Overall Survival in Patients with Systemic Mastocytosis with Associated Hematologic Neoplasm Treated with Avapritinib versus Best Available Therapy

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Abstract P0103

Objective

To present an updated overall survival analysis from the phase 3, open-label, randomized, controlled BASELINE trial (NCT04695431) comparing avapritinib to best available therapy (BAT) in patients with systemic mastocytosis with an associated hematologic neoplasm (SM-AHN) who were not candidates for stem cell transplantation.

Methods

Inclusion criteria:
- Patients with SM-AHN who were not candidates for stem cell transplantation
- SM-AHN patients with a measurable cytogenetic abnormality
- SM-AHN patients with a WHO performance status of 0-2
- Age ≥18 years

Exclusion criteria:
- Patients with a history of severe adverse events (grade 3 or higher) within the 6 months before study entry
- Patients with a history of grade 3 or higher neutropenia, anemia, or thrombocytopenia
- Patients with a history of grade 3 or higher hydration requirements

Data sources:
- Real-world data from 119 patients with SM-AHN who were treated with avapritinib
- Data from patients treated with BAT in single-arm studies

Statistical analysis:
- Kaplan-Meier estimates were used to estimate the overall survival
- The log-rank test was used to compare the Kaplan-Meier survival curves

Results

Baseline demographics:
- The analysis included 119 patients with SM-AHN who were treated with avapritinib and 83 patients with BAT (Table 1)
- Median age of the avapritinib cohort was 59 (range: 12-84) years and 65 (range: 23-87) years for the BAT cohort
- More patients in the BAT cohort had a history of prior treatments
- Patients in the avapritinib cohort had a higher median serum tryptase level

Overall survival:
- The analysis compared overall survival between the avapritinib cohort and the BAT cohort
- Patients in the avapritinib cohort had a significantly longer overall survival compared to patients in the BAT cohort (hazard ratio [HR] 0.42, 95% CI 0.24-0.74; P < 0.001)

Conclusions

Avapritinib significantly improves overall survival compared to BAT in patients with SM-AHN who were not candidates for stem cell transplantation.