

Claims

- 1) Use of a gene therapy vehicle comprising a nucleic acid that encodes a coagulation factor having Factor IX activity in the manufacture of a medicament for preventing, arresting and/or treating hemophilic arthropathy of a joint in a patient having hemophilia B.
- 2) Use of the gene therapy vehicle according to claim 1, wherein the patient having hemophilia B has level of Factor IX activity of less than 1 IU/dL, or from 1 to up to 5 IU/dL, or from 5 to up to 40 IU/dL; preferably, the patient has a pre-treatment (baseline) level of Factor IX activity from 1 to up to 40 IU/dL.
- 3) Use of the gene therapy vehicle according to claim 1 or 2, wherein the vehicle is a viral vector, preferably an AAV based particle.
- 4) Use of the gene therapy vehicle according to claim 3, wherein the AAV based particle is an AAV5 particle.
- 5) Use of the gene therapy vehicle according to any one of the preceding claims, wherein the nucleic acid comprising a wildtype Factor IX, or a hyperactive variant of Factor IX; preferably, the nucleic acid comprises a hyperactive variant of Factor IX, for example FIX-R338L.
- 6) Use of the gene therapy vehicle according to any one of the preceding claims, wherein the nucleic acid further comprises a promoter, for example a liver-specific promoter.
- 7) Use of the gene therapy vehicle according to any one of the preceding claims, wherein the gene therapy vehicle is to be administered at a dose of from 8×10^{10} vg/kg to 2×10^{13} vg/kg.
- 8) Use of the gene therapy vehicle according to any one of the preceding claims, wherein the gene therapy vehicle is to be administered in a single dose.
- 9) Use of the gene therapy vehicle according to any one of the preceding claims, wherein the joint is selected from the group consisting of one or both elbows, one or both knees, one or both ankles, and any combination thereof.
- 10) Use of the gene therapy vehicle according to any one of preceding claims, wherein the hemophilic arthropathy comprises joint bleeding and/or synovitis.
- 11) Use of the gene therapy vehicle according to any one of the preceding claims, wherein the patient is a human.

- 12) Use of a pharmaceutical composition comprising a gene therapy vehicle according to any one of preceding claims in the manufacture of a medicament for preventing, arresting and/or treating hemophilic arthropathy of a joint in a patient having hemophilia B.
- 13) Use of the pharmaceutical composition according to claim 12, wherein the pharmaceutical composition is in a form suitable for intravenous infusion.
- 14) Use of the pharmaceutical composition according to claim 13, wherein the pharmaceutical composition is a liquid, or a lyophilized solid.
- 15) A method of preventing, arresting and/or treating hemophilic arthropathy of a joint in a patient having hemophilia B, comprising administering to the patient an effective amount of a gene therapy vehicle for use according to any one of claims 1 to 11, or a pharmaceutical composition for use according to any one of claims 12 to 14.
- 16) The method of claim 15, wherein the gene therapy vehicle for use, or the pharmaceutical composition for use, is administered via intravenous infusion.
- 17) The method of claim 15 or 16, wherein the patient has a pre-treatment baseline HJHS 2.1 score and a post-treatment HJHS 2.1 score, the post-treatment HJHS 2.1 score being no higher than the pre-treatment baseline HJHS 2.1 score.
- 18) The method of claim 17, wherein the post-treatment HJHS 2.1 score is lower than the pre-treatment baseline HJHS 2.1 score; preferably, the post-treatment HJHS 2.1 score is at least 2 points lower than the pre-treatment baseline HJHS 2.1 score; more preferably, the post-treatment HJHS 2.1 score is at least 4 points lower than the pre-treatment baseline HJHS 2.1 score
- 19) The method of claim 17 or 18, wherein the post-treatment HJHS 2.1 score is taken at least 1 year after the administration of the gene therapy vehicle for use or the pharmaceutical composition for use; preferably, the post-treatment HJHS 2.1 score is taken at least 2 years after the administering of the gene therapy vehicle for use or the pharmaceutical composition for use.