Introduction

Systemic mastocytosis (SM) is a mast cell (MC) neoplasm driven by KIT D816V mutation in ~90% of cases, characterized by the accumulation of neoplastic MC in various organs.1

Advanced systemic mastocytosis (ASM) includes three subsets: mast cell leukemia (MCL), SM with an associated hematologic neoplasm (SM-AHN), and aggressive SM (ASM), and all subtypes typically have a poor prognosis.2

Diagnosis of SM includes:
- Evaluation of MC aggregates in bone marrow and extracutaneous organs
- Asymmetric MC morphology
- Expression of CD25 or without CD25 on MCs
- Detection of the KIT D816V mutation
- Serum tryptase level of >20 ng/ml (if no associated myeloid neoplasm)

The proportion of CD25+ and CD30+ MCs appearance and immunophenotype and reduction in fibrosis. This was accompanied by a decrease in circulating MCs.

Results

Table 1. Baseline characteristics (n=173)

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<tr>
<th>Variable</th>
<th>ASM 29 (16.5)</th>
<th>ASM 29 (16.5)</th>
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<tr>
<td>Median age, years (range)</td>
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<td>ECOG PS 0–3</td>
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<td>BMB, bone marrow biopsy</td>
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<td>Neutrophil counts (10^9/L)</td>
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<td>Bone marrow infiltration</td>
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Figure 5. Reduction of CD25+ and CD30+ MCs in ASM patients

- **Clinical improvement** (defined as the achievement of MCL CR, complete response by BM examination and after 2.24 ± 1.56 years)
- **Efficacy and safety**

- **Methods**
  - **Patients** (n=173), including 105 with SM and 68 with ASM (29 with MCL and 39 with ASM-29).
  - **Treatment** with Avapritinib (500 mg once daily) for 8 weeks.
  - **Endpoints** included changes in bone marrow MC burden, morphology, and immunohistochemistry in BM, BM cellularity and fibrosis, as well as changes in circulating MC and levels of hematologic parameters.

- **Study design** (Figure 1): A phase 2, open-label, non-comparative, single-arm trial in 173 patients with SM or ASM.

- **Endpoints** (Figure 2): Changes in MC multifocal dense aggregates

- **Results** (Table 1): Baseline characteristics of patients with SM or ASM.

- **Discussion**
  - **Findings** from the study include a comprehensive pooled analysis of the effect of Avapritinib in patients with SM and ASM.

- **Acknowledgements**
  - **References**
  - **Disclosures**

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