# Verastem Oncology to Present Phase 2 PRIMO Study Data Evaluating Duvelisib in Relapsed or Refractory Peripheral T-Cell Lymphoma at the American Society of Hematology 2019 Annual Meeting

November 6, 2019

*Results from the Dose Optimization Portion of the Study to be Presented*

*PRIMO Expansion Phase To Investigate Duvelisib 75mg Twice Daily for Two Cycles, Followed by 25mg Twice Daily*

*Four Additional Abstracts Selected for Presentation, Including Results from a Phase 1 Study Investigating Duvelisib in Combination with Venetoclax in Patients with Relapsed or Refractory CLL/SLL*

BOSTON--(BUSINESS WIRE)--Nov. 6, 2019-- Verastem, Inc. (Nasdaq:VSTM) (Verastem Oncology or the Company), a biopharmaceutical company focused on developing and commercializing medicines seeking to improve the survival and quality of life of cancer patients, today announced that five abstracts highlighting data for COPIKTRA™ (duvelisib) have been selected for presentation at the upcoming American Society of Hematology 2019 Annual Meeting taking place December 7-10, 2019, in Orlando.

"We are pleased to share additional data on duvelisib across multiple areas of research that continues to expand our understanding of its potential benefit and utility for patients with certain types of blood cancers," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "The results of the dose optimization portion of the PRIMO study provide important guidance to support our ongoing evaluation of duvelisib for the treatment of relapsed or refractory PTCL."

A key abstract at the meeting will feature clinical data from the dose optimization portion of the registration-directed Phase 2 PRIMO study evaluating duvelisib in patients with relapsed or refractory peripheral T-cell lymphoma (PTCL). This open-label, multicenter trial is currently ongoing and is expected to enroll approximately 120 patients. In the dose optimization portion of the study, patients were randomized to receive duvelisib 25mg twice daily with an option for dose escalation (cohort 1) or duvelisib 75mg twice daily continuously (cohort 2) until disease progression or unacceptable toxicity (cycle=28 days).

The primary endpoint of the dose optimization portion is investigator-assessed overall response rate (ORR). Based on the efficacy and safety data to be reported at the meeting, the investigators have elected to investigate duvelisib starting at 75mg twice daily for two cycles, followed by 25mg twice daily, during the dose expansion portion of the study which is currently ongoing. Updated data from those shown in the abstract will be presented at the meeting. Duvelisib is not approved for the treatment of PTCL.

"Identifying additional options for this aggressive type of T-cell lymphoma is critical. In the initial cohort of this trial, we identified that the dose of 75mg twice daily for two cycles helped to achieve more rapid tumor control in what are often aggressive diseases. We will assess if following the induction by 25mg twice daily dose we are able to maintain longer-term disease control and mitigate the potential for later onset toxicities in patients with relapsed or refractory PTCL," said Steven Horwitz, MD, Memorial Sloan Kettering Cancer Center, and principal investigator of Phase 2 PRIMO study. "We look forward to sharing updated data from this ongoing study at ASH 2019."

Other abstracts at the meeting include: Preliminary results from a Phase 1 study investigating duvelisib in combination with venetoclax in patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL); an analysis of cytogenetic and molecular markers associated with improved outcomes in the Phase 3 DUO study; and a description of the [soon to be initiated] TEMPO study investigating an intermittent dosing regimen of duvelisib in patients with indolent non-Hodgkin lymphoma. Preclinical data highlighting the effectiveness of duvelisib in combination with venetoclax in Richter syndrome will also be presented.

**Details for the ASH 2019 presentations are as follows:**

***Poster Presentations***

**Title:** Dose Optimization of Duvelisib in Patients with Relapsed or Refractory Peripheral T-Cell Lymphoma from the Phase 2 PRIMO Trial: Selection of Regimen for the Dose-Expansion Phase
**Lead author:** Steven Horwitz, Memorial Sloan Kettering Cancer Center
**Poster #:** 1567
**Session:** 624. Hodgkin Lymphoma and T/NK Cell Lymphoma – Clinical Studies: Poster I
**Date and Time:** Saturday, December 7, 2019; 5:30-7:30PM ET
**Location:** Orange County Convention Center, Hall B

**Title:** A Phase I Study of Duvelisib and Venetoclax in Patients with Relapsed or Refractory CLL / SLL
**Lead author:** Jennifer Crombie, Dana-Farber Cancer Institute
**Poster #:** 1763
**Session:** 642. CLL: Therapy, excluding Transplantation: Poster I
**Date and Time:** Saturday, December 7, 2019; 5:30-7:30 PM ET
**Location:** Orange County Convention Center, Hall B

**Title:** Cytogenetic and Molecular Marker Associations to Outcomes with Duvelisib and Ofatumumab Treatment in Patients with Relapsed or Refractory CLL/SLL in the DUO Trial
**Lead author:** Jennifer Brown, Dana-Farber Cancer Institute

**Poster #:** 4312
**Session:** 642. CLL: Therapy, excluding Transplantation: Poster III
**Date and Time:** Monday, December 9, 2019; 6:00-8:00 PM ET
**Location:** Orange County Convention Center, Hall B

**Title:** The Dual PI3K-δ/γ Inhibitor Duvelisib in Combination with the Bcl-2 Inhibitor Venetoclax Shows Promising Responses in Richter Syndrome-PDX Models
**Lead author:** Andrea Iannello, Università degli Studi di Torino
**Poster #:** 2862
**Session:** 625. Lymphoma: Pre-Clinical—Chemotherapy and Biologic Agents: Poster II
**Date and Time:** Sunday, December 8, 6:00-8:00 PM ET
**Location:** Orange County Convention Center, Hall B

***Publication Only Presentations***

**Title:** *Trial in Progress (TiP)**:*** A Phase 2, Randomized, Open-Label, 2-Arm Study Comparing 2 Intermittent Dosing Schedules of Duvelisib in Patients with Indolent Non-Hodgkin Lymphoma (iNHL) (TEMPO)
**Lead author:** Reem Karmali, Lurie Cancer Center, Northwestern University
**Session:** 623. Mantle cell, follicular, and other indolent B Cell Lymphoma – Clinical studies

PDF copies of these poster presentations will be available **here** after the meeting.

COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL)_after at least two prior therapies and in relapsed or refractory follicular lymphoma (FL) after at least two prior system therapies. Accelerated approved in FL was based on overall response rate and continued approval may be contingent upon confirmatory trials.

COPIKTRA includes a Boxed Warning for fatal and serious toxicities including infections, diarrhea or colitis, cutaneous reactions and pneumonitis. See full Prescribing Information for complete Boxed Warning and other important safety information.

**SELECT IMPORTANT SAFETY INFORMATION**

This does not include all information needed to use COPIKTRA™ (duvelisib) safely and effectivelySee full Prescribing 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% (4% fatal) 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% (<1% fatal) of COPIKTRA-treated patients. Monitor for the development of severe diarrhea or colitis. Withhold COPIKTRA.
- Fatal and/or serious cutaneous reactions occurred in 5% (<1% fatal) of COPIKTRA-treated patients. Withhold COPIKTRA.
- Fatal and/or serious pneumonitis occurred in 5% (<1% fatal) 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 Adverse Reactions, contact FDA at 1-800-FDA-1088 (1-800-332-1088) or www.fda.gov/medwatch and Verastem Oncology at 1-877-7RXVSTM (1-877-779-8786).**

**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.

**USE IN SPECIFIC POPULATIONS**
Lactation: Advise women not to breastfeed.

**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.[1,2,3] 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 Orphan Drug Designation, and is being investigated in combination with other agents through investigator-sponsored studies.[4] For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

**About Verastem Oncology**

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

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

**Forward looking statements notice**

This press release includes forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995 that are subject to risks, uncertainties and other factors, 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; that enrollment of clinical trials may take longer than expected; and 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. These risks, uncertainties and other factors could cause actual results to differ materially from those referred to in the forward-looking statements. The reader is cautioned not to rely on these forward-looking statements.

Other risks and uncertainties include those identified under the heading "Risk Factors" in the Company's Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2019, as filed with the Securities and Exchange Commission (SEC) on August 1, 2019, 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**

[1] 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.
[2] 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.
[3] 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.
[4] www.clinicaltrials.gov, NCT03372057.

*Dr. Horwitz has been compensated for consulting services by Verastem Oncology and also receives research support from the company.*

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Source: Verastem, Inc.

*Investors:*
John Doyle
Vice President, Investor Relations & Finance
+1 781-469-1546
jdoyle@verastem.com

*Media:*
Lisa Buffington

Corporate Communications
+1 781-292-4205
lbuffington@verastem.com