CLAIMS

1. Use of a composition comprising a recombinant adeno-associated virus (rAAV) vector comprising an open reading frame encoding a lysosomal storage enzyme to be administered to a mammal having mucopolysaccharidosis type II disorder, for use in the manufacture of a medicament having an amount of the rAAV effective to enhance neurocognition throughout the brain of the mammal relative to a mammal with the MPS type II disorder that is not administered the rAAV, wherein the rAAV is AAV9 or AAVrh10.

2. The use of claim 1 wherein the mammal is not treated with an immunosuppressant.

3. The use of claim 1 wherein the mammal is to be or has been treated with an immunosuppressant.

4. The use of claim 3 wherein the immune suppressant comprises cyclophosphamide.

5. The use of claim 3 wherein the immune suppressant comprises a glucocorticoid, cytostatic agents including an alkylating agent, an anti-metabolite, a cytotoxic antibiotic, an antibody, or an agent active on immunophilin.

6. The use of claim 3 wherein the immune suppressant comprises a nitrogen mustard, nitrosourea, platinum compound, methotrexate, azathioprine, mercaptopurine, fluorouracil, dactinomycin, an anthracycline, mitomycin C, bleomycin, mithramycin, IL-2 receptor- (CD25-) or CD3-directed antibodies, anti-IL-2 antibodies, ciclosporin, tacrolimus, sirolimus, IFN-β, IFN-γ, an opioid, or TNF-α (tumor necrosis factor-alpha) binding agent.

7. The use of any one of claims 3 to 6 wherein the rAAV and the immune suppressant are to be co-administered or the immune suppressant is to be administered after the rAAV.

8. The use of any one of claims 1 to 7 wherein the mammal is not immunotolerized prior to administration of rAAV.

9. The use of any one of claims 1 to 7 wherein the mammal is immunotolerized prior to administration of rAAV.

10. The use of claim 1 wherein the mammal is an immunocompetent adult.

11. The use of any one of claims 1 to 10 wherein the rAAV vector is a rAAV9 vector.

12. The use of any one of claims 1 to 11 wherein the open reading frame encodes iduronate-2-sulfatase.

13. The use of any one of claims 1 to 12 wherein the mammal is a human.

14. The use of any one of claims 1 to 13 wherein the mammal is deficient in iduronate-2-sulfatase.

15. The use of any one of claims 1 to 14 wherein multiple doses are to be administered.

16. The use of any one of claims 1 to 14 wherein the composition is to be administered weekly.

17. The use of any one of claims 1 to 16 wherein the amount inhibits growth delay, inhibits hepatospenomegaly, inhibits cardiopulmonary disease, or inhibits skeletal dysplasia, or any combination thereof.

18. The use of any one of claims 1 to 10 or 12 to 17 wherein the rAAV is rAAVrh10.

19. The use of any one of claims 1 to 18 wherein the AAV is to be administered intranasally.