Verastem Oncology Presents Duvelisib Development Program Data at the American Society of Hematology 2018 Annual Meeting

December 4, 2018

Multiple Clinical Data Presentations Highlighting Updated Long-Term Follow-Up from the Phase 3 DUO and the DUO Crossover Extension Studies

New Preclinical Research Suggests Duvelisib Overcomes Ibrutinib Resistance in CLL Models through Elimination of Malignant B Cells and Disruption of the Supportive Tumor Microenvironment

BOSTON--(BUSINESS WIRE)--Dec. 4, 2018-- Verastem, Inc. (Nasdaq:VSTM) (Verastem Oncology or the Company), focused on developing and commercializing medicines to improve the survival and quality of life of cancer patients, today announced the presentation of seven posters highlighting new and updated clinical and preclinical data from its duvelisib development program at the American Society of Hematology (ASH) 2018 Annual Meeting, taking place December 1-4, 2018, in San Diego. Duvelisib is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma.

“The PI3K pathway is critical for the survival and proliferation of many types of cancer cells,” said Robert Forrester, Verastem President and Chief Executive Officer. “At Verastem Oncology we are committed to progressing the scientific research and clinical development with our corporate, clinical and academic research partners worldwide to unlock the potential of PI3K inhibition and usher in new treatment strategies for patients in need.”

“Research being presented at ASH this year by Chen, *et al* used CLL patient samples to demonstrate critical points about dual PI3K-delta and PI3K-gamma inhibition,” said Jonathan Pachter, PhD, Chief Scientific Officer at Verastem Oncology. “This research suggests that while PI3K-delta inhibition targets the malignant B cells directly, PI3K-gamma inhibition blocks the support of CLL growth by macrophages and T cells in the tumor microenvironment. Data presented show that when CLL cells from patients who progressed on ibrutinib were implanted in mice, dual PI3K-delta and PI3K-gamma inhibition effectively reduced the CLL burden thereby suggesting the potential value of the dual inhibition in tumors resistant to BTK inhibition. The importance of dual inhibition of PI3K-delta and PI3K-gamma, in this case in combination with BCL-2 inhibition, was also described by Ye, *et al* in an aggressive lymphoma model. This study highlights the synergistic activity of the combination in inhibiting ibrutinib resistance compensatory pathways and inducing apoptosis in preclinical models of Mantle Cell Lymphoma.”

“We are delighted to have presented a wide range of data from our ongoing duvelisib development programs, including updated long-term follow-up data from the Phase 3 DUO study as well as the DUO crossover extension study,” said Hagop Youssoufian, MSc, MD, Head of Medical Strategy at Verastem Oncology. “Other key presentations include the Zinzani and Lehmberg data, which describe compelling new biomarker research being conducted relating to predictive factors for response to duvelisib in certain hematologic malignancies.”

Details for the ASH 2018 poster presentations are as follows:

Poster Presentations

Title: Clinical and Biological Indicators of Duvelisib Efficacy in CLL from the Phase 3 DUO Study
Presenter: Jennifer Brown, Harvard Medical School and Dana-Farber Cancer Institute
Abstract Number/Publication ID: 1856
Session: 642. CLL: Therapy, excluding Transplantation: Poster I

Title: The Efficacy and Safety of Duvelisib Following Disease Progression on Ofatumumab in Patients with Relapsed/Refractory CLL or SLL: Updated Results from the DUO Crossover Extension Study
Presenter: Matthew Davids, Dana-Farber Cancer Institute
Abstract Number/Publication ID: 3140
Session: 642. CLL: Therapy, excluding Transplantation: Poster II

Title: Characterization of the Long-Term Efficacy and Safety of Duvelisib Monotherapy in Patients with Relapsed/Refractory CLL/SLL on Treatment for > 2 Years across 4 Clinical Studies
Presenter: Ian Flinn, Sarah Cannon Research Institute
Abstract Number/Publication ID: 3146
Session: 642. CLL: Therapy, excluding Transplantation: Poster II

Title: Simultaneous inhibition of BCL-2 and PI3K signaling overcomes ibrutinib resistance in mantle cell lymphoma
Presenter: Haige Ye, MD Anderson Cancer Center
Abstract Number/Publication ID: 2950
Session: 625. Lymphoma: Pre-Clinical—Chemotherapy and Biologic Agents: Poster II

Title: Prognostic and Immune-Related Factors for Response to Duvelisib in the Phase 2 DYNAMO Clinical Trial in iNHL
Presenter: Pier Luigi Zinzani, University of Bologna Institute of Hematology
Abstract Number/Publication ID: 4167
Session: 623. Mantle Cell, Follicular, and Other Indolent B-Cell Lymphoma—Clinical Studies: Poster III

Title: Dual Inhibition of PI3K-δ and PI3K-γ by Duvelisib Impairs CLL B Cells and CLL-Supporting Cells and Overcomes Ibrutinib Resistance in a Patient-Derived Xenograft Model

Presenter: Shih-Shih Chen, The Feinstein Institute for Medical Research, Northwell Health
Abstract Number/Publication ID: 4420
Session: 642. CLL: Therapy, excluding Transplantation: Poster III

Title: Dynamic BH3 Profiling Predicts Patient Response and MRD Status in Chronic Lymphocytic Leukemia (CLL) Patients Undergoing Frontline Treatment with Kinase Inhibitor Augmented (KIA) FCR
Presenter: Timothy Z. Lehmberg, Dana-Farber Cancer Institute
Abstract Number/Publication ID: 4395
Session: 641. CLL: Biology and Pathophysiology, excluding Therapy: Poster III

PDF copies of these poster presentations will be available here following the conclusion of the meeting.

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 about Verastem Oncology's strategy, future plans and prospects, including statements regarding the development and activity of Verastem Oncology's lead product duvelisib, and Verastem Oncology's PI3K and FAK programs generally, its intent to commercialize duvelisib, the potential commercial success of duvelisib, the anticipated adoption of duvelisib by patients and physicians, the structure of its planned and pending clinical trials and the timeline and indications for clinical development, regulatory submissions and commercialization activities. 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, among other things, uncertainties regarding the commercial success of duvelisib in the United States; uncertainties regarding physician and patient adoption of duvelisib, including those related to the safety and efficacy of duvelisib; the uncertainties inherent in research and development of duvelisib, such as negative or unexpected results of clinical trials; whether and when any applications for duvelisib may be filed with regulatory authorities in any other jurisdictions; whether and when regulatory authorities in any other jurisdictions may approve any such other applications that may be filed for duvelisib, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted and, if approved, whether duvelisib will be commercially successful in such jurisdictions; Verastem Oncology's ability to obtain, maintain and enforce patent and other intellectual property protection for duvelisib and its other product candidates; the scope, timing, and outcome of any legal proceedings; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of duvelisib; that regulatory authorities in the U.S. or other jurisdictions, if approved, could withdraw approval; whether preclinical testing of Verastem Oncology's product candidates and preliminary or interim data from clinical trials will be predictive of the results or success of ongoing or later clinical trials; that the timing, scope and rate of reimbursement for Verastem Oncology's product candidates is uncertain; the risk that third party payors (including government agencies) will not reimburse for duvelisib; that there may be competitive developments affecting its product candidates; that data may not be available when expected; that enrollment of clinical trials may take longer than expected; that duvelisib or Verastem Oncology's 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; 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 FL in other jurisdictions; 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 Quarterly Report on Form 10-Q for the quarterly period ended September 30, 2018 as filed with the Securities and Exchange Commission (SEC) on November 7, 2018, its Annual Report on Form 10-K for the year ended December 31, 2017 as filed with the 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20181204005276/en/

Source: Verastem, Inc.

Verastem Oncology:
Brian Sullivan
Senior Director, Corporate Development
+1-781-469-1636
bsullivan@verastem.com

Media:
Jeff Stoecker
FleishmanHillard
+1-617-692-0509
media@verastem.com

Investors:
Joseph Rayne
Argot Partners
+1-617-340-6075
joseph@argotpartners.com