Verastem Oncology and Yakult Honsha Co., Ltd. Sign Exclusive License Agreement for the Development and Commercialization of Duvelisib in Japan

June 5, 2018

Verastem to Receive Upfront Payment of $10 Million USD,Then Eligible to Receive Up To $90 Million USD in Future Milestones, Plus Royalties

Yakult Obtains Rights to First-in-class Oral Dual Inhibitor of Phosphoinositide-3-kinase (PI3K)-delta and PI3K-gamma (PI3K-δ,γ), Duvelisib for Oncology Indications in Japan

BOSTON & TOKYO--(BUSINESS WIRE)--Jun. 5, 2018-- Verastem, Inc. (President and CEO: Robert Forrester)(NASDAQ:VSTM) and Yakult Honsha Co., Ltd. (President: Takashige Negishi)(Tokyo:2267), today announced their entry into an exclusive licensing agreement for Yakult to develop and commercialize Verastem's duvelisib, a first-in-class oral dual inhibitor of phosphoinositide 3-kinase (PI3K)-delta and PI3K-gamma, for the treatment, prevention or diagnosis of all oncology indications in Japan. Verastem's New Drug Application (NDA) for duvelisib is currently under review with the U.S. Food and Drug Administration (FDA) and is seeking full approval for the treatment of relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) and accelerated approval for the treatment of relapsed or refractory follicular lymphoma (FL). On April 9, 2018, Verastem announced that the FDA had accepted the NDA for filing with Priority Review.

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Under the terms of the agreement, Verastem will receive a one-time upfront payment of $10 million from Yakult. Verastem is eligible to receive up to an additional $90 million if certain future pre-specified development and commercialization milestones are successfully achieved by Yakult, plus double-digit royalties based on future net sales of duvelisib in Japan. In exchange, Yakult will receive exclusive rights to develop and commercialize duvelisib in Japan, at its own cost and expense. Yakult will also fund certain global development costs on a pro-rata basis. Verastem will retain all rights to duvelisib outside of Japan.

"In Japan, current therapies to treat CLL/SLL and FL are extremely limited and duvelisib has robust clinical data supporting its efficacy and safety in both indications, which we can build upon," said Masanori Ito, Head of Pharmaceutical Business Division/Managing Executive Officer, Member of the Board of Yakult. "We are eager to collaborate with Verastem to develop duvelisib in these initial hematologic malignancies, and then plan to later expand development to include the additional indications of PTCL and DLBCL. We believe this collaboration underscores our commitment to innovation, growing our oncology franchise, and commercializing medicines that positively impact the lives of patients in Japan."

"This agreement is an important, validating achievement for both duvelisib and Verastem Oncology and speaks to the significant global potential of this novel therapeutic for a broad range of hematologic malignancies," said Robert Forrester, President and Chief Executive Officer of Verastem. "Yakult is an established oncology leader in Japan that successfully markets several branded anti-cancer therapies, including Elplat® and Campto®. We look forward to working with the world-class development, regulatory and commercial teams at Yakult as they advance oral duvelisib toward commercialization in Japan."

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.

About Yakult Honsha Co., Ltd.

Yakult is a leading Japanese company focused on the development and marketing of pharmaceuticals, foods, beverages, and cosmetics. With respect to its pharmaceutical business, Yakult has a strong presence in development and commercialization of the therapeutic products in the field of oncology. The company, led by Takashige Negishi, in 2017 recorded ¥378.3 Billion Revenues.

For more information on Yakult, visit: http://www.yakult.co.jp/english/index.html or view the following company profile: http://www.yakult.co.jp/english/pdf/profile2017-2018_en.pdf

Verastem, Inc. 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 potential to receive milestone and royalty payments under the agreement with Yakult, 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 in any jurisdiction 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 partnerships or collaborations for the development of duvelisib outside of the United States may not be successful; 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

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[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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