CLAIMS

1. A nucleic acid construct for use as a medicament, which comprises:

a) a nucleotide sequence of an eukaryotic promoter;

b) a nucleotide sequence encoding a truncated ATP7B in which the N-terminal Heavy-Metal-Associated sites HMA 1, HMA 2, HMA 3, and HMA 4 are not present and wherein HMA 5 and HMA 6 are present;

c) a polyadenylation signal sequence; and,

d) a 5' Terminal Repeat sequence and a 3'Terminal Repeat sequence of an adeno-associated virus (AAV).

2. The nucleic acid construct for use according to claim 1, wherein the N-terminal Heavy-Metal-Associated sites HMA 1, HMA 2, HMA 3 and HMA4 comprise amino acids 57 to 486 of SEQ ID NO: 2.

3. The nucleic acid construct for use according to any of claims 1 or 2, wherein the ATP7B comprises the amino acid sequence of SEQ ID NO: 7.

4. The nucleic acid construct for use according to claim 1, wherein the nucleotide sequence encoding the truncated ATP7B is selected from the group consisting of:

a) nucleotide bases 473 through 3580 of the nucleotide sequence of SEQ ID NO:6;

b) the nucleotide sequence SEQ ID NO: 8; and

c) a nucleotide sequence wherein at least 827 of the codons encoding the truncated ATP7B are identical to the codons of SEQ ID NO: 8; and

d) a nucleotide sequence encoding the amino acid sequence of SEQ ID NO: 7.

5. The nucleic acid construct for use according to any of claims 1 – 4, wherein the nucleotide sequence of the eukaryotic promoter is a nucleotide sequence of the α1-antitrypsin gene promoter, or a chimeric promoter sequence that comprises an α1-antitrypsin gene promoter sequence combined with an albumin gene enhancer element.

6. The nucleic acid construct for use according to any of claims 1 – 5, wherein the nucleotide sequence of the eukaryotic promoter is from nucleotide bases 156 through 460 of SEQ ID NO: 1 (AAT), or is the nucleotide sequence of SEQ ID NO: 5 (EalbPa1AT).

7. The nucleic acid construct for use according to claim 6, wherein the 5' ITR and 3' ITR sequences of an AAV are of a serotype selected from the group consisting of AAV1, AAV2, and AAV4, preferably are of the AAV2 serotype.

8. A viral expression vector for use as a medicament, comprising the nucleic acid construct according to any of claims 1 – 7.

9. The viral expression vector for use according to claim 8, wherein the vector is an AAV vector.

10. A recombinant AAV (rAAV) virion for use as a medicament that comprises a nucleic acid construct according to any of claims 1 – 7 or a viral expression vector according to claim 8 or claim 9.

11. The rAAV virion for use according to claim 10, wherein the rAAV virion further comprises a capsid protein of an AAV.

12. The rAAV virion for use according to claim 11, wherein the capsid protein of an AAV is of a serotype selected from the group consisting of: AAV1, AAV3, AAV5, AAV7, AAV8, AAV9 and AAV10.

13. The rAAV virion for use according to claim 12, wherein the 5' ITR and 3' ITR sequences of the nucleic acid construct are of an AAV2 serotype and the capsid protein is of an AAV8 serotype.

14. A pharmaceutical composition that comprises the nucleic acid construct according to any of claims 1–7, the expression vector according to claim 8-9, or a rAAV virion according to any of claims 10-13, and a pharmaceutically acceptable carrier.

15. The nucleic acid construct according to any of claims 1–7, the expression vector according to claims 8-9, or the rAAV virion according to any of claims 10-13 or a pharmaceutical composition according to claim 14, for use in the treatment of a condition caused by a deficiency or dysfunction of Copper-transporting ATPase 2, preferably in the treatment of Wilson's disease.

16 Use of a nucleic acid construct according to any of claims 1–7, the expression vector according to claims 8-9, the rAAV virion according to any of claims 10-13 or the pharmaceutical composition according to claim 14 in the preparation of a medicament for the treatment of a condition caused by a deficiency or dysfunction of Copper-transporting ATPase 2, preferably in the treatment of Wilson's disease.

17. An rAAV virion for use as a medicament, comprising:

a nucleic acid construct comprising :

a) a nucleotide sequence of an eukaryotic promoter;

b) a nucleotide sequence encoding a truncated ATP7B in which the N-terminal Heavy-Metal-Associated sites HMA 1, HMA 2, HMA 3, and HMA 4 are not present and HMA 5 and HMA 6 are present, and wherein the N-terminal Heavy-Metal-Associated sites HMA 1, HMA 2, HMA 3, and HMA 4 comprise amino acids 57 to 486 of SEQ ID NO:2;

c) a polyadenylation signal sequence; and

d) a 5' ITR sequence and a 3' ITR sequence of AAV2; and

e) a capsid protein of AAV3B.

18. An rAAV virion for use as a medicament comprising:

a nucleic acid construct comprising:

a) a nucleotide sequence of an eukaryotic promoter;

b) a nucleotide sequence encoding the amino acid sequence of SEQ ID NO:7;

c) a polyadenylation signal sequence; and

d) a 5' ITR sequence and a 3' ITR sequence of AAV2; and

e) a capsid protein of AAV3B.

19. A pharmaceutical composition comprising the rAAV virion of claim 17 and a pharmaceutically acceptable carrier or excipient.

20. A pharmaceutical composition comprising the rAAV virion of claim 18 and a pharmaceutically acceptable carrier or excipient.