geq 20\%$ of patients) were diarrhea or colitis, neutropenia, pyrexia, upper respiratory tract infection, pneumonia, rash, fatigue, nausea, anemia and cough.

FL: Serious adverse reactions were reported in 58% of patients and most often involved diarrhea or colitis, pneumonia, renal insufficiency, rash, and sepsis. The most common adverse reactions ($\geq 20\%$ of patients) were diarrhea or colitis, nausea, fatigue, musculoskeletal pain, rash, neutropenia, cough, anemia, pyrexia, headache, mucositis, abdominal pain, vomiting, transaminase elevation, and thrombocytopenia. Adverse reactions resulted in

COPIKTRA discontinuation in 29% of patients, most often due to diarrhea or colitis and rash. COPIKTRA was dose reduced in 23% due to adverse reactions, most often due to transaminase elevation, diarrhea or colitis, lipase increased and infection.

DRUG INTERACTIONS

- CYP3A Inducers: Coadministration with a strong CYP3A inducer may reduce COPIKTRA efficacy. Avoid coadministration with strong CYP3A4 inducers.
- CYP3A Inhibitors: Coadministration with a strong CYP3A inhibitor may increase the risk of COPIKTRA toxicities. Reduce COPIKTRA dose to 15 mg BID when coadministered with a strong CYP3A4 inhibitor.
- CYP3A Substrates: Coadministration of COPIKTRA with sensitive CYP3A4 substrates may increase the risk of toxicities of these drugs. Consider reducing the dose of the sensitive CYP3A4 substrate and monitor for signs of toxicities of the coadministered sensitive CYP3A substrate.

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 Follicular Lymphoma

Follicular lymphoma (FL) is typically a slow-growing or indolent form of non-Hodgkin lymphoma (NHL) that arises from B-lymphocytes, making it a B-cell lymphoma. This lymphoma subtype accounts for 20 to 30 percent of all NHL cases, with more than 140,000 people in the US with FL and more than 13,000 newly diagnosed patients this year. Common symptoms of FL include enlargement of the lymph nodes in the neck, underarms, abdomen, or groin, as well as fatigue, shortness of breath, night sweats, and weight loss. Often, patients with FL have no obvious symptoms of the disease at diagnosis. Follicular lymphoma is usually not considered to be curable, but more of a chronic disease, with patients living for many years with this form of lymphoma. 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 FL.

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.^{2,3,4} 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.⁵ 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, Inc. (Nasdaq:VSTM) (Verastem Oncology or the Company), is a biopharmaceutical company focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients. Verastem Oncology currently markets COPIKTRA™ (duvelisib), an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, which 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. The indication in FL is approved under accelerated approval based on overall response rate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials. In addition, Verastem Oncology is developing the focal adhesion kinase (FAK) inhibitor defactinib, which is currently being evaluated in three separate clinical collaborations in combination with immunotherapeutic agents for the treatment of several different cancer types, including pancreatic cancer, ovarian cancer, non-small cell lung cancer (NSCLC), and mesothelioma. Verastem Oncology's product candidates seek to treat cancer by modulating the local tumor microenvironment and enhancing anti-tumor immunity. 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 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.

¹ Decision Resources Group 2018 Estimates.

² 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.

³ 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.

⁴ 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.

⁵ www.clinicaltrials.gov, NCT03372057.

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