ELSEVIER

Blood 142 (2023) 6480

The 65th ASH Annual Meeting Abstracts

ONLINE PUBLICATION ONLY

637.MYELODYSPLASTIC SYNDROMES - CLINICAL AND EPIDEMIOLOGICAL

Decitabine in Combination with Aclacinomycin, Cytarabine and Granulocyte Colony-Simulating Factor (CAG) Had a High Response Rate in Children with Advanced Myelodysplastic Syndrome and Acute Myeloid Leukemia with Myelodysplasia-Related Changes

Yuanyuan Ren[1,2], *Yumei Chen*[1,2], *Shuchun Wang*[1,2], *Tianfeng Liu*[1,2], *Yang Wan*[2,1], *Jingliao Zhang, MD*[2,3], *Wenyu Yang*[2,3], *Ye Guo, MD*[1,2], *Xiaofan Zhu*[4,2]

[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, Chinese Academy of Medical Sciences&Peking Union Medical College, Tianjin, China
[2] Tianjin Institutes of Health Science, Tianjin, China
[3] 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
[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 & Peking Union Medical Colleg, Tianjin, China

Advanced myelodysplastic syndrome (MDS) is a rare disease in children with poor prognosis. Allogeneic transplantation is the only curative option. However, there has been no established treatment prior to transplant. We conducted a retrospective study exploring the response rates and safety of decitabine combined with aclacinomycin, cytarabine and granulocyte colony-stimulating factor (CAG) for induction treatment of advanced MDS and acute myeloid leukemia with myelodysplasia-related changes (MDR-AML) in children. Patients from 1 to 14 years administrated in Pediatric Blood Disease Center of CAMS Blood Disease Hospital between January 1the 2019 and December 31th 2022 were screening. All diagnosed with MDS-EB or MDR-AML according to WHO 2016 criteria and treated with decitabine combined with CAG were identified. The efficacy of one-cycle treatment of MDS-ED was evaluated based on The International Working Group Response Criteria 2006. The response for MDR-AML was evaluated according to the criteria for AML published by ELN. The response of MDR-AML was defined as complete remission (CR) or CR with incomplete count recovery (CRi). The response of MDS-EB was defined as CR or bone marrow CR with hematological improvement (HI). A total of 13 patients, including MDS-EB (n=7) and MDR-AML (n=6), were enrolled for analysis. 5-day decitabine was administrated, combined with modified CAG regimen for 7 days. Eventually 12 patients of those were available for evaluation. For patients with MDS-EB, four achieved CR and three reached marrow CR with HI. Four patients with MDR-AML, three achieved CR and two reached CRi. The overall response rate was 100%(12/12). The most frequent adverse events were myelosuppression and suppression-induced infections, but no infection-related mortality was recorded. There had been no cardiotoxic event happened, either. Only one patient experienced fatal bleeding in early stage after treatment. The median follow-up time was 16 months (range 1-48). At last follow-up, 10 patients who had underwent allo-HCT were alive without disease. The remaining 2 patients died of disease progression. The 2-year estimated overall survival was 62.9%. Decitabine combined with modified CAG had a high response rate for children with advanced MDS and MDR-AMl, and it can bridge patients to curative allogeneic transplantation.

Disclosures No relevant conflicts of interest to declare.

OffLabel Disclosure: Decitabine In instructionsï¼Œthe safety and effectiveness of decitabine in pediatric patients have not been established. But it has widely reported in clinical trials to treat myeloid malignancies.

https://doi.org/10.1182/blood-2023-184620