Claims

1. Use of a recombinant human acid sphingomyelinase (rhASM) or modified rhASM for an acid sphingomyelinase (ASM) deficiency for the manufacture of a medicament for treating the deficiency in a human subject, wherein

   (a) the medicament is to be administered to the subject; and

   (b) the level of lyso-sphingomyelin (lyso-SPM) in a biological sample collected from the subject three or more days after the administration is obtained, wherein the biological sample is whole blood, a dried blood spot, plasma, or serum, and wherein a decrease of the level of lyso-SPM compared to a reference level indicates efficacy of the medicament.

2. The use of claim 1, wherein the medicament is to be administered repeatedly to the subject.

3. The use of claim 1 or 2, wherein additional doses of the medicament are to be administered to the subject.

4. The use of any one of the preceding claims, wherein the reference level is the baseline lyso-SPM level of the subject before any treatment with the rhASM or modified rhASM.

5. The use of any one of claims 2-4, wherein the biological sample is to be collected prior to a subsequent dose.

6. The use of any one of claims 1-5, wherein the ASM deficiency is Niemann-Pick Disease type B.

7. The use of any one of claims 1-5, wherein the ASM deficiency is Niemann-Pick Disease type A.

8. The use of any one of the preceding claims, wherein a maintenance dose of the rhASM or modified rhASM at a dose concentration equal to or less than the highest dose concentration of the rhASM or modified rhASM is to be administered to the subject.

9. The use of any one of the preceding claims, wherein each successive dose of the rhASM or modified rhASM is to be administered two weeks after the previous dose.

10. The use of any one of the preceding claims, wherein one or more dose concentrations of the rhASM or modified rhASM are to be administered at least twice.

11. The use of any one of the preceding claims, wherein each dose of the rhASM or modified rhASM is to be administered at 0.03 mg/kg to 3 mg/kg.

12. The use of any one of the preceding claims, wherein the first dose of the rhASM or modified rhASM that the subject receives is to be administered at 0.1 mg/kg.

13. The use of claim 12, wherein a maintenance dose of the rhASM or modified rhASM at 1, 2, or 3 mg/kg is to be administered to the subject.

14. The use of any one of the preceding claims, wherein the rhASM or modified rhASM is to be administered to the subject intravenously.

15. Use of a recombinant human acid sphingomyelinase (rhASM) for the manufacture of a medicament for treating Niemann-Pick Disease type A or type B in a human subject, wherein:

    (a) a starting dose of rhASM at 0.03 or 0.1 mg/kg and subsequent doses that increase in dose concentration until reaching 3 mg/kg are to be administered to the human subject;

    (b) maintenance doses are to be administered to the subject at 3 mg/kg or less; and

    (c) the level of lyso-sphingomyelinase (lyso-SPM) in a biological sample collected from the subject (i) within 24 hours, (ii) within 48 hours, or (iii) 72 or more hours after administration of a dose is to be obtained;

    wherein the biological sample is a dried blood spot, whole blood, plasma, or serum;

    wherein each dose is to be administered two weeks after the previous dose, and wherein a decrease of the level of lyso-SPM compared to a reference level indicates efficacy of the medicament.

16. The use of claim 15, wherein the maintenance doses are to be administered at 1, 2, or 3 mg/kg.

17. The use of claim 15 or 16, wherein the subject has been determined prior to treatment to have an elevated level of lyso-SPM compared to a healthy control.

18. The use of any one of claims 15-17, wherein the biological sample is to be collected prior to a subsequent dose.

19. The use of any one of the preceding claims, wherein the subject is an adult patient.

20. The use of any one of the preceding claims, wherein the subject is a pediatric patient.