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’s Annual Report on Form 10-K for the fiscal year ended December 31, 2018 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.
Novel drug candidates targeting malignant cells both directly and through modulation of the tumor microenvironment

- **NASDAQ**: VSTM
- **Headquarters**: Needham, MA
- **Incorporated**: 2010

**Corporate Overview**

**Products**

The first approved oral inhibitor of PI3K-δ and PI3K-γ
Exclusively marketed in the US by Verastem Oncology

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

**Investigational Research & Pipeline**

**Duvelisib Program**

- Ongoing clinical expansion in PTCL (FDA Fast Track Designation)
- Ongoing clinical investigation as monotherapy and in combination in multiple hematologic malignancies
- **IP**: COM 2030 before extensions
- Partnered in Japan and China

**Defactinib Program**

- Investigational FAK inhibitor
- Clinical Proof-of-Concept of FAK/Immuno-Oncology combinations in 2018
- **IP**: COM 2028 before extensions
- **Orphan Designation**: Ovarian & mesothelioma in the US & EU
COPIKTRA™ (Duvelisib) Capsules

COPIKTRA® is a dual inhibitor of PI3K-δ and PI3K-γ targeting both malignant B cells and their supportive tumor microenvironment

FIRST APPROVED

CLINICAL OUTCOMES AND NEED

COPIKTRA is an effective oral monotherapy regimen with a consistent safety profile

PATIENT BENEFIT

COPIKTRA patients can maintain flexibility in daily life with at-home dosing

HEALTH ECONOMICS

COPIKTRA offers monotherapy administration, with no required hospitalization or infusion

δ & γ
(d e l t a) (gamma)

1 capsule, twice a day

COPIKTRA has been granted approval for the treatment of adult patients with relapsed or refractory CLL or SLL after at least two prior therapies.

COPIKTRA has been granted accelerated approval in adult patients with relapsed or refractory follicular lymphoma after two prior systemic therapies. Safety and efficacy in this patient population have not been confirmed. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

For full prescribing and safety information, including BOXED WARNING, please refer to the Package Insert and Important Safety Information available at www.COPIKTRA.com.
Building a Global Brand
Duvelisib Global Expansion Strategy

UNITED STATES
- Wholly owned & commercialization ready

CANADA
- Regional license
  - Planning to file
  - Evaluating Build or Partner

EU
- Commercialization strategies under review
  - Planning to file in EU
  - Evaluation of Build or Partner Ongoing

CHINA
- Regional license
  - $15M Up-front
  - $160M Development and Sales milestones
  - Double digit royalty

JAPAN
- Regional license
  - $10M Up-front
  - $90M Development and Sales milestones
  - Double digit royalty
Launch Update

Established Teams ahead of Launch: Sales, Marketing, Access, Med Affairs

NCCN Guidelines: CLL/SLL, Follicular, and Marginal Zone Lymphoma

Distribution Established with SD and SP: Product Shipped Day 1*

Health Plans: 90% covering COPIKTRA™

Pricing: $11,800 per 25mg and 15mg Dose Pack (One Month)

Established HUB to support patient access to COPIKTRA™

Majority of Territories have COPIKTRA™ treated patients

*Revenue recognized upon delivery to specialty distributor and specialty pharmacy
Opportunity: Additional Therapy Options are Needed for Chronic iNHL Patients

- **CLL/SLL**
  - US PREVALENCE 2018\(^1\)
  - 197,000
  - 22,205

- **FL**
  - US PREVALENCE 2018\(^1\)
  - 141,000
  - 13,500

**1ST LINE TREATABLE PATIENTS/YEAR (AVG.)\(^1\)**

**Increasing Elderly At-Risk Patient Population**
- 65-75 AGE AT DIAGNOSIS\(^2\)
- AGING BABY BOOMER POPULATION
- INCREASED DIAGNOSES

**Aging Baby Boomer Population**

**Additional Therapy Options Needed for Chronic Disease Control**
- MEDIAN OS
  - 10+ YEARS\(^3\)
- NEED FOR MORE LINES OF THERAPY
- INCREASED DEMAND FOR ORAL TARGETED THERAPIES

**Sources**
1. Decision Resources, 2016-2018 annual estimates; 2018 annual estimates; 2. SEER, FL and CLL statistics; 3. NHI, NHL and CLL PDQ
CLL and SLL Remain Incurable Hematologic Malignancies and Patients Often Relapse or Develop Refractory Disease

Duration of response in the relapsed/refractory setting is shorter\textsuperscript{2}

\begin{itemize}
  \item Patients May Not Respond to or Become Resistant to Targeted Therapies
  \item High-risk Patients Remain Difficult to Treat
  \item Additional Treatment Options are Needed
\end{itemize}

\textbf{PFS by CLL-IPI Risk Stratification (N = 845)}\textsuperscript{1}

\textbf{CLL-ISI = International Prognostic Index for Chronic Lymphocytic Leukemia}

The COPIKTRA™ Opportunity in Relapsed or Refractory CLL/SLL After Two Prior Therapies

COPIKTRA Expands Oral Monotherapy Opportunities

Preference for oral targeted therapies is steadily growing:

- 30% of patients now initiate treatment on a BTK inhibitor, instead of a chemo- or anti-CD20 based regimen

For CHRONIC LYMPHOCYTIC LEUKEMIA / SMALL LYMPHOCYTIC LYMPHOMA patients considering their next therapy

COPIKTRA™ allows for continuation of an oral monotherapy regimen for patients who have relapsed after 2 prior therapies.

1. ATU 2018, Verastem Oncology; 2. Copiktra USPI, Full Approval

For full prescribing and safety information, please refer to the Package Insert and Important Safety information available at www.COPIKTRA.com.
PFS per IRC in Patients with at Least 2 Prior Therapies (N = 196)

COPIKTRA is approved for the treatment of adult patients with relapsed or refractory CLL or SLL after at least two prior therapies.

For full prescribing and safety information, please refer to the Package Insert and Important Safety information available at www.COPIKTRA.com.

Sources
Copiktra USPI, 2018
Kaplan-Meier estimate. CI, confidence interval; HR, hazard ratio; ITT, intention to treat; SE, standard error
COPIKTRA for CLL/SLL Patients with at Least 2 Prior Therapies

PFS Analysis by Selected Variables

PFS Analysis in High-Risk Patient Subgroups (N = 196)*

<table>
<thead>
<tr>
<th>Variable</th>
<th>COPIKTRA</th>
<th>PFS analysis</th>
<th>Ofatumumab</th>
<th>N</th>
<th>HR</th>
</tr>
</thead>
<tbody>
<tr>
<td>Overall</td>
<td></td>
<td></td>
<td></td>
<td>196</td>
<td>0.40</td>
</tr>
<tr>
<td>High-risk cytogenetics</td>
<td></td>
<td></td>
<td></td>
<td>43</td>
<td>0.32</td>
</tr>
<tr>
<td>No high-risk cytogenetics</td>
<td></td>
<td></td>
<td></td>
<td>133</td>
<td>0.38</td>
</tr>
<tr>
<td>Refractory/early relapse</td>
<td></td>
<td></td>
<td></td>
<td>49</td>
<td>0.50</td>
</tr>
<tr>
<td>No refractory/early relapse</td>
<td></td>
<td></td>
<td></td>
<td>147</td>
<td>0.34</td>
</tr>
<tr>
<td>Grade 4 cytopenia(s) at baseline</td>
<td></td>
<td></td>
<td></td>
<td>13</td>
<td>0.19</td>
</tr>
<tr>
<td>No grade 4 cytopenia(s) at baseline</td>
<td></td>
<td></td>
<td></td>
<td>183</td>
<td>0.39</td>
</tr>
<tr>
<td>Male</td>
<td></td>
<td></td>
<td></td>
<td>115</td>
<td>0.47</td>
</tr>
<tr>
<td>Female</td>
<td></td>
<td></td>
<td></td>
<td>81</td>
<td>0.28</td>
</tr>
<tr>
<td>Age &lt;65 years</td>
<td></td>
<td></td>
<td></td>
<td>59</td>
<td>0.42</td>
</tr>
<tr>
<td>Age ≥65 years</td>
<td></td>
<td></td>
<td></td>
<td>137</td>
<td>0.38</td>
</tr>
<tr>
<td>Prior anticancer therapy &lt;12 months</td>
<td></td>
<td></td>
<td></td>
<td>82</td>
<td>0.34</td>
</tr>
<tr>
<td>Prior anticancer therapy ≥12 months</td>
<td></td>
<td></td>
<td></td>
<td>114</td>
<td>0.42</td>
</tr>
<tr>
<td>Not previously treated with ofatumumab</td>
<td></td>
<td></td>
<td></td>
<td>190</td>
<td>0.40</td>
</tr>
<tr>
<td>del(17p) or TP53</td>
<td></td>
<td></td>
<td></td>
<td>59</td>
<td>0.36</td>
</tr>
<tr>
<td>No del(17p) or TP53</td>
<td></td>
<td></td>
<td></td>
<td>103</td>
<td>0.45</td>
</tr>
</tbody>
</table>

Hazard Ratio (95% CI): 0.0 0.5 1.0 1.5 2.0

COPIKTRA is approved for the treatment of adult patients with relapsed or refractory CLL or SLL after at least two prior therapies.

For full prescribing and safety information, please refer to the Package Insert and Important Safety information available at www.COPIKTRA.com.

* Pre-specified patient subgroups; Analysis not powered to show statistical significance in PFS

Sources
Data on file
**COPIKTRA for CLL/SLL Patients with at Least 2 Prior Therapies**

**ORR and LNRR**

**Overall Response Rate (ORR) per IRC**

- **COPIKTRA (n=95)**: 78% (n=74/95)
- **Ofatumumab (n=101)**: 39% (n=39/101)

**Lymph Node Response Rate (LNRR) per IRC**

- **COPIKTRA (n=95)**: 88% (n=84/95) 95% CI: 82.0-94.9
- **Ofatumumab (n=101)**: 14% (n=14/101) 95% CI: 7.1-20.6

Data were evaluated based on the International Workshop on CLL or revised International Working Group response criteria, with modification for treatment-related lymphocytosis.

LNRR was not ranked or formally tested in the hierarchy of key secondary endpoints. Lymph node response was defined as ≥50% reduction in target lesion size.

Sources
1. Copiktra USPI, 2018;
2. Data on file
New FL Promotional Campaign Launched in Q1 2019

Unlike CLL where full promotional materials were available upon FDA approval, all FL materials are based solely on the package insert for the first 120 days. Following the first 120 days, FL promotional materials including an FL campaign, health care professional and patient websites, an FL core visual aid, and more are now available.
FL is an Incurable and Heterogeneous Hematologic Malignancy

- FL is the most common indolent NHL
- High-risk characteristics complicate treatment
- Patient lifestyle must be considered
- Chemoimmunotherapy alternatives are needed

Duration of response becomes shorter after every relapse

PFS According to FLIPI2 (N=832)

<table>
<thead>
<tr>
<th>Score</th>
<th>N</th>
<th>3-year PFS (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>168</td>
<td>90.9</td>
</tr>
<tr>
<td>1-2</td>
<td>444</td>
<td>69.3</td>
</tr>
<tr>
<td>3-5</td>
<td>220</td>
<td>51.3</td>
</tr>
</tbody>
</table>

Sources
Adapted from Federico et al.2
The COPIKTRA™ Opportunity in Relapsed or Refractory FL After Two Prior Systemic Therapies

COPIKTRA Provides a Targeted Therapy Option After Chemo-Immunotherapy

>80% of 2\textsuperscript{nd} line treated FL patients are still re-challenged with a chemotherapy or anti-CD20 based regimen

Oral targeted therapies provide new treatment options

For FOLLICULAR LYMPHOMA patients considering their next therapy

\begin{itemize}
  \item INITIATE THERAPY
  \item RE-CHALLENGE
  \item LONG-TERM DISEASE CONTROL
\end{itemize}

1. Chemo ± CD20
2. Chemo ± CD20 or CD20
3. COPIKTRA™ is an additional option for FL patients who have relapsed or are refractory to 2 prior systemic therapies\textsuperscript{2}

\textsuperscript{1} 2\textsuperscript{ND} LINE

COPIKTRA is approved for the treatment of adult patients with relapsed or refractory CLL or SLL after at least two prior therapies.

For full prescribing and safety information, please refer to the Package Insert and Important Safety information available at www.COPIKTRA.com.

Sources
1. ATU 2018, Verastem Oncology; 2. Copiktra USPI, Accelerated Approval
## Efficacy in Patients with Relapsed or Refractory FL

<table>
<thead>
<tr>
<th>Outcome per IRC</th>
<th>FL N = 83</th>
</tr>
</thead>
<tbody>
<tr>
<td>ORR, n (%) (^a)</td>
<td>35 (42%)</td>
</tr>
<tr>
<td>95% CI</td>
<td>(31, 54)</td>
</tr>
<tr>
<td>CR, n (%)</td>
<td>1 (1%)</td>
</tr>
<tr>
<td>PR, n (%)</td>
<td>34 (41%)</td>
</tr>
</tbody>
</table>

### Duration of response

<table>
<thead>
<tr>
<th></th>
<th>FL N = 83</th>
</tr>
</thead>
<tbody>
<tr>
<td>Range, months</td>
<td>0.0(^<em>) to 41.9(^</em>)</td>
</tr>
<tr>
<td>Patients maintaining response at 6 months, n/N (%)</td>
<td>15/35 (43%)</td>
</tr>
<tr>
<td>Patients maintaining response at 12 months, n/N (%)</td>
<td>6/35 (17%)</td>
</tr>
</tbody>
</table>

*Abbreviations: CI = confidence interval; CR = complete response; IRC = Independent Review Committee; ORR = overall response rate; PR = partial response*

\(^a\) Per IRC according to Revised International Working Group criteria

\(^*\) Denotes censored observation

- Primary data supporting accelerated approval is from the DYNAMO™ Phase 2 trial of duvelisib in patients with refractory indolent NHL
- Heavily pre-treated double refractory patient population, with median of 3 prior lines of therapy

_inclusion criteria required that patients be refractory to both rituximab and a chemotherapy regimen or RT._

_Refractory is defined as no response while on therapy, or progressive disease within 6 months of the last dose._

---

**FL: Data Supporting Accelerated Approval**

- **Copiktra** USPI, 2018

**Copiktra** is approved for the treatment of adult patients with relapsed or refractory CLL or SLL after at least two prior therapies.

For full prescribing and safety information, please refer to the Package Insert and Important Safety information available at www.Copiktra.com.
DYNAMO™
88% of Patients in the DYNAMO™ Study had Reduction in Target Lymph Nodes by IRC

88% of pts had reduction in target lymph nodes (per IRC)

Duvelisib is an investigational agent available for clinical trial use only. Safety and efficacy have not been established.

Sources
Zinzani et al., 14-ICML, 14 June 2017, Lugano Switzerland
Indication
COPIKTRA is a kinase inhibitor indicated for the treatment of adult patients with:

- Relapsed or refractory chronic lymphocytic leukemia (CLL) or small lymphocytic lymphoma (SLL) after at least two prior therapies.
- Relapsed or refractory follicular lymphoma (FL) after at least two prior systemic therapies.

This indication is approved under accelerated approval based on overall response rate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

Dosing & Administration
25 mg orally, twice daily. Modify dosage for toxicity.

Selected Important Safety Information
WARNING: FATAL AND SERIOUS TOXICITIES: INFECTIONS, DIARRHEA OR COLITIS, CUTANEOUS REACTIONS, and PNEUMONITIS

- Fatal and/or serious infections occurred in 31% of COPIKTRA-treated patients. Monitor for signs and symptoms of infection. Withhold COPIKTRA if infection is suspected.
- Fatal and/or serious diarrhea or colitis occurred in 18% of COPIKTRA-treated patients. Monitor for the development of severe diarrhea or colitis. Withhold COPIKTRA.
- Fatal and/or serious cutaneous reactions occurred in 5% of COPIKTRA-treated patients. Withhold COPIKTRA.
- Fatal and/or serious pneumonitis occurred in 5% of COPIKTRA-treated patients. Monitor for pulmonary symptoms and interstitial infiltrates. Withhold COPIKTRA.

Warnings and Precautions
- Hepatotoxicity: Monitor hepatic function.
- Neutropenia: Monitor blood counts.
- Embryo-Fetal toxicity: COPIKTRA can cause fetal harm. Advise patients of potential risk to a fetus and to use effective contraception.

Contraindications: None.

Most common adverse reactions (> 20%): Diarrhea or colitis, neutropenia, rash, fatigue, pyrexia, cough, nausea, upper respiratory infection, pneumonia, musculoskeletal pain, and anemia.
### Most Common Adverse Reactions (≥ 10% Grade ≥ 3 or ≥ 20% Any Grade) in Patients with B-cell Malignancies Receiving COPIKTRA

<table>
<thead>
<tr>
<th>Adverse Reactions</th>
<th>COPIKTRA 25 mg BID (N = 442)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Grade ≥ 3 n (%)</td>
</tr>
<tr>
<td>Neutropenia †</td>
<td>132 (30%)</td>
</tr>
<tr>
<td>Diarrhea or colitis †</td>
<td>101 (23%)</td>
</tr>
<tr>
<td>Pneumonia †</td>
<td>67 (15%)</td>
</tr>
<tr>
<td>Anemia †</td>
<td>48 (11%)</td>
</tr>
<tr>
<td>Rash †</td>
<td>41 (9%)</td>
</tr>
<tr>
<td>Fatigue †</td>
<td>22 (5%)</td>
</tr>
<tr>
<td>Pyrexia</td>
<td>7 (2%)</td>
</tr>
<tr>
<td>Musculoskeletal pain †</td>
<td>6 (1%)</td>
</tr>
<tr>
<td>Nausea †</td>
<td>4 (&lt;1%)</td>
</tr>
<tr>
<td>Cough †</td>
<td>2 (&lt;1%)</td>
</tr>
<tr>
<td>Upper respiratory tract infection †</td>
<td>2 (&lt;1%)</td>
</tr>
</tbody>
</table>

Serious adverse reactions were reported in 289 patients (65%). The most frequent serious adverse reactions that occurred were:

- infection (31%) †
- diarrhea or colitis (18%) †
- pneumonia (17%) †
- rash (5%) †
- pneumonitis (5%) †

† Grouped term for reactions with multiple preferred terms

* Diarrhea or colitis includes the preferred terms: colitis, enterocolitis, colitis microscopic, colitis ulcerative, diarrhea, diarrhea hemorrhagic

* Pneumonia includes the preferred terms: All preferred terms containing “pneumonia” except for “pneumonia aspiration”; bronchopneumonia, bronchopulmonary aspergillosis

* Rash includes the preferred terms: dermatitis (including allergic, exfoliative, perivascular), erythema (including multiforme), rash (including exfoliative, erythematous, follicular, generalized, macular & papular, pruritic, pustular), toxic epidermal necrolysis and toxic skin eruption, drug reaction with eosinophilia and systemic symptoms, drug eruption, Stevens-Johnson syndrome

COPIKTRA is approved for the treatment of adult patients with relapsed or refractory CLL or SLL after at least two prior therapies.

For full prescribing and safety information, please refer to the Package Insert and Important Safety Information available at www.COPIKTRA.com.
Grow COPIKTRA™ Through Clinical Growth

**TODAY:**

**ANCHOR**
Monotherapy for R/R FL and CLL/SLL after 2 Prior Lines

- FL: 13,000 incidence, 141,000 prevalence
- CLL: 23,000 incidence, 197,000 prevalence

**BROADEN REACH**
Expand in CLL/SLL and FL
Expand into PTCL†

**BOLD STEPS**
Aggressive NHL Subtypes
- DLBCL, MCL, Richter’s, Transformed FL†

**MAXIMIZE POTENTIAL**
Combinations with I-O and CAR-T
- Solid Tumors, NHL†

**Sources:**
1. Copiktra USPI, 2018 – Accelerated Approval in FL, Full approval in CLL/SLL;
2. Decision Resources, US 2018

**STEP 1**
Composition of Matter: 2030

COPIKTRA is approved for the treatment of adult patients with relapsed or refractory CLL or SLL after at least two prior therapies.
For full prescribing and safety information, please refer to the Package Insert and Important Safety Information available at www.COPIKTRA.com.
R/R PTCL: Duvelisib Clinical Development

**UNMET NEED**

- Median OS is < 6 months\(^1\)
- NCCN guidelines still recommend clinical trials for relapsed patients\(^2\)
- 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><strong>INVESTIGATIONAL</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>duvelisib (oral monotherapy) Ph 1 subpopulation, n = 16 (Horwitz et al., Blood 2018)</td>
<td>50%</td>
<td>19%</td>
<td>Fast Track Designation</td>
</tr>
<tr>
<td>duvelisib + romidepsin Ph 1 IST, n = 27 (Horwitz et al., ASH 2018)</td>
<td>59%</td>
<td>36%</td>
<td>-</td>
</tr>
<tr>
<td><strong>APPROVED(^3)</strong></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Folotyn (pralatrexate IV) Single arm, n = 109</td>
<td>27%</td>
<td>8%</td>
<td>AA 2009</td>
</tr>
<tr>
<td>Istodax (romidepsin IV) Single arm, n = 130</td>
<td>25.4%</td>
<td>14.6%</td>
<td>AA 2011</td>
</tr>
<tr>
<td>Beleodaq (belinostat IV) Single arm, n = 120</td>
<td>25.8%</td>
<td>10.8%</td>
<td>AA 2014</td>
</tr>
</tbody>
</table>

**ONGOING DEVELOPMENT**

- Enrolling
- IST expansion (total enrollment ~50)

---

**Sources**


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.
### Pipeline Overview

#### Duvelisib (PI3K Delta/PI3K Gamma Inhibitor)

<table>
<thead>
<tr>
<th>Phase 1 / 1B</th>
<th>Phase 2</th>
<th>Phase 3</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Relapsed/Refractory CLL/SLL</strong>&lt;br&gt;Randomized open label vs. ofatumumab</td>
<td>DUO™&lt;br&gt;Complete, in long-term follow-up</td>
<td><strong>Collaborator</strong></td>
</tr>
<tr>
<td><strong>Refractory iNHL</strong>&lt;br&gt;Single arm, monotherapy</td>
<td>DYNAMO™&lt;br&gt;Complete, in long-term follow-up</td>
<td><strong>Collaborator</strong></td>
</tr>
<tr>
<td><strong>Relapsed/Refractory PTCL</strong>&lt;br&gt;Single arm, monotherapy</td>
<td>PRIMO™&lt;br&gt;Enrolling</td>
<td><strong>Collaborator</strong></td>
</tr>
<tr>
<td><strong>1st line, younger CLL/SLL patients</strong>&lt;br&gt;Single arm, with FCR</td>
<td>In long term follow-up</td>
<td><strong>Collaborator</strong></td>
</tr>
<tr>
<td><strong>Relapsed/Refractory T Cell Lymphoma</strong>&lt;br&gt;With Romidepsin or Bortezomib</td>
<td>Enrolling</td>
<td><strong>Collaborator</strong></td>
</tr>
<tr>
<td><strong>Relapsed/Refractory CLL/SLL</strong>&lt;br&gt;With Venetoclax</td>
<td>Enrolling</td>
<td><strong>Collaborator</strong></td>
</tr>
</tbody>
</table>

#### Defactinib (FAK Inhibitor)

<table>
<thead>
<tr>
<th>NSCLC, Pancreatic, Mesothelioma&lt;sup&gt;*&lt;/sup&gt;&lt;br&gt;With pembrolizumab</th>
<th>Enrolling</th>
<th><strong>Collaborator</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td>Pancreatic, relapsed&lt;sup&gt;*&lt;/sup&gt;&lt;br&gt;With pembrolizumab + gemcitabine</td>
<td>Dose-escalation complete; In expansion phase</td>
<td><strong>Collaborator</strong></td>
</tr>
<tr>
<td>Advanced Solid Tumors&lt;sup&gt;*&lt;/sup&gt;&lt;br&gt;With RAF/MEK Inhibitor</td>
<td>Dose-escalation complete; In expansion phase</td>
<td><strong>Collaborator</strong></td>
</tr>
<tr>
<td>Carboplatin Resistant Ovarian&lt;sup&gt;*&lt;/sup&gt;&lt;br&gt;With Platinum + Taxane</td>
<td>Dose-escalation</td>
<td><strong>Collaborator</strong></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.*

*Investigator Sponsored Trial (IST)*
## Key Financial Statistics

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash, cash equivalents &amp; investments as of 03/31/2019</td>
<td>$211.7M</td>
</tr>
<tr>
<td>Shares outstanding as of 3/31/2019</td>
<td>73.9M</td>
</tr>
<tr>
<td>Shares fully diluted as of 3/31/2019</td>
<td>109.7M</td>
</tr>
<tr>
<td>Hercules Term Loan Facility</td>
<td>$35.0M*</td>
</tr>
<tr>
<td>5.00% Convertible Senior Notes Due 2048</td>
<td>$150.0M**</td>
</tr>
<tr>
<td>QTD net loss as of 3/31/2019</td>
<td>$38.1M</td>
</tr>
<tr>
<td>QTD cash used in operating activities as of 3/31/2019</td>
<td>$38.8M</td>
</tr>
<tr>
<td>Full-time employees as of 3/31/2019</td>
<td>182</td>
</tr>
<tr>
<td>Insider ownership (outstanding / vested) as of 3/31/2019</td>
<td>17.3% / 8.7%</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.

**The Senior Convertible Notes have an initial conversion rate of 139.5771 shares of Common Stock per $1,000, which translates to an initial conversion price of approximately $7.16 per share of Common Stock.
### 2019 Milestones

<table>
<thead>
<tr>
<th>Category</th>
<th>Description</th>
</tr>
</thead>
<tbody>
<tr>
<td>Commercial Traction</td>
<td>Continuing to expand on the commercial traction of COPIKTRA in CLL/SLL and FL</td>
</tr>
<tr>
<td>Commercial Traction</td>
<td>Expansion of the PRIMO study evaluating duvelisib monotherapy in PTCL</td>
</tr>
<tr>
<td>Commercial Traction</td>
<td>Initiating confirmatory Phase 3 study evaluating duvelisib for the treatment of R/R FL after at two prior systemic therapies</td>
</tr>
<tr>
<td>Commercial Traction</td>
<td>Initiating additional investigational studies of duvelisib as a monotherapy and in combination with other anti-cancer agents, such as checkpoint inhibitors, in both hematological and solid tumor malignancies</td>
</tr>
<tr>
<td>Working with LLS</td>
<td>Working with LLS to advance the PTCL program including the expansion of the Phase 2 combination study of duvelisib and romidepsin for patients with relapsed or refractory PTCL</td>
</tr>
<tr>
<td>Additional Ex-U.S. Partnership</td>
<td>Additional ex-U.S. partnership for duvelisib</td>
</tr>
<tr>
<td>Presenting and Publishing</td>
<td>Presenting and publishing additional duvelisib data</td>
</tr>
<tr>
<td>Advanced Defactinib</td>
<td>Advancing defactinib in three separate clinical collaborations in combination with immunotherapeutic agents for the treatment of several different cancer types including pancreatic cancer, non-small cell lung cancer (NSCLC), and mesothelioma.</td>
</tr>
</tbody>
</table>
We’re driven by the strength, tenacity, and courage of those battling cancer—

Single minded in our resolve to deliver new therapies that not only keep cancer at bay, but improve the lives of patients diagnosed with cancer. Dedicated to improving how physicians care for their patients, and how caregivers cope with looking after a loved one or friend.

Because for us, it’s personal.
COPIKTRA TARGETS Malignant B Cells and Disrupts the tumor microenvironment

**PI3K-δ (delta) inhibition predominantly restricts malignant B-cell growth and survival**

- Prohibit proliferation and reduce viability in malignant B cells with PI3K-δ inhibition

**PI3K-γ (gamma) inhibition helps modulate the tumor microenvironment, a network of nonneoplastic cells essential to malignant B-cell survival and proliferation**

- Block CXCL12-induced T-cell migration and M2 macrophage polarization with PI3K-γ inhibition (based on pre-clinical studies)

COPIKTRA maintains pressure on an established cancer-growth pathway in CLL/SLL

By targeting both PI3K-δ and PI3K-γ, COPIKTRA can help address the complex pathogenesis of CLL/SLL

3. Data on File, Verastem Oncology.
Phase 1b/2 IST of Duvelisib + FCR for Younger Patients with Previously Untreated CLL

- Best response of MRD- seen in 76% of patients, a significantly higher rate than historical data with FCR, and similar to the ibrutinib + FCR regimen
  - High rates of MRD- observed even in higher risk CLL populations, such as patients with unmutated IGHV
- Responses and bone marrow MRD negativity (MRD-) deepened on duvelisib maintenance
- DFCR toxicities are comparable to duvelisib and FCR individually, with infectious, immune-mediated toxicities, and secondary malignancies observed

**Best response on study***

<table>
<thead>
<tr>
<th></th>
<th>ORR (n = 31)</th>
<th>CR† with MRD negativity</th>
<th>MRD negativity</th>
</tr>
</thead>
<tbody>
<tr>
<td>PR</td>
<td>42%</td>
<td></td>
<td></td>
</tr>
<tr>
<td>CR*</td>
<td>52%</td>
<td>55%</td>
<td>76%</td>
</tr>
<tr>
<td>MRD negativity</td>
<td>n = 29</td>
<td>n = 29</td>
<td></td>
</tr>
</tbody>
</table>

*Interim data, as presented on 16 June 2018 at EHA

† Includes CR and CRi

COPIKTRA is not indicated for use in the treatment of previously untreated CLL patients or in combination with FCR. The safety and efficacy of COPIKTRA in this setting has not been established. Any such use is investigational only.

**Source**

Davids et al. EHA 2018
CONTEMPO: Phase 1b/2 study of duvelisib + rituximab or obinutuzumab in previously untreated CD20+ FL

- Safety profile of duvelisib in combination with anti-CD20 mAbs observed to date has been consistent with previously established safety profile of duvelisib monotherapy
- Both DR and DO combination therapies exhibited preliminary activity and modulation of tumor-supportive factors in the tumor microenvironment
- Interim data is supportive of the potential role of duvelisib + anti-CD20 as initial treatment for FL patients

### ORR by investigator

<table>
<thead>
<tr>
<th></th>
<th>DR Arm (N = 28)</th>
<th>DO Arm (N = 27)</th>
</tr>
</thead>
<tbody>
<tr>
<td>CR</td>
<td>36%</td>
<td>41%</td>
</tr>
<tr>
<td>PR</td>
<td>57%</td>
<td>48%</td>
</tr>
<tr>
<td>100%</td>
<td>93%</td>
<td>89%</td>
</tr>
<tr>
<td>80%</td>
<td>80%</td>
<td>75%</td>
</tr>
<tr>
<td>60%</td>
<td>60%</td>
<td>55%</td>
</tr>
<tr>
<td>40%</td>
<td>40%</td>
<td>35%</td>
</tr>
<tr>
<td>20%</td>
<td>20%</td>
<td>15%</td>
</tr>
<tr>
<td>0%</td>
<td>0%</td>
<td>0%</td>
</tr>
</tbody>
</table>

#### Pharmacodynamics:
In both arms, chemokines reflective of the tumor microenvironment were inhibited

#### Safety, DR arm:
- TEAE ≥ Gr 3: 68%
- TEAE leading to discontinuation: 36%

#### Safety, DO arm:
- TEAE ≥ Gr 3: 89%
- TEAE leading to discontinuation: 48%

### Most common AEs ≥ Gr 3

<table>
<thead>
<tr>
<th>AEs</th>
<th>DR (N = 28)</th>
<th>DO (N = 27)</th>
</tr>
</thead>
<tbody>
<tr>
<td>ALT increased</td>
<td>25%</td>
<td>26%</td>
</tr>
<tr>
<td>Diarrhea</td>
<td>25%</td>
<td>15%</td>
</tr>
<tr>
<td>Infections</td>
<td>14%</td>
<td>22%</td>
</tr>
<tr>
<td>AST increased</td>
<td>11%</td>
<td>15%</td>
</tr>
<tr>
<td>Neutropenia</td>
<td>11%</td>
<td>19%</td>
</tr>
<tr>
<td>Rash</td>
<td>14%</td>
<td>11%</td>
</tr>
</tbody>
</table>

Source: 1. CSR, data cut-off 30 March 2017; 2. Casulo et al., ASCO 2018
Duvelisib & Venetoclax Show Synergy in R/R CLL Cell Lines

### Combination Index

<table>
<thead>
<tr>
<th>Venetoclax / 0.17 μM</th>
<th>Duvelisib / 0.33 μM</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>0.1</td>
<td>0.5</td>
</tr>
<tr>
<td>0.2</td>
<td>1.0</td>
</tr>
</tbody>
</table>

**DOHH-2 (cell line)**

**Combination Index**

**Growth Inhibition (%) = 180.0**

**Isobologram**

**None**

**GM**

**Z Factor**

**Infinity Compound**

**1 / 0.33μM**

**ABT-199 / 0.17μM**

### Study Design

- **Arm 1:** duvelisib 25 mg BID + venetoclax 100 mg
- **Arm 2:** duvelisib 25 mg BID + venetoclax 200 mg
- **Arm 3:** duvelisib 25 mg BID + venetoclax 400 mg

### Safety lead-in

### Phase I/II IST in patients with R/R CLL/SLL
- Up to 23 patients
- Strong scientific rationale for duvelisib/venetoclax combo
  - Duvelisib treatment of CLL patients increases pro-apoptotic proteins (primed for apoptosis) and BCL2 (guardian against apoptosis)
  - CLL cells from duvelisib–treated patients show increased cell death in response to venetoclax (BCL2 inhibitor) ex vivo

**COIKTRA is not indicated for use in combination with venetoclax. The safety and efficacy of COPIKTRA in this setting has not been established. Any such use is investigational only.**
Duvelisib is Synergistic with PD-1 and OX40 Antibodies in B-cell Lymphoma (A20) Preclinical Model

- Duvelisib @ 50 mg/kg po, BID
- Anti-PD-1 @ 100 mg/mouse ip, biweekly x 2

- PI3K-delta inhibition is known to reduce immunosuppressive Tregs & enrich memory T cells

- PI3K-gamma inhibition is known to reduce immunosuppressive myeloid cells


COPIKTRA is not indicated for use in the treatment of B-cell lymphoma or in combination with PD-1. The safety and efficacy of COPIKTRA in this setting has not been established. Any such use is investigational only.
Senior Management Team

Robert Forrester  
President/Chief Executive Officer  
CEO/CFO - CombinatoRx, COLY  
MeesPierson, Barclays, UBS

Cathy Carew  
Chief People & Organizational Strategy Officer  
Principal - HR Collaborative  
Ironwood, ActiveBiotics, Dynogen, Tufts Health Plan

Rob Gagnon  
Chief Financial Officer  
CFO – Harvard Bioscience, Clean Harbors  
VP of Finance – Biogen Idec

Joseph Lobacki  
Chief Commercial Officer  
CCO – Medivation and Micromet  
SVP and General Manager, Genzyme  
Xtandi®, Mozobil® and Clolar/Evoltra®

Jonathan Pachter, Ph.D.  
Chief Scientific Officer  
Head of Cancer Biology - OSI (now Astellas)

Hagop Youssoufian, MSc, M.D.  
Head of Medical Strategy  
CMO, BIND Therapeutics, EVP, Progenics,  
CMO & EVP, Ziopharm Oncology, SVP, Imclone

Daniel Paterson  
Chief Operating Officer  
CEO - The DNA Repair Co. (now On-Q-ity)  
PharMetrics (now IMS), Axion