# Verastem Oncology Reports First Quarter 2019 Financial Results

May 9, 2019

*Company Reports $1.7 Million in Net Product Revenues from COPIKTRA™; Issues Product Revenue Guidance for 2019*

*Cash, Cash Equivalents and Short-Term Investments of $211.7 Million as of March 31, 2019*

*Company to Host Conference Call Today at 4:30 PM ET*

BOSTON--(BUSINESS WIRE)--May 9, 2019-- Verastem, Inc. (Nasdaq: VSTM), operating as Verastem Oncology, (or "the Company"), focused on developing and commercializing medicines seeking to improve the survival and quality of life of cancer patients, today reported financial results for the three months ended March 31, 2019.

"We are now into the second full quarter of the COPIKTRA launch and sales were up approximately 38% compared to the prior quarter," said Robert Forrester, President and Chief Executive Officer of Verastem Oncology. "We have also made substantial progress securing broader reimbursement for our product, with over 92% of targeted health plans now listing and providing reimbursement for COPIKTRA. We continue to receive positive feedback from physicians using COPIKTRA for the treatment of patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies or follicular lymphoma (FL) after at least two prior systemic therapies."

"The commercial team has been diligently working to enhance physician and advocacy group awareness of COPIKTRA, and to overcome certain historical misperceptions concerning the PI3K class. We believe the groundwork we have laid over the past several months will have an increasingly positive impact through 2019 and into next year. In parallel, we continue to advance duvelisib in additional lines of therapy, both as a monotherapy and in combination, as well as in additional indications like peripheral T-cell lymphoma (PTCL) for which preliminary data are expected by the end of this year," concluded Mr. Forrester.

**Key First Quarter 2019 and Recent Accomplishments:**

***COPIKTRA (duvelisib)***

- ***Launched COPIKTRA in FL*** – In mid-March 2019, upon completion of the required 120-day waiting period following receipt of accelerated approval from the FDA, the Company launched its physician education and marketing campaign for COPIKTRA for the treatment of patients with 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 confirmatory trials, the first of which is expected to start in 2019.
- ***Presented COPIKTRA Data at the 23rd 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.
- ***Phase 2 DYNAMO Study Results Published in the Journal of Clinical Oncology*** – In February 2019, results of the Phase 2 DYNAMO™ study, which evaluated COPIKTRA in patients with indolent non-Hodgkin lymphoma (iNHL) who were refractory to both rituximab and chemotherapy or radioimmunotherapy, was published online in the peer-reviewed Journal of Clinical Oncology. The full manuscript, titled "DYNAMO: A Phase II Study of Duvelisib (IPI-145) in Patients with Refractory Indolent Non-Hodgkin Lymphoma," (Flinn, et al. DOI: 10.1200/JCO.18.00915) can be accessed at www.ascopubs.org.
- ***Continued Commercialization of 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 CLL/SLL after at least two prior therapies. COPIKTRA also received accelerated approval for the treatment of adult patients with relapsed or refractory FL after at least two prior systemic therapies.

  COPIKTRA contains a BOXED WARNING for fatal and/or serious toxicities including 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 other adverse reactions which may also require dose reduction, treatment delay or discontinuation of COPIKTRA.

Please see www.COPIKTRAHCP.com/prescribinginformation for full Prescribing Information including BOXED WARNING and Select Important Safety Information provided below.

***Corporate and Financial***

- ***Chief Commercial Officer Joseph Lobacki to Step Down in 2019*** –Joseph Lobacki, Chief Commercial Officer of Verastem Oncology will be stepping down from the Company in 2019 to pursue other professional opportunities, including Board of Director roles. Mr. Lobacki intends to continue in his role until the Company identifies and appoints a successor. During this transition, Brian Stuglik, RPh, a member of the Company's Board of Directors and the former Chief Marketing Officer of Lilly Oncology, will provide strategic oversight and advisory support for the commercial organization.
- ***Amended Hercules Loan Facility*** – In April 2019, the Company announced an amendment to its existing Loan and Security Agreement with Hercules Capital, Inc., changing key terms of the agreement, including a lower overall interest rate and an extended principal repayment timeline. The amendment also increases the borrowing limit from $50 million to $75 million in financing.

**First Quarter 2019 Financial Results**

Net product revenue for the 2019 Quarter was $1.7 million, which reflects the second full quarter of recorded sales for COPIKTRA. The Company did not have any product revenue for the 2018 Quarter as the FDA approved COPIKTRA on September 24, 2018.

Research and development (R&D) expense for the 2019 Quarter was $9.8 million, compared to $10.9 million for the 2018 Quarter. The decrease of $1.1 million, or 11%, was primarily related to a decrease in consulting fees as a result of activities to file a New Drug Application for COPIKTRA in the 2018 Quarter and lower R&D costs associated with the development of COPIKTRA as a result of site closures in the Company's Phase 3 DUO and Phase 2 DYNAMO studies throughout 2018 and 2019 as patients continue to complete treatment. All of these lower costs were partially offset by an increase in costs related to the Phase 2 PRIMO study for the treatment of patients with relapsed or refractory PTCL.

Selling, general and administrative expense for the 2019 Quarter was $26.0 million, compared to $9.8 million for the 2018 Quarter. The increase of $16.2 million, or 165%, was primarily due to higher personnel and related costs, as well as promotional and consulting costs in support of the launch of COPIKTRA.

Net loss for the three months ended March 31, 2019 (2019 Quarter) was $38.1 million, or $0.52 per share (basic and diluted), as compared to $21.1 million, or $0.41 per share (basic and diluted), for the three months ended March 31, 2018 (2018 Quarter). In addition, net loss includes non-cash stock-based compensation expense of $2.2 million and $1.3 million for the 2019 and 2018 Quarters, respectively.

As of March 31, 2019, Verastem Oncology had cash, cash equivalents and short-term investments of $211.7 million.

**Financial Outlook**

For 2019, the Company expects net product revenue from sales of COPIKTRA to be in the range of $10-12 million, based on product revenue to date, current run rates and near-term expectations.

**Conference Call and Webcast Information**

The Verastem Oncology management team will host a conference call and webcast today, Thursday, May 9, 2019, at 4:30 PM (ET). The call can be accessed by dialing (877) 341-5660 (U.S. and Canada) or (315) 625-3226 (international), five minutes prior to the start of the call and providing the passcode 7199457.

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.

**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" [1] which circulate with the lymphatic system.[2] PTCL accounts for between 10-15% of all non-Hodgkin lymphomas (NHLs) and generally affects people aged 60 years and older.[1] Although there are many different subtypes of peripheral T-cell lymphoma, they often present in a similar way, with widespread, enlarged, painless lymph nodes in the neck, armpit or groin.[2] There is currently no established standard of care for patients with relapsed or refractory disease.[1]

**About COPIKTRA™ (duvelisib)**

COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-cells. PI3K signaling may lead to the proliferation of malignant B-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.[3,4,5] COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. COPIKTRA is also being developed by Verastem Oncology for the treatment of peripheral T-cell lymphoma (PTCL), for which it has received Fast Track status, and is being investigated in combination with other agents through investigator-sponsored studies.[6] For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

**About Verastem Oncology**

Verastem Oncology (Nasdaq: VSTM) is a commercial biopharmaceutical company committed to the development and commercialization of medicines to improve the lives of patients diagnosed with cancer. We are driven by the strength, tenacity and courage of those battling cancer – single-minded in our resolve to deliver new therapies that not only keep cancer at bay, but improve the lives of patients diagnosed with cancer. Because for us, it's personal.

Our first FDA approved product is now available for the treatment of patients with certain types of indolent non-Hodgkin's lymphoma (iNHL). Our pipeline comprises product candidates that seek to treat cancer by modulating the local tumor microenvironment. For more information, please visit www.verastem.com.

**COPIKTRA™ (duvelisib) – Select 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**

- Hepatotoxicity: Monitor hepatic function.
- Neutropenia: Monitor blood counts.
- Embryo-Fetal toxicity: COPIKTRA can cause fetal harm. Advise patients of potential risk to a fetus and to use effective contraception.

**ADVERSE REACTIONS:** The most common adverse reactions (> 20%) are diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain, and anemia.

To report SUSPECTED ADVERSE REACTIONS, contact Verastem, Inc. (Verastem) at 877-7RXVSTM or 1-877-779-8786, or U.S. Food and Drug Administration (FDA) at 1-800-FDA-1088 or www.fda.gov/medwatch.

**DRUG INTERACTIONS**

- CYP3A inducers: Avoid co-administration with strong CYP3A inducers.
- CYP3A inhibitors: Monitor for COPIKTRA toxicities when co-administered with strong or moderate CYP3A inhibitors. Reduce COPIKTRA dose to 15 mg twice daily when co-administered with strong CYP3A4 inhibitors.
- CYP3A substrates: Monitor for signs of toxicities when co-administering COPIKTRA with sensitive CYP3A substrates.

**See full Prescribing Information, including Boxed Warning, at www.COPIKTRA.com**

**Forward looking statements notice**

This press release and the commentary in the conference call to be held today each include 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, including financial guidance and patient population estimates, 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 Annual Report on Form 10-K for the year ended December 31, 2018 as filed with the SEC on March 12, 2019 and in any subsequent filings with the SEC. The forward-looking statements contained in this press release reflect Verastem Oncology's views as of the date hereof, and the Company does not assume and specifically disclaims any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by law.

**References**

[1]The Leukemia & Lymphoma Society. Peripheral T-Cell Lymphoma Facts. July 2014.

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

[3] Winkler D.G., Faia K.L., DiNitto J.P. et al. PI3K-delta and PI3K-gamma inhibition by IPI-145 abrogates immune responses and suppresses activity in autoimmune and inflammatory disease models. Chem Biol 2013; 20:1-11.

[4] Reif K et al. Cutting Edge: Differential Roles for Phosphoinositide 3 kinases, p110-gamma and p110-delta, in lymphocyte chemotaxis and homing. J Immunol 2004:173:2236-2240.

[5] Schmid M et al. Receptor Tyrosine Kinases and TLR/IL1Rs Unexpectedly activate myeloid cell PI3K, a single convergent point promoting tumor inflammation and progression. Cancer Cell 2011;19:715-727.

[6]www.clinicaltrials.gov, NCT03372057

**Verastem, Inc.**

**Condensed Consolidated Balance Sheets**

(in thousands)

| | March 31, 2019 | December 31, 2018 |
|---|---|---|
| Cash, cash equivalents and investments | $ 211,659 | $ 249,653 |
| Accounts receivable, net | 558 | 306 |
| Inventory | 306 | 327 |

| | | |
|---|---|---|
| Prepaid expenses and other current assets | 4,073 | 2,973 |
| Property and equipment, net | 1,256 | 1,369 |
| Intangible assets, net | 21,185 | 21,577 |
| Other assets | 4,328 | 1,031 |
| **Total assets** | **$ 243,365** | **$ 277,236** |
| | | |
| Current Liabilities | $ 28,624 | $ 37,077 |
| Long-term debt | 24,814 | 19,506 |
| Convertible senior notes | 97,159 | 95,231 |
| Other liabilities | 4,308 | 1,123 |
| Stockholders' equity | 88,460 | 124,299 |
| **Total liabilities and stockholders' equity** | **$ 243,365** | **$ 277,236** |

**Verastem, Inc.**

**Unaudited Condensed Consolidated Statements of Operations**

(in thousands, except per share amounts)

| | **Three months ended March 31,** | |
|---|---|---|
| | **2019** | **2018** |
| Revenue: | | |
| Product revenue, net | $ 1,671 | $ — |
| Total revenue | 1,671 | — |
| Operating expenses: | | |
| Costs of revenues, excluding amortization of acquired intangible assets | 158 | — |
| Research and development | 9,758 | 10,934 |
| Selling, general and administrative | 26,033 | 9,827 |
| Amortization of acquired intangible assets | 392 | — |
| Total operating expenses | 36,341 | 20,761 |
| Loss from operations | (34,670) | (20,761) |
| Interest income | 1,497 | 191 |
| Interest expense | (4,929) | (480) |
| Net loss | **$ (38,102)** | **$ (21,050)** |
| Net loss per share—basic and diluted | **$ (0.52)** | **$ (0.41)** |
| | | |
| Weighted average common shares outstanding used in computing net loss per share—basic and diluted | **73,854** | **50,835** |

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