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GENETIC SURGERY: CRISPR/Cas 9

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Özet

CRISPR/Cas9 (Clustered Regularly Interspaced Short Palindromic Repeats and CRISPR associated Cas9) mediated new generation genome modification system which is one of the recently discovered genome editing tools. It makes possible for us to correct the functions of the gene, to learn about a possible function of the gene or turn it on. CRISPR/ Cas 9 acts as a genetic scissors thus why we call it genetic surgery. The cell is forced to fix the mutation that was induced by DNA repair systems. The beauty of the CRISPR/Cas9 is the opportunities it gives us to learn and fix a variety of genetical conditions which will open and pave the way for future clinical treatments. This system can be thought of as the adaptive immune system of bacteria. There are CRISPR locuses in the bacterial DNA which consists of CRISPR DNA and spacer DNA where the host hides the genetic information of the virus. Next to these locuses there are Cas enzyme locuses. Cas9 is a type of enzyme that uses CRISPR sequences as guide RNA (gRNA) to find the location that it needs to cleave the DNA at. In order not to cleave its own DNA, PAM sequences are recognized in the viral DNA which the bacterial DNA doesn't consist of. After CRISPR/Cas 9 system makes the double strand break of DNA which we could call an induced mutation there are two possibilities. First one would be HDR (Homology Directed Repair) where knock in procedure can be performed by adding a gene/sequence and the second would be NHEJ (Non-Homologous End-Joining) where knock down procedure happens by gene silencing or knock off procedure which the expression of the gene is reduced. Basically the cell is forced to repair a mutation that we have induced and with this gene knock out, knock in and knock down can happen. Many studies are done all over the world about B-thalassemia, cancer, DMD, HBV, leukemia, cataracts, familial amyloidosis. However CRISPR/Cas9 system is far from being a perfect system that can be used in clinics but that doesn't mean that it doesn't have a bright future. The simplicity of the CRISPR/Cas9 system will enable it to be used effectively in ex vivo gene therapy studies in humans in the future.

Anahtar kelimeler: Crispr, cas9, gen terapisi, genetik cerrahi