CLAIMS

1. Use of a composition comprising apitegromab in the manufacture of a medicament for the treatment of spinal muscular atrophy (SMA) in a human subject, wherein the treatment comprises intravenous administration of apitegromab in an amount of greater than 2 mg/kg and no more than 20 mg/kg at an interval of once every four weeks or once a month for at least 24 weeks or six months to treat SMA.

2. The use according to claim 1, wherein the administration is sufficient to achieve a clinical benefit.

3. The use according to claim 1 or claim 2, wherein the treatment is sufficient to prevent loss of motor function.

4. The use according to claim 1 or claim 2, wherein the treatment is sufficient to delay loss of motor function.

5. The use according to claim 1 or claim 2, wherein the treatment is sufficient to to increase motor function.

6. The use according to any one of claims 3-5, wherein the motor function is measured by a Hammersmith Functional Motor Scale Expanded score (HFMSE) at 6 months after the start of treatment relative to HFMSE at baseline.

7. The use according to any one of claims 1-6, wherein the apitegromab is to be administered for at least 52 weeks or twelve months.

8. The use according to claim 1, wherein the administration is sufficient (a) to prevent or delay a reduction in an HFMSE score relative to a baseline prior to treatment; (b) for the human subject to achieve an increase in an HFMSE score relative to baseline, an increase in a Revised Upper Limb Module relative to baseline, an increase in the Revised Hammersmith Score relative to baseline, or an increase in a number of WHO motor development milestones relative to baseline; and/or (c) for the human subject to achieve an increase in an HFMSE score of at least one point, at least two points or at least three points relative to baseline.

9. The use according to any of the preceding claims, wherein the human subject is 2 years of age or older.

10. The use according to any of the preceding claims, wherein the human subject is 2-21 years of age.

11. The use according to any of the preceding claims, wherein the human subject is 2-10 years of age.

12. The use according to any of the preceding claims, wherein the human subject is 2-5 years of age.

13. The use according to any of the preceding claims, wherein the spinal muscular atrophy is later onset SMA.

14. The use according to claim 13, wherein the later-onset SMA is Type 2 SMA or non-ambulatory Type 3 SMA.

15. The use according to claim 13, wherein the later-onset SMA is ambulatory Type 3 SMA.

16. The use according to any of the preceding claims, wherein apitegromab is to be administered intravenously in an amount of 10 mg/kg.

17. The use according to any one of claims 1-15, wherein apitegromab is to be administered intravenously in an amount of 20 mg/kg.

18. The use according to any of the preceding claims, wherein the subject has an SMN1 mutation and at least two copies of the SMN2 gene.

19. The use according to any of the preceding claims wherein the human subject is also to be administered an SMN upregulation therapy.

20. The use according to claim 19, wherein the SMN upregulation therapy is selected from nusinersen and/or risdiplam.

21. The use according to any of the preceding claims, wherein the baseline serum concentration of latent myostatin in the human subject selected for treatment is at least 1 ng/mL (e.g., greater than 1.5 ng/ml, greater than 2 ng/ml, greater than 2.5 ng/ml, or greater than 3 ng/ml), and optionally wherein the human subject has later-onset SMA, preferably type 2 SMA.

22. Use of a composition comprising apitegromab in the manufacture of a medicament for the treatment of spinal muscular atrophy (SMA) in a human subject, wherein the treatment comprises administration of apitegromab in an amount sufficient to

(a) achieve a serum concentration of latent myostatin in the human subject of at least 250 ng/ml (e.g., 550-2400 ng/ml) at steady state after the administration of apitegromab; and/or

(b) achieve a serum concentration of apitegromab in the human subject of at least 50 ug/ml at steady state after the administration of apitegromab.

23. The use according to claim 22, wherein the steady state is at 14 days or later after administration of apitegromab.