CLAIMS

1. Use of a composition comprising an effective amount of a rAAV vector including an open reading frame encoding alpha L-iduronidase, the expression of which in a human prevents, inhibits or treats the one or more neurological symptoms, in the manufacture of a medicament for the prevention, inhibition or treatment of one or more neurological symptoms in a human, having a deficiency in alpha L-iduronidase, wherein the medicament is to be administered to a cisterna magna of the human and wherein the rAAV is rAAV-9 or rAAVrh10.

2. Use of a composition comprising an effective amount of a rAAV vector including an open reading frame encoding iduronate-2-sulfatase, the expression of which in a human prevents, inhibits or treats the one or more neurological symptoms, in the manufacture of a medicament for the prevention, inhibition or treatment of one or more neurological symptoms in a human, having a deficiency in iduronate-2-sulfatase, wherein the medicament is to be administered to a cisterna magna of the human and wherein the rAAV is rAAV-9 or rAAVrh10.

3. The use of claim 1 or claim 2 wherein a permeation enhancer comprising mannitol, sodium glycocholate, sodium taurocholate, sodium deoxycholate, sodium salicylate, sodium caprylate, sodium caprate, sodium lauryl sulfate, polyoxyethylene-9-laurel ether, or EDTA is also to be administered to the human.

4. The use of claim 1 or claim 2, wherein an immune suppressant is to be administered to the human.

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

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

7. The use of any one of claims 4 to 6, 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-P, IFN-y, an opioid, or tumor necrosis factor-alpha binding agent.

8. The use of any one of claims 4 to 7, wherein the medicament and the immune suppressant are to be co-administered or the immune suppressant is to be administered after the medicament.

9. The use of any one of claims 5 to 9, wherein the immune suppressant is to be intrathecally or intracerebroventricularly administered to the human.

10. Use of a composition comprising an effective amount of a rAAV vector including an open reading frame encoding alpha L-iduronidase, the expression of which in the human prevents, inhibits or treats the one or more symptoms associated with a deficiency in alpha L-iduronidase in a human, in the manufacture of a medicament for the prevention, inhibition or treatment of one or more symptoms associated with a deficiency in alpha L-iduronidase in a human, wherein the human has been immunotolerized to alpha-L-iduronidase; wherein the medicament is to be administered to a cisterna magna of the human, and wherein the rAAV is rAAV-9 or rAAVrh10.

11. Use of a composition comprising an effective amount of a rAAV vector including an open reading frame encoding iduronate-2-sulfatase, the expression of which in the human prevents, inhibits or treats the one or more symptoms associated with a deficiency in iduronate-2-sulfatase in a human, in the manufacture of a medicament for the

prevention, inhibition or treatment of one or more symptoms associated with a deficiency in iduronate-2-sulfatase in a human, wherein the human has been immunotolerized to iduronate-2-sulfatase; wherein the medicament is to be administered to a cisterna magna of the human, and wherein the rAAV is rAAV-9 or rAAVrh10.

12. The use of any one of claims 1 to 11, wherein the human is an immuno-competent adult.

13. The use of any one of claims 1 to 12, wherein the rAAV vector is a rAAV-9 vector.

14. The use of any one of claims 1 to 13, wherein multiple doses are to be administered.

15. The use of any one of claims 1 to 13, wherein the medicament is to be administered weekly.

16. The use of any one of claims 1 to 15, wherein neurodegeneration is prevented, inhibited or treated by the administration.

17. The use of any one of claims 1 to 12, wherein the rAAV vector is rAAVrh10.