

CLAIMS

1. Use of fitusiran in the manufacture of a medicament for prophylactic treatment of hemophilia A or B in a patient with or without inhibitors,
 - (a) wherein the medicament is to be subcutaneously administered to the patient in need thereof at a starting dose amount at a selected dosing frequency;
 - (b) wherein a measurement of an antithrombin (AT) level is to be obtained in the patient; and
 - (c) wherein:
 - (i) if the AT level is 15-35%, the medicament is to be further administered according to (a),
 - (ii) if the AT level is >35%, the medicament is to be further subcutaneously administered to the patient at a higher dose amount at the selected dosing frequency, or at the starting dose amount at a higher dosing frequency, or
 - (iii) if the AT level is <15%, the treatment is to be discontinued or paused.
2. The use of claim 1, wherein the thrombotic risk in the patient is reduced.
3. The use of claim 1 or 2, wherein the starting dose amount of fitusiran is selected from about 10 mg to about 50 mg.
4. The use of any one of claims 1-3, wherein the selected dosing frequency is about every two months (Q2M) or about every eight weeks (Q8W), optionally wherein the higher dosing frequency is about every month (QM) or about every four weeks (Q4W).
5. The use of claim 4, wherein the starting dose amount is about 20 mg and the selected dosing frequency is about Q2M or about Q8W.
6. The use of claim 4, wherein the starting dose amount is about 50 mg and the selected dosing frequency is about Q2M or about Q8W, optionally wherein

the medicament is to be administered according to (c)(ii) if two measurements of the AT level are >35%, or

the treatment is to be discontinued or paused according to (c)(iii) if more than one measurement, optionally two measurements, of the AT level is <15%.

7. The use of claim 6, wherein according to (c)(ii) the medicament is to be administered subcutaneously to the patient at a dose of about 50 mg fitusiran about QM.

8. The use of claim 7, wherein the medicament is to be administered subcutaneously to the patient at a dose of about 80 mg fitusiran about QM if the AT level in the patient following treatment according to (c)(ii) is >35%, optionally according to two measurements.

9. The use of any one of claims 6-8, wherein, after discontinuing or pausing treatment according to (c)(iii):

(A) the medicament is to be administered to the patient subcutaneously at a dose of about 20 mg of fitusiran about Q2M; and

(B) if the AT level of the patient treated according to (A) is
<15%, optionally according to more than measurement, fitusiran treatment is to be discontinued,

15-35%, the medicament is to be further administered according to (A), or
>35%, optionally according to two measurements, the medicament is to be administered subcutaneously to the patient at a dose of about 20 mg fitusiran about QM.

10. The use of any one of the preceding claims, wherein the medicament is to be administered subcutaneously to the patient at a dose amount and a dosing frequency sufficient to maintain the AT level in the patient at 15-35%, optionally wherein the medicament is to be administered subcutaneously at a dose of:

about 10 mg fitusiran about QM or about Q4W,
about 20 mg fitusiran about QM or about Q4W,
about 20 mg fitusiran about Q2M or about Q8W,
about 50 mg fitusiran about QM or about Q4W,

about 50 mg fitusiran about Q2M or about Q8W, or
about 80 mg fitusiran about QM or about Q4W.

11. The use of any one of the preceding claims, wherein each AT level measurement is to be obtained after the patient has received at least two doses of the medicament at a given dose amount.
12. Use of fitusiran in the manufacture of a medicament for prophylactic treatment of hemophilia A or B in a patient with or without inhibitors, wherein the medicament is to be administered subcutaneously to the patient in need thereof at a dose of about 50 or about 80 mg of fitusiran about every two months or about every eight weeks.
13. Use of fitusiran in the manufacture of a medicament for prophylactic treatment of hemophilia A or B in a patient with or without inhibitors, wherein the medicament is to be administered subcutaneously to the patient in need thereof at a dose of about 20 mg of fitusiran about every month or about every four weeks, or about every two months or about every eight weeks.
14. Use of fitusiran in the manufacture of a medicament for prophylactic treatment of hemophilia A or B in a patient with or without inhibitors, wherein the medicament is to be administered subcutaneously to the patient in need thereof at a dose of about 10 mg of fitusiran about every month or about every four weeks.
15. The use of any one of the preceding claims, wherein measurement of an AT level in the patient is to be obtained about every four weeks, about every eight weeks, about every month, about every two months, about every four months, about every six months, or about every 12 months.
16. The use of any one of claims 1-15, wherein the patient is twelve years of age or older.

17. The use of any one of claims 1-16, wherein fitusiran is provided in a phosphate-buffered saline (PBS) at about 50-200 mg/mL, optionally about 100 mg/mL, and optionally wherein the PBS has a pH of about 7.
18. An article of manufacture, wherein the article of manufacture is a container containing one or more doses of fitusiran, each dose being about 80 mg, about 50 mg, about 20 mg, or about 10 mg,
optionally wherein
the about 80 mg of fitusiran is in about 0.8 mL of a phosphate-buffered saline (PBS),
the about 50 mg of fitusiran is in about 0.5 mL of a PBS,
the about 20 mg of fitusiran is in about 0.2 mL of a PBS, or
the about 10 mg of fitusiran is in about 0.1 mL of a PBS, and
optionally wherein the PBS has a pH of about 7.
19. The article of manufacture of claim 18, wherein the article of manufacture is a kit.
20. The article of manufacture of claim 18 or 19, wherein the container is a single-use, single-dose prefilled syringe.