CLAIMS

1. Use of a recombinant vector carrying an expression cassette encoding a human α-L-iduronidase (IDUA) in the manufacture of a medicament for treatment of mucopolysaccharidosis I (MPS I) in a human subject, wherein the recombinant vector is for administration to the human subject via an intracisternal (IC) injection, intracerebroventricular (ICV) injection, or lumbar puncture, and wherein a glycosylated recombinant human IDUA is produced by human neuronal cells or human glial cells after administration of the recombinant vector to the human subject.

2. Use of a glycosylated recombinant human α-L-iduronidase (IDUA) produced by human neuronal cells or human glial cells, for treatment of mucopolysaccharidosis I (MPS I) in a human subject, wherein the glycosylated recombinant human IDUA is for delivery to the human subject after administration of a composition comprising a recombinant vector carrying an expression cassette encoding a human IDUA and a physiologically compatible carrier to the human subject via an intracisternal (IC) injection, intracerebroventricular (ICV) injection, or lumbar puncture.

3. The use of claim 1 or 2, wherein the recombinant vector comprises a CB7 promoter.

4. The use of any one of claims 1-3, wherein the recombinant vector is a recombinant adeno-associated virus (AAV) vector.

5. The use of claim 4, wherein the recombinant AAV comprises an AAV9 capsid protein or a recombinant AAV9 capsid protein.

6. The use of claim 4, wherein the recombinant AAV comprises an AAVrh10 capsid protein or a recombinant AAVrh10 capsid protein.

7. The use of any one of claims 1-6, wherein the human subject is a pediatric human subject.

8. The use of any one of claims 1-6, wherein the human subject is 4 months of age or older.

9. The use of any one of claims 1-6, wherein the human subject is less than 3 years of age.

10. The use of any one of claims 1 and 3-9, wherein the human subject has severe MPS I prior to a first administration of the recombinant vector to the human subject.

11. The use of any one of claims 2-9, wherein the human subject has severe MPS I prior to a first administration of the composition to the human subject.

12. The use of any one of claims 1-11, wherein the glycosylated recombinant human IDUA is to be delivered to the cerebrospinal fluid (CSF) of the brain of the human subject.

13. The use of any one of claims 1-12, wherein the glycosylated recombinant human IDUA: (a) is α2,6-sialylated; (b) does not contain detectable NeuGc; (c) does not contain detectable α-Gal antigen; and/or (d) is tyrosine-sulfated.

14. The use of any one of claims 1, 3-10, and 12-13, wherein the recombinant vector is for a single administration to the human subject.

15. The use of any one of claims 2-9 and 11-13, wherein the composition is for a single administration to the human subject.

16. The use of claim 14 or 15, wherein the single administration is 6.0×10^{12} GC, 1.0×10^{13} GC, 1.1×10^{13} GC, 3.0×10^{13} GC, 5.0×10^{13} GC, or 5.5×10^{13} GC.

17. The use of claim 14 or 15, wherein the single administration is from 2×10^{9} GC/g brain mass to 5×10^{10} GC/g brain mass.

18. The use of any one of claims 1-17, wherein the human IDUA comprises SEQ ID NO: 1.

19. The use of any one of claims 1, 3-10, 12-14, and 16-18, wherein an immune suppression therapy is for administration to the human subject before or concurrently with administration of the recombinant vector to the human subject, and continuing immune suppression therapy thereafter.

20. The use of any one of claims 2-9, 11-13, and 15-18, wherein an immune suppression therapy is for administration to the human subject before or concurrently with administration of the composition to the human subject, and continuing immune suppression therapy thereafter.

21. The use of claim 19 or 20, wherein the immune suppression therapy comprises administration of a combination of (a) tacrolimus and mycophenolic acid, (b) rapamycin and mycophenolic acid, or (c) tacrolimus, rapamycin, and a corticosteroid to the human subject.

22. The use of any one of claims 19-21, wherein the immune suppression therapy is withdrawn after 180 days.

23. The use of claim 21 or 22, wherein the corticosteroid is prednisolone, methylprednisolone, or a combination thereof.

24. The use of any one of claims 1, 3-10, 12-14, 16-19, and 21-23, wherein the human subject had previously received hematopoietic stem cell transplantation (HSCT) and/or enzyme replacement therapy (ERT) treatment prior to the first administration of the recombinant vector to the human subject.

25. The use of any one of claims 2-9, 11-13, 15-18, and 20-23, wherein the human subject had previously received hematopoietic stem cell transplantation (HSCT) and/or enzyme replacement therapy (ERT) treatment prior to the first administration of the composition to the human subject.