Verastem Oncology Outlines Strategic Priorities for 2019 and Highlights Recent Progress

January 3, 2019

2018 Highlighted by Approval and Commercial Launch of Lead Asset COPIKTRA™

Entering 2019 with a Strong Balance Sheet to Achieve Corporate Objectives

Company's Focus in 2019 Will Be on Continued Investigation of Duvelisib in Additional Tumor Types, Including Peripheral T-Cell Lymphoma, Both as a Monotherapy and in Combination with Other Anti-Cancer Agents

BOSTON--(BUSINESS WIRE)--Jan. 3, 2019-- Verastem, Inc. (Nasdaq: VSTM) (Verastem Oncology or the Company), a biopharmaceutical company focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients, today highlighted the company's recent progress and outlined strategic priorities for 2019.

"2018 was a pivotal year for Verastem Oncology, as the U.S. Food and Drug Administration's (FDA) approval of COPIKTRA™ and other key accomplishments strongly positioned us to execute on our 2019 corporate priorities that are focused on increasing revenues, initiating additional clinical studies of COPIKTRA and advancing our pipeline," said Robert Forrester, President and Chief Executive Officer of Verastem Oncology. "We are pleased with the strong vote of confidence we have received in duvelisib, including validating licensing agreements in key Asian markets, recognition of our pivotal Phase 3 data in the medical journal *Blood***,** and more. We are also entering 2019 with a strong balance sheet derived from the successful completion of multiple financing transactions, which we believe provides us with important financial strength to achieve our planned corporate objectives. We look forward to keeping the momentum going, and to sharing ongoing updates on our progress."

"Since the launch of COPIKTRA, we've been encouraged by the positive feedback we are hearing from physicians and other healthcare providers about this important new oral monotherapy within the treatment landscape," said Joseph Lobacki, Executive Vice President and Chief Commercial Officer of Verastem Oncology. "Following the approval, COPIKTRA was quickly added to the National Comprehensive Cancer Network® (NCCN) guidelines, which has led to its inclusion on formularies and extensive reimbursement coverage, including on the top national health plans, reaching approximately 75% of U.S. Pharmacy lives and providing critical access to treatment for appropriate patients. In 2019, the commercial team will be diligently working to engage with physicians and other health care professionals to focus on ensuring COPIKTRA reaches the patients who need it."

2018 Accomplishments

- **COPIKTRA (duvelisib) Capsules Approved by the U.S. FDA** – On September 24, 2018, the 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 a confirmatory trial. The commercial launch of COPIKTRA is ongoing.

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

  Additionally, use of COPIKTRA is also associated with adverse reactions which may require dose reduction, treatment delay or discontinuation of COPIKTRA.

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

- **Established a Commercial Franchise in the U.S.** – In 2018, the Company established a full commercial infrastructure in the U.S. The sales, market access and medical affairs teams are fully deployed and calling on medical institutions, oncology healthcare professionals and payors in support of the COPIKTRA launch. COPIKTRA product was available at specialty distributors and specialty pharmaceutical providers immediately following approval. Top national health plans are now offering reimbursement coverage and the majority of COPIKTRA sales territories have patients being treated. The Company also successfully launched Verastem Cares™, a comprehensive, personalized program designed to provide information and assistance to patients who have been prescribed COPIKTRA, which is now fully operational nationwide.
- **COPIKTRA Added to NCCN Guidelines for CLL/SLL and FL** – 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.

- **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 at al). The publication was accompanied by a review article by Jennifer R. Brown, M.D., Ph.D., Director of the Chronic Lymphocytic Leukemia Center at Dana-Farber Cancer Center, 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.
- **Eight Abstracts Presented at the American Society of Hematology 2018 Annual Meeting (ASH 2018)** – The Company presented eight abstracts, including one oral presentation, at ASH 2018 in San Diego. The oral presentation highlighted data from the Phase 1 study evaluating duvelisib in combination with romidepsin in relapsed or refractory peripheral T-cell lymphoma. Additional poster presentations showcased preclinical and clinical data reinforcing the potential of duvelisib.
- **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, 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 million and is entitled to receive aggregate payments of up to $160 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 million, includes a one-time upfront payment of $10 million and up to an additional $90 million if certain future pre-specified 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 duvelisib in Japan.
- **Collaboration with The Leukemia & Lymphoma Society for Development of Duvelisib in Peripheral T-Cell Lymphoma** – 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.
- **Entering 2019 with a Strong Balance Sheet**– In May 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. The Company also raised funds through the sale of shares of common stock under its at-the-market equity offering program. The Company has approximately $279 million in cash and cash equivalents pro-forma to the close of the third quarter of 2018[1].

2019 Priorities

Verastem Oncology's 2019 focus is to execute on business priorities aimed at increasing the company's sales and revenues:

- Continuing to expand on the commercial traction of COPIKTRA in CLL/SLL and FL for appropriate patients;
- Expansion of the open-label, multicenter, Phase 2 clinical trial (the PRIMO study) evaluating the efficacy and safety of duvelisib monotherapy in adult patients with histologically confirmed relapsed or refractory PTCL. This study is expected to enroll approximately 120 patients;
- Initiating a confirmatory Phase 3 study evaluating duvelisib for the treatment of patients with relapsed or refractory FL after at least two prior systemic therapies. The confirmatory study is expected to start in the second half of 2019;
- Initiating additional investigational studies of duvelisib as a monotherapy and in combination with other anti-cancer agents, such as checkpoint inhibitors, in both hematological and solid tumor malignancies;
- Working with the LLS to advance the PTCL program including the expansion of the Phase 2 combination study of duvelisib and romidepsin for patients with relapsed or refractory PTCL;
- Additional ex-U.S. partnerships for duvelisib;
- Presenting and publishing additional duvelisib data; and
- Advancing the Company's focal adhesion kinase (FAK) inhibitor defactinib, which is designed to treat cancer through modulation of the tumor microenvironment and enhancement of anti-tumor immunity. Defactinib is currently being evaluated in three separate clinical collaborations in combination with immunotherapeutic agents for the treatment of several different cancer types including pancreatic cancer, non-small cell lung cancer (NSCLC), and mesothelioma.

For more information about Verastem Oncology, including its leadership, product and pipeline, please visit 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. (6)

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.

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 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" [2] which circulate with the lymphatic system.[3] PTCL accounts for between 10-15% of all non-Hodgkin lymphomas (NHLs) and generally affects people aged 60 years and older.[2] 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.[3] There is currently no established standard of care for patients with relapsed or refractory disease.[2]

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.[4,5,6] 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.[7] For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

About Defactinib

Defactinib is an investigational inhibitor of focal adhesion kinase (FAK), a non-receptor tyrosine kinase that mediates oncogenic signaling in response to cellular adhesion and growth factors.[8] Based on the multi-faceted roles of FAK, defactinib is used to treat cancer through modulation of the tumor microenvironment and enhancement of anti-tumor immunity.[9,10] Defactinib is currently being evaluated in three separate clinical collaborations in combination with immunotherapeutic agents for the treatment of several different cancer types including pancreatic cancer, ovarian cancer, non-small cell lung cancer (NSCLC), and mesothelioma. These studies are combination clinical trials with pembrolizumab and avelumab from Merck & Co. and Pfizer/Merck KGaA, respectively.[11,12,13] Information about these and additional clinical trials evaluating the safety and efficacy of defactinib 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 duvelisib, and Verastem Oncology's PI3K and FAK programs generally, its intent to commercialize duvelisib, the potential commercial success of duvelisib, the anticipated adoption of duvelisib 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 commercial success of duvelisib in the United States; uncertainties regarding physician and patient adoption of duvelisib, including those related to the safety and efficacy of duvelisib; the uncertainties inherent in research and development of duvelisib, such as negative or unexpected results of clinical trials; whether and when any applications for duvelisib 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 duvelisib, 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 duvelisib will be commercially successful in such jurisdictions; Verastem Oncology's ability to obtain, maintain and enforce patent and other intellectual property protection for duvelisib 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 duvelisib; 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 duvelisib; that there may be competitive developments affecting its product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that duvelisib 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 duvelisib 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.

References

[1] Pro-forma cash and cash equivalents represents cash and cash equivalents at September 30, 2018 of $145.6 million, plus estimated net proceeds of $145.1 million from the issuance of our 5.00% Convertible Senior Notes in October 2018, and the remaining $10.0 million of the $15.0M non-refundable upfront payment due from CSPC pursuant to the exclusive license agreement executed in September 2018, less the $22.0 million payment the Company owes to Infinity Pharmaceuticals, Inc. pursuant to the terms of the amended and restated license agreement.

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[7] www.clinicaltrials.gov, NCT03372057

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[10] Sulzmaier F.J. et al. FAK in cancer: mechanistic findings and clinical applications. Nature Rev Cancer. 2014 14: 598-610.

[11] www.clinicaltrials.gov, NCT02546531

[12] www.clinicaltrials.gov, NCT02943317

[13] www.clinicaltrials.gov, NCT02758587

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