CLAIMS

1. Use of a composition in the manufacture of a medicament for the treatment of a hereditary bleeding disorder in a subject wherein the composition comprises a VWF targeting agent.

2. The use of claim 1, wherein the hereditary bleeding disorder is an acquired platelet function defect, congenital platelet function defects, disseminated intravascular coagulation (DIC), prothrombin deficiency, fibrinogen deficiency, FV deficiency, FVII deficiency, FX deficiency, FXI deficiency (hemophilia C), FXIII deficiency, combined FV and FVIII deficiency (F5F8D), VKCFD, Glanzmann thrombasthenia, hemophilia A, hemophilia B, immune thrombocytopenic purpura (ITP), von Willebrand disease (VWD) Type 1, von Willebrand disease (VWD) Type 2, and Von Willebrand disease (VWD) Type 3; optionally, wherein the hereditary bleeding disorder is hemophilia A, mild hemophilia, moderate hemophilia, severe hemophilia, VWD Type 1, VWD Type 1 Vicenza subtype, VWD Type 2a, VWD Type 2b, VWD Type 2b with thrombocytopenia, VWD Type 2m, VWD Type 2n,, or VWD Type 3.

3. The use of claim 1 or 2, wherein the VWF targeting agent is a VWF binding agent selected from the group consisting of a synthetic polynucleotide, an antibody, a nanobody, a peptide, an oligonucleotide, a siRNA, a microRNA, and a small molecule.

4. The use of claim 3, wherein the VWF binding agent is a synthetic polynucleotide selected from the group consisting of BT200 (SEQ ID No.: 6), SEQ ID No.: 3, BT99 (SEQ ID No.: 4), BT100 (SEQ ID No.: 5), ARC15105 (SEQ ID No.: 7), and ARC1779 (SEQ ID No.: 8) and variants thereof.

5. The use of claim 4, wherein the VWF binding agent is BT200 (SEQ ID No.: 6) or a variant thereof.

6. The use of claim 5, wherein BT200 increases the levels and activity of VWF, the levels and activity of FVIII, and/or the platelet counts in the blood.

7. The use of claim 5, wherein BT200 is to be administered to the subject at a dose ranging from 0.1 mg to 48.0 mg, or from 0.1mg to 20.0 mg, or from 1.0mg to 10mg; and optionally, wherein BT200 is to be administered to the subject at a dose ranging from 0.6 mg to 6.0 mg.

8. The use of claim 4, wherein the VWF binding agent is to be administered to the subject via subcutaneous injection.

9. The use of claim 4, wherein the VWF binding agent is to be administered to the subject with multiple doses, and wherein each dose is to be administered once every other day, or once every three days, or once every five days, or once a week, or once every other week.

10. The use of any one of claims 1-9, wherein the treatment of the hereditary bleeding disorder is a therapy treatment or a prophylactic treatment.

11. The use of claim 2, wherein the VWF targeting agent is to be administered in combination with coagulation factor substitute treatment; the coagulation factor substitute is plasma derived FVIII/VWF concentrates, recombinant FVIII preparations, or recombinant VWF preparations.

12. Use of a composition in the manufacture of a medicament for use in a method for
i) preventing a chronic bleeding condition, and/or spontaneous bleeding in a subject;
ii) blocking VWF clearance in the blood of a subject;
iii) elevating the FVIII levels in the blood of a subject; or
iv) increasing platelet counts in the blood of a patient having a low platelet count (thrombocytopenia);
wherein the composition comprises a VWF targeting agent that is a VWF binding agent that is a synthetic polynucleotide comprising PEGylated nucleic acids.

13. The use of claim 12, wherein the VWF binding agent is BT200 (SEQ ID No.: 6), or a variant thereof.

14. The use of claim 13, wherein

i) when used in preventing a chronic bleeding condition, and/or spontaneous bleeding in a subject, BT200 is to be administered at a dose ranging from 1.0mg to 10.0mg, or alternatively at a dose ranging from 1.0mg to 6.0mg; or

ii) when used in blocking VWF clearance in the blood of a subject, BT200 is to be provided at an amount ranging from 0.6mg to 6.0mg, or from 1.0mg to 6.0mg, or from 1.0mg to 10.0mg.

15. The use of claim 13, wherein when used for blocking VWF clearance in the blood of a subject, BT 200 increases VWF levels in the blood.

16. The use of claim 13, wherein when used in elevating the FVIII levels in the blood of a subject, the subject is diagnosed with hemophilia A, VWD Type 1, VWD Type 2, or VWD Type 3.

17. The use of claim 16, wherein BT200 increases FVIII clotting activity.

18. The use of claim 13, wherein when used in increasing platelet counts in the blood of a patient having a low platelet count, the thrombocytopenia is associated with a genetic disorder that causes a low platelet count, an enlarged spleen that holds abnormal platelets, a side reaction to a medicine, an autoimmune disease, or a viral infection; optionally
wherein the disorder is VWD Type 2a, VWD Type 2b with thrombocytopenia, VWD Type 2b, VWD Type 2m, or VWD Type 2n.

19. Use of a pharmaceutical composition in the manufacture of a medicament for use in method for treating a hereditary bleeding disorder in a patient, wherein the pharmaceutical composition comprises a VWF binding agent and BT200, and wherein the method comprises:

a) assessing the hereditary bleeding disorder of said patient and the baseline coagulation function in said patient;

b) determining the optimal dose of BT200 with the assessment of step a) for initial treatment; and

c) providing a weekly dose of BT200 after one-week treatment with the initial dose of BT200 in said patient;

optionally

wherein the assessment includes FVIII activity, VWF antigen levels and/or platelet counts in the blood.

20. The use of claim 19, wherein the hereditary bleeding disorder is hemophilia A, severe hemophilia, VWD Type 1, VWD Type 2a, VWD Type 2b, VWD Type 2m, VWD Type 2n, or VWD Type 3.

21. The use of claim 20, wherein the patient is treated with a coagulation factor substitute.

22. A composition for use in therapy, wherein the composition comprises a VWF targeting agent wherien the VWF targeting agent is BT200 that comprises the sequence of SEQ ID NO. 6, or variant thereof.