Claims:

1. Use of acetyl-leucine, or a pharmaceutically acceptable salt thereof, in the manufacture of a medicament, for delaying progression of Niemann-Pick Type C disease or one or more symptoms of Niemann-Pick Type C disease over time as compared to typical disease progression in a subject in need thereof, wherein the subject is asymptomatic.

2. The use according to claim 1, wherein the subject has been found to have a genetic and/or biochemical marker of Niemann-Pick Type C disease.

3. Use of acetyl-leucine, or a pharmaceutically acceptable salt thereof, in the manufacture of a medicament, for delaying onset of one or more symptoms of Niemann-Pick Type C disease that would otherwise be expected to manifest according to typical disease progression.

4. The use according to any of claims 1-3, wherein a therapeutically effective amount of acetyl-leucine is to be administered to the subject in need thereof when the subject is asymptomatic.

5. The use according to any of claims 1-3, wherein a therapeutically effective amount of the acetyl-leucine is to be administered to the subject in need thereof for a duration chosen from at least about 3 months, at least about 6 months, at least about 1 year, at least about 2 years, and at least about 5 years.

6. The use according to any of claims 1-5, wherein the acetyl-leucine is acetyl-DL-leucine.

7. The use according to any of claims 1-5, wherein the acetyl-leucine has an enantiomeric excess of the L-enantiomer or the D-enantiomer.

8. The use according to any of claims 1-5, wherein the acetyl-leucine is in a single enantiomeric form of either the L-enantiomer or the D-enantiomer.

9. The use according claim 8, wherein the single enantiomeric form is the L-enantiomer.

10. The use according to any of claims 1-3, wherein the acetyl-leucine is to be administered to the subject in need thereof at a therapeutically effect amount chosen from 1 g to 15 g per day, from 1 g to 10 g per day, from 1.5 g to 7 g per day, from 4 g to 6 g per day, or from 4 g to 5 g per day.

11. The use according to any of claims 4-5, wherein the therapeutically effective amount of acetyl-leucine is chosen from 1 g to 15 g per day, from 1 g to 10 g per day, from 1.5 g to 7 g per day, from 4 g to 6 g per day, or from 4 g to 5 g per day.

12. Use of acetyl-leucine, or a pharmaceutically acceptable salt thereof, in the manufacture of a medicament, for providing neuroprotection in a subject having, suspected of having, or at risk of having Niemann-Pick Type C disease, wherein a therapeutically effective amount of the acetyl-leucine is to be administered to the subject for a duration chosen from at least about 3 months, at least about 6 months, at least about 1 year, at least about 2 years, and at least about 5 years.