CLAIMS

1. A pharmaceutical composition comprising

a recombinant adeno-associated virus (rAAV) vector,

sodium chloride (NaCl)

potassium chloride (KCl),

disodium phosphate (Na_2HPO_4),

monopotassium phosphate (KH_2PO_4),

magnesium chloride ($MgCl_2$),

a polyol, and

a poloxamer, wherein the composition comprises no more than 0.1±0.01 mM calcium chloride and has a pH of 7.1±10% to 7.5±10%.

2. The composition of claim 1, wherein the polyol is sucrose.

3. The composition of claim 1 or 2, wherein the poloxamer is poloxamer 188.

4. The composition of any one of claims 1–3, wherein the composition contains 0.1±0.01 to 2.0±0.20 mM magnesium chloride.

5. The composition of claim 4, wherein the composition contains 0.5±0.05 mM or higher magnesium chloride.

6. The composition of claim 4, wherein the composition contains 1.4±0.14 mM magnesium chloride.

7. The composition of claim 4, wherein the composition contains more than 1.3±0.13 mM magnesium chloride.

8. The composition of any one of claims 1–7, wherein the composition contains 150±15 to 200±20 mM, optionally 172±17.2 mM, sodium chloride.

9. The composition of any one of claims 1–8, wherein the composition contains 2.5±0.25 to 3.0±0.30 mM, optionally 2.7±0.27 mM, potassium chloride.

10. The composition of any one of claims 1–9, wherein the composition contains 5 to 10±1 mM, optionally 8±0.8 mM, disodium phosphate.

11. The composition of any one of claims 1–10, wherein the composition contains 1.0±0.1 to 2.0±0.2 mM, optionally 1.5±0.15 mM, monopotassium phosphate.

12. The composition of any one of claims 1–11, wherein the composition contains 0.5±0.05% to 2±0.2% (w/v), optionally 1±0.1% (w/v), sucrose.

13. The composition of any one of claims 1–12, wherein the composition contains 0.01±0.001% to 0.1±0.01% (w/v), optionally 0.05±0.005% (w/v), poloxamer 188.

14. A pharmaceutical composition comprising

a rAAV vector,

171.81±17.181 mM sodium chloride,

2.68±0.268 mM potassium chloride,

8.10±0.081 mM disodium phosphate,

1.47±0.147 mM monopotassium phosphate,

1.40±0.140 mM magnesium chloride,

1.00±0.100% (w/v) sucrose, and

0.05±0.005% (w/v) poloxamer 188, optionally wherein the composition comprises no more than 0.1±0.01 mM calcium chloride and has a pH of 7.1±10% to 7.5±10%.

15. A pharmaceutical composition comprising

a rAAV vector,

172±17.2 mM sodium chloride,

2.68±0.268 mM potassium chloride,

8.10±0.810 mM disodium phosphate,

1.47±0.147 mM monopotassium phosphate,

0.49±0.049 mM magnesium chloride,

1.00±0.100% (w/v) sucrose, and

0.05±0.005% (w/v) poloxamer 188, optionally wherein the composition comprises no more than 0.1±0.01 mM calcium chloride and has a pH of 7.1±10% to 7.5±10%.

16. The composition of any one of claims 1–15, wherein the rAAV comprises a genome comprising an expression cassette for a therapeutic protein.

17. The composition of claim 16, wherein the therapeutic protein is a human factor VIII polypeptide.

18. A pharmaceutical composition comprising:

a (rAAV vector, wherein the rAAV comprises a genome comprising an expression cassette for expressing a human factor VIII polypeptide,

171.81±17.181 mM sodium chloride,

2.68±0.268 mM potassium chloride,

8.10±0.810 mM disodium phosphate,

1.47±0.147 mM monopotassium phosphate,

1.40±0.140 mM magnesium chloride,

1.00±0.100% (w/v) sucrose, and

0.05±0.005% (w/v) poloxamer 188, optionally wherein the composition comprises no more than 0.1±0.01 mM calcium chloride and has a pH of 7.1±10% to 7.5±10%.

19. A pharmaceutical composition comprising:

a rAAV vector, wherein the rAAV comprises a genome comprising an expression cassette for expressing a human factor VIII polypeptide

172±17.2 mM sodium chloride,

2.68±0.268 mM potassium chloride,

8.10±0.810 mM disodium phosphate,

1.47±0.147 mM monopotassium phosphate,

0.49±0.049 mM magnesium chloride,

1.00±0.100% (w/v) sucrose, and

0.05±0.005% (w/v) poloxamer 188, optionally wherein the composition comprises no more than 0.1±0.01 mM calcium chloride and has a pH of 7.1±10% to 7.5±10%.

20. The composition of any one of claims 17–19, wherein the human factor VIII polypeptide comprises SEQ ID NO: 1.

21. The composition of any one of claims 17–19, wherein the genome of the rAAV comprises SEQ ID NO:2 or nucleotides 131–5,024 of SEQ ID NO:2.

22. The composition of any one of claims 1–21, the composition contains the rAAV at $1.0\pm0.1\times10^{12}$ to $1.0\pm0.1\times10^{14}$ vector genomes (vg) per mL, optionally $1.0\pm0.1\times10^{13}$ to $5.0\pm0.5\times10^{13}$ vg per mL.

23. The composition of claim 22, wherein the composition contains $1.0\pm0.1\times10^{13}$ vg per mL.

24. The composition of any one of the preceding claims, wherein the rAAV comprises an AAV6 capsid protein, optionally inverted terminal repeats (ITRs) of AAV2.

25. A vial comprising 5–10 mL, optionally 6.4 mL, of the composition of any one of claims 1–24.

26. The vial of claim 25, wherein the vial is made of cyclo-olefin copolymer.

27. The vial of claim 25 or 26, wherein the vial has an in-place thermoplastic elastomer stopper.

28. Use of the pharmaceutical composition of any one of claims 1–24 or the entire content of the vial of any one of claims 25–27 for the manufacture of a medicament for treating hemophilia A in a human subject in need thereof.