中文題目：去甲基化藥物 Azacitidine 使用於急性骨髓性白血病(AML)的治療成果

英文題目：Clinical Characteristics, Response and Outcome of Acute Myeloid Leukemia (AML) Patients Treated with Hypomethylating Agents

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INTRODUCTION：Hypermethylation of tumor suppressor genes plays a major role in cancer pathogenesis. Hypomethylating agents azacitidine (AZA) and decitabine (DAC) have shown efficacy for the treatment of hematologic malignancies where gene hypermethylation occurs. AZA had been approved by FDA for myelodysplastic syndrome (MDS), and recent studies also showed the efficacy of AZA in acute myeloid leukemia (AML). However, the reports regarding the response and outcome of AML patients treated with AZA are limited in Asia, and it interaction with hematopoietic stem cell transplantation (HSCT) remains to be delineated.

METHODS：We enrolled 100 AML patients who received AZA between 2007 and 2015 in National Taiwan University Hospital. The response criteria were defined by the IWG-2003 criteria for AML. Chromosomal analyses were performed by Giemsa banding.

RESULTS：The patients had a median age of 67 years. 55 (55.0%) patients had de novo AML. 77 patients had intermediate cytogenetic risk, 18 had poor cytogenetic risk, but none had good-risk cytogenetics. The median AZA cycles were 4 (range 1 to 20), and 54 (54.0%) patients received AZA as first-line treatment. Totally, 33 (33.0%) patients had received HSCT.

The overall response rate was 31.9%. After a median follow-up time of 13 months (range 0.6-100), the median overall survival (OS) was 8.1 months. Univariate analysis revealed BM blast <20% (P=0.005), HSCT (P=0.007), and AZA responder (P=0.027) were correlated with longer OS. In multivariate analysis, HSCT (P=0.001) and responder (p=0.034) were independent predictors for longer OS. The estimated 1-year, 2-year OS were 49%, 39% in the HSCT group and 31%, 11% in the non-HSCT group (P=0.019)..

CONCLUSION：Hypomethylating agents AZA has shown efficacy for AML patients,

including those with poor-risk cytogenetics and those refractory to conventional chemotherapies. AZA in combination with HSCT confers OS benefit. AZA may represent a suitable option for AML patients in particular as a bridge to allogeneic transplantation.