ELSEVIER

Blood 142 (2023) 6482

The 65th ASH Annual Meeting Abstracts

ONLINE PUBLICATION ONLY

637.MYELODYSPLASTIC SYNDROMES - CLINICAL AND EPIDEMIOLOGICAL

The Efficacy and Prognosis of Advanced Myelodysplastic Syndrome in Children

Chenmeng Liu[1], *Xiaolan Li*[2], *Yunlong Chen*[2], *Yang Wan*[2], *Yang Lan*[3], *Jingliao Zhang, MD*[2], *Xiaofan Zhu, MD*[2], *Wenyu Yang*[4]

[1] State Key Laboratory of Experimental Hematology, National Clinical Research Center for Blood Diseases, Haihe Laboratory of Cell Ecosystem, Institute of Hematology & Blood Diseases Hospital, CAMS & PUMC 2.Tianjin Institutes of Health Science, Tianjin, China
[2] Tianjin Institutes of Health Science, Tianjin, China
[3] Insitute of Hematology & Blood Diseases Hospital Chinese Academy of Medical Scie, Tianjin, CHN
[4] Pediatric Blood Diseases Center, State Key Laboratory of Experimental Hematology, National Clinical Research Center for Blood Diseases, Haihe Laboratory of Cell Ecosystem, Institute of Hematology & Blood Diseases Hospital, Chinese Academy of Medical Sciences, Tianjin, China

Pediatric myelodysplastic syndrome (MDS) is a clonal hematopoietic stem cell neoplasm with an annual incidence of 1-4 cases/million. The low incidence of advanced MDS in children and poor prognosis attract our attention. However, there are limited reports on the scheme and efficacy of pediatric advanced MDS. In this study, we retrospectively analyzed the efficacy of chemotherapy in pediatric advanced MDS and assessed the prognosis of patients. According to 2016 World Health Organization (WHO) classification of haematolymphoid tumours, 30 newly diagnosed pediatric advanced MDS patients during Dec 2007 to Apr 2022 from our single center were selected as the research object. The clinical data and prognosis were analyzed. The median age of patients is 8 years (range: 1-15years). The median bone marrow blast is 15.75% (range: 5-29.5%), while the median peripheral blood blast is 4% (range: 0-29%). Cytogenetic analysis was available for 29 patients, 69% (20/29) cases had abnormal karyotype. Monosomy 7 (37.9%) was the most common cytogenetic abnormality. Out of the total, 7 patients were treated with CAG/HAG regimen (C: aclarubicin 6mg d1-14, H: homoharringtonine 1mg/m ^{2}d1-14, A: cytarabine 10mg/m ^{2} q12h d1-14, and G: Granulocyte Colony-stimulating Factor 200mg/m ^{2} d1-14), 8 patients with decitabine (20mg/m ^{2} d1-5), 11 patients with decitabine and CAG/HAG, 4 patients with AML-like chemotherapy (Daunorubicin+cytarabine+etoposide) or other. The evaluation of response of therapy was available in 25 patients, 5 cases (20%) in partial remission (PR), 11 cases (44%) in complete remission (CR), 9 cases in(36%)non-remission (NR). The total response rate of chemotherapy is 60%, with CAG/HAG therapy 33%, decitabine therapy 43%, combined CAG/HAG and decitabine therapy 78%, AML-like chemotherapy 100%. The total complete remission rate of chemotherapy is 44%, with CAG/HAG therapy 17%, decitabine therapy 43%, combined CAG/HAG and decitabine therapy 56%, AML-like chemotherapy 67%. Patients receiving CAG/HAG in combination with decitabine had higher response rates compared to CAG/HAG or decitabine alone (OR=17.5, 95% CI 1.2-250.3, P=0.035). With a median follow-up of 20 months (range: 1-158months), 23 patients received HSCT after chemotherapy, and they had a higher 2-year overall survival rate compared to those who did not undergo transplantation (86% vs 0%, p<0.001). Decitabine combined with CAG/HAG improve the response rate of advanced pediatric MDS. Chemotherapy bridged hematopoietic stem cell transplantation significantly improves the prognosis of advanced pediatric MDS.

Disclosures No relevant conflicts of interest to declare.

https://doi.org/10.1182/blood-2023-186054