Verastem Oncology Reports Third Quarter 2018 Financial Results

November 7, 2018

Quarter Highlighted by FDA Approval of COPIKTRA™ (duvelisib) Capsules

Exclusive License Agreement Executed with CSPC Pharmaceutical Group Limited for the Development and Commercialization of COPIKTRA in China

Company Secures $150 Million in Gross Proceeds Through an Offering of Convertible Notes

BOSTON--(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 reported financial results for the third quarter ended September 30, 2018 and provided an overview of certain corporate developments.

“During the third quarter, we achieved a remarkable milestone with our lead product COPIKTRA™ (duvelisib) receiving its first regulatory approval from the U.S. Food and Drug Administration,” said Robert Forrester, President and Chief Executive Officer of Verastem Oncology. “We were also delighted to have signed an exclusive licensing agreement with CSPC Pharmaceutical Group Limited for the development and commercialization of COPIKTRA for all oncology indications in China, further extending the global reach of our product. This is an exciting time at Verastem Oncology, and we believe this is just the beginning for both COPIKTRA and the Company. It is our goal to unlock the potential of PI3K inhibition, initially as a monotherapy, and through novel combinations to potentially expand its use to broader hematologic and solid tumors.”

“The COPIKTRA launch is well underway and proceeding on track,” said Joseph Lobacki, Executive Vice President and Chief Commercial Officer of Verastem Oncology. “We had the necessary staffing in place and were ready to distribute COPIKTRA on the day of approval. Our commercial field force and medical affairs teams are engaging with physicians, and our contracted specialty pharmacy partners are receiving prescriptions. We are thrilled that within six weeks of the approval date, COPIKTRA has been included in the NCCN guidelines and we’ve secured reimbursement coverage, including the top national plans, for 72% of the U.S. Pharmacy lives. This is an exciting time for Verastem Oncology, and I am proud of the team’s diligent efforts and early progress in the launch.”

Third Quarter 2018 and Recent Highlights:

COPIKTRA (duvelisib)

- ***COPIKTRA (duvelisib) Capsules Approved by the FDA*** – On September 24, 2018, the U.S. Food and Drug Administration (FDA) approved COPIKTRA, an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma. COPIKTRA was 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 is contingent upon verification and description of clinical benefit in confirmatory trials. The commercial launch of COPIKTRA is ongoing.
- ***COPIKTRA Added to NCCN Guidelines for CLL/SLL and FL*** – In early October 2018, following its approval by the FDA, the National Comprehensive Cancer Network® (NCCN) added COPIKTRA to the Clinical Practice Guidelines in Oncology (NCCN Guidelines). Physicians use the NCCN Guidelines as the standard resource for determining the best course of treatment for patients, and the Company believes these updated guidelines will increase awareness for COPIKTRA and help health care providers make informed decisions for patients battling these difficult to treat advanced cancers.
- ***Phase 3 DUO Study Results Published in the Journal* BLOOD** – In early October 2018, the results of the randomized, multicenter, open-label Phase 3 DUO™ study (NCT02004522), which evaluated COPIKTRA versus ofatumumab in patients with relapsed or refractory CLL/SLL, were published online in the peer-reviewed journal *Blood*. The full manuscript titled “The phase 3 DUO trial: duvelisib versus ofatumumab in relapsed and refractory CLL/SLL,” is available at www.bloodjournal.org.
- ***Investigator-Sponsored Study Initiated Evaluating COPIKTRA in Combination with Venetoclax*** – In early September 2018, the first patient was dosed in a multicenter Phase 1/2 clinical trial investigating COPIKTRA in combination with venetoclax, an oral selective inhibitor of BCL-2, in patients with relapsed or refractory CLL/SLL. Preclinical data support this combination, as COPIKTRA has been shown to upregulate BCL-2 transcript and protein expression levels and potentially enhance the ability of venetoclax to induce apoptosis in ex vivo human CLL cells. The primary objectives of the Phase 1 portion of the trial are to determine the maximum tolerated dose and the recommended Phase 2 dose of venetoclax for this combination regimen. The trial is being led by Matthew Davids, M.D., MMSc, Assistant Professor of Medicine, Harvard Medical School, and Associate Director, Center for Chronic Lymphocytic Leukemia, Dana-Farber Cancer Institute.
- ***Eight Abstracts Selected for Presentation at the Upcoming American Society of Hematology 2018 Annual Meeting***

(ASH 2018) – In November 2018, the Company announced that eight abstracts were selected for presentation, including one oral presentation, at ASH 2018 which is being held December 1-4, 2018 in San Diego, CA. The oral presentation will highlight data from the Phase 1 study evaluating COPIKTRA in combination with romidepsin in relapsed or refractory peripheral T-cell lymphoma. Additional poster presentations will showcase preclinical and clinical data reinforcing the potential of COPIKTRA.

Corporate and Financial

- ***Signed Exclusive License Agreement with CSPC Pharmaceutical Group Limited (CSPC) for the Development and Commercialization of Duvelisib in China*** – In September 2018, Verastem Oncology announced its entry into an exclusive license agreement with CSPC to develop and commercialize COPIKTRA in China, Hong Kong, Macau and Taiwan (collectively, the CSPC Territory) for all oncology indications. Under the terms of the agreement, Verastem Oncology is to receive an upfront payment of $15.0 million and is entitled to receive aggregate payments of up to $160.0 million if certain development, regulatory and commercial milestones are successfully achieved, plus double-digit royalties on net sales of products containing duvelisib in the CSPC Territory. CSPC will receive exclusive rights to develop and commercialize COPIKTRA and hold the marketing authorization and product license for COPIKTRA in the CSPC Territory. Additionally, CSPC will have the right to collaborate with Verastem Oncology on certain global development and clinical trial activities and will share pro-rata in the cost of studies that they elect to participate in. CSPC is a leading pharmaceutical group in China. CSPC has been listed on the Main Board of the Hong Kong Stock Exchange since 1994 and is currently a constituent stock of the Hang Seng Index. CSPC is a leading developer and manufacturer of innovative and generic drugs in China.
- ***Collaboration with The Leukemia & Lymphoma Society for Development of Duvelisib in Peripheral T-Cell Lymphoma -*** Verastem Oncology's duvelisib was selected for The Leukemia & Lymphoma Society's (LLS) 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 TAP funds to conduct certain translational and clinical activities relating to the development of duvelisib for the treatment of Peripheral T-Cell Lymphoma (PTCL). LLS and Verastem Oncology will share the cost of the PTCL 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.
- ***Sale of Convertible Senior Notes for Gross Proceeds of $150 Million –*** In October 2018, the Company completed an offering of 5.00% convertible senior notes due 2048 through a registered direct offering. The Company received net proceeds of $145.1 million, after transaction fees and expenses.
- ***Robert E. Gagnon Appointed Chief Financial Officer*** – In August 2018, Mr. Gagnon was appointed Chief Financial Officer. He comes to Verastem Oncology from Harvard Bioscience, Inc., where he served as Chief Financial Officer. Prior to Harvard Bioscience, he served as Executive Vice President, Chief Financial Officer and Treasurer at Clean Harbors, Inc., as well as Chief Accounting Officer and Controller at Biogen Idec, Inc. Earlier, he worked in a variety of senior positions at Deloitte & Touche, LLP, and Price Waterhouse Coopers, LLP. Mr. Gagnon holds an M.B.A. from the MIT Sloan School of Management and a Bachelor of Arts degree in accounting from Bentley College. His prior experience heading global finance operations, and his overall business acumen, will be a great asset to the Company as it executes on its growth strategy.
- ***Gina Consylman Appointed to Board of Directors –*** In October 2018, Ms. Consylman was appointed to the Company's Board of Directors (the Board) and will serve as Chair of the Board's Audit Committee. Ms. Consylman replaces Louise Phanstiel who left the Board to pursue other professional opportunities. Ms. Consylman currently serves as Senior Vice President and Chief Financial Officer of Ironwood Pharmaceuticals, Inc., a commercial biotech company, where she oversees the finance, planning, accounting, tax, treasury and insurance functions. Prior to joining Ironwood, she held various senior level accounting and corporate controller positions at Analogic Corporation, Biogen Inc., and Varian Semiconductor Equipment Associates, Inc. Ms. Consylman holds a Bachelor of Science degree in accounting from Johnson & Wales University, a Master of Science degree in taxation from Bentley University and is a Certified Public Accountant.
- ***Hagop Youssoufian, MSc, M.D., Appointed Head of Medical Strategy –*** In October 2018, the Company announced that Dr. Youssoufian would transition to Head of Medical Strategy from his prior role as Head of Hematology and Oncology Development. Dr. Youssoufian will be taking over responsibilities from Diep Le, M.D., Ph.D., who stepped down as Chief Medical Officer.

Third Quarter 2018 Financial Results

License revenue for the three months ended September 30, 2018 (2018 Quarter) was $15.0 million and was related to an upfront payment pursuant to the license and collaboration agreement executed between Verastem Oncology and CSPC in September 2018. Verastem Oncology had no license revenue during the three months ended September 30, 2017 (2017 Quarter).

Verastem Oncology began commercial sales of COPIKTRA within the United States in September 2018, following receipt of FDA marketing approval on September 24, 2018. For the 2018 Quarter, the Company recorded approximately $508,000 of net product revenue. Verastem Oncology had no

product revenue during the 2017 Quarter.

Costs of revenues, excluding amortization of acquired intangible assets (cost of revenues) of approximately $49,000 for the 2018 Quarter, consisted of costs associated with the manufacturing of COPIKTRA, royalties owed to Infinity Pharmaceuticals, Inc. (Infinity) on such sales, and certain period costs. Verastem Oncology expensed the manufacturing costs of COPIKTRA as operating expenses in the periods prior to July 1, 2018. Verastem Oncology had no cost of revenues during the 2017 Quarter.

Research and development expense for the 2018 Quarter was $11.6 million compared to $17.7 million for the 2017 Quarter. The $6.1 million decrease from the 2017 Quarter to the 2018 Quarter was primarily related to a decrease of $6.0 million in license fees related to a one-time milestone payment pursuant to the Infinity license agreement that was recognized in the 2017 Quarter and a decrease of $1.2 million in consulting fees. These decreases were offset by an increase of $1.1 million in personnel related costs, including non-cash stock-based compensation.

Selling, general and administrative expense for the 2018 Quarter was $25.4 million compared to $5.4 million for the 2017 Quarter. The increase of $20.0 million from the 2017 Quarter to the 2018 Quarter primarily resulted from an increase in personnel related costs, including non-cash stock-based compensation, of $9.7 million, primarily related to the hiring and staffing of Verastem Oncology's sales and commercial teams, an increase in consulting and professional fees of $9.1 million, primarily related to the support of commercial launch preparation activities, and travel and other costs of $1.2 million.

Amortization of acquired intangible assets for the 2018 Quarter of approximately $31,000 was related to the COPIKTRA finite-lived intangible asset which Verastem Oncology recognized and began amortizing in September 2018. There was no amortization of acquired intangible assets in the 2017 Quarter.

Net loss for the 2018 Quarter was $21.7 million, or $0.29 per share, as compared to a net loss of $23.1 million, or $0.61 per share, for 2017 Quarter.

As of September 30, 2018, Verastem Oncology had cash, cash equivalents and investments of $145.6 million compared to $86.7 million of cash, cash equivalents and investments as of December 31, 2017. Cash, cash equivalents and investments for the 2018 Quarter does not include the $145.1 million in net proceeds from the registered direct offering of convertible notes in October 2018.

The number of outstanding common shares as of September 30, 2018 was 73,703,423.

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 ≥ 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** **N = 95**	**Ofatumumab** **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]		
COPIKTRA/ofatumumab	0.40 (0.2)	
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 International Working Group 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 ≥ 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/μL. 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 ≥ 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 ≥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 (≥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 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, including statements regarding the development and activity of Verastem Oncology's lead product COPIKTRA, and Verastem Oncology's PI3K and FAK programs generally, its commercialization of 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 timeline 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 Verastem Oncology's 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 September 30, 2018 as filed with the Securities and Exchange Commission (SEC) on November 7, 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.

Verastem, Inc.

Condensed Consolidated Balance Sheets

(in thousands)

	September 30, 2018 (unaudited)	December 31, 2017
Cash, cash equivalents and investments	$ 145,639	$ 86,672
Accounts receivable, net	10,562	—
Inventory	131	—
Prepaid expenses and other current assets	2,397	1,115
Property and equipment, net	1,210	861
Intangible assets, net	21,969	—
Other assets	1,247	1,143
Total assets	**$ 183,155**	**$ 89,791**
Accounts payable, accrued expenses and other current liabilities	$ 53,441	$ 17,128
Long-term debt	21,535	14,828
Other liabilities	566	151
Stockholders' equity	107,613	57,684
Total liabilities and stockholders' equity	**$ 183,155**	**$ 89,791**

Verastem, Inc.

Unaudited Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended September 30,		Nine months ended September 30,	
	2018	2017	2018	2017
Revenue:				
License revenue	$ 15,000	$ —	$ 25,000	$ —
Product revenue, net	508	—	508	—
Total revenue	15,508	—	25,508	—
Operating expenses:				
Costs of revenues, excluding amortization of acquired intangible assets	49	—	49	—
Research and development	11,571	17,743	34,886	35,170
Selling, general and administrative	25,426	5,394	51,066	14,582
Amortization of acquired intangible assets	31	—	31	—
Total operating expenses	37,077	23,137	86,032	49,752
Loss from operations	(21,569)	(23,137)	(60,524)	(49,752)
Interest income	763	121	1,297	416
Interest expense	(862)	(110)	(1,858)	(231)
Net loss	**$ (21,668)**	**$ (23,126)**	**$ (61,085)**	**$ (49,567)**
Net loss per share—basic and diluted	**$ (0.29)**	**$ (0.61)**	**$ (0.99)**	**$ (1.33)**
Weighted-average number of common shares used in net loss per share-basic and diluted	**73,644**	**37,630**	**61,995**	**37,207**

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