Claims:

1. An isolated nucleic acid for producing a gene therapy viral product, said isolated nucleic acid comprising a nucleic acid that encodes the SMN1 protein (survival motor neuron protein) having the amino acid sequence of SEQ ID NO: 1, and a nucleic acid that encodes the microRNA miR-23a.

2. The isolated nucleic acid according to claim 1, wherein the nucleic acid that encodes the SMN1 protein having the amino acid sequence of SEQ ID NO: 1 comprises the nucleotide sequence of SEQ ID NO: 2.

3. The isolated nucleic acid according to claim 1, wherein the microRNA miR-23a has the nucleotide sequence of SEQ ID NO: 3.

4. The isolated nucleic acid according to claim 1, wherein the nucleic acid that encodes the microRNA miR-23a comprises the nucleotide sequence of SEQ ID NO: 4.

5. An expression cassette that comprises the nucleic acid according to any one of claims 1 to 4.

3. The expression cassette according to claim 5, said expression cassette comprising the following elements in the 5'-end to 3'-end direction:

a left-hand (first) ITR (inverted terminal repeats);

a CMV (cytomegalovirus) enhancer;

a CMV (cytomegalovirus) promoter;

an intron of the hBG1 gene (hemoglobin subunit gamma 1 gene);

a nucleic acid that encodes the SMN1 protein;

an hGH1 polyadenylation signal (human growth hormone gene polyadenylation signal);

an SV40 promoter (simian virus 40 promoter);

a nucleic acid that encodes the microRNA miR-23a;

an SV40 polyadenylation signal (simian virus 40 polyadenylation signal), and

a right-hand (second) ITR.

7. The expression cassette according to claim 6, said expression cassette comprising a nucleic acid with SEQ ID NO: 6.

8. An expression vector that comprises the nucleic acid according to any one of claims 1 to 4 or the cassette according to any one of claims 5 to 7.

9. An AAV9 (adeno-associated virus serotype 9)-based recombinant virus for the expression of the SMN1 gene in target cells, said AAV9-based recombinant virus comprising a capsid and the expression cassette according to any one of claims 5 to 7.

10. The AAV9-based recombinant virus according to claim 9, wherein the capsid comprises the AAV9 protein VP1.

11. The AAV9-based recombinant virus according to claim 10, wherein the capsid comprises the AAV9 protein VP1 having the amino acid sequence of SEQ ID NO: 7.

12. The AAV9-based recombinant virus according to claim 10, wherein the capsid includes the AAV9 protein VP1 having the amino acid sequence of SEQ ID NO: 7 with one or more point mutations.

13. The AAV9-based recombinant virus according to any one of claims 9 to 12, wherein the capsid comprises the AAV9 protein VP1 having the amino acid sequence of SEQ ID NO: 7 or the amino acid sequence of SEQ ID NO: 7 with one or more point mutations, and the expression cassette comprises the following elements in the 5'-end to 3'-end direction:

a left-hand (first) ITR (inverted terminal repeats);

a CMV (cytomegalovirus) enhancer;

a CMV (cytomegalovirus) promoter;

an intron of the hBG1 gene (hemoglobin subunit gamma 1 gene);

a nucleic acid that encodes the SMN1 protein;

an hGH1 polyadenylation signal (human growth hormone gene polyadenylation signal);

an SV40 promoter (simian virus 40 promoter);

a nucleic acid that encodes the microRNA miR-23a;

an SV40 polyadenylation signal (simian virus 40 polyadenylation signal), and

a right-hand (second) ITR.

14. The AAV9-based recombinant virus according to claim 10, wherein the capsid comprises the AAV9 protein VP1 having the amino acid sequence of SEQ ID NO: 7 or the amino acid sequence of SEQ ID NO: 7 with one or more point mutations, and the expression cassette comprises a nucleic acid with SEQ ID NO: 6.

15. A pharmaceutical composition for delivering the SMN1 gene to target cells, said pharmaceutical composition comprising the AAV9-based recombinant virus according to claims 9 to 14 in combination with one or more pharmaceutically acceptable excipients.

16. Use of the AAV9-based recombinant virus according to claims 9 to 14 or the composition according to claim 15 for delivering the SMN1 gene to target cells.

17. Use of the AAV9-based recombinant virus according to claims 9 to 14 or the composition according to claim 15 for survival of a subject that has spinal muscular atrophy and/or that does not have fully functional copies of the SMN1 gene.

18. Use of the AAV9-based recombinant virus according to claims 9 to 14 or the composition according to claim 15 for providing the SMN1 protein to a subject that has spinal muscular atrophy and/or that does not have fully functional copies of the SMN1 gene.

19. Use of the AAV9-based recombinant virus according to claims 9 to 14 or the composition according to claim 15 for treating spinal muscular atrophy in a subject that has spinal muscular atrophy.