Outcome prediction of chronic myeloid leukaemia (CML) in children

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Abstract

Purpose We evaluated the existing risk assessment tools for CML in children. Patients and Methods A total of 55 patients from 1.4 to 18.0 years with newly diagnosed CML between 1996 and 2019 were included. Forty-nine patients presented in the chronic phase, thirty-six of whom were treated with upfront tyrosine kinase inhibitor (CP-TKI group); one presented in the accelerated phase and 4 in the blastic phase. Treatment, survival, responses, and tolerance were evaluated. Results The median follow-up time was 8.7 years (range, 2 months to 24.3 years). All patients in the CP-TKI group received imatinib as their first TKI treatment. Allogenic stem cell transplantation was performed in one patient after complete cytogenetic response was achieved with imatinib and in one patient with imatinib failure. Dasatinib and nilotinib were prescribed as second-line TKI in 5 patients and 4 patients respectively. The 10-year overall survival (OS), progression-free survival (PFS) and event-free survival (EFS) of TKI treated group was 97%, 91.4% and 72.3% respectively. The rates of major molecular response and deep molecular response of TKIs were 81.2% and 67.5% at 60 months. The EUTOS long-term survival (ELTS) risk grouping did not predict OS, PFS or EFS. The IMAFAIL risk groups are correlated with the risk of imatinib failure. Conclusion TKIs resulted in excellent long-term overall and progression-free survival in children and adolescents with newly diagnosed CML in the chronic phase. Further studies are required to modify the existing prognostic scoring system or develop new ones for children.

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