Claims

1. A pharmaceutical composition of a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype comprising:

(i)	a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype,	
(ii)	a buffering agent being	
	tris(hydroxymethyl)aminomethane	2.42 – 7.26 mg/ml and
	hydrochloric acid	to pH 7.0 – 8.0
	or	
	L-histidine	2.79 – 8.37 mg/ml and
	L-arginine	0.348 – 1.044 mg/ml,
(iii)	hydroxypropyl beta cyclodextrin	50.0 – 100.0 mg/ml,
(iv)	poloxamer 188	0.5 – 1.5 mg/ml,
(v)	sodium chloride	3.0 – 13.14 mg/ml,
(vi)	magnesium chloride	0.204 – 0.609 mg/ml,
(vii)	water for injection	to 1.0 ml.

2. The pharmaceutical composition according to claim 1, wherein tris(hydroxymethyl)aminomethane is present at a concentration of 2.42 mg/ml or at a concentration of 7.26 mg/ml.

3. The pharmaceutical composition according to claim 1, wherein hydrochloric acid is added to pH 7.5.

4. The pharmaceutical composition according to claim 1, wherein L-histidine is present at a concentration of 2.79 mg/ml or at a concentration of 8.37 mg/ml.

5. The pharmaceutical composition according to claim 1, wherein L-arginine is present at a concentration of 0.348 mg/ml or at a concentration of 1.044 mg/ml.

6. The pharmaceutical composition according to claim 1, wherein hydroxypropyl-beta-cyclodextrin is present at a concentration of 50.0 mg/ml or at a concentration of 100.0 mg/ml.

7. The pharmaceutical composition according to claim 1, wherein the poloxamer 188 is present at a concentration of 0.5 mg/ml or at a concentration of 1.5 mg/ml.

8. The pharmaceutical composition according to claim 1, wherein sodium chloride is present at a concentration of 3.0 mg/ml; or

at a concentration of 4.38 mg/ml; or

at a concentration of 4.5 mg/ml; or

at a concentration of 6.0 mg/ml; or

at a concentration of 13.14 mg/ml.

9. The pharmaceutical composition according to claim 1, wherein magnesium chloride is present at a concentration of 0.203 mg/ml or at a concentration of 0.609 mg/ml.

10. The pharmaceutical composition according to claim 1, wherein magnesium chloride is magnesium chloride hexahydrate.

11. The pharmaceutical composition according to claim 1, the composition further comprising disaccharide at a concentration of 10 – 200 mg/ml.

12. The pharmaceutical composition according to claim 11, wherein disaccharide is present at a concentration of 30.0 – 70.0 mg/ml or at a concentration of 90.0 – 200.0 mg/ml.

13. The pharmaceutical composition according to claim 12, wherein disaccharide is present at a concentration of 25.0 mg/ml; or

at a concentration of 33.3 mg/ml; or

at a concentration of 50.0 mg/ml; or

at a concentration of 100.0 mg/ml.

14. The pharmaceutical composition according to claim 11, wherein disaccharide is trehalose.

15. The pharmaceutical composition according to claim 14, wherein trehalose is trehalose dihydrate.

16. The pharmaceutical composition according to claim 1, comprising:

(i)	a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype,	
(ii)	a buffering agent being	
	tris(hydroxymethyl)aminomethane	2.42 mg/ml and
	hydrochloric acid	to pH 7.0 – 8.0
	or	
	L-histidine	2.79 mg/ml and
	L-arginine	0.348 mg/ml,

(iii)	hydroxypropyl beta cyclodextrin	100.0 mg/ml,
(iv)	a surfactant being poloxamer 188	0.5 mg/ml,
(v)	sodium chloride	6.0 mg/ml,
(vi)	magnesium chloride	0.203 mg/ml,
(vii)	water for injection	to 1.0 ml.

17. The pharmaceutical composition according to claim 1, comprising:

(a)

(i)	a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype,	
(ii)	a buffering agent being	
	tris(hydroxymethyl)aminomethane	2.42 mg/ml and
	hydrochloric acid	to pH 7.0 – 8.0
	or	
	L-histidine	2.79 mg/ml and
	L-arginine	0.348 mg/ml,
(iii)	hydroxypropyl beta cyclodextrin	100.0 mg/ml,
(iv)	a surfactant being poloxamer 188	0.5 mg/ml,
(v)	sodium chloride	4.5 mg/ml,
(vi)	magnesium chloride	0.203 mg/ml,
(vii)	water for injection	to 1.0 ml and
(viii)	further comprises a disaccharide being trehalose	25.0 mg/ml; or

(b)

(i)	a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype,	
(ii)	a buffering agent being	
	tris(hydroxymethyl)aminomethane	2.42 mg/ml and
	hydrochloric acid	to pH 7.0 – 8.0
	or	
	L-histidine	2.79 mg/ml and
	L-arginine	0.348 mg/ml,
(iii)	hydroxypropyl beta cyclodextrin	100.0 mg/ml,
(iv)	a surfactant being poloxamer 188	0.5 mg/ml,
(v)	sodium chloride	3.0 mg/ml,
(vi)	magnesium chloride	0.203 mg/ml,

(vii)	water for injection	to 1.0 ml and
(viii)	further comprises a disaccharide being trehalose	50.0 mg/ml; or

(c)

(i)	a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype,	
(ii)	a buffering agent being	
	tris(hydroxymethyl)aminomethane	2.42 mg/ml and
	hydrochloric acid	to pH 7.0 – 8.0
	or	
	L-histidine	2.79 mg/ml and
	L-arginine	0.348 mg/ml,
(iii)	hydroxypropyl beta cyclodextrin	33.3 mg/ml,
(iv)	a surfactant being poloxamer 188	0.5 mg/ml,
(v)	sodium chloride	4.38 mg/ml,
(vi)	magnesium chloride	0.203 mg/ml,
(vii)	water for injection	to 1.0 ml and
(viii)	further comprises a disaccharide being trehalose	33.3 mg/ml; or

(d)

(i)	a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype,	
(ii)	a buffering agent being	
	tris(hydroxymethyl)aminomethane	7.26 mg/ml and
	hydrochloric acid	to pH 7.0 – 8.0
	or	
	L-histidine	8.37 mg/ml and
	L-arginine	1.044 mg/ml,
(iii)	hydroxypropyl beta cyclodextrin	100.0 mg/ml,
(iv)	a surfactant being poloxamer 188	1.5 mg/ml,
(v)	sodium chloride	13.14 mg/ml,
(vi)	magnesium chloride	0.609 mg/ml,
(vii)	water for injection	to 1.0 ml and
(viii)	further comprises a disaccharide being trehalose	100.0 mg/ml.

18. The pharmaceutical composition according to claim 17, wherein trehalose is trehalose dihydrate.

19. The pharmaceutical composition according to claim 16, 17, wherein magnesium chloride is magnesium chloride hexahydrate.

20. The pharmaceutical composition according to claim 16, 17, wherein hydrochloric acid is added to pH 7.5.

21. The pharmaceutical composition according to claim 1, wherein the rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotypeis present at a concentration of $1.0*10^{9}$ – $1.0*10^{14}$ viral genomes/ml.

22. The pharmaceutical composition according to claim 1, wherein the rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotypeis is present at a concentration of $1.0*10^{9}$ – $1.0*10^{13}$ viral genomes/ml.

23. The pharmaceutical composition according to claim 1, wherein the rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotypeis is present at a concentration of $(1.0 \pm 0.5)*10^{9}$ viral genomes/ml, $(1.0 \pm 0.5)*10^{10}$ viral genomes/ml, $(1.0 \pm 0.5)*10^{11}$ viral genomes/ml, $(1.0 \pm 0.5)*10^{12}$ viral genomes/ml, $(1.0 \pm 0.5)*10^{13}$ viral genomes/ml или $(1.0 \pm 0.5)*10^{14}$ viral genomes/ml.

24. The pharmaceutical composition according to claim 1, wherein the capsid of AAV5 serotype or AAV9 serotype may be a modified capsid.

25. The pharmaceutical composition according to any one of claims 1-24, the composition being suitable for lyophilization.

26. A lyophilized pharmaceutical composition of a recombinant non-enveloped virus vector produced by lyophilization of the pharmaceutical composition according to any one of claims 1-24.

27. The lyophilized pharmaceutical composition according to claim 26, the composition being intended for preparing an injectable solution.

28. The lyophilized pharmaceutical composition according to claim 26, the composition being intended for preparing a solution for intramuscular, intravenous or subcutaneous administration.

29. The lyophilized pharmaceutical composition according to claim 26, wherein the lyophilized pharmaceutical composition is present in a vial.

30. The lyophilized pharmaceutical composition according to claim 26, wherein the vial is a glass or polymer vial.

31. A lyophilized pharmaceutical composition of a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype, said composition following being reconstituted in an aqueous solvent comprises:

(i)	a rAAV vector which comprises a capside of AAV5 serotype or AAV9 serotype,	
(ii)	a buffering agent being	
	tris(hydroxymethyl)aminomethane	2.42 mg/ml and
	hydrochloric acid	to pH 6.0 – 8.0
	or	
	L-histidine	0.4 – 10.0 mg/ml and
	L-arginine	0.05 – 10.0 mg/ml,
(iii)	hydroxypropyl beta cyclodextrin	20.0 – 100.0 mg/ml,
(iv)	poloxamer 188	0.01 – 1.0 mg/ml,
(v)	sodium chloride	1.0 – 6.0 mg/ml,
(vi)	magnesium chloride	0.203 mg/ml,
(vii)	water for injection	to 1.0 ml.

32. The lyophilized pharmaceutical composition according to claim 31, wherein hydrochloric acid is added to pH 7.5.

33. The lyophilized pharmaceutical composition according to claim 31, wherein L-histidine is present at a concentration of 2.79 mg/ml; or at a concentration of 0.348 mg/ml.

34. The lyophilized pharmaceutical composition according to claim 31, wherein hydroxypropyl-beta-cyclodextrin is present at a concentration of 33.3 mg/ml; or at a concentration of 100.0 mg/ml.

35. The lyophilized pharmaceutical composition according to claim 31, wherein the poloxamer 188 is present at a concentration of 0.5 mg/ml.

36. The lyophilized pharmaceutical composition according to claim 31, wherein sodium chloride is present at a concentration of 3.0 mg/ml; or

at a concentration of 4.38 mg/ml; or

at a concentration of 4.5 mg/ml; or

at a concentration of 6.0 mg/ml.

37. The lyophilized pharmaceutical composition according to claim 31, wherein magnesium chloride is magnesium chloride hexahydrate.

38. The lyophilized pharmaceutical composition according to claim 31, the composition further comprising disaccharide.

39. The lyophilized pharmaceutical composition according to claim 38, wherein disaccharide is present at a concentration of 20 – 100 mg/ml.

40. The lyophilized pharmaceutical composition according to claim 38, wherein disaccharide is present at a concentration of 25.0 mg/ml; or

at a concentration of 33.3 mg/ml; or

at a concentration of 50.0 mg/ml.

41. The lyophilized pharmaceutical composition according to claim 38, wherein disaccharide is trehalose.

42. The lyophilized pharmaceutical composition according to claim 41, wherein trehalose is trehalose dihydrate.

43. The lyophilized pharmaceutical composition according to claim 31, wherein the recombinant non-enveloped virus vector is present at a concentration of $1.0*10^{9} – 1.0*10^{14}$ viral genomes/ml.

44. The lyophilized pharmaceutical composition according to claim 31, wherein the recombinant non-enveloped virus vector is present at a concentration of $1.0*10^{9} – 1.0*10^{13}$ viral genomes/ml.

45. The lyophilized pharmaceutical composition according to claim 31, wherein the recombinant non-enveloped virus vector is present at a concentration of $(1.0 \pm 0.5)*10^{11}$ viral genomes/ml, $(1.0 \pm 0.5)*10^{10}$ viral genomes/ml, $(1.0 \pm 0.5)*10^{11}$ viral genomes/ml, $(1.0 \pm 0.5)*10^{12}$ viral genomes/ml, $(1.0 \pm 0.5)*10^{13}$ viral genomes/ml или $(1.0 \pm 0.5)*10^{14}$ viral genomes/ml.

46. The lyophilized pharmaceutical composition according to claim 31, wherein the capsid of AAV5 serotype or AAV9 serotype may be a modified capsid.