



Verastem Oncology to Present Data on Two Lead Drug Candidates at ASCO 2018 Annual Meeting

May 16, 2018 at 5:09 PM EDT

Results from the DUO™ crossover study with duvelisib demonstrate superior ORR and PFS compared to prior ofatumumab treatment and consistent with ORR in patients who were initially treated with duvelisib in the DUO™ Study

Promising initial clinical activity of defactinib, an oral FAK inhibitor, in combination with pembrolizumab and gemcitabine in patients with pancreatic cancer whose disease progressed on one or more prior lines of chemotherapy

BOSTON--(BUSINESS WIRE)--May 16, 2018-- 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 announced that five abstracts have been selected for poster presentations at the 54th Annual Meeting of the American Society of Clinical Oncology (ASCO) being held June 1-5, 2018 in Chicago.

The Company will present data on its lead product candidate, duvelisib, a first-in-class oral dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma, as well as defactinib, its oral small molecule inhibitor of Focal Adhesion Kinase (FAK). The poster presentations will highlight the latest findings from the Company's ongoing clinical trials for duvelisib, the Phase 3 DUO study crossover extension in patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL), in addition to a Phase 1 study of defactinib in combination with pembrolizumab and gemcitabine in patients with relapsed/refractory pancreatic cancer.

A poster will also be presented on PRIMO, a Phase II study that Verastem Oncology has initiated to evaluate duvelisib monotherapy in peripheral T-cell lymphoma (PTCL), one of the most aggressive forms of non-Hodgkin lymphoma (NHL). The U.S. Food and Drug Administration (FDA) has granted fast track designation to the investigation of duvelisib for the potential treatment of patients with peripheral T-cell lymphoma (PTCL) who have received at least one prior therapy. This study was initiated based on promising activity observed with duvelisib monotherapy in T-cell lymphomas in the Phase I study. In addition, posters on biomarker measurements in the DUO™ and DYNAMO™ studies, as well as in a treatment naive follicular lymphoma study, will be presented.

"In the crossover extension trial of the Phase 3 DUO study, patients treated with duvelisib following confirmed disease progression on prior ofatumumab therapy exhibited a 73% overall response rate (ORR), compared to a 28% ORR with ofatumumab in the DUO study. These results were consistent with the ORR of patients originally initiated on duvelisib. This response rate was accompanied by a longer median progression-free survival (mPFS), with duvelisib-treated patients achieving 15-month mPFS, compared to 9 months when they were treated with ofatumumab," said Diep Le, MD, PhD, Chief Medical Officer of Verastem Oncology.

Dr. Le added, "For our lead FAK inhibitor defactinib, Dr. Andrea Wang-Gillam will describe initial results from the ongoing Phase 1 study evaluating defactinib in combination with pembrolizumab and gemcitabine in patients with advanced pancreatic cancer. In addition to defining a recommended Phase 2 dose, the combination regimen has been well tolerated with promising signs of clinical activity, including one confirmed partial response in a pancreatic cancer patient and stable disease in additional pancreatic patients."

"Data presented at this year's ASCO meeting by Verastem Oncology and collaborative researchers also directly demonstrate inhibition of both PI3K-delta and PI3K-gamma in patients treated with duvelisib," said Jonathan Pachter, PhD, Chief Scientific Officer of Verastem Oncology. "Accordingly, biomarker changes observed in duvelisib-treated patients indicate targeting of malignant B-cells directly along with targeting of the supportive tumor microenvironment."

Details for the ASCO 2018 presentations are as follows:

Duvelisib

Title: The efficacy of duvelisib monotherapy following disease progression on ofatumumab monotherapy in patients with relapsed/refractory CLL or SLL in the DUO crossover extension study

Lead author: Dr. Bryone Kuss, Flinders Medical Center

Session: Hematologic Malignancies – Lymphoma and Chronic Lymphocytic Leukemia

Poster #: 170

Abstract #: 7533

Location: McCormick Place Hall A

Date and Time: Monday, June 4, 8:00 – 11:30 a.m. CT

Title: The effect of duvelisib, a dual inhibitor of PI3K- δ,γ , on components of the tumor microenvironment in previously untreated follicular lymphoma

Lead author: Dr. Carla Casulo, University of Rochester, Wilmot Cancer Center

Session: Hematologic Malignancies – Lymphoma and Chronic Lymphocytic Leukemia

Poster #: 216

Abstract #: 7579

Location: McCormick Place Hall A

Date and Time: Monday, June 4, 8:00 – 11:30 a.m. CT

Title: The PRIMO study: A phase 2 study of duvelisib efficacy and safety in patients with relapsed or refractory peripheral t-cell lymphoma (PTCL)
Lead author: Dr. Steven Horwitz, Memorial Sloan Kettering Cancer Center
Session: Hematologic Malignancies – Lymphoma and Chronic Lymphocytic Leukemia
Poster #: 222b
Abstract #: TPS7590
Location: McCormick Place Hall A
Date and Time: Monday, June 4, 8:00 – 11:30 a.m. CT

Title: Duvelisib inhibition of chemokines in patients with CLL (DUO study) and iNHL (DYNAMO study)
Lead author: Dr. David Weaver, Verastem Oncology
Session: Tumor Biology
Poster #: 161
Abstract #: 12048
Location: McCormick Place Hall A
Date and Time: Monday, June 4, 1:15 – 4:45 p.m. CT

Defactinib

Title: Phase I study of defactinib combined with pembrolizumab and gemcitabine in patients with advanced cancer
Lead author: Dr. Andrea Wang-Gillam, Washington University School of Medicine in St. Louis
Session: Developmental Therapeutics – Clinical Pharmacology and Experimental Therapeutics
Poster #: 387
Abstract #: 2561
Location: McCormick Place Hall A
Date and Time: Monday, June 4, 8:00 – 11:30 a.m. CT

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 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.⁷ 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.^{8,9} 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.^{10,11,12} 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, Inc. (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 2019 or at all; that even if data from clinical trials is positive, regulatory authorities may require additional studies for approval or may approve for indications or patient populations that are not as broad as intended and the product may not prove to be safe and effective or may require labeling with use or distribution restrictions; 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

- 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, NCT02004522
- ⁵ www.clinicaltrials.gov, NCT01882803
- ⁶ www.clinicaltrials.gov, NCT02783625, NCT02158091
- ⁷ Schaller M.D. and Parsons J.T. Focal adhesion kinase: an integrin-linked protein tyrosine kinase. *Trends Cell Biol.* 1993 3: 258-62.
- ⁸ Jiang H et al. Targeting focal adhesion kinase renders pancreatic cancers responsive to checkpoint immunotherapy. *Nat Med* 2016: Aug 22(8) 851-60.
- ⁹ Sulzmaier F.J. et al. FAK in cancer: mechanistic findings and clinical applications. *Nature Rev Cancer.* 2014 14: 598-610.
- ¹⁰ www.clinicaltrials.gov, NCT02546531
- ¹¹ www.clinicaltrials.gov, NCT02943317
- ¹² www.clinicaltrials.gov, NCT02758587

View source version on businesswire.com: <https://www.businesswire.com/news/home/20180516006497/en/>

Source: Verastem, Inc.

Verastem Oncology

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