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.

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.
Portfolio Targets Opportunities With High Unmet Need

The first-approved oral inhibitor of PI3K-δ and PI3K-γ
- Exclusively marketed in the US by Verastem Oncology
- Partnered in Japan, China, Russia/CIS, Turkey, Middle East, & Africa

Full prescribing information, including BOXED WARNING and Medication Guide, is available at www.COPIKTRA.com

Investigational Research & Pipeline

<table>
<thead>
<tr>
<th>VS-6766 Program</th>
<th>Defactinib Program</th>
<th>Duvelisib Program</th>
</tr>
</thead>
<tbody>
<tr>
<td>• First in Class Investigational RAF/MEK inhibitor</td>
<td>• First in Class Investigational FAK inhibitor</td>
<td>• Ongoing registration study in PTCL (FDA Fast Track Designation)</td>
</tr>
<tr>
<td>• Acquired WW Rights from Chugai in Jan-20</td>
<td>• Activity in KRAS Mutant Tumors</td>
<td>• Ongoing clinical investigation as monotherapy and in combination in multiple hematologic malignancies</td>
</tr>
<tr>
<td>• Activity in KRAS Mutant Tumors</td>
<td>• Phase 2 I-O Combinations</td>
<td>• Phase 2 I-O Combination in Solid Tumors</td>
</tr>
<tr>
<td>• Novel Dosing Schedule</td>
<td>• Orphan Designation: Ovarian &amp; mesothelioma in the US &amp; EU</td>
<td>• Pre-Clinical data completed and planned clinical study in combo with CAR-T</td>
</tr>
</tbody>
</table>

- Oral Combination study in KRAS Mutant Tumors
- Phase 2 Dose defined, ongoing basket trial
- Initiate Regulatory Discussions in 1H 2020
We are a biopharmaceutical company committed to developing and commercializing new medicines for patients battling cancer

Portfolio Represents Multiple Growth Opportunities

**New lead clinical program has best-in-class potential**

- VS-6766 (RAF/MEKi) and defactinib (FAKi) are clinically active against KRAS mutant cancers
- Clinical proof-of-concept achieved in KRAS mutant low-grade serous ovarian cancer (LGSOC); goal to initiate registration-directed trial in 2020
- 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 into the fourth quarter of 2021; recent financing funded by several premier life science investors

**Rapid development pathway to market**

**Significant downstream market opportunity and blockbuster potential**

**Strong balance sheet and investor syndicate**

**Revenue-generating commercial asset with multiple planned indication expansion opportunities**

COPIKTRA® (duvelisib) generated $12.3M in 2019 and $5.0M in 1Q20 in approved indications; actively working toward label expansions in PTCL and other hematologic malignancies

We are a biopharmaceutical company committed to developing and commercializing new medicines for patients battling cancer.
### Key Pipeline Programs Aligned with New Strategic Direction

<table>
<thead>
<tr>
<th>PROGRAM</th>
<th>PRECLINICAL</th>
<th>PHASE 1 / 1B</th>
<th>PHASE 2</th>
<th>PHASE 3</th>
<th>MARKET</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>VS-6766; RAF/MEK</strong></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td><strong>Combinations</strong></td>
<td></td>
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</tr>
<tr>
<td>Advanced solid tumors (LGSOC, NSCLC, CRC)*</td>
<td></td>
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<tr>
<td>VS-6766 + defactinib</td>
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<tr>
<td>Advanced solid tumors (KRASm lung)*</td>
<td></td>
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<td></td>
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<tr>
<td>VS-6766 + everolimus</td>
<td></td>
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<td></td>
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<tr>
<td><strong>DEFACTINIB; FAK</strong></td>
<td></td>
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<tr>
<td>In combination with PD-1 inhibitors</td>
<td></td>
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<tr>
<td>R/R pancreatic ductal adenocarcinoma*</td>
<td></td>
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<td></td>
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<tr>
<td>Defactinib + pembrolizumab + gemcitabine</td>
<td></td>
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<td></td>
</tr>
<tr>
<td>NSCLC, pancreatic, mesothelioma*</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Defactinib + pembrolizumab</td>
<td></td>
<td></td>
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</tr>
<tr>
<td><strong>COPIKTRA (duvelisib); Pi3K</strong></td>
<td></td>
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<tr>
<td><strong>Monotherapy</strong></td>
<td></td>
<td></td>
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<td></td>
<td></td>
</tr>
<tr>
<td>R/R CLL/SLL (following two prior therapies)</td>
<td></td>
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<tr>
<td>R/R FL (following two prior systemic therapies)</td>
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<td></td>
<td></td>
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<tr>
<td>R/R PTCL (registration directed)</td>
<td></td>
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<td></td>
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<tr>
<td><strong>Combinations</strong></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>R/R CLL/SLL* duvelisib + venetoclax</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>R/R CLL/SLL* bridging from Ibrutinib</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HNSCC duvelisib + pembrolizumab</td>
<td></td>
<td></td>
<td></td>
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<td></td>
</tr>
</tbody>
</table>

*Investigator-sponsored study
RAS Pathway: Current Approaches and Unmet Needs

VS-6766 & Defactinib: A novel targeted therapy platform for high unmet medical need 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:
3. 85% of lung cancer is NSCLC (Lu et. al. Cancer Manag Res. 2019)
4. 90% of all uterine cancers are of the endometrial type (ACS)
6. 8 out of 10 thyroid cancers are of the papillary type (ACS)

### References:
- McCormick F Clin Cancer Res 15April2015
- Abderley 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

Reference:
Compelling Preclinical Synergy Observed with RAF/MEKi + FAKi Combination

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

**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

---

**H441 CELLS KRASm NON-SMALL-CELL LUNG CANCER**

- Defactinib
- Defactinib + Trametinib
- Loewe Model

**SW982 CELLS SARCOMA BRAF:pV600E**

- Defactinib
- Defactinib + VS-6766
- Loewe Model

---

**KRASm Ovarian TOV-21G In Vivo Model**

- Vehicle
- Trametinib (MEKi)
- FAKi
- VS-6766
- VS-6766 + FAKi

**BRAF-V600E mt Melanoma 5555 In Vivo Model**

- Vehicle
- FAKi
- PLX4720 (BRAFi)
- BRAFi + FAKi

---

Coma and Pachter, Verastem (unpublished)

More Complete Shutdown of Tumor Growth Requires Addressing Multiple Resistance Mechanisms

- BRAF inhibition induces compensatory activation of pFAK \(^1\)
- MEK inhibition induces compensatory activation of pFAK preclinically and clinically\(^2\)
  - 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

\(\Leftrightarrow\) Feedback Reactivation

References:
1. Chen, Mol Cancer Res 2018
2. Banerji, BTOG Dublin, Jan 23, 2019
Low Grade Serous Ovarian Cancer (LGSOC)

Opportunity for Precision Medicine
LGSOC: Key Drivers are KRAS/NRAS/BRAF Mutations

<table>
<thead>
<tr>
<th>Incidence</th>
<th>10 Yr Prevalence</th>
</tr>
</thead>
<tbody>
<tr>
<td>Worldwide</td>
<td>~13,000</td>
</tr>
<tr>
<td></td>
<td>~80,000</td>
</tr>
<tr>
<td>US</td>
<td>~1,000</td>
</tr>
<tr>
<td></td>
<td>~6,000</td>
</tr>
</tbody>
</table>

*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; Sieben et al., J Pathol 2004; Nieuwenhuysen et al., Neoplasia 2019

G12D
G12V
G12R
G12A
Q61H
G12C
G12S

KRAS Mutation

Total patients: 321
Patients with KRAS mutations: 78
McIntyre et al., Histopathology 2017; Haas et al., Virchows Arch 1999; Jones et al., J Pathol 2012; Gershenson et al., Br J Cancer 2015; Hunter et al., Oncotarget 2015; Wong et al., Am J Path 2010; Sadlecki et al., Tumor Biology 2017; Sieben et al., J Pathol 2004; Nieuwenhuysen et al., Neoplasia 2019

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

*BRaf, KRas and NRas mutations were mutually exclusive
LIMITED RESPONSE RATES FOR AVAILABLE TREATMENTS:

- Platinum based chemotherapy ORR=~10%
- 13% ORR for letrozole
- Trametinib ORR=~26%; 35% of patients stopped for adverse events
- 24% ORR for binimetinib
- 15% ORR for selumetinib
**FRAME: Focusing on Low Grade Serous Ovarian Cancer**

**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

**Advanced NSCLC KRAS mutant***

**Advanced CRC RAS mutant***

**Advanced Solid Tumors Enriched for RAS***

(*Refactory to conventional treatment or for which no conventional treatment exists)

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**Patient Disposition**

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

<table>
<thead>
<tr>
<th>Escalation</th>
<th>VS-6766/ Defactinib</th>
<th>Total</th>
<th>LGSOC</th>
<th>NSCLC</th>
<th>Other</th>
</tr>
</thead>
<tbody>
<tr>
<td>3.2mg/200mg</td>
<td>3</td>
<td>2</td>
<td>1</td>
<td></td>
<td></td>
</tr>
<tr>
<td>4.0mg/200mg</td>
<td>6</td>
<td>3a</td>
<td>1</td>
<td>2</td>
<td></td>
</tr>
<tr>
<td>3.2mg/400mg</td>
<td>3</td>
<td>1</td>
<td></td>
<td>2</td>
<td></td>
</tr>
<tr>
<td>Biopsy</td>
<td>4.0mg/200mg</td>
<td>7</td>
<td>3</td>
<td>0</td>
<td>4</td>
</tr>
<tr>
<td>Expansion</td>
<td>4.0mg/200mg</td>
<td>10</td>
<td>10</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Subtotal**

<table>
<thead>
<tr>
<th></th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td>LGSOC</td>
<td>9c</td>
</tr>
<tr>
<td>NSCLC</td>
<td>11d</td>
</tr>
<tr>
<td>CRC</td>
<td>9b</td>
</tr>
</tbody>
</table>

**RP2D dose**

<table>
<thead>
<tr>
<th>Expansion</th>
<th>VS-6766/ Defactinib</th>
<th>Total</th>
<th>LGSOC</th>
<th>NSCLC</th>
<th>CRC</th>
</tr>
</thead>
<tbody>
<tr>
<td>3.2mg/200mg</td>
<td>Goal</td>
<td>20</td>
<td>10</td>
<td>10</td>
<td></td>
</tr>
<tr>
<td>3.2mg/200mg</td>
<td>Apr-20</td>
<td>9</td>
<td>4a</td>
<td>10</td>
<td></td>
</tr>
</tbody>
</table>

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*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*G12V* patient as part of combined analysis

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

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**

<table>
<thead>
<tr>
<th>Adverse Event Details*</th>
<th>Dose Escalation Phase</th>
<th>Dose Expansion Phase</th>
<th>Total N=46</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>VS-6766 3.2mg Def 200mg Cohort 1 n=3</td>
<td>VS-6766 4mg Def 200mg Cohort 2a n=6</td>
<td>VS-6766 3.2mg Def 400mg Cohort 2b n=3</td>
</tr>
<tr>
<td>Rash</td>
<td>Gr1/2 2</td>
<td>Gr3/4 6</td>
<td>Gr1/2 3</td>
</tr>
<tr>
<td>CK elevation</td>
<td>Gr1/2 2</td>
<td>Gr3/4 2</td>
<td>Gr3/4 1</td>
</tr>
<tr>
<td>AST elevation</td>
<td>Gr1/2 1</td>
<td>Gr3/4 1</td>
<td>Gr3/4 5</td>
</tr>
<tr>
<td>Hyperbilirubinemia</td>
<td>Gr1/2 1</td>
<td>Gr3/4 1</td>
<td>Gr3/4 1</td>
</tr>
<tr>
<td>Visual disturbance</td>
<td>Gr1/2 1</td>
<td>Gr3/4 2</td>
<td>Gr1/2 5</td>
</tr>
<tr>
<td>ALT elevation</td>
<td>Gr1/2 1</td>
<td>Gr3/4 1</td>
<td>Gr3/4 3</td>
</tr>
<tr>
<td>Diarrhoea</td>
<td>Gr1/2 2</td>
<td>Gr3/4 1</td>
<td>Gr3/4 4</td>
</tr>
<tr>
<td>Fatigue</td>
<td>Gr1/2 2</td>
<td>Gr3/4 3</td>
<td>Gr3/4 8</td>
</tr>
<tr>
<td>Oral Mucositis</td>
<td>Gr1/2 4</td>
<td>Gr3/4 6</td>
<td>Gr1/2 2</td>
</tr>
<tr>
<td>Nausea</td>
<td>Gr1/2 1</td>
<td>Gr3/4 3</td>
<td>Gr3/4 2</td>
</tr>
<tr>
<td>Peripheral Edema</td>
<td>Gr1/2 4</td>
<td>Gr3/4 6</td>
<td>10</td>
</tr>
</tbody>
</table>

*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

- 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)

References: Banerji, AACR VM 1, April 27, 2020, CT143; Data on file
VS-6766 in Combination with Defactinib Shows Robust ORR with Durability in KRASm in Refractory LGSOC

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

**CONFIRMED RESPONSES BY RECIST**

- Partial response
- Stable disease

**TIME ON TREATMENT**

- * G12V
- * G12A
- * G12D

Dose

- 3.2/200
- 3.2/200
- 4/200
- 4/200
- 4/200
- 4/200
- 4/200
- 4/200
- 3.2/400
- 4/200
- 4/200

**Sources:**
- Annals of Oncology, 10/2019, V30, v897-898
- Journal of Clinical Oncology 2015 33:15, suppl, TP55610; Farley, J. et al. Lancet Oncol. (2013); Banerji, AACR VM 1, April 27, 2020, CT143; Data on file

- **67% ORR (4/6) FOR KRASm LGSOC; All LGSOC ORR = 50% (4/8)**
- **1 patient with KRASm mucinous ovarian cancer had PR (> 60% reduction) with > 1 year on therapy** (not included in these charts)
VS-6766 Monotherapy Shows Activity Across RAS Pathway Mutations in Refractory Gynecologic Cancers

Best response by RECIST v1.1

Progression Free Survival

G12o = G12D, R or S

ASCO 2017, presented by: Maxime Chénard-Poirier, MD
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 KRASm LGSOC represents Best-in-Class opportunity
- Two potential product revenue streams

NEXT STEPS

- Initiate dialogue with FDA for registration trial this year
  - Focus on KRASm patient population
- Confirm added benefit of defactinib in the FRAME Study
- Expand into non-KRASm LGSOC

---

\(^1\) [http://molecularcasestudies.cshlp.org/content/5/6/a004341.full](http://molecularcasestudies.cshlp.org/content/5/6/a004341.full)
NSCLC opportunity

Additional Opportunity for Precision Medicine
High Unmet Need in Refractory KRASm NSCLC Adenocarcinoma

NSCLC Adenocarcinoma\(^3\)

- KRAS mutations represent 25% of lung cancer adenocarcinoma (EGFR 17%, ALK 7%)\(^4\)

<table>
<thead>
<tr>
<th>KRAS Mutation</th>
<th>US Annual Incidence(^1,2): 92K</th>
<th>WW Annual Incidence(^1,2): 836K</th>
</tr>
</thead>
<tbody>
<tr>
<td>G12C</td>
<td>15%</td>
<td>10%</td>
</tr>
<tr>
<td>G12V</td>
<td>5%</td>
<td>5%</td>
</tr>
<tr>
<td>G12D</td>
<td>5%</td>
<td>5%</td>
</tr>
<tr>
<td>G12A</td>
<td>10%</td>
<td>10%</td>
</tr>
<tr>
<td>G13C</td>
<td>1%</td>
<td>1%</td>
</tr>
<tr>
<td>G12S</td>
<td>1%</td>
<td>1%</td>
</tr>
<tr>
<td>G13D</td>
<td>1%</td>
<td>1%</td>
</tr>
</tbody>
</table>

Advanced or Metastatic NSCL Cancer
Recommend Histologic and Molecular Subtyping\(^5\)

- EGFR/ALK/ROS1/BRAF (targeted)
- Non-targeted PD-(L)1 ≥ 1%
- Non-Targeted PD-(L)1 < 1%

Recurrence

Prior PD-(L)1

- Chemotherapy
  - Docetaxel
  - Gemcitabine
  - Pemetrexed

No Prior PD-(L)1

- PD-(L)1

Recurrence

Chemotherapy or clinical trials

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

\(^1\)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
**VS-6766 inhibits CRAF**

*A precision approach to KRAS-G12V driven NSCLC*

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

---

**CRAF drives KRAS<sup>G12V</sup> NSCLC<sup>1,3</sup>**

- CRAF KO vs. WT
  - Survival (%)
  - 100 80 60 40 20 0
  - Age (weeks)
  - c-Raf<sup>lox/lox</sup> ▲ +83% ↑ OS
  - c-Raf<sup>+/+</sup> ▲

- BRAF KO vs. WT
  - Survival (%)
  - 100 80 60 40 20
  - Age (weeks)
  - B-Raf<sup>lox/lox</sup> ▲
  - B-Raf<sup>+/+</sup> ▲

---

*CRAF, but not BRAF, ablation improves survival of mice with KRAS<sup>G12V</sup> induced lung tumor formation across two different models*

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**FRAME: Focusing on Advanced Non-Small Cell Lung Cancer**

**Phase I**
Advanced Solid Cancers
- VS-6766 (V) oral twice weekly x 3 weeks every 4 weeks
- Defactinib (D) oral BID daily x 3 weeks q 4 weeks
- 3 cohorts with increasing doses explored

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

**Patient Disposition**

<table>
<thead>
<tr>
<th>Dose Escalation: Disclosed at AACR &amp; Verastem Investor Meeting</th>
<th>Total</th>
<th>LGSOC</th>
<th>NSCLC</th>
<th>Other</th>
</tr>
</thead>
<tbody>
<tr>
<td>VS-6766/ Defactinib 3.2mg/200mg</td>
<td>3</td>
<td>2</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>Escalation 4.0mg/200mg</td>
<td>6</td>
<td>3&lt;sup&gt;a&lt;/sup&gt;</td>
<td>1</td>
<td>2</td>
</tr>
<tr>
<td>3.2mg/400mg</td>
<td>3</td>
<td>1</td>
<td>1</td>
<td>2</td>
</tr>
<tr>
<td>Biopsy 4.0mg/200mg</td>
<td>7</td>
<td>3</td>
<td>0</td>
<td>4</td>
</tr>
<tr>
<td>Expansion 4.0mg/200mg</td>
<td>10</td>
<td>10</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Subtotal**
29 9<sup>c</sup> 11<sup>d</sup> 9<sup>b</sup>

**RP2D dose**

<table>
<thead>
<tr>
<th>Ongoing Dose Expansion cohorts; data not mature</th>
<th>Total</th>
<th>LGSOC</th>
<th>NSCLC</th>
<th>CRC</th>
</tr>
</thead>
<tbody>
<tr>
<td>VS-6766/ Defactinib 3.2mg/200mg</td>
<td>Goal</td>
<td>20</td>
<td>10</td>
<td>10</td>
</tr>
<tr>
<td>Expansion 4.0mg/200mg</td>
<td>Apr-20</td>
<td>9</td>
<td>4&lt;sup&gt;e&lt;/sup&gt;</td>
<td>10</td>
</tr>
</tbody>
</table>

<sup>a</sup> includes one KRASm mucinous ovarian carcinoma
<sup>b</sup> non LGSOC or NSCLC phase 1 patients included to determine recommended dose or PD modeling
<sup>c</sup> response rate data reported for LGSOC at AACR 2020
<sup>d</sup> response rate data reported for NSCLC at AACR 2020; one patient not evaluable for response, included in time on treatment
<sup>e</sup> data not disclosed, except for one NSCLC<sup>G12V</sup> 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 KRASm Refractory NSCLC

Combination of VS-6766 (2x/wk) + Defactinib (BID) q3/4 wks; Initial Results; KRASm Cohorts to be Expanded

- Median time on treatment ~18 weeks (range 4-38 weeks)
- 1 additional confirmed PR in KRAS<sup>G12V</sup> patient as of Mar-2020
VS-6766 Monotherapy Active in Refractory KRAS Mutant NSCLC Adenocarcinoma

Best response by RECIST v1.1

Progression Free Survival

KRAS\textsuperscript{mut} NSCLC

G12\textsuperscript{a} = G12D, R or S

PFS (weeks)

KRAS\textsuperscript{mut} NSCLC

Partial Response
Stable Disease

Reason off study
- Ongoing
* PD
# Toxicity
+ Withdrew consent
^ Deteriorating performance

Best Response

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

<table>
<thead>
<tr>
<th>Time on Treatment for KRAS(^{G12V}) NSCLC</th>
</tr>
</thead>
<tbody>
<tr>
<td>Weeks on Treatment</td>
</tr>
<tr>
<td>0</td>
</tr>
<tr>
<td>Mono</td>
</tr>
<tr>
<td>Mono</td>
</tr>
<tr>
<td>Combo</td>
</tr>
<tr>
<td>Mono</td>
</tr>
<tr>
<td>Mono</td>
</tr>
<tr>
<td>Mono</td>
</tr>
</tbody>
</table>

*On Treatment

- 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

(2) Banerji, AACR VM 1, April 27, 2020, CT143
KEY TAKEAWAYS

- High unmet need across KRASm lung adenocarcinoma
- High disease control and time on therapy seen across KRASm 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
- Prospective validation of signal in KRAS$^{G12V}$ NSCLC, potential for accelerated approval
- Confirm added benefit of Defactinib
- 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$^{1,2}$

Expansion Opportunities
- Pancreatic$^{1,2}$
- Additional G12V & G12D mt cancers$^{1}$
- Uveal Melanoma$^{2}$
- BRAF mt melanoma$^{1,2}$
- BRAF mt colorectal
- BRAF mt prostate$^{2}$

Other Mutation Opportunities
- GNAQ mutations in uveal melanoma$^{2}$
- NF1 mutations in melanoma
- MAP3K1 mutations in breast cancer

Other Combinations
- KRAS$^{G12C}$ inhibitors
- EGFR inhibitors
- Everolimus$^{2}$
- Anti-PD-1$^{1,2}$

1 Supported by clinical data
2 Supported by preclinical data
Strong Growth for COPIKTRA in 1Q 2020

- **Continue to grow market share in approved indications:**
  - Relapsed/Refractory CLL or SLL after at least two prior therapies
  - Relapsed/Refractory FL after at least two prior systemic therapies - this indication is approved under accelerated approval based on overall response rate

- **Revenue performance:**
  - FY19 net sales of $12.3m vs. FY’18 of $1.7m
  - Q1 20 net sales of $5m, 39% growth over Q4’19

- **Shift in focus of promotional resources toward large community-based practices and academic institutions**
  - Smaller commercial team of 30 (down from 50)
  - Refocus on account based selling using specific account strategies

- **Access restrictions as a result of COVID-19 pandemic**
  - Virtual meetings and speaker programs replace face-to-face HCP interactions
  - New Lab Assist Program introduced to provide assistance to eligible patients that have been prescribed COPIKTRA for required blood tests to be done at home

**FY’20 Net Product Revenue Guidance: ~$16 Million**

**Net Sales of COPIKTRA®**

<table>
<thead>
<tr>
<th>Period</th>
<th>Sales ($M)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Q1’19</td>
<td>$1.7</td>
</tr>
<tr>
<td>Q2’19</td>
<td>$3.0</td>
</tr>
<tr>
<td>Q3’19</td>
<td>$4.0</td>
</tr>
<tr>
<td>Q4’19</td>
<td>$3.6</td>
</tr>
<tr>
<td>Q1’20</td>
<td>$5.0</td>
</tr>
</tbody>
</table>
Ongoing Registration Directed Effort in Relapsed/Refractory Peripheral T-Cell Lymphoma (PTCL)

**US PREVALENCE**
- 1st Line Treatable: 4,000
- R/R Treatable: 2,800

**UNMET NEED**
- Median OS is < 6 months
- NCCN guidelines still recommend clinical trials for relapsed patients
- KOLs are unsatisfied with the available treatment options

---

**EARLY CLINICAL SIGNALS**

<table>
<thead>
<tr>
<th>Drug / Trial</th>
<th>ORR</th>
<th>CR</th>
<th>FDA decision</th>
</tr>
</thead>
<tbody>
<tr>
<td>duvelisib (oral monotherapy)</td>
<td>54%</td>
<td>31%</td>
<td>Fast Track Designation</td>
</tr>
<tr>
<td>Ph 2 Dose Optimization, n = 33 (Horwitz et al., ASH 2019)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>duvelisib + romidepsin</td>
<td>59%</td>
<td>36%</td>
<td>-</td>
</tr>
<tr>
<td>Ph 1 IST, n = 27 (Horwitz et al., ASH 2018)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Folotyn (pralatrexate IV)</td>
<td>27%</td>
<td>8%</td>
<td>AA 2009</td>
</tr>
<tr>
<td>Single arm, n = 109</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Istodax (romidepsin IV)</td>
<td>25.4%</td>
<td>14.6%</td>
<td>AA 2011</td>
</tr>
<tr>
<td>Single arm, n = 130</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Beleodaq (belinostat IV)</td>
<td>25.8%</td>
<td>10.8%</td>
<td>AA 2014</td>
</tr>
<tr>
<td>Single arm, n = 120</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**ONGOING DEVELOPMENT**

**PRIMO** Enrolling
- (~22 sites in US; ~28 sites in Germany, Italy, UK, and Japan)

**IST expansion** (total enrollment ~50)

---

**COPIKTRA is not indicated for use in the treatment of PTCL, and the safety and efficacy of COPIKTRA in PTCL has not been established. Any such use is investigational only. No head-to-head studies have been conducted comparing Duvelisib to these approved products.**

**Sources**
1. Mak et al., Blood 2011 – mOS for relapsed patients ineligible for HDC/SCT;
2. NCCN Guidelines T-cell Lymphoma Version 2.2017; 3. FDA PTCL approval packages

**AA = accelerated approval; CR = complete response; ORR = overall response rate**
**Duvelisib Pipeline – PI3K DELTA / PI3K GAMMA INHIBITOR Focused on Growth Opportunities**

### Company Sponsored Trials

<table>
<thead>
<tr>
<th>Condition</th>
<th>Phase</th>
<th>Collaborator</th>
<th>Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>Relapsed/Refractory CLL/SLL</td>
<td>PHASE 1 / 1B</td>
<td>DUO™</td>
<td>Complete, in long-term follow-up</td>
</tr>
<tr>
<td>Refractory iNHL</td>
<td>PHASE 2</td>
<td>DYNAMO™</td>
<td>Complete, in long-term follow-up</td>
</tr>
<tr>
<td>Relapsed/Refractory PTCL</td>
<td>PHASE 2</td>
<td>PRIMO</td>
<td>Enrolling</td>
</tr>
<tr>
<td>Relapsed/Refractory iNHL – Intermittent Dosing</td>
<td>PHASE 2</td>
<td>TEMPO</td>
<td>Enrolling</td>
</tr>
<tr>
<td>Head and Neck Squamous Cell Carcinoma</td>
<td>PHASE 3</td>
<td>I-O COMBO</td>
<td>Not yet recruiting</td>
</tr>
</tbody>
</table>

### Investigator Sponsored Trials

<table>
<thead>
<tr>
<th>Condition</th>
<th>Phase</th>
<th>Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>1st line, younger CLL/SLL patients</td>
<td></td>
<td>In long term follow-up</td>
</tr>
<tr>
<td>Relapsed/Refractory T Cell Lymphoma</td>
<td>2019</td>
<td>Enrolling</td>
</tr>
<tr>
<td>Relapsed/Refractory CLL/SLL</td>
<td>2019</td>
<td>Enrolling</td>
</tr>
<tr>
<td>Relapsed/Refractory CLL/SLL</td>
<td>2019</td>
<td>Enrolling</td>
</tr>
<tr>
<td>Richter’s Syndrome / Transformed FL</td>
<td></td>
<td>Enrolling</td>
</tr>
</tbody>
</table>

These studies are investigating treatments or outcomes that have not received approval from a Health Authority. The information presented is not intended to convey conclusions of safety or efficacy. There is no guarantee that the outcome of these studies will result in approval by a Health Authority.
2020 Milestones Focus on Growth Opportunities

**VS-6766 & Defactinib**
- Regulatory Discussions on VS-6766 & defactinib
- Initiate Registration Directed Trial in LGSOC
- Initiate Prospective Validation of KRAS$^{G12V}$ NSCLC

**Duvelisib**
- NCCN Guidelines – PTCL
- Complete Accrual on PRIMO
- Interim Analysis of PRIMO
- Japan First Patient In – PRIMO
- DUV + I/O – First Patient In, Safety Data
- Updates on multiple ISTs

**COPIKTRA® Commercial**
- EU Regulatory Opinion
- EU Partnership
- CSPC First Patient in (FL)
- Sanofi Regulatory Filings

**Financial**
- Maintain a Strong Balance Sheet
- FY’20 Net Product Revenue Guidance ~$16M
- FY’20 Operating Expense Guidance $70-$85M
## Key Financial Statistics

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>QTD ended 3/31/2020 Net Product Revenue</td>
<td>$5.0M</td>
</tr>
<tr>
<td>Cash, cash equivalents &amp; short-term investments as of 3/31/2020</td>
<td>$170.3M</td>
</tr>
<tr>
<td>Shares outstanding as of 3/31/2020</td>
<td>162.4M</td>
</tr>
<tr>
<td>Shares fully diluted as of 3/31/2020</td>
<td>182.1M</td>
</tr>
<tr>
<td>Hercules Term Loan Facility as of 3/31/2020</td>
<td>$35.0M*</td>
</tr>
<tr>
<td>5.00% Convertible Senior Notes Due 2048 (2018 Notes) as of 3/31/2020</td>
<td>$28.3M**</td>
</tr>
<tr>
<td>QTD ended 3/31/2020 Non-GAAP Loss</td>
<td>$21.3M</td>
</tr>
<tr>
<td>Full-time equivalent employee as of 3/31/2020</td>
<td>106</td>
</tr>
<tr>
<td>Insider ownership (outstanding / vested) as of 3/31/2020</td>
<td>9.0% / 4.3%</td>
</tr>
</tbody>
</table>

*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 3/31/2020.

**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