Forward Looking Statements

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, 2017 and in our subsequent reports on Form 10-Q, 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.
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.
Corporate Overview

Novel drug candidates targeting malignant cells both directly and through modulation of the tumor microenvironment

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

Changing the way cancer is treated

Product

The first approved 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
The first approved dual inhibitor of PI3K delta and gamma is

NOW AVAILABLE

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.

Full Prescribing Information, including BOXED WARNING and Medication Guide, is available at www.COPIKTRA.com
Approved Oncology Drugs are Rare and Valuable

First NDA drug approvals for small to midcap oncology biotech companies from 2010

Source: MTS Health Partners, March 2018
**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.

Source: Copiktra USPI, 2018
### Efficacy in Patients with Relapsed or Refractory FL

<table>
<thead>
<tr>
<th>Endpoint</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>
<tr>
<td><strong>Duration of response</strong></td>
<td></td>
</tr>
<tr>
<td>Range, months</td>
<td>0.0(^+) to 41.9(^+)</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

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**Primary data supporting accelerated approval** is from the DYNAMO™ Phase 2 trial of duvelisib in patients with refractory indolent NHL.

**Heavily pre-treated refractory patient population**
- Median of 3 prior lines of therapy
- 94% refractory to their last therapy
- 81% refractory to 2 or more prior lines of therapy

**Inclusion criteria for the study required that patients be refractory to both rituximab and a chemotherapy regimen or RIT**

**Refractory is defined as no response while on therapy, or progressive disease within 6 months of the last dose**

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**Source:** Copiktra USPI, 2018

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**COPIKTRA is approved for the treatment of adult patients with relapsed or refractory follicular lymphoma after at least two prior systemic therapies. Accelerated approval was granted in this indication based on overall response rate (ORR). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.**

For full prescribing and safety information, please refer to the Package Insert and Important Safety Information available at www.COPIKTRA.com.
# Efficacy in Patients with CLL or SLL After at Least Two Prior Therapies

<table>
<thead>
<tr>
<th>Outcome per IRC</th>
<th>COPIKTRA N = 95</th>
<th>Ofatumumab N = 101</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>PFS</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Number of events, n (%)</td>
<td>55 (58%)</td>
<td>70 (69%)</td>
</tr>
<tr>
<td>Progressive disease</td>
<td>44</td>
<td>62</td>
</tr>
<tr>
<td>Death</td>
<td>11</td>
<td>8</td>
</tr>
<tr>
<td>Median PFS (SE), months&lt;sup&gt;a&lt;/sup&gt;</td>
<td>16.4 (2.1)</td>
<td>9.1 (0.5)</td>
</tr>
<tr>
<td>Hazard Ratio (SE)&lt;sup&gt;b&lt;/sup&gt;, COPIKTRA/ofatumumab</td>
<td>0.40 (0.2)</td>
<td></td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Response rate</th>
<th>COPIKTRA</th>
<th>Ofatumumab</th>
</tr>
</thead>
<tbody>
<tr>
<td>ORR n (%)&lt;sup&gt;c&lt;/sup&gt;</td>
<td>74 (78%)</td>
<td>39 (39%)</td>
</tr>
<tr>
<td>CR</td>
<td>0 (0%)</td>
<td>0 (0%)</td>
</tr>
<tr>
<td>PR</td>
<td>74 (78%)</td>
<td>39 (39%)</td>
</tr>
<tr>
<td>Difference in ORR, % (SE)</td>
<td>39% (6.4)</td>
<td></td>
</tr>
</tbody>
</table>

Abbreviations: CR = complete response; IRC = Independent Review Committee; PFS = progression-free survival; PR = partial response; SE = standard error
<sup>a</sup> Kaplan-Meier estimate
<sup>b</sup> Standard Error of ln(hazard ratio) = 0.2
<sup>c</sup> IWCLL or revised IWG response criteria, with modification for treatment-related lymphocytosis

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**Source:** Copiktra USPI, 2018

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- Primary data supporting full approval is from the DUO™ Phase 3 trial of duvelisib vs. ofatumumab in patients with relapsed/refractory CLL (N = 319)
- Heavily pre-treated overall patient population
- Full approval is received based on analysis of the 61% of patients who received at least 2 prior lines of therapy

**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.

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**Verastem Oncology**

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COPIKTRA for CLL/SLL patients with at least 2 prior therapies

COPIKTRA demonstrated >7 month mPFS advantage vs. ofatumumab & decreased risk of progression across high-risk patient subgroups

Kaplan-Meier Curve of PFS per IRC in Patients with at Least 2 Prior Therapies (N = 196)

PFS analysis for high-risk patient subgroups

<table>
<thead>
<tr>
<th>Variable</th>
<th>PFS analysis</th>
<th>N</th>
<th>HR</th>
</tr>
</thead>
<tbody>
<tr>
<td>Overall</td>
<td><img src="image" alt="Diagram" /></td>
<td>196</td>
<td>0.40</td>
</tr>
<tr>
<td>High-risk cytogenetics</td>
<td><img src="image" alt="Diagram" /></td>
<td>43</td>
<td>0.32</td>
</tr>
<tr>
<td>No high-risk cytogenetics</td>
<td><img src="image" alt="Diagram" /></td>
<td>133</td>
<td>0.38</td>
</tr>
<tr>
<td>Refractory/early relapse</td>
<td><img src="image" alt="Diagram" /></td>
<td>49</td>
<td>0.50</td>
</tr>
<tr>
<td>No refractory/early relapse</td>
<td><img src="image" alt="Diagram" /></td>
<td>167</td>
<td>0.34</td>
</tr>
<tr>
<td>Grade 4 cytopenia(s) at baseline</td>
<td><img src="image" alt="Diagram" /></td>
<td>13</td>
<td>0.19</td>
</tr>
<tr>
<td>No grade 4 cytopenia(s) at baseline</td>
<td><img src="image" alt="Diagram" /></td>
<td>183</td>
<td>0.39</td>
</tr>
<tr>
<td>Male</td>
<td><img src="image" alt="Diagram" /></td>
<td>115</td>
<td>0.47</td>
</tr>
<tr>
<td>Female</td>
<td><img src="image" alt="Diagram" /></td>
<td>81</td>
<td>0.26</td>
</tr>
<tr>
<td>Age &lt;65 years</td>
<td><img src="image" alt="Diagram" /></td>
<td>59</td>
<td>0.42</td>
</tr>
<tr>
<td>Age &gt;65 years</td>
<td><img src="image" alt="Diagram" /></td>
<td>137</td>
<td>0.38</td>
</tr>
<tr>
<td>Prior antitumor therapy &lt;12 months</td>
<td><img src="image" alt="Diagram" /></td>
<td>82</td>
<td>0.34</td>
</tr>
<tr>
<td>Prior antitumor therapy ≥12 months</td>
<td><img src="image" alt="Diagram" /></td>
<td>114</td>
<td>0.42</td>
</tr>
<tr>
<td>Not previously treated with ofatumumab</td>
<td><img src="image" alt="Diagram" /></td>
<td>100</td>
<td>0.40</td>
</tr>
<tr>
<td>ddi(17p) or TP53</td>
<td><img src="image" alt="Diagram" /></td>
<td>59</td>
<td>0.36</td>
</tr>
<tr>
<td>No ddi(17p) or TP53</td>
<td><img src="image" alt="Diagram" /></td>
<td>103</td>
<td>0.45</td>
</tr>
</tbody>
</table>

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

Source: 1. Copiktra USPI, 2018; 2. Data on file

**Note:**

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.
COPIKTRA for CLL/SLL patients with at least 2 prior therapies

The majority of patients achieved a partial response with COPIKTRA & 88% saw a ≥50% reduction in target lymph nodes

Overall Response Rate (ORR) per IRC

- 78% (n=74/95)
- 39% (n=39/101)

Lymph Node Response Rate (LNRR) per IRC

- 88% (n=84/95) 95% CI: 82.0-94.9
- 14% (n=14/101) 95% CI: 71-20.6

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.

Source: 1. Copiktra USPI, 2018; 2. Data on file

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.
### 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 †a</td>
<td>101 (23%)</td>
</tr>
<tr>
<td>Pneumonia †b</td>
<td>67 (15%)</td>
</tr>
<tr>
<td>Anemia †</td>
<td>48 (11%)</td>
</tr>
<tr>
<td>Rash †c</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>

<table>
<thead>
<tr>
<th>Note:</th>
</tr>
</thead>
<tbody>
<tr>
<td>† Grouped term for reactions with multiple preferred terms</td>
</tr>
<tr>
<td>†a Diarrhea or colitis includes the preferred term: colitis, enterocolitis, colitis microscopic, colitis ulcerative, diarrhea, diarrhea hemorrhagic</td>
</tr>
<tr>
<td>†b Pneumonia includes the preferred terms: All preferred terms containing &quot;pneumonia&quot; except for &quot;pneumonia aspiration&quot;; bronchopneumonia, bronchopulmonary aspergillosis</td>
</tr>
<tr>
<td>†c Rash includes the preferred terms: dermatitis (including allergic, exfoliative, perivascular), erythema (including multiforme), rash (including exfoliative, erythematous, follicular, generalized, macular &amp; papular, pruritic, purpuric, toxic epidermal necrolysis and toxic skin eruption, drug reaction with eosinophilia and systemic symptoms, drug eruption, Stevens-Johnson syndrome</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%) †

For full prescribing and safety information, please refer to the Package Insert and Important Safety Information available at www.COPIKTRA.com.
Consistent safety and tolerability profile across B-cell malignancies

- Serious opportunistic infections < 4%: PCP (unconfirmed) (n=1); CMV (n=2); fungal pneumonia (n=1)
- Deaths attributed to treatment (n=6)*
  * colitis (n=1); toxic epidermal necrolysis/sepsis syndrome (n=1); drug reaction/eosinophilia/systemic symptoms (n=1); pneumonitis/pneumonia (n=1); viral infection (n=1); septic shock (n=1)
- Severe opportunistic infections (6%): bronchopulmonary aspergillosis (n=4), fungal infection (n=2), Pneumocystis jirovecii pneumonia (n=1)*, and cytomegalovirus colitis (n=1)
  - No severe herpes zoster infections
- Deaths attributed to treatment (n=4)**
  * Neither patient on prophylaxis at the time of the event
  ** general health deterioration (n=1); pneumonia staphylococcal (n=2); sepsis (n=1)

Source: Zinzani et al., ICML 2017
Source: Flinn et al., ASH 2017
COPIKTRA™ (duvelisib) capsules

**FIRST APPROVED**

δ & γ (delta) (gamma)  
COPIKTRA is a dual inhibitor of PI3K-δ and PI3K-γ targeting both malignant B cells and their supportive tumor microenvironment

**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

COPIKTRA has been granted full 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.
Additional therapy options are needed for chronic iNHL patients

**CLL/SLL**
- US PREVALENCE 2018: 197,000
- 1ST LINE TREATABLE PATIENTS/YEAR (AVG.): 22,205
- AGE AT DIAGNOSIS: 65-75
- MEDIAN OS: 10+ YEARS

**FL**
- US PREVALENCE 2018: 141,000
- MEDIAN OS: 10+ YEARS

**Sources:**
1. Decision Resources, 2016-2018 annual estimates; 2018 annual estimates; 2. SEER, FL and CLL statistics; 3. NHI, NHL and CLL PDQ

**Increasing Elderly At-Risk Patient Population**
- AGING BABY BOOMER POPULATION
- INCREASED DIAGNOSES

**Additional Therapy Options Needed for Chronic Disease Control**
- NEED FOR MORE LINES OF THERAPY
- INCREASED DEMAND FOR ORAL TARGETED THERAPIES

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

COPIKTRA is approved for the treatment of adult patients with relapsed or refractory follicular lymphoma after at least two prior systemic therapies. Accelerated approval was granted in this indication based on overall response rate (ORR). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.
The COPIKTRA™ opportunity in relapsed or refractory FL after two prior systemic therapies

COPIKTRA provides a targeted therapy option after chemo-immunotherapy

For FOLLICULAR LYMPHOMA patients considering their next therapy

Oral targeted therapies provide new treatment options

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

2ND LINE

INITIATE THERAPY

RE-CHALLENGE

LONG-TERM DISEASE CONTROL

COPIKTRA™ is an additional option for FL patients who have relapsed or are refractory to 2 prior systemic therapies

Sources: 1. ATU 2018, Verastem Oncology; 2. Copiktra USPI, Accelerated Approval

COPIKTRA is approved for the treatment of adult patients with relapsed or refractory follicular lymphoma after at least two prior systemic therapies. Accelerated approval was granted in this indication based on overall response rate (ORR). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

For full prescribing and safety information, please refer to the Package Insert and Important Safety Information available at www.COPIKTRA.com.
The COPIKTRA™ opportunity in relapsed or refractory CLL/SLL after two prior therapies

COPIKTRA expands oral monotherapy opportunities

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

- 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

- 70% initiate therapy with chemo or anti-CD20
- 30% start with targeted therapy

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

1. Chemotherapy or CD20
2. BTK inhibitor or BCL-2i or PI3Ki + CD20
3+ Continuation

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

**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.
Making COPIKTRA available

“How can we better Care Differently for you today?”

Targeting key HCPs and reimbursement coverage

Experienced senior commercial and medical affairs leadership, with a focused field force providing One Voice to the customer

- Senior leadership has an average of 24+ years industry experience and participation in over 30 drug launches
- 50 person Oncology sales team
- Dedicated Medical Affairs & Patient Advocacy teams
- Oncology nurse advocates providing access support and education
- Experienced supporting team in Marketing, Patient Services, Reimbursement, and Market Access

Specialty Pharmacy Providers
- 92% coverage of all US cases

Specialty Distributors
- 100% coverage

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For full prescribing and safety information, please refer to the Package Insert and Important Safety Information available at www.COPIKTRA.com.
VerastemCare: Go beyond the expected

**ACCESS**  *Cancer is hard enough – let’s keep it simple.*
For the majority of patients and their healthcare providers, stay with the system that already works for you

**ASSISTANCE**  *Patients prescribed COPIKTRA should have access to therapy.*
- **Bridge Program** provides product for eligible patients with delays in reimbursement coverage over 5 days
- **Prescription Assistance Program (PAP)** provides therapy for eligible patients in need

**SUPPORT**  *Patients should always have somewhere to turn.*
Oncology nurse advocates are here to listen and assist

**COMMUNITY**  *Patients should never feel alone.*
Let us make connections to patients and caregivers like you through external cancer support organizations

All logos and trademarks are the property of their respective owners.
For full prescribing and safety information, please refer to the Package Insert and Important Safety Information available at www.COPIKTRA.com.
**Future Potential of COPIKTRA**

**TODAY:**

**ANCHOR**

Approved in the US as monotherapy for R/R FL and CLL/SLL after 2 prior lines \(^1\)
- FL: 13,000 incidence, 141,000 prevalence \(^2\)
- CLL: 23,000 incidence, 197,000 prevalence \(^2\)

**BROADEN REACH**

Expand in FL and CLL/SLL
Expand into PTCL \(^\dagger\)

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**BOLD STEPS**

Combinations with I-O and SOC in aggressive NHL subtypes
- DBLCL, MCL, Richter’s, Transformed FL \(^\dagger\)

**MAXIMIZE POTENTIAL**

Combinations with novel agents and CAR-T
- NHL, Myeloma, Solid Tumors \(^\dagger\)

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

\(^\dagger\) COPIKTRA is not indicated for use in the treatment of these indications, and the safety and efficacy of COPIKTRA in these indications has not been established. Any such use is investigational only.

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

COPIKTRA is approved for the treatment of adult patients with relapsed or refractory follicular lymphoma after at least two prior systemic therapies. Accelerated approval was granted in this indication based on overall response rate (ORR). Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

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

- Hematologic toxicities: neutropenia, 59% (50% Gr3/4); thrombocytopenia, 65 (34% Gr3/4); anemia, 38% (16% Gr3/4)
- Immune-mediated toxicities: transaminitis, 34% (28% Gr3/4); inflammatory arthritis, 9% (all Gr2); colitis, 6% (1 Gr3); pericarditis and pancreatitis, 3% (all Gr2)
- Additional SAEs: Pneumonia, 19% (including 3 cases of PJP despite prophylaxis); Gr3 febrile neutropenia, 19%

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 is consistent with previously established safety profile of duvelisib monotherapy
- Both DR and DO combination therapies exhibited preliminary efficacy and modulation of tumor-supportive factors in the tumor microenvironment
- Data is supportive of the potential role of duvelisib + anti-CD20 as initial treatment for FL patients


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

Pharmacokinetics: No drug-drug interactions
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%
Duvelisib clinical development in R/R PTCL

**UNMET NEED**

Standard of care remains to be established in relapsed/refractory PTCL

- Recently approved 2nd+ line treatment options have low response rates with limited durability
- Median OS is < 6 months\(^1\)
- NCCN guidelines still recommend clinical trials for relapsed patients\(^4\)
- KOLs are unsatisfied with the available treatment options

**EARLY CLINICAL SIGNALS**

<table>
<thead>
<tr>
<th>Drug / Trial(^2,3)</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 = 12 (Horwitz, ASH 2017)</td>
<td>60%</td>
<td>27%</td>
<td>-</td>
</tr>
<tr>
<td><strong>APPROVED</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**

1. New trial initiation
2. IST Expansion (total n = 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.**


AA = accelerated approval; CR = complete response; ORR = overall response rate
## DUVELISIB (PI3K DELTA/PI3K GAMMA INHIBITOR)

<table>
<thead>
<tr>
<th>Patient Type</th>
<th>Phase</th>
<th>Collaborator</th>
</tr>
</thead>
<tbody>
<tr>
<td>Relapsed/Refractory CLL/SLL</td>
<td>PHASE 1 / 1B</td>
<td>Randomized open label vs. ofatumumab</td>
</tr>
<tr>
<td>Refractory iNHL</td>
<td>PHASE 2</td>
<td>Single arm, monotherapy</td>
</tr>
<tr>
<td>Relapsed/Refractory PTCL</td>
<td>PHASE 3</td>
<td>Single arm, monotherapy</td>
</tr>
<tr>
<td>1st line, younger CLL/SLL patients*</td>
<td>PHASE 2</td>
<td>Single arm, with FCR</td>
</tr>
<tr>
<td>Relapsed/Refractory T Cell Lymphoma*</td>
<td>PHASE 2</td>
<td>With Romidepsin or Bortezomib</td>
</tr>
<tr>
<td>Relapsed/Refractory CLL/SLL*</td>
<td>PHASE 3</td>
<td>With Venetoclax</td>
</tr>
</tbody>
</table>

## DEFACTINIB (FAK INHIBITOR)

<table>
<thead>
<tr>
<th>Patient Type</th>
<th>Phase</th>
<th>Collaborator</th>
</tr>
</thead>
<tbody>
<tr>
<td>NSCLC, Pancreatic, Mesothelioma*</td>
<td>PHASE 1 / 1B</td>
<td>With pembrolizumab</td>
</tr>
<tr>
<td>Pancreatic, relapsed*</td>
<td>PHASE 2</td>
<td>With pembrolizumab + gemcitabine</td>
</tr>
<tr>
<td>Advanced Solid Tumors*</td>
<td>PHASE 3</td>
<td>With RAF/MEK Inhibitor</td>
</tr>
<tr>
<td>Carboplatin Resistant Ovarian*</td>
<td>PHASE 3</td>
<td>With Platinum + Taxane</td>
</tr>
</tbody>
</table>

* Investigator Sponsored Trial (IST)

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.
Senior Management Team

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

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

**Steven Bloom**
Chief Strategy Officer  
SVP Commercial Strategy and Business Dev, Ziopharm PharMetrics (now IMS), Eli Lilly and Company

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

**Diep Le, M.D., Ph.D.**
Chief Medical Officer  
VP, I/O Innovative Medicines, MedImmune  
Exec Medical Director & Head, Global Clinical Program, Novartis

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

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

**Jonathan Pachter, Ph.D.**
Chief Scientific Officer  
Head of Cancer Biology - OSI (now Astellas)
# Key Financial Statistics

<table>
<thead>
<tr>
<th>Top Holders</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Consonance Capital</td>
<td></td>
</tr>
<tr>
<td>Fidelity Management &amp; Research Company</td>
<td></td>
</tr>
<tr>
<td>BlackRock Institutional Trust Company</td>
<td></td>
</tr>
<tr>
<td>The Vanguard Group, Inc.</td>
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<tr>
<td>BVF Partners L.P.</td>
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<tr>
<td>Eastern Capital, Ltd.</td>
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<tr>
<td>1Globe Capital, LLC</td>
<td></td>
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<tr>
<td>Bessemer Venture Partners</td>
<td></td>
</tr>
<tr>
<td>Renaissance Technologies LLC</td>
<td></td>
</tr>
<tr>
<td>State Street Global Advisors</td>
<td></td>
</tr>
</tbody>
</table>

| **Cash and cash equivalents as of 6/30/2018**                      | **$168.7M** |
| **Shares outstanding as of 6/30/2018**                             | **73.6M**   |
| **Shares fully diluted as of 6/30/2018**                            | **85.1M**   |
| **Hercules facility undrawn as of 6/30/2018**                        | **$25.0M**  |
| **YTD net loss as of 6/30/2018**                                     | **$39.4M** (including non-cash stock-based expense) |
| **YTD cash used in operating activities as of 6/30/2018**            | **$42.8M*** |
| **Full-time Employees as of 6/30/2018**                              | **101**    |
| **Insider ownership (outstanding/vested) as of 6/30/2018**            | **14.3%/7.8%** |

*Based on $32.8M YTD cash used in operating activities as of 6/30/2018, adjusted for the Yakult $10.0M upfront payment.*
## Upcoming Milestones

### 2H 2018
- ✔ Commercial organization launch ready Q3
- ✔ Defactinib dose escalation Immuno-Oncology combination data
- ✔ Duvelisib + venetoclax trial initiated
- ✔ Duvelisib FDA Target Action date October 5, 2018
  - **COPIKTRA™ (duvelisib) approved September 24, 2018**
- ✔ Additional Business Development partnership for duvelisib ex-US
  - **Exclusive License Agreement with CSPC for duvelisib in China**
- ○ Phase 3 DUO™ study manuscript published
- ○ Clinical and preclinical data reported at ASH

### 2019
- ○ Initiation of FL Confirmatory Study
- ○ Expansion of PRIMO study
- ○ Additional duvelisib publications
- ○ Initiation of additional sponsored trials for duvelisib
- ○ Interim data from duvelisib ISTs
- ○ Additional Business Development partnership for duvelisib ex-US
- ○ Final data from defactinib dose escalation Immuno-Oncology combinations
Focused Growth of Verastem Oncology

**Build** a team & organization dedicated to reaching patients

**Anchor** with launch of our first drug & first indications

**Reach** duvelisib’s full potential in additional tumors

**Repeat:** Unlock the full potential of defactinib

**Evolve** to continue meeting patient needs

---

**Care Differently**

At Verastem Oncology, we take a different approach. One that goes beyond the expected. When others see a problem, we see an opportunity. When others give up, we step up.

Because for us, and for our patients, it’s personal.