Recent Advances in Hematologic Oncology: A 4-Part Live Webinar Series Reviewing Key Data and Presentations from the 62nd ASH Annual Meeting

Part 1 — Acute Myeloid Leukemia

Wednesday, January 20, 2021
5:00 PM – 6:00 PM ET

Faculty

Daniel A Pollyea, MD, MS
Eytan M Stein, MD
Andrew H Wei, MBBS, PhD

Moderator

Neil Love, MD

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Faculty

Daniel A Pollyea, MD, MS
Associate Professor of Medicine
Clinical Director of Leukemia Services
Robert H Allen, MD Chair in Hematology Research
Division of Hematology
University of Colorado School of Medicine
Aurora, Colorado

Andrew H Wei, MBBS, PhD
Adjunct Professor, Department of Haematology
Alfred Hospital and Monash University
Melbourne, Australia

Eytan M Stein, MD
Assistant Attending Physician
Director, Center for Drug Development in Leukemia
Leukemia Service, Department of Medicine
Memorial Sloan Kettering Cancer Center
New York, New York

Moderator
Neil Love, MD
Research To Practice
Miami, Florida

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Commercial Support

This activity is supported by educational grants from AbbVie Inc, Astellas, Bristol-Myers Squibb Company, Daiichi Sankyo Inc, Genentech, a member of the Roche Group, Helsinn Healthcare SA and Pfizer Inc.

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Dr Love — Disclosures

Dr Love is president and CEO of Research To Practice. Research To Practice receives funds in the form of educational grants to develop CME activities from the following commercial interests: AbbVie Inc, Acerta Pharma — A member of the AstraZeneca Group, Adaptive Biotechnologies Corporation, Agendia Inc, Agios Pharmaceuticals Inc, Amgen Inc, Array BioPharma Inc, a subsidiary of Pfizer Inc, Astellas, AstraZeneca Pharmaceuticals LP, Bayer HealthCare Pharmaceuticals, Biodesix Inc, bioTheranostics Inc, Blueprint Medicines, Boehringer Ingelheim Pharmaceuticals Inc, Bristol-Myers Squibb Company, Celgene Corporation, Clovis Oncology, Daiichi Sankyo Inc, Dendreon Pharmaceuticals Inc, Eisai Inc, EMD Serono Inc, Epizyme Inc, Exact Sciences Inc, Exelixis Inc, Foundation Medicine, Genentech, a member of the Roche Group, Genmab, Gilead Sciences Inc, GlaxoSmithKline, Grail Inc, Guardant Health, Halozyme Inc, Helsinn Healthcare SA, ImmunoGen Inc, Incyte Corporation, Infinity Pharmaceuticals Inc, Ipsen Biopharmaceuticals Inc, Janssen Biotech Inc, administered by Janssen Scientific Affairs LLC, Jazz Pharmaceuticals Inc, Karyopharm Therapeutics, Kite, A Gilead Company, Lexicon Pharmaceuticals Inc, Lilly, Loxo Oncology Inc, a wholly owned subsidiary of Eli Lilly & Company, Merck, Merrimack Pharmaceuticals Inc, Myriad Genetic Laboratories Inc, Natera Inc, Novartis, Novocure Inc, Oncopeptides, Pfizer Inc, Pharmacyclics LLC, an AbbVie Company, Prometheus Laboratories Inc, Puma Biotechnology Inc, Regeneron Pharmaceuticals Inc, Sandoz Inc, a Novartis Division, Sanofi Genzyme, Seagen Inc, Sirtex Medical Ltd, Spectrum Pharmaceuticals Inc, Sumitomo Dainippon Pharma Oncology Inc, Taiho Oncology Inc, Takeda Oncology, Tesaro, A GSK Company, Teva Oncology, Tokai Pharmaceuticals Inc and Verastem Inc.

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Dr Pollyea — Disclosures

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Contracted Research	AbbVie Inc
Data and Safety Board/Committee	GlycoMimetics Inc, Takeda Oncology

Dr Stein — Disclosures

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Dr Wei — Disclosures

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Consulting Agreement	Servier
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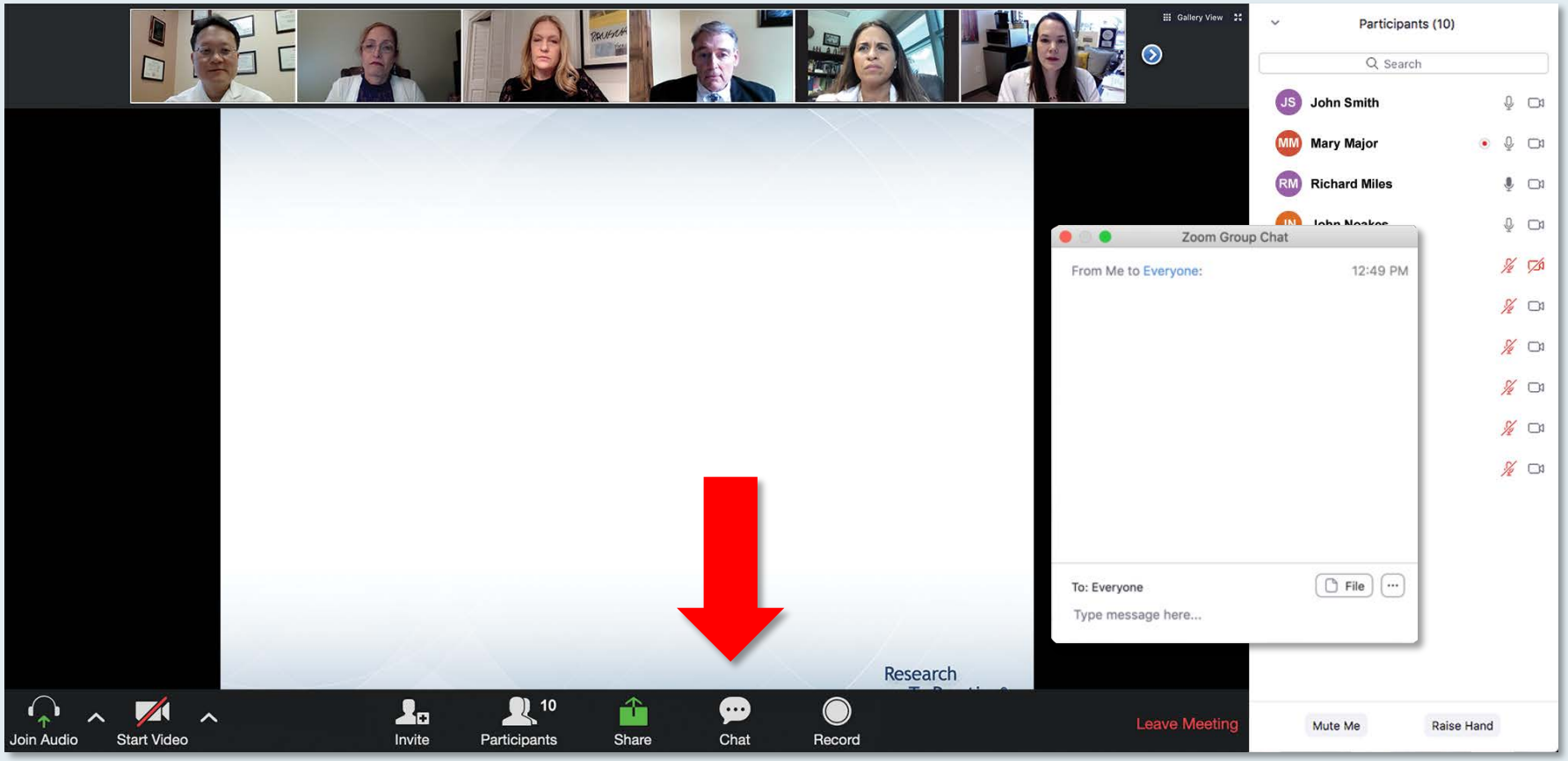
Royalties	Former employee of the Walter and Eliza Hall Institute of Medical Research receiving a fraction of its royalty stream related to venetoclax
Speakers Bureau	AbbVie Inc, Genentech, a member of the Roche Group

We Encourage Clinicians in Practice to Submit Questions



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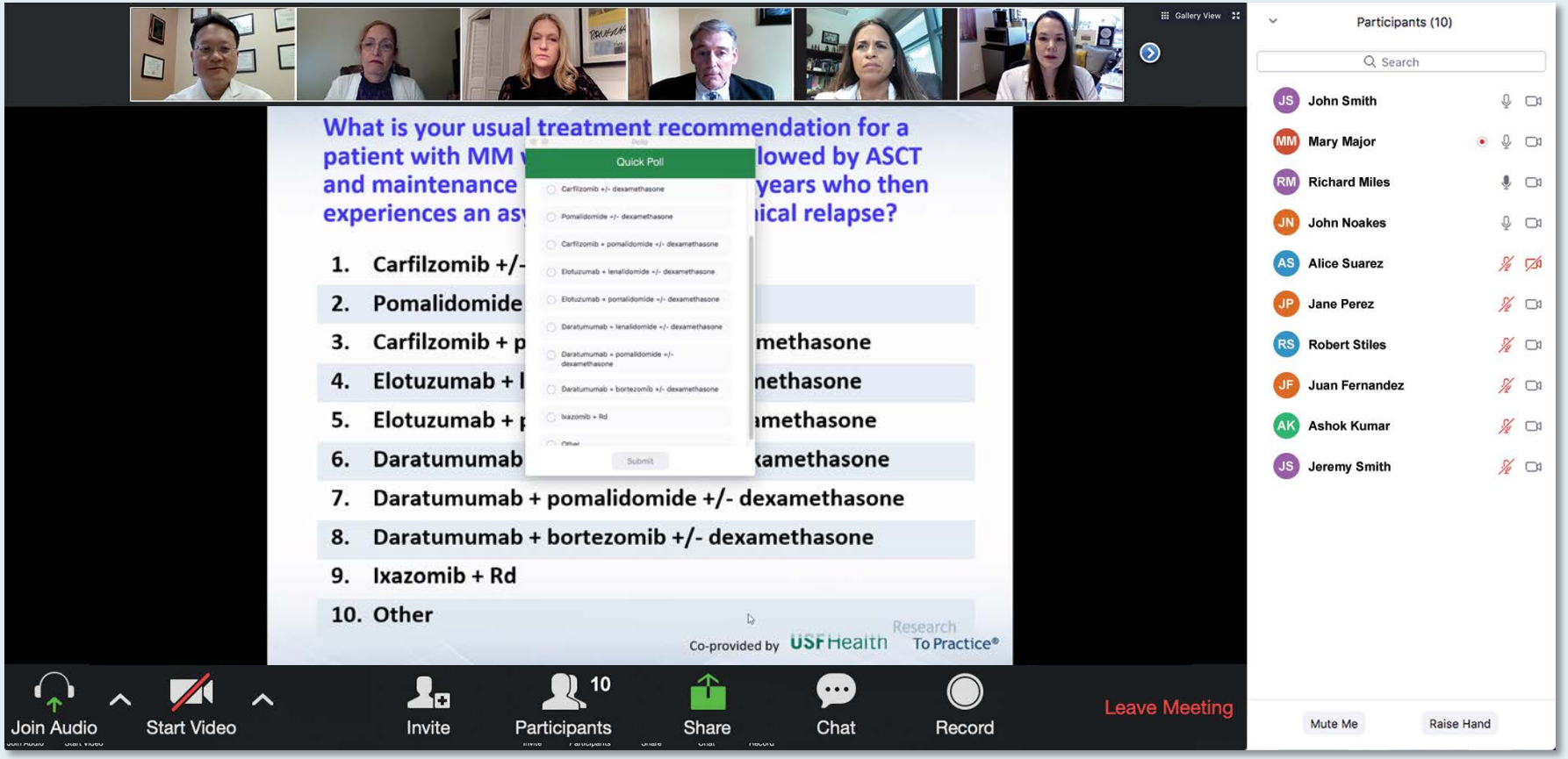

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ACUTE MYELOID LEUKEMIA WITH FLT3 MUTATIONS

DR KEITH PRATZ
UNIVERSITY OF PENNSYLVANIA

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Year in Review — Clinical Investigators Provide Perspectives on the Most Relevant New Publications, Data Sets and Advances in Oncology:

Chronic Lymphocytic Leukemia

Thursday, January 21, 2021
5:00 PM – 6:00 PM ET

Faculty

Matthew S Davids, MD, MMSc
Jennifer Woyach, MD

Moderator

Neil Love, MD

RTP Year in Review 2020

Meet The Professor
Management of Ovarian Cancer

Friday, January 22, 2021
1:15 PM – 2:15 PM ET

Faculty

Professor Jonathan A Ledermann, MD

Moderator

Neil Love, MD

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Cancer Conference Update: What Happened at the 2020 San Antonio Breast Cancer Symposium®

Management of HER2-Positive Breast Cancer

Monday, January 25, 2021
5:00 PM – 6:00 PM ET

Faculty

Erika Hamilton, MD

Moderator

Neil Love, MD

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Year in Review — Clinical Investigators Provide Perspectives on the Most Relevant New Publications, Data Sets and Advances in Oncology:

Targeted Therapy for Lung Cancer

Tuesday, January 26, 2021
5:00 PM – 6:00 PM ET

Faculty

Joel W Neal, MD, PhD
Paul K Paik, MD

Moderator

Neil Love, MD

RTP Year in Review 2020

Cases from the Community: Investigators Discuss Emerging Research and Actual Patients with Hepatocellular Carcinoma (Part 1 of a 3-Part Series)

Wednesday, January 27, 2021
5:00 PM – 6:30 PM ET

Faculty

Richard S Finn, MD
Tim Greten, MD
James J Harding, MD
Ahmed Omar Kaseb, MD, CMQ

Moderator

Neil Love, MD

RTP RESEARCH TO PRACTICE

Year in Review — Clinical Investigators Provide Perspectives on the Most Relevant New Publications, Data Sets and Advances in Oncology:

Multiple Myeloma

Thursday, January 28, 2021
5:00 PM – 6:00 PM ET

Faculty

Rafael Fonseca, MD
Jonathan L Kaufman, MD

Moderator

Neil Love, MD

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Thank you for joining us!

CME credit information will be emailed to each participant within 3 business days.

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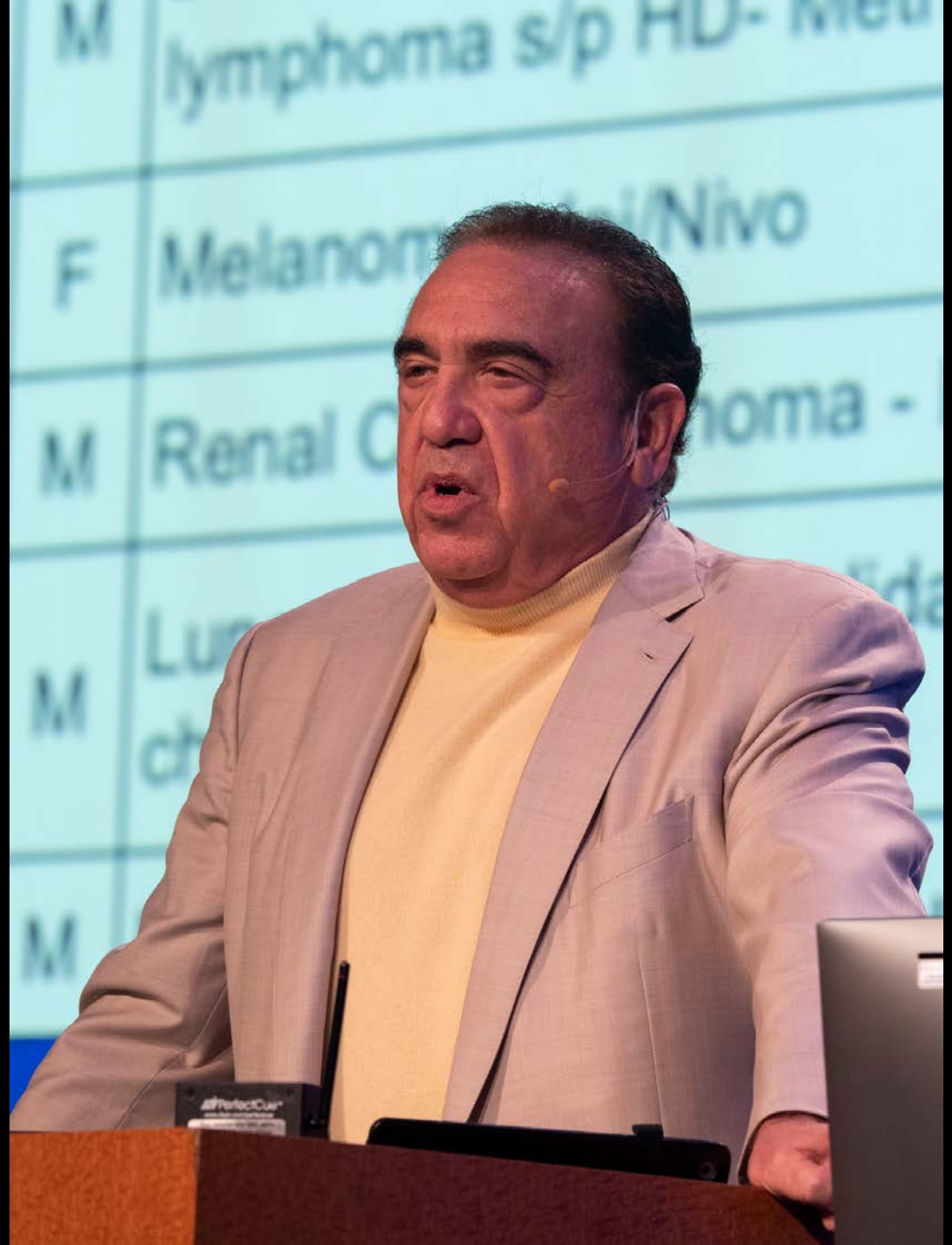
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Exploring Current Management Paradigms
for Patients with Acute Myeloid Leukemia
Not Eligible for Intensive Therapy
Friday, October 25, 2019, 1:15 PM – 3:45 PM
Orlando, Florida
Faculty
Courtney D DiNardo,
Keith W Pratz,
Richard M Ston
Moderat
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Levis, MD, PhD
el A Pollyea, MD,

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Alfred Hospital and Monash University
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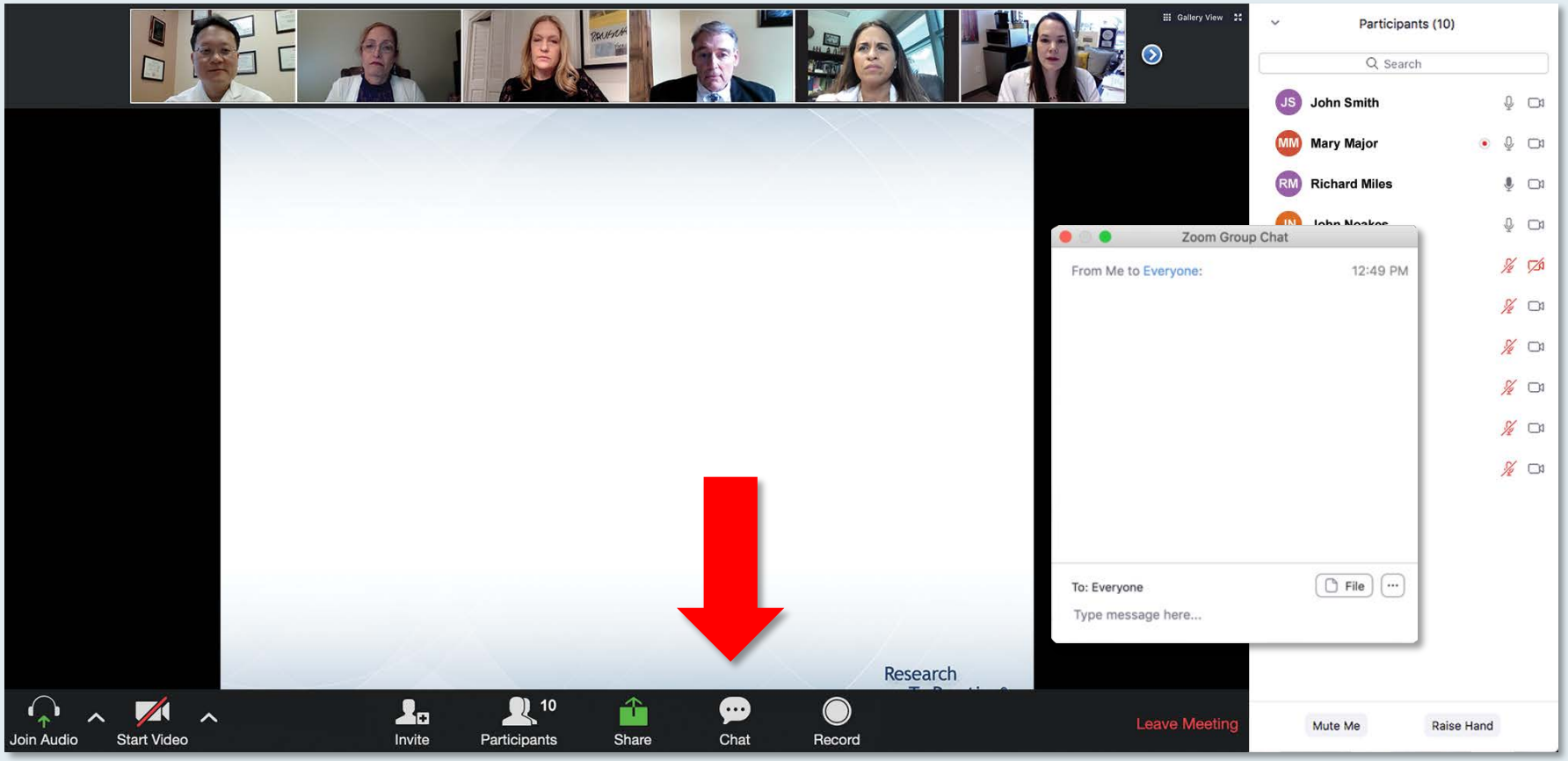

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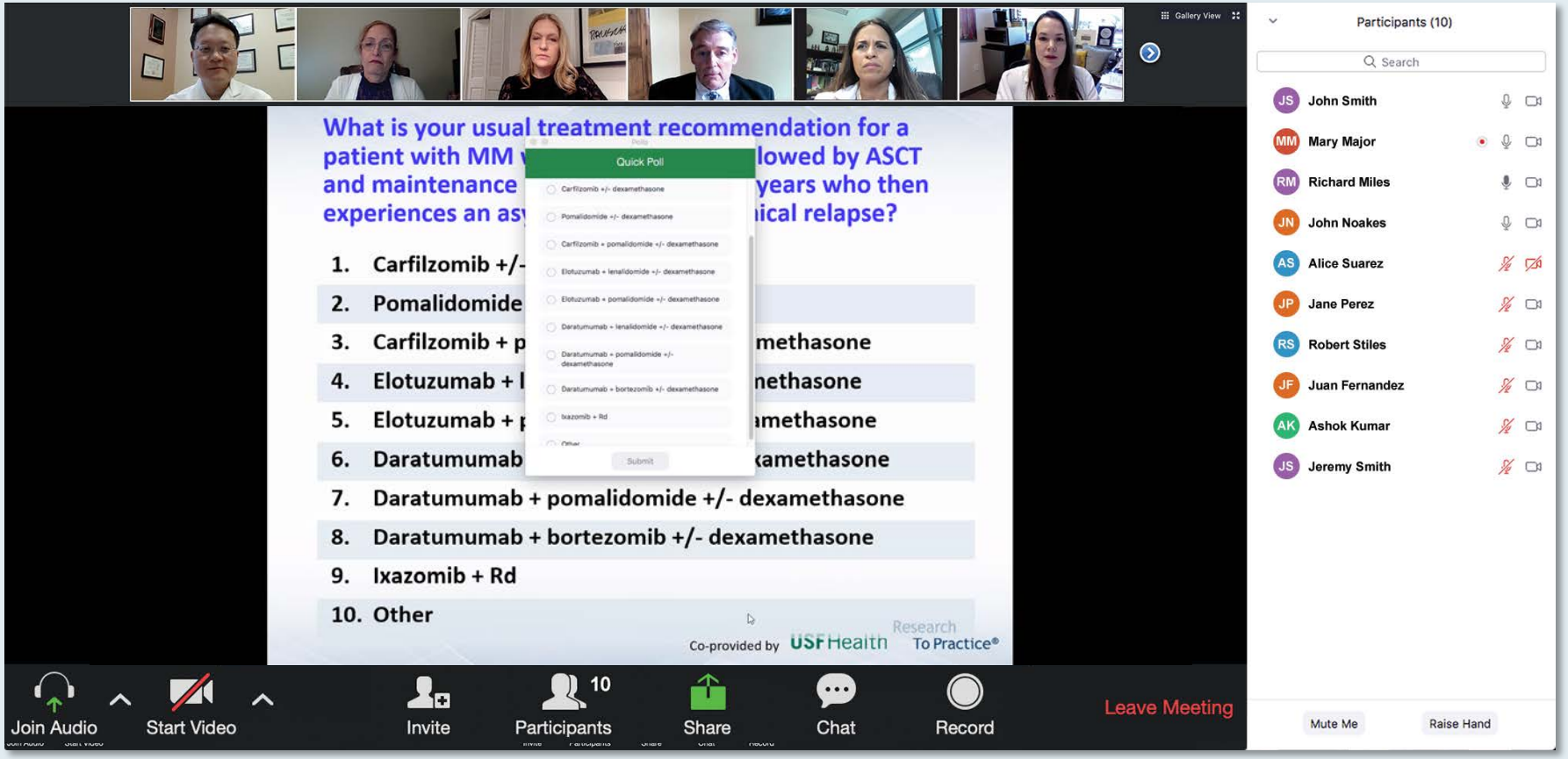

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Agenda

Module 1: Venetoclax combinations — Azacitidine, decitabine, LDAC, pracinostat

Module 2: FLT3 inhibitors — Midostaurin, gilteritinib, quizartinib

Module 3: IDH inhibitors — Ivosidenib, enasidenib

Module 4: Oral azacitidine (CC-486)

Module 5: Secondary AML — CPX-351

Module 6: Novel agents and strategies — Gemtuzumab ozogamicin, glasdegib, magrolimab

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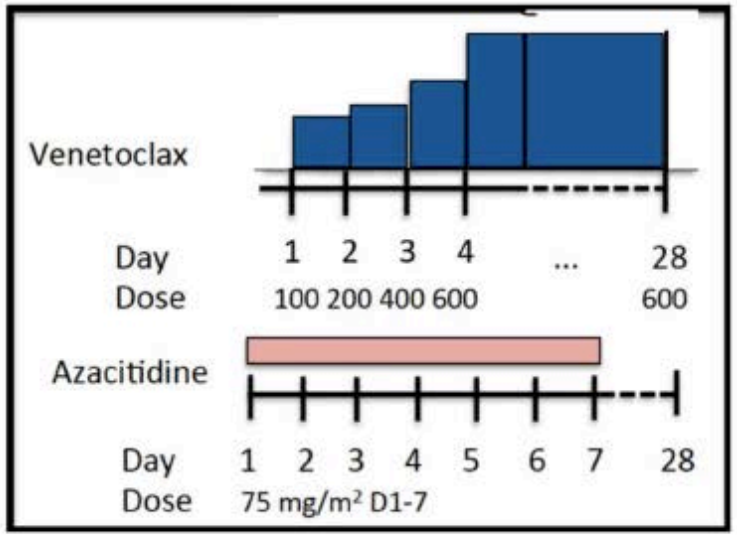
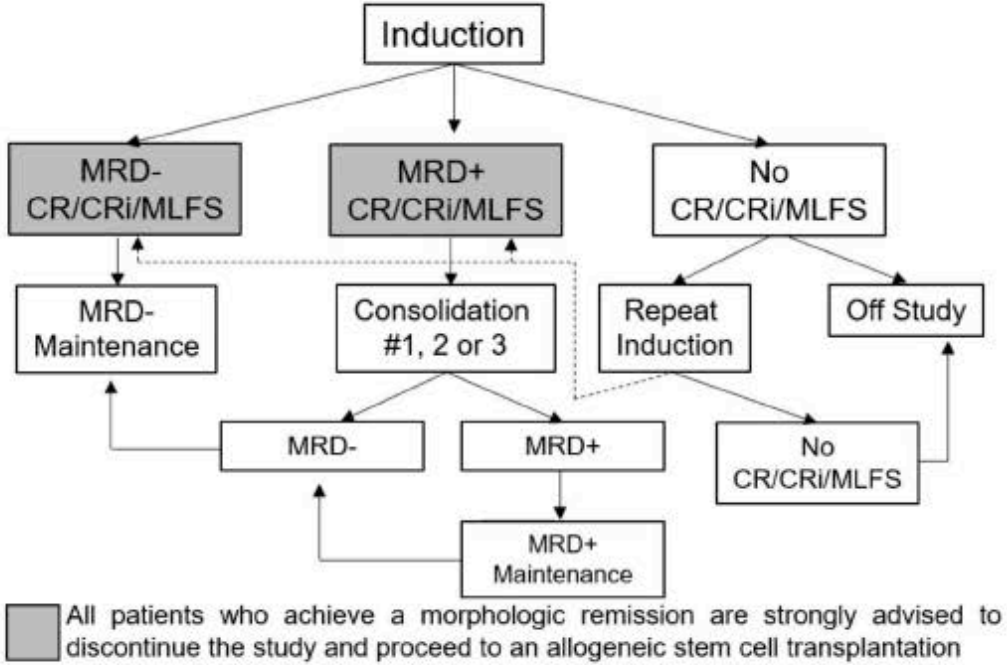
Venetoclax and Azacitidine for Newly Diagnosed Non-Elderly Adult Patients with Acute Myeloid Leukemia and Adverse Risk Features

Gutman JA et al.

ASH 2020;Abstract 2855.

Phase 2 Study Design





Frequent analysis (6 stages) to ensure CR rate ≥60%

Gutman JA et al. ASH 2020;Abstract 2855.

Outcomes

- 6/8 responded
  - 6/6 responses were CR
  - 4/6 cytogenetic remissions
  - 5/6 MRD negative by multi-parameter flow cytometry
  - 1/6 MRD negative by digital droplet PCR
- 2 relapses, 35 and 84 days after remission
- 7/8 proceeded to transplant; no relapses after transplant, median 340 days
- 7/8 alive

Gutman JA et al. ASH 2020;Abstract 2855.

Phase II Study of Venetoclax Added to Cladribine + Low Dose AraC (LDAC) Alternating with 5-Azacytidine Demonstrates High Rates of Minimal Residual Disease (MRD) Negative Complete Remissions (CR) and Excellent Tolerability in Older Patients with Newly Diagnosed Acute Myeloid Leukemia

Kadia TM et al.

ASH 2020;Abstract 25.

Cladribine/LDAC + Venetoclax in Older AML

Responses

Response / Outcome	N	%	MRD(-)
Evaluable for Response	54	98	
CR	42	78	39 (93)
CRi	8	15	3 (38)
CR + CRi (CRc)	50	93	42 (84)
No Response	4	7	
Died ≤ 4 weeks	1	2	
Died ≤ 8 weeks	2	4	
Median # of cycles given (Range)	2 (1 – 14)		
Median # of cycles to response (Range)	1 (1 – 3)		

Median follow-up in Surviving Patients : 14.2 months

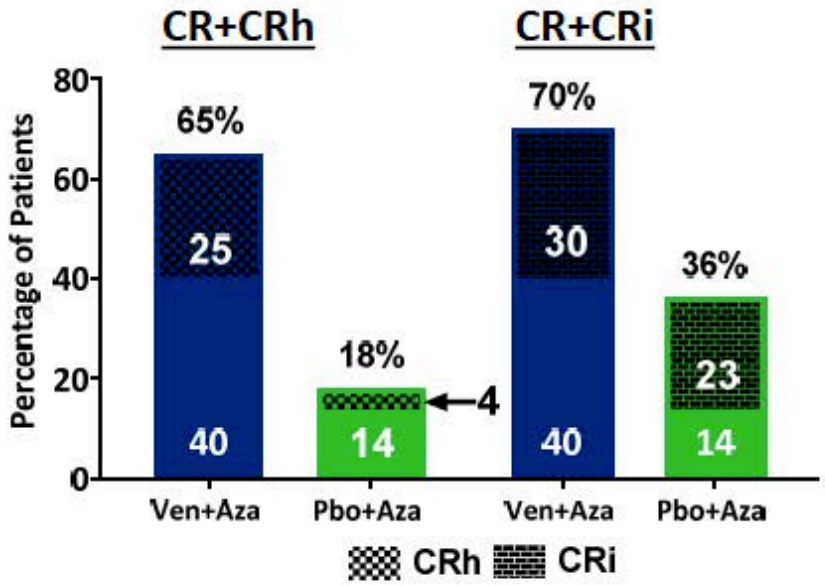
Kadia TM et al. ASH 2020;Abstract 25.

Results of Venetoclax and Azacitidine Combination in Chemotherapy Ineligible Untreated Patients with Acute Myeloid Leukemia with *FLT3* Mutations

Konopleva M et al.

ASH 2020;Abstract 1904.

Response rates in patients with *FLT3* mutation



	Ven+Aza (n=40)	Pbo+Aza (n=22)
Median treatment duration, cycles (range)	7.0 (1.0 — 31.0)	5.0 (1.0 — 21.0)
Median time to CR/CRh, months (range)	1.0 (0.8 — 4.8)	3.2 (1.8 — 3.6)
Median time to CR/CRi, months (range)	1.2 (0.8 – 7.7)	2.8 (1.0 – 11.2)

Aza: Azacitidine; Pbo: Placebo; Ven: Venetoclax; CR: Complete remission; CRi: CR with incomplete hematologic recovery; NR: not reached; CR was defined as absolute neutrophil count >10^3/µL, platelets >10^5/µL, red cell transfusion independence (TI), and bone marrow with <5% blasts; CRi was defined as all criteria for CR, except for neutropenia ≤10^3/µL or thrombocytopenia <10^5/µL; sample size for time to response analysis included responders only

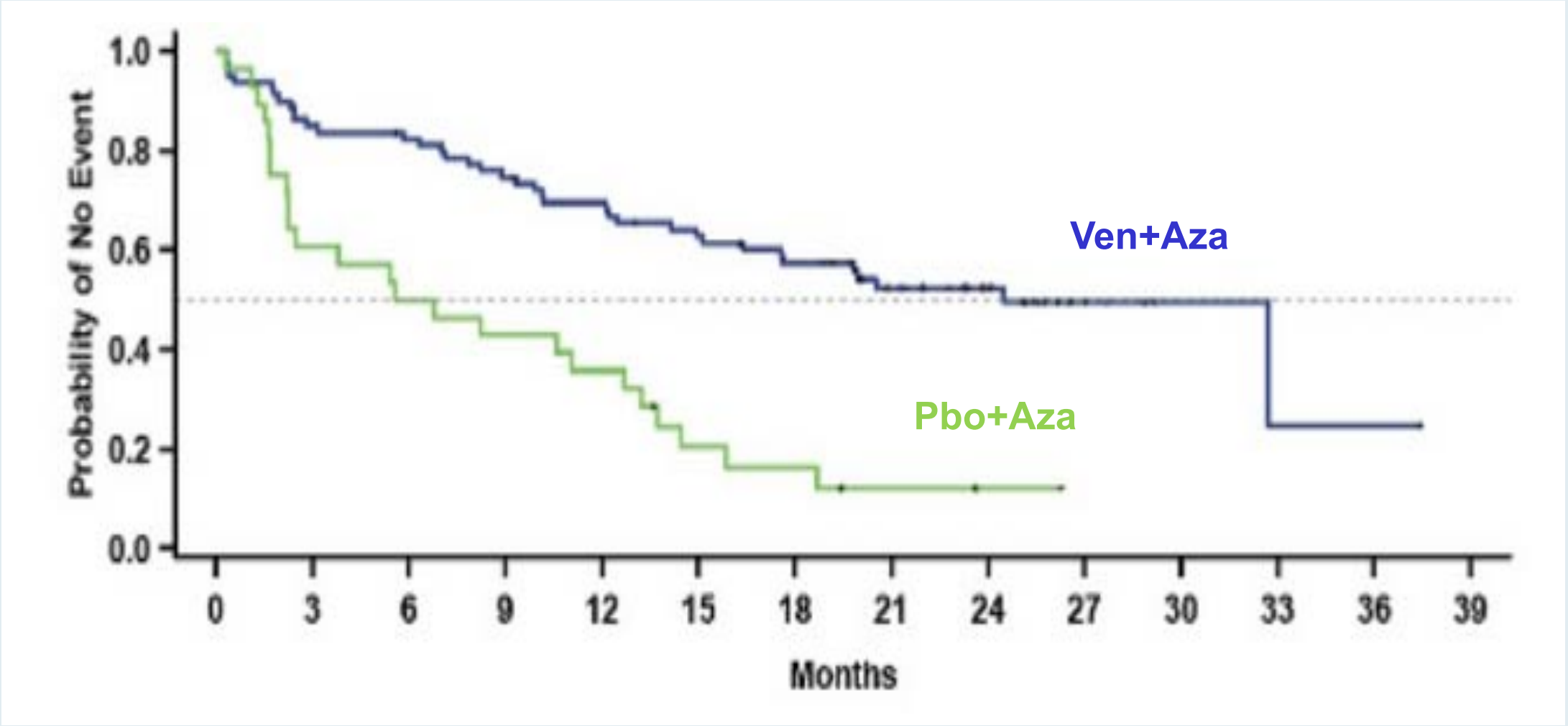
Konopleva M et al. ASH 2020;Abstract 1904.

Results of Venetoclax and Azacitidine Combination in Chemotherapy Ineligible Untreated Patients with Acute Myeloid Leukemia with *IDH 1/2* Mutations

Pollyea DA et al.

ASH 2020;Abstract 461.

Venetoclax with Azacitidine plus chemotherapy for AML with IDH1/2 Mutation: Overall Survival



Pollyea DA et al. ASH 2020;Abstract 461.

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Delays in Time to Deterioration of Health-Related Quality of Life Were Observed in Patients with Acute Myeloid Leukemia Receiving Venetoclax in Combination with Azacitidine or in Combination with Low-Dose Cytarabine

Pratz KW et al.

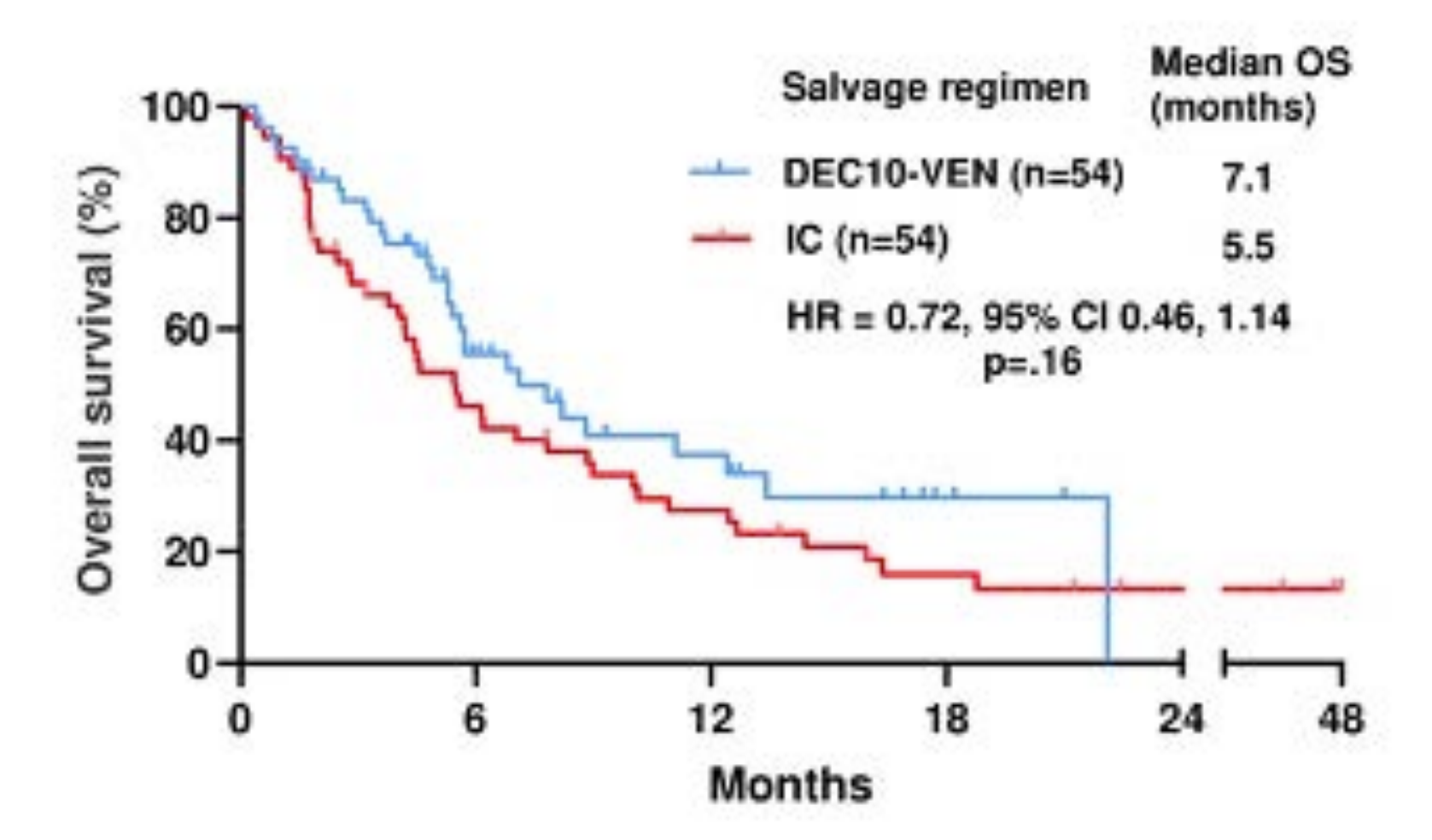
ASH 2020;Abstract 589.

Ten-Day Decitabine with Venetoclax versus Intensive Chemotherapy in Relapsed or Refractory Acute Myeloid Leukemia: A Propensity Score Matched Analysis

Maiti A et al.

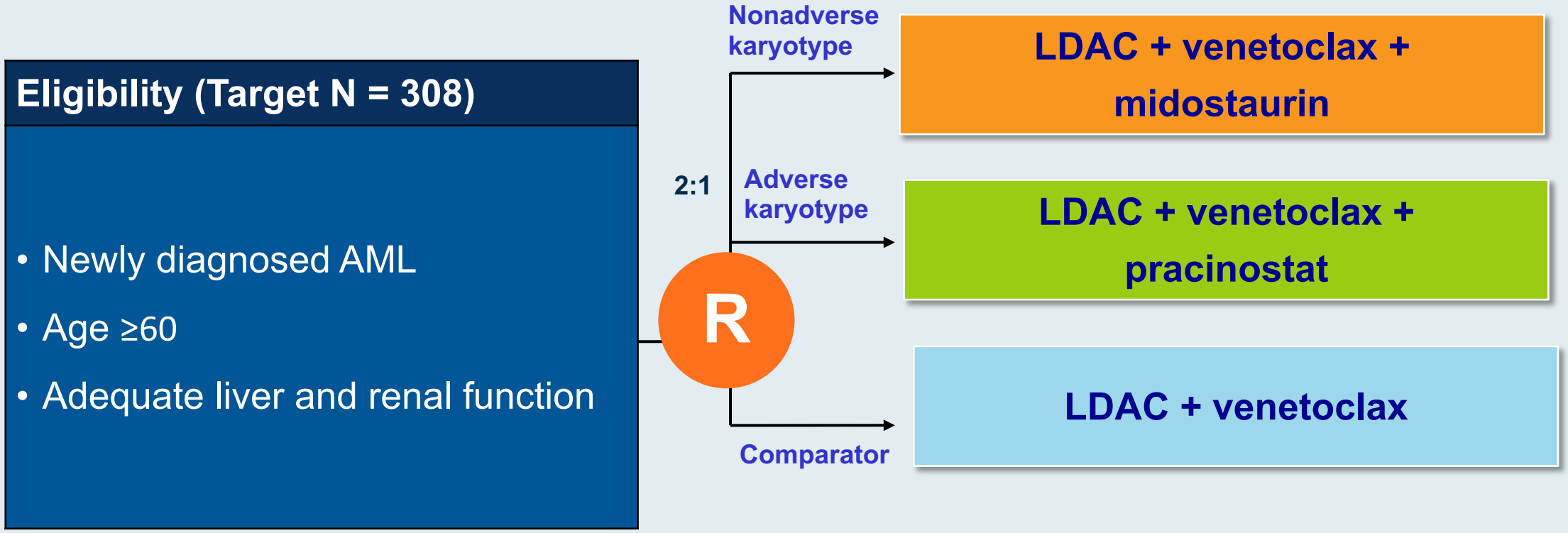
ASH 2020;Abstract 637.

Ten-Day Decitabine with Venetoclax versus Chemotherapy: Overall Survival



Maiti A et al. ASH 2020;Abstract 637.

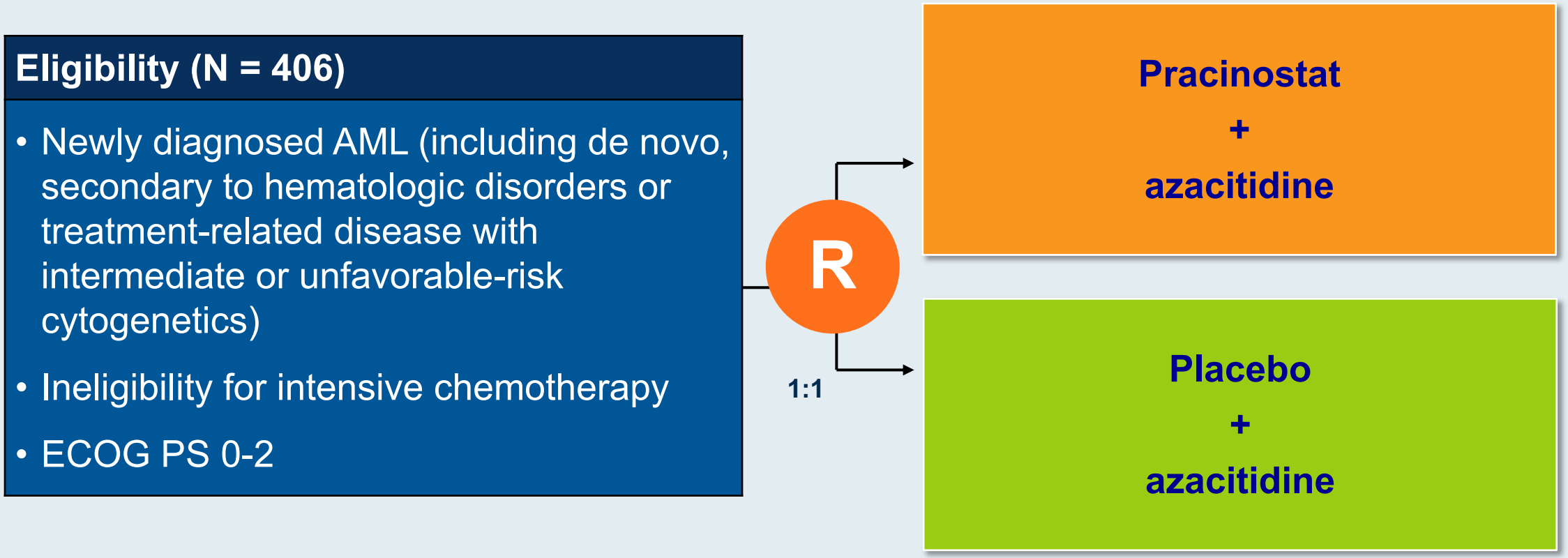
Phase II AMLM25 (INTERVENE) Trial Design



Endpoints: Objective response, safety/DLT, overall survival

https://www.anzctr.org.au/Trial/Registration/TrialReview.aspx?id=378194

Phase III PRAN-16-52 Trial Design



Primary endpoint: Overall survival

Secondary endpoints include morphologic CR rate, CR without MRD, cytogenetic CR rate and transfusion independence

www.clinicaltrials.gov (NCT03151408) – Accessed November 2020. Garcia-Manero G et al. ASCO 2018;Abstract TPS7078.

Phase III PRAN-16-52 Trial Discontinued After Completing Interim Analysis
Press Release: July 02, 2020

“An interim futility analysis of the ongoing Phase 3 study of pracinostat in combination with azacitidine in patients with AML who are unfit to receive standard intensive chemotherapy, undertaken by the study Independent Data Monitoring Committee (‘IDMC’), has demonstrated it was unlikely to meet the primary endpoint of overall survival compared to the control group.

Based on the outcome of the interim analysis, the decision was made to discontinue the recruitment of patients and end the study. The decision was based on a lack of efficacy and not on safety concerns.

Pending further evaluation, patients currently enrolled in other pracinostat studies will continue treatment.”

https://www.globenewswire.com/news-release/2020/07/02/2056824/0/en/Helsinn-Group-and-MEI-Pharma-Discontinue-the-Phase-3-Study-with-Pracinostat-in-AML-after-Completing-Interim-Analysis.html

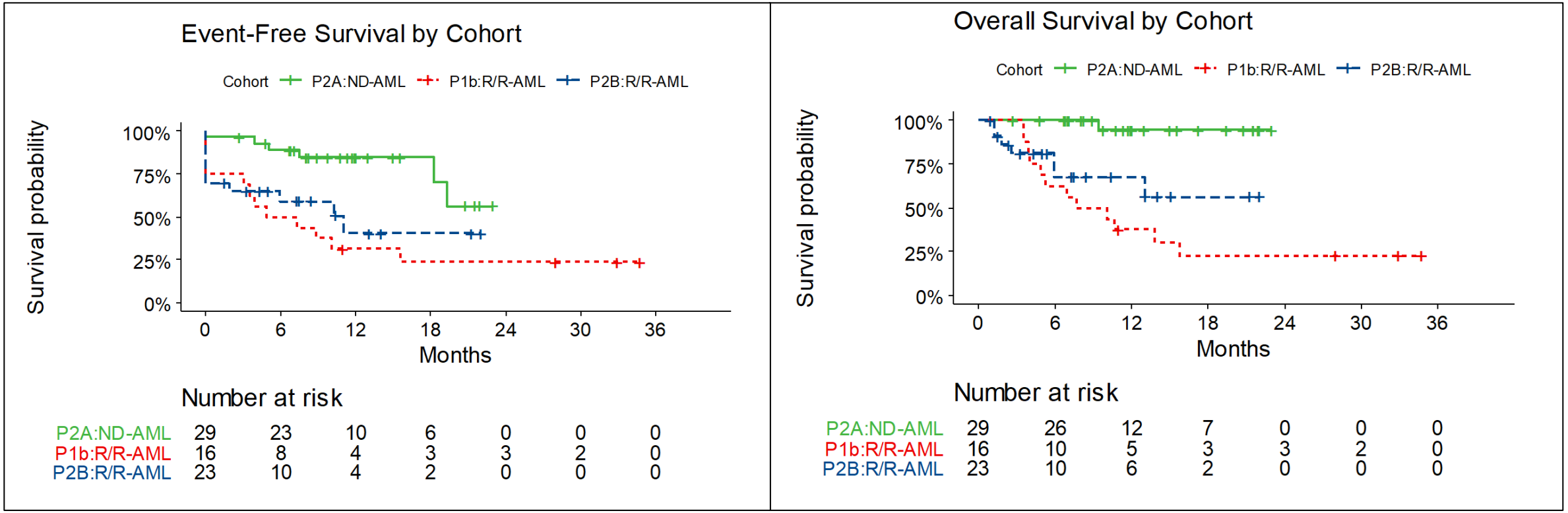
RTP RESEARCH TO PRACTICE

Interim Analysis of the Phase 1b/2 Study of the BCL-2 Inhibitor Venetoclax in Combination with Standard Intensive AML Induction/Consolidation Therapy with FLAG-IDA in Patients with Newly Diagnosed or Relapsed/Refractory AML

Lachowiez C et al.

ASH 2020;Abstract 332.

FLAG-IDA-VEN: Event-Free and Overall Survival



Variable	Phase 2A	Phase 1b	Phase 2B
Event-Free Survival	NR	6 (3-NE)	11 (2-NE)
Overall Survival	NR	9 (4.9-NE)	NR
1-year OS	94%	38%	68%

*Median study follow up: 12 months

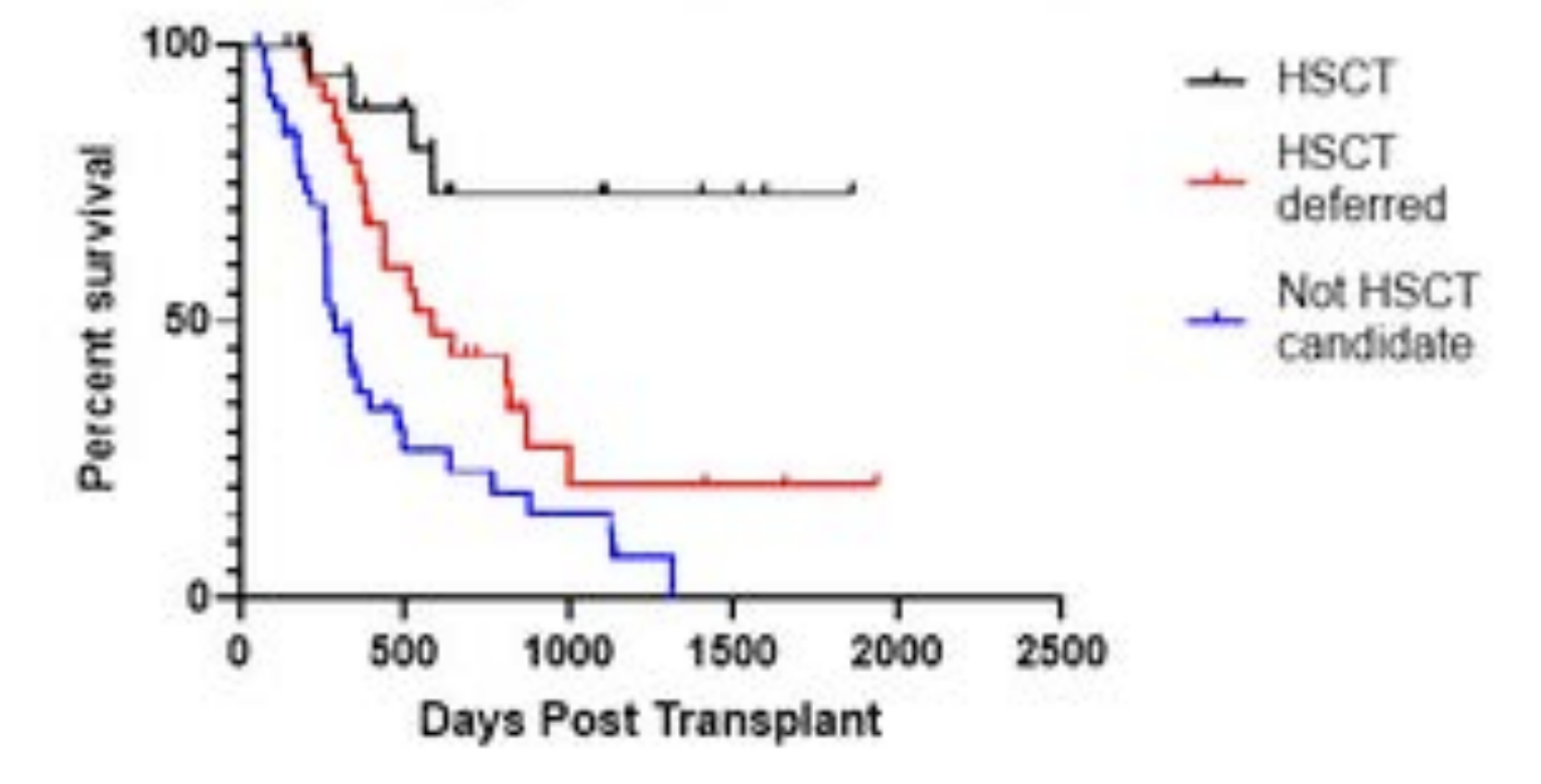
Lachowiez C et al. ASH 2020;Abstract 332.

Allogeneic Transplant Improves AML Outcomes Compared to Maintenance Venetoclax and Azacitidine Following Response to Initial Venetoclax and Azacitidine Therapy

Pollyea DA et al.

ASH 2020;Abstract 78.

Allogeneic Transplant: Overall Survival



Pollyea DA et al. ASH 2020;Abstract 78.

What initial treatment would you recommend for a 65-year-old man with AML with a PS of 1 and pancytopenia, 35% marrow myeloblasts, a complex karyotype and a TP53 mutation?

 MARK LEVIS, MD	Azacitidine + venetoclax	 ANDREW H WEI, MBBS, PHD	Azacitidine + venetoclax → SCT
 ALEXANDER PERL, MD	Azacitidine + venetoclax	 COURTNEY D DINARDO, MD, MSCE	Azacitidine + APR-246 OR Azacitidine + magrolimab
 DANIEL A POLLYEA, MD, MS	Azacitidine + venetoclax	 ALICE S MIMS, MD	Azacitidine + venetoclax
 EYTAN M STEIN, MD	Azacitidine + venetoclax	 EUNICE S WANG, MD	Decitabine + venetoclax

GENERAL MEDICAL ONCOLOGISTS (N = 75)	Azacitidine + venetoclax

What initial treatment would you generally recommend for an 80-year-old patient with AML and intermediate-risk cytogenetics?

 MARK LEVIS, MD	Azacitidine + venetoclax	 ANDREW H WEI, MBBS, PHD	Azacitidine + venetoclax
 ALEXANDER PERL, MD	Azacitidine + venetoclax	 COURTNEY D DINARDO, MD, MSCE	Azacitidine + venetoclax
 DANIEL A POLLYEA, MD, MS	Azacitidine + venetoclax	 ALICE S MIMS, MD	Azacitidine + venetoclax
 EYTAN M STEIN, MD	Azacitidine + venetoclax	 EUNICE S WANG, MD	Azacitidine + venetoclax
GENERAL MEDICAL ONCOLOGISTS (N = 75)	Azacitidine + venetoclax		

What initial treatment would you generally recommend for an 80-year-old patient with AML and poor-risk cytogenetics?

 MARK LEVIS, MD	Azacitidine + venetoclax	 ANDREW H WEI, MBBS, PHD	Azacitidine + venetoclax
 ALEXANDER PERL, MD	Azacitidine + venetoclax	 COURTNEY D DINARDO, MD, MSCE	Azacitidine + venetoclax
 DANIEL A POLLYEA, MD, MS	Azacitidine + venetoclax	 ALICE S MIMS, MD	Azacitidine + venetoclax
 EYTAN M STEIN, MD	Azacitidine + venetoclax	 EUNICE S WANG, MD	Azacitidine + venetoclax
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Module 2: FLT3 inhibitors — Midostaurin, gilteritinib, quizartinib

Module 3: IDH inhibitors — Ivosidenib, enasidenib

Module 4: Oral azacitidine (CC-486)

Module 5: Secondary AML — CPX-351

Module 6: Novel agents and strategies — Gemtuzumab ozogamicin, glasdegib, magrolimab

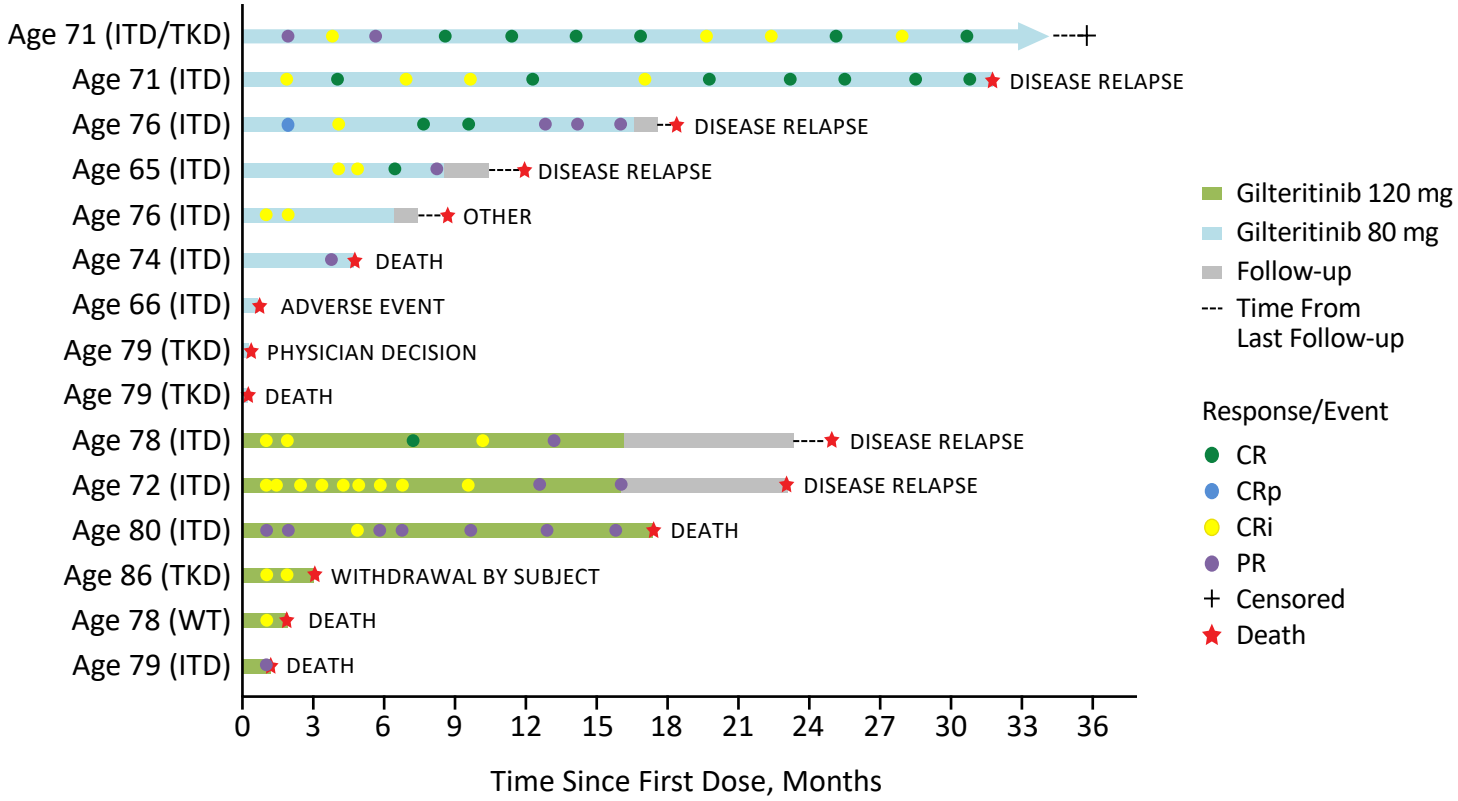
Phase 3, Multicenter, Open-Label Study of Gilteritinib, Gilteritinib plus Azacitidine, or Azacitidine Alone in Newly Diagnosed FLT3 Mutated (FLT3mut+) Acute Myeloid Leukemia (AML) Patients Ineligible for Intensive Induction Chemotherapy

Wang ES et al.

ASH 2020;Abstract 27.

Type and Duration of Response with Gilteritinib in Combination With AZA and End of Treatment Reasons

Safety Cohort (N = 15)



- CR and CRc were achieved by 33% (n = 5/15) and 67% (n = 10/15), respectively, of patients in the safety cohort.
- Among the 10 patients with CRc, the median (95% CI) duration of remission was 10.4 (0.95–NR) months, with 5 patients being censored.

AZA, azacitidine; CR, complete remission; CRc, composite complete remission; CRi, complete remission with incomplete hematologic recovery; CRp, complete remission with incomplete platelet recovery; ITD, internal tandem duplication; NR, not reached; PR, partial remission; TKD, tyrosine kinase domain; WT, wild type.

Wang ES et al. ASH 2020;Abstract 27.

Clinical Outcomes Following Treatment with Gilteritinib or Quizartinib in Patients with Relapsed/Refractory FLT3-ITD+ Acute Myeloid Leukemia

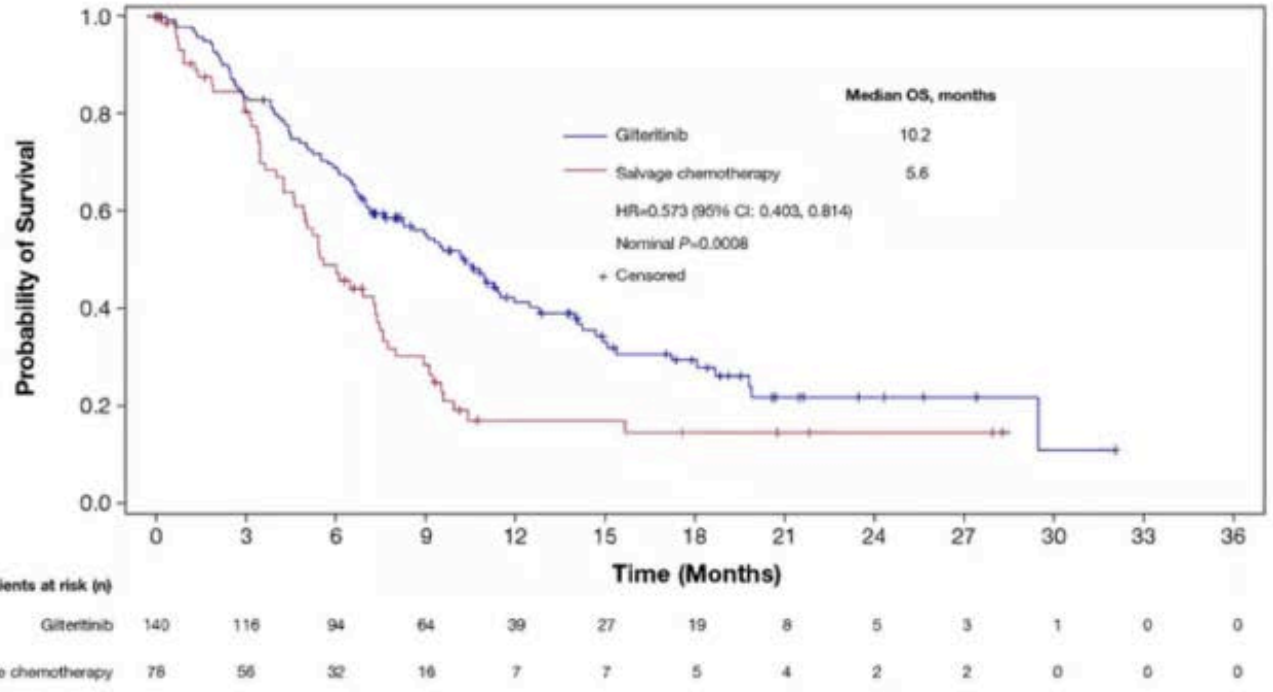
Perl AE et al.

ASH 2020;Abstract 995.

Results

Overall Survival

- Median overall survival (OS) in patients treated with quizartinib or gilteritinib was longer than that observed with salvage chemotherapy (SC)
  - After a median follow-up of 17.4 mos, median OS in the matched ADMIRAL ITT population was 10.2 mos with gilteritinib versus 5.6 mos with SC (HR=0.573 [95% CI: 0.403, 0.814]; one-sided nominal *P*=0.0008) (**Figure**)
  - After a median follow-up of 23.5 mos, median OS in the QuANTUM-R ITT population was 6.2 mos with quizartinib versus 4.7 mos with SC (HR=0.76 [95% CI: 0.58-0.98]; one-sided *P*=0.02)[1]



1. Cortes JE, et al. *Lancet Oncol*. 2019; 20(7):984-997.

American Society *of* Hematology

Abbreviations: CI, confidence interval; HR, hazard ratio; mos, months; OS, overall survival..

Perl AE et al. ASH 2020;Abstract 995.

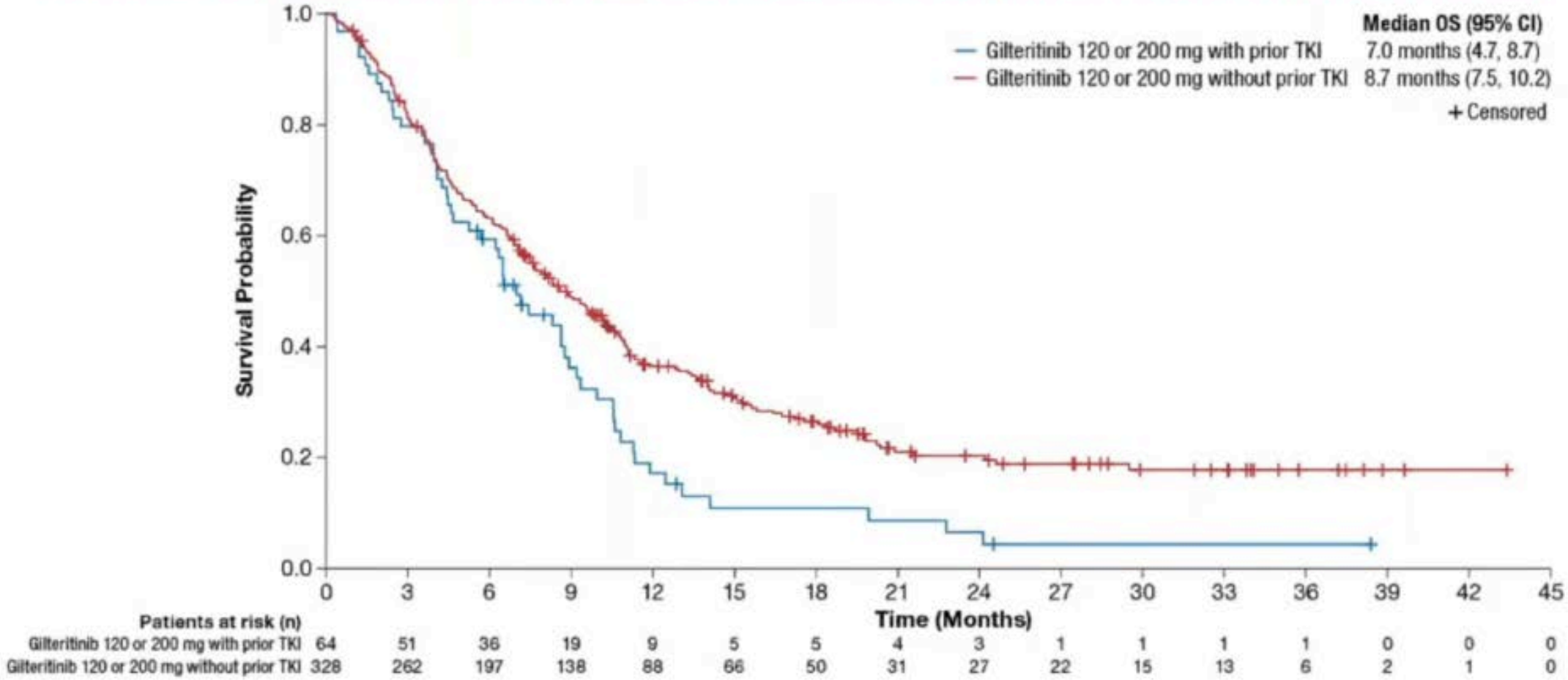
RTP RESEARCH TO PRACTICE

Clinical Outcomes in Patients with Relapsed/Refractory Acute Myeloid Leukemia Treated with Gilteritinib Who Received Prior Midostaurin or Sorafenib

Perl AE et al.

ASH 2020;Abstract 334.

Overall Survival: CHRYSALIS and ADMIRAL (120- or 200-mg Gilteritinib)



American Society of Hematology

Abbreviations: CI, confidence interval; HR, hazard ratio; OS, overall survival; TKI, tyrosine kinase inhibitor.

Perl AE et al. ASH 2020;Abstract 334.

RTP RESEARCH TO PRACTICE

A Phase 1 Study of Gilteritinib in Combination with Induction and Consolidation Chemotherapy in Patients with Newly Diagnosed AML: Final Results

Pratz KW et al.

ASH 2020;Abstract 24.

Gilterinitib in Combination with 7 + 3 Induction: Clinical Response

Response Parameter, n (%)	*FLT3*$^{mut+}$ Patients who Received 120 mg/d (N=38)
CR	15 (39.5)
CRp	1 (2.6)
CRi	15 (39.5)
CRc	31 (81.6)

Pratz KW et al. ASH 2020;Abstract 24.

Gilteritinib Remains Clinically Active in Relapsed/Refractory FLT3 Mutated AML Previously Treated with FLT3 Inhibitors

Numan Y et al.

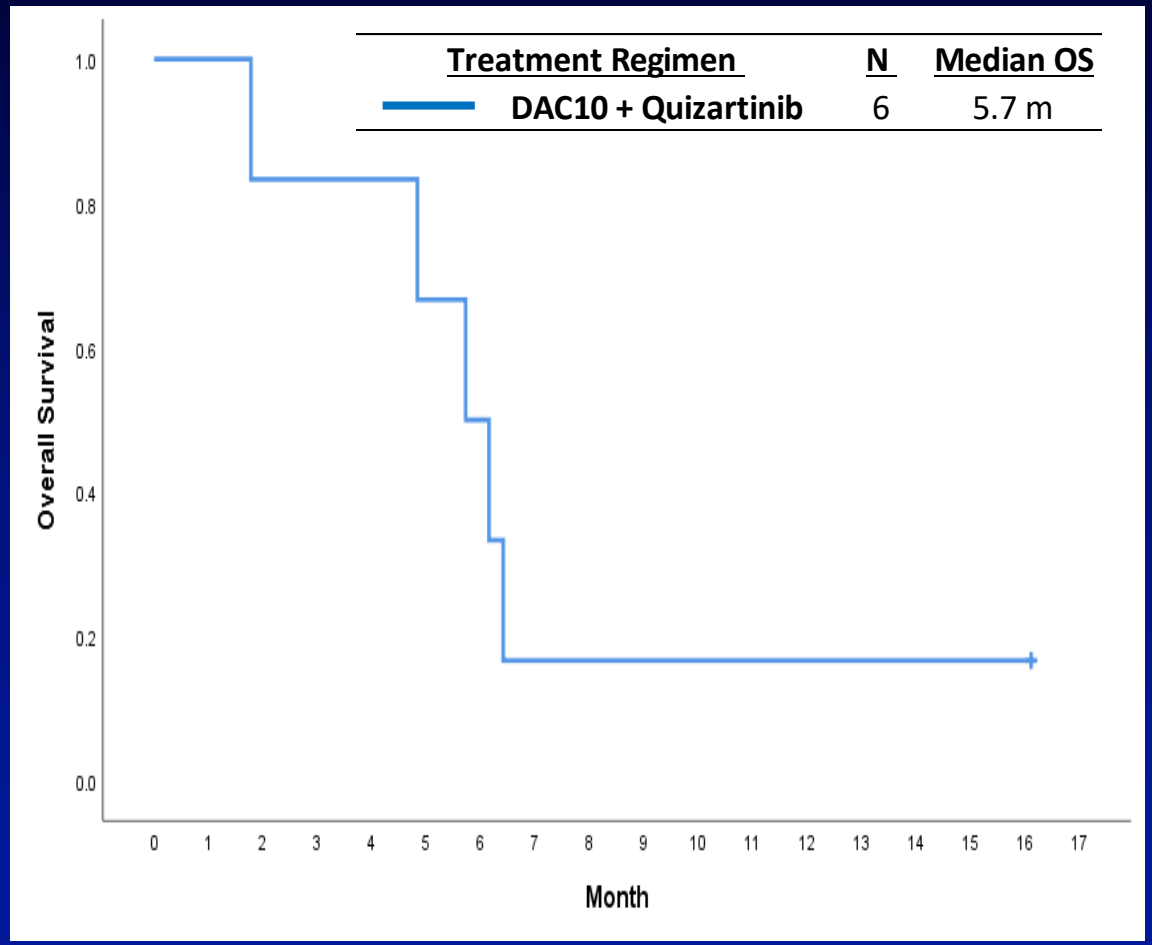
ASH 2020;Abstract 262.

Quizartinib with Decitabine +/- Venetoclax Is Highly Active in Patients (Pts) with FLT3-ITD Mutated (mut) Acute Myeloid Leukemia (AML): Clinical Report and Signaling Cytof Profiling from a Phase IB/II Trial

Yilmaz M et al.

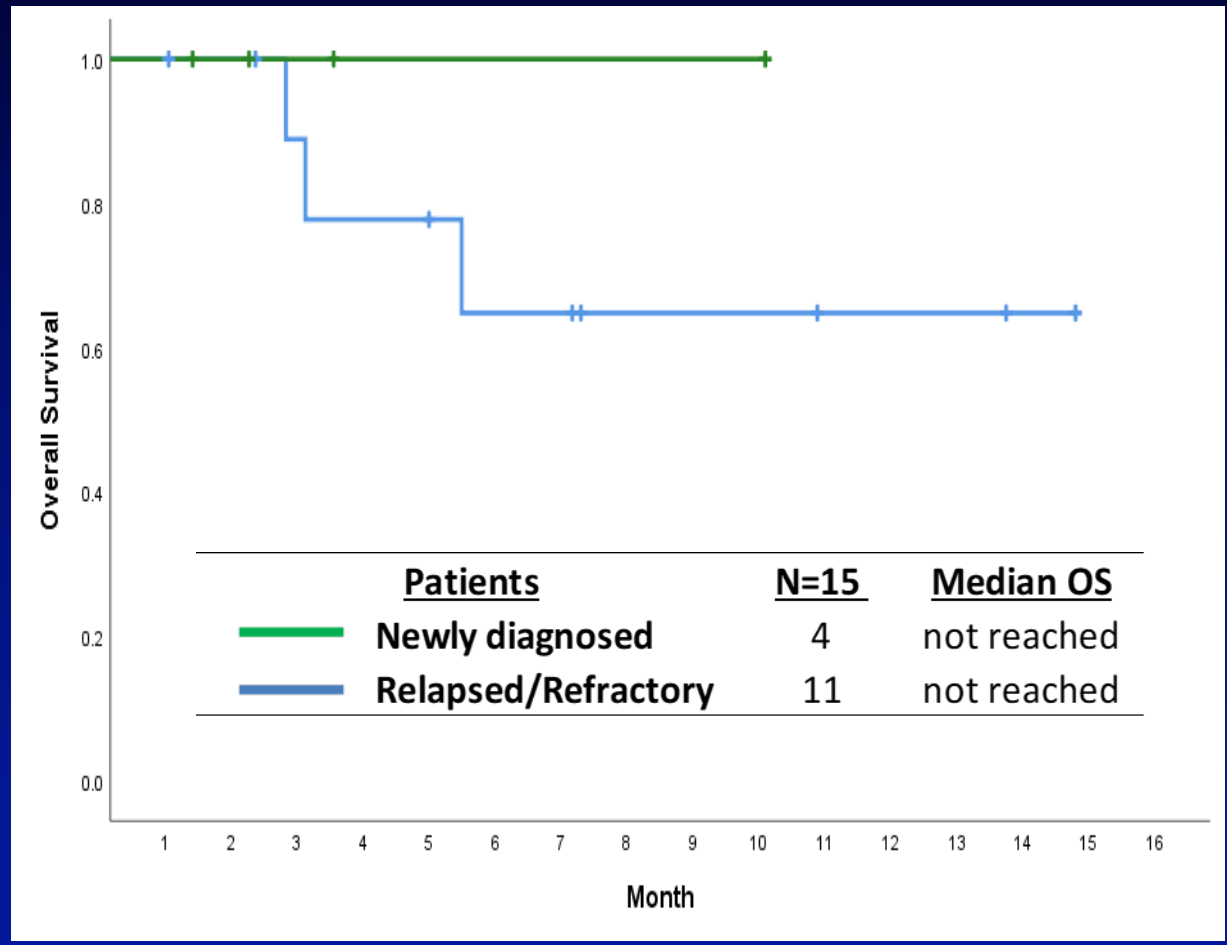
ASH 2020;Abstract 26.

OS – DAC + Quizartinib (R/R FLT3 AML, N = 6)



Median follow-up: 16 months

OS – DAC + Quizartinib + Venetoclax in Front-Line and R/R FLT3 AML



Median follow-up: 7 months

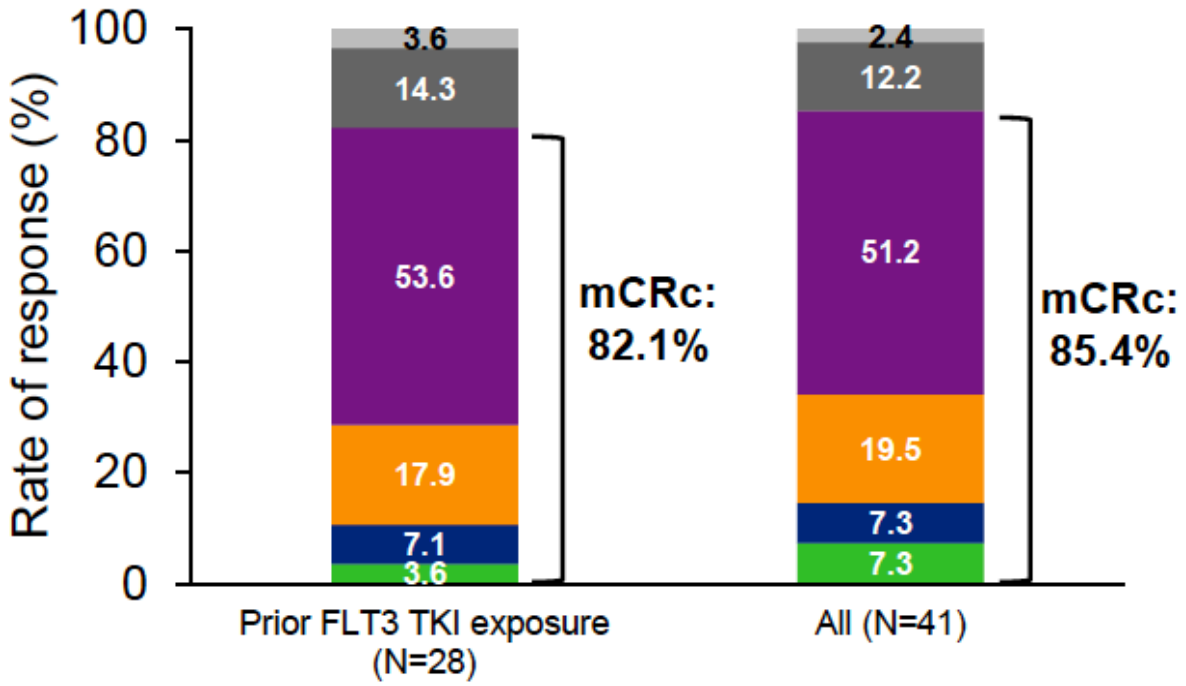
Yilmaz M et al. ASH 2020;Abstract 26.

Efficacy and Safety of Venetoclax in Combination with Gilteritinib for Relapsed/Refractory FLT3-Mutated Acute Myeloid Leukemia in the Expansion Cohort of a Phase 1b Study

Daver N et al.

ASH 2020;Abstract 333.

Summary of Best Responses



	All (N=41)
mCRc, n (%)	35 (85.4%)
Time to best response (mCRc), median (range), months	0.9 (0.7–4.2)

The 85% mCRc rate compares favorably to the 52% CRc rate (using the same response parameters), with single agent Gilt in the ADMIRAL phase 3 study[1]

Data cut off: April 15, 2020. Analyses were conducted using data from all treated ITD and/or TKD patients irrespective of the availability of postbaseline disease assessment data prior to data cut-off date (ITT analysis), including patients who received non-RP2D dose during dose expansion phase. Two on-treatment patients did not have their first disease assessment at the cutoff date and were not included in the efficacy analyses. No patients achieved partial remission. One patient (TKD only) discontinued with no response data

AML, acute myeloid leukemia; CI, confidence interval; CR, complete remission; CRi, CR with incomplete blood count recovery; CRp, CR with incomplete platelet recovery; FLT3, FMS-like tyrosine kinase 3; Gilt, gilteritinib; ITD, internal tandem duplications; mCRc, ITT, intention to treat; modified composite complete remission; MLFS, morphologic leukemia free state; NE, not estimable; PD, progressive disease; RD, resistant disease; TKI, tyrosine kinase inhibitor; TKD, tyrosine kinase domain

1. Perl AE, et al. N Engl J Med. 2019;381(18):1728–1740

Daver N et al. ASH 2020;Abstract 333.

What would you recommend as first-line therapy to a 60-year-old patient who presents with intermediate-risk AML and a FLT3-TKD mutation?

 MARK LEVIS, MD	7 + 3 induction + midostaurin	 ANDREW H WEI, MBBS, PHD	7 + 3 induction + midostaurin
 ALEXANDER PERL, MD	7 + 3 induction + midostaurin	 COURTNEY D DINARDO, MD, MSCE	7 + 3 induction + midostaurin
 DANIEL A POLLYEA, MD, MS	7 + 3 induction + midostaurin	 ALICE S MIMS, MD	7 + 3 induction + midostaurin
 EYTAN M STEIN, MD	7 + 3 induction + midostaurin	 EUNICE S WANG, MD	7 + 3 induction + midostaurin

GENERAL MEDICAL ONCOLOGISTS (N = 75)	7 + 3 induction + midostaurin

What would you recommend as first-line therapy to a 60-year-old patient who presents with intermediate-risk AML and a FLT3-ITD mutation?

 MARK LEVIS, MD	7 + 3 induction + midostaurin	 ANDREW H WEI, MBBS, PHD	7 + 3 induction + midostaurin
 ALEXANDER PERL, MD	7 + 3 induction + midostaurin	 COURTNEY D DINARDO, MD, MSCE	7 + 3 induction + midostaurin OR 7 + 3 + gilteritinib
 DANIEL A POLLYEA, MD, MS	7 + 3 induction + midostaurin	 ALICE S MIMS, MD	7 + 3 induction + midostaurin
 EYTAN M STEIN, MD	7 + 3 induction + midostaurin	 EUNICE S WANG, MD	7 + 3 induction + midostaurin

GENERAL MEDICAL ONCOLOGISTS (N = 75)	7 + 3 induction + midostaurin

What would you recommend as first-line therapy to a 78-year-old patient (PS 0) who presents with intermediate-risk AML with a FLT3-ITD mutation?

1. Midostaurin
2. 7 + 3 induction + midostaurin
3. Hypomethylating agent (HMA)
4. HMA + venetoclax
5. HMA + venetoclax + FLT3 inhibitor
6. Low-dose cytarabine + venetoclax
7. Low-dose cytarabine + venetoclax + FLT3 inhibitor
8. Gilteritinib
9. Other

What would you recommend as first-line therapy to a 78-year-old patient (PS 0) who presents with intermediate-risk AML with a FLT3-ITD mutation?

 MARK LEVIS, MD	Azacitidine + venetoclax x 2 → azacitidine + gilteritinib x 2 → gilteritinib	 ANDREW H WEI, MBBS, PHD	Azacitidiine + venetoclax + gilteritinib
 ALEXANDER PERL, MD	Azacitidine + venetoclax	 COURTNEY D DINARDO, MD, MSCE	Azacitidine + venetoclax + gilteritinib
 DANIEL A POLLYEA, MD, MS	Azacitidine + venetoclax	 ALICE S MIMS, MD	Azacitidine + gilteritinib
 EYTAN M STEIN, MD	Azacitidine + venetoclax	 EUNICE S WANG, MD	Azacitidine + venetoclax
GENERAL MEDICAL ONCOLOGISTS (N = 75)	Azacitidine + venetoclax		

Agenda

Module 1: Venetoclax combinations — Azacitidine, decitabine, LDAC, pracinostat

Module 2: FLT3 inhibitors — Midostaurin, gilteritinib, quizartinib

Module 3: IDH inhibitors — Ivosidenib, enasidenib

Module 4: Oral azacitidine (CC-486)

Module 5: Secondary AML — CPX-351

Module 6: Novel agents and strategies — Gemtuzumab ozogamicin, glasdegib, magrolimab

A Phase I Study of the IDH2 Inhibitor Enasidenib as Maintenance Therapy for IDH2-Mutant Myeloid Neoplasms Following Hematopoietic Cell Transplantation

Fathi AT et al.

ASH 2020;Abstract 2402.

Safety and Tolerability

Dose Level	Enrollment	Dose Limiting Toxicity	Attributable ≥ Grade 3 Adverse Events
1 (50mg QD)	3	No	G3 Anemia
2 (100mg QD)	6	No	G3 Bilirubinemia, G4 Neutropenia
Expansion (100mg QD)	7 (of 10)	NA	

- Six patients (38%) have required dose interruptions lasting a median of 19 days (range 7-25 days).
- Four patients have required a dose reduction to 50mg from 100mg daily.
- In total, 3 patients (18%) have to date discontinued study treatment, one for G3 bilirubinemia, one to pursue another trial for GVHD, and one for relapse.
- Three patients have experienced ≥ G2 acute GVHD, and four patients experienced moderate chronic GVHD.

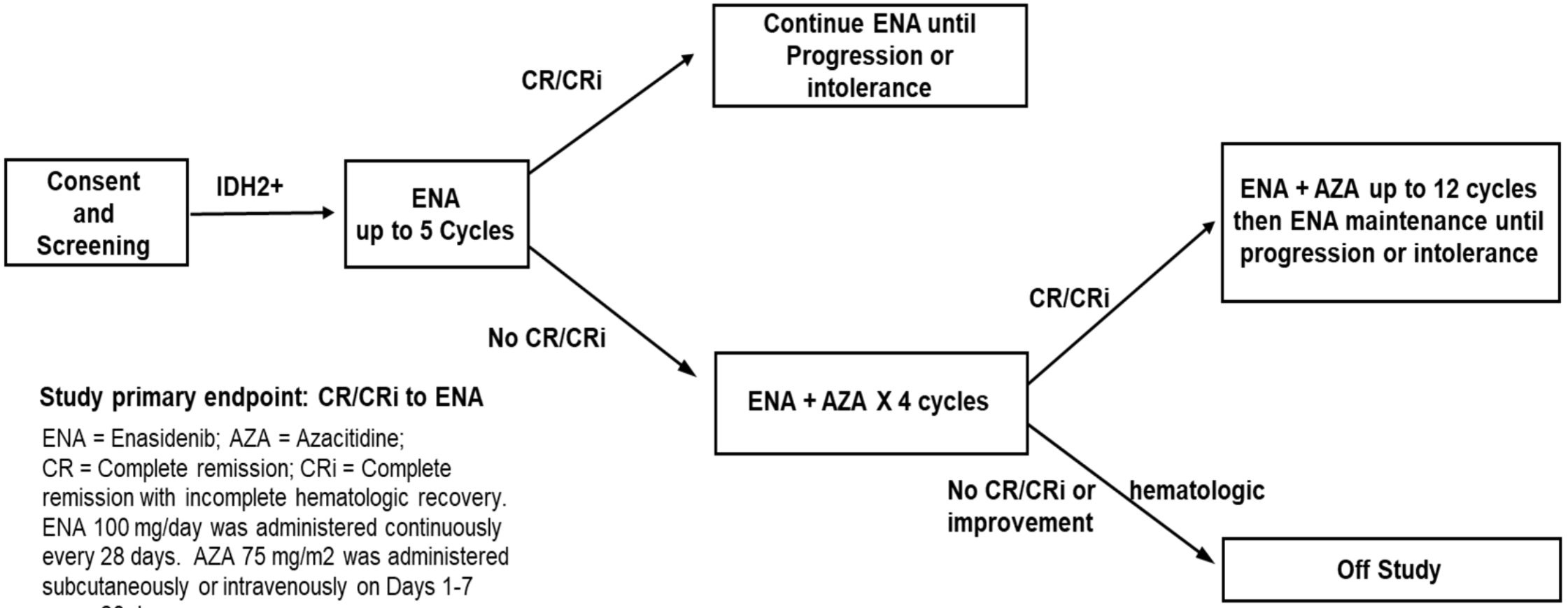
Fathi AT et al. ASH 2020;Abstract 2402.

Enasidenib (ENA) Monotherapy with Addition of Azacitidine in Non-Responders Is Effective in Older Patients with Newly Diagnosed IDH2 Mutated Acute Myeloid Leukemia (AML): A Completed Phase 2/1b Sub-Study of the Beat AML Master Trial

Stein EM et al.

ASH 2020;Abstract 636.

S3 - STUDY DESIGN AND OBJECTIVES



Study primary endpoint: CR/CRi to ENA

ENA = Enasidenib; AZA = Azacitidine; CR = Complete remission; CRi = Complete remission with incomplete hematologic recovery. ENA 100 mg/day was administered continuously every 28 days. AZA 75 mg/m2 was administered subcutaneously or intravenously on Days 1-7 every 28 days.

Stein EM et al. ASH 2020;Abstract 636.

RESPONSE

Parameter	Phase 2 Enasidenib 100 mg/day (n=60)	Phase 1b Enasidenib + Azacitidine (n=17)
Duration of Response in months Median, 95% CI	NR, 7.1-NR	NR, 2.5 – NR
Overall Survival Median in months, 95% CI	24.4, 10.6-NE	8.9, 5.2-NE
Median Follow-up in months (range)	14.6 (6.2-33.5)	12.7 (1.1-31.4)

Stein EM et al. ASH 2020;Abstract 636.

Molecular Characterization of Clinical Response and Relapse in Patients with IDH1-Mutant Newly Diagnosed Acute Myeloid Leukemia Treated with Ivosidenib and Azacitidine

Daigle SR et al.

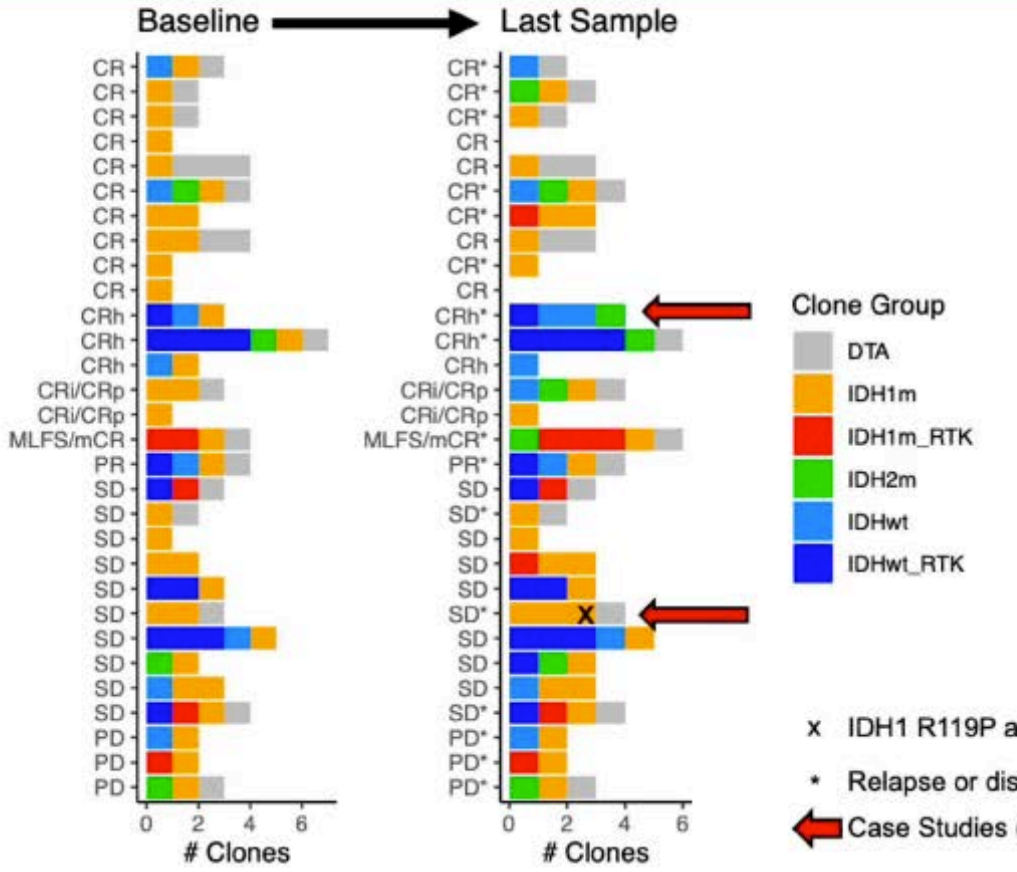
ASH 2020;Abstract 1943.

Longitudinal Molecular Profiling in Patients with IDH1-Mutant Newly Diagnosed Acute Myeloid Leukemia Treated with Ivosidenib

Choe S et al.

ASH 2020;Abstract 2900.

Single-cell DNAseq dataset summary (n = 30)



- Baseline and longitudinal single-cell DNAseq profiling was performed on PBMC samples from 30 patients using MissionBio's Tapestri™ 20-gene AML panel
- As a result of the higher sensitivity of the single-cell DNAseq platform (0.1%), *IDH2* mutations were detected more frequently upon treatment than with bulk sequencing (8 out of 30 patients). In this dataset, *IDH2* mutations did not co-occur with m*IDH1* in the same cell
- *RTK* pathway mutations were also frequently detected in *IDH1* wild type clones (8 out of 12 patients with mutations in *NRAS, KRAS,* or *PTPN11*)

CR = complete response; CRh = complete response with partial hematologic recovery; DNAseq = deoxyribonucleic acid sequencing; *IDH* = isocitrate dehydrogenase; m = mutant; PD = progressive disease; SD = stable disease; wt = wild type; DTA = DNMT3A, TET2, and/or ASXL1

Choe S et al. ASH 2020;Abstract 2900.

Transplant Outcomes for IDH-Mutated AML: Good Outcomes Thanks to Keeping Good Company

Ambinder AJ et al.

ASH 2020;Abstract 1525.

What would you recommend as first-line therapy to a 60-year-old patient who presents with intermediate-risk AML with an IDH1 mutation?

What would you recommend as first-line therapy to a 78-year-old patient (PS 0) who presents with intermediate-risk AML with an IDH1 mutation?

1. 7 + 3 induction
2. HMA
3. HMA + venetoclax
4. HMA + venetoclax + ivosidenib
5. Low-dose cytarabine + venetoclax
6. Low-dose cytarabine + venetoclax + ivosidenib
7. HMA + ivosidenib
8. Ivosidenib
9. Other

What would you recommend as first-line therapy to a 78-year-old patient (PS 0) who presents with intermediate-risk AML with an IDH1 mutation?

 MARK LEVIS, MD	Azacitidine + ivosidenib	 ANDREW H WEI, MBBS, PHD	Azacitidine + venetoclax
 ALEXANDER PERL, MD	Azacitidine + venetoclax	 COURTNEY D DINARDO, MD, MSCE	Azacitidine + ivosidenib
 DANIEL A POLLYEA, MD, MS	Azacitidine + venetoclax	 ALICE S MIMS, MD	Ivosidenib
 EYTAN M STEIN, MD	Azacitidine + venetoclax	 EUNICE S WANG, MD	Azacitidine + venetoclax

GENERAL MEDICAL ONCOLOGISTS (N = 75)	Azacitidine + venetoclax, Ivosidenib

What would you generally recommend as the next line of treatment for a 60-year-old patient with AML with an IDH2 mutation who has experienced disease progression after 7 + 3 induction, consolidation therapy and transplant?

1. Chemotherapy
2. HMA + venetoclax
3. HMA + venetoclax + enasidenib
4. Low-dose cytarabine
5. Low-dose cytarabine + venetoclax
6. Low-dose cytarabine + venetoclax + enasidenib
7. HMA + enasidenib
8. Enasidenib
9. Other

What would you generally recommend as the next line of treatment for a 60-year-old patient with AML with an IDH2 mutation who has experienced disease progression after 7 + 3 induction, consolidation therapy and transplant?

 MARK LEVIS, MD	Azacitidine + enasidenib	 ANDREW H WEI, MBBS, PHD	Azacitidine + venetoclax
 ALEXANDER PERL, MD	Azacitidine + venetoclax	 COURTNEY D DINARDO, MD, MSCE	Azacitidine + venetoclax
 DANIEL A POLLYEA, MD, MS	Enasidenib	 ALICE S MIMS, MD	Enasidenib
 EYTAN M STEIN, MD	Enasidenib	 EUNICE S WANG, MD	Azacitidine + venetoclax
GENERAL MEDICAL ONCOLOGISTS (N = 75)	Enasidenib		

What would you generally recommend as the next line of treatment for a 78-year-old patient with AML with an IDH2 mutation who has experienced disease progression after venetoclax/azacitidine?

 MARK LEVIS, MD	Enasidenib	 ANDREW H WEI, MBBS, PHD	Enasidenib
 ALEXANDER PERL, MD	Enasidenib	 COURTNEY D DINARDO, MD, MSCE	Azacitidine + venetoclax + enasidenib or enasidenib
 DANIEL A POLLYEA, MD, MS	Enasidenib	 ALICE S MIMS, MD	Enasidenib
 EYTAN M STEIN, MD	Enasidenib	 EUNICE S WANG, MD	Enasidenib
GENERAL MEDICAL ONCOLOGISTS (N = 75)	Enasidenib		

A 65-year-old patient presents with new-onset shortness of breath, hypoxemia and fever 3 weeks into therapy with ivosidenib for relapsed AML. Chest CT reveals diffuse ground glass infiltrates. The patient has an ANC of 600, 27% blasts in the blood and has been receiving prophylaxis with levofloxacin and acyclovir only. What would you recommend?

 MARK LEVIS, MD	Continue ivosidenib and begin antibiotics and corticosteroids
 ALEXANDER PERL, MD	Test for COVID-19; continue ivosidenib and begin antibiotics and corticosteroids
 DANIEL A POLLYEA, MD, MS	Continue ivosidenib and begin antibiotics and corticosteroids
 EYTAN M STEIN, MD	Continue ivosidenib and begin antibiotics and corticosteroids
 ANDREW H WEI, MBBS, PHD	Continue ivosidenib and begin antibiotics and corticosteroids
 COURTNEY D DINARDO, MD, MSCE	Continue ivosidenib and begin antibiotics and corticosteroids
 ALICE S MIMS, MD	Continue ivosidenib and begin antibiotics and corticosteroids
 EUNICE S WANG, MD	Continue ivosidenib and begin antibiotics and corticosteroids
GENERAL MEDICAL ONCOLOGISTS (N = 75)	Continue ivosidenib and begin antibiotics and corticosteroids

Agenda

Module 1: Venetoclax combinations — Azacitidine, decitabine, LDAC, pracinostat

Module 2: FLT3 inhibitors — Midostaurin, gilteritinib, quizartinib

Module 3: IDH inhibitors — Ivosidenib, enasidenib

Module 4: Oral azacitidine (CC-486)

Module 5: Secondary AML — CPX-351

Module 6: Novel agents and strategies — Gemtuzumab ozogamicin, glasdegib, magrolimab

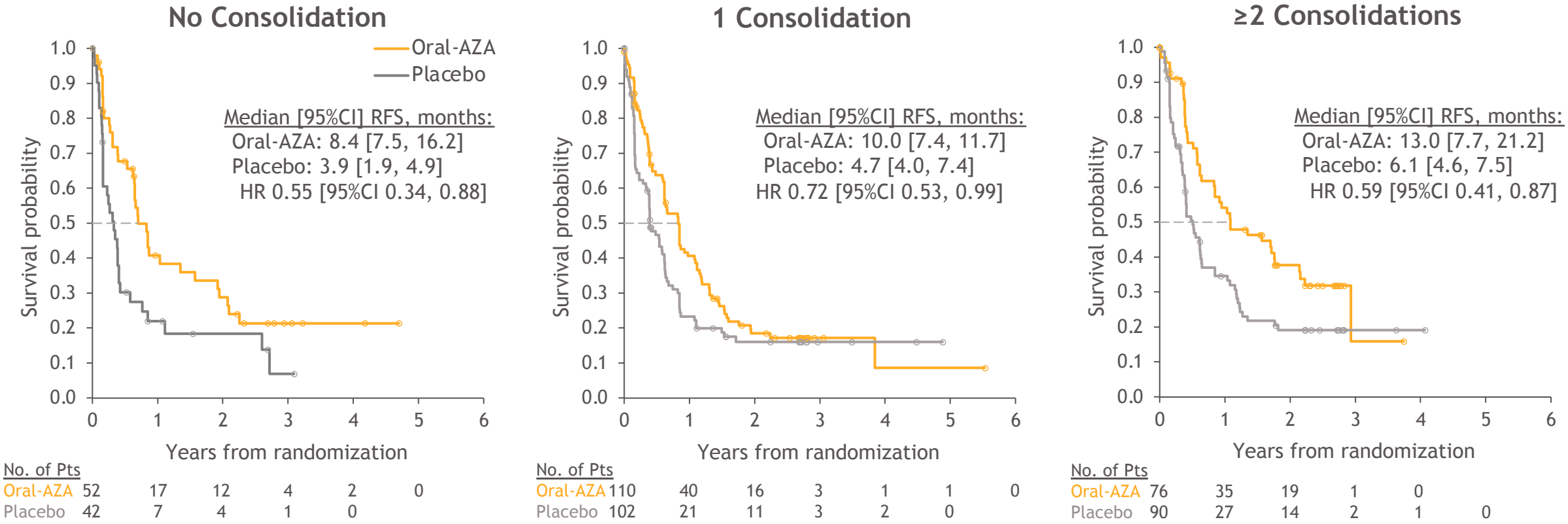
CC-486 Improves Overall Survival (OS) and Relapse-Free Survival (RFS) for Patients with Acute Myeloid Leukemia (AML) in First Remission after Intensive Chemotherapy (IC), Regardless of Amount of Consolidation Received: Results from the Phase III QUAZAR AML-001 Maintenance Trial

Wei AH et al.

ASH 2020;Abstract 1036.

Relapse-free survival by number of consolidations

- Within each consolidation-based cohort, RFS was significantly prolonged with Oral-AZA vs placebo
- Median RFS ranged from 8.4–13.0 mo in the Oral-AZA arm and 3.9–6.1 mo in the placebo arm



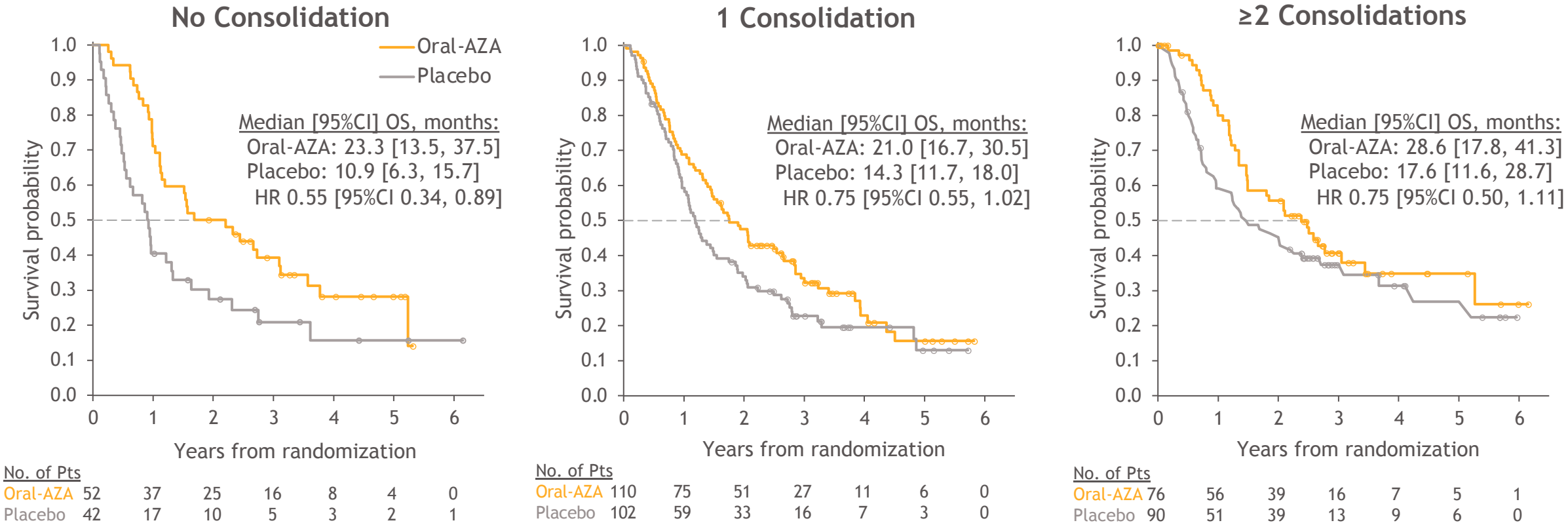
RFS estimates were derived using Kaplan-Meier methods and compared for Oral-AZA vs. placebo using log-rank test. Hazard ratios (HRs) and 95% CIs were generated using a stratified Cox proportional hazards model.

95%CI, 95% confidence interval; AZA, azacitidine; HR, hazard ratio; No., number; pts, patients; RFS, relapse-free survival.

Wei AH et al. ASH 2020;Abstract 1036.

Overall survival by number of consolidations

- OS was also prolonged with Oral-AZA within each consolidation cohort
- Median OS ranged from 21.0–28.6 months in the Oral-AZA arm and 10.9–17.6 months in the placebo arm



OS estimates were derived using Kaplan-Meier methods and compared for Oral-AZA vs. placebo using log-rank test. Hazard ratios (HRs) and 95% CIs were generated using a stratified Cox proportional hazards model.

95%CI, 95% confidence interval; AZA, azacitidine; HR, hazard ratio; No., number; OS, overall survival; pts, patients.

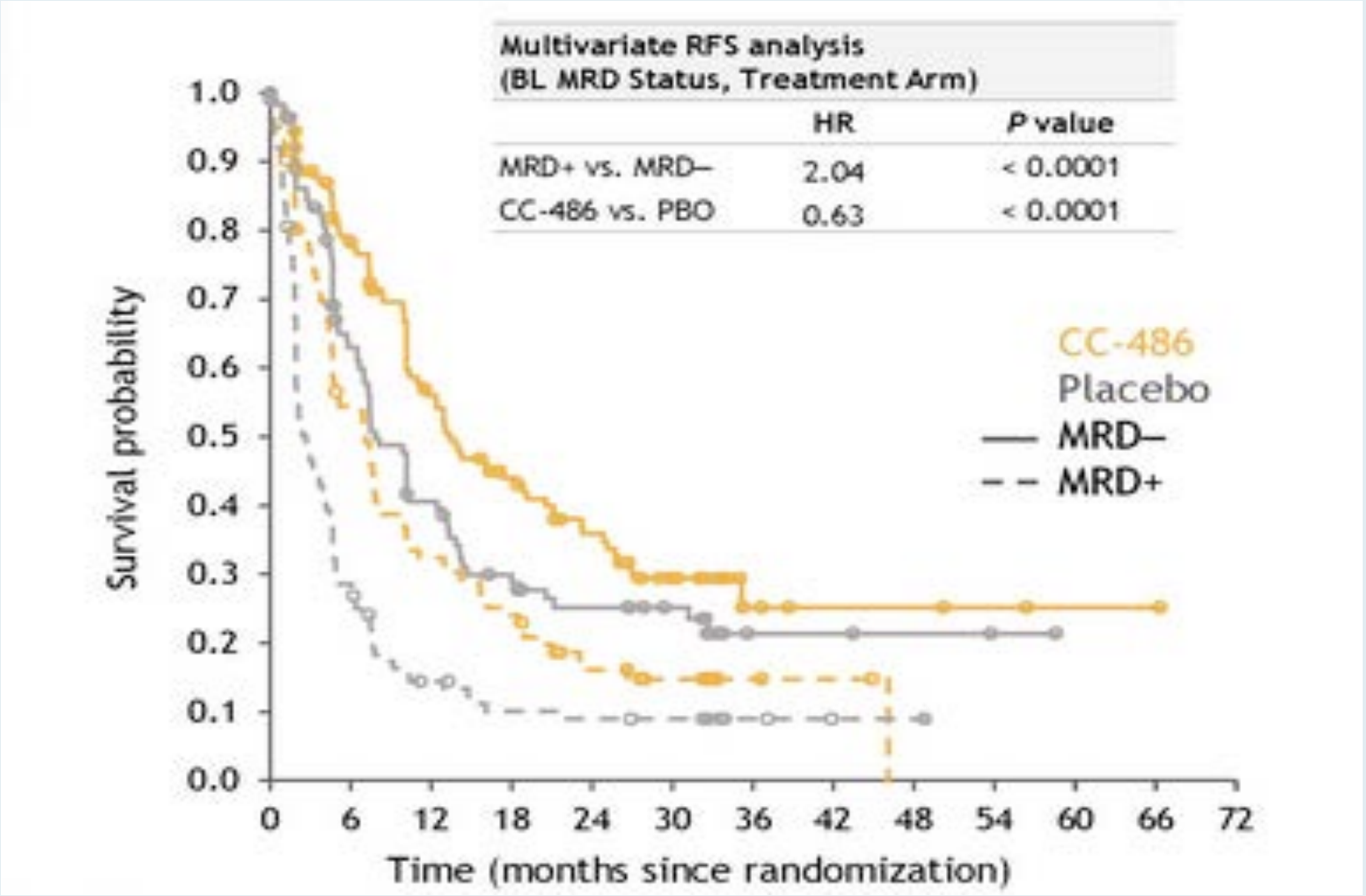
Wei AH et al. ASH 2020;Abstract 1036.

CC-486 Prolongs Survival for Patients with Acute Myeloid Leukemia (AML) in Remission After Intensive Chemotherapy (IC) Independent of the Presence of Measurable Residual Disease (MRD) at Study Entry: Results from the QUAZAR AML-001 Maintenance Trial

Roboz GJ et al.

ASH 2020;Abstract 692.

QUAZAR AML-001: Relapse-Free Survival by Baseline MRD



Roboz GJ et al. ASH 2020;Abstract 692.

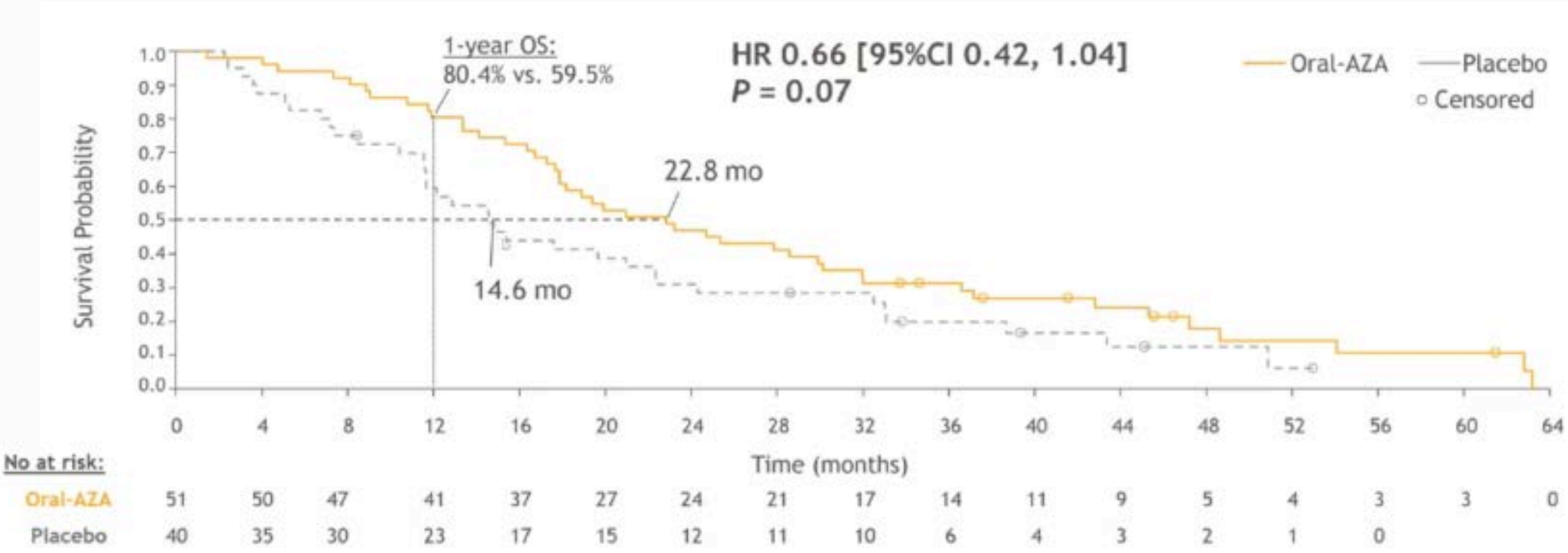
RTP RESEARCH TO PRACTICE

Escalated Dosing Schedules of CC-486 Are Effective and Well Tolerated for Patients Experiencing First Acute Myeloid Leukemia (AML) Relapse: Results from the Phase III QUAZAR AML-001 Maintenance Trial

Dohner H et al.

ASH 2020;Abstract 111.

Escalated dosing cohort: Overall survival



No at risk:																	
Oral-AZA	51	50	47	41	37	27	24	21	17	14	11	9	5	4	3	3	0
Placebo	40	35	30	23	17	15	12	11	10	6	4	3	2	1	0		

Overall survival estimated using Kaplan-Meier methods. The hazard ratio (HR) and 95% confidence intervals comparing Oral-AZA vs. placebo are from a Cox proportional hazards model, and the *P* value is from an unstratified log-rank test.

95%CI, 95% confidence interval; AZA, azacitidine; HR, hazard ratio; mo, months; OS, overall survival; No., number.

Dohner H et al. ASH 2020;Abstract 111.

Health-Related Quality of Life with CC-486 in Patients with Acute Myeloid Leukemia (AML) in First Remission Following Induction Chemotherapy (IC): Results from the Phase III QUAZAR AML-001 Maintenance Trial

Roboz GJ et al.

ASH 2020;Abstract 214.

A 65-year-old with intermediate-risk AML, no actionable mutations and a PS of 0 receives standard 7 + 3 induction. He achieves a complete remission after 2 cycles of induction and then receives 2 cycles of high-dose cytarabine as consolidation but ultimately declines transplant. Would you offer this patient maintenance therapy?

1. Yes
2. Yes, with oral azacitidine (CC-486)
3. No

A 65-year-old patient with intermediate-risk AML, no actionable mutations and a PS of 0 receives standard 7 + 3 induction. He achieves a complete remission after 2 cycles of induction and then receives 2 cycles of high-dose cytarabine as consolidation but ultimately declines transplant. Would you offer this patient maintenance therapy?

 MARK LEVIS, MD	Yes, oral azacitidine (CC-486)	 ANDREW H WEI, MBBS, PHD	Yes, oral azacitidine (CC-486)
 ALEXANDER PERL, MD	Yes, oral azacitidine (CC-486)	 COURTNEY D DINARDO, MD, MSCE	Yes, oral azacitidine (CC-486)
 DANIEL A POLLYEA, MD, MS	Yes, oral azacitidine (CC-486)	 ALICE S MIMS, MD	Yes, oral azacitidine (CC-486)
 EYTAN M STEIN, MD	Yes, oral azacitidine (CC-486)	 EUNICE S WANG, MD	Yes, oral azacitidine (CC-486)
GENERAL MEDICAL ONCOLOGISTS (N = 75)	No		

Agenda

Module 1: Venetoclax combinations — Azacitidine, decitabine, LDAC, pracinostat

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Module 3: IDH inhibitors — Ivosidenib, enasidenib

Module 4: Oral azacitidine (CC-486)

Module 5: Secondary AML — CPX-351

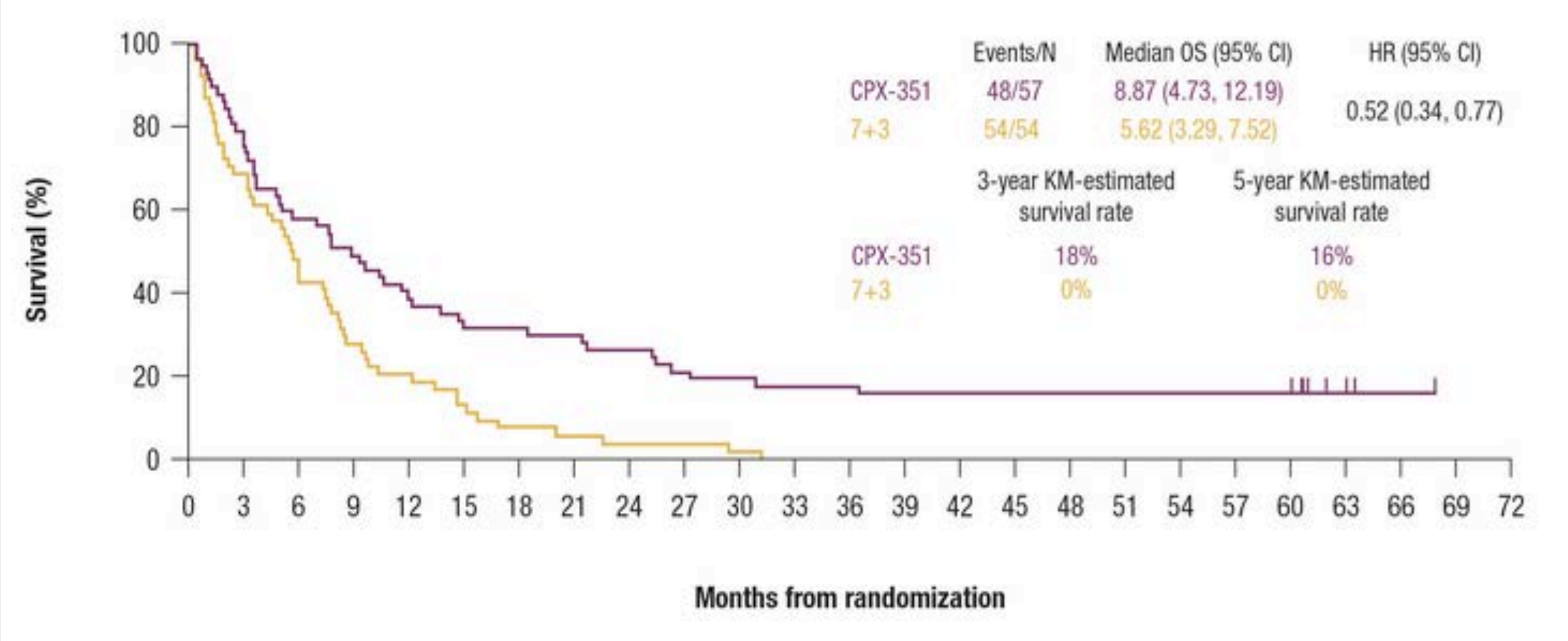
Module 6: Novel agents and strategies — Gemtuzumab ozogamicin, glasdegib, magrolimab

Five-Year Final Results of a Phase 3 Study of CPX-351 versus 7 + 3 in Older Adults with Newly Diagnosed High-Risk/Secondary Acute Myeloid Leukemia (AML): Outcomes by Age Subgroup and Among Responders

Lancet JE et al.

ASH 2020;Abstract 635.

CPX-351 versus 7 + 3: Five-Year Overall Survival for Patients Aged 70 to 75 years



Lancet JE et al. ASH 2020;Abstract 635.

Phase II Study of CPX-351 plus Venetoclax in Patients with Acute Myeloid Leukemia (AML)

Kadia TM et al.

ASH 2020;Abstract 28.

CPX351 + Venetoclax in AML

Responses

Response / Outcome	N	%
Evaluable for Response	18	90
CR	1	6
CRi	6	33
MLFS	1	6
ORR	8	44
Died ≤ 4 weeks	2	10
Died ≤ 8 weeks	4	20
Median # of cycles given [Range]	1 [1 – 2]	
Median # of cycles to response	1 [1 – 2]	
No. of Responding Pts Receiving SCT	7	88
Median time to count recover (days)	41 [23 – 60]	

Kadia TM et al. ASH 2020;Abstract 28.

V-FAST: A Phase 1b Master Trial to Investigate CPX-351 Combined with Various Targeted Agents in Patients with Previously Untreated Acute Myeloid Leukemia

Lin T et al.

ASH 2020;Abstract 1025.

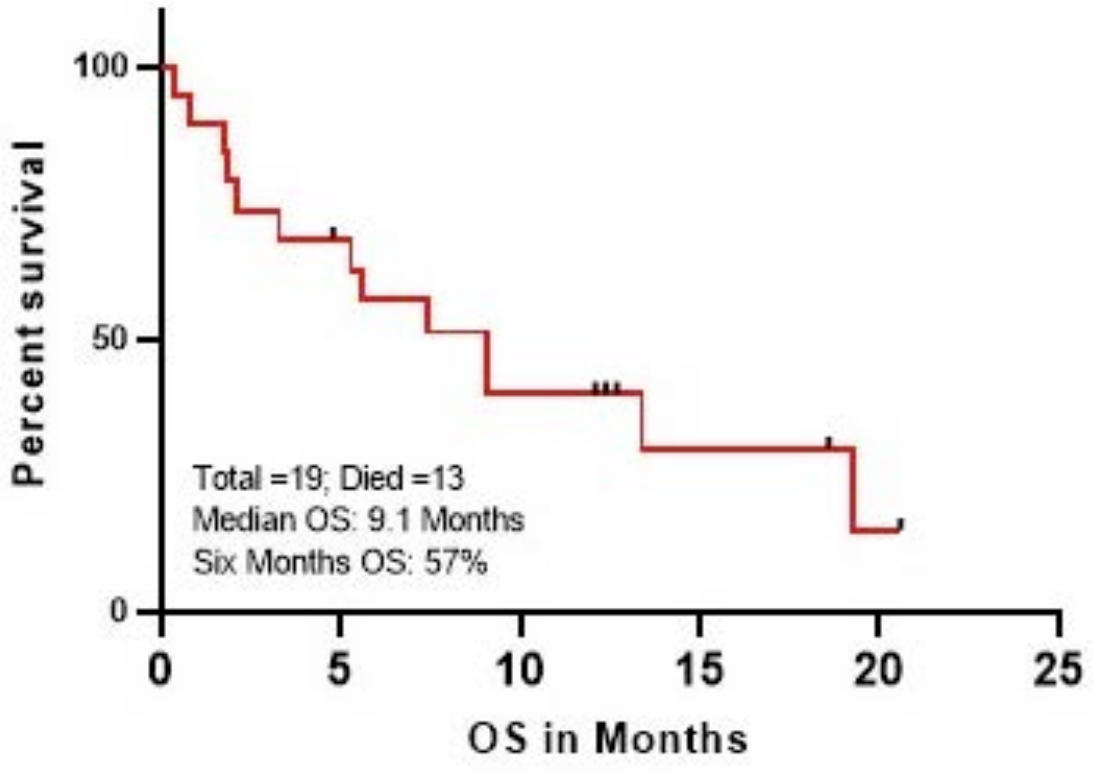
Liposomal Cytarabine and Daunorubicin (CPX-351) in Combination with Gemtuzumab Ozogamicin (GO) in Relapsed Refractory (R/R) Patients with Acute Myeloid Leukemia (AML) and Post-Hypomethylating Agent (Post-HMA) Failure High-Risk Myelodysplastic Syndrome (HR-MDS)

Ramos Perez JM et al.

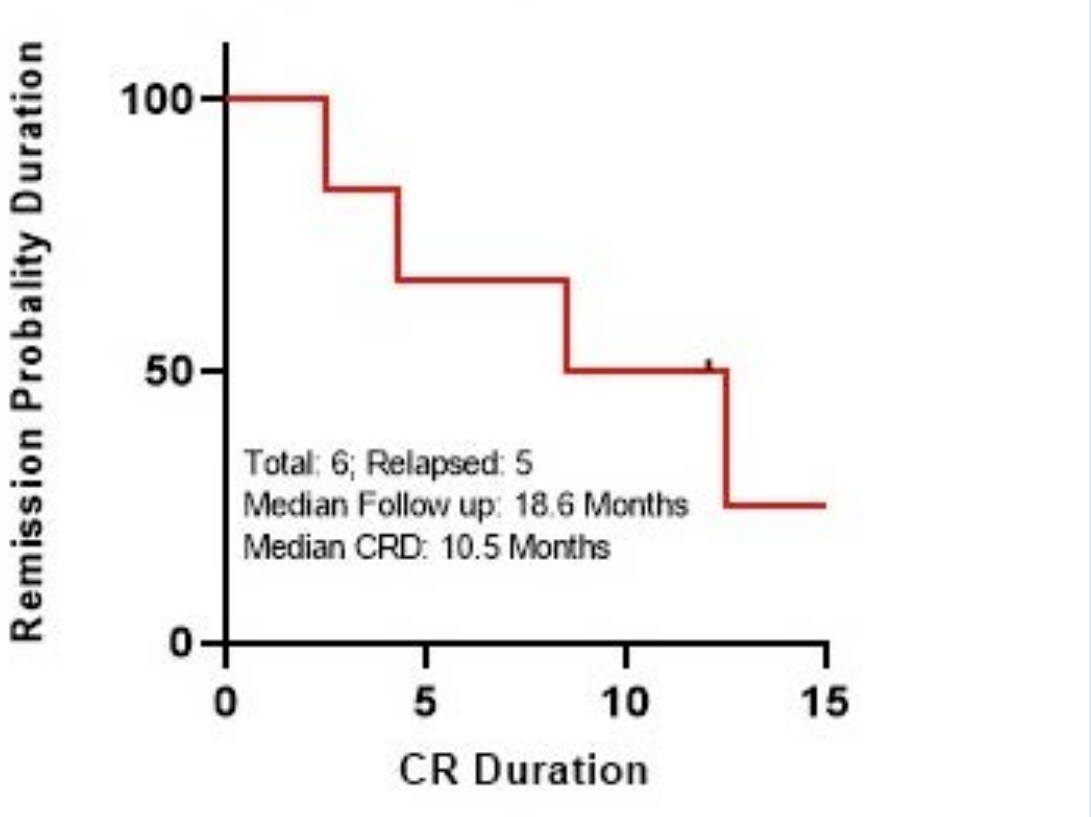
ASH 2020;Abstract 987.

CPX-351 in Combination with Gemtuzumab Ozogamicin in Relapsed/Refractory AML

Overall Survival



Complete Remission Duration



Ramos Perez JM et al. ASH 2020;Abstract 987.

RTP RESEARCH TO PRACTICE

Routine Laboratory Values Can Predict Therapy-Related Myeloid Neoplasms in Patients with New Cytopenias After Treatment for Breast Cancer

Petrone G et al.

ASH 2020;Abstract 1918.

A 65-year-old patient with a history of myelodysplastic syndrome treated with azacitidine for 10 months presents 1 year later with AML with 35% marrow blasts, trisomy 8 and ASXL1, NRAS and U2AF1 mutations (VAFs 45, 20 and 45, respectively). What would you recommend?

1. 7 + 3 induction
2. CPX-351
3. Decitabine
4. Decitabine + venetoclax
5. Low-dose cytarabine + venetoclax
6. Low-dose cytarabine + glasdegib
7. Other

A 65-year-old patient with a history of myelodysplastic syndrome treated with azacitidine for 10 months presents 1 year later with AML with 35% marrow blasts, trisomy 8 and ASXL1, NRAS and U2AF1 mutations (VAFs 45, 20 and 45, respectively). What would you recommend?

 MARK LEVIS, MD	CPX-351	 ANDREW H WEI, MBBS, PHD	CPX-351 → SCT
 ALEXANDER PERL, MD	CPX-351	 COURTNEY D DINARDO, MD, MSCE	CPX-351
 DANIEL A POLLYEA, MD, MS	Azacitidine + venetoclax	 ALICE S MIMS, MD	CPX-351
 EYTAN M STEIN, MD	CPX-351	 EUNICE S WANG, MD	CPX-351

GENERAL MEDICAL ONCOLOGISTS (N = 75)	CPX-351

What initial treatment would you recommend for a 64-year-old woman with a history of breast cancer, for which she received adjuvant chemotherapy, who now presents with bone marrow findings consistent with therapy-related AML?

 MARK LEVIS, MD	CPX-351	 ANDREW H WEI, MBBS, PHD	Azacitidine + venetoclax
 ALEXANDER PERL, MD	CPX-351	 COURTNEY D DINARDO, MD, MSCE	CPX-351
 DANIEL A POLLYEA, MD, MS	Azacitidine + venetoclax	 ALICE S MIMS, MD	CPX-351
 EYTAN M STEIN, MD	CPX-351	 EUNICE S WANG, MD	CPX-351

GENERAL MEDICAL ONCOLOGISTS (N = 75)	CPX-351, Azacitidine + venetoclax

Agenda

Module 1: Venetoclax combinations — Azacitidine, decitabine, LDAC, pracinostat

Module 2: FLT3 inhibitors — Midostaurin, gilteritinib, quizartinib

Module 3: IDH inhibitors — Ivosidenib, enasidenib

Module 4: Oral azacitidine (CC-486)

Module 5: Secondary AML — CPX-351

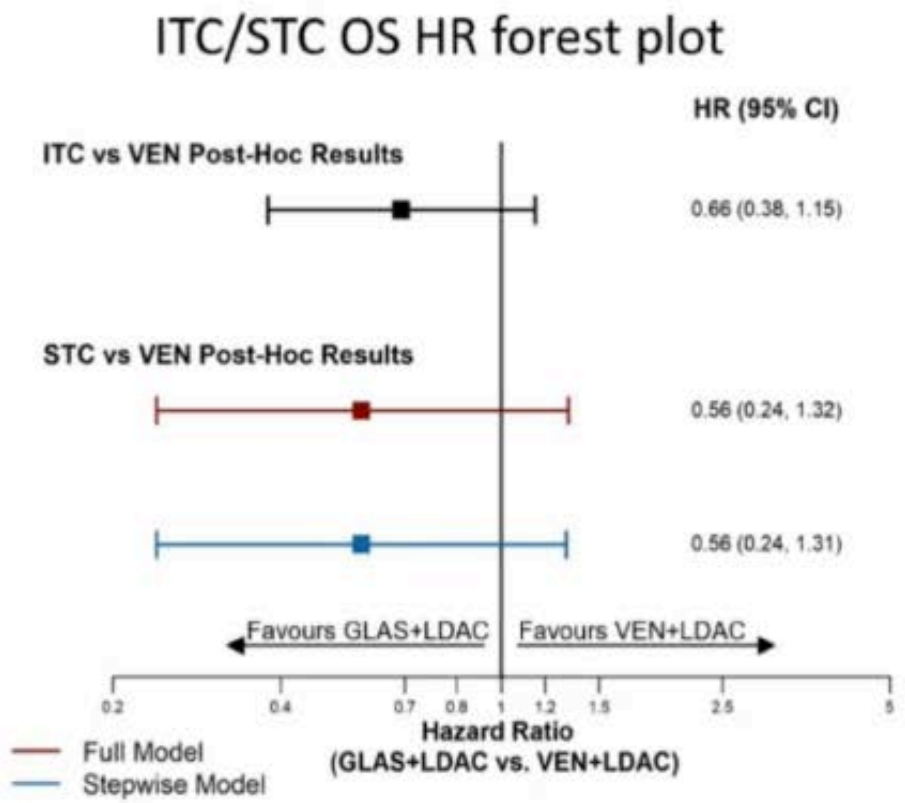
Module 6: Novel agents and strategies — Gemtuzumab ozogamicin, glasdegib, magrolimab

Comparative Effectiveness of Glasdegib or Venetoclax in Combination with Low-Dose Cytarabine Using Simulated Treatment Comparisons

Tremblay G et al.

ASH 2020;Abstract 624.

Treatment comparison results



- The unadjusted ITC and adjusted STC estimated that GLAS+LDAC had lower mortality than VEN+LDAC
  - However, the results were not statistically significant
- Strengthening of the comparative OS advantage is likely because patient population in the glasdegib study had on average a worse prognosis based on more unfavorable baseline characteristics

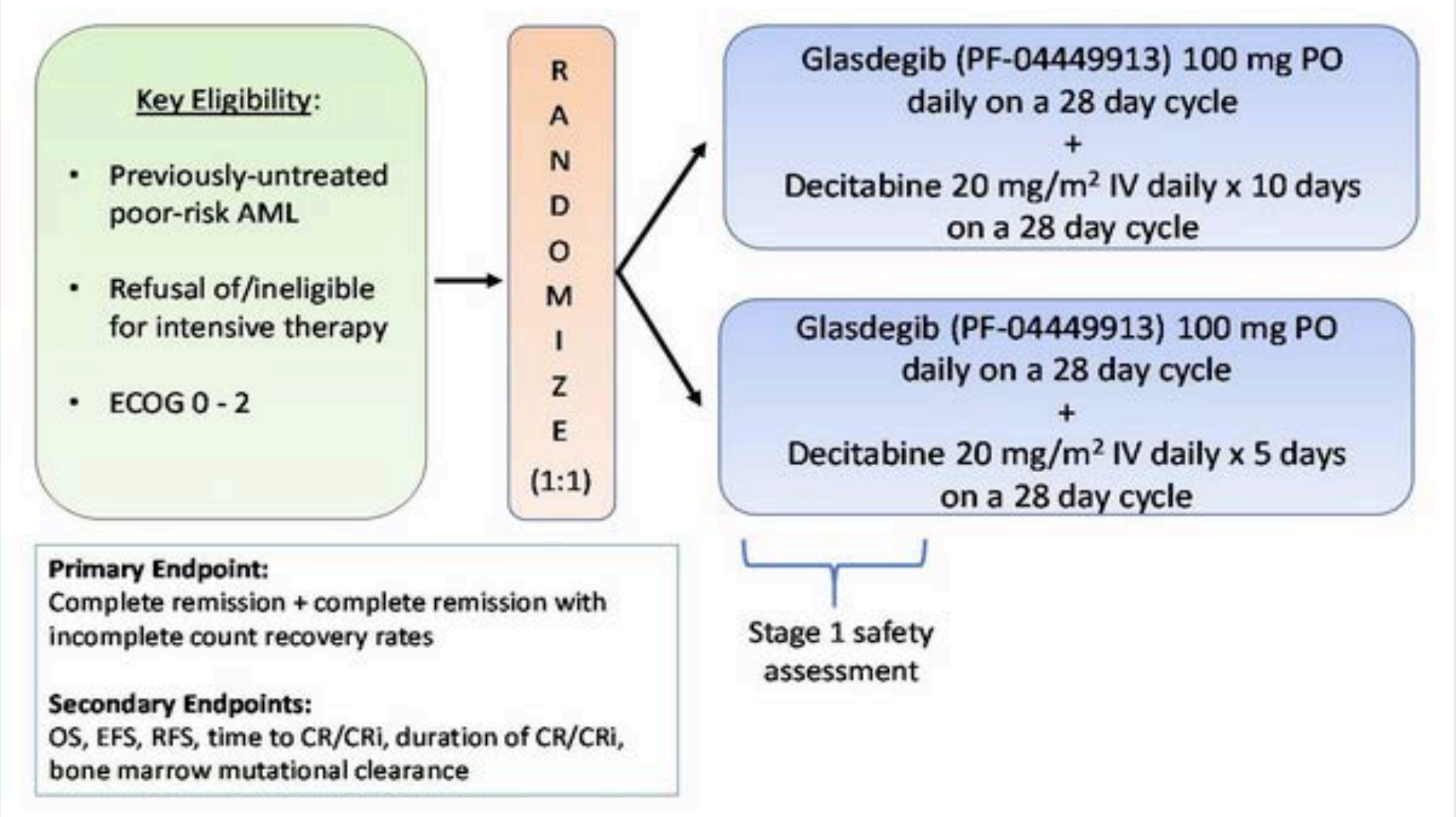
Tremblay G et al. ASH 2020;Abstract 624.

Trial in Progress: Glad-AML – A Randomized, Phase 2 Trial of Glasdegib with Two Standard Decitabine Regimens for Older Patients with Newly-Diagnosed, Poor-Risk Acute Myeloid Leukemia

Shallis R et al.

ASH 2020;Abstract 2825.

GLAD-AML: Glasdegib in Combination with Decitabine



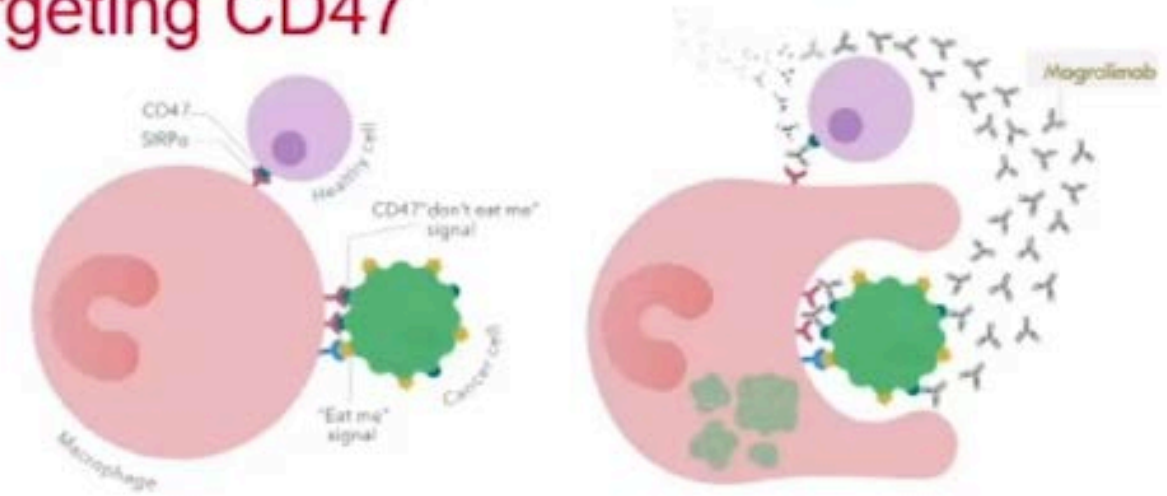
Shallis R et al. ASH 2020;Abstract 2825.

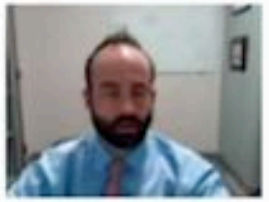
The First-in-Class Anti-CD47 Antibody Magrolimab Combined with Azacitidine Is Well-Tolerated and Effective in AML Patients: Phase 1b Results

Sallman DA et al.

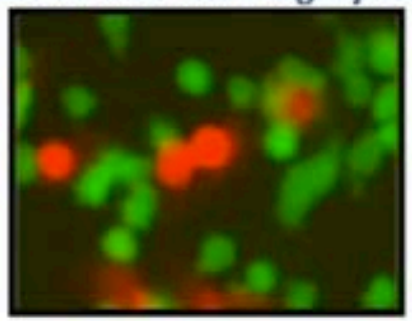
ASH 2020;Abstract 330.

Magrolimab (Formerly 5F9) Is a First-in-Class Macrophage Immune Checkpoint Inhibitor Targeting CD47

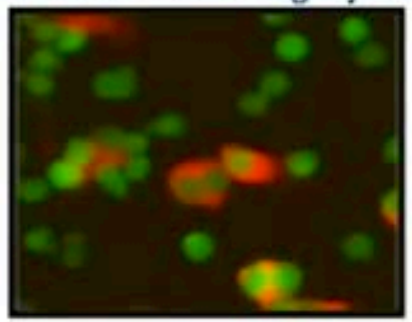




Control mAb: No Phagocytosis



Anti-CD47 mAb: Phagocytosis



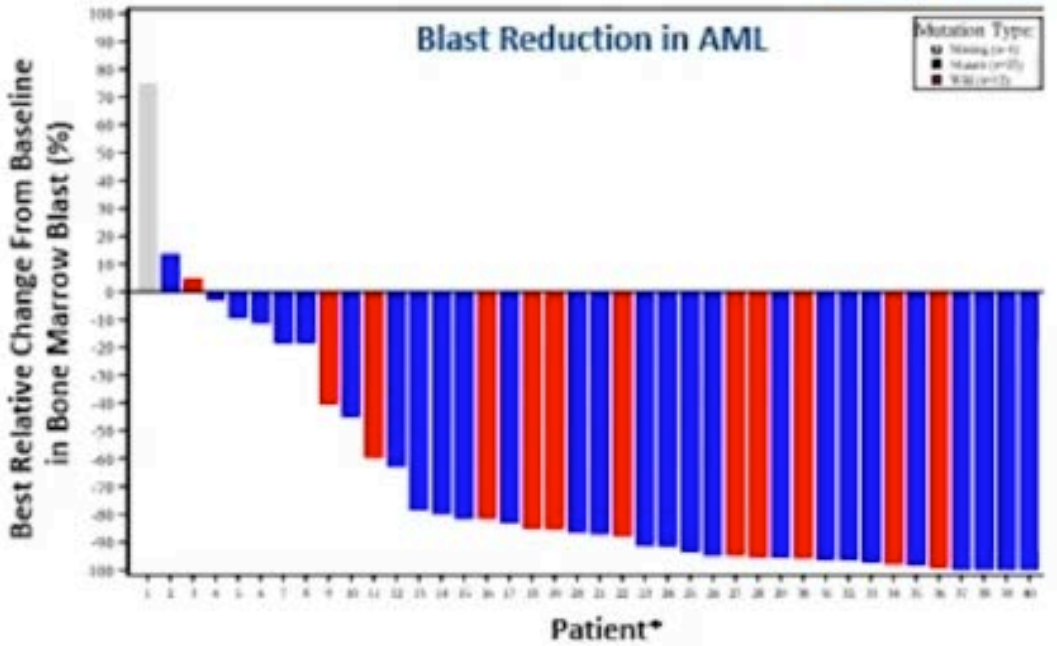
Macrophages
Cancer cells

- CD47 is a "do not eat me" signal that is overexpressed in multiple cancers, including acute myeloid leukemia, leading to macrophage immune evasion
- Magrolimab, an IgG4 anti-CD47 monoclonal antibody (mAb), eliminates tumor cells through macrophage phagocytosis
- Magrolimab is being investigated in multiple cancers with >500 patients dose

Sallman DA et al. ASH 2020;Abstract 330.

Magrolimab + AZA Induces High Response Rates in AML

Best Overall Response	All AML (N=43)	*TP53*-mutant AML (29)
ORR	27 (63%)	20 (69%)
CR	18 (42%)	13 (45%)
CRi	5 (12%)	4 (14%)
PR	1 (2%)	1 (3%)
MLFS	3 (7%)	2 (7%)
SD	14 (33%)	8 (28%)
PD	2 (5%)	1 (3%)



- Magrolimab + AZA induces a 63% ORR and 42% CR rate in AML, including similar responses in *TP53*-mutant patients
- Median time to response is 1.95 months (range 0.95 to 5.6 mo), more rapid than AZA monotherapy
- 9.6% of patients proceeded to bone marrow stem cell transplantation
- Magrolimab + AZA efficacy compares favorably to AZA monotherapy (CR rate 18%–20%)[1,2]

Response assessments per 2017 AML ELN criteria. Patients with at least 1 post-treatment response assessment are shown. *Three patients not shown due to missing values; <5% blasts imputed as 2.5%.

1. Fenaux P, et al. *J Clin Oncol*. 2010;28(4):562-569. 2. Dombret H, et al. *Blood*. 2015;126(3):291-299.

Sallman DA et al. ASH 2020;Abstract 330.

A Phase 1 Study of LY3410738, a First-in-Class Covalent Inhibitor of Mutant IDH in Advanced Myeloid Malignancies (Trial in Progress)

Stein EM et al.

ASH 2020;Abstract 2877.

Flotetuzumab as Salvage Therapy for Primary Induction Failure and Early Relapse Acute Myeloid Leukemia

Aldoss I et al.

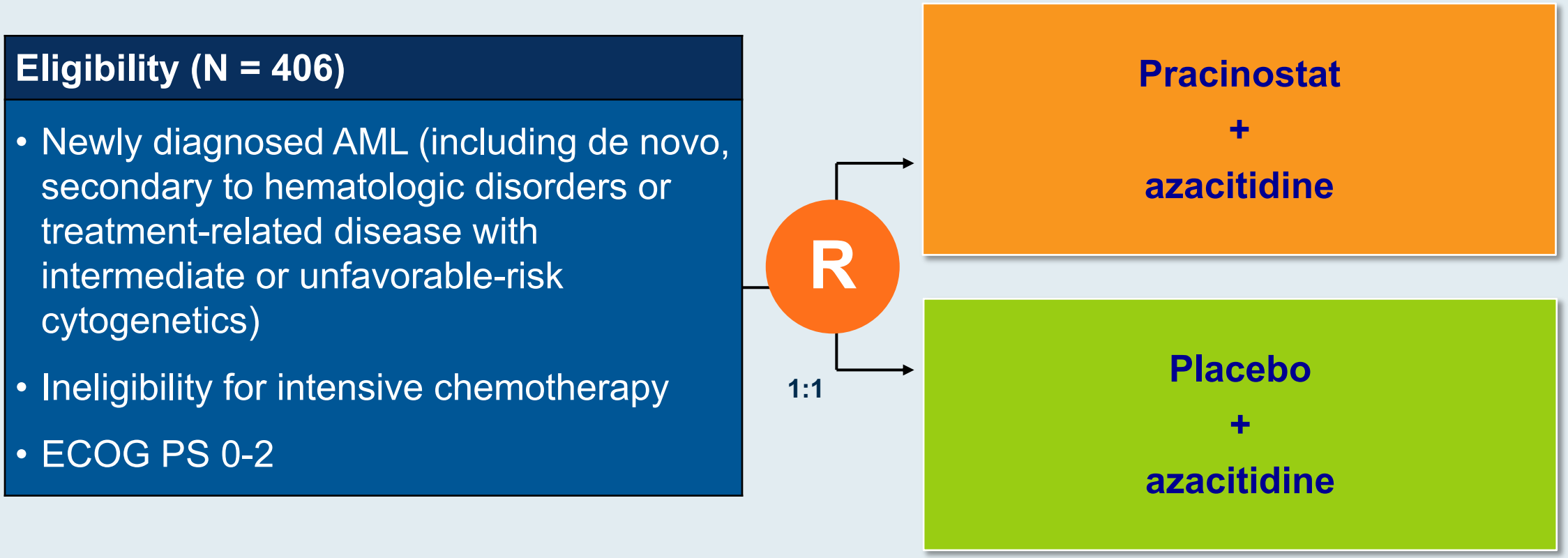
ASH 2020;Abstract 331.

SY-1425, a Potent and Selective RARα Agonist, in Combination with Azacitidine Demonstrates a High Complete Response Rate and a Rapid Onset of Response in RARA-Positive Newly Diagnosed Unfit Acute Myeloid Leukemia

DeBotton S et al.

ASH 2020;Abstract 112.

Phase III PRAN-16-52 Trial Design



Primary endpoint: Overall survival

Secondary endpoints include morphologic CR rate, CR without MRD, cytogenetic CR rate and transfusion independence

www.clinicaltrials.gov (NCT03151408) – Accessed November 2020. Garcia-Manero G et al. ASCO 2018;Abstract TPS7078.

Phase III PRAN-16-52 Trial Discontinued After Completing Interim Analysis
Press Release: July 02, 2020

“An interim futility analysis of the ongoing Phase 3 study of pracinostat in combination with azacitidine in patients with AML who are unfit to receive standard intensive chemotherapy, undertaken by the study Independent Data Monitoring Committee (‘IDMC’), has demonstrated it was unlikely to meet the primary endpoint of overall survival compared to the control group.

Based on the outcome of the interim analysis, the decision was made to discontinue the recruitment of patients and end the study. The decision was based on a lack of efficacy and not on safety concerns.

Pending further evaluation, patients currently enrolled in other pracinostat studies will continue treatment.”

https://www.globenewswire.com/news-release/2020/07/02/2056824/0/en/Helsinn-Group-and-MEI-Pharma-Discontinue-the-Phase-3-Study-with-Pracinostat-in-AML-after-Completing-Interim-Analysis.html

RTP RESEARCH TO PRACTICE

Year in Review — Clinical Investigators Provide Perspectives on the Most Relevant New Publications, Data Sets and Advances in Oncology:

Chronic Lymphocytic Leukemia

Thursday, January 21, 2021
5:00 PM – 6:00 PM ET

Faculty

Matthew S Davids, MD, MMSc
Jennifer Woyach, MD

Moderator

Neil Love, MD

RTP Year in Review 2020

Thank you for joining us!

CME credit information will be emailed to each participant within 3 business days.

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