CLAIMS

What is claimed is:

1. Use of a composition in the manufacture of a medicament for treating a disease or disorder in a subject in need thereof, wherein the composition comprises a dose comprising a therapeutically effective amount of ribitol for administration to the subject, wherein the dose is an immediate-release dose and/or not a controlled-release dose, and wherein the disease or disorder is associated with a defect in fukutin (FKTN) or Fukutin-related protein (FKRP).

2. Use of a composition in the manufacture of a medicament for treating a disease or disorder in a subject in need thereof, wherein the composition comprises a dose comprising an amount of ribitol effective to achieve an area under the concentration-time curve (AUC_{0-24}) steady-state level of between 100 (µg·h)/mL and 2500 (µg·h)/mL upon administration to the subject, and wherein the disease or disorder is associated with a defect in fukutin (FKTN) or Fukutin-related protein (FKRP).

3. The use of claim 1 or 2, wherein the defect causes a partial or complete loss of function in FKRP or FKTN.

4. The use of any of claims 1 to 3, wherein the dose is to be administered at most four times daily, at most three times daily, or at most twice daily.

5. The use of any of claims 1 to 4, wherein the dose is to be administered between 0.5 grams per day (g/day) and 40 g/day of ribitol.

6. The use of any one of claims 1 to 5, wherein the dose of ribitol is to be administered for between one week and four months.

7. The use of any one of claims 1 to 6, wherein the dose of ribitol is to be administered chronically.

8. The use of any one of claims 1 to 7, wherein the disease or disorder is a muscular dystrophy.

9. The use of claim 8, wherein the muscular dystrophy is an FKRP-related alpha-dystroglycanopathy, an FKTN-related alpha-dystroglycanopathy, an Isoprenoid Synthase Domain-Containing Protein (ISPD)-related alpha-dystroglycanopathy, Muscle Eye Brain Disease (MEB), or Congenital Muscular Dystrophy (CMD).

10. The use of claim 9, wherein the disease or disorder is Limb-Girdle Muscular Dystrophy type 2i (LGMD2i/R9), Limb-Girdle Muscular Dystrophy Type 2M (LGMD2M), Limb-Girdle Muscular Dystrophy Type 2U (LGMD2U), or Fukuyama congenital muscular dystrophy (FCMD).

11. The use of any one of claims 1 to 10, wherein the maximum observed concentration (C_{max}) of ribitol is between 50 and 400 µg/mL.

12. The use of any one of claims 1 to 11, wherein the subject is a human.

13. The use of claim 12, wherein the human is a child.

14. The use of any one of claims 1 to 13, wherein the medicament restores and/or enhances functional glycosylation of α-DG.

15. A pharmaceutical composition comprising ribitol and a pharmaceutically acceptable carrier or excipient.

16. The pharmaceutical compositions of claim 15, wherein the pharmaceutical composition is a solid.

17. The pharmaceutical compositions of claim 15, wherein the pharmaceutical composition is a solution.

18. The pharmaceutical composition of claim 17, wherein the solution comprises a carrier, and wherein the carrier is water or saline.

19. The pharmaceutical composition of claim 18, wherein the carrier is substantially pure water.

20. The pharmaceutical composition of any one of claims 15 to 19, wherein the pharmaceutical composition comprises ribitol at between 0.2 g/mL and 10 g/mL.

21. A kit comprising the pharmaceutical composition of any one of claims 15 to 20 and instructions for use in treating a disease or disorder.

22. A unit dose, comprising between 0.5 g and 40 g of ribitol.

23. The unit dose of claim 22, wherein the ribitol is dissolved in water.

24. A unit dose, comprising an amount of ribitol that is effective to achieve a steady-state AUC_{0-24} level for ribitol of between 100 (μg·h)/mL and 2500 (μg·h)/mL.

25. The unit dose of claim 24, wherein the unit dose is formulated as a solid.

26. The unit dose of claim 24, wherein the unit dose is formulated as a liquid.

27. The unit dose of claim 25, wherein the ribitol is dissolved in water.

28. The unit dose of any one of claims 22-27, wherein the unit dose is formulated for oral administration.

29. Use of the pharmaceutical composition of any one of claims 15-20 or unit dose of any one of claims 22-28 in the manufacture of a medicament for treating a disease or disorder in a subject in need thereof, wherein the disease or disorder is associated with a defect in fukutin (FKTN) or Fukutin-related protein (FKRP).

30. The use of claim 29, wherein the defect causes a partial or complete loss-of-function in FKRP or FKTN.