CLAIMS

1. Use of a composition comprising a recombinant adeno-associated virus (rAAV) vector comprising an open reading frame encoding iduronate-2-sulfatase to be administered to a human having mucopolysaccharidosis (MPS) type II disorder, for use in the manufacture of a medicament having an amount of the rAAV effective to enhance neurocognition in the human relative to a human with the MPS type II disorder that is not administered the rAAV, wherein the rAAV is AAV9.

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

3. The use of claim 1 wherein the human 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 human is not immunotolerized prior to administration of rAAV.

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

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

11. The use of any one of claims 1 to 10 wherein the human is deficient in iduronate-2-sulfatase.

12. The use of any one of claims 1 to 11 wherein multiple doses are to be administered.

13. The use of any one of claims 1 to 11 wherein the composition is to be administered weekly.

14. The use of any one of claims 1 to 13 wherein the amount inhibits growth delay, inhibits hepatospenomegaly, inhibits cardiopulmonary disease, or inhibits skeletal dysplasia, or any combination thereof.

15. The use of any one of claims 1 to 14 wherein the rAAV is to be administered intranasally.

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

17. The use of any one of claims 1 to 14 wherein the rAAV is to be administered cerebroventricularly.