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(54) Title: SYNERGISTIC EFFECT OF SMN1 AND MIR-23A IN TREATING SPINAL MUSCULAR ATROPHY

(57) Abstract: The present application relates to the fields of biotechnology, virology, genetics, and molecular biology. More specifically, the present invention relates to an isolated nucleic acid for producing a gene therapy viral product, said isolated nucleic acid comprising a nucleic acid that encodes the SMN1 protein having the amino acid sequence of SEQ ID NO: 1, and a nucleic acid that encodes the microRNA miR-23a, an expression cassette and a vector based thereon, as well as an AAV9-based recombinant virus for expressing the SMN1 gene in target cells, a pharmaceutical composition that includes said recombinant virus, and various uses of the above recombinant virus and the above composition.

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