In a preferred example, a biotin-labelled lipidated Rab6a product is detected using a detection reagent specific for biotin, for example streptavidin. Preferably, the biotin-labelled lipidated Rab6a product is detected using Western blot analysis using a detection reagent specific for biotin, for example streptavidin (e.g. a streptavidin-horseradish peroxidase conjugate). More preferably, the biotin-labelled lipidated Rab6a product is detected using an ELISA using a detection reagent specific for biotin, for example streptavidin.

In one example, the method is for determining the activity of a REP1-encoding gene therapy vector for use in the treatment of choroideremia.

In another aspect, the present disclosure refers to use of Rab6a for determining *in vitro* the activity of Rab escort protein 1 (REP1)-encoding gene therapy vector for use in the treatment of choroideremia, wherein the Rab6a is contacted with Rab geranylgeranyltransferase (Rab GGTase), a lipid donor substrate, and REP1 from a cell genetically engineered to express the REP1 using the REP1-encoding gene therapy vector.

The method of determining the activity of REP1, the Rab6a, Rab GGTase, lipid donor substrate and the REP1 may be as described herein.

Disclosed is a method for determining the efficacy of a vector comprising a Rab escort protein 1 (REP1) encoding nucleotide sequence, wherein the method comprises the steps:

(a) providing a sample comprising REP1, wherein the REP1 is expressed using the vector comprising a REP1-encoding nucleotide sequence;

(b) contacting the sample of step (a) with Rab6a, Rab geranylgeranyltransferase (Rab GGTase) and a lipid donor substrate; and

(c) detecting the lipidated Rab6a product.

Disclosed is the use of Rab6a for determining the efficacy of a vector comprising a Rab escort protein 1 (REP1) encoding nucleotide sequence.

Preferably, the method and use are for determining the efficacy of a vector for use in the treatment of choroideremia.

The vector, REP1, Rab6a, Rab GGTase, lipid donor substrate, lipidated Rab6a product, method of detection and other features of the method and use may be as described herein.

DESCRIPTION OF THE DRAWINGS

Figure 1

CLAIMS

1. An *in vitro* method for determining the activity of Rab escort protein 1 (REP1)-encoding gene therapy vector for use in the treatment of choroideremia, the method comprising the steps:

    (a) contacting a sample comprising REP1 with Rab6a, Rab geranylgeranyltransferase (Rab GGTase) and a lipid donor substrate, wherein the sample comprising REP1 is from a cell genetically engineered to express REP1 using the REP1-encoding gene therapy vector; and

    (b) detecting the lipidated Rab6a product.

2. The method of claim 1, wherein the REP1 encoding gene therapy vector is a viral vector comprising a REP1-encoding nucleotide sequence.

3. The method of claim 2, wherein the viral vector is an adeno-associated viral (AAV) vector, preferably, wherein the adeno-associated viral vector is AAV serotype 2 (AAV2).

4. The method of any preceding claim, wherein the Rab6a and/or Rab GGTase are pure.

5. The method of any preceding claim, wherein the Rab6a:Rab GGTase molar ratio is 1:2-3, preferably 1:2.5.

6. The method of any preceding claim, wherein the lipid donor substrate is geranylgeranylpyrophosphate (GGPP) or an analogue thereof, preferably biotin-geranylpyrophosphate (BGPP).

7. The method of any preceding claim, wherein the lipidated Rab6a product is detected using an enzyme-linked immunosorbent assay (ELISA), Western blot analysis or autoradiography.

8. The method of any preceding claim, wherein the method is for quality control analysis of a REP1-encoding gene therapy vector for use in the treatment of choroideremia.

9. The method of any preceding claim, wherein the detection of the lipidated Rab6a product comprises quantifying the amount of the lipidated Rab6a product, preferably quantifying the amount relative to a control or reference level.

10. Use of Rab6a for determining *in vitro* the activity of Rab escort protein 1 (REP1)-encoding gene therapy vector for use in the treatment of choroideremia, wherein the Rab6a is contacted with Rab geranylgeranyltransferase (Rab GGTase), a lipid donor substrate, and REP1 from a cell genetically engineered to express the REP1 using the REP1-encoding gene therapy vector.

11. The use of claim 10, wherein the REP1 is in a lysate from the cell genetically engineered to express the REP1.

12. The use of claim 10 or 11, wherein the REP1 is expressed using a viral vector comprising a REP1-encoding nucleotide sequence.

13. The use of claim 12, wherein the viral vector is an adeno-associated viral (AAV) vector.

14. The use of any one of claims 10-13, wherein the Rab6a is pure.

15. The method of any one of claims 1-9 or the use of any one of claims 10-14, wherein the REP-1 encoding nucleotide sequence of the REP1-encoding gene therapy vector comprises a nucleotide sequence that encodes an amino acid sequence that has at least 80% identity to SEQ ID NO: 5, wherein the amino acid sequence substantially retains the natural function of the protein represented by SEQ ID NO: 5.