CLAIMS

1. Use of a recombinant human α-L-iduronidase (rIDUA) produced by human neuronal or human glial cells in the preparation of a composition for treating a human subject diagnosed with mucopolysaccharidosis I (MPS I), wherein a recombinant expression vector encoding the rIDUA is suitable for a single dose administration to the human subject, and wherein the single dose is determined based on the average brain mass of human subjects in the same age group of the human subject.

2. Use of a recombinant expression vector encoding a recombinant human α-L-iduronidase (rIDUA) in the manufacture of a medicament for treating a human subject diagnosed with mucopolysaccharidosis I (MPS I), wherein the medicament is suitable for administration at a single dose that is determined based on the average brain mass of human subjects in the same age group of the human subject.

3. The use of claim 2, wherein the rIDUA is produced by the human subject's neuronal or glial cells after the medicament is to be administered to the human subject.

4. The use of claim 1 or 3, wherein the rIDUA produced by the neuronal or glial cells is to be delivered to the cerebrospinal fluid of the human subject.

5. The use of any one of claims 1-4, wherein the recombinant expression vector is a recombinant adeno-associated virus (rAAV).

6. The use of claim 5, wherein the rAAV comprises an AAV9 capsid protein or a recombinant AAV9 capsid protein.

7. The use of claim 5, wherein the rAAV comprises an AAVrh10 capsid protein or a recombinant AAVrh10 capsid protein.

8. The use of any one of claims 1-7, wherein an immune suppression therapy is suitable for administration to the human subject before or concurrently with the rIDUA treatment and optionally the immune suppression therapy thereafter is to be continued thereafter.

9. The use of claim 8, wherein the immune suppression therapy comprises a combination of (a) tacrolimus and mycophenolic acid, (b) rapamycin and mycophenolic acid, or (c) tacrolimus, rapamycin, and a corticosteroid.

10. The use of claim 9, wherein the corticosteroid is a prednisolone and/or methylprednisolone.

11. The use of any one of claims 8-10, wherein the immune suppression therapy is withdrawn after 180 days.

12. The use of any one of claims 1-11, wherein the rIDUA: (a) is α2,6-sialylated; (b) does not contain detectable NeuGc; (c) does not contain detectable α-Gal antigen; (d) contains tyrosine-sulfation; and/or (e) is mannose-6-phosphorylated.

13. The use of any one of claims 1 to 12, wherein the rIDUA comprises the amino acid sequence of SEQ ID NO. 1.

14. The use of any one of claims 1-13, wherein the recombinant expression vector comprises a CB7 promoter.

15. The use of any one of claims 1-14, wherein the recombinant expression vector encodes a signal peptide.

16. The use of any one of claims 1-15, wherein the human subject is about or at least about 4 months to less than about 9 months of age, and the same age group is human subjects who are about or at least about 4 months to less than about 9 months of age.

17. The use of any one of claims 1-15, wherein the human subject is about or at least about 9 months to less than about 18 months of age, and the same age group is human subjects who are about or at least about 9 months to less than about 18 months of age.

18. The use of any one of claims 1-15, wherein the human subject is about or at least about 18 months to less than about 3 years of age, and the same age group is human subjects who are about or at least about 18 months to less than about 3 years of age.

19. The use of any one of claims 1-18, wherein the human subject is a pediatric subject.

20. The use of claim 19, wherein the pediatric subject is about or less than about 3 years of age.

21. The use of any one of claims 16-20, wherein the single dose is an amount of about or from about 2×10^9 GC/g brain mass to about 1×10^{10} GC/g brain mass, wherein the GC/g brain mass is based on the average brain mass of human subjects in the same age group of the human subject.

22. The use of any one of claims 16-20, wherein the single dose is an amount of about or from about 1×10^{10} GC/g brain mass to about 5×10^{10} GC/g brain mass, wherein the GC/g brain mass is based on the average brain mass of human subjects in the same age group of the human subject.

23. The use of claim 16, wherein the single dose is an amount of about or from about 1.2×10^{12} GC to about 6.0×10^{12} GC or of about or from about 6.0×10^{12} GC to about 3.0×10^{13} GC.

24. The use of claim 17, wherein the single dose is an amount of about or from about 2×10^{12} GC to about 1.0×10^{13} GC or of about or from about 1.0×10^{13} GC to about 5.0×10^{13} GC.

25. The use of claim 18, wherein the single dose is an amount of about or from about 2.2×10^{12} GC to about 1.1×10^{13} GC or of about or from about 1.1×10^{13} GC to about 5.5×10^{13} GC.

26. The use of claim 16, wherein the single dose is an amount of 1.2×10^{12} GC, 6.0×10^{12} GC, or 3.0×10^{13} GC.

27. The use of claim 17, wherein the single dose is an amount of 2.0×10^{12} GC, 1.0×10^{13} GC, or 5.0×10^{13} GC.

28. The use of claim 18, wherein the single dose is an amount of 2.2×10^{12} GC, 1.1×10^{13} GC, or 5.5×10^{13} GC.

29. The use of claim 19 or 20, wherein the single dose is an amount of 1.2×10^{12} GC, 2.0×10^{12} GC, 2.2×10^{12} GC, 6.0×10^{12} GC, 3.0×10^{13} GC, 1.0×10^{13} GC, 1.1×10^{13} GC, 5.0×10^{13} GC, or 5.5×10^{13} GC.

30. The use of any one of claims 1-29, wherein the recombinant expression vector is suitable for intracerebroventricular (ICV) administration or intracisternal (IC) injection.