EFFICACY OF MIDOSTAURIN THERAPY AND ALLOGENEIC BONE MARROW TRANSPLANTATION IN PATIENTS WITH ADVANCED SYSTEMIC MASTOCYTOSIS

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INTRODUCTION

Advanced systemic mastocytosis (AdvSM) includes aggressive systemic mastocytosis (ASM), mast cell leukemia (MCL), and systemic mastocytosis with associated hematologic neoplasm (SM-AHN) and has an unfavorable course. Treatment requires active drug therapy and, in some cases, allogeneic hematopoietic stem cell transplantation (allo-HSCT).

AIM

To evaluate treatment outcomes in patients with AdvSM.

METHOD

Diagnosis was established according to the 2022 WHO criteria and response to therapy per the 2022 ECNM criteria. This retrospective study Of the 78 patients with mastocytosis included in this retrospective study, 18% had cutaneous mastocytosis, 44% had indolent/smoldering systemic mastocytosis, and 42% (n=33) had AdvSM. In AdvSM, the median time from symptom onset to consultation with a hematologist was 374 days (range, 1.3–42.5 months), the median time from consultation to diagnosis verification was 3.2 months (range, 1 day to 6.7 years), and the median age at diagnosis was 57 years (range, 30–83 years). The AdvSM subgroup (n=33) included 8 cases of ASM (24%), 4 of MCL (12%), and 21 of SM-AHN (64%). Associated hematologic neoplasms included myelodysplastic syndrome with excess blasts-1/2 (n=5), acute myeloid leukemia/myeloid sarcoma (n=4), plasma cell dyscrasias (n=2), unclassifiable myeloproliferative neoplasm (n=3), non-Hodgkin lymphoma (n=2), chronic lymphocytic leukemia (n=1), and chronic myelomonocytic leukemia (n=4). Twenty-eight patients received treatment, 13 (46.4%) midostaurin alone, 6 (21.4%) allo-HSCT, and 9 (32.1%) chemotherapy.

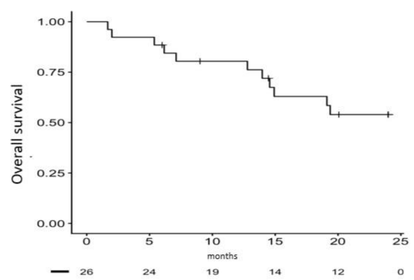
CONCLUSIONS

Both drug therapy and allo-HSCT show unsatisfactory outcomes in AdvSM patients, mainly due to late diagnosis and aggressive associated neoplasms.

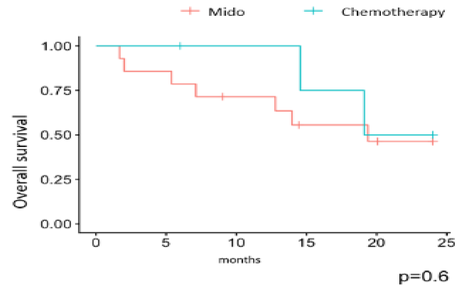
RESULTS

The median midostaurin duration (n=13) was 14 months (range, 1.7–48.5 months). At 12 months, partial response was registered in 1 patient, clinical improvement in 4, stable disease in 3, progression in 4, and loss of response in 1. The allo-HSCT group included 6 patients, 2 with MCL and 4 with SM-AHN. Overall survival was 54% (95% CI, 32%–71%) at median follow-up of 19.2 months. Progression-free survival at 24 months was 48% (95% CI, 26%–68%), and median time to progression was 20.8 months (range, 4.3–68.6 months). Progression occurred in 19 patients (13 with SM-AHN). Overall survival at 24 months was 46% in the midostaurin group (n=13) and 33% in the allo-HSCT group (n=6).

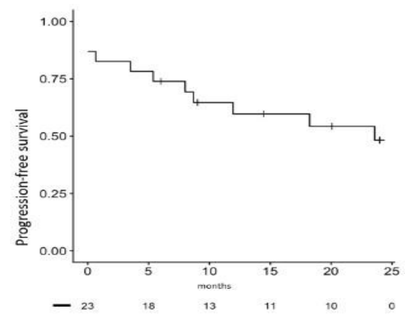
The 2-year overall survival



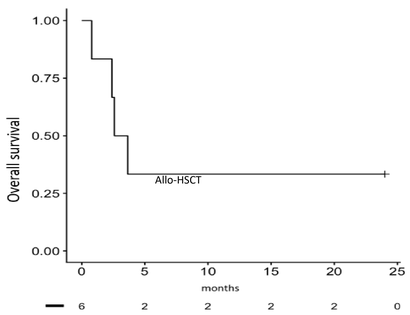
The 2-year overall survival



Progression-free survival



The 2-year overall survival after allo-HSCT



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