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Prognostic implication of molecular aberrations in cytogenetically normal acute myeloid leukemia patients receiving allogeneic hematopoietic stem cell transplantation

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ABSTRACT. Different molecular aberrations can be discriminated into certain prognostic subgroups in cytogenetically normal acute myeloid leukemia (CN-AML) patients but their impact on allogeneic hematopoietic stem cell transplantation (allo-HSCT) remains controversial and studies from Asian populations are lacking. Forty-two adult non-M3 AML patients receiving allo-HSCT from 2002 to 2009 in southern Taiwan were retrospectively reviewed for survey,

23 (54.7%) of whom were CN-AML. *NPM1*, *FLT3*-ITD, and *CEBPA* were analyzed. After a median follow-up of 104 weeks (range, 8 to 384), patients in the good risk group (harboring either *NPM1* or *CEBPA* mutation without concurrent *FLT3*-ITD) showed a borderline worse overall survival (OS) compared with the intermediate/poor risk group (P = 0.08). Interestingly, a poorer OS was found in patients with the *CEBPA* mutation (P = 0.003) but not the *NPM1* mutation (P = 0.96). No OS difference was found between patients with or without *FLT3*-ITD (P = 0.15). In patients receiving allo-HSCT at first remission, there was no significant OS benefit in the good risk group (P = 0.33). In patients receiving allo-HSCT beyond first remission, disease status played a major role (P = 0.006), irrespective of molecular aberrations. Allo-HSCT in good risk patients should be carefully evaluated in Taiwanese, especially in patients with the *CEBPA* mutation. Conversely, allo-HSCT should be considered in first remission in patients with an intermediate/poor risk, where it may overcome the adverse impact of *FLT3*-ITD. Disease status remained a main issue in patients receiving allo-HSCT beyond first remission.

Key words: Acute myeloid leukemia; Cytogenetic; Mutation; Hematopoietic stem cell transplantation; Molecular aberrations