Acute myeloid leukemia (AML) has a poor prognosis, and is associated
in a preclinical study using an m

Central or local confirmation of m

Mutations in isocitrate dehydrogenase 1 (IDH1) occur in ~6–10% of AML cases.

The active phase 3 AGILE study is currently recruiting in 20 countries, with

Ivosidenib (AG-120) is a first-in-class, oral, potent, targeted, small-molecule

Objective responses were observed in 18 of 23 (78.3%) patients with newly diagnosed AML (CR rate 60.9% and CRh rate 8.7%) support the development

The favorable safety profile and encouraging clinical activity observed in the phase 1b ivosidenib + azacitidine combination study of the treatment of de novo AML (CR rate 50.0% and CRh rate 11.1%) support the development of this combination in the phase 3 AGILE study. The phase 3 AGILE study is currently recruiting in 20 countries, with a total of 172 study centers in North America, South America, Asia, and Europe participating in the study.

Further information is available at https://clinicaltrials.gov/show/NCT03173248.

Contact madeirol@agios.com.

| Study design and methods
| Preclinical rationale for combining ivosidenib and azacitidine

• Patients with antecedent myelodysplasia
• Patients with a history of other myeloid neoplasms
• Patients with antecedent hematologic tumors

• Patients who previously have received an intensive induction chemotherapy
• Patients who are candidates for and willing to receive intensive chemotherapy

• Patients with antecedent hematologic tumors
• Patients with a history of other myeloid neoplasms
• Patients with antecedent myelodysplasia

• Event-free survival
• Overall survival
• Safety
• Transfusion requirements
• Rates of infection and hospitalization
• Quality of life (EORTC QLQ-C30 and EQ-5D-5L)

• Overall survival, event-free survival, response rates
• Secondary

• Overall survival
• Rate of CR+CRh
• Rate of CR

• Eligible patients with untreated AML

Table 1. Phase 1b study: response rates

Table 2. Phase 1b study: IDH mutation clearance rate by best overall response (BEAMing digital PCR)

Figure 1. Phase 1b study: treatment duration and best overall response

Figure 2. AGILE study design

SUMMARY AND CURRENT STATUS

• The global phase 3 AGILE study is currently recruiting in 20 countries, with a total of 172 study centers in North America, South America, Asia, and Europe participating in the study. Further information is available at https://clinicaltrials.gov/show/NCT03173248.

| Key inclusion criteria
| Key exclusion criteria

| Phase 3 AGILE STUDY DESIGN

• AML is a global, phase 3, multicenter, randomized, double-blind, placebo-controlled study of ivosidenib in combination with azacitidine in adult patients with previously untreated AML who do not have candidates for intensive treatment.

- Preclinical rationale for combining ivosidenib and azacitidine
- Background
- Methods
- Results
- Conclusions
- Acknowledgments
- Disclosures
- References

...Continued 28-day cycles...