



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

508.BONE MARROW FAILURE: ACQUIRED

IST Combined with Full-Dose Rhtpo in the Treatment of Severe Aplastic Anemia -a Prospective Single-Center Clinical Study

Huihui Fan¹, Jing Hu², Wenrui Yang¹, Xin Zhao¹, Li Zhang¹, Kang Zhou¹, Youzhen Xiong¹, Liping Jing, MD³, Fengkui Zhang⁴

¹Anemia Therapeutic Center, Institute of Hematology and Blood Diseases Hospital, CAMS & PUMC, Tianjin, China

²Institute of Hematology and Blood Diseases Hospital, CAMS & PUMC, Tianjin, China, Tianjin, China

³Anemia Therapeutic Center, Institute of Hematology and Blood Diseases Hospital, CAMS & PUMC, Tianjin, China

⁴State Key Laboratory of Experimental Hematology, National Clinical Research Center for Blood Diseases, Anemia Therapeutic Center, Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences & Peking Union Medical College, Tianjin, China

BACKGROUND: Immunosuppressive therapy (IST) (anti-thymocyte immunoglobulin (ATG) and cyclosporine (CSA)) is the treatment of choice for patients with acquired severe aplastic anemia (SAA) who are ineligible for hematopoietic stem cell transplantation (HSCT), which results in a hematological response (HR) in about 60-70% of SAA patients, while 1/3 of patients fail to achieve a hematological response. TPO is a hepatic-secreted hematopoietic growth factor that specifically regulates platelet production, induces hematopoietic stem cell differentiation to megakaryocytes by binding to the TPO receptor (MPL), stimulates megakaryocyte proliferation and differentiation, and promotes platelet production and release. Previous retrospective studies have demonstrated that IST combined with rhTPO improves the early hematologic response in SAA patients, but efficacy reported by centers varied depending on the dose and duration of rhTPO. There are no prospective studies of rhTPO application in SAA to confirm its efficacy. So this study was designed to observe the early efficacy and safety of the SAA patients who were treated with IST combination with full-dose rhTPO.

METHODS: This study was a single-center, single-arm, prospective cohort clinical study, enrolling 33 naive SAA patients aged between 14-70 years who diagnosed after April 2021 in our study center. The patients treated with IST combined with full-dose rhTPO (300U/kg.d) for 8 weeks starting from the first day of ATG. The primary endpoint of the observational study protocol good response rate, blood product transfusion volume and safety profile at 3 months of treatment of initial SAA. The patients were also evaluated for total hematologic response rate and adverse effects of drug application, clonal shift rate, and recurrence rate at 3 and 6 months after treatment.

Efficacy Criteria: Complete response (CR): HGB>100 g/L, PLT> 100×10⁹/L and ANC>1.5×10⁹/L; ② Good partial response (GPR): HGB>80g/L, PLT> 50×10⁹/L and ANC>1.0×10⁹/L; ③ Partial response (PR): withdrawal from blood product transfusion dependence (HGB>70g/L, PLT> 20×10⁹/L), hematologic tests improved and no longer met the SAA criteria, but blood count did not meet the GPR criteria. ④No response to treatment (NR): Continuous transfusion dependency was classified as no response. and/or blood count still met SAA criteria. We defined CR+GPR+PR as HR and CR+GPR as good HR.

RESULTS: The rhTPO group enrolled 33 patients, and the control group was 99 patients who applied the caliper value of 0.2 to match the previous unapplied TPO treatment of SAA. At 3 months after IST treatment efficacy, the HR rate of 33 patients in the rhTPO group was 66.67% (22 /33), and the rate of good HR was 36.4% (12/33). The HR rate in the control group was 32.32% (32 /99), and the rate of good HR was 12.12% (12/99). The HR rate and the good HR rate between the two group were significantly statistical differences (66.67% v 32.32%; 36.4% v 12.12%), (P value=0.001; 0.002). At 6 months after IST treatment, the HR rate and good HR rate in the rhTPO group had a significantly higher than those of the control group (66.67% v 32.32%; 36.4% v 12.12%), (P value=0.001; 0.002).

No disease transformation such as myelofibrosis, myelodysplastic syndrome and acute myeloid leukemia occurred in all enrolled patients during the follow-up period.

CONCLUSION: 22 of the 33 SAA patients in this study achieved HR at 3 months after IST treatment, including 36.4% cases achieved good HR, which is the highest good efficacy response in a prospective study of SAA patients in China known to us to date. This confirms that full-dose rhTPO, as a macromolecular peptide, can also promote the recovery of bone marrow

hematopoietic function, significantly improved the rapidity and strength of HR in SAA patients. And its clinical effect is no less than that of the various TPO-Ras that have been widely used so far, and may even be higher.
 [Keywords] severe aplastic anemia; recombinant human thrombopoietin; immunosuppression treatment.

Disclosures No relevant conflicts of interest to declare.

Tab 1. The efficacy at 3 month after IST therapy

efficacy	CR	GPR	PR	Good HR	HR
IST+full-dose rhTPO (33)	3	9	10	12	22
N (%)	(9.1%)	(27.3%)	(35.7%)	(36.4%)	(66.7%)
IST only (99)	2	10	20	12	32
N (%)	(2.0%)	(10.1%)	(20.2%)	(12.1%)	(32.3%)
<i>P</i>	<i>0.188</i>	<i>0.032</i>	<i>0.23</i>	<i>0.002</i>	<i>0.001</i>

Tab 2. The efficacy at 6 month after IST therapy

efficacy	CR	GPR	PR	Good HR	HR
IST+full-dose rhTPO (33)	6	6	12	12	24
N (%)	(18.2%)	(18.2%)	(36.4%)	(36.4%)	(72.7%)
IST only (99)	12	12	25	24	49
N (%)	(12.1%)	(12.1%)	(25.3%)	(24.2 %)	(49.5%)
<i>P 值</i>	<i>0.558</i>	<i>0.558</i>	<i>0.267</i>	<i>0.176</i>	<i>0.026</i>

Downloaded from http://ashpublications.net/blood/article-pdf/142/Supplement_1/4086/2192023/blood-687-main.pdf by guest on 29 May 2024

Figure 1

<https://doi.org/10.1182/blood-2023-182717>

Downloaded from http://ashpublications.net/blood/article-pdf/142/Supplement_1/4086/2192023/blood-687-main.pdf by guest on 29 May 2024