CRISPR/Cas9

A genome editing tool derived from bacteria. Consists of a guide RNA (gRNA) ❶, which is complementary to a target DNA sequence, and an endonuclease (Cas9), which makes a single- or double-strand break at the target site ❷. Break imperfectly repaired by nonhomologous end joining (NHEJ) → accidental frameshift mutations ("knock-out") 3A, or a donor DNA sequence can be added to fill in the gap using homology-directed repair (HDR) 3B.

Not used clinically. Potential applications include removing virulence factors from pathogens, replacing disease-causing alleles of genes with healthy variants, and specifically targeting tumor cells.