

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT
Pursuant to Section 13 or 15(d) of the
Securities Exchange Act of 1934

Date of report (Date of earliest event reported): **August 10, 2020**

Verastem, Inc.

(Exact Name of Registrant as Specified in Charter)

Delaware
(State or Other Jurisdiction
of Incorporation)

001-35403
(Commission
File Number)

27-3269467
(IRS Employer
Identification No.)

117 Kendrick Street, Suite 500, Needham, MA

(Address of Principal Executive Offices)

02494

(Zip Code)

Registrant's telephone number, including area code: **(781) 292-4200**
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

<u>Title of each class</u>	<u>Trading Symbol(s)</u>	<u>Name of each exchange on which registered</u>
Common stock, \$0.0001 par value per share	VSTM	The Nasdaq Global Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Item 1.01. Entry into a Material Definitive Agreement

On August 10, 2020, Verastem, Inc. (the “Company”) entered into an Asset Purchase Agreement (the “Asset Purchase Agreement”) with Secura Bio, Inc. (“Secura Bio”), pursuant to which the Company will divest its rights, title and interest in and to COPIKTRA (duvelisib) (“COPIKTRA”), including certain related assets, in all oncology indications, to Secura Bio (the “Transaction”). Pursuant to the Asset Purchase Agreement, Secura Bio has agreed to pay the Company (i) an up-front payment of \$70.0 million in cash payable at the closing of the Transaction (the “Closing”) and (ii) after the Closing (a) regulatory milestone payments of up to \$45.0 million, consisting of a payment of \$35.0 million upon receipt of regulatory approval of COPIKTRA in the United States for the treatment of peripheral T-cell lymphoma and a payment of \$10.0 million upon receipt of the first regulatory approval for the commercial sale of COPIKTRA in the European Union for the treatment of peripheral T-cell lymphoma, (b) sales milestone payments of up to \$50.0 million, consisting of \$10.0 million when total worldwide net sales of COPIKTRA exceed \$100.0 million, \$15.0 million when total worldwide net sales of COPIKTRA exceed \$200.0 million and \$25.0 million when total worldwide net sales of COPIKTRA exceed \$300.0 million, (c) low double-digit royalties on the annual aggregate net sales above \$100.0 million in the United States and Europe and (d) 50% of all royalty, milestone and sublicense revenue payments payable to Secura Bio under the Company’s existing license agreements with Sanofi, Yakult Honsha Co., Ltd. and CSPC Pharmaceutical Group Limited, each of which will transfer to Secura Bio at the Closing, and 50% of all royalty and milestone payments payable to Secura Bio under any license or sublicense agreement entered into by Secura Bio after the Closing in certain jurisdictions. Pursuant to the terms of the Asset Purchase Agreement, Secura Bio will assume certain contracts, liabilities and obligations of the Company relating to COPIKTRA. The Asset Purchase Agreement contains customary representations, warranties, covenants, termination rights, and indemnification provisions. In addition, the completion of the Transaction is subject to certain customary conditions, including the expiration or termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended.

The foregoing description of the Asset Purchase Agreement does not purport to be complete and is qualified in its entirety by reference to the Asset Purchase Agreement, which will be filed as an exhibit to a future filing by the Company with the Securities and Exchange Commission pursuant to the Securities Exchange Act of 1934, as amended (the “Exchange Act”).

Item 2.02. Results of Operations and Financial Condition

On August 10, 2020, the Company announced its financial results for the quarter ended June 30, 2020. In connection with the announcement, the Company issued a press release, which is being furnished as Exhibit 99.2 to this current report on Form 8-K.

Item 7.01. Other Events

On August 10, 2020, the Company issued a press release announcing the Transaction and posted its corporate presentation. Copies of the press release and the presentation are furnished hereto as Exhibits 99.1 and 99.3.

Item 9.01. Financial Statements and Exhibits

Exhibit No.	Description
99.1	Press Release, dated August 10, 2020, regarding the asset sale transaction
99.2	Press Release, dated August 10, 2020, regarding financial results
99.3	Corporate Presentation, dated August 10, 2020

SIGNATURE

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

VERASTEM, INC.

Date: August 10, 2020

By: /s/ Brian M. Stuglik
Brian M. Stuglik
Chief Executive Officer



Verastem Oncology Signs Definitive Agreement to Sell COPIKTRA® (duvelisib) Rights to Secura Bio to Focus on Development of VS-6766 and Defactinib in KRAS Mutant Solid Tumors

Verastem Will Receive \$70 Million Up-Front with Total Deal Value up to \$311 Million, Plus Double-Digit Sales Royalties

Upon Closing, Verastem's Current Programs Will Be Funded Until At Least 2024 to Develop VS-6766 and Defactinib in Low-Grade Serous Ovarian Cancer and KRAS Mutant Non-Small Cell Lung Cancer

Phase 2 Registration-Directed Trials Expected to Commence by Year End 2020 in Both Low-Grade Serous Ovarian Cancer and KRAS Mutant Non-Small Cell Lung Cancer

Enrollment in Ongoing Investigator-Initiated Phase 1/2 FRAME Study of VS-6766 and Defactinib Now Expanding to Include Pancreatic, KRAS Mutant Endometrial and KRAS-G12V Non-Small Cell Lung Cancer Cohorts

BOSTON – August 10, 2020 – Verastem, Inc. (Nasdaq:VSTM) (also known as Verastem Oncology), a biopharmaceutical company committed to advancing new medicines for patients battling cancer, today announced that it has entered into a definitive agreement to sell its global commercial and development rights to COPIKTRA (duvelisib), its marketed oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first FDA-approved dual inhibitor of PI3K-delta and PI3K-gamma, to Secura Bio, Inc., an integrated biopharmaceutical company dedicated to the worldwide commercialization of significant oncology therapies.

Verastem's sale of COPIKTRA follows the Company's previously announced strategic direction to focus on maximizing the broad potential of its RAF/MEK inhibitor (VS-6766) and FAK inhibitor (defactinib) program in KRAS mutant (KRASmt) solid tumors. Upon closing of the transaction with Secura Bio, Verastem will be dedicated to the development of this program and to deliver on clinical and regulatory milestones for the first potential indications in low-grade serous ovarian cancer (LGSOC) and KRASmt non-small cell lung cancer (NSCLC). Both LGSOC and KRASmt NSCLC are areas of high unmet patient need as there are no approved treatments and existing therapies have low response rates.

"By focusing our expertise and efforts on rapidly advancing the RAF/MEK/FAK development program, we believe we will be providing the best path forward for patients, customers, our shareholders and our company. These strategic decisions will enable us to best deliver on our mission to advance new medicines on behalf of cancer patients," said Brian Stuglik, Chief Executive Officer of Verastem Oncology. "The agreement with Secura Bio will ensure COPIKTRA continues to help more patients, leveraging the established commercial structure, support of ongoing clinical study and potential expansion into new indications."

Terms of the Definitive Sale Agreement

Verastem will receive an up-front payment of \$70 million upon the closing of the transaction and is eligible to receive up to a total deal value of \$311 million if certain regulatory and sales-based milestones are successfully met by Secura Bio and COPIKTRA's other rest-of-world partners, including:

- A total of \$45 million from two separate milestone payments for U.S. Food and Drug Administration (FDA) and European Medicines Agency approvals of COPIKTRA with label indicated for peripheral T-cell lymphoma
- A total of \$50 million for cumulative worldwide net sales of COPIKTRA beginning at \$100 million of cumulative net sales
- Verastem will receive low double-digit royalties on net sales over \$100 million in U.S., Europe and the United Kingdom
- Verastem will also receive 50% of licensing milestones (up to \$146 million) and royalties outside of U.S., Europe and the United Kingdom

In exchange, Secura Bio will receive an exclusive worldwide license for the research, development, commercialization and manufacture of COPIKTRA in all oncology indications. Secura Bio will assume all operational and financial responsibility for activities that were previously part of Verastem's duvelisib program, including commercialization efforts in the United States and Europe, ongoing clinical trials, Verastem's partnerships with Yakult, CSPC and Sanofi and existing royalty obligations. Secura Bio and Verastem are also in discussions related to the transfer of Verastem's field sales and medical professionals.

The transaction with Secura Bio is subject to customary closing conditions and is expected to close in the third quarter of 2020.*

VS-6766 and Defactinib Program Progress and Registration-Directed Trials

Verastem announced today that the company met with the FDA in July 2020 to discuss the registration-directed study design for the VS-6766/defactinib combination in patients with LGSOC. The FDA was supportive of the Company's development strategy and adaptive design for LGSOC.

Verastem's NSCLC study will also be an adaptive design with a focus on patients with KRAS-G12V mutant tumors. Verastem intends to seek input from the FDA after completing the initial cohort of the lung cancer study. Verastem expects to commence registration-directed clinical trials for potential accelerated approval in LGSOC and KRASmt NSCLC by the end of 2020.

Verastem is continuing its clinical collaboration with the Drug Development Unit at ICR/Royal Marsden Hospital. The ongoing investigator-initiated Phase 1/2 FRAME study evaluating the combination of VS-6766 with defactinib in LGSOC, KRASmt NSCLC and colorectal cancer (CRC) has resumed normal accrual and reporting rates following the global lockdown resulting from the COVID-19 pandemic. The FRAME study is now expanding to include new cohorts in pancreatic cancer, KRASmt endometrial cancer and KRAS-G12V NSCLC. Verastem expects that additional data from the LGSOC cohort of the FRAME study will be made available in September, including presentation at the 2nd Annual RAS-Targeted Drug Development Conference. The Company also expects that additional data from the NSCLC cohort of the FRAME study will be submitted to the International Association for the Study of Lung Cancer (IASLC) World Lung Cancer Conference, taking place in January 2021.

The Company has also begun preclinical combination studies investigating VS-6766 and defactinib in combination with KRAS-G12C inhibitors and initial data will be presented at the 2nd Annual RAS-Targeted Drug Development Conference. Based on the positive preclinical data presented at the AACR 2020 Virtual Annual Meeting II, Verastem plans to support a Phase 2 investigator-initiated study evaluating the combination of VS-6766 and defactinib in uveal melanoma, which is expected to begin in late 2020.

Corporate and Financial Overview

With the sale of COPIKTRA, Verastem will become a focused development company with reduced annual expenses of approximately \$50 million. The company is in a position of financial strength with a cash runway expected to fund the clinical and regulatory milestones and development of VS-6766 and defactinib in LGSOC and KRASmt NSCLC until at least 2024.

About VS-6766

VS-6766 (formerly known as CH5126766, CKI27 and RO5126766) is a unique inhibitor of the RAF/MEK signaling pathway. In contrast to other MEK inhibitors in development, VS-6766 blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. This unique mechanism allows VS-6766 to block MEK signaling without the compensatory activation of MEK that appears to limit the efficacy of other inhibitors.

About Defactinib

Defactinib (VS-6063) is an oral small molecule inhibitor of FAK and PYK2 that is currently being evaluated as a potential combination therapy for various solid tumors. The Company has received Orphan Drug designation for defactinib in ovarian cancer and mesothelioma in the US, EU and Australia. Preclinical research by Verastem Oncology scientists and collaborators at world-renowned research institutions has described the effect of FAK inhibition to enhance immune response by decreasing immuno-suppressive cells, increasing cytotoxic T cells, and reducing stromal density, which allows tumor-killing immune cells to enter the tumor.^{1,2}

About the VS-6766/Defactinib Combination

RAS mutant tumors are present in 30% of all human cancers and have historically presented a difficult treatment challenge and are often associated with significantly worse prognosis. Challenges associated with identifying new treatment options for these types of cancers include resistance to single agents, identifying tolerable combination regimens with MEK inhibitors and new RAS inhibitors in development addressing only a minority of all RAS mutated cancers.

The combination of VS-6766 and defactinib has been found to be clinically active in KRASmt. In an ongoing investigator-initiated Phase I/2 FRAME study, the combination of VS-6766 and defactinib is being evaluated in patients with LGSOC, KRASmt NSCLC and colorectal cancer (CRC). Preliminary data from this study presented at the American Association for Cancer Research (AACR) 2020 Virtual Annual Meeting I demonstrated a 67% overall response rate and long duration of therapy among patients with KRASmt LGSOC. Based on an observation of higher response rates seen in patients with KRAS-G12V mutations in the study, Verastem will also be further exploring the role of VS-6766 and defactinib in KRAS-G12V NSCLC. The FRAME study is expanding in August 2020 to include new cohorts in pancreatic, KRASmt endometrial and KRAS-G12V NSCLC.

About COPIKTRA[®] (duvelisib)

COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-cells. PI3K signaling may lead to the proliferation of malignant B-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.^{3,4,5} COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. COPIKTRA is also being developed by Verastem Oncology for the treatment of peripheral T-cell lymphoma (PTCL), for which it has received Fast Track status and Orphan Drug Designation, and is being investigated in combination with other agents through investigator-sponsored studies.⁶ For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

*MTS Health Partners, L.P and Ropes & Gray acted as advisors to Verastem Oncology on this transaction.

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a commercial biopharmaceutical company committed to the development and commercialization of new medicines to improve the lives of patients diagnosed with cancer. Our pipeline is focused on novel small molecule drugs that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, including phosphoinositide 3-kinase (PI3K), focal adhesion kinase (FAK) and RAF/MEK inhibition.

Our first FDA approved product is available for the treatment of patients with certain types of indolent non-Hodgkin's lymphoma (iNHL).

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 related to the expected sale of COPIKTRA, the Company's future funding requirements and the potential clinical value of the RAF/MEK/FAK combination. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," "can," "promising" 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 and uncertainties, among other things, regarding: the satisfaction of closing conditions with respect to the sale of the COPIKTRA assets to Secura Bio; the ability of Secura Bio to achieve the clinical and sales milestones necessary to result in additional consideration payable to Verastem; the inherent uncertainty in forecasting expected funding needs of the Company in advancing its product candidates; the success in the development and potential commercialization of our product candidates, including defactinib in combination with VS-6766; the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis or result in unmanageable safety profiles as compared to their levels of efficacy; our ability to obtain, maintain and enforce patent and other intellectual property protection for our 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 our product candidates; whether preclinical testing of our 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 our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse; 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 experience manufacturing or supply interruptions or failures; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we may not have sufficient cash to fund our contemplated operations; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will be unable to execute on our partnering strategies for defactinib in combination with VS-6766; that we will not pursue or submit regulatory filings for our product candidates, and that our 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, 2019 as filed with the Securities and Exchange Commission (SEC) on March 11, 2020 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.

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- ¹ Gerber D. et al. Phase 2 study of the focal adhesion kinase inhibitor defactinib (VS-6063) in previously treated advanced KRAS mutant non-small cell lung cancer. *Lung Cancer* 2020; 139:60-67.
- ² Chénard-Poirier, M. et al. Results from the biomarker-driven basket trial of RO5126766 (CH5127566), a potent RAF/MEK inhibitor, in RAS- or RAF-mutated malignancies including multiple myeloma. *Journal of Clinical Oncology* 2017; 35. 10.1200/JCO.2017.35.15_suppl.2506.
- ³ 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.
- ⁴ 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.
- ⁵ 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.
- ⁶ www.clinicaltrials.gov, NCT03372057.
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Verastem Oncology Reports Second Quarter 2020 Financial Results and Highlights Recent Company Progress

Announced Path Forward for VS-6766 and Defactinib Combination Following Meeting with FDA

Phase 2 Registration-Directed Trials Expected to Commence by Year End 2020 in Both Low-Grade Serous Ovarian Cancer and KRAS Mutant Non-Small Cell Lung Cancer

Company Monetizes COPIKTRA® (duvelisib) Providing Cash Runway Until at Least 2024

BOSTON, MA – August 10, 2020 – Verastem, Inc. (Nasdaq:VSTM) (also known as Verastem Oncology), a biopharmaceutical company committed to developing and commercializing new medicines for patients battling cancer, today reported financial results for the three months ending June 30, 2020, and provided an overview of recent corporate highlights.

“The first half of 2020 has been a time of transformational change at Verastem Oncology. We recently announced our newest strategic transaction, the sale of COPIKTRA to Secura Bio, which allows us to monetize this asset while focusing our resources and efforts on advancing the VS-6766 and defactinib combination program in KRAS mutant solid tumors,” commented Brian Stuglik, Chief Executive Officer of Verastem Oncology. “We are now looking forward to a catalyst-driven second half of 2020, including reporting updated data from the LGSOC arm of the investigator-initiated Phase 1/2 FRAME study in September and commencing registration-directed clinical trials in low-grade serous ovarian cancer (LGSOC) and KRAS mutant non-small cell lung cancer (NSCLC) by the end of this year.”

Second Quarter 2020 and Recent Highlights

- **Announced Path Forward for VS-6766/Defactinib Combination in LGSOC Following Meeting with U.S. FDA.** Verastem announced today that the company met with the FDA in July 2020 to discuss the registration-directed study design for the VS-6766/defactinib combination in patients with LGSOC. The FDA was supportive of the Company’s development strategy and adaptive design for LGSOC. Verastem’s NSCLC study will also be an adaptive design with a focus on patients with KRAS-G12V mutant tumors. Verastem intends to seek input from the FDA after completing the initial cohort of the lung cancer study. Verastem expects to commence registration-directed clinical trials for potential accelerated approval in LGSOC and KRAS mutant NSCLC by the end of 2020.
 - **Selling COPIKTRA Franchise to Secura Bio in a Deal Totaling \$311 Million, Plus Royalties.** Verastem recently announced its entry into a definitive agreement to sell its global commercial and development rights to COPIKTRA in all oncology indications to Secura Bio, Inc. The transaction, which carries a total deal value of up to \$311 million, plus royalties, will provide Verastem’s current programs with a cash runway until at least 2024 and will allow the Company to focus its resources and efforts on the clinical development of VS-6766, its RAF/MEK inhibitor, and defactinib, its FAK inhibitor, in KRAS mutant solid tumors. Verastem is pursuing development of this combination in LGSOC and KRAS mutant NSCLC.
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- **Presented Preliminary Results from Investigator-initiated Phase 1 FRAME Study Evaluating the Combination of VS-6766 and Defactinib in KRAS Mutant Solid Tumors at AACR 2020 Virtual Meeting I.** In a virtual poster presentation, Udai Banerji, MBBS, MD, DNB, PhD, FRCP, NIHR, Professor of Molecular Cancer Pharmacology at The Institute of Cancer Research and Honorary Consultant in Medical Oncology at The Royal Marsden NHS Foundation Trust, highlighted data from this ongoing, open-label, dose-escalation and expansion study in patients with KRAS mutant advanced solid tumors, including LGSOC and NSCLC. Preliminary data demonstrated a 67% overall response rate and long duration of therapy among patients with LGSOC. Based on higher response rates seen in NSCLC patients with KRAS-G12V mutations, Verastem will also be further exploring the role of the VS-6766/defactinib combination in KRAS-G12V NSCLC. Expansion cohorts remain ongoing in LGSOC and NSCLC and the study will be expanding to include new cohorts in pancreatic, KRAS mutant endometrial and KRAS-G12V NSCLC.
- **Presented New Preclinical VS-6766/Defactinib Combination Data in Uveal Melanoma at AACR 2020 Virtual Meeting II.** In this study, researchers identified and reinforced that FAK inhibition is a viable pathway to inhibit downstream from the GNAQ pathway, which is constitutively active in uveal melanoma. It was observed that co-targeting FAK and RAF/MEK signaling led to tumor collapse in uveal melanoma xenograft and liver metastasis models *in vivo*. Based on these encouraging results, Verastem plans to support an investigator-sponsored, Phase 2 clinical testing of the VS-6766/defactinib combination in uveal melanoma, which is expected to commence by the end of 2020.
- **Appointed John H. Johnson to the Board of Directors.** In April, Verastem Oncology announced the appointment of John H. Johnson to its Board of Directors. Mr. Johnson's career spans multiple executive management roles at leading global corporations where he was responsible for overseeing oncology and immunology drug development initiatives and commercialization. Mr. Johnson will serve on the Compensation and Nominating and Governance Committees.

Upcoming Milestones

- Close transaction with Secura Bio during the third quarter of 2020.
- Present updated data from the LGSOC cohort of the investigator-initiated Phase 1/2 FRAME study evaluating VS-6766 and defactinib in KRAS mutant solid tumors in September, including at the 2nd Annual RAS-Targeted Drug Development Conference on September 16, 2020.
- Present new preclinical data from studies investigating VS-6766 and defactinib in combination with KRAS-G12C inhibitors in September, including at the 2nd Annual RAS-Targeted Drug Development Conference on September 16, 2020.
- Commence registration-directed clinical trials in LGSOC and KRAS mutant NSCLC by the end of 2020.
- Submit updated data from the NSCLC cohort of the investigator-initiated Phase 1/2 FRAME study to the International Association for the Study of Lung Cancer (IASLC) World Lung Cancer Conference, taking place in January 2021.

Second Quarter 2020 Financial Results

Net product revenue for the three months ending June 30, 2020 (2020 Quarter) was \$4.2 million, compared to \$3.0 million for the three months ending June 30, 2019 (2019 Quarter). License and collaboration revenue for both the 2020 Quarter and 2019 Quarter was \$0.1 million.

Total operating expenses for the 2020 Quarter were \$25.6 million, compared to \$41.4 million for the 2019 Quarter.

Research and development (R&D) expense for the 2020 Quarter was \$9.3 million, compared to \$11.3 million for the 2019 Quarter. The decrease of \$2.0 million, or 18%, was primarily related to a decrease in contract research organization (CRO) costs and lower employee related expense.

Selling, general and administrative (SG&A) expense for the 2020 Quarter was \$15.4 million, compared to \$29.3 million for the 2019 Quarter. The decrease of \$13.9 million, or 47%, primarily resulted from the company's shift in strategic direction which led to lower commercial program and employee related expense.

Net loss for the 2020 Quarter was \$23.0 million, or \$0.14 per share (basic and diluted), compared to \$42.2 million, or \$0.57 per share (basic and diluted), for the 2019 Quarter.

For the 2020 Quarter, non-GAAP adjusted net loss was \$20.5 million, or \$0.12 per share (diluted), compared to non-GAAP adjusted net loss of \$35.6 million, or \$0.48 per share (diluted), for the 2019 Quarter. Please refer to the GAAP to Non-GAAP Reconciliation attached to this press release.

Verastem Oncology ended the second quarter of 2020 with cash, cash equivalents and short-term investments of \$160.8 million.

Financial Guidance and Outlook

With the proceeds from the sale of COPIKTRA, Verastem has a cash runway until at least 2024 to deliver on the current programs for VS-6766 and defactinib, including clinical and regulatory milestones and development in LGSOC and KRASmt NSCLC. Verastem expects its 2020 operating expenses to be approximately 40% lower than its 2019 operating expenses. As a result of its new strategic direction and operating plans, along with the expected sale of the COPIKTRA franchise during the third quarter and associated transition activities, the Company expects total operating expenses for the full year 2020 to be in the range of \$80 million to \$90 million. Beginning in 2021 Verastem expects its annual operating expenses to be approximately \$50 million.

Use of Non-GAAP Financial Measures

To supplement Verastem Oncology's condensed consolidated financial statements, which are prepared and presented in accordance with generally accepted accounting principles in the United States (GAAP), the Company uses the following non-GAAP financial measures in this press release: non-GAAP adjusted net loss and non-GAAP net loss per share. These non-GAAP financial measures exclude certain amounts or expenses from the corresponding financial measures determined in accordance with GAAP. Management believes this non-GAAP information is useful for investors, taken in conjunction with the Company's GAAP financial statements, because it provides greater transparency and period-over-period comparability with respect to the Company's operating performance and can enhance investors' ability to identify operating trends in the Company's business. Management uses these measures, among other factors, to assess and analyze operational results and trends and to make financial and operational decisions. Non-GAAP information is not prepared under a comprehensive set of accounting rules and should only be used to supplement an understanding of the Company's operating results as reported under GAAP, not in isolation or as a substitute for, or superior to, financial information prepared and presented in accordance with GAAP. In addition, these non-GAAP financial measures are unlikely to be comparable with non-GAAP information provided by other companies. The determination of the amounts that are excluded from non-GAAP financial measures is a matter of management judgment and depends upon, among other factors, the nature of the underlying expense or income amounts. Reconciliations between these non-GAAP financial measures and the most comparable GAAP financial measures for the three months ended March 31, 2020 and 2019 are included in the tables accompanying this press release after the unaudited condensed consolidated financial statements.

About VS-6766

VS-6766 (formerly known as CH5126766, CKI27 and RO5126766) is a unique inhibitor of the RAF/MEK signaling pathway. In contrast to other MEK inhibitors in development, VS-6766 blocks both MEK kinase activity and the ability of RAF to phosphorylate MEK. This unique mechanism allows VS-6766 to block MEK signaling without the compensatory activation of MEK that appears to limit the efficacy of other inhibitors.

About Defactinib

Defactinib (VS-6063) is an oral small molecule inhibitor of FAK and PYK2 that is currently being evaluated as a potential combination therapy for various solid tumors. The Company has received Orphan Drug designation for defactinib in ovarian cancer and mesothelioma in the US, EU and Australia. Preclinical research by Verastem Oncology scientists and collaborators at world-renowned research institutions has described the effect of FAK inhibition to enhance immune response by decreasing immuno-suppressive cells, increasing cytotoxic T cells, and reducing stromal density, which allows tumor-killing immune cells to enter the tumor.^{i,ii}

About the VS-6766/Defactinib Combination

RAS mutant tumors are present in 30% of all human cancers and have historically presented a difficult treatment challenge and are often associated with significantly worse prognosis. Challenges associated with identifying new treatment options for these types of cancers include resistance to single agents, identifying tolerable combination regimens with MEK inhibitors and new RAS inhibitors in development addressing only a minority of all RAS mutated cancers.

The combination of VS-6766 and defactinib has been found to be clinically active in KRAS mutant tumors. In an ongoing investigator-initiated Phase I/2 FRAME study, the combination of VS-6766 and defactinib is being evaluated in patients with LGSOC, KRAS mutant NSCLC and colorectal cancer (CRC). Preliminary data from this study presented at the American Association for Cancer Research (AACR) 2020 Virtual Annual Meeting I demonstrated a 67% overall response rate and long duration of therapy among patients with KRASmt LGSOC. Based on an observation of higher response rates seen in patients with KRAS-G12V mutations in the study, Verastem will also be further exploring the role of VS-6766 and defactinib in KRAS-G12V NSCLC. The FRAME study is expanding in August 2020 to include new cohorts in pancreatic, KRAS mutant endometrial and KRAS-G12V NSCLC.

About COPIKTRA[®] (duvelisib)

COPIKTRA is an oral inhibitor of phosphoinositide 3-kinase (PI3K), and the first approved dual inhibitor of PI3K-delta and PI3K-gamma, two enzymes known to help support the growth and survival of malignant B-cells. PI3K signaling may lead to the proliferation of malignant B-cells and is thought to play a role in the formation and maintenance of the supportive tumor microenvironment.^{iii,iv,v} COPIKTRA is indicated for the treatment of adult patients with relapsed or refractory chronic lymphocytic leukemia/small lymphocytic lymphoma (CLL/SLL) after at least two prior therapies and relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies. COPIKTRA is also being developed by Verastem Oncology for the treatment of peripheral T-cell lymphoma (PTCL), for which it has received Fast Track status and Orphan Drug Designation, and is being investigated in combination with other agents through investigator-sponsored studies.^{vi} For more information on COPIKTRA, please visit www.COPIKTRA.com. Information about duvelisib clinical trials can be found on www.clinicaltrials.gov.

About Verastem Oncology

Verastem Oncology (Nasdaq: VSTM) is a commercial biopharmaceutical company committed to the development and commercialization of new medicines to improve the lives of patients diagnosed with cancer. Our pipeline is focused on novel small molecule drugs that inhibit critical signaling pathways in cancer that promote cancer cell survival and tumor growth, including phosphoinositide 3-kinase (PI3K), focal adhesion kinase (FAK) and RAF/MEK inhibition.

Our first FDA approved product is available for the treatment of patients with certain types of indolent non-Hodgkin's lymphoma (iNHL).

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 related to the expected sale of COPIKTRA, the Company's future funding requirements and the potential clinical value of the RAF/MEK/FAK combination. The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," "can," "promising" 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 and uncertainties, among other things, regarding: the satisfaction of closing conditions with respect to the sale of the COPIKTRA assets to Secura Bio; the ability of Secura Bio to achieve the clinical and sales milestones necessary to result in additional consideration payable to Verastem; the inherent uncertainty in forecasting expected funding needs of the Company in advancing its product candidates; the success in the development and potential commercialization of our product candidates, including defactinib in combination with VS-6766; the occurrence of adverse safety events and/or unexpected concerns that may arise from additional data or analysis or result in unmanageable safety profiles as compared to their levels of efficacy; our ability to obtain, maintain and enforce patent and other intellectual property protection for our 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 our product candidates; whether preclinical testing of our 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 our product candidates is uncertain; that third-party payors (including government agencies) may not reimburse; 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 experience manufacturing or supply interruptions or failures; that we will be unable to successfully initiate or complete the clinical development and eventual commercialization of our product candidates; that the development and commercialization of our product candidates will take longer or cost more than planned; that we may not have sufficient cash to fund our contemplated operations; that we may be unable to make additional draws under our debt facility or obtain adequate financing in the future through product licensing, co-promotional arrangements, public or private equity, debt financing or otherwise; that we will be unable to execute on our partnering strategies for defactinib in combination with VS-6766; that we will not pursue or submit regulatory filings for our product candidates, and that our 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, 2019 as filed with the Securities and Exchange Commission (SEC) on March 11, 2020 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.

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Verastem, Inc.
Condensed Consolidated Balance Sheets
(in thousands)
(unaudited)

	June 30,	December 31,
	2020	2019
Cash, cash equivalents, & investments	\$ 125,328	\$ 75,506
Accounts receivable, net	1,500	2,524
Inventory	6,316	3,096
Restricted cash, Prepaid expenses and other current assets	11,448	3,835
Property and equipment, net	791	947
Intangible assets, net	19,223	20,008
Right-of-use asset, net	2,909	3,077
Restricted cash and other assets	31,017	36,053
Total assets	\$ 198,532	\$ 145,046
Current Liabilities	\$ 28,784	\$ 29,890
Long-term debt	30,899	35,067
Convertible senior notes	20,381	68,556
Lease Liability, long-term	3,225	3,489
Other liabilities	870	870
Stockholders' equity	114,373	7,174
Total liabilities and stockholders' equity	\$ 198,532	\$ 145,046

Verastem, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except per share amounts)
(unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
Revenue:				
Product revenue, net	\$ 4,235	\$ 3,019	\$ 9,269	\$ 4,690
License and collaboration revenue	72	117	94	117
Total revenue	4,307	3,136	9,363	4,807
Operating expenses:				
Cost of sales - product	392	377	887	534
Cost of sales - intangible amortization	393	392	785	785
Research and development	9,344	11,346	20,268	21,103
Selling, general and administrative	15,442	29,298	35,046	55,331
Total operating expenses	25,571	41,413	56,986	77,753
Loss from operations	(21,264)	(38,277)	(47,623)	(72,946)
Other expense	—	—	(1,313)	—
Interest income	122	1,268	478	2,765
Interest expense	(1,868)	(5,185)	(12,542)	(10,115)
Net Loss	\$ (23,010)	\$ (42,194)	\$ (61,000)	\$ (80,296)
Net loss per share—basic and diluted	\$ (0.14)	\$ (0.57)	\$ (0.45)	\$ (1.09)
Weighted average common shares outstanding used in computing net loss per share—basic and diluted	165,395	73,877	136,775	73,865

Verastem, Inc.
Reconciliation of GAAP to Non-GAAP Financial Information
(in thousands, except per share amounts)
(unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2020	2019	2020	2019
Net Loss Reconciliation				
Net Loss (GAAP basis)	\$ (23,010)	\$ (42,194)	\$ (61,000)	\$ (80,296)
Adjust:				
Amortization of acquired intangible asset	393	392	785	785
Stock-based compensation expense	1,659	3,065	3,029	5,313
Non-cash interest, net	480	1,207	9,259	2,815
Severance and Other	11	1,957	1,798	1,994
Change in fair value of derivative	—	—	1,313	—
Chugai license payment	—	—	3,000	—
Adjusted Net Loss (non-GAAP basis)	\$ (20,467)	\$ (35,573)	\$ (41,816)	\$ (69,389)
Reconciliation of Net Loss Per Share				
Net Loss per share – diluted (GAAP Basis)	(0.14)	(0.57)	(0.45)	(1.09)
Adjust per diluted share				
Amortization of acquired intangible asset	—	—	0.01	0.01
Stock-based compensation expense	0.01	0.04	0.02	0.07
Non-cash interest, net	0.01	0.02	0.07	0.04
Severance and Other	—	0.03	0.01	0.03
Change in fair value of derivative	—	—	0.01	—
Chugai license payment	—	—	0.02	—
Adjusted Net Loss per share – diluted (non-GAAP Basis)	\$ (0.12)	\$ (0.48)	\$ (0.31)	\$ (0.94)
Weighted average common shares outstanding used in computing net loss per share—diluted	165,395	73,877	136,775	73,865

ⁱ Gerber D. et al. Phase 2 study of the focal adhesion kinase inhibitor defactinib (VS-6063) in previously treated advanced KRAS mutant non-small cell lung cancer. Lung Cancer 2020: 139:60-67.

ⁱⁱ Chénard-Poirier, M. et al. Results from the biomarker-driven basket trial of RO5126766 (CH5127566), a potent RAF/MEK inhibitor, in RAS- or RAF-mutated malignancies including multiple myeloma. Journal of Clinical Oncology 2017: 35. 10.1200/JCO.2017.35.15_suppl.2506.

ⁱⁱⁱ 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.

^{iv} 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.

^v 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.

^{vi} www.clinicaltrials.gov, NCT03372057.



Corporate Overview

August 10, 2020

NASDAQ: VSTM

Safe Harbor Statement

This presentation includes forward-looking statements about, among other things, Verastem Oncology's products and product candidates, including anticipated regulatory submissions, approvals, performance and potential benefits of Verastem Oncology products and product candidates, that are subject to substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Applicable risks and uncertainties include the risks and uncertainties, among other things, regarding: the satisfaction of closing conditions with respect to the sale of the COPIKTRA assets to Secura Bio; the ability of Secura Bio to achieve the clinical and sales milestones necessary to result in additional consideration payable to Verastem.

Additional information regarding these factors can be found in Verastem Oncology's Annual Report on Form 10-K for the fiscal year ended December 31, 2019 and in any subsequent filings with the SEC, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors that May Affect Future Results," as well as in our subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission (SEC) and available at www.sec.gov and www.verastem.com.

The forward-looking statements in this presentation speak only as of the original date of this presentation, and we undertake no obligation to update or revise any of these statements.

Investigational Research & Pipeline

VS-6766 Program

- First in Class Investigational RAF/MEK inhibitor
- Acquired WW Rights from Chugai in Jan-20
- Activity in KRAS Mutant Tumors
- Novel Dosing Schedule

Defactinib Program

- First in Class Investigational FAK inhibitor
- Activity in KRAS Mutant Tumors
- Phase 2 I-O Combinations
- Orphan Designation: Ovarian & mesothelioma in the US & EU

Investigator-Initiated Phase 1 Oral Combination study in KRAS Mutant Tumors – FRAME Study

Activity seen in Low Grade Serous Ovarian Cancer & KRAS Mutant Non-Small Cell Lung Cancer

Phase 2 Registration-Directed Trials to Commence by Year End 2020 in LGSOC and KRAS mt NSCLC



Well Positioned to Capitalize on Growth Opportunities



We are a biopharmaceutical company committed to developing and commercializing new medicines for patients battling cancer

New lead clinical program has best-in-class potential

VS-6766 (RAF/MEKi) and defactinib (FAKi) are clinically active against KRAS mutant cancers

Rapid development paths to market

Clinical proof-of-concept achieved in KRAS mutant low-grade serous ovarian cancer (LGSOC), strong signal in KRAS mutant G12V NSCLC; goal to initiate registration-directed trials in 2020

Significant downstream market opportunity and blockbuster potential

30% of all human cancers are driven by mutations in RAS family of genes; VS-6766 combinations broadly applicable across a variety of tumor types

Cash runway until at least 2024

Monetization of COPIKTRA® (duvelisib) provides funding until at least 2024 to develop VS-6766 and defactinib in LGSOC and KRAS mt NSCLC

Proforma cash before Transaction Costs is \$230 million

Starting in 2021, Annual Operating Expense of ~\$50 million

Verastem Oncology Signs Definitive Agreement to Sell COPIKTRA® (duvelisib) Rights in All Oncology Indications to Secura Bio, Inc.



➤ Total Deal Value up to \$311 million

\$70 million upon closing of the transaction

A total of \$45 million from two separate milestones for U.S. FDA and EMA Approvals of COPIKTRA® with label indicated for Peripheral T-cell lymphoma

A total of \$50 million for cumulative worldwide net sales of COPIKTRA® beginning at \$100 million of cumulative net sales

Verastem will receive low double-digit royalties on net sales over \$100 million in U.S., Europe and the United Kingdom

Verastem will also receive 50% of licensing milestones (up to \$146 million) and royalties outside of U.S., Europe and the United Kingdom

➤ Secura Bio will assume all operational and financial responsibility for activities that were previously part of Verastem's duvelisib program, including existing royalty obligations

➤ Verastem will be right-sized as headcount is reduced from ~85 to 50 and beginning in 2021 annual operating expenses are expected to be ~\$50 million

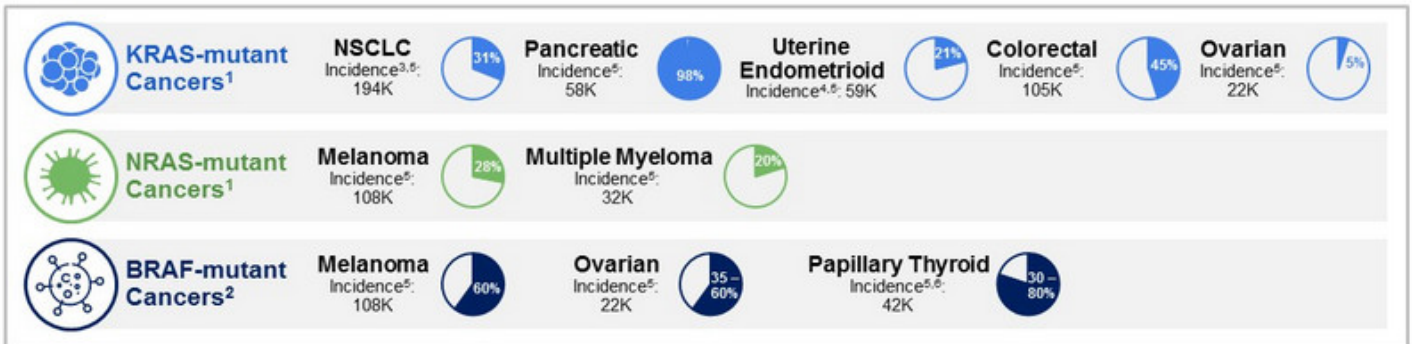


RAS Pathway: Current Approaches and Unmet Needs

VS-6766 & Defactinib:
Novel targeted therapies for high unmet
medical need cancers



High Unmet Needs in RAS/RAF/MEK/ERK-Driven Cancers



Breadth of potential opportunity

- 30% of all human cancers are driven by mutations of the RAS family of genes

Established prognostic significance

- Patients with mutations of the RAS family have an overall worse prognosis

Challenges with conventional approaches

- Modest progress; limited number of approved therapies
- Single agent therapies (e.g. MEK inhibitors) associated with resistance
- Tolerable combination regimens with MEK inhibitors have been challenging
- Current RAS inhibitors in development address only a minority of all RAS mutated cancers

Incidence Sources:

¹Reference for RAS mt frequencies – Cox et al. *Nature Reviews* 13: 828, 2014; ²Reference for BRAF mt frequencies – Turski et al. *Mol Cancer Ther* 15: 533, 2016
³85% of lung cancer is NSCLC (Lu et al. *Cancer Manag Res* 2019); ⁴90% of all uterine cancers are of the endometrial type (ACS); ⁵Cancer Statistics 2020, Siegel et al. *CA Cancer J Clin* 2020;70:7-30; ⁶8 out of 10 thyroid cancers are of the papillary type (ACS)

References:

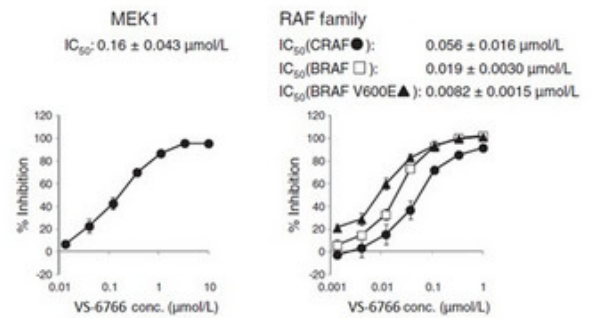
McCormick F. *Clin Cancer Res* 15April2015; Adjerley H et al. *EBioMedicine* 01Mar2019; Papke B et al. *Science* 17Mar2017; Ryan M et al. *Nature Reviews Clinical Oncology* 01Oct2018; NIH cancer.gov/research/key-initiatives/ras

VS-6766 is a Unique Small Molecule RAF/MEK Inhibitor

- VS-6766 inhibits both MEK & RAF kinase activities
- MEK inhibitors paradoxically induce MEK phosphorylation (pMEK) by relieving ERK-dependent feedback inhibition of RAF

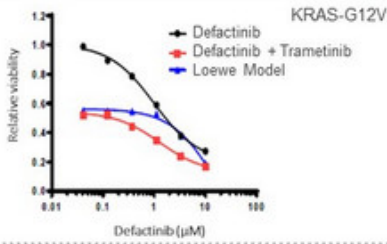


- By inhibiting RAF phosphorylation of MEK, VS-6766 has advantage of not inducing pMEK
- VS-6766 inhibits ERK signaling more completely; may confer enhanced therapeutic activity

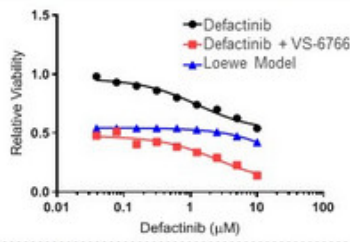


Compelling Preclinical Synergy Observed with RAF/MEKi + FAKi Combination

H441 CELLS KRAS^m Non-small-cell Lung Cancer

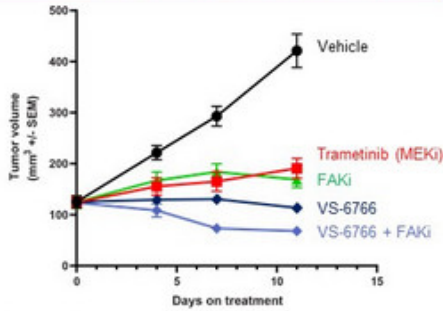


SW982 Cells Sarcoma BRAF:pV600E



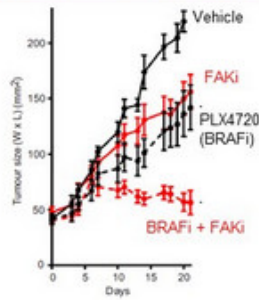
- *In vitro* screen for synergy with defactinib showed MEKi as top hit
 - Included both VS-6766 & trametinib
 - Included both KRAS^m & BRAF^{mt} cancer cell lines

KRAS^m Ovarian TOV-21G In Vivo Model



Coma and Pachter, Verastem (unpublished)

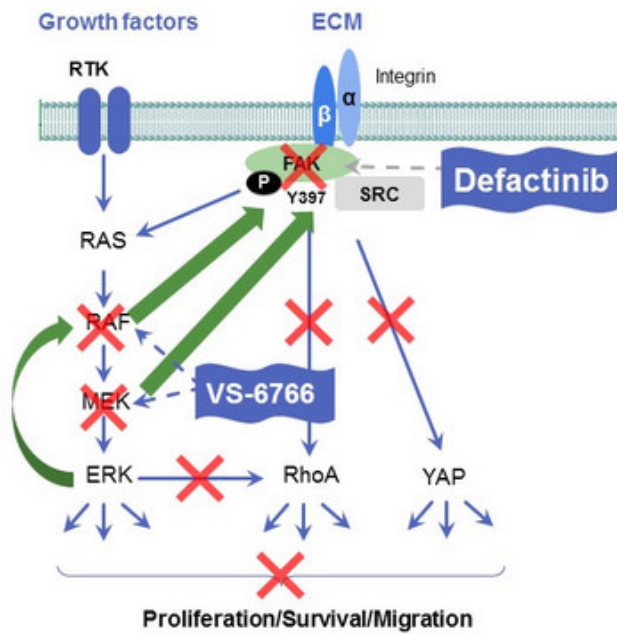
BRAF-V600E mt Melanoma 5555 In Vivo Model



Hirata et al. Cancer Cell (2015)

- *In vivo* xenograft models show improved tumor growth inhibition & tumor regression with FAKi in combo with MEKi or BRAF inhibitor
 - Tumor regression required FAKi combination across models
 - VS-6766 + FAKi induces tumor regression

More Complete Shutdown of Tumor Growth Requires Addressing Multiple Resistance Mechanisms



- BRAF inhibition induces compensatory activation of pFAK¹
- MEK inhibition induces compensatory activation of pFAK preclinically and clinically²
 - Trametinib induced ↑ pFAK (Y397) preclinically in KRAS mt NSCLC cell lines
 - Also observed in patients
 - VS-6766 induced ↑ pFAK (Y397) as a potential resistance mechanism in the majority of patients
 - Combination with defactinib reduced this compensatory pFAK signal
- Upon MEK blockade, ERK feeds back to activate RAF kinase

➔ = Feedback Reactivation



Low Grade Serous Ovarian Cancer (LGSOC)

Opportunity for Precision Medicine

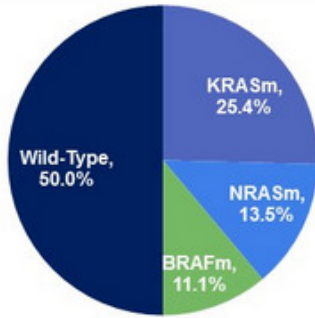


LGSOC: Key Drivers Are KRAS/NRAS/BRAF Mutations

	Incidence	10 Yr Prevalence
Worldwide	~13,000	~80,000
US	~1,000	~6,000

*Based on LGSOC representing 5% of epithelial ovarian cancer

50% of LGSOC Have KRAS/NRAS/BRAF Mutations*

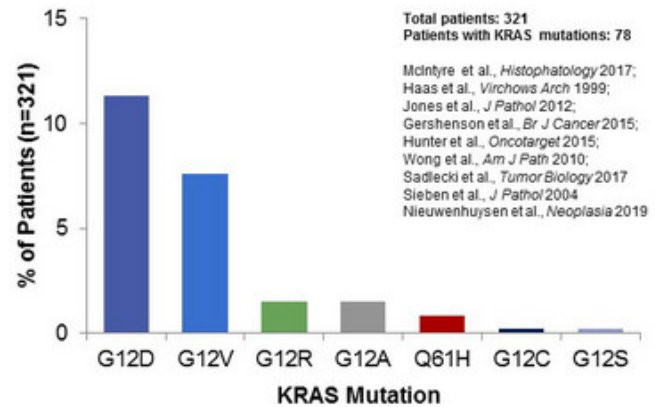


Total patients: 126
Patients with KRAS mutations: 63

McIntyre et al., *Histopathology* 2017;
Emmanuel et al., *Clin Cancer Res* 2014;
Etemadmoghadam et al., *Cancer Res* 2017
Hunter et al., *Oncotarget* 2015
Nieuwenhuysen et al., *Neoplasia* 2019

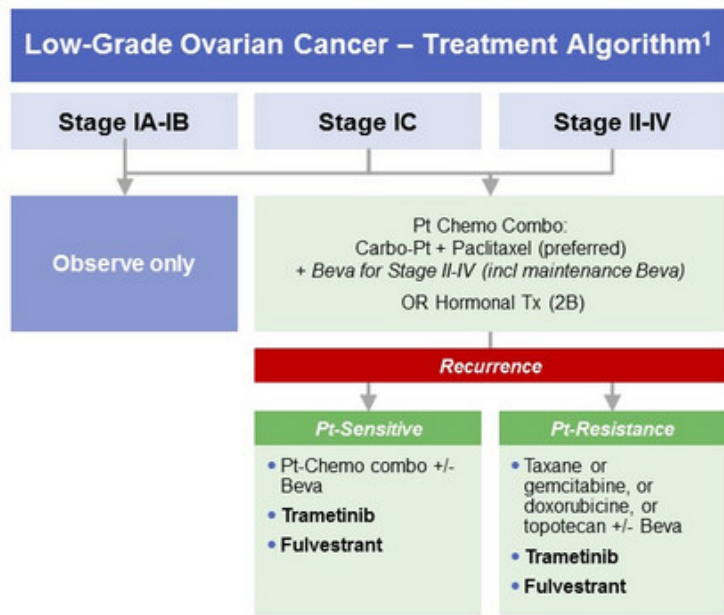
*BRAF, KRAS and NRAS mutations were mutually exclusive

LGSOC (KRASm in 25% of patients)



In LGSOC, G12V & G12D are the dominant KRASm and G12V confers a more aggressive phenotype (Tsang et al., *J. Pathology* 231: 449, 2013)

LGSOC: Limited Treatment Options With High Unmet Need



Limited Response Rates for Available Treatments:

- Platinum based chemotherapy
ORR=~10%
- 13% ORR for letrozole
- MEK inhibitors in the range of approx. 15-25% with high toxicities

¹ Morice, P., Gouy, S. & Leary, A. *N. Engl. J. Med.* (2019)

FRAME¹: Focusing on Low Grade Serous Ovarian Cancer



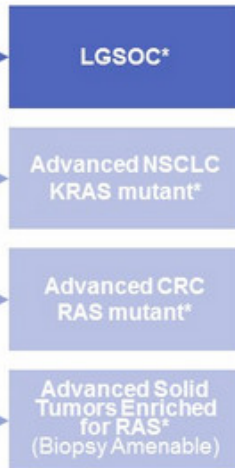
Dr. Udai Banerji
Royal Marsden Hospital

Phase I

Advanced Solid Cancers

- VS-6766 (V) oral twice wkly x 3 wks every 4 wks
- Defactinib (D) oral BID daily x 3 wks q 4 wks
- 3 cohorts with increasing doses explored

¹ Investigator-initiated Phase 1/2 Study



Patient Disposition

Dose **Escalation**: Disclosed at AACR & Verastem Investor Meeting

	VS-6766/Defactinib	Total	LGSOC	NSCLC	Other
Escalation	3.2mg/200mg	3	2		1
	4.0mg/200mg	6	3 ^a	1	2
	3.2mg/400mg	3	1		2
Biopsy	4.0mg/200mg	7	3	0	4
Expansion	4.0mg/200mg	10		10	
Subtotal		29	9 ^c	11 ^d	9 ^b

RP2D dose
Ongoing Dose **Expansion** cohorts; data not mature

	VS-6766/Defactinib	Total	LGSOC	NSCLC	Other
Expansion 3.2mg/200mg	Goal		20	10	10
	Apr-20		9	4 ^e	10

 Data from AACR VM 1, April 27, 2020, CT143; Data Cut-off Nov 2019

*Refractory to conventional treatment or for which no conventional treatment exists
^a Includes one KRASm mucinous ovarian carcinoma
^b Non LGSOC or NSCLC phase 1 patients included to determine recommended dose or PD modeling
^c Response rate data reported for LGSOC at AACR 2020
^d Response rate data reported for NSCLC at AACR 2020; one patient not evaluable for response, included in time on treatment
^e Data not disclosed, except for one NSCLC^{PD} patient as part of combined analysis
 References: Banerji, AACR VM 1, April 27, 2020, CT143; Data on file

VS-6766 3.2 mg + Defactinib 200 mg Selected as RP2D

Treatment Related Adverse Events Occurring in ≥ 10 Patients (Total) Q4 2019 Update

Adverse Event Details*	Dose Escalation Phase						Dose Expansion Phase				Total N=46
	VS-6766 3.2mg Def 200mg Cohort 1 n=3		VS-6766 4mg Def 200mg Cohort 2a n=6		VS-6766 3.2mg Def 400mg Cohort 2b n=3		VS-6766 3.2mg Def 200mg Cohort 1 n=17		VS-6766 4mg Def 200mg Cohort 2a n=17		
	Gr1/2	Gr3/4	Gr1/2	Gr3/4	Gr1/2	Gr3/4	Gr1/2	Gr3/4	Gr1/2	Gr3/4	
Rash	2		6		3		16		12	3	42
CK Elevation	2		2	1	1		7	1	8	3	25
AST Elevation			1		1		5		10	1	18
Hyperbilirubinemia	1	1	1	1	1		7		6		18
Visual Disturbance			1		2		5		8		16
ALT Elevation			1		1		3		8		13
Diarrhoea	2		1		1		4		5		13
Fatigue			2				3		8		13
Oral Mucositis [^]							4		6	2	12
Nausea	1		3		2				6		12
Peripheral Edema							4		6		10

- Most Adverse Events (AE) were Grade 1/2
 - All changes were reversible
- No DLTs in Cohort 1 or 2a
- DLTs Cohort 2b: Gr 2 rash in 2/3 of patients; MTD not reached
- Due to chronic Grade 2 AEs in patients on treatment > 6 months

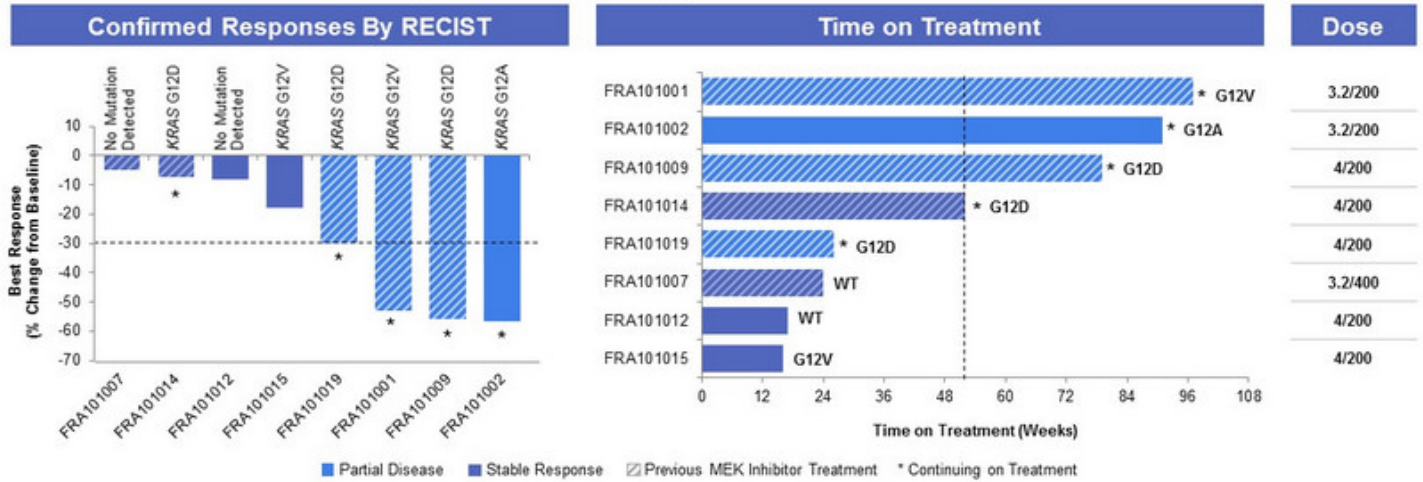
RP2D

- VS-6766 3.2 mg oral twice wkly (3 wks of every 4 wks)
- Defactinib 200 mg oral BID (3 wks of every 4 wks)

*AEs were graded by NCI CTC v4; highest grade only recorded for each patient; data preliminary and subject to change; [^]also includes glossitis/mouth ulcers
References: Banerji, AACR VM 1, April 27, 2020, CT143; Data on file

VS-6766 in Combination with Defactinib Shows Robust ORR with Durability in KRAS^M Refractory LGSOC

Combination of VS-6766 (2x/wk) + Defactinib (BID) q3/4 Wks; Initial Results

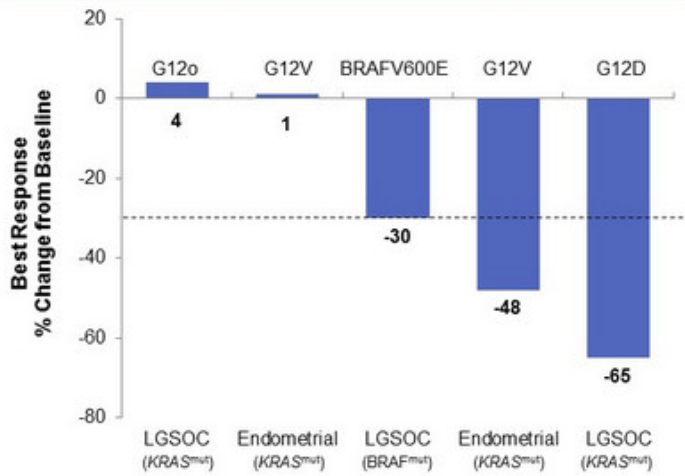


- 67% ORR (4/6) FOR KRAS^M LGSOC; All LGSOC ORR = 50% (4/8)
- 1 patient with KRAS^M mucinous ovarian cancer had PR (> 60% reduction) with > 1 year on therapy (not included in these charts)

Sources: *Annals of Oncology*, 10/2019, V30, v897-898; *Journal of Clinical Oncology* 2015 33:15_suppl TPS5610; Farley, J. et al. *Lancet Oncol.* (2013); Banerji, *AACR VM 1*, April 27, 2020, CT143; Data on file

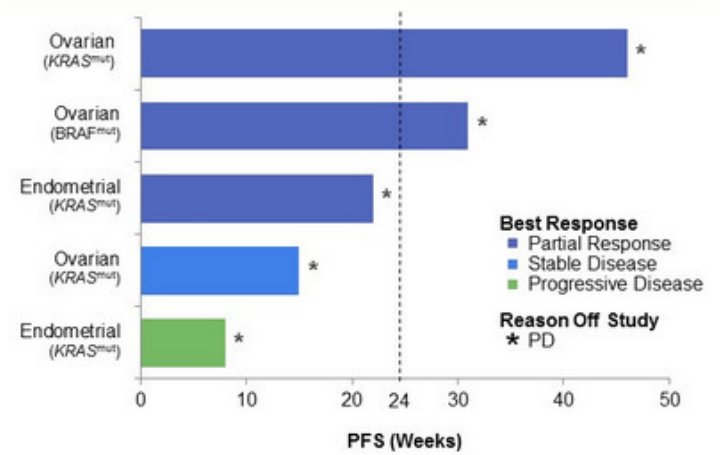
VS-6766 Monotherapy Shows Activity Across RAS Pathway Mutations in Refractory Gynecologic Cancers

Best Response by RECIST v1.1



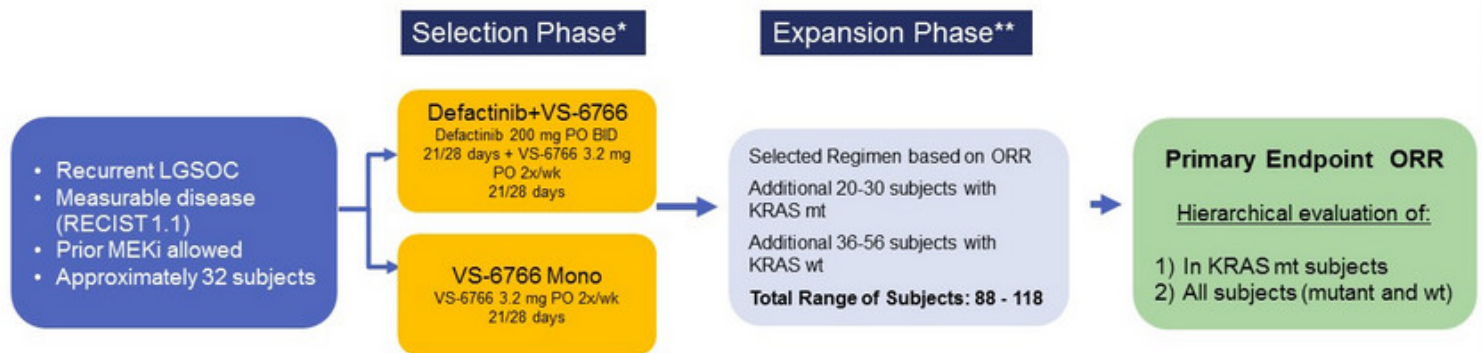
G12o = G12D, R or S

Progression Free Survival



ASCO 2017, presented by: Maxime Chénard-Poirier, MD

KRAS Mutated (mt) and Wild Type (wt), Phase 2, Recurrent LGSOC Adaptive Design for Potential Accelerated Approval



*Selection Phase – KRAS mt only

**Expansion Phase – final sample size to be adjusted based on adaptive design

Proof of Concept Established in LGSOC

VS-6766 ± Defactinib Represents Best in Class Market Opportunity in LGSOC



Key Takeaways

- RAS pathway mutation frequency 50%¹ in LGSOC
- No FDA-approved therapy; limited treatment options
- Unmet medical need creates large market opportunity
- FRAME study: 67% ORR in KRAS^m LGSOC represents Best-in-Class opportunity
- Two potential product revenue streams



Next Steps

- Present additional data from the LGSOC cohort of the FRAME study in September
- Commence Phase 2 Registration-Directed Trial by the end of 2020

¹<http://molecularcasestudies.cshb.org/content/5/6/a004341.full>



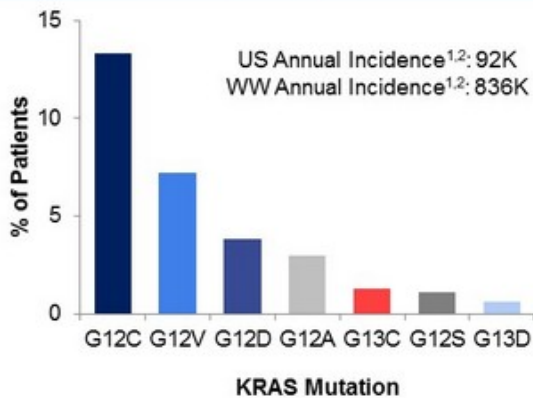
NSCLC Opportunity

Additional Opportunity for
Precision Medicine



High Unmet Need in Refractory KRASm NSCLC Adenocarcinoma

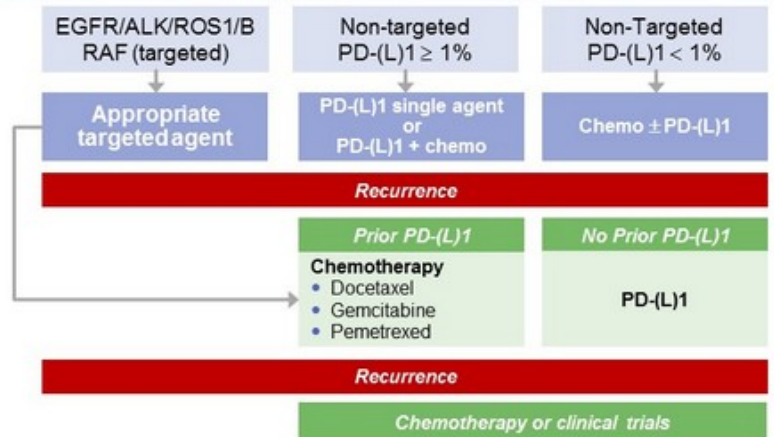
NSCLC Adenocarcinoma³



KRAS Mutations Represent 25% of Lung Cancer Adenocarcinoma (EGFR 17%, ALK 7%)⁴

¹ Globocan, 2018
² <https://www.ncbi.nlm.nih.gov/books/NBK519578/>
³ TCGA PanCancer Atlas (cBioPortal analysis)
⁴ www.thelancet.com Vol 389 January 21, 2017
⁵ Adapted from NCCN Non-small cell lung cancer guidelines Version 3.2020

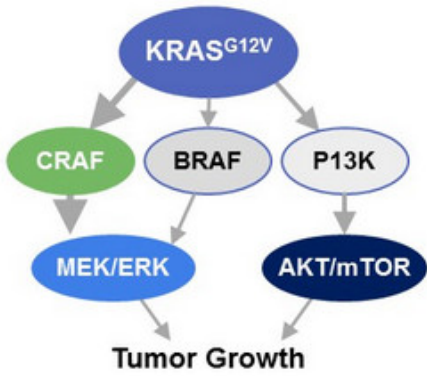
Advanced or Metastatic NSCLC Cancer Recommend Histologic and Molecular Subtyping⁵



- SOC in recurrent disease is chemotherapy
- Pre-PD-(L)1 era, chemotherapy response rate ~10% in recurrent disease; 12w PFS of 30–45%

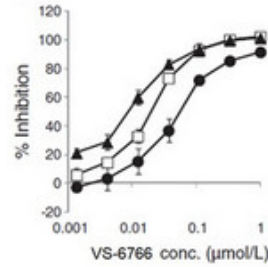
VS-6766 Inhibits CRAF

A Precision Approach to KRAS-G12V Driven NSCLC



- KRAS^{G12V} signals mainly through RAF/MEK in contrast to other variants, such as KRAS-G12D, which signal more through PI3K/AKT
- KRAS^{G12V} models are especially dependent on CRAF

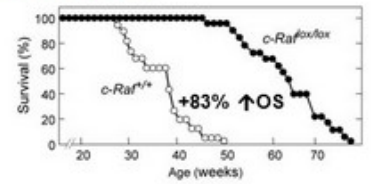
RAF family
 IC_{50} (CRAF●): 0.056 ± 0.016 μmol/L
 IC_{50} (BRAF□): 0.019 ± 0.0030 μmol/L
 IC_{50} (BRAF V600E▲): 0.0082 ± 0.0015 μmol/L



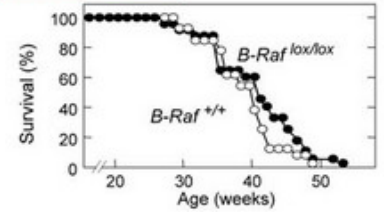
CRAF, but not BRAF, ablation improves survival of mice with KRAS^{G12V} induced lung tumor formation across two different models

CRAF Drives KRAS^{G12V} NSCLC^{1,3}

CRAF KO vs. WT



BRAF KO vs. WT



FRAME¹: Focusing on Advanced Non-Small Cell Lung Cancer



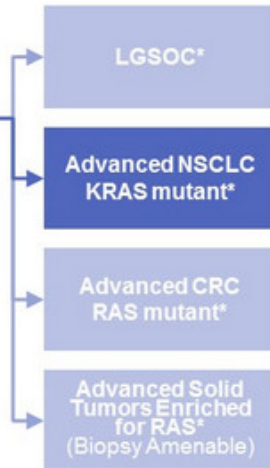
Dr. Udai Banerji
Royal Marsden Hospital

Phase I

Advanced Solid Cancers

- VS-6766 (V) oral twice wklly x 3 wks every 4 wks
- Defactinib (D) oral BID daily x 3 wks q 4 wks
- 3 cohorts with increasing doses explored

¹ Investigator-initiated Phase 1/2 Study



Patient Disposition

Dose **Escalation**: Disclosed at AACR & Verastem Investor Meeting

	VS-6766/Defactinib	Total	LGSOC	NSCLC	Other
Escalation	3.2mg/200mg	3	2		1
	4.0mg/200mg	6	3 ^a	1	2
	3.2mg/400mg	3	1		2
Biopsy	4.0mg/200mg	7	3	0	4
Expansion	4.0mg/200mg	10		10	
Subtotal		29	9 ^c	11 ^d	9 ^b

RP2D dose
Ongoing Dose **Expansion** cohorts; data not mature

	VS-6766/Defactinib	Total	LGSOC	NSCLC	Other
Expansion 3.2mg/200mg	Goal		20	10	10
	Apr-20		9	4 ^e	10

 Data from AACR VM 1, April 27, 2020, CT143; Data Cut-off Nov 2019

*Refractory to conventional treatment or for which no conventional treatment exists

^a Includes one KRAS^m mucinous ovarian carcinoma

^b Non LGSOC or NSCLC phase 1 patients included to determine recommended dose or PD modeling

^c Response rate data reported for LGSOC at AACR 2020

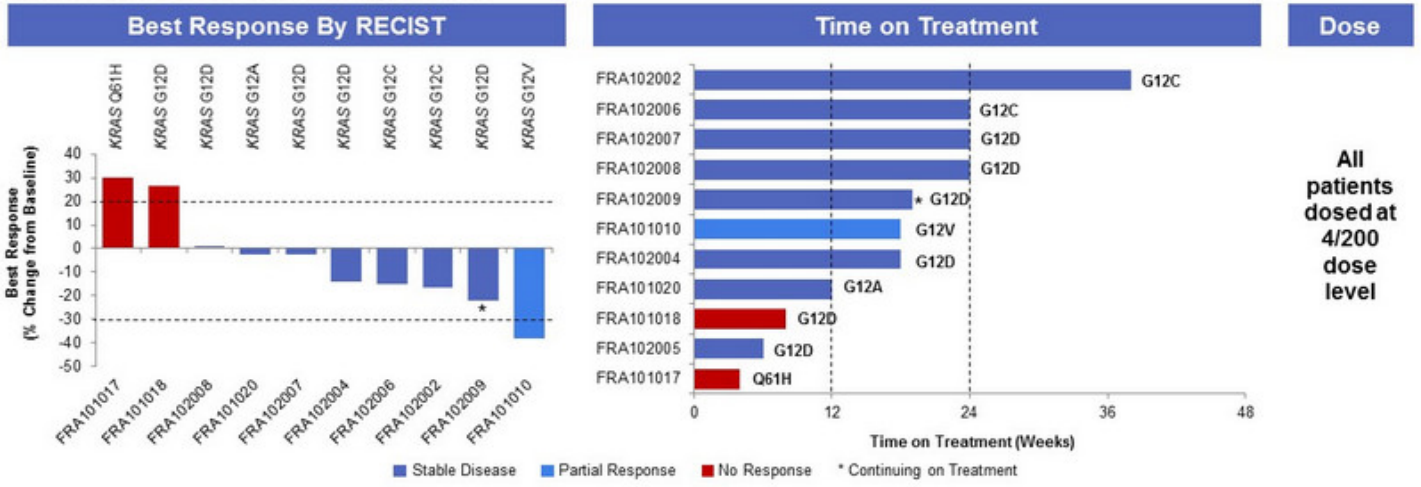
^d Response rate data reported for NSCLC at AACR 2020; one patient not evaluable for response, included in time on treatment

^e Data not disclosed, except for one NSCLC^{2/12} patient as part of combined analysis

References: Banerji, AACR VM 1, April 27, 2020, CT143; Data on file

VS-6766 in Combination with Defactinib: Evidence of Durable Activity Across KRAS^m Refractory NSCLC

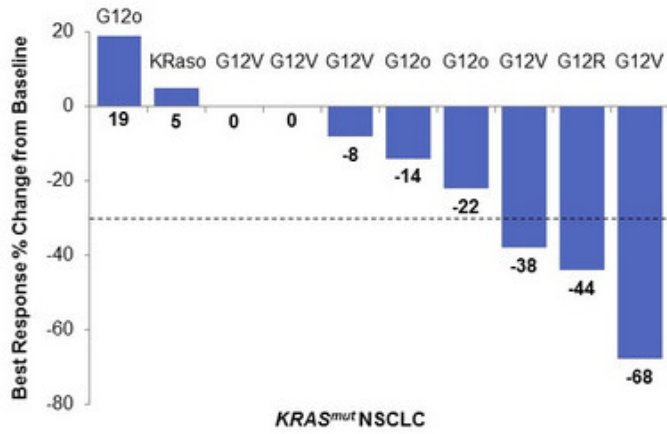
Combination of VS-6766 (2x/wk) + Defactinib (BID) q3/4 wks; Initial Results; KRAS^m Cohorts to Be Expanded



- Median time on treatment ~18 weeks (range 4-38 weeks)
- 1 additional confirmed PR in KRAS^{G12V} patient as of Mar-2020

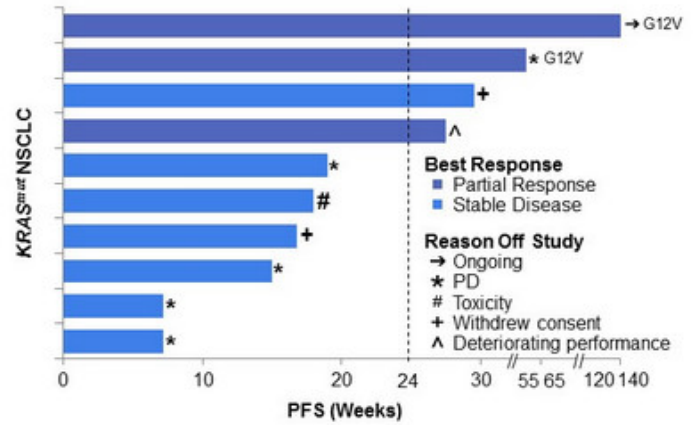
VS-6766 Monotherapy Active in Refractory KRAS Mutant NSCLC Adenocarcinoma

Best Response by RECIST v1.1



G12o = G12D, R or S

Progression Free Survival



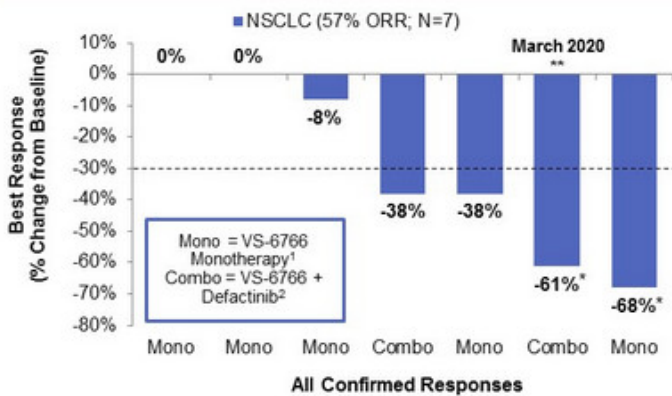
ASCO 2017, presented by: Maxime Chénard-Poirier, MD

PROPERTY OF VERASTEM INC. 25

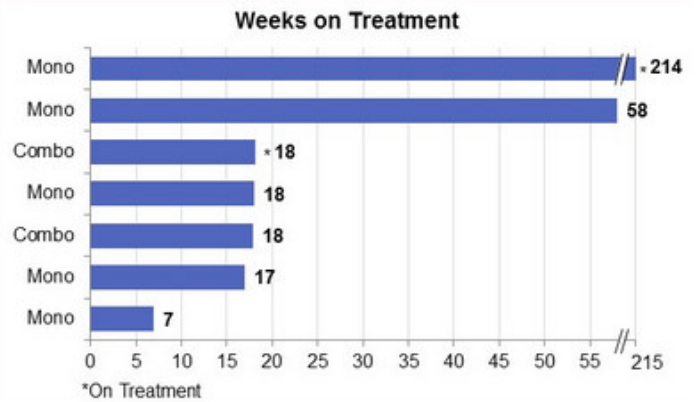
Strong Signal Identified in KRAS^{G12V} to Be Further Validated

VS-6766 ± Defactinib Has a Confirmed 57% ORR in KRAS^{G12V} NSCLC in Integrated Analysis

Best Response by RECIST in KRAS^{G12V} NSCLC



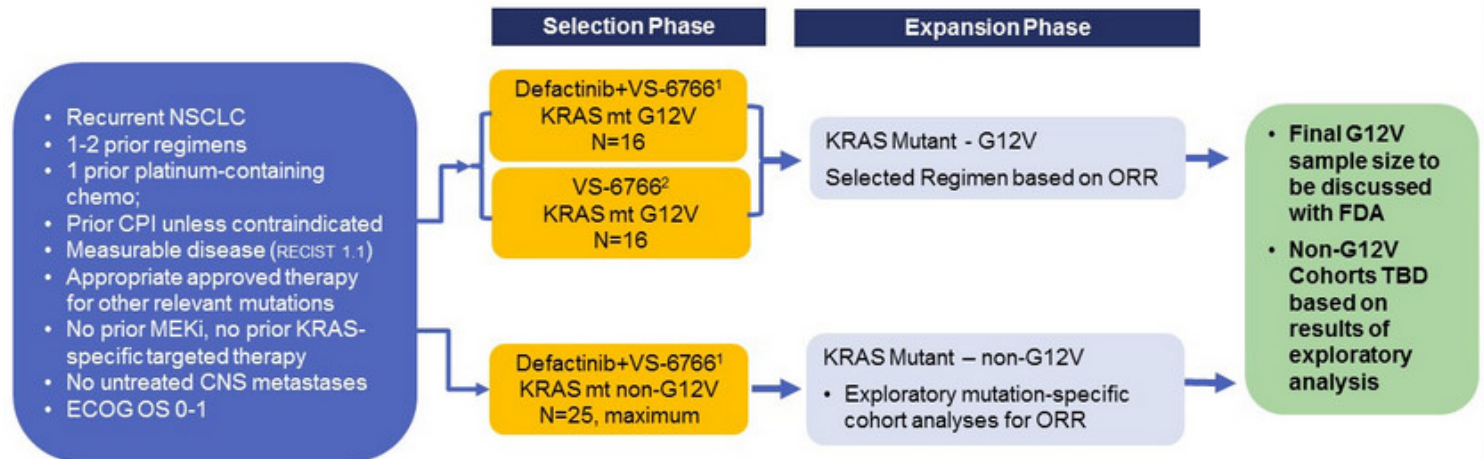
Time on Treatment for KRAS^{G12V} NSCLC



- Preclinical evidence suggests combination with Defactinib may improve efficacy in KRAS^{G12V}
- Activity of VS-6766 as a single agent and in combo with Defactinib in KRAS^{G12V}
- 1 additional confirmed PR in KRAS^{G12V} mutant patient as of Mar-2020

Source: ¹ Martinez-Garcia, M. et al. Clin. Cancer Res. (2012); ² Banerji, AACR VM 1, April 27, 2020, CT143

NSCLC Clinical Strategy: KRAS Mutant (mt), enriched G12V, Phase 2, Recurrent NSCLC for Potential Accelerated Approval



¹ Defactinib 200 mg PO BID (21/28 days) + VS-6766 3.2 mg PO 2x/wk (21/28 days)

² VS-6766 3.2 mg PO 2x/wk (21/28 days)

NSCLC Represents Significant Market Potential for VS-6766 ± Defactinib



Key Takeaways

- High unmet need across KRAS^m lung adenocarcinoma
- High disease control and time on therapy seen across KRAS^m in a heavily refractory patient population
- Strong signal seen in KRAS^{G12V} with VS-6766 monotherapy and with Defactinib combination
- VS-6766 yields more complete blockade of pMEK and pERK than other MEK inhibitors
- Safety profile allows for combination therapy



Next Steps

- Complete NSCLC cohort in FRAME study; add G12V cohort
- Commence Phase 2 Registration-Directed Trial by the end of 2020
- Submit additional data to the International Association for the Study of Lung Cancer (IASLC) World Lung Cancer Conference, January 2021
- Complete ongoing preclinical combo studies of KRAS^{G12C} inhibitors with VS-6766 and Defactinib; Expand into the clinic if positive

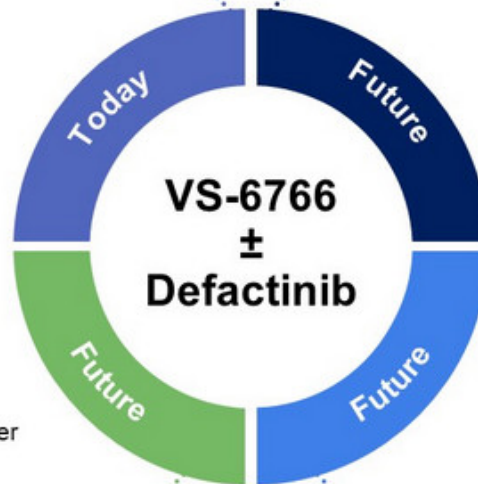
High Priority Lead Indications with Multiple Growth Opportunities

High Priority Indications Supported by Initial Data

- LGSOC^{1,2}
- KRAS^{G12V} NSCLC^{1,2}

Other Mutation Opportunities

- GNAQ mutations in uveal melanoma²
- NF1 mutations in melanoma
- MAP3K1 mutations in breast cancer



Expansion Opportunities

- Pancreatic^{1,2}
- Endometrial¹
- Additional G12V & G12D mt cancers¹
- Uveal Melanoma²
- BRAF mt melanoma^{1,2}
- BRAF mt colorectal
- BRAF mt prostate²

Other Combinations

- KRAS^{G12C} inhibitors
- EGFR inhibitors
- Everolimus²
- Anti-PD-1^{1,2}

¹ Supported by clinical data
² Supported by preclinical data



Corporate



VS-6766 & Defactinib

- ✓ Communicate a regulatory path forward in LGSOC and KRAS mutant NSCLC during the [third quarter/second half] of 2020
- Expand Phase 1/2 FRAME study to include new cohorts in pancreatic cancer, KRAS mutant endometrial cancer and KRAS-G12V NSCLC
- Present updated data from the LGSOC cohort of the Phase 1/2 FRAME study in September 2020
- Present new preclinical data in combination with KRAS-G12C inhibitors in September 2020
- Commence registration-directed clinical trials in LGSOC and KRAS mutant NSCLC by the end of 2020
- Submit updated data from the NSCLC cohort of the Phase 1/2 FRAME study to the IASLC World Lung Cancer Conference, taking place in January 2021



Key Financial Statistics

Cash, cash equivalents & short-term investments as of 6/30/2020	\$160.8M
Proforma cash after transaction close; prior to any transaction expenses	\$230M
Shares fully diluted as of 6/30/2020	188.2M
Hercules Term Loan Facility as of 6/30/2020	\$35.0M*
5.00% Convertible Senior Notes Due 2048 (2018 Notes) as of 6/30/2020	\$28.3M**
Insider ownership (outstanding / vested) as of 6/30/2020	8.3% / 4.3%

*On April 23, 2019, we entered into a 4th Amendment to our existing Agreement with Hercules Capital, Inc. whereas we may borrow up to an aggregate amount of \$75.0 million, of which \$35.0 million was outstanding as of the date of amendment and 6/30/2020.

**The 2018 Notes have an initial conversion rate of 139.5771 shares of Common Stock per \$1,000 which translates to an initial conversion price of \$7.16 per share of Common Stock.

Experienced Senior Management Team



Brian Stuglik
Chief Executive Officer

- Global VP & Chief Marketing Officer – Lilly Oncology
- Founding Member – Proventus Health Solutions



Cathy Carew
Chief People & Organizational Strategy Officer

- Principal – HR Collaborative
- Ironwood, ActiveBiotics, Dynogen, Tufts Health Plan



Hagop Youssoufian, MSc, M.D.
Head of Medical Strategy

- CMO, BIND Therapeutics, EVP, Progenics,
- CMO & EVP, Ziopharm Oncology, SVP, Imclone



Daniel Paterson
President and Chief Operating Officer

- CEO – The DNA Repair Co. (now On-Q-ity)
- PharMetrics (now IMS), Axion



Jonathan Pachter, Ph.D.
Chief Scientific Officer

- Head of Cancer Biology – OSI (now Astellas)



Rob Gagnon
Chief Business and Financial Officer

- CFO – Harvard Bioscience, Clean Harbors
- VP of Finance – Biogen Idec