



The 65th ASH Annual Meeting Abstracts

POSTER ABSTRACTS

615.ACUTE MYELOID LEUKEMIAS: COMMERCIALY AVAILABLE THERAPIES, EXCLUDING TRANSPLANTATION AND CELLULAR IMMUNOTHERAPIES**Decitabine Combined with Low-Dose Chemotherapy Is Effective in the Treatment of Pediatric Refractory/Relapsed Acute Myeloid Leukemia**

Liyan Fan¹, Hua Jiang, MDPhD², Hongjun Liu, MD³, Ning Liao, MD⁴, Ningling Wang, MD⁵, Xiaowen Zhai, MDPhD⁶, Xiuli Ju, MDPhD⁷, Yufeng Liu, MDPhD⁸, Jixia Luo, MD⁹, Peifang Xiao¹, Jun Lu¹, Hailong He¹, Yi Wang¹, Raul C. Ribeiro, MD¹⁰, Shaoyan Hu, MDPhD¹

¹ Department of Hematology and Oncology, Children's Hospital of Soochow University, Suzhou, China

² Department of Hematology and Oncology, Guangzhou Women and Children's Medical Center, Guangzhou, China

³ Department of Hematology and Oncology, Anhui Provincial Hospital, University of Science and Technology of China, Hefei, China

⁴ Department of Pediatrics, The First Affiliated Hospital of Guangxi Medical University, Nanning, China

⁵ Department of Hematology and Oncology, The Second Affiliated Hospital of Anhui Medical University, Hefei, China

⁶ Department of Hematology and Oncology, Children's Hospital of Fudan University, Shanghai, China

⁷ Department of Hematology and Oncology, Qilu Hospital of Shandong University, Qingdao, China

⁸ Department of Hematology and Oncology, The First Affiliated Hospital of Zhengzhou University, Zhengzhou, China

⁹ Department of Hematology and Oncology, Kaifeng Children's Hospital, Kaifeng, China

¹⁰ Division of Leukemia/Lymphoma, Department of Oncology, St. Jude Children's Research Hospital, Memphis, TN

B ackground : Refractory and relapsed acute myeloid leukemia are usually associated with poor outcomes. Allogeneic hematopoietic stem cell transplantation (allo-HSCT) is the only curative option for children suffering from refractory/relapsed AML. Complete remission before transplantation can reduce the recurrence rate after transplantation and improve the survival rate. Previous studies have found that low-dose chemotherapy has a complete response rate and long-term survival rate comparable to standard dose chemotherapy, and chemotherapy-related toxicities are lower. Decitabine is active in both initial induction therapy and post-relapse therapy of AML, and has a synergistic effect on apoptosis of leukemia cells when combined with cytarabine. We therefore conducted a prospective study to confirm the efficacy of decitabine combined with low-dose chemotherapy in the treatment of pediatric refractory/relapsed AML. The trial is registered under ChiCTR1800015872(A multicenter clinical study of decitabine combined with low-dose chemotherapy in the treatment of refractory/relapsed AML in children).

Methods: We randomized 89 participants < 18 years with refractory/relapsed AML to receive decitabine combined with low-dose chemotherapy. All patients received decitabine 20 mg/ (m²·d) ×5 days, intravenous; combined with idarubicin 5 mg/ (m²·d), qod×3 times, intravenous; cytarabine 10 mg/ (m²·d), q12h×10 days, subcutaneous; granulocyte stimulating factor 5μg/ (kg·d), qd×10 days, intravenous. Subsequently, children received additional intensive chemotherapy or hematopoietic stem cell transplantation (HSCT) as post-remission consolidation. The primary endpoint was overall survival.

Results: From June 2018 to June 2022, the trial included 31 children with relapsed AML and 58 children with refractory AML. The male to female ratio was 57:32, median age 100 months (range 59-133months). The most common fusion genes were AML-ETO(17, 19.1%) and MLL-r (17, 19.1%), followed by NUP98- (7, 7.9%),CBFβ-MYH11(5, 5.6%). The most common mutation was WT1(18,20.2%), while FLT3, NRAS, and PTPN11 had the same proportions. (14, 15.7%).After one course of chemotherapy, 48 children (53.9%) achieved CR/CRi, 21 children (23.6%) achieved PR, and 20 children (22.5%) achieved NR, with an overall response rate of 77.5%. Thirty-one of these children received a second course of decitabine combined with low-dose chemotherapy. 11 children (35.5%) achieved CR/CRi, 17 children (54.8%) achieved PR, and 3 children (9.7%) remained NR. Of the 89 children, 66 (74.2%) children eventually received HSCT, while the remaining 23 did not. The five-year cumulative survival rate was 64.7%. Among them, the five-year survival rate of relapsed AML is 52.6%,while that of refractory AML was 76.1%. The survival rate of refractory AML was better, but there was no statistical difference in P value between the two groups. The five-year survival rate was 82.7% for those who received HSCT, compared with 19.8% for those who only received chemotherapy, the P-value was statistically significant. The survival rate of different fusion genes was shown in figure 1.

Conclusions: Decitabine combined with low-dose chemotherapy is a favorable benefit-risk profile and may be a promising option for refractory/relapsed AML in children. Hematopoietic stem cell transplantation after remission can improve survival rate.

Disclosures No relevant conflicts of interest to declare.

<https://doi.org/10.1182/blood-2023-191115>

Survival analysis function

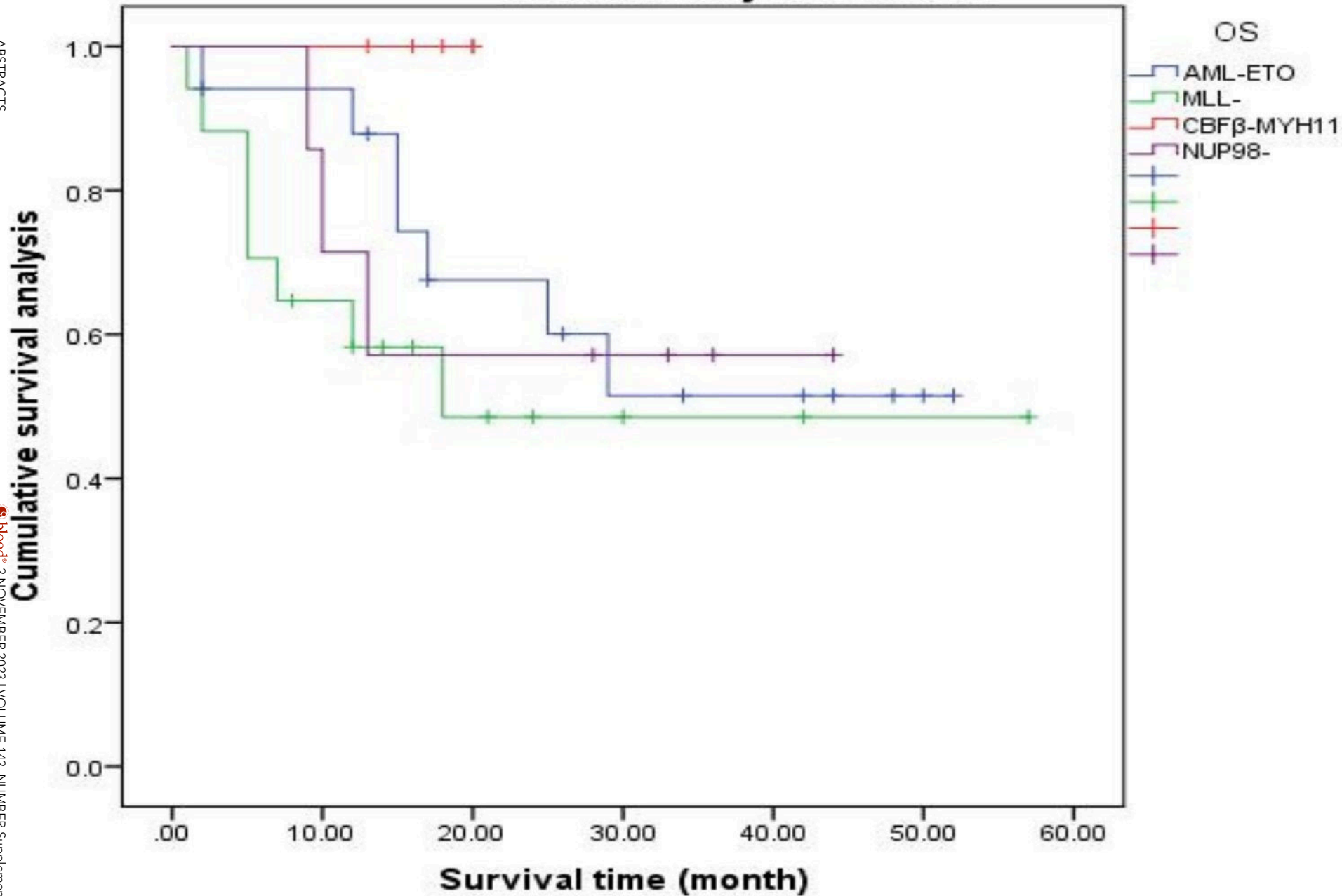


Figure 1 OS of different fusion genes

Figure 1