Ivosidenib (IVO) prior to hematopoietic cell transplantation for patients with IDH1-mutant relapsed or refractory acute myeloid leukemia (R/R AML)

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BACKGROUND

- Allelic hematogentic cell transplantation (HCT) provides a potentially curative option for patients with relapsed or refractory (R/R) acute myeloid leukemia (AML). 
- Pre-HCT remission status is a major determinant of long-term prognosis.2
- Older and/or heavily pretreated patients frequently cannot tolerate intensive salvage chemotherapy to obtain adequate disease control prior to HCT.3

OBJECTIVE

– To assess HCT outcomes in 18 patients with mIDH1 R/R AML who underwent HCT after responding to treatment with IVO in the AG120-C-001 phase 1 study

METHODS

- Here we report outcomes in patients with mIDH1 R/R AML from the phase 1 study who received a starting dose of IVO 500 mg once daily (QD), responded to treatment, and then proceeded to HCT.

RESULTS

- This was a multicenter, open-label, dose-escalation and expansion study enrolling patients 18 years of age with an advanced mIDH1 hematologic malignancy. (ClinicalTrials.gov ID: NCT02164128)
- IVO monotherapy was administered orally, daily, in continuous 28-day cycles (Figure 1)
- During dose-escalation, IVO was administered at doses of 200–1200 mg daily (QD) and 500 mg QD was selected for expansion
- Per protocol patients with R/R AML achieving an adequate response to IVO and meeting other criteria required for transplant could proceed to HCT after discontinuation of IVO

CONCLUSIONS

- In the HCT subgroup:
  - Median (95% CI) OS was 16.8 months (9.0, 21.0) from start of IVO treatment for patients with mIDH1, calculated from the start of IVO treatment compared with 0 months (7.1, 10.2) in the overall AML study arm (Table 2)
  - 6-month OS was 94.4% and 12-month OS was 61.1% (Table 3)