Verastem Oncology to Present Duvelisib Data at EHA 2018 Annual Meeting

May 17, 2018

In a phase Ib/II investigational study of duvelisib, a dual inhibitor of PI3K-δ,γ in combination with FCR (dFCR) for frontline therapy of younger CLL patients, dFCR was observed to be a highly active regimen, achieving ORR of 97% and 81% bone marrow MRD negativity

Additional data presentations support the hypothesis that duvelisib targets malignant B-cells directly and modulates the tumor microenvironment, as well as demonstrate robust clinical activity of duvelisib in patients relapsed/refractory to ofatumamab

BOSTON--(BUSINESS WIRE)--May 17, 2018-- Verastem Oncology (Nasdaq:VSTM), a biopharmaceutical company focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients, today announced the selection of four abstracts for oral and poster presentations at the 23rd Congress of the European Hematology Association (EHA) being held June 14-17, 2018 in Stockholm, Sweden.

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. An oral presentation by Dr. Matthew Davids, Dana-Farber Cancer Institute, will highlight the latest data from a Phase Ib/II study of duvelisib in combination with FCR (dFCR) as a frontline treatment in younger patients with chronic lymphocytic leukemia (CLL). Three posters will highlight additional duvelisib data, including crossover extension results from the Phase 3 DUO™ study in patients with relapsed or refractory CLL/small lymphocytic lymphoma (CLL/SLL), and new biomarker analyses on the tumor microenvironment modulation from DUO™ and the Phase 2 DYNAMO™ study in patients with refractory indolent non-Hodgkin lymphoma (iNHL), and the dual PI3K-delta and PI3K-gamma activity of duvelisib in the CONTEMPO™ study in patients with untreated follicular lymphoma who are treated with duvelisib in combination with CD20 antibody immunotherapy.

"At EHA 2018, Dr. Matthew Davids will deliver an oral presentation describing results from the ongoing Phase Ib/II study evaluating duvelisib in combination with FCR (chemo-immunotherapy) in younger CLL patients," said Diep Le, MD, PhD, Chief Medical Officer of Verastem Oncology. "To date, the combination regimen has shown to be effective as an initial therapy with an ORR of 97%, including 28% of evaluable patients achieving a complete response or complete response with incomplete blood count recovery, and 69% achieving a partial response. A high rate of 81% bone marrow MRD negativity was observed in patients with at least one evaluation. Collectively, the data being presented at EHA this year continue to provide important insights to guide the future clinical development of duvelisib across a wide range of hematologic malignancies, both as a monotherapy and in combination with other agents."

Details for the EHA 2018 presentation and posters are as follows:

Oral Presentation

Title: A Phase IB/II Study of duvelisib in combination with FCR (DFCR) for Frontline Therapy of Younger CLL Patients
Lead author: Dr. Matthew Davids, Dana-Farber Cancer Institute
Topic: Chronic lymphocytic leukemia and related disorders - Clinical
Session Title: Combination treatment with targeted agents in CLL
Date and Time: Saturday, June 16, 12:15 - 12:30 CEST
Location: Victoria Hall
Final Abstract Code: S807

Poster Presentations

Title:The Efficacy of Duvelisib Monotherapy Following Disease Progression on Ofatumumab Monotherapy in Patients with Relapsed/Refractory CLL or SLL in a Phase 3 Crossover Extension Study
Lead author: Dr. Bryone Kuss, Flinders Medical Center
Topic: Chronic lymphocytic leukemia and related disorders - Clinical
Session Title: Chronic lymphocytic leukemia and related disorders - Clinical
Date and Time: Friday, June 15, 17:30 - 19:00 CEST
Location: Poster area
Final Abstract Code: PF354

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
Topic: Non-Hodgkin lymphoma Biology & Translational Research
Session Title: Non-Hodgkin lymphoma Biology & Translational Research
Date and Time: Friday, June 15, 17:30 - 19:00 CEST
Location: Poster area
Final Abstract Code: PF646

Title: Duvelisib inhibition of chemokines in patients with CLL (DUO study) and iNHL (DYNAMO study).
Lead author: Dr. David Weaver, Verastem Oncology
Topic: Non-Hodgkin lymphoma Biology & Translational Research
Session Title: Non-Hodgkin lymphoma Biology & Translational Research

Date and Time: Friday, June 15, 17:30 - 19:00 CEST
Location: Poster area
Final Abstract Code: PF649

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, Inc. (Nasdaq:VSTM), operating as Verastem Oncology, is a biopharmaceutical company focused on developing and commercializing medicines 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

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