



# Jefferies Virtual Healthcare Conference

June 2, 2020

# Forward-Looking Statements

- This presentation contains, and our officers and representatives may from time to time make, statements that are “forward-looking statements” within the meaning of the safe harbor provisions of the U.S. Private Securities Litigation Reform Act of 1995. Examples of forward-looking statements include, among others, statements regarding our development strategy; potential advantages of our product candidates; the initiation and completion of preclinical and clinical studies and the reporting of the results thereof; the timing of regulatory submissions and actions; the sufficiency of our existing cash; and all other statements relating to our plans, objectives, expectations and beliefs regarding future performance, operations, financial condition and other future events (including assumptions underlying or relating to any of the foregoing).
- These forward-looking statements rely on a number of assumptions concerning future events and are subject to a number of risks, uncertainties, and other factors, many of which are outside of our control. Important factors that could cause our actual results and financial condition to differ materially from those indicated in forward-looking statements include, among others: uncertainties relating to the initiation and completion of preclinical and clinical studies; whether preclinical and clinical study results will validate and support the safety and efficacy of our product candidates; the outcome of regulatory reviews of our product candidates; varying interpretation of research and development and market data; the impact of the COVID-19 pandemic on our industry and individual companies, including on our counterparties, the supply chain, the execution of our clinical development programs, our access to financing and the allocation of government resources; risks and uncertainties relating to intellectual property and the other factors discussed under the caption “Item 1A. Risk Factors” in our most recent annual report on Form 10-K and our most recent quarterly report on Form 10-Q.
- Any forward-looking statement made by us in this presentation is based only on information currently available to us and speaks only as of the date on which it is made. In addition, we operate in a highly competitive and rapidly changing environment, and new risks may arise. Accordingly, you should not place any reliance on forward-looking statements as a prediction of actual results. We disclaim any intention to, and undertake no obligation to, update or revise any forward-looking statement. You are urged to carefully review and consider the various disclosures in our most recent annual report on Form 10-K, our most recent Form 10-Q and our other public filings with the SEC since the filing of our most recent annual report.

# Building a Leading Oncology Franchise



- 4 Clinical-Stage Oncology Programs: Focus On HemOnc



- 2 Candidates in Studies Intended to Support FDA Marketing Approval Applications



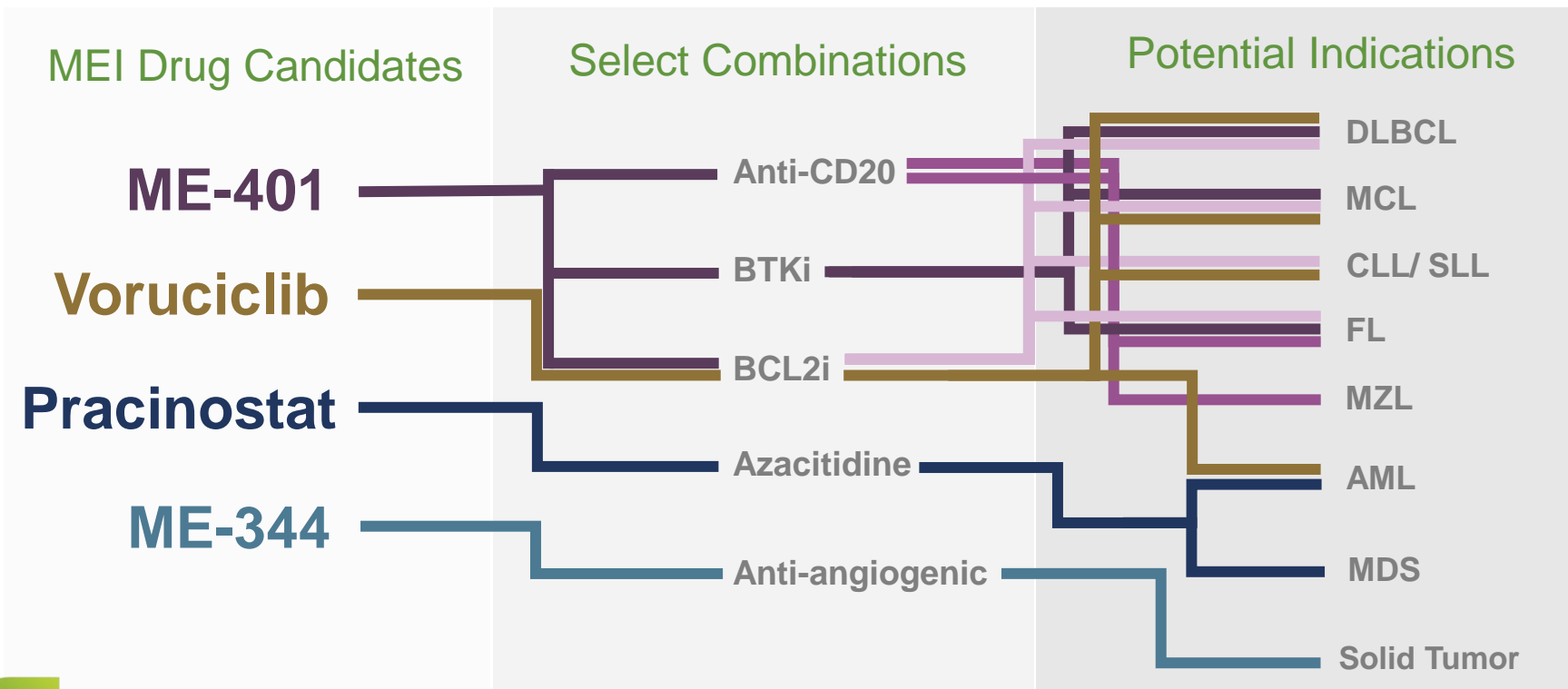
- ME-401: TIDAL Study Supporting Accelerated Approval Submission in r/r Follicular Lymphoma



- **\$193 Million** (Giving Effect to Kyowa Kirin \$100M Upfront)
  - \$93 Million as of March 31, 2020\*
  - Kyowa Kirin Agreement Announced April 2020

# Pipeline of Compatibility

Shift Towards Versatile Drugs in Potent Combinations





## **ME-401 ± Rituximab in Relapsed/Refractory (R/R) B-cell Malignancies**

# Global License, Development and Commercialization Agreement Optimizes ME-401 Value (April 2020)

**\$100M**

upfront to MEI

**\$582M**

in potential development, regulatory and commercial milestones

**50/50**

U.S. co-promote, MEI books U.S. sales



**KYOWA KIRIN**

KKC exclusive ex-U.S. rights, MEI to receive escalating tiered royalties starting in teens

■ **U.S. COST SHARING**, KKC responsible for incremental ex-U.S. costs

■ **AGREED ON BROAD DEVELOPMENT PLAN** for B-cell malignancies



# The Time for PI3K $\delta$ Has Come: Targeting a Profile to Meet B-Cell Malignancy Medical Need

Oral  
Administration

Low G3+  
TEAEs

High  
Responses

Low  
Discontinuation  
Rate

Durable  
Responses

**ME-401**

# **Tolerability and Durable Responses of the PI3K $\delta$ Inhibitor ME-401 Administered on an Intermittent Schedule in Relapsed/Refractory (R/R) Follicular Lymphoma (FL) and Other B-cell Malignancies**

**( Abstract # 8016)**

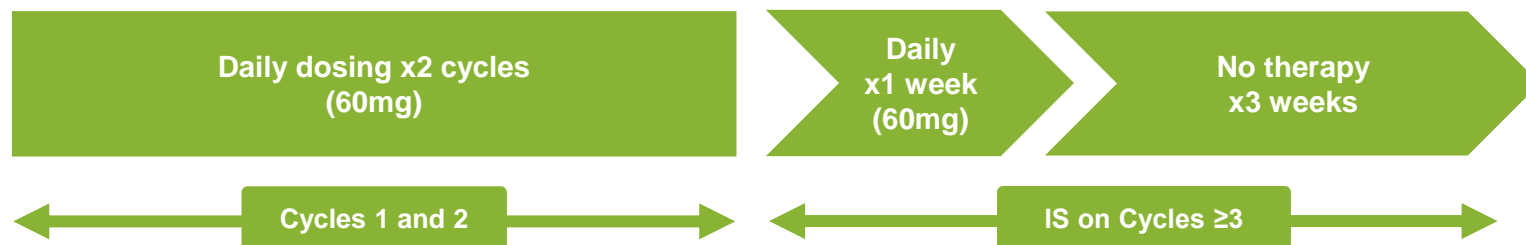
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# ME-401 Phase 1b Study (NCT02914938)

- Phase 1b single arm, dose escalation/expansion study with safety and tolerability endpoints
  - Main Eligibility Criteria: ECOG 0–2, failure of  $\geq 1$  prior therapy for B-cell malignancies, measurable disease, no prior PI3K or BTK therapy
- 57 patients treated on Intermittent Schedule (IS) beginning in Cycle 3
- Treatment groups: 60 mg daily as monotherapy / 60 mg in combination with rituximab



**Intermittent schedule (IS) being evaluated as a strategy to mitigate delayed immune-related toxicities associated with continuous daily administration of oral PI3K inhibitors**

# Patient Characteristics

	<b>FL N=36</b>	<b>CLL N=10</b>	<b>MZL N=4</b>	<b>DLBCL N=7</b>	<b>Total N=57</b>
Age					
Median (range), in years	62 (38 -87)	71 (46 -80)	73(70 -94)	69(60 -84)	66 (38 – 94)
≥ 65 years, No. (%)	16 (44%)	8 (80%)	4 (100%)	6 (86%)	34 (60%)
Prior anti-lymphoma therapy					
Median (range)	2 ( 1, 5)	2( 1, 3)	1( 1, 1)	2( 1, 8)	2 (1 – 8)
≥ 2 prior lines	20 (56%)	5 (50%)	0	6 (86%)	31 (54%)
Prior anti-CD20 antibody	36 (100%)	9 (90%)	4 (100%)	7 (100%)	56 (98%)
Diameter of largest tumor size					
< 5 cm	17 (47%)	6 (60%)	3 (75%)	5 (71%)	31 (54%)
≥ 5 cm	19 (53%)	4 (40%)	1 (25%)	2 (29%)	26 (46%)

# Overall Responses: R/R FL

Diagnosis	Evaluable Subjects (N)	ORR N (%)	CR Rate N (%)
FL	36	30 (83%)	8 (22%)
By treatment group			
ME-401 monotherapy	17	13 (76%)	4 (24%)
ME-401 + rituximab	19	17 (89%)	4 (21%)
By prior lines of therapy			
1 prior	16	13 (81%)	3 (19%)
≥ 2 prior	20	17 (85%)	5 (25%)

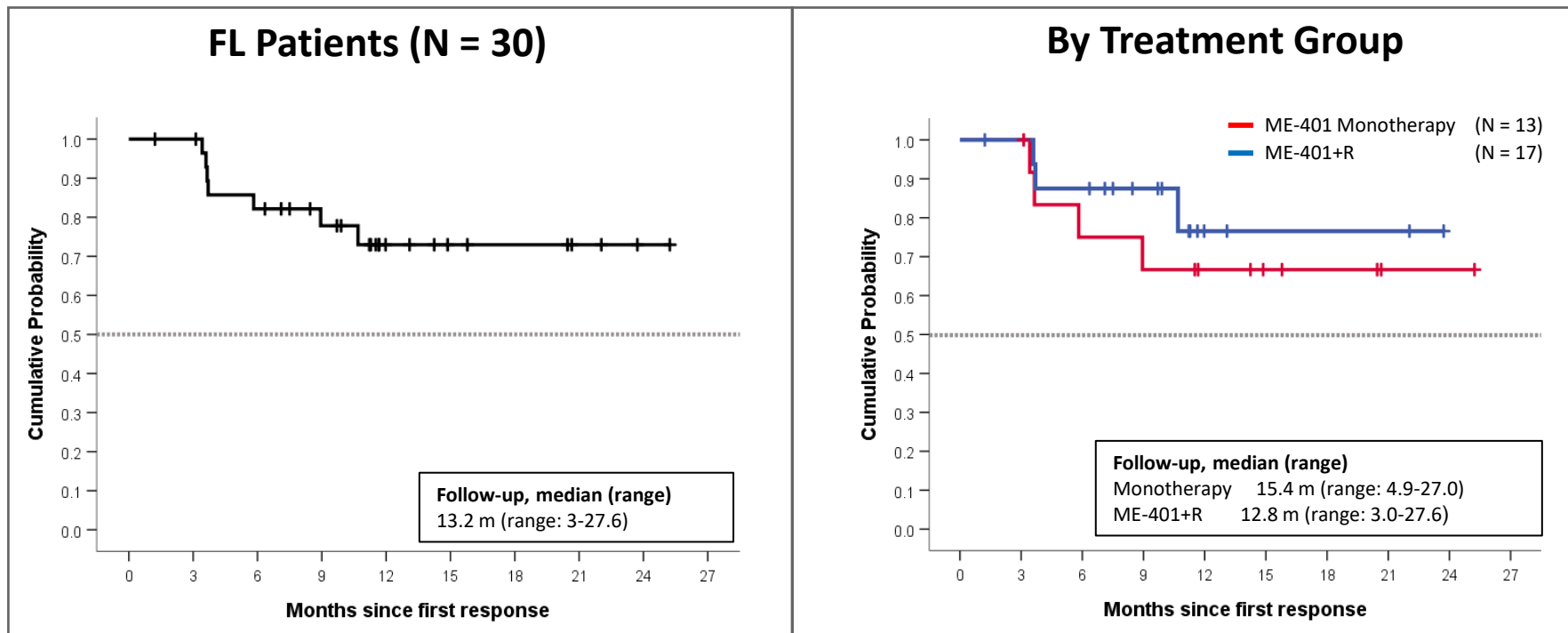
Response criteria : Lugano

# Overall Responses: Other R/R B-Cell Malignancies

Diagnosis	Evaluable Subjects (N)	ORR N (%)	CR Rate N (%)
CLL/SLL	9	8 (89%)	1 (11%)
By treatment group			
ME-401 monotherapy	3	3 (100%)	0
ME-401 + rituximab	6	5 (83%)	1 (17%)
MZL			
ME-401 + rituximab	4	4 (100%)	1 (25%)
DLBCL			
ME-401 + rituximab	6	1 (17%)	1 (17%)

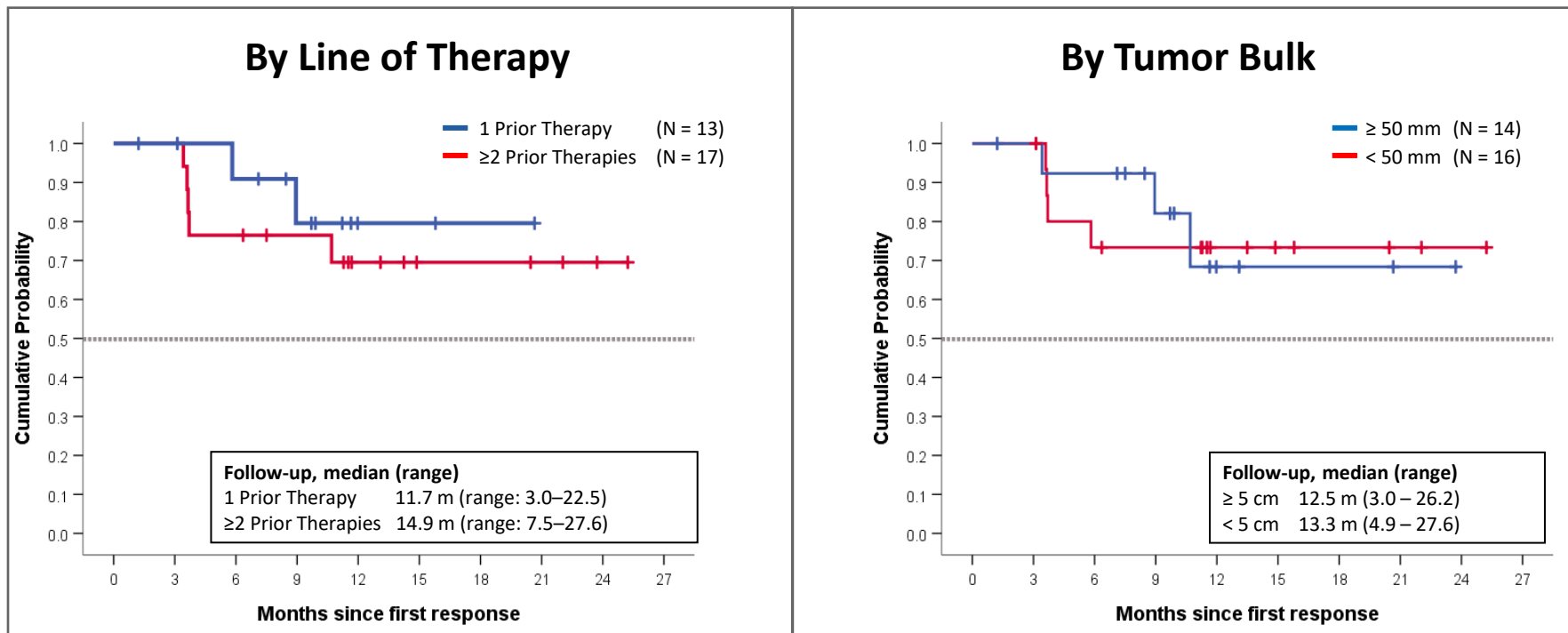
Response criteria : iwCLL

# Duration of Response in FL



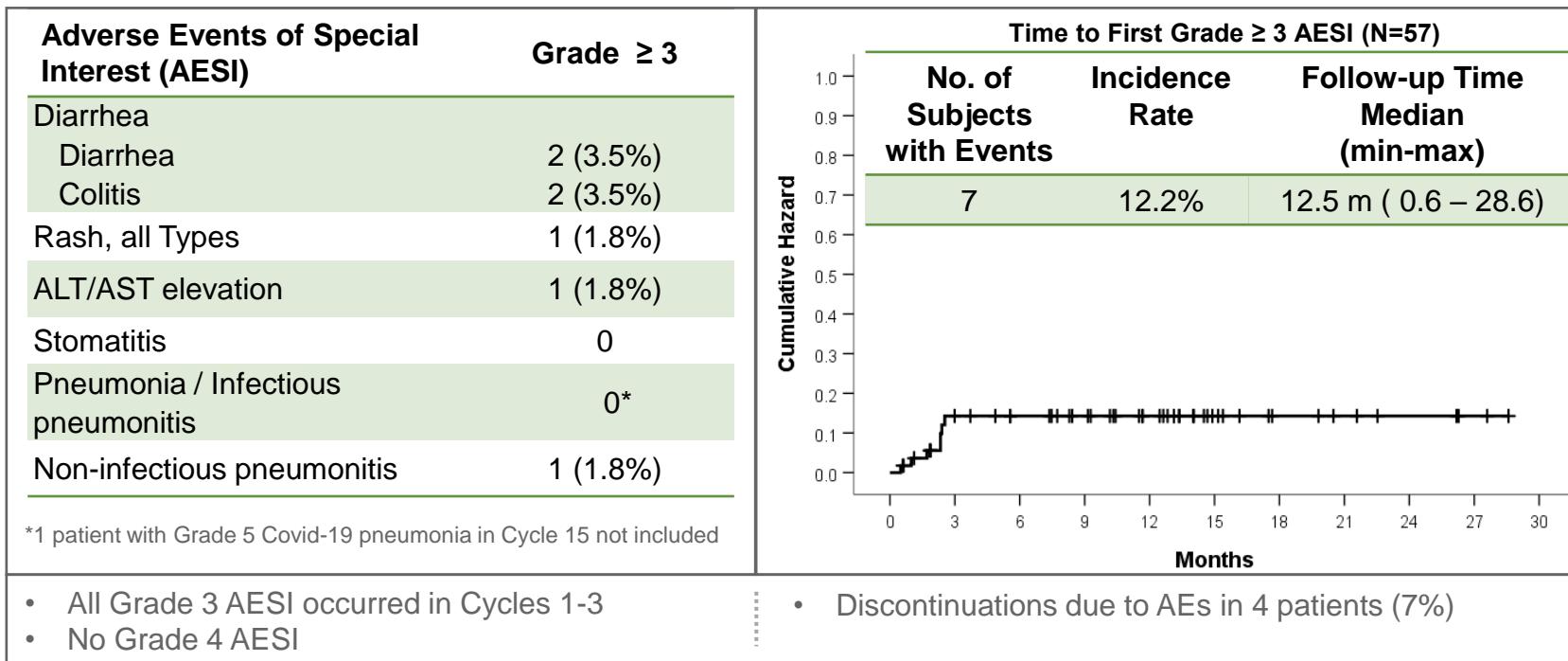
Follow-up = Time from first day on study to treatment discontinuation or data cutoff date

# Duration of Response in FL



Follow-up = Time from first day on study to treatment discontinuation or data cutoff date

# Low Incidence of Adverse Events of Special Interest (AESI): No Increased Toxicity Over Time



# Low Incidence of Grade $\geq 3$ Myelosuppression and AST/ALT Elevation Based on Laboratory Tests

<b>Grade <math>\geq 3</math></b>	<b>ME-401 Alone (N=21)</b>	<b>ME-401 + Rituximab (N=36)</b>	<b>Total (N = 57)</b>
Increased Transaminases			
AST	0 (0.0%)	0 (0.0%)	0 (0.0%)
ALT	0 (0.0%)	2 (5.6%)	2 (3.5%)
Hematology			
Neutropenia	3 (14.3%)	8 (22.2%)	11 (19.3%)
Anemia	0 (0.0%)	0 (0.0%)	0 (0.0%)
Thrombocytopenia	0 (0.0%)	2 (5.6%)	2 (3.5%)



# Conclusions: ASCO 2020 Data Update

- **ME-401 on IS has a high response rate and is well tolerated in R/R FL and CLL/SLL**
  - ORR = 83% in FL and 89% in CLL/SLL
  - Low incidence of Grade  $\geq 3$  AESI
  - No Grade  $\geq 3$  AESI reported after Cycle 3
  - Discontinuation rate due to adverse events = 7%
- **Median DOR in FL is not reached with a median follow-up 13.2 months**
  - Durable responses regardless of prior lines of therapy, treatment group ( $\pm$  rituximab) or tumor bulk

# ME-401 Emerging Profile: Potential for Broad Acceptance Across B-Cell Malignancies & Supports Combinations with Other Modalities

Discontinuation Rate: TEAEs (Any Grade)

**ME-401**

**7.0%**

**BTKi<sup>1</sup>**

**8.3%** (avg.)  
(Range: 6.5% - 10.0%)

**BCL2i<sup>2</sup>**

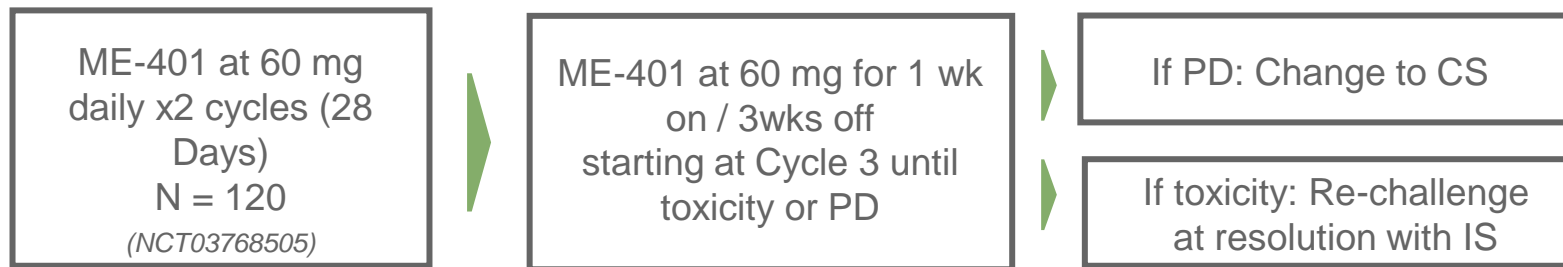
**12.5%** (avg.)  
(Range: 9% - 16%)

**R2<sup>2</sup>**

**14.6%**

	Diarrhea/ Colitis	Neutropenia	Anemia	Thrombocytopenia	Pneumonia/ Pneumonitis	Hemorrhage	Atrial Fibrillation/Flutter	Secondary Malignancy
<b>ME-401</b>	<b>7%</b>	<b>0%</b>	<b>0%</b>	<b>0%</b>	<b>2%</b>	<b>0%</b>	<b>0%</b>	<b>0%</b>
BTKi	0.6%-5%	23%-27%	8%-10%	7%-8%	6%-13%	2%-4%	0.6%-4%	9%-12%
BCL2i	3%-4%	45%-62%	8%-18%	20-28%	8%	0%	0%	0%
R2	2.8%	50%	4.5%	2.3%	2.2%	0%	0%	0%

# Phase 2 TIDAL Trial to Support Accelerated Approval Marketing Application – The Initial Opportunity in r/r FL



- Histologically confirmed diagnosis of FL, Grade 1, 2, or 3a
- FL, relapsed or refractory to 2 prior systemic therapies including an anti-CD20 antibody and chemotherapy
- No prior therapy with PI3K $\delta$  inhibitors
- No histological transformation to an aggressive lymphoma

# Focus on Select B-Cell Malignancy Indications\*

**>65,000** Addressable  
Patients Across Multiple  
Indications

(U.S. Only)

**\$1B+Markets**

**Follicular Lymphoma** 15,000

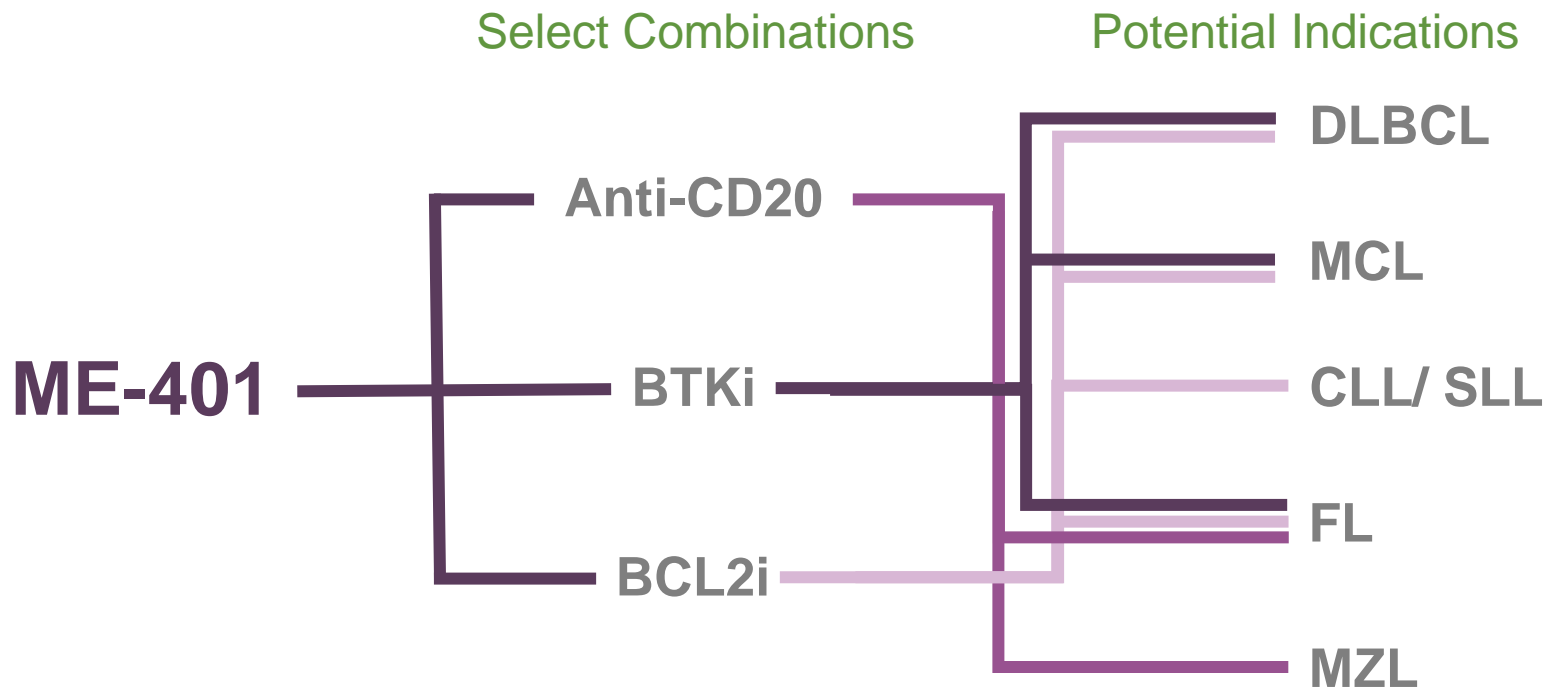
**Mantle Cell Lymphoma** 4,500

**DLBCL** 22,000

**Marginal Zone Lymphoma** 6,000

**Chronic Lymphocytic Leukemia  
& Small Lymphocytic Lymphoma** 20,000

# Select Combinatorial Potential of ME-401



## **ME-401: Regaining the Promise of PI3K $\delta$**

**Best-in-class  
Potential**

**Emerging Profile  
to Meet Need  
Across B-cell  
Disease and  
Supports Broad  
Acceptance**

**Many  
Combinatorial  
Options  
Across B-Cell  
Malignancies**



## **Voruciclib: Oral CDK Inhibitor with Potent CDK9 Activity**

# Voruciclib: Potential to Overcome Venetoclax Resistance

Increased MCL1 is an established venetoclax resistance mechanism<sup>1</sup>

Venetoclax inhibits BCL2  
but can lead to  
stabilization of MCL1



Voruciclib inhibits MCL1  
via CDK9 inhibition

- Combination opportunities across multiple indications, including:
  - AML, CLL & DLBCL

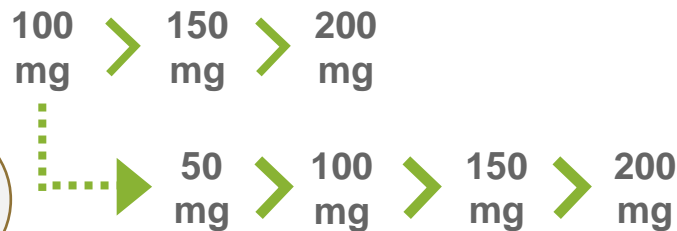


# MEI Phase 1 Study: Daily Dosing Design in R/R B-Cell Malignancies and AML

- Study population
  - Relapsed/Refractory B-cell Malignancies
  - AML After Treatment with Standard Therapy
- Dose escalation with standard 3+3 design
- Endpoints
  - Safety and tolerability
  - Pharmacokinetics
  - Biologic correlative studies
    - BH3 profiling, MCL-1 expression (Dana Farber)
    - Molecular mutations analysis (City of Hope)
  - Objective response rates
  - CR/CRi rate

Voruciclib single agent  
dose escalation

Venetoclax + Voruciclib dose  
escalation





## **Pracinostat: Potential Best-in-Class Phase 3 HDAC Inhibitor**

# Pracinostat in Two Ongoing Studies: AML and MDS

## Phase 3

### AML

- 75+ w/ newly diagnosed AML
- Unfit for intensive chemo

**500 patients:**  
Pracinostat (60 mg) + Aza  
vs. Aza monotherapy

**Primary endpoint:**  
Overall Survival

## Phase 2

### MDS

- High/Very high risk
- No prior HMA's

**≤ 40 patients:**  
Pracinostat (45 mg) + Aza

**Enrollment expansion:**  
60 pts  
Pracinostat + Aza

**Primary endpoints:**  
Overall Response  
&  
One Year Survival

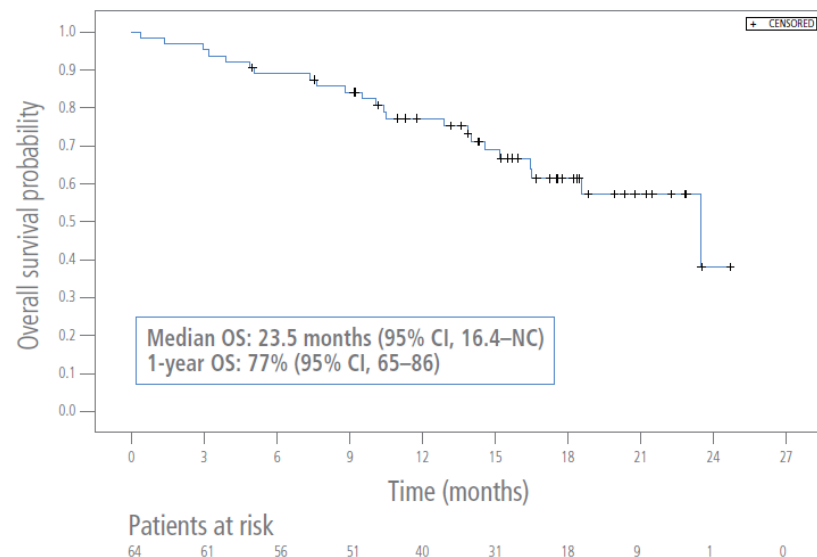


# ASCO 2020: Phase 2 Study in Patients with High/Very-High Risk MDS

ASCO20 Virtual  
SCIENTIFIC PROGRAM

- 77%. 1-year OS (median follow-up 17.6 months (range, 15.7–18.8))
- 33% ORR (21/64), all CRs
- 77% (49/64) Clinical Benefit rate\*
- 27% (17/64) proceeded to a stem cell transplant
- 11% (7/64) discontinued treatment because AE
- Most common grade  $\geq 3$  TEAEs were hematologic:
  - Decreased neutrophil count (50%)
  - Anemia (39%)
  - Febrile neutropenia (34%)
  - Decreased platelet count (33%)
  - Thrombocytopenia (27%)
  - Decreased white blood cell count (20%)

Figure 3. Overall Survival



NC: not computable; OS: overall survival.

# Key Upcoming 12 Month Milestones Across Portfolio



- **ME-401**

- TIDAL, accelerated approval study in R/R follicular lymphoma, complete enrollment
- Initiation of new clinical studies to expand into additional indications
- Initial data, phase 1b study evaluating ME-401 with Brukinsa™ (zanubrutinib) under clinical collaboration with BeiGene

- **Voruciclib**

- Initial data, Phase 1 monotherapy and +BCL2i data updates

- **Pracinostat**

- Phase 3 study in AML, complete enrollment

- **ME-344**

- Institute plan to leverage clinically demonstrated anti-tumor activity in combination with anti-VEGF



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