Lenalidomide is Effective Treatment Option for Patients with Refractory Anemia with Ring Sideroblasts and Thrombocytosis

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Introduction

- Refractory anemia with ring sideroblasts and thrombocytosis (RARS-T) an MDS/MPN overlap syndrome with features of refractory anemia with ring sideroblasts (RARS) and thrombocytosis (platelet count $>450 \times 10^9/L$).
- Management of RARS-T is primarily aimed at alleviating symptoms of anemia and thrombocytosis.
- Individual case reports suggest lenalidomide may be effective in treating RARS-T.
- In non-del 5q, low-risk MDS, 25% of patients (pts) became red blood cell transfusion independent (RBC-TI). In the phase II study of lenalidomide in non-del 5q lower risk MDS, 35% of pts with RARS treated with lenalidomide achieved RBC-TI.
- The primary objective of this study is to evaluate a series of pts with RARS-T and compare HI responses among different treatment options.

Methods

- This is a retrospective, single-center study. Patients with RARS-T were identified from the MDS Database at Moffitt Cancer Center. Samples for somatic mutation testing were obtained at time of referral.
- Descriptive statistics were used to describe baseline characteristics and response rates to treatment.
- The primary endpoint was HI using international working group criteria (IWG) 2006. The median overall survival (OS) was calculated from time of diagnosis.

Table-1

Descriptive Characteristics	
Characteristics	N=33 (%)
Gender	
Male	15 (45.5%)
Female	18 (54.5%)
Race	
White	27 (82%)
Other	6 (18%)
Mean Age	67 (38-80)
IPSS Prognostic Risk Score	
Low	24 (73%)
Intermediate	9 (27%)
R-IPSS Prognostic Risk	
Very Low	11 (33%)
Low	18 (55%)
Intermediate	3 (9%)
High	1 (3%)
Mean Blood Counts	
WBC	7.7 (2.8-25.7)
HgB	9.9 (7.3-12.4)
Platelets	682 (372-1644)
Available Nex Gen Seq N=20 (60.6%)	
SF3B1	19 (95%)
JAK-2 V617 F	4 (20%)

Results

- Of the 33pt with RARS-T, the median OS was 90 mo. Median OS for JAK-2 V617F mutations was 110 mo vs 74 mo for wild type ($p=0.24$). The rate of AML transformation was 3% (1 pt).
- 8 pts had no treatment, 22 pts received ESA prior to active therapy, 2 patients received lenalidomide with no prior ESA, and 1 pt ruxolitinib with no prior ESA.
- 12 pts (36%) received lenalidomide (7 as first line active therapy after ESA and 2 without prior ESA exposure) and 13 pts (33%) received azacitidine (6 as first line active therapy after ESA).
- The HI rate for ESA was 36% (12/22) with a median duration of ESA treatment of 33mo.
- The HI rate for lenalidomide was 50% (6/12) with a median duration of lenalidomide treatment of 10.3mo and the HI rate for the 9 pts who received lenalidomide as first line active therapy was 56%.
- The HI rate for azacitidine was 15% (2/13) with a median duration of azacitidine treatment of 6.3mo.

Conclusions

- Pts with RARS-T had favorable outcomes with long overall survival and a low rate of AML transformation.
- Rates of hematological improvement with the use of azacitidine were lower than expected.
- Lenalidomide is an effective treatment option and should be considered when treating patients with RARS-T.