

## 去甲基化药物治疗骨髓增生异常综合征的研究进展

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**摘要** 近年来表观遗传学研究在恶性肿瘤分型以及临床治疗方面发挥了重要作用。表观遗传是一种不涉及DNA序列变化的、可以在细胞分裂中传递的基因表达调控机制, 主要包括DNA甲基化和组蛋白乙酰化。其中DNA甲基化是目前人们研究最为深入的一种表观遗传学修饰方式, 主要发生在CpG二核苷酸序列的胞嘧啶上, 已经证实其与多种肿瘤发生密切相关。DNA甲基化的可诱导性和可逆性特点也为肿瘤发生机制的探讨和肿瘤治疗提供了新的途径。大量证据表明DNA甲基化在骨髓增生异常综合征(Myelodysplastic syndrome, MDS)的形成与发展中发挥作用。两个去甲基化药物(阿扎胞苷和地西他滨)在临床上应用治疗高危和中高危的MDS病人取得的成功, 为MDS的病因研究和临床治疗带来了新的思路。文章主要就这两种药物对MDS的作用机制、应用效果和新的临床问题等方面进行综述, 增加对药物作用的理解, 为临床治疗提供更好的手段。

**关键词:** 骨髓增生异常综合征 去甲基化 治疗

**Abstract:** Epigenetic research plays an important role in the malignant tumor genotyping and tumor clinical treatment recently. Epigenetics is the study of changes in gene function that are mitotically and/or meiotically heritable and that do not entail a change in DNA sequence, including DNA methylation and histone modifications. DNA methylation is one of the most important epigenetic modifications often occurring on the cytosine of CpG islands located in gene promoter regions, which is thought to be closely correlated with tumorigenesis. The inducibility and reversibility of DNA methylation provide us an insight into tumor development and treatment. Aberrant DNA hypermethylation is associated with the progress of myelodysplastic syndrome (MDS). The DNA methyltransferase inhibitors (azacytidine and decitabine) have achieved success in treating high- and intermediate-risk MDS. This will bring new ideas to understand the cause and develop the treatment of MDS. This review mainly introduces the latest progress of the action mechanism of those two medicines, the clinical effect and new problems during the clinical application on MDS.

**Keywords:** myelodysplastic syndrome(MDS), DNA demethylation, treatment

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