liver-specific promoter; the nucleic acid sequence encoding the GAA protein; and a polyadenylation signal, the construct comprising preferably, in this order: an ApoE control region; a HBB2 intron, in particular a modified HBB2 intron; a hAAT promoter; the nucleic acid sequence encoding the truncated GAA polypeptide; and a bovine growth hormone polyadenylation signal. In specific embodiments, said nucleic acid construct more particularly comprises the nucleotide sequence of any one of SEQ ID NO:22 to 26.

In another aspect, the invention relates to a vector comprising the nucleic acid molecule or the nucleic acid construct herein disclosed. The vector of the invention may be in particular a viral vector, preferably a retroviral vector, such as a lentiviral vector, or an AAV vector. Preferably, the vector is a single-stranded or double-stranded self-complementary AAV vector, preferably an AAV vector with an AAV-derived capsid, such as an AAV1, AAV2, variant AAV2, AAV3, variant AAV3, AAV3B, variant AAV3B, AAV4, AAV5, AAV6, variant AAV6, AAV7, AAV8, AAV9, AAV10 such as AAVcy10 and AAVrh10, AAVrh74, AAVdj, AAV-Anc80, AAV-LK03, AAV2i8, a porcine AAV capsid, such as AAVpo4 and AAVpo6 capsid, or with a chimeric capsid. In a specific embodiment, the vector is an AAV vector with an AAV8, AAV9, AAVrh74 or AAV2i8 capsid, in particular an AAV8, AAV9 or AAVrh74 capsid, more particularly an AAV8 capsid.

In yet another aspect, the invention provides a cell transformed with the nucleic acid molecule, the nucleic acid construct or the vector of the invention. More particularly, the cell is a liver cell or a muscle cell.

In a particular aspect, the invention provides a pharmaceutical composition, comprising, in a pharmaceutically acceptable carrier, the truncated GAA polypeptide, the nucleic acid molecule, the nucleic acid construct, the vector, or the cell of the invention.

The invention further relates to the truncated GAA polypeptide, the nucleic acid molecule, the nucleic acid construct, the vector, or the cell of the invention, for use as a medicament.

The invention further provides the use of the truncated GAA polypeptide, the nucleic acid molecule, the nucleic acid construct, the vector, or the cell of the invention, in the manufacture of a medicament for treating a glycogen storage disease. In a particular embodiment, the glycogen storage disease is GSDI, GSDII, GSDIII, GSDIV, GSDV, GSDVI, GSDVII, GSDVIII or lethal congenital glycogen storage disease of the heart. In a more particular embodiment, the glycogen storage disease is selected in the group consisting of GSDI, GSDII and GSDIII, more particularly in the group consisting of GSDII and GSDIII. In an even more particular embodiment, the glycogen storage disease is GSDII.

LEGENDS TO THE FIGURES

13. A pharmaceutical composition, comprising, in a pharmaceutically acceptable carrier, the truncated GAA polypeptide of any one of claims 1 to 5, the nucleic acid molecule of claim 6, the nucleic acid construct of any one of claims 7 to 8, the vector of any one of claims 9 to 11, or the cell according to claim 12.

14. The truncated GAA polypeptide of any one of claims 1 to 5, the nucleic acid molecule of claim 6, the nucleic acid construct of any one of claims 7 to 8, the vector of any one of claims 9 to 11, or the cell according to claim 12, for use as a medicament.

15. Use of the truncated GAA polypeptide of any one of claims 1 to 5, the nucleic acid molecule of claim 6, the nucleic acid construct of any one of claims 7 to 8, the vector of any one of claims 9 to 11, or the cell according to claim 12, in the manufacture of a medicament for treating a glycogen storage disease, such as GSDI (von Gierke's disease), GSDII (Pompe disease), GSDIII (Cori disease), GSDIV, GSDV, GSDVI, GSDVII, GSDVIII and lethal congenital glycogen storage disease of the heart, more particularly GSDI, GSDII or GSDIII, even more particularly GSDII and GSDIII, and most particularly GSDII.