CLAIMS

1. A composition comprising recombinant human acid α-glucosidase (rhGAA) molecules for use in the treatment of Pompe disease in combination with miglustat in a patient in need thereof;
wherein the composition is administered intravenously at a dose of 5 mg/kg to 20 mg/kg and the miglustat is administered orally at a dose of 260 mg or 130 mg;
wherein the rhGAA molecules are produced in Chinese hamster ovary (CHO) cells, the rhGAA molecules comprise seven potential N-glycosylation sites, 40%-60% of the N-glycans on the rhGAA molecules are complex type N-glycans, and at least 50% of the rhGAA molecules bear a bis-mannose-6-phosphate (bis-M6P) unit at the first potential N-glycosylation site.

2. The composition for use according to claim 1, wherein the rhGAA molecules are expressed having
(i) a sequence at least 95% identical to SEQ ID NO: 4; or
(ii) the sequence of SEQ ID NO: 4, wherein the rhGAA molecules undergo post-translational modification to remove the first 56 amino acids.

3. The composition for use according to claim 1 or claim 2, wherein at least 55% of the rhGAA molecules bear a bis-M6P unit at the first potential N-glycosylation site.

4. The composition for use according to any one of claims 1-3, wherein at least 70% of the rhGAA molecules are phosphorylated at the first potential N-glycosylation site.

5. The composition for use according to any one of claims 1-4, wherein at least 40% of the rhGAA molecules bear a mono-mannose-6-phosphate (mono-M6P) unit at the second potential N-glycosylation site.

6. The composition for use according to any one of claims 1-5, wherein at least 40% of the rhGAA molecules bear a bis-M6P unit at the fourth potential N-glycosylation site.

7. The composition for use according to any one of claims 1-6, wherein at least 25% of the rhGAA molecules bear a mono-M6P unit at the fourth potential N-glycosylation site.

8. The composition for use according to any one of claims 1-7, wherein the composition is administered at a dose of 20 mg/kg by intravenous infusion over approximately four hours every 2 weeks, wherein the miglustat is administered one hour prior to the intravenous infusion of the composition, and wherein the patient fasts for at least two hours before and at least two hours after the oral administration of miglustat.

9. The composition for use according to any one of claims 1-8, wherein the composition is administered intravenously at a dose of 20 mg/kg and the miglustat is administered orally at a dose of 260 mg.

10. A kit for combination therapy of Pompe disease in a patient in need thereof, the kit including a pharmaceutically acceptable dosage form comprising miglustat configured for oral administration at a dose of 260 mg or 130 mg, a pharmaceutically acceptable dosage form comprising recombinant human acid α-glucosidase (rhGAA) molecules configured for intravenous administration at a dose of 5 mg/kg to 20 mg/kg, and instructions for administering the pharmaceutically acceptable dosage form comprising miglustat and the pharmaceutically acceptable dosage form comprising rhGAA molecules to a patient in need thereof;
wherein the rhGAA molecules are produced in Chinese hamster ovary (CHO) cells, the rhGAA molecules comprise seven potential N-glycosylation sites, and at least 50% of the rhGAA molecules bear a bis-mannose-6-phosphate (bis-M6P) unit at the first potential N-glycosylation site.

11. The kit according to claim 10, wherein the instructions comprise instructions to administer the pharmaceutically acceptable dosage form comprising rhGAA molecules at a dose of 20 mg/kg by intravenous infusion over approximately four hours every 2 weeks, wherein the instructions comprise instructions to administer the pharmaceutically acceptable dosage form comprising rhGAA molecules one hour after the oral administration of the pharmaceutically acceptable dosage form comprising miglustat, and wherein the instructions further comprise instructions that the patient fasts for at least two hours before and at least two hours after the oral administration of the pharmaceutically acceptable dosage form comprising miglustat.