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RNA/DNA嵌合分子介导的高效基因修复 Targeted Gene Correction Directed by Chimeric RNA/DNA Oligonucleotides

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摘要　本文介绍了RNA/DNA嵌合分子介导的高效基因修复技术。这一技术是1996年开始发展起来的全新技术，它通过人工合成的双链开环RNA/DNA嵌合分子转染细胞而使特定基因靶位点产生单碱基改变，从而修复突变基因。这一技术高效（目前最高可达50%以上）、特异性强、安全、无随机插入致变的危险、无免疫反应、无明显毒性，能够用于定点突变、基因敲除、动植物功能基因组学、药物遗传学等很多方面的研究，在不久的将来能够应用于人类基因治疗，具有很高的应用价值和医学前景。

Abstract: We introduce a new technique?targeted gene correction directed by chimeric RNA/DNA oligonucleotides which began at 1996. It uses synthetic double?stranded non?circular RNA/DNA chimeric oligonucleotides to transfect cells and make a single?based change at the targeted site of the target gene. It is highly efficient (the highest efficiency is more than 50%), highly special, safe, without danger of mutation caused by random insertion, without immune response, and without obvious toxicity. It can be used to make point mutation, or gene knock?out plants and animals, and is very likely to be used in human gene therapy in the near future. It is also valuable in the study of functional genomics, pharmacogenetics, and medicine.

关键词　RNA/DNA嵌合分子　基因治疗　基因修复　Key words　RNA/DNA chimeric oligonucleotides　gene therapy　gene correction

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