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

Courtney D DiNardo¹, Eytan Stein², Arnaud Pignon², Jessica K Altman³, Robert Collins, Harry P Erba, Justin M Watts, Geoffrey L Uy, Bin Wu, Sung Choe, Stephanie M Kapsalis, Hua Liu, Thomas Winkler, Gail J Roboz, Stéphane de Botton

¹University of Texas MD Anderson Cancer Center, Houston, TX, USA; ²Mayo Clinic, Rochester, MN, USA; ³University of Alabama at Birmingham, Birmingham, AL, USA; ⁴Regional Cancer Institute, University of Miami, Miami, FL, USA; ⁵Washington University School of Medicine, St. Louis, MO, USA; ⁶Tegik Pharmaceutical, Inc., Cambridge, MA, USA; ⁷Weill Cornell Medicine and The New York Presbyterian Hospital, New York, NY, USA; ⁸Institut Gustave-Roussy, Villejuif, France.

Email: medtnr@agps.org

BACKGROUND
- Allogeneic hematopoietic cell transplantation (HCT) provides a potentially curative option for patients with relapsed or refractory (R/R) AML.
- Pre-HCT remission status is a major determinant of long-term prognosis.
- CR (complete remission) is associated with an excellent survival outcome.

METHODS
- This was a single-arm, open-label, phase 1, multicenter trial (ClinicalTrials.gov NCT02074839) that enrolled patients ≥ 18 years of age with R/R AML.
- IVO (ivosidenib) was administered orally, daily, in continuous 28-day cycles (BID = twice daily).
- Duration of follow-up was 33.2 months (3.2–41.9).
- HCT subgroup: Patients with R/R AML who underwent HCT are shown in Figure 3.

RESULTS
- Baseline demographics and disease characteristics are reported in Table 1.
- The median age of patients with R/R AML treated with IVO in the phase 1 study was 60.1 (20.6–79.6) years.
- In the overall cohort of 179 patients with R/R AML treated with IVO in the phase 1 study, 60 (33.4) patients achieved a BOR of CR, and median (95% CI) OS was 6.0 (8–9) months.
- In the HCT subgroup, the best overall response (BOR) or IVO prior to HCT was complete remission (CR) in 35 (18.6) patients, and median (range) time from last IVO dose to HCT was 13.3 (9–24) days.
- The overall cohort of 179 patients with R/R AML treated with IVO in the phase 1 study was 140 (78.1) men and 39 (21.9) women.
- In the overall cohort of 179 patients with R/R AML treated with IVO in the phase 1 study, 8 (4.4) patients achieved a BOR of CR, and median (95% CI) OS was 6.0 (8–9) months.

CONCLUSIONS
- The results of this study suggest that IVO may have a role in the pre-HCT setting for patients with R/R AML and may improve outcomes for patients undergoing HCT.
- Further studies are needed to evaluate the potential benefit of IVO in the pre-HCT setting for patients with R/R AML.