



Verastem Oncology Reports on Chronic Lymphocytic Leukemia/Small Lymphocytic Lymphoma and Follicular Lymphoma Opportunity, Landscape and Advancements in Pre-Commercial Initiatives at Analyst and Investor Day

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Duvelisib is Well Positioned to Meet the Evolving Unmet Need in CLL/SLL and FL

Company is Laying the Foundation to Optimize the Launch and Commercial Value of Duvelisib

BOSTON--(BUSINESS WIRE)--May 3, 2018-- Verastem, Inc. (NASDAQ:VSTM) (Verastem Oncology or the Company), focused on developing and commercializing drugs to improve the survival and quality of life of cancer patients, yesterday hosted in New York City an Analyst and Investor Day, titled, "Duvelisib: Harnessing the Power of Dual PI3K Inhibition."

The program featured key opinion leaders in the hematologic oncology field: **Jennifer Brown, MD, PhD**, Associate Professor of Medicine, Harvard Medical School Director, and Director, CLL Center of the Division of Hematologic Malignancies, Dana-Farber Cancer Institute; **Ian Flinn, MD, PhD**, Director, Blood Cancer Research Program at Sarah Cannon Research Institute, and Lead Investigator of the DUO and DYNAMO Studies; **Steven Horwitz, MD**, Medical Oncologist, Memorial Sloan Kettering Cancer Center and NYC Health + Hospitals/Bellevue; **Brian Koffman, MDCM, DCFP, FCFP, DABFP, MSED**, Physician, Medical Director of the Chronic Lymphocytic Leukemia (CLL) Society and CLL Patient; and **Lori Kunkel, MD**, Former Chief Medical Officer, Pharmacyclics.

These leading experts in hematologic malignancies, including chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL), follicular lymphoma (FL), and peripheral T-cell lymphoma (PTCL), provided an in-depth discussion regarding the current U.S. treatment landscape, where phosphoinositide-3-kinase (PI3K)- inhibitors fit into the treatment paradigm and the need for new anti-cancer agents such as duvelisib, the Company's first-in-class oral dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma. Joe Lobacki, Chief Commercial Officer of Verastem, outlined the Company's commercialization strategy and plans, including an overview of ongoing pre-commercial and launch initiatives, in preparation for the potential launch of duvelisib in the U.S. following the assigned target action date of October 5, 2018. Following the presentations, Robert Forrester, President and Chief Executive Officer of Verastem, moderated an expert panel discussion which highlighted the unmet need in CLL/SLL, FL and T-cell lymphomas, the clinical utility of PI3K-inhibitors and in particular duvelisib, as well as current treatment practices and their perspectives on the medical need for new treatment options.

"At Verastem Oncology, we care differently. We are driven by the strength, tenacity and courage of those battling cancer and are single-minded in our resolve to deliver new therapies to patients in need," said Robert Forrester, President and Chief Executive Officer of Verastem. "I want to extend my thanks to the outstanding panel of experts for their presentations and thoughtful discussion. Their comments further reinforce our belief in the unmet need of CLL/SLL and FL patients, the importance of PI3K inhibitors, as well as the gap that duvelisib will fill, if approved."

Presentation Highlights

Brian Koffman, MDCM, DCFP, FCFP, DABFP, MSED, Physician, Founder & President of the CLL Society, and CLL patient kicked off the morning by taking the audience through the CLL patient journey, along with the learnings and challenges of living with high risk disease. Dr. Koffman, who was diagnosed twelve years ago with CLL, highlighted his perspective on what patients want, "I believe that more targeted options are needed for relapsed patients and therapy should be matched to each individual's profile and preference."

Lori Kunkel, MD, former CMO at Pharmacyclics with global approval of cancer therapeutic IMBRUVICA®, presented the evolving landscape of therapies for hematologic malignancies. As Dr. Kunkel noted, there are a number of factors to consider when treating patients with CLL/SLL or FL, including resistance or intolerance to first-line therapies, patient sub-types, and comorbidities. She went on to illustrate that every patient is different, concluding that there is no single solution for all patients and more options, like duvelisib, are needed for these incurable diseases.

Jennifer Brown, MD, PhD, Director of the CLL Center of the Division of Hematologic Malignancies, Dana-Farber Cancer Institute, and Associate Professor of Medicine at Harvard Medical School, gave a compelling presentation on the role of PI3K inhibitors as treatment evolves towards a chemo-free future for many patients with B-Cell malignancies. "While there are other, efficacious targeted therapies available, each comes with its own limitations," noted Dr. Brown. Her presentation focused on the role of the PI3K inhibitor, the large and growing population of patients intolerant of BTK inhibitors, particularly older patients, and a steadily increasing population progressing on BTK and BCL-2 inhibitors. "While venetoclax is an effective therapy, it can be very challenging to give in a community setting. Duvelisib has a novel mechanism that is easily given with no infusions required. This may provide a benefit to older patients in the community, which represents the majority of the patients," she concluded.

Ian Flinn, MD, PhD, Director, Blood Cancer Research Program at Sarah Cannon Research Institute was the lead Investigator of the DUO™ and DYNAMO™ studies, the basis for the New Drug Application for duvelisib. "In the Phase 3 DUO study, oral duvelisib monotherapy achieved a statistically significant improvement in progression-free survival (PFS) versus the approved standard of care treatment ofatumumab, along with a well characterized and manageable safety profile, in patients with previously treated CLL/SLL," noted Dr. Flinn. During an overview of the Phase 2 DYNAMO data, Dr. Flinn commented, "The clinical activity and durability of responses observed in the DYNAMO study, seen across highly refractory disease subtypes such as FL, highlight the potential of this drug in lymphoid malignancies. These results were seen in patients who were refractory to both rituximab and chemotherapy, a specific population with unmet medical need." Dr. Flinn concluded by saying, "Additional options are needed for a

physician's armamentarium in the treatment of chronic indolent lymphomas and leukemias and the sequential use of clinically manageable treatments may extend the period of disease control. Continued development of oral, targeted therapies such as duvelisib, is necessary to address the medical unmet need. The DYNAMO and DUO results support duvelisib oral monotherapy as a potential new and convenient treatment option for previously treated CLL/SLL or FL patients."

Dr. Steve Horwitz, Medical Oncologist at Memorial Sloan Kettering, presented an overview of the unmet need for new strategies in T-cell lymphoma. He presented encouraging Phase 1 clinical data where duvelisib demonstrated a 50% investigator-assessed overall response rate in 16 heavily pre-treated patients with relapsed or refractory PTCL, including a 19% complete response rate and a 31% partial response rate. Dr. Horwitz also presented data showing that oral duvelisib, in combination with either romidepsin or bortezomib, has an acceptable safety profile in patients with relapsed or refractory TCL with meaningful response rates, noting, "While still preliminary, the results appear promising when compared to those seen with currently approved therapies. We were especially pleased to see that these response rates were even higher in patients with PTCL, a rare and aggressive type of non-Hodgkin lymphoma." Dr. Horwitz is an investigator on PRIMO, an open-label, multicenter, Phase 2 clinical trial evaluating the efficacy and safety of duvelisib in patients with relapsed or refractory PTCL that is currently being initiated in the US, EU and Japan.

Mr. Lobacki, former Chief Commercial Officer at Medivation, concluded the presentations by highlighting the current limited options for CLL/SLL and FL patients and where duvelisib meets the unmet needs of both patients and physicians. He provided an overview of how duvelisib could potentially fit into the treatment landscape, given its profile as a first in class dual PI3K inhibitor with demonstrated clinical efficacy in relapsed lymphomas, a single CLL/SLL and FL therapy option with a safety profile that is well characterized and manageable, a chemo-free option that has shown signs of clinical efficacy regardless of tumor burden or genetic alterations, and a daily oral monotherapy that can be taken at home with no planned hospitalization or infusions required. Mr. Lobacki also outlined Verastem's plan to commercialize duvelisib in the U.S. including sales force size, geographic coverage, reimbursement and access as well as plans to prioritize a seamless patient experience. "With this go-to-market plan and this commercial launch, we anchor duvelisib in relapsed CLL and FL. However, this is just the start, as we have a strong foundation for future growth into other important markets," he concluded.

At the Analyst and Investor Day event, the Company also announced that it will change its name to Verastem Oncology. Shares of the Company's common stock will continue to trade on the Nasdaq Global Market under the symbol "VSTM." The name change reinforces Verastem's commitment to advance innovative treatment options to improve the lives of patients battling cancer.

An archived webcast of the event is available on the "Events and Presentations" page in the "Investors" section of the Company's website at www.verastem.com.

About Duvelisib

Duvelisib is a first-in-class investigational oral, dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-cells and T-cells. PI3K signaling may lead to the proliferation of malignant B- and T-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.^{1,2,3} Duvelisib was evaluated in late- and mid-stage extension trials, including DUO™, a randomized, Phase 3 monotherapy study in patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL),⁴ and DYNAMO™, a single-arm, Phase 2 monotherapy study in patients with refractory indolent non-Hodgkin lymphoma (iNHL).⁵ Both DUO and DYNAMO achieved their primary endpoints. Verastem Oncology's New Drug Application (NDA) requesting the full approval of duvelisib for the treatment of patients with relapsed or refractory CLL/SLL, and accelerated approval for the treatment of patients with relapsed or refractory follicular lymphoma (FL) was accepted for filing by the U.S. Food and Drug Administration (FDA), granted Priority Review and assigned a target action date of October 5, 2018. Duvelisib is also being developed by Verastem Oncology for the treatment of peripheral T-cell lymphoma (PTCL), and is being investigated in combination with other agents through investigator-sponsored studies.⁶ Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

About Verastem Oncology

Verastem, (NASDAQ:VSTM), operating as Verastem Oncology, is a biopharmaceutical company focused on developing and commercializing drugs to improve the survival and quality of life of cancer patients. Verastem Oncology is currently developing duvelisib, a dual inhibitor of PI3K-delta and PI3K-gamma, which has successfully met its primary endpoint in a Phase 2 study in indolent Non-Hodgkin Lymphoma (iNHL) and a Phase 3 clinical trial in patients with chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL). Verastem Oncology's New Drug Application (NDA) requesting the full approval of duvelisib for the treatment of patients with relapsed or refractory CLL/SLL, and accelerated approval for the treatment of patients with relapsed or refractory follicular lymphoma (FL) was accepted for filing by the U.S. Food and Drug Administration (FDA), granted Priority Review and assigned a target action date of October 5, 2018. In addition, Verastem Oncology is developing the FAK inhibitor defactinib, which 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. Verastem Oncology's product candidates seek to treat cancer by modulating the local tumor microenvironment and enhancing anti-tumor immunity. 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 investigational product candidates, including duvelisib and defactinib, and Verastem Oncology's PI3K and FAK programs generally, the structure of our planned and pending clinical trials, Verastem Oncology's financial guidance and the timeline and indications for clinical development and regulatory submissions. 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 that approval of Verastem Oncology's New Drug Application for duvelisib will not occur on the expected timeframe or at all, including by the U.S. Food and Drug Administration's target action date; that a filing of a European Marketing Application may not be achieved in fiscal year 2018 or at all; that even if data from clinical trials is positive, regulatory authorities may require additional studies for approval and the product may not prove to be safe and effective; that the preclinical testing of Verastem Oncology's product candidates and preliminary or interim data from clinical trials may not be predictive of the results or success of ongoing or later clinical trials; that the full data from the DUO™ study will not be consistent with the

previously presented results of the study; that data may not be available when expected, including for the Phase 3 DUO study; that the degree of market acceptance of product candidates, if approved, may be lower than expected; that the timing, scope and rate of reimbursement for our product candidates is uncertain; 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 our product candidates will cause unexpected safety events or result in an unmanageable safety profile as compared to their level 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 iNHL; 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 Annual Report on Form 10-K for the year ended December 31, 2017 as filed with the Securities and Exchange Commission (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

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Marianne M. Lambertson, +1 781-292-4273
Vice President, Corporate Communications
Investor Relations/Public Relations
mlambertson@verastem.com