CLAIMS

What is claimed is:

1. Use of a pharmaceutical composition comprising an oligosaccharide-protein conjugate and a pharmaceutically acceptable carrier, in the manufacture of a medicament for treating infantile-onset Pompe disease (IOPD) in a human individual, wherein the oligosaccharide-protein conjugate is avalglucosidase alfa, and wherein the pharmaceutical composition is to be administered intravenously to the individual at a dose of 20 mg/kg to 40 mg/kg for at least 25 weeks.

2. The use of claim 1, wherein the pharmaceutical composition is to be administered at a dose of 20 mg/kg.

3. The use of claim 1, wherein the pharmaceutical composition is to be administered at a dose of 40 mg/kg.

4. The use of any one of claims 1-3, wherein the pharmaceutical composition is to be administered to the individual once every two weeks.

5. The use of any one of claims 1-4, wherein the pharmaceutical composition is reconstituted from a lyophilized formulation comprising the oligosaccharide-protein conjugate.

6. The use of claim 5, wherein the pharmaceutically acceptable carrier comprises a sugar that is not degraded by GAA, optionally wherein the sugar that is not degraded by GAA is mannitol.

7. The use of claim 5 or 6, wherein the pharmaceutically acceptable carrier further comprises glycine and/or histidine.

8. The use of any one of claims 5-7, wherein the pharmaceutical composition has a pH of 6.2.

9. The use of any one of claims 5-8, wherein the pharmaceutically acceptable carrier comprises 10-50 mM histidine, 0.25-2% glycine, 1-4% mannitol, and 0.005-0.05% polysorbate 80.

10. The use of claim 9, wherein the pharmaceutically acceptable carrier comprises 10 mM histidine, 2% glycine, 2% mannitol, and 0.01% polysorbate 80.

11. The use of any one of claims 1-10, wherein the individual:

a. has cardiomyopathy at the time of diagnosis in the first year of life;

b. has arrhythmia;

c. has cardiomegaly;

d. is 18 years old or younger;

e. is 6 months old or younger; and/or

f. has not received treatment with a recombinant GAA.

12. The use of any one of claims 1-11, wherein the individual has received at least 6 months of treatment with a recombinant GAA, optionally, wherein the individual shows clinical decline after treatment with the recombinant GAA, wherein the clinical decline is determined by assessing one or more parameters selected from the group consisting of respiratory functions, motor skills and cardiac parameters.

13. The use of claim 12, wherein the individual has suboptimal clinical response to treatment with the recombinant GAA, wherein the clinical response is determined by assessing one or more parameters selected from the group consisting of respiratory functions, motor skills and cardiac parameters.

14. The use of claim 12 or 13, wherein the recombinant GAA is alglucosidase alfa.

15. The use of any one of claims 1-14, wherein the individual is cross-reactive immunologic material (CRIM)-negative or the individual is CRIM-positive.

16. The use of any one of claims 1-15, wherein an effective amount of methotrexate is to be further administered to the individual, optionally, wherein the methotrexate is to be administered in a single cycle or in three cycles.

17. The use of claim 16, wherein an effective amount of an additional immune tolerance induction therapy is to be further administered to the individual, optionally, wherein the additional immune tolerance induction therapy comprises rituximab and intravenous immunoglobulin (IVIG).

18. The use of any one of claims 1-17, wherein:

a. the individual has decreasing level of antidrug antibody (ADA) against the oligosaccharide-protein conjugate over time;

b. creatine kinase (CK) level of the individual decreases by at least 100 IU/L when measured after at least 25 weeks of treatment;

c. urinary hexose tetrasaccharide (Hex4) level of the individual decreases by at least 10 mmol/mol when measured after at least 25 weeks of treatment;

d. Gross Motor Function Measure (GMFM-88) score of the individual increases by at least 5% when measured after at least 25 weeks of treatment; and/or

e. the individual shows improvement or stabilization of one or more parameters selected from the group consisting of respiratory functions, motor skills, cardiac parameters and eyelid positions.

19. The use of claim 18, wherein the improvement or stabilization is assessed based on one or more parameters selected from the group consisting of Alberta Infant Motor Scale (AIMS) score, Pompe-Pediatric Evaluation of Disability Inventory (PEDI) functional skills scale, Echocardiographic (ECHO)-left ventricular mass (LVM) Z-score, ECHO LVMI score, Gross Motor Function Classification System-Expanded and Revised (GMFCS-E&R) score, Quick Motor Function Test, 6 Minute Walk test (6MWT), interpalpebral fissure distance (IPFD), margin reflex distance-1 (MRD-1), margin pupil distance (MPD), onset of ptosis, and use of respiratory support.