

Abstract

The present application relates to the fields of genetics, gene therapy, and molecular biology. More specifically, the present invention relates to an isolated codon-optimized nucleic acid that encodes the SMN1 protein (survival motor neuron protein), an expression cassette and a vector based thereon, as well as an AAV9 (adeno-associated virus serotype 9)-based recombinant virus for increasing the expression of the SMN1 gene in target cells, and use thereof.