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.
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

**Products**

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

**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

FIRST APPROVED

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 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

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

Canada
Regional license
Planning to file
Evaluating Build or Partner

United States
Wholly owned & commercialization ready

Wholly owned & commercialization ready
Launch Update: 4 months ahead of schedule

1. Established Teams ahead of Launch: Sales, Marketing, Access, Med Affairs
2. NCCN Guidelines: CLL/SLL, Follicular, and Marginal Zone Lymphoma
3. Distribution Established with SD and SP: Product Shipped Day 1*
4. Payers: 75.5% covering Copiktra, 241M of 320M Patient Lives
5. Pricing: $11,800 per 25mg and 15mg Dose Pack (One Month)
6. Established HUB to support patient access to Copiktra
7. Majority of Territories have Copiktra treated patients

*Revenue recognized upon delivery to specialty distributor and specialty pharmacy
CLL/SLL Promotional HCP Resources at Launch

CLL/SLL Promo Leave Behind

CLL/SLL Visual Aid

CLL/SLL Journal Ad
PFS per IRC in Patients with at Least 2 Prior Therapies (N=196)

Source: Copiktra USPI, 2018

Kaplan-Meier estimate.
CI, confidence interval; HR, hazard ratio; ITT, intention to treat; SE, standard error

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

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

PFS analysis in high-risk patient subgroups (N = 196)*

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

Source: 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.
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) for COPIKTRA (n=95)
- **39%** (n=39/101) for Ofatumumab (n=101)

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

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

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
FL Promotional Launch

• Unlike CLL where full promotional materials were available upon FDA approval, all FL materials will be 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 will be ready and available for use.

NEW FL Promotional Campaign to Launch in Q1 2019!
### Efficacy in Patients with Relapsed or Refractory FL

<table>
<thead>
<tr>
<th>Outcome per IRC</th>
<th>FL</th>
</tr>
</thead>
<tbody>
<tr>
<td>N = 83</td>
<td></td>
</tr>
<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>

<table>
<thead>
<tr>
<th>Duration of response</th>
</tr>
</thead>
<tbody>
<tr>
<td>Range, months</td>
</tr>
<tr>
<td>Patients maintaining response at 6 months, n/N (%)</td>
</tr>
<tr>
<td>Patients maintaining response at 12 months, n/N (%)</td>
</tr>
</tbody>
</table>

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

* 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.

Source: Copiktra USPI, 2018

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](http://www.COPIKTRA.com).
88% of patients in the DYNAMO™ study had reduction in target lymph nodes by IRC.

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

Source: 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.

Source: Copiktra USPI, 2018
Pooled analysis of safety supporting approval
442 patients with previously treated hematologic malignancies

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 †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>

 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.

Source: Copiktra USPI, 2018

Notes:
- † Grouped term for reactions with multiple preferred terms
- †a Diarrhea or colitis includes the preferred terms: colitis, enterocolitis, colitis microscopic, colitis ulcerative, diarrhea, diarrhea hemorrhagic
- †b Pneumonia includes the preferred terms: All preferred terms containing "pneumonia" except for "pneumonia aspiration"; bronchopneumonia, bronchopulmonary aspergillosis
- †c 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
Opportunity: Additional therapy options are needed for chronic iNHL patients

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

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

1ST LINE TREATABLE PATIENTS/YEAR (AVG.)\(^1\)
22,205

13,500

**Increasing Elderly At-Risk Patient Population**

\(65-75\)
AGE AT DIAGNOSIS\(^2\)

**Aging Baby Boomer Population**

INCREASED DIAGNOSES

**MEDIAN OS**
10+ YEARS\(^3\)

**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.*

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

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

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

Sources: 1. ATU 2018, Verastem Oncology; 2. Copikra 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.
The COPIKTRA™ opportunity in relapsed or refractory FL after two prior systemic therapies

COPIKTRA provides a targeted therapy option after chemo-immunotherapy

>80% of 2nd 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... 

INITIATE THERAPY

1

Chemo ± CD20

RE-CHALLENGE

2

Chemo ± CD20 or CD20

LONG-TERM DISEASE CONTROL

3+

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.
# 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>

\(^1\) 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

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.
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

**ORR (n = 31)**

- 52% CR*
- 42% PR
- 94% Best response on study*

**CR† with MRD negativity (n = 29)**

- 55%

**MRD negativity (n = 29)**

- 76%

*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.

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

† Includes CR and CRi

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

### 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></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>

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.

**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

- **DOHH-2 (cell line)**

<table>
<thead>
<tr>
<th>Combining Doses</th>
<th>Safety lead-in</th>
</tr>
</thead>
<tbody>
<tr>
<td>Arm 1: duvelisib 25 mg BID + venetoclax 100 mg</td>
<td>Duvelisib 25 mg BID + venetoclax 100 mg</td>
</tr>
<tr>
<td>Arm 2: duvelisib 25 mg BID + venetoclax 200 mg</td>
<td>Duvelisib 25 mg BID + venetoclax 200 mg</td>
</tr>
<tr>
<td>Arm 3: duvelisib 25 mg BID + venetoclax 400 mg</td>
<td>Duvelisib 25 mg BID + venetoclax 400 mg</td>
</tr>
</tbody>
</table>

- **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

*COPIKTRA 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

- Duvelisib @ 50 mg/kg po, BID
- Anti-OX40 @ 100 µg/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.

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

† 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 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.

Composition of Matter: 2030

**STEP 1**

**STEP 2**

**STEP 3**

**STEP 4**
### Pipeline Overview

#### DUVELISIB (PI3K DELTA/PI3K GAMMA INHIBITOR)

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

#### DEFACTINIB (FAK INHIBITOR)

<table>
<thead>
<tr>
<th>Phase</th>
<th>Study Type</th>
<th>Patient Population</th>
</tr>
</thead>
<tbody>
<tr>
<td>PHASE 1/1B</td>
<td>DUO™</td>
<td>NSCLC, Pancreatic, Mesothelioma* (With pembrolizumab)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>Pancreatic, relapsed* (With pembrolizumab + gemcitabine)</td>
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<tr>
<td></td>
<td></td>
<td>Advanced Solid Tumors* (With RAF/MEK Inhibitor)</td>
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<td></td>
<td></td>
<td>Carboplatin Resistant Ovarian* (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.
### Key financial statistics

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Pro forma cash and cash equivalents as of 9/30/2018</td>
<td>$278.7M*</td>
</tr>
<tr>
<td>Shares outstanding as of 9/30/2018</td>
<td>73.7M</td>
</tr>
<tr>
<td>Shares fully diluted as of 9/30/2018</td>
<td>86.9M</td>
</tr>
<tr>
<td>Hercules Term Loan Facility</td>
<td>$25.0M</td>
</tr>
<tr>
<td>5.00% Convertible Senior Notes Due 2048</td>
<td>$150.0M**</td>
</tr>
<tr>
<td>YTD net loss as of 9/30/2018</td>
<td>$61.1M (including non-cash stock-based expense)</td>
</tr>
<tr>
<td>YTD cash used in operating activities as of 9/30/2018</td>
<td>$70.3M***</td>
</tr>
<tr>
<td>Full-time employees as of 9/30/2018</td>
<td>169</td>
</tr>
<tr>
<td>Insider ownership (outstanding/vested) as of 9/30/2018</td>
<td>15.9%/8.0%</td>
</tr>
</tbody>
</table>

*Pro forma cash and cash equivalents represents cash and cash equivalents at 9/30/18 of $145.6M, plus (1) estimated net proceeds of $145.1M from the issuance of our 5.00% Convertible Senior Notes in October 2018, and (2) the remaining $10.0 million of the $15.0M non-refundable upfront payment due from CSPC pursuant to the exclusive license agreement executed in September 2018, less the $22.0M payment we owe to Infinity Pharmaceuticals, Inc. pursuant to the terms of the amended and restated license agreement.

**The 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.

***Based on $55.3M YTD cash used in operating activities as of 9/30/2018, adjusted for the Yakult $10.0M upfront payment and $5.0M of the $15.0M CSPC upfront payment received prior to 9/30/2018.
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 Strategy, Ironwood, ActiveBiotics, Dynogen, Tufts Health Plan

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)

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

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