



## Verastem Oncology Announces Fourth Quarter and Full-Year 2018 Financial Results and Corporate Developments

March 12, 2019

*U.S. Commercial Launch of COPIKTRA™ Underway*

*\$1.7 Million in 2018 Net Product Revenues from COPIKTRA*

*Cash, Cash Equivalents and Short-Term Investments of \$249.7 million as December 31, 2018*

BOSTON--(BUSINESS WIRE)--Mar. 12, 2019-- Verastem, Inc. (Nasdaq:VSTM), operating as 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 three and twelve months ended December 31, 2018, including revenue from its first commercial product, COPIKTRA™ (duvelisib), which was approved by the U.S. Food and Drug Administration (FDA) on September 24, 2018.

"Upon the early FDA approval we received, our commercial team was mobilized the same day and began educating physicians, patients and payors on the clinical benefits and appropriate use of COPIKTRA and to secure access to therapy," said Robert Forrester, President and Chief Executive Officer of Verastem Oncology. "This year is poised to be an exciting one as we continue to drive awareness of COPIKTRA and work to expand upon the potential of PI3K inhibition through the investigation of duvelisib, initially as a monotherapy, and through novel combinations, in additional hematologic malignancies like peripheral T-cell lymphoma (PTCL)."

"Following FDA approval, COPIKTRA was quickly added to the National Comprehensive Cancer Network® (NCCN) guidelines, and as of December 31, 2018, we had secured formulary listing and reimbursement for approximately 75% of targeted health plans. As of March 11, 2019, that number increased to 90%, underscoring our efforts to provide access to treatment for appropriate patients," said Joseph Lobacki, Executive Vice President and Chief Commercial Officer of Verastem Oncology. "Looking ahead to 2019, we are focused on further identifying appropriate patients for treatment with COPIKTRA, and we intend to continue to work with the leukemia and lymphoma community to increase awareness and help ensure physicians and patients are able to get the support they need."

### Key 2018 Accomplishments:

- **Launched COPIKTRA in the United States**—Verastem Oncology launched COPIKTRA, an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, in the United States following FDA approval 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. 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, the first of which is expected to start in 2019.

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 has implemented a 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 associated with additional adverse reactions which may also require dose reduction, treatment delay or discontinuation of COPIKTRA.

Please see [www.COPIKTRAHCP.com/prescribinginformation](http://www.COPIKTRAHCP.com/prescribinginformation) for full Prescribing Information including BOXED WARNING and Medication Guide in addition to the Important Safety Information provided below.

- **COPIKTRA Added to NCCN Guidelines for CLL/SLL, FL and Marginal Zone Lymphoma (MZL)** – The NCCN added COPIKTRA to the Clinical Practice Guidelines in Oncology (NCCN Guidelines), the standard physician resource for determining the appropriate course of treatment for patients. 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. COPIKTRA is not approved for use in MZL.
- **Presented COPIKTRA Data at the 23<sup>rd</sup> Annual International Congress on Hematologic Malignancies (ICHM)** – The Company presented four COPIKTRA abstracts at ICHM 2019, including an abstract highlighting Phase 3 DUO data in patients with relapsed or refractory CLL/SLL who have progressed following two prior lines of the therapy. This is the same indication for which COPIKTRA received approval from the FDA in September 2018. In this analysis, COPIKTRA demonstrated progression-free survival (PFS) of 16.4 months and an ORR of 78%, with a manageable safety profile. The remaining three abstracts featured data from a long-term (>2 years) efficacy and safety analysis, the Phase 3 DUO crossover extension study, and prognostic and immune-related factors associated with response to duvelisib from the Phase 2 DYNAMO™ study in indolent non-Hodgkin's lymphoma (iNHL). Collectively, the data presented at ICHM 2019

continue to support the use of COPIKTRA in its approved indications of relapsed or refractory CLL/SLL after at least two prior therapies and FL after at least two prior systemic therapies. PDF copies of all of the ICHM 2019 poster presentations are available [here](#).

- **Presented Updated Duvelisib Combination Data in PTCL at the American Society of Hematology 2018 Annual Meeting (ASH 2018)** – The oral presentation highlighted updated data from an investigator-sponsored Phase 1 study evaluating duvelisib in combination with romidepsin in relapsed or refractory T-cell lymphomas, including PTCL and cutaneous T-cell lymphoma (CTCL). Of the 27 patients with PTCL evaluable for efficacy, 16 responded (9 complete responses (CRs) and 7 partial responses (PRs)) for an overall response rate (ORR) of 59%. Importantly, of the 27 patients with PTCL treated with the combination of duvelisib and romidepsin, 6 (22%) responded deeply enough to allow them to bridge to potentially curative stem cell transplant (SCT). Median progression-free survival (PFS) for patients with PTCL was 6.72 months, which was confounded by 6 subjects that proceeded to SCT. Among the 31 patients at the maximum tolerated dose who were evaluable for safety, the most common Grade  $\geq 3$  adverse events occurring in  $\geq 10\%$  of patients were neutropenia (32%), diarrhea (19%), increased transaminase (23%; alanine aminotransferase 16% and aspartate aminotransferase 6%), hyponatremia (13%) and platelet count decrease (10%).
- **Presented Frontline Duvelisib Combination Data in Younger CLL Patients at European Hematology Association 2018 Annual Meeting (EHA 2018)** – Dr. Matthew Davids, M.D., MMSc, Assistant Professor of Medicine, Harvard Medical School, and Associate Director, Center for Chronic Lymphocytic Leukemia at the Dana-Farber Cancer Institute, presented Phase 1b/2 clinical data from 31 patients who received duvelisib in combination with fludarabine (F), cyclophosphamide (C), and rituximab (R) (dFCR) as frontline therapy. The ORR was 94%, with 26% (n=8) of patients achieving a CR or CRi, and 68% achieving a PR. The best rate of minimum residual disease (MRD) negativity in the bone marrow (BM) in patients with at least one evaluation was 76% (22 of 29 patients). The two-year progression-free survival and overall survival rates for patients in the study were both 97%. The recommended Phase 2 dose for duvelisib in combination with FCR was established as 25mg twice daily. The most common all grade non-hematologic adverse events (AEs) were nausea (72%, all Grade 1/2), fatigue (69%, 3% Grade 3), fever (53%, all Grade 1/2), diarrhea (47%, 3% Grade 3), transaminitis (34%, 28% Grade 3/4), anorexia (34%, all Grade 1/2), vomiting (28%, all Grade 1/2), pruritus (16%, 3% Grade 3), arthritis (9%, all Grade 2) and Cytomegalovirus (CMV) reactivation (6%, both Grade 2). The most common all grade hematologic adverse events were thrombocytopenia (65%; 34% Grade 3/4), neutropenia (59%; 50% Grade 3/4), and anemia (38%, 16% Grade 3/4). Serious AEs included febrile neutropenia (n=6, all Grade 3) and pneumonia (n=6, including 3 cases of PJP despite planned prophylaxis).
- **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 Dr. Matthew Davids.
- **Signed Exclusive License Agreements in China and Japan**– Verastem Oncology entered into exclusive license agreements with CSPC Pharmaceutical Group Limited (CSPC) to develop and commercialize COPIKTRA in China, Hong Kong, Macau and Taiwan (collectively, the CSPC Territory), and Yakult Honsha Co., Ltd. (Yakult) to develop and commercialize COPIKTRA in Japan. Both agreements are for the treatment, prevention or diagnosis of all oncology indications.
- Under the terms of the agreement with CSPC, Verastem Oncology received 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 is a leading pharmaceutical group in China.
- The transaction with Yakult carries a total deal value of up to \$100.0 million, includes a one-time upfront payment of \$10.0 million and up to an additional \$90.0 million if certain development, regulatory and commercial milestones are successfully achieved by Yakult. In addition, Verastem Oncology is also eligible to receive double-digit royalties based on future net sales of products containing duvelisib in Japan. Yakult has a strong presence in development and commercialization of therapeutic products in the field of oncology and markets several branded anti-cancer therapies, including Elplat® and Campto®.
- **Collaboration with The Leukemia & Lymphoma Society for Development of Duvelisib in PTCL** –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 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.

- **Phase 3 DUO Study Results Published in the Journal BLOOD** – 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 in the peer-reviewed journal Blood (Flinn *et al*). The publication was accompanied by a review article by Jennifer R. Brown, M.D., Ph.D., Director of the Center for Chronic Lymphocytic Leukemia at the Dana-Farber Cancer Institute, discussing the role of PI3K inhibitors and duvelisib in current CLL therapy. The full manuscript titled “The phase 3 DUO trial: duvelisib versus ofatumumab in relapsed and refractory CLL/SLL,” is available at [www.bloodjournal.org](http://www.bloodjournal.org).
- **Entering 2019 with Cash, Cash Equivalents and Short-Term Investments of \$249.7 Million**– During 2018, Verastem Oncology successfully completed multiple fundraising transactions, including an underwritten registered offering in May 2018, a registered offering in June 2018, and a registered direct offering of 5.00% Convertible Senior Notes in October 2018 (Convertible Senior Notes). The Company also raised funds through the sale of shares of common stock under its at-the-market equity offering program. These fundraising transactions helped to provide the Company with a strong cash, cash equivalents and short-term investments balance of \$249.7 million as of December 31, 2018.
- **Key Commercial, Clinical and Investor Relations Team Additions** – In February 2019, the Company expanded its commercial and clinical teams through the appointment of several new employees, including Amy Cavers as Senior Vice President, Strategic Engagement and Alignment, Robert Morgan as Senior Vice President, Development Operations, and Erin Cox, Senior Director, Investor Relations and Corporate Communications.

Selected posters and presentations are available within the “Media” section of the Company’s website at [www.verastem.com](http://www.verastem.com).

#### Fourth Quarter 2018 Financial Results

Net loss for the three months ended December 31, 2018 (2018 Quarter) was \$11.3 million, or \$0.15 per share (basic), as compared to \$18.2 million, or \$0.43 per share (basic), for the three months ended December 31, 2017 (2017 Quarter). Net loss for the 2018 Quarter includes a non-cash gain of \$25.6 million, or \$0.35 per share (basic), relating to the accounting impact of a financial derivative related to our Convertible Senior Notes. In addition, net loss includes non-cash stock-based compensation expense of \$1.8 million and \$1.0 million for the 2018 and 2017 Quarters, respectively.

Net product revenue for the 2018 Quarter was \$1.2 million which reflects the first full quarter of recorded sales for COPIKTRA. The Company did not have any product revenue for the 2017 Quarter as the FDA approved COPIKTRA on September 24, 2018.

Research and development expense for the 2018 Quarter was \$8.8 million compared to \$11.3 million for the 2017 Quarter, a decrease of \$2.5 million or 22%, primarily related to lower R&D costs associated with the development of COPIKTRA.

Selling, general and administrative expense for the 2018 Quarter was \$26.2 million compared to \$6.8 million for the 2017 Quarter. The increase of \$19.4 million, or 285%, from the 2017 Quarter to the 2018 Quarter was due to higher personnel and related costs, as well as promotional and consulting costs in support of the commercial launch of COPIKTRA.

Other income of \$25.6 million for the 2018 Quarter relates entirely to a non-cash gain for the accounting impact of a financial derivative related to our Convertible Senior Notes.

#### Full-Year 2018 Financial Results

Net loss for the year ended December 31, 2018 (2018 Period) was \$72.4 million, or \$1.12 per share (basic), as compared to \$67.8 million, or \$1.76 per share (basic), for the year ended December 31, 2017 (2017 Period). Net loss for the 2018 Period includes a non-cash gain of \$25.6 million, or \$0.39 per share (basic), relating to the accounting impact of a financial derivative related to our Convertible Senior Notes. In addition, net loss includes non-cash stock-based compensation expense of \$6.7 million and \$5.0 million for the 2018 and 2017 Periods, respectively.

Total revenue for the 2018 Period was \$26.7 million which reflects net product revenue of \$1.7 million for sales of COPIKTRA and license revenue of \$25.0 million relating to our license agreements with Yakult and CSPC. The Company did not have any product revenue for the 2017 Period as the FDA approved COPIKTRA on September 24, 2018. The Company did not have any license revenue for the 2017 Period.

Research and development expense for the 2018 Period was \$43.6 million compared to \$46.4 million for the 2017 Period. The decrease of \$2.8 million, or 6%, from the 2017 Period to the 2018 Period was primarily related to a decrease of \$6.0 million in license fees related to a one-time milestone earned pursuant to our Infinity license agreement which was recognized in the 2017 Period, offset, in part, by a net increase of \$3.2 million in personnel related costs, including non-cash stock-based compensation, clinical trial costs and consulting fees for COPIKTRA.

Selling, general and administrative expense for the 2018 Period was \$77.3 million compared to \$21.4 million for the 2017 Period. The increase of \$55.9 million, or 261%, from the 2017 Period to the 2018 Period primarily resulted from higher personnel and related costs, promotional and consulting costs in support of the commercial launch of COPIKTRA.

Other income of \$25.6 million for the 2018 Period relates entirely to a non-cash gain for the accounting impact of a financial derivative related to our Convertible Senior Notes.

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 \$145.3 million, after transaction fees and expenses. Verastem Oncology ended 2018 with cash, cash equivalents and short-term investments of \$249.7 million.

For more information about Verastem Oncology, including its leadership, product and pipeline, please visit [verastem.com](http://verastem.com)

#### Important Safety Information

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

- 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/ $\mu$ 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  $\geq$ 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.

See full Prescribing Information, including Boxed Warning, at [www.COPIKTRA.com](http://www.COPIKTRA.com)

### 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. In 2018, there were approximately 200,000 patients in the United States affected by CLL/SLL with nearly 20,000 new diagnoses. 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. In 2018, this lymphoma subtype accounted for 20 to 30 percent of all NHL cases, with more than 140,000 people in the United States with FL and more than 13,000 newly diagnosed patients. 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 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" <sup>1</sup> which circulate with the lymphatic system.<sup>2</sup> PTCL accounts for between 10-15% of all non-Hodgkin lymphomas (NHLs) and generally affects people aged 60 years and older.<sup>1</sup> 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.<sup>2</sup> There is currently no established standard of care for patients with relapsed or refractory disease.<sup>1</sup>

### **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.<sup>3,4,5</sup> 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.<sup>6</sup> For more information on COPIKTRA, please visit [www.COPIKTRA.com](http://www.COPIKTRA.com). Information about duvelisib clinical trials can be found on [www.clinicaltrials.gov](http://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](http://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 program 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 the risks and uncertainties, among other things, regarding: the commercial success of COPIKTRA in the United States; physician and patient adoption of COPIKTRA, including those related to the safety and efficacy of COPIKTRA; the uncertainties inherent in research and development of COPIKTRA, such as negative or unexpected results of clinical trials; whether and when any applications for COPIKTRA may be filed with regulatory authorities in any other jurisdictions; whether and when regulatory authorities in any other jurisdictions may approve any such other applications that may be filed for COPIKTRA, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted and, if approved, whether COPIKTRA will be commercially successful in such jurisdictions; our ability to obtain, maintain and enforce patent and other intellectual property protection for COPIKTRA and our other product candidates; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of COPIKTRA; the fact that regulatory authorities in the U.S. or other jurisdictions, if approved, could withdraw approval; whether preclinical testing of our product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse for COPIKTRA; that there may be competitive developments affecting our product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that COPIKTRA or our other product candidates will cause unexpected safety events, experience manufacturing or supply interruptions or failures, or result in unmanageable safety profiles as compared to their levels of efficacy; that COPIKTRA will be ineffective at treating patients with lymphoid malignancies; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we may not have sufficient cash to fund our contemplated operations; that we, CSPC Pharmaceutical Group, Yakult Honsha Co., Ltd. or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreements; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will not pursue or submit regulatory filings for our product candidates, including for duvelisib in patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) or indolent non-Hodgkin lymphoma (iNHL) in other jurisdictions; and that our product candidates will not receive regulatory approval, become commercially successful products, or result in new treatment options being offered to patients.

Other risks and uncertainties include those identified under the heading "Risk Factors" in the Company's 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**

<sup>1</sup>The Leukemia & Lymphoma Society. Peripheral T-Cell Lymphoma Facts. July 2014.

<sup>2</sup>Leukemia Foundation. <http://www.leukaemia.org.au/blood-cancers/lymphomas/non-hodgkin-lymphoma-nhl/peripheral-t-cell-lymphoma>

<sup>3</sup>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.

<sup>4</sup>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.

<sup>5</sup>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.

<sup>6</sup>[www.clinicaltrials.gov](http://www.clinicaltrials.gov), NCT03372057

## Verastem, Inc.

### Consolidated Balance Sheets

(in thousands)

	December 31, 2018	December 31, 2017
Cash, cash equivalents and investments	\$ 249,653	\$ 86,672
Accounts receivable, net	306	—
Inventory	327	—
Prepaid expenses and other current assets	2,973	1,115
Property and equipment, net	1,369	861
Intangible assets, net	21,577	—
Other assets	1,031	1,143
<b>Total assets</b>	<b>\$ 277,236</b>	<b>\$ 89,791</b>
Accounts payable, accrued expenses and other current liabilities	\$ 37,077	\$ 17,128
Long-term debt	19,506	14,828
Convertible senior notes	95,231	—
Other liabilities	1,123	151
Stockholders' equity	124,299	57,684
<b>Total liabilities and stockholders' equity</b>	<b>\$ 277,236</b>	<b>\$ 89,791</b>

## Verastem, Inc.

### Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three months ended		Year ended	
	December 31,		December 31,	
	2018	2017	2018	2017
Revenue:				
Product revenue, net	\$ 1,210	\$ —	\$ 1,718	\$ —
License revenue	—	—	25,000	—
Total revenue	1,210	—	26,718	—
Operating expenses:				
Costs of revenues, excluding amortization of acquired intangible assets	116	—	165	—
Research and development	8,762	11,253	43,648	46,423
Selling, general and administrative	26,199	6,799	77,265	21,381
Amortization of acquired intangible assets	392	—	423	—
Total operating expenses	35,469	18,052	121,501	67,804
Loss from operations	(34,259)	(18,052)	(94,783)	(67,804)
Other income	25,556	—	25,556	—

Interest income	1,306	145	2,603	561
Interest expense	(3,952)	(328)	(5,810)	(559)
Net loss	<b>\$ (11,349)</b>	<b>\$ (18,235)</b>	<b>\$ (72,434)</b>	<b>\$ (67,802)</b>
Net loss per share—basic	<b>\$ (0.15)</b>	<b>\$ (0.43)</b>	<b>\$ (1.12)</b>	<b>\$ (1.76)</b>
Net loss per share—diluted	<b>\$ (0.37)</b>	<b>\$ (0.43)</b>	<b>\$ (1.37)</b>	<b>\$ (1.76)</b>
Weighted average common shares outstanding used in computing:				
Net loss per share—basic	<b>73,766</b>	<b>42,027</b>	<b>64,962</b>	<b>38,422</b>
Net loss per share—diluted	<b>91,061</b>	<b>42,027</b>	<b>69,321</b>	<b>38,422</b>

View source version on businesswire.com: <https://www.businesswire.com/news/home/20190312005881/en/>

Source: Verastem, Inc.

*Verastem Oncology:*

Erin S. Cox  
Senior Director, Investor Relations & Corporate Communications  
+1 781-469-1553  
[ecox@verastem.com](mailto:ecox@verastem.com)

*Investors:*

Joseph Rayne  
Argot Partners  
+1 617-340-6075  
[joseph@argotpartners.com](mailto:joseph@argotpartners.com)