CLAIMS:

1. A vector carrying an expression cassette comprising a human alpha-L-iduronidase (hIDUA) gene having the nucleotide sequence of SEQ ID NO: 1 or a sequence at least about 80% identical to SEQ ID NO: 1 which encodes a functional human alpha-L-iduronidase in human cells, wherein said expression cassette further comprises regulatory control sequences which direct expression of the human alpha-L-iduronidase in human cells, said regulatory control sequences comprising a liver-specific promoter.

2. The vector according to claim 1, wherein the modified hIDUA gene is at least about 85 % to about 90 % or at least about 95% to about 99% identical to SEQ ID NO: 1.

3. The vector according to claim 1, wherein the functional human alpha-L-iduronidase is selected from the group consisting of:

(a) about amino acid 1 to about 653 of SEQ ID NO: 2;

(b) a synthetic human enzyme comprising a heterologous leader sequence fused to about acids 27 to about 653 of SEQ ID NO: 2;

(c) a variant of amino acid sequence of SEQ ID NO:2 having one or more of the modifications comprising: an H to Q reduction at amino acid position 82; a change from R to Q at position 105; a change from G to R at position 116; a change from V to A at position 279; a change from L to R at position 346; a change from A to T at position 361; a change from H to N at position 449; a change from V to I at position 454; a change from A to T at position 591; and a change from A to T at position 622.

4. The vector according to claim 1, wherein the expression cassette further comprises a poly A selected from rabbit globulin poly A.

5. The vector according to claim 1 which is plasmid pENN.TBG.hIDUA.nRBG.

6. A recombinant adeno-associated virus (rAAV) particle having an AAV capsid and having packaged therein a 5' inverted terminal repeat (ITR), a human alpha-L-iduronidase (hIDUA) gene under the control of regulatory sequences which control expression thereof, and an AAV 3' ITR, wherein said hIDUA gene has a sequence shown in SEQ ID NO: 1 or a sequence at least about 95% identical thereto which encodes a functional human alpha-L-iduronidase.

7. The rAAV according to claim 6, wherein the modified hIDUA gene is expressed under the control of a liver-specific promoter

8. The vector according to claim 1 or the rAAV according to claim 6 or 7, wherein the promoter is a TBG promoter.

9. The vector according to claim 1 or the rAAV according to claim 6, wherein the vector further comprises one or more enhancers, optionally wherein the enhancers are the same or different.

10. The vector according to claim 9 or the rAAV according to claim 9, wherein the enhancers are selected from the group consisting of an intron, a CMV enhancer, and an Alpha mic/bik enhancer, optionally wherein more than one copy of a selected enhancer is present in the vector, further optionally wherein the more than one copy of the enhancer are located in tandem.

11. The rAAV according to claim 6, wherein the rAAV particle comprises an AAV capsid selected from AAV8 and AAV9.

12. The rAAV according to claim 11, wherein the rAAV is pseudotyped, optionally wherein the ITRs are from an AAV2.

13. A recombinant adeno-associated viral particle AAV2/8.TBG.hIDUA.co.

14. A composition for use in treating mucopolysaccharidosis type I (MPS I) comprising an effective amount of the composition comprising the rAAV according to any of claims 6 to 13 and a pharmaceutically acceptable carrier.

15. The composition for the use according to claim 14, wherein said rAAV is delivered intravenously or wherein rAAV is delivered to the subject in an amount of about 3 x 10 9 to about 3 x 10 12.