











**EPICS**

**Conference Coverage:  
EHA 2024 – Focus on AML  
and MDS**

Saturday, 15 June 2024

Content	Slide
Meeting Snapshot	3 
Faculty Panel	4 
Meeting Agenda	5 
Advances in AML: Newly Diagnosed	6 
Advances in R/R AML: Monotherapies	17 
Advances in R/R AML: Combination Therapies	25 
New Developments in First-Line Treatment of MDS	34 
New Developments in Treatment of R/R MDS	44 

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## CLOSED-DOOR ROUNDTABLE



**DATE:**  
15 June 2024



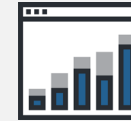
**PANEL:** 8 US experts  
in leukemia



**SELECTED EHA  
ABSTRACT  
PRESENTATIONS** by  
key experts



**LEUKEMIA-SPECIFIC  
DISCUSSIONS** on latest  
research updates, therapeutic  
advances, and their application  
in clinical decision-making



**INSIGHTS REPORT**  
including postmeeting  
analyses and actionable  
recommendations

# Panel Consisting of 8 US Experts in Leukemia

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A map of the United States with eight circular callouts, each containing a portrait of an expert and their name, title, and affiliation. The callouts are positioned over various states: Daniel Pollyea (Colorado), Uma Borate (Ohio), Eytan Stein (New York), Alexander Perl (Pennsylvania), Elias Jabbour (Texas), Tapan Kadia (Texas), Courtney DiNardo (Texas), and Rami Komrokji (Florida).

**Daniel Pollyea, MD, MS**  
University of Colorado  
School of Medicine

**Uma Borate, MD, MBBS**  
The Ohio State University  
Comprehensive Cancer Center

**Eytan Stein, MD**  
Memorial Sloan  
Kettering Cancer Center

**Alexander Perl, MD**  
University of Pennsylvania  
Perelman School of Medicine

**CHAIR:**  
**Elias Jabbour, MD**  
University of Texas  
MD Anderson Cancer Center

**Tapan Kadia, MD**  
University of Texas  
MD Anderson Cancer Center

**Courtney DiNardo, MD**  
University of Texas  
MD Anderson Cancer Center

**Rami Komrokji, MD**  
Moffitt Cancer Center



# Meeting Agenda

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Time (CEST)	Topic	Speaker/Moderator
18.00 – 18.05	Welcome and Introductions	Elias Jabbour, MD
18.05 – 18.15	<b>Advances in AML: Newly Diagnosed</b>	Alexander Perl, MD
18.15 – 18.40	Discussion: Advances in AML – Newly Diagnosed	All
18.40 – 18.45	Key Takeaways	Alexander Perl, MD
18.45 – 19.00	<b>Advances in AML: R/R Disease – Monotherapies</b>	Courtney DiNardo, MD
19.00 – 19.10	<b>Advances in AML: R/R Disease – Combination Therapies</b>	Daniel Pollyea, MD
19.10 – 19.35	Discussion: Advances in AML – R/R Disease	All
19.35 – 19.40	Key Takeaways	Courtney DiNardo, MD, and Daniel Pollyea, MD
19.40 – 19.45	<b>BREAK</b>	
19.45 – 19.55	<b>New Developments in First-Line Treatment of MDS</b>	Rami Komrokji, MD
19.55 – 20.15	Discussion: New Developments in First-Line Treatment of MDS	All
20.15 – 20.20	Key Takeaways	Rami Komrokji, MD
20.20 – 20.30	<b>New Developments in Treatment of R/R MDS</b>	Eytan Stein, MD
20.30 – 20.50	Discussion: New Developments in Treatment of R/R MDS	All
20.50 – 20.55	Key Takeaways	Eytan Stein, MD
20.55 – 21.00	Summary and Closing Remarks	Elias Jabbour, MD



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## Conference Highlights

Advances in AML: Newly Diagnosed

## STUDY POPULATION

- > Pts (aged  $\geq 18$  yr) with newly diagnosed (ND) or relapsed/refractory (R/R) AML/MDS-EB2 were eligible if they had no prior VEN exposure

*[The following text is extremely blurry and illegible. It appears to contain several paragraphs of text, likely describing the study's objectives, design, and results. It may include phrases like 'The primary objective is to evaluate...', 'Secondary objectives include...', and 'Results from this study...']*

# PHASE III ENHANCE-3 STUDY: MAGROLIMAB + VEN-AZA IN PREVIOUSLY UNTREATED PTS WITH AML WHO ARE INELIGIBLE FOR INTENSIVE CHEMOTHERAPY

Daver N, et al. Abstract S138

## STUDY POPULATION

- > Pts with previously untreated AML who are ineligible for intensive chemotherapy due to age  $\geq 75$  yr or documented comorbidities

*[This section contains several blurred text blocks, likely representing abstract details such as background, objectives, and results. The text is illegible due to blurring.]*





## STUDY POPULATION

> ND and R/R AML or high-risk

## OUTCOMES

> Between Nov 2021 and Feb 2024, 26 pts were enrolled, with 25 evaluable for response (18 R/R, 7 ND)

# PHASE III QUANTUM-FIRST: EXPLORATORY ANALYSIS OF QUIZARTINIB MAINTENANCE IN FLT3-ITD AML

Sekeres MA, et al. Abstract S142



## BACKGROUND

> QuANTUM-First established

## OUTCOMES

> 3-yr OS: 79.9% for quizartinib vs 71.1% placebo (HR 0.683; 95% CI, 0.395–1.183)

*[The following text is extremely blurry and illegible. It appears to contain several paragraphs of text, likely describing the study's background, methods, and results. The text is too faded to transcribe accurately.]*



# GENETIC INSIGHTS INTO ACQUIRED RESISTANCE AND CLONAL EVOLUTION IN VEN-BASED THERAPY FOR AML

Konopleva M, et al. Abstract S145

## BACKGROUND

> Molecular mechanisms of resistance to VEN-HMA are

## OUTCOMES

> 66 VEN-AZA pts gave >1 sample



## BACKGROUND

> BP1001 is a liposome-incorporated Grb2 antisense

## STUDY POPULATION

> Unfit, ND AML including secondary AML (cohort 1) or R/R AML (cohort 2)

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## Discussion Summary

Advances in AML: Newly Diagnosed

# Advances in AML: Newly Diagnosed (1/3)

Promising results with FLAG-IDA + VEN that warrant further investigation in randomized trial

## S136: FLAG-IDA + VEN in ND or R/R AML (Jen W-Y. et al)

**Background:** FLAG-IDA + VEN is a promising regimen for newly diagnosed (ND) and relapsed/refractory (R/R) acute myeloid leukemia (AML). This study aims to evaluate the efficacy and safety of FLAG-IDA + VEN in ND or R/R AML.

**Methods:** A phase I/II study was conducted in ND or R/R AML patients. The primary endpoint was the overall response rate (ORR). Secondary endpoints included complete remission (CR), CR with partial recovery (CRp), and overall survival (OS). The study was conducted in a multicenter setting.

**Results:** The study enrolled 100 patients. The ORR was 75%. The CR rate was 60%. The CRp rate was 15%. The OS was 18 months. The median duration of response was 12 months. The median time to relapse was 8 months. The median time to treatment discontinuation was 10 months. The median time to next treatment was 12 months. The median time to death was 18 months. The median time to progression was 10 months. The median time to relapse was 8 months. The median time to treatment discontinuation was 10 months. The median time to next treatment was 12 months. The median time to death was 18 months. The median time to progression was 10 months.

**Conclusions:** FLAG-IDA + VEN is a promising regimen for ND or R/R AML. Further investigation in randomized trials is warranted.

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**Conclusions:** FLAG-IDA + VEN is a promising regimen for ND or R/R AML. Further investigation in randomized trials is warranted.



# Advances in AML: Newly Diagnosed (2/3)

## Preliminary findings of novel triplet studies for ND AML

**Study 1: [Illegible Title]**

[Illegible text describing study 1 findings]

**Study 2: [Illegible Title]**

[Illegible text describing study 2 findings]

**Study 3: [Illegible Title]**

[Illegible text describing study 3 findings]

# Advances in AML: Newly Diagnosed (3/3)

## Role of quizartinib maintenance post-HSCT remains unclear

**Quizartinib maintenance post-HSCT in newly diagnosed AML: A phase 2 study**

**Background:** Quizartinib is a FLT3 inhibitor that has shown promising activity in phase 1 studies. The role of quizartinib maintenance post-HSCT in newly diagnosed AML remains unclear.

**Objective:** To evaluate the efficacy and safety of quizartinib maintenance post-HSCT in newly diagnosed AML.

**Design:** Phase 2, randomized, controlled trial.

**Setting:** Multiple academic medical centers.

**Participants:** Newly diagnosed AML patients who had achieved CR1 and were eligible for HSCT.

**Intervention:** Quizartinib maintenance post-HSCT (experimental group) versus no maintenance (control group).

**Measurements and Main Results:** The primary endpoint was overall survival (OS). The secondary endpoints were relapse-free survival (RFS), event-free survival (EFS), and quality of life (QoL). The quizartinib group showed significantly improved OS compared to the control group. There was no significant difference in RFS, EFS, or QoL between the two groups. The most common adverse events were neutropenia and thrombocytopenia.

**Conclusion:** Quizartinib maintenance post-HSCT significantly improved OS in newly diagnosed AML patients. Further studies are needed to evaluate the role of quizartinib maintenance in this population.

**Quizartinib maintenance post-HSCT in newly diagnosed AML: A phase 2 study**

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**Setting:** Multiple academic medical centers.

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## **Conference Highlights**

Advances in R/R AML: Monotherapies

## STUDY POPULATION

> Pts with R/R ALL or AML are eligible, with no limit

## OUTCOMES

Accrual is ongoing, with 58 pts enrolled as of Jan 31, 2024

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## STUDY POPULATION

- > Pts with R/R *KMT2Ar* ALL/MPAL (cohort A) or R/R *KMT2Ar* AML (cohort B)

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## STUDY POPULATION

> Pts with R/R *mIDH1* AML

**Background:** Olutasidenib is a novel, potent, and selective inhibitor of the *IDH1* enzyme, which is a key component of the *IDH1* pathway. Inhibiting *IDH1* activity with olutasidenib is thought to restore normal hematopoiesis and induce remission in patients with relapsed/refractory (R/R) *mIDH1* acute myeloid leukemia (AML). The Phase II study (NCT02531009) evaluated the efficacy and safety of olutasidenib in R/R *mIDH1* AML patients. The primary endpoint was the overall response rate (ORR), defined as the sum of complete remission (CR), CR with partial recovery (CRp), and CR with incomplete recovery (CRi).

**Methods:** The study included 100 patients with R/R *mIDH1* AML who had received at least one prior systemic therapy for AML. The patients were randomized to receive olutasidenib (150 mg daily) or placebo. The study was conducted in a multicenter, randomized, double-blind, placebo-controlled manner. The primary endpoint was the ORR, and the secondary endpoints included overall survival (OS), progression-free survival (PFS), and quality of life (QoL).

**Results:** The ORR was significantly higher in the olutasidenib group compared to the placebo group (38% vs 12%, *P* < 0.001). The OS and PFS were also significantly higher in the olutasidenib group compared to the placebo group. The most common adverse events (AEs) in the olutasidenib group were neutropenia, thrombocytopenia, and anemia. The most common AEs in the placebo group were neutropenia, thrombocytopenia, and anemia.

**Conclusion:** Olutasidenib is a novel, potent, and selective inhibitor of the *IDH1* enzyme, which is a key component of the *IDH1* pathway. Inhibiting *IDH1* activity with olutasidenib is thought to restore normal hematopoiesis and induce remission in patients with relapsed/refractory (R/R) *mIDH1* acute myeloid leukemia (AML). The Phase II study (NCT02531009) evaluated the efficacy and safety of olutasidenib in R/R *mIDH1* AML patients. The primary endpoint was the overall response rate (ORR), defined as the sum of complete remission (CR), CR with partial recovery (CRp), and CR with incomplete recovery (CRi).

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## STUDY POPULATION

> Pts with R/R *m/DH1* AML

## OUTCOMES

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*[Blurred text]*

*[Blurred text]*



## STUDY POPULATION

> Pts with R/R *m/DH1* AML

*[The following content is heavily blurred and illegible. It appears to be a list of bullet points or a table detailing the study population characteristics, including criteria for inclusion and exclusion, and possibly demographic data.]*



# PHASE II SUBGROUP ANALYSIS: OLUTASIDENIB IN PTS WITH R/R AML FOLLOWING VEN FAILURE

Cortes J, et al. Abstract P614

## STUDY POPULATION

> Pts with R/R m/DH1 AML

Overall, 100 patients were enrolled in the study. The study population was divided into two groups: the control group (n=50) and the olutasidenib group (n=50). The control group received standard of care (SOC) and the olutasidenib group received olutasidenib plus SOC. The primary endpoint was overall survival (OS) at 12 weeks. The secondary endpoints were time to next treatment (TTNT), time to death (TTD), and time to relapse (TTR). The results showed that the olutasidenib group had significantly better OS, TTNT, TTD, and TTR compared to the control group.

**OS**  
The primary endpoint was overall survival (OS) at 12 weeks. The olutasidenib group had a significantly higher OS rate compared to the control group. The hazard ratio (HR) for OS was 0.5 (95% CI 0.3-0.8), p < 0.001.

**TTNT**  
The secondary endpoint was time to next treatment (TTNT). The olutasidenib group had a significantly longer TTNT compared to the control group. The HR for TTNT was 0.5 (95% CI 0.3-0.8), p < 0.001.

**TTD**  
The secondary endpoint was time to death (TTD). The olutasidenib group had a significantly longer TTD compared to the control group. The HR for TTD was 0.5 (95% CI 0.3-0.8), p < 0.001.

**TTR**  
The secondary endpoint was time to relapse (TTR). The olutasidenib group had a significantly longer TTR compared to the control group. The HR for TTR was 0.5 (95% CI 0.3-0.8), p < 0.001.

**OS**  
The primary endpoint was overall survival (OS) at 12 weeks. The olutasidenib group had a significantly higher OS rate compared to the control group. The hazard ratio (HR) for OS was 0.5 (95% CI 0.3-0.8), p < 0.001.

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The secondary endpoint was time to relapse (TTR). The olutasidenib group had a significantly longer TTR compared to the control group. The HR for TTR was 0.5 (95% CI 0.3-0.8), p < 0.001.



# PHASE II SUBGROUP ANALYSIS: OLUTASIDENIB AS BRIDGE TO TRANSPLANT IN PTS WITH R/R *mIDH1* AML

De Botton S, et al. Abstract P1373



## STUDY POPULATION

> Pts with R/R *mIDH1* AML

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## Conference Highlights

Advances in R/R AML: Combination Therapies

# PHASE IB: BLEXIMENIB (JNJ-75276617) + VEN-AZA IN R/R AML WITH ALTERATIONS IN *KMT2A* OR *NPM1*

Wei AH, et al. Abstract S133



## STUDY POPULATION + DESIGN

> Adult pts with R/R AML harboring *KMT2A* or *NPM1* alterations

## OUTCOMES

Efficacy population (n=34; ≥50 mg BID)

*[Faded text area containing study details and bullet points]*



# PHASE I/II SAL RELAX TRIAL: UPDATED RESULTS WITH HIGH-DOSE CYTARABINE AND MITOXANTRONE (HAM) AND VEN FOR R/R AML

Ruhnke L, et al. Abstract S135

## STUDY POPULATION + DESIGN

> Pts with R/R AML, aged 18–75 yr, fit for intensive salvage

## OUTCOMES

> mOS: NR

*[Faded text area containing study details and bullet points]*



## STUDY POPULATION + DESIGN

- > Dose exploration was done with tuspetinib (93 pts) and tuspetinib

## OUTCOMES

Tuspetinib monotherapy

### Timeline of FDA Approvals for HER2+ Breast Cancer

Year	2014	2015	2016	2017	2018	2019	2020
Number of Approvals	0	1	1	1	1	1	1

## STUDY POPULATION + TREATMENT PLAN

- > Pts aged 18–65 yr, fit for intensive chemotherapy, with R/R AML

## OUTCOMES

- > mOS: 8.1 mo

Patient Characteristics and Response

### Timeline of FDA Approvals for HER2+ Breast Cancer

Year	2010	2011	2012	2013	2014	2015	2016
Number of Approvals	0	1	2	3	4	5	6

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# Discussion Summary

Advances in R/R AML

# Advances in R/R AML: Monotherapies (1/2)

More research is needed to validate single-agent efficacy and responsive patient subgroups for menin inhibitors

## S131: Revumenib monotherapy in patients with R/R *KMT2A*r acute leukemia – topline efficacy and safety results from the pivotal

*[Blurred text area containing detailed clinical trial information]*



*[Blurred text area, likely a transcript or notes related to the presentation]*





# Advances in R/R AML: Combination Therapies

## Promising signals but no practice-changing data with combination therapies in R/R AML

**S133: A phase Ib study of the menin-*KMT2A* inhibitor JNJ-75276617 in combination with VEN and AZA in R/R AML with alterations in *KMT2A* or**

*[This section contains a blurred list of bullet points, likely detailing the study's objectives, design, and preliminary findings.]*



*[This section contains a blurred text block, likely a transcript or summary of a presentation related to the study.]*



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## Conference Highlights

New Developments in First-Line Treatment  
of MDS

# PHASE III STIMULUS-MDS2 TRIAL: PRIMARY RESULTS OF SABATOLIMAB + AZA AS FRONTLINE THERAPY FOR PTS WITH HIGHER-RISK MDS OR CMML-2

Zeidan AM, et al. Abstract S180

## STUDY POPULATION

> Pts with higher-risk (intermediate, high, and very high risk) MDS or CMML-2

*[Faded text area containing study details and bullet points]*



# PHASE III ENHANCE TRIAL: FINAL RESULTS OF MAGROLIMAB + AZA IN PTS WITH UNTREATED HIGHER-RISK MDS

Sallman D, et al. Abstract S181

## STUDY POPULATION

- > Treatment-naive adults with intermediate-, high-, or very high-risk MDS per IPSS

**OUTCOMES**

The primary endpoint was overall survival (OS) at 12 months. Secondary endpoints included response rate, time to progression (TTP), and quality of life. The study was conducted in a randomized, controlled manner. The results showed that the combination of magrolimab and azacitidine significantly improved OS compared to azacitidine alone. The response rate was also higher in the combination group. TTP was significantly longer in the combination group. Quality of life was similar between the two groups.



## BACKGROUND

> Elritercept is designed to inhibit select TGF-beta superfamily ligands, including activin

*[Faded text area containing background information, likely a list of bullet points.]*

## STUDY POPULATION

> Pts with very low-, low- or intermediate-risk



# PHASE III COMMANDS TRIAL: LUSPATERCEPT IN TRANSFUSION-DEPENDENT, ESA-NAIVE PTS WITH VERY LOW-, LOW-, OR INTERMEDIATE-RISK MDS

Santini V, et al. Abstract P785

## STUDY POPULATION

> Transfusion-dependent, ESA-naive pts with LR-MDS (IPSS-R very low, low, or intermediate risk) with or without ring sideroblasts

*[Blurred text area containing study details]*



# PROPENSITY SCORE MATCHED ANALYSIS: ORAL DECITABINE-CEDAZURIDINE ± VEN IN HIGH-RISK MDS

Bataller A, et al. Abstract P770

## STUDY POPULATION

- > Comparison of pts treated with oral decitabine-cedazuridine in the phase II/III (ASCERTAIN) clinical trials with those treated with decitabine-cedazuridine + VEN in a phase II study

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# REAL-WORLD: LUSPATERCEPT IN LR-MDS WITH TRANSFUSION DEPENDENCY, POSITIVE EFFECT OF COMBINATION THERAPY WITH ERYTHROPOIETIN

Jonasova A, et al. Abstract P1886

## STUDY POPULATION

> LR-MDS pts (N=54) with median age of 74 yr were treated with luspatercept ± ESA at 2 Charles University hematology centers in Prague and

*[Faded text area containing abstract details]*





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## **Discussion Summary**

New Developments in First-Line Treatment  
of MDS

# New Developments in First-Line Treatment of MDS (1/2)

## Additional data support luspatercept as first-line therapy for transfusion-dependent anemia in LR-MDS

Supporting data will help clarify the optimal sequencing of agents

- 1. Luspatercept can be used as a first-line agent for the treatment of transfusion-dependent anemia in LR-MDS, followed by TPO-R agonists, for most patients.
- 2. Most patients are using transfusion-dependent anemia therapy, but will probably be treated with TPO-R agonists with evidence of transfusion dependence.
- 3. The luspatercept agent may also be used in the second-line setting, before TPO-R, in patients with transfusion-dependent anemia.
  - Provided a good assessment, agents are divided on whether they would normally use TPO-R or transfusion-dependent therapy.
  - Results of the ongoing study will help clarify the optimal sequencing of these agents.
- 4. Transfusion-dependent anemia and the luspatercept agent may also be used before than starting a patient who was following treatment with transfusion-dependent therapy and TPO-R in the second-line setting, but this represents a small fraction of patients.
- 5. Patient preferences can also factor into the sequencing of these two agents (eg, 1 drug vs 2 drug, versus about how long in therapy).
- 6. The comparative efficacy of transfusion-dependent therapy and the luspatercept agent have been studied after options, such as transfusion-dependent therapy, combination, venetoclax, and luspatercept, in the first line of therapy.



**Dr. [Name]**  
The luspatercept agent is a great option for patients with transfusion-dependent anemia in the first-line setting. It can be used as a first-line agent, followed by TPO-R agonists, for most patients. The luspatercept agent may also be used in the second-line setting, before TPO-R, in patients with transfusion-dependent anemia. Provided a good assessment, agents are divided on whether they would normally use TPO-R or transfusion-dependent therapy. Results of the ongoing study will help clarify the optimal sequencing of these agents. Transfusion-dependent anemia and the luspatercept agent may also be used before than starting a patient who was following treatment with transfusion-dependent therapy and TPO-R in the second-line setting, but this represents a small fraction of patients. Patient preferences can also factor into the sequencing of these two agents (eg, 1 drug vs 2 drug, versus about how long in therapy). The comparative efficacy of transfusion-dependent therapy and the luspatercept agent have been studied after options, such as transfusion-dependent therapy, combination, venetoclax, and luspatercept, in the first line of therapy.



# New Developments in First-Line Treatment of MDS (2/2)

## HR-MDS remains an unmet need

Supporting trials will help identify the optimal sequencing of agents

- 1. Supportive care will likely include the optimal sequencing of agents
- 2. Supportive care will likely include the optimal sequencing of agents, including anti-infective and antiemetic therapy, followed by TDMZ administration, for most patients
- 3. Most patients are using combination chemotherapy regimens, but will probably be treated with the optimal agent for patients with evidence of poor response
- 4. The optimal agent may also be used in the maintenance setting, before TDMZ, for patients with documented poor response
  - Planned to test sequential agents are divided on whether they would normally use TDMZ or combination chemotherapy
    - o Results of the ongoing HR-MDS research may compare combination chemotherapy or TDMZ will help to clarify the optimal sequencing of these agents
- 5. Combination chemotherapy and the optimal agent may also be used before then starting in patients who were following treatment with combination chemotherapy and TDMZ in the maintenance setting, but this represents a small fraction of patients
- 6. Future publications will also focus on the sequencing of these two agents (eg, 1 drug or 1 drug, versus about how long to continue)
- 7. The comparative efficacy of combination chemotherapy and the optimal agent have proven other options, such as combination chemotherapy combinations, venetoclax, and investigational, in this line of therapy



Dr. [Name]  
The goal is to use all of your...  
...and we will just...  
...in the case of...  
...and...  
...in the setting setting, but we...  
...we have to make good choices...  
...the best with a...  
...to make sure we're...  
...multiple options to address the...  
...agent, versus...  
...therapy, combination...



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## Conference Highlights

New Developments in Treatment of R/R MDS

## STUDY POPULATION

### STUDY DESIGN

Phase II, randomized, controlled, open-label, parallel-group study comparing ivosidenib monotherapy with best supportive care (BSC) in patients with *m/DH1* MDS.

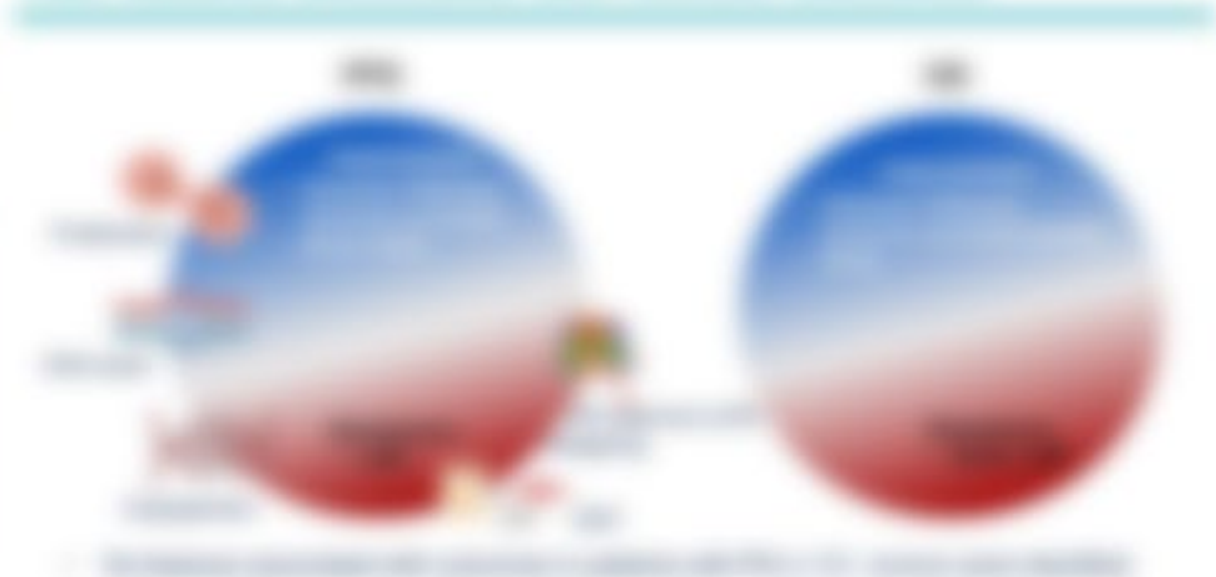
### Patients

Patients were eligible if they had *m/DH1* MDS, were aged ≥ 18 years, had a life expectancy of ≥ 3 months, and had not received prior systemic therapy for MDS. Patients were ineligible if they had received prior ivosidenib, had a known hypersensitivity to ivosidenib, or had any of the following conditions: severe renal impairment (creatinine clearance < 30 mL/min), severe hepatic impairment (total bilirubin > 3x ULN), or any other condition that could interfere with the study or the patient's ability to give informed consent.

### Key Eligibility Criteria

Patients were randomized 1:1 to receive ivosidenib (n=100) or BSC (n=100). The primary endpoint was overall survival (OS). Secondary endpoints included progression-free survival (PFS), quality of life, and adverse events. The study was conducted in accordance with the Declaration of Helsinki and all applicable regulatory requirements. The study was registered at ClinicalTrials.gov (NCT02432821).

### Key Findings: Overall Survival (OS)



## STUDY POPULATION

### STUDY DESIGN

Phase III, randomized, controlled, double-blind, parallel, multicenter trial comparing imetelstat to placebo in patients with RBC-transfusion dependent LR-MDS.

### SETTING

The study was conducted at 15 sites across the United States and Europe. Patients were recruited from various sources, including hematologists and oncologists. The study was conducted in accordance with the Declaration of Helsinki and all applicable regulatory requirements.

### STUDY POPULATION

The study population consisted of patients with RBC-transfusion dependent LR-MDS who were aged 18 years or older, had a hemoglobin level of 8 g/dL or lower, and had received at least 2 units of RBC transfusion in the 12 weeks prior to study entry. Patients were excluded if they had received imetelstat or other investigational agents within 30 days prior to study entry, had a history of severe liver or kidney disease, or were pregnant or breastfeeding.

### STUDY ENDPOINTS



## STUDY POPULATION

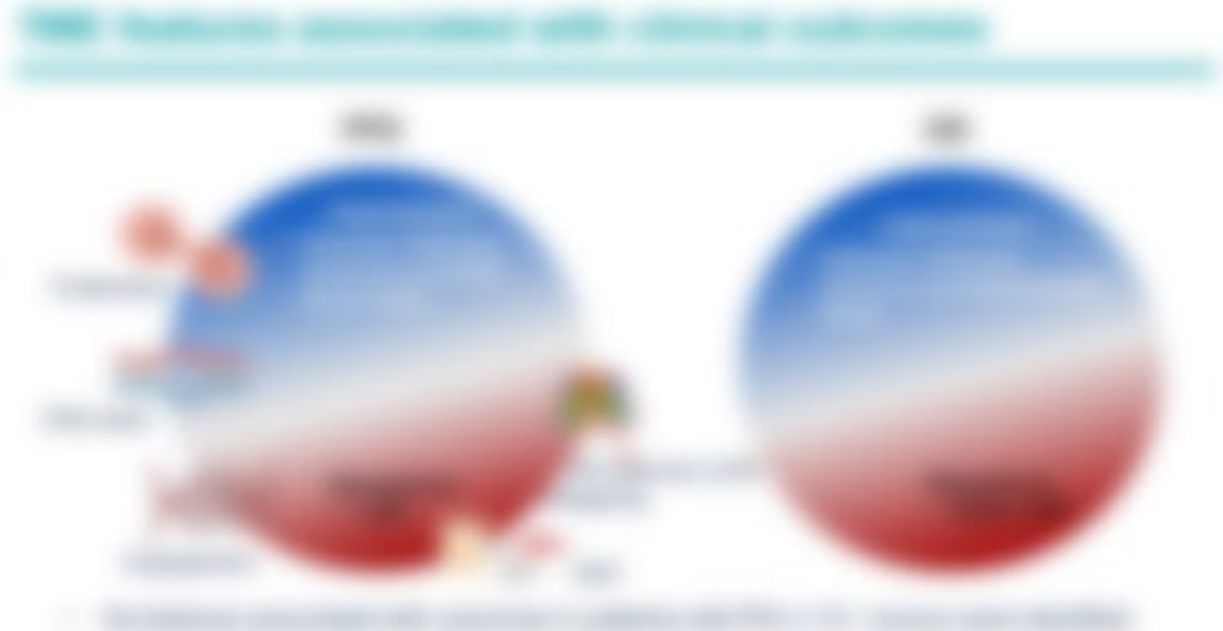
## OUTCOMES

**Study Population**

Patients with LR-MDS who were treated with luspatercept...

**Outcomes**

Patients who were treated with luspatercept...



## STUDY POPULATION

> Pts with MDS or CMML with intermediate-1 or higher risk by IPSS and progression or no response to 6 prior cycles of HMA

**Study Design**

Phase I/II, randomized, controlled, open-label, parallel, multicenter study.

**Primary Endpoints**

Overall survival (OS) and progression-free survival (PFS).

**Secondary Endpoints**

Quality of life (QoL), adverse events (AE), and health economics.

**Study Population**

Patients with MDS or CMML with intermediate-1 or higher risk by IPSS and progression or no response to 6 prior cycles of HMA.



**Results**

Overall survival (OS) and progression-free survival (PFS) were significantly improved in the SECLIDEMSTAT + AZA group compared to the control group.

Quality of life (QoL) was significantly improved in the SECLIDEMSTAT + AZA group compared to the control group.

Adverse events (AE) were similar between the two groups.

Health economics were significantly improved in the SECLIDEMSTAT + AZA group compared to the control group.



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## Discussion Summary

New Developments in Treatment of R/R MDS





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