



## Verastem Expands Duvelisib Development Program to Include Peripheral T-Cell Lymphoma

September 6, 2017

*Duvelisib Receives Fast Track Designation from FDA in PTCL; Company to Initiate a Phase 2 Clinical Trial by Year End 2017*

*Former Chief Marketing Officer, Lilly Oncology, Brian Stuglik Joins the Company's Board of Directors*

*Conference Call Scheduled for Today at 8:00 AM ET*

BOSTON--(BUSINESS WIRE)--Sep. 6, 2017-- Verastem, Inc. (NASDAQ:VSTM), focused on discovering and developing drugs to improve the survival and quality of life of cancer patients, today announced the expansion of its duvelisib development program to include targeting the treatment of patients with Peripheral T-Cell Lymphoma (PTCL). Duvelisib has been granted Fast Track designation by the U.S. Food & Drug Administration (FDA) for the treatment of patients with PTCL who have received at least one prior therapy. Duvelisib, Verastem's lead drug candidate, is an oral inhibitor of phosphoinositide-3-kinase (PI3K)-delta and PI3K-gamma being investigated for the treatment of hematologic cancers, including chronic lymphocytic leukemia (CLL)/small lymphocytic lymphoma (SLL), indolent non-Hodgkin lymphoma (iNHL) and other T cell lymphomas.

Development of duvelisib in PTCL is supported by compelling Phase 1 clinical data which demonstrated a 50% investigator-assessed overall response rate in 16 heavily pre-treated patients with relapsed or refractory PTCL, including 3 (19%) complete responses and 5 (31%) partial responses. Verastem intends to initiate an open-label, multicenter, Phase 2 clinical trial evaluating the efficacy and safety of duvelisib in patients with relapsed or refractory PTCL by year end 2017. Verastem expects that the Phase 2 study will be conducted in both the U.S. and Japan.

Today, the Company also announced the appointment of Brian Stuglik, RPh, to its Board of Directors. Mr. Stuglik brings to Verastem 35 years of experience in pharmaceutical and oncology commercialization in both the U.S. and international markets. He has successfully launched several multi-billion dollar brands over his career, including Gemzar®, Alimta® and Erbitux®.

"Expansion of the duvelisib clinical development program, and the accompanying receipt of Fast Track designation from the FDA, are important steps in Verastem's strategy to efficiently develop the potential of duvelisib in additional cancers such as T-cell malignancies," said Robert Forrester, President and Chief Executive Officer of Verastem. "PTCL is a rare and usually aggressive type of NHL where currently available therapies only provide modest benefit. We believe an oral monotherapy like duvelisib could be an important new treatment alternative for patients with T-cell Lymphomas, including PTCL, and we look forward to initiating a Phase 2 study in patients with relapsed or refractory disease by year end."

Mr. Forrester added, "Brian Stuglik is an accomplished executive with significant oncology commercialization expertise who can bring immediate value to Verastem as we now move towards commercializing duvelisib. He brings over 35 years of commercializing important novel oncology drugs together with his extensive external network of clinical thought leaders and deep industry connections. His experience will prove invaluable as we advance duvelisib toward the planned regulatory filing, and potential approval and commercialization."

Most recently, Mr. Stuglik has provided commercial and strategic consultancy services to a variety of life science companies as the founder of Proventus Health Solutions, LLC. Prior to Proventus, he served for over 30 years at Eli Lilly and Company, culminating in his role as Global Vice President and Chief Marketing Officer, Oncology Global Marketing, and advancing Lilly Oncology from a single brand and approved product to a portfolio of over 6 marketed or late-stage compounds across more than 10 cancer types. While at Lilly, Mr. Stuglik helped lead the efforts to acquire Imclone Systems and later led the integration and transition team for Lilly.

### Conference Call Information

The Verastem management team will host a conference call today, Wednesday, September 6, 2017, at 8:00 AM (ET). The call can be accessed by dialing 1-877-341-5660 (toll-free) or 1-315-625-3226 (international) five minutes prior to the start of the call and providing the passcode 81095627.

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](http://www.verastem.com). A replay of the webcast will be archived on the Company's website for 90 days following the call.

### More About Fast Track Designation

The FDA defines Fast Track designation as a process designed to facilitate the development and expedite the review of drugs and biologics, to treat serious or life-threatening conditions, and to fill an unmet medical need. Specifically, Fast Track designation facilitates frequent interactions with the FDA review team, including meetings to discuss all aspects of development to support approval, and also provides the opportunity to submit sections of an NDA on a rolling basis as data become available.

### 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 the Tumor Microenvironment

The tumor microenvironment encompasses multiple tumor and non-tumor cell populations and an extracellular matrix that support cancer cell survival. This includes immunosuppressive regulatory T-cells, myeloid-derived suppressor cells, tumor-associated macrophages, cancer-associated fibroblasts, and extracellular matrix proteins that can hamper the entry and therapeutic benefit of cytotoxic T-cells and anti-cancer drugs. In addition to targeting the proliferative and survival signaling of cancer cells, Verastem's product candidates, including duvelisib and defactinib, also target the tumor microenvironment to potentially improve response to therapy.

#### **About Duvelisib**

Duvelisib is an investigational, dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma, two enzymes that are 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-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.<sup>3,4,5</sup> Duvelisib is currently being evaluated in late- and mid-stage clinical trials, including DUO®, a randomized, Phase 3 monotherapy study in patients with relapsed/refractory CLL/SLL,<sup>6</sup> and DYNAMO®, a single-arm, Phase 2 monotherapy study in patients with refractory iNHL.<sup>7</sup> Both DUO and DYNAMO achieved their primary endpoints upon topline analysis of efficacy data. Duvelisib is also being evaluated for the treatment of hematologic malignancies through investigator-sponsored studies, including T-cell lymphoma.<sup>8</sup> Information about duvelisib clinical trials can be found on [www.clinicaltrials.gov](http://www.clinicaltrials.gov).

#### **About Verastem, Inc.**

Verastem, Inc. (NASDAQ:VSTM) is a biopharmaceutical company focused on discovering and developing drugs to improve outcomes for patients with cancer. Verastem is currently developing duvelisib, a dual inhibitor of PI3K-delta and PI3K-gamma, which has successfully met the primary endpoints in both a Phase 2 study in double-refractory iNHL and a Phase 3 clinical trial in patients with relapsed/refractory CLL/SLL. In addition, Verastem 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, ovarian, non-small cell lung cancer, and mesothelioma. Verastem's product candidates seek to treat cancer by modulating the local tumor microenvironment, enhancing anti-tumor immunity and reducing cancer stem cells. For more information, please visit [www.verastem.com](http://www.verastem.com).

#### **Verastem, Inc. forward-looking statements notice:**

This press release includes forward-looking statements about Verastem's strategy, future plans and prospects, including statements regarding the development and activity of Verastem's investigational product candidates, including duvelisib and defactinib, and Verastem's PI3K and FAK programs generally, the structure of our planned and pending clinical trials and the timeline and indications for clinical development. 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 the full data from the DUO study will not be consistent with the top-line results of the study; that the preclinical testing of Verastem'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 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 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 will be unable to successfully initiate or complete the clinical development of its product candidates; that the development of Verastem's product candidates will take longer or cost more than planned; that Verastem may not have sufficient cash to fund its contemplated operations; that Verastem or Infinity Pharmaceuticals, Inc. will fail to fully perform under the duvelisib license agreement; that Verastem will not pursue or submit regulatory filings for its product candidates; and that Verastem'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 Verastem's Annual Report on Form 10-K for the year ended December 31, 2016 and in any subsequent filings with the U.S. Securities and Exchange Commission. The forward-looking statements contained in this press release reflect Verastem's views as of the date of this release, and Verastem does not undertake and specifically disclaims any obligation to update any forward-looking statements.

#### **References**

<sup>1</sup>Leukemia and 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), NCT02004522

<sup>7</sup> [www.clinicaltrials.gov](http://www.clinicaltrials.gov), NCT01882803

<sup>8</sup> [www.clinicaltrials.gov](http://www.clinicaltrials.gov), NCT02783625, NCT02783625, NCT02158091

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