CLAIMS

1. An antisense nucleic acid molecule targeting a C9orf72 transcript, wherein the antisense nucleic acid molecule comprises or consists of a sequence as shown in SEQ ID NO: 1, a sequence having from 13 to 25 consecutive nucleotides of SEQ ID NO: 1, or a sequence having at least 85%, 90%, 95%, 96% , 97%, 98% or at least 99% identity with the sequence of SEQ ID NO:1.

2. The antisense nucleic acid molecule of claim 1, wherein the antisense nucleic acid molecule consists of SEQ ID NO: 1.

3. The antisense nucleic acid molecule of claim 1, wherein the antisense nucleic acid molecule comprises from 40 to 50 nucleotides and comprises a nucleic acid sequence as shown in SEQ ID NO: 1.

4. The antisense nucleic acid molecule according to any one of claims 1 to 3, wherein said antisense nucleic acid molecule is fused to a small nuclear RNA such as the U7 small nuclear RNA.

5. A nucleic acid construct comprising at least two antisense nucleic acid molecules, wherein at least one antisense nucleic acid molecule is the nucleic acid molecule according to anyone of claims 1 to 4.

6. The nucleic acid construct of claim 5 comprising a first antisense nucleic acid molecule targeting the sense C9orf72 transcript and a second antisense nucleic acid molecule targeting the antisense C9orf72 transcript, wherein the second antisense nucleic acid molecule is the nucleic acid molecule according to any one of claims 1 to 4.

7. A vector for delivering the antisense nucleic acid molecule according to any one of claims 1 to 4 or the nucleic acid construct of claims 5-6.

8. The vector according to claim 7, which is a viral vector coding said antisense nucleic acid molecule or said nucleic acid construct.

9. The vector according to claim 8, wherein said viral vector is an AAV vector, in particular an AAV9 or AAV10 vector.

10. The antisense nucleic acid molecule according to any one of claims 1 to 4, the nucleic acid construct of claims 5 or 6 or the vector according to any one of claims 7 to 9, for use as a medicament.

11. The antisense nucleic acid molecule according to any one of claims 1 to 4, the nucleic acid construct of claim 5 or 6 or the vector according to any one of claims 7 to 9, wherein said antisense nucleic acid molecule or said vector is for an administration via the intravenous and/or intracerebroventricular routes.

12. Use of the antisense nucleic acid molecule according to any one of claims 1 to 4, the nucleic acid construct of claim 5 or 6 or the vector according to any one of claims 7 to 9, in the preparation of a medicament for treating a C9orf72 hexanucleotide repeat expansion associated disease, wherein the disease is amyotrophic lateral sclerosis (ALS) or frontotemporal dementia (FTD), in particular amyotrophic lateral sclerosis (ALS).

13. Use according to claim 12, wherein said antisense nucleic acid molecule or said vector is to be administered via an intravenous and/or intracerebroventricular routes.