



April 10, 2018

**To Our Shareholders,**

Verastem is focused on developing and commercializing new therapeutics to improve outcomes for patients with cancer. 2017 was a transformative year for Verastem. We have made tremendous progress with our clinical program for duvelisib. The most significant event of 2017 was the reporting of positive results from the pivotal Phase 3 DUO™ study investigating our lead oncology asset, duvelisib, in patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL). Following the successful outcome of the Phase 3 DUO™ study, we have now been transformed into a pre-commercial enterprise preparing for the potential approval of duvelisib by the U.S Food and Drug Administration (FDA) and a subsequent product launch in the United States.

**Duvelisib**

Oral duvelisib is a potent and selective oral inhibitor of PI3K- $\delta$  (delta) and PI3K- $\gamma$  (gamma) and is the only dual inhibitor of PI3K- $\delta,\gamma$  in late-stage development. In December 2017, at the American Society of Hematology (ASH) Annual Meeting, we presented the positive results from the randomized Phase 3 DUO™ study, which successfully achieved its primary endpoint. In this study, oral duvelisib monotherapy significantly improved progression-free survival (PFS) versus the approved standard of care, ofatumumab, in patients with relapsed or refractory CLL/SLL. Similar PFS advantages were also observed across all analyzed patient subgroups, including patients with 17p deletion, a genotype associated with poor clinical outcomes. In addition to a consistent and manageable safety profile, duvelisib also achieved a statistically significant improvement in overall response rate (ORR) and significantly reduced lymph node burden in the vast majority of patients.

In February 2018, we submitted a New Drug Application (NDA) to the FDA seeking full approval for duvelisib for the treatment of relapsed or refractory CLL/SLL and accelerated approval for the treatment of relapsed or refractory follicular lymphoma (FL).

The NDA is supported by positive clinical data for duvelisib from both the Phase 3 DUO™ study and the Phase 2 DYNAMO™ study, which also achieved its primary endpoint of ORR in patients with double-refractory FL. Results from the Phase 2 DYNAMO™ study were reported at the 2016 annual ASH meeting.

As a result of recent advances, CLL/SLL and FL patients are living longer. However, many will be refractory or intolerant to their initial therapy, or relapse following their initial therapy, which points to the need for new treatment options. In addition, CLL/SLL and FL affect mostly elderly patients, and many are unable or unwilling to be hospitalized or visit the clinic for frequent IV infusions. As a result, we believe the CLL/SLL and FL treatment landscape is moving away from chemotherapies and toward more targeted, oral regimens. Oral duvelisib is the first PI3K inhibitor to show efficacy as an oral monotherapy in a randomized Phase 3 study, and we believe it may offer an appealing alternative for patients with CLL/SLL or FL who have progressed or relapsed.

Beyond CLL/SLL and FL, we are also developing oral duvelisib for the treatment of peripheral T-cell lymphoma (PTCL), a rare and typically aggressive type of non-Hodgkin lymphoma. PRIMO™ is a Phase 2 clinical trial evaluating duvelisib in patients with relapsed or refractory PTCL who have received at least one prior therapy. The primary endpoint of



the study is ORR and secondary and exploratory endpoints include safety, duration of response, PFS, disease control rate and overall survival, among others. This trial is expected to enroll approximately 120 patients in the U.S., European Union and Japan.

### **Defactinib**

Beyond duvelisib, our most advanced product candidate is defactinib, an oral focal adhesion kinase (FAK) inhibitor. Following the observation of promising defactinib activity in both preclinical and early clinical research, a key initiative for Verastem in 2017 was to explore the use of defactinib in combination with immunotherapies and other standard of care oncology agents. To that end, we entered into several important clinical collaborations evaluating various defactinib combinations across several high unmet need cancer indications. The first is a Phase 1 study by Washington University in St. Louis evaluating defactinib in combination with Merck's PD-1 inhibitor Keytruda® (pembrolizumab) and gemcitabine in patients with pancreatic cancer. The second study is being led by Cancer Research UK in conjunction with MSD (known as Merck & Co., Inc. in the U.S. and Canada) to evaluate defactinib and MSD's PD-1 inhibitor Keytruda® in a Phase 1/2 trial in patients with non-small cell lung cancer, pancreatic cancer or mesothelioma. The third clinical study is being conducted in collaboration with Pfizer and Merck KGaA investigating defactinib in combination with their PD-L1 inhibitor Bavencio® (avelumab) in patients with advanced ovarian cancer.

Through these important collaborations, we hope to advance our understanding of how FAK inhibition may complement these leading immuno-oncology agents, with the ultimate goal of achieving better clinical outcomes.

### **Corporate Operations**

Over the last several quarters, we've added a number of new members to the Verastem executive leadership team, including Joseph Lobacki, former CCO of Medivation, as Chief Commercial Officer, Diep Le, MD, PhD, former VP of Immuno-Oncology at MedImmune, as Chief Medical Officer and Nadeem Mirza, MD, MHA, former Global Head Hematology, Global Medical Affairs at Abbvie Oncology, as Senior Vice President, Hematology and Oncology Development. In addition, former Chief Marketing Officer of Lilly Oncology, Brian Stuglik, joined our Board of Directors, and Lori Kunkel, MD, former Chief Medical Officer of Pharmacyclics, joined our Clinical and Scientific Advisory Board.

As we await the potential approval of the duvelisib NDA, we are diligently working to build a targeted commercial capability and prepare for our first potential product launch. We are extremely proud of all that has been accomplished to date, and we look forward to building on the momentum we've created. I would like to thank you, our shareholders, for your continued support.

Sincerely,

A handwritten signature in black ink, appearing to read 'Robert Forrester', is positioned above the printed name.

Robert Forrester  
*President and Chief Executive Officer*



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

This letter 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, Verastem's potential collaboration opportunities 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 the NDA will not occur on the expected timeframes or at all, including by the FDA's target action date; that a filing of a European Marketing Application may not be achieved before the end of the year, if 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'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 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. (Infinity) will fail to fully perform under the duvelisib license agreement; that Verastem 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 will not pursue or submit regulatory filings for its product candidates, including for duvelisib in patients with CLL/SLL or iNHL; 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, 2017 and in any subsequent filings with the U.S. Securities and Exchange Commission. The forward-looking statements contained in this letter reflect Verastem's views as of the date of this letter, and Verastem does not undertake and specifically disclaims any obligation to update any forward-looking statements.