CLAIMS

1. Use of a therapeutically effective amount of fitusiran in the manufacture of a medicament for treating a hemophilia patient in need thereof, wherein the medicament is to be subcutaneously administered to the hemophilia patient in need thereof, wherein the medicament improves a patient-reported outcome (PRO) in the hemophilia patient, optionally wherein the patient is a hemophilia A or B patient with or without inhibitors and optionally wherein the PRO is improved in one or more quality of life (QoL) domains.

2. Use of fitusiran in the manufacture of a medicament for treating a hemophilia patient in need thereof, wherein the medicament is to be subcutaneously administered to the hemophilia patient in need thereof, wherein the medicament improves quality of life (QoL) in the hemophilia patient, wherein the QoL is improved in one or more QoL domains and optionally wherein the patient is a hemophilia A or B patient with or without inhibitors.

3. The use of claim 1 or 2, wherein fitusiran is to be administered at about 10 to about 90 mg per dose.

4. The use of any one of claims 1-3, wherein the one or more QoL domains are domains in a QoL questionnaire, optionally wherein the QoL questionnaire is Haemophilia Quality of Life Questionnaire for Adults (Haem-A-QoL).

5. The use of claim 4, wherein the medicament results in a clinically meaningful improvement indicated by a reduction of 7 or more units, optionally 8 or more, 9 or more, or 10 or more units, in one or more of Total Score, Sports and Leisure domain score, and Physical Health domain score of the questionnaire.

6. The use of any one of claims 1-3, wherein the one or more QoL domains are domains in a QoL questionnaire, optionally wherein the QoL questionnaire is Haemophilia Quality of Life Questionnaire for children and adolescents (Haemo-QoL).

7. The use of claim 6, wherein the medicament results in a clinically meaningful improvement indicated by a reduction of 7 or more units, optionally 8 or more, 9 or more, or 10 or more units, in the Total Score of the questionnaire.

8. The use of any one of claims 1-3, wherein the one or more QoL domains are domains in a QoL questionnaire, optionally wherein the QoL questionnaire is Hemophilia Activities List (HAL).

9. The use of claim 8, wherein the medicament results in an improvement indicated by an increase of 7 or more units, optionally 8 or more, 9 or more, or 10 or more units, in the Total Score of the questionnaire.

10. The use of any one of claims 1-3, wherein the one or more QoL domains are domains in a QoL questionnaire, optionally wherein the QoL questionnaire is pediatric Hemophilia Activities List (pedHAL).

11. The use of claim 10, wherein the medicament results in an improvement indicated by an increase of 7 or more units, optionally 8 or more, 9 or more, or 10 or more units, in the Total Score of the questionnaire.

12. The use of any one of claims 1-3, wherein the one or more QoL domains are domains in a QoL questionnaire, optionally wherein the QoL questionnaire is Treatment Satisfaction Questionnaire for Medication Version 9 (TSQM-9).

13. The use of claim 12, wherein the medicament results in an improvement indicated by an increase of 7 or more units, optionally 8 or more, 9 or more, or 10 or more units, in one or more of Effectiveness, Satisfaction, and Convenience domain score of the questionnaire.

14. The use of any one of claims 1-3, wherein the one or more QoL domains are domains in a QoL questionnaire, optionally wherein the QoL questionnaire is EuroQol 5-Dimensions (EQ-5D-5L).

15. The use of claim 14, wherein the medicament results in an improvement indicated by an increase of 0.02 or more units, optionally 0.03 or more, 0.04 or more, or 0.05 or more units, in the total score of the questionnaire or an increase of 5 or more units, optionally 6 or more, 7 or more, or 8 or more units, in the visual analog scale (VAS) score of the questionnaire.

16. The use of any one of claims 1-15, wherein the patient is an adult or adolescent patient twelve years or older with hemophilia A or B with or without inhibitors.

17. The use of any one of claims 1-16, wherein the patient has hemophilia A.

18. The use of claim 17, wherein the patient has been treated with factor VIII or a bypassing agent (BPA).

19. The use of claim 18, wherein the patient is with inhibitors, optionally wherein the level of inhibitors is more than 0.6 BU/mL as determined by Bethesda inhibitor assay.

20. The use of claim 17 or 18, wherein the patient is without inhibitors.

21. The use of any one of claims 1-16, wherein the patient has hemophilia B.

22. The use of claim 21, wherein the patient has been treated with factor IX or a BPA.

23. The use of claim 22, wherein the patient is with inhibitors, optionally wherein the level of inhibitors is more than 0.6 BU/mL as determined by Bethesda inhibitor assay.

24. The use of claim 21 or 22, wherein the patient is without inhibitors.

25. The use of any one of claims 18-20 or 22-24, wherein the BPA is activated prothrombin complex concentrates (aPCC) and/or recombinant activated Factor VII (rFVIIa).

26. The use of any one of claims 1-25, wherein a plurality of doses of fitusiran is to be administered to the patient at about 10 mg, about 20 mg, or about 50 mg per dose.

27. The use of any one of claims 1-26, wherein fitusiran is to be administered to the patient about once every four weeks or about once a month or about once every eight weeks or about once every two months.

28. The use of any one of claims 1-25, wherein fitusiran is to be administered to the patient at a dose of about 50 mg about once every two months or about once every eight weeks.

29. The use of any one of claims 1-27, wherein fitusiran is to be provided at a concentration of about 1 to about 200 mg/mL, optionally at a concentration of about 6.25, about 12.5 mg/mL, or about 100 mg/mL, in a phosphate-buffered saline (pH 7).

30. The use of any one of claims 1-28, wherein the patient does not have
(i) presence of clinically significant liver disease,
(ii) ALT >1.5× upper limit of normal reference range (ULN),
(iii) AST >1.5× upper limit of normal reference range (ULN),
(iv) hepatitis C,
(v) hepatitis A,
(vi) hepatitis E, and/or
(vii) hepatitis B.

31. An article of manufacture for use according to any one of claims 1-30.

32. The article of manufacture for use of claim 31, wherein the article of manufacture is a single-use container containing

i. about 50 mg of fitusiran in about 0.5 mL of a phosphate-buffered saline (pH 7),
ii. about 20 mg of fitusiran in about 0.2 mL of a phosphate-buffered saline (pH 7), or
iii. about 10 mg of fitusiran in about 0.1 mL of a phosphate-buffered saline (pH 7).

33. The article of manufacture for use of claim 31, wherein the article of manufacture is a single-use prefilled syringe containing

i. about 50 mg of fitusiran in about 0.5 mL of a phosphate-buffered saline (pH 7),
ii. about 20 mg of fitusiran in about 0.2 mL of a phosphate-buffered saline (pH 7), or
iii. about 10 mg of fitusiran in about 0.1 mL of a phosphate-buffered saline (pH 7).