

Claims

- 1) Use of a gene therapy vehicle comprising a nucleic acid encoding a hyperactive variant of Factor IX which is FIX-R338L 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 a 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 further comprises a promoter, for example a liver-specific promoter.
- 6) 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.
- 7) 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.
- 8) 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.
- 9) Use of the gene therapy vehicle according to any one of preceding claims, wherein the hemophilic arthropathy comprises joint bleeding and/or synovitis.
- 10) Use of the gene therapy vehicle according to any one of the preceding claims, wherein the patient is a human.
- 11) Use, according to any one of claims 1 to 8, this being use of a pharmaceutical composition comprising the gene therapy vehicle which comprises a nucleic acid encoding a hyperactive variant of Factor IX which is FIX-R338L in the manufacture of a medicament for preventing, arresting and/or treating hemophilic arthropathy of a joint in a patient having hemophilia B.
- 12) Use according to claim 11, wherein the pharmaceutical composition is in a form suitable for intravenous infusion.

- 13) Use according to claim 12, wherein the pharmaceutical composition is a liquid, or a lyophilized solid.
- 14) The use of any preceding claim, wherein the gene therapy vehicle is to be administered via intravenous infusion.
- 15) The use of claim 14, wherein the gene therapy vehicle is to be administered to a patient having 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.
- 16) The use of claim 15, 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
- 17) The use of claim 15 or 16, wherein the post-treatment HJHS 2.1 score is to be taken at least 1 year after the administration of the gene therapy vehicle; preferably, the post-treatment HJHS 2.1 score is to be taken at least 2 years after the administration of the gene therapy vehicle.