CLAIMS

1. Use of a vector, which comprises an adeno-associated virus (AAV) genome or a derivative thereof and a polynucleotide sequence encoding REP1 or a variant thereof in the manufacture of a medicament for the treatment or prevention of choroideremia.
2. The use of a vector according to claim 1, wherein said derivative is a chimeric, shuffled or capsid modified derivative.
3. The use of a vector according to claim 1 or 2, wherein said AAV genome is from a naturally derived serotype or isolate or clade of AAV.
4. The use of a vector according to claim 3, wherein said serotype is AAV serotype 2 (AAV2).
5. The use of a vector according to claim 4, which comprises SEQ ID NO: 1 or a derivative thereof.
6. The use of a vector according to any one of the preceding claims, wherein said polynucleotide sequence encodes a polypeptide having at least 70% homology to SEQ ID NO: 3 over its entire sequence.
7. The use of a vector according to claim 6, wherein said polynucleotide sequence has at least 70% homology to SEQ ID NO: 2 over its entire sequence.
8. The use of a vector according to any one of the preceding claims, which comprises a promoter sequence operably linked to said polynucleotide sequence encoding REP1 or a variant thereof.
9. The use of a vector according to claim 8, wherein said promoter is constitutively active.
10. The use of a vector according to claim 8, wherein expression from said promoter is retinal-cell specific.
11. The use of a vector according to any one of the preceding claims, which comprises one or more additional regulatory sequences.
12. The use of a vector according to claim 11, which comprises a sequence having at least 70% homology to SEQ ID NO: 5 over its entire sequence.
13. The use of a vector according to any of the preceding claim in the form of a vector particle.
14. The use of a vector according to claim 13 wherein the vector particle is serotype AAV2.

15. The use of a vector according to any preceding claim wherein the vector is to be administered to said patient by direct retinal, subretinal or intravitreal injection.

16. The use of a vector according to claim 15, wherein said vector is to be administered directly into the subretinal space.

CLAIMS

1. A vector, which comprises an adeno-associated virus (AAV) genome or a derivative thereof and a polynucleotide sequence encoding REP1 or a variant thereof.
2. A vector according to claim 1, wherein said derivative is a chimeric, shuffled or capsid modified derivative.
3. A vector according to claim 1 or 2, wherein said AAV genome is from a naturally derived serotype or isolate or clade of AAV.
4. A vector according to claim 3, wherein said serotype is AAV serotype 2 (AAV2).
5. A vector according to claim 4, which comprises SEQ ID NO: 1 or a derivative thereof.
6. A vector according to any one of the preceding claims, wherein said polynucleotide sequence encodes a polypeptide having at least 70% homology to SEQ ID NO: 3 over its entire sequence.
7. A vector according to claim 6, wherein said polynucleotide sequence has at least 70% homology to SEQ ID NO: 2 over its entire sequence.
8. A vector according to any one of the preceding claims, which comprises a promoter sequence operably linked to said polynucleotide sequence encoding REP1 or a variant thereof.
9. A vector according to claim 8, wherein said promoter is constitutively active.
10. A vector according to claim 8, wherein expression from said promoter is retinal-cell specific.
11. A vector according to any one of the preceding claims, which comprises one or more additional regulatory sequences.
12. A vector according to claim 11, which comprises a sequence having at least 70% homology to SEQ ID NO: 5 over its entire sequence.

13. Use of a vector according toany one of claims 1-12 in the manufacture of a medicament for treating or preventing choroideremia in a patient in need thereof; wherein the vector is to be administered to said patient by direct retinal, subretinal or intravitreal injection.

14. The use according to claim 13, wherein said vector is to be administered directly into the subretinal space.

15. A vector according to any one of claims 1 to 12 for use in a method of treating or preventing choroideremia by administering said vector to a patient by direct retinal, subretinal or intravitreal injection.