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Conference Abstract A8

Results of Targeted Therapy for Systemic Mastocytosis at the R.M. Gorbacheva Research Institute

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Background

Midostaurin is the first targeted agent approved for the treatment of systemic mastocytosis (SM). Its efficacy is independent of C-KIT mutation status and it has improved overall survival outcomes without the need for allogeneic bone marrow transplantation.

Objective

To evaluate the outcomes of targeted therapy for systemic mastocytosis in clinical practice.

Materials and Methods

The diagnosis of systemic mastocytosis was established according to WHO 2017 criteria. Diagnostic workup included complete blood count with microscopy, cytological and histological examination of bone marrow, serum tryptase measurement, C-KIT mutation analysis (KITD816V, mutations in exons 8–11 and 17), abdominal ultrasound, and low-dose whole-body CT scan. Treatment response was assessed according to the 2024 National Clinical Guidelines for the diagnosis and therapy of mastocytosis.

Results

Between 2019 and 2024, systemic mastocytosis was confirmed in 41 patients. Distribution of SM subtypes was as follows: indolent SM (ISM) – 53.6% (n=22), smoldering SM (SSM) – 2.6% (n=1), aggressive SM (ASM) – 7.2% (n=3), SM with associated hematologic neoplasm (SM-AHN) – 26.8% (n=11), mast cell leukemia (MCL) – 4.9% (n=2), unclassified SM – 4.9% (n=2). KITD816V mutation was detected in 75% of cases, C-KIT wild type in 22%, and Phe522Cys mutation in 3%.Midostaurin therapy was administered in 31% (n=13): ISM (n=4), SSM (n=1), ASM (n=2), SM-AHN (n=4), MCL (n=2). At therapy initiation (dose 200 mg/day), clinical manifestations were present in all patients: general symptoms 85% (n=11), cutaneous involvement 77% (n=10), splenomegaly 77% (n=10), hepatomegaly 54% (n=7), lymphadenopathy 61% (n=8), anaphylaxis episodes 15% (n=2), skeletal involvement 39% (n=5), gastrointestinal symptoms 46% (n=6), transfusion dependence on platelet concentrates 7.6% (n=1) and on erythrocyte products 23% (n=3). Median follow-up was 18 months (range 6–30). After 6 months of therapy: partial response – 15% (n=2), clinical improvement – 38.75% (n=5), stable disease – 38.75% (n=5), disease progression – 7.5% (n=1). No differences in response were observed depending on C-KIT status. Median duration of response was not reached in any SM subgroup. Median overall survival (OS) was not reached in ISM and SSM groups; in advanced SM it was 6 months, with a single fatal case (progression) in MCL. Dose reduction of midostaurin (to 50 mg/day) was required in 30% (n=4): due to Grade 4 hematologic toxicity in 15% (n=2) and Grade 4 emetic syndrome in 15% (n=2).

Conclusion

Targeted therapy with midostaurin results in clinical response across all systemic mastocytosis subgroups within 6 months of treatment initiation; however, long-term follow-up is required.