CLAIMS

1. Use of (i) a viral vector comprising a transgene encoding myotubularin 1 (MTM1) and (ii) an anti-cholestatic agent in the manufacture of a medicament for treating X-linked myotubular myopathy (XLMTM) in a human patient in need thereof, wherein the anti-cholestatic agent is to be administered to the patient in one or more doses that commence within about six weeks of administration of the viral vector to the patient.

2. Use of (i) a viral vector comprising a transgene encoding MTM1 and (ii) an anti-cholestatic agent in the manufacture of a medicament for reducing stiffness and/or joint contractures in a human patient diagnosed as having XLMTM, wherein the anti-cholestatic agent is to be administered to the patient in one or more doses that commence within about six weeks of administration of the viral vector to the patient.

3. Use of (i) a viral vector comprising a transgene encoding MTM1 and (ii) an anti-cholestatic agent in the manufacture of a medicament for increasing diaphragm and/or respiratory muscle progression in a human patient diagnosed as having XLMTM, wherein the anti-cholestatic agent is to be administered to the patient in one or more doses that commence within about six weeks of administration of the viral vector to the patient.

4. The use of any one of claims 1-3, wherein the anti-cholestatic agent is to be administered to the patient in one or more doses that commence within about five weeks of administration of the viral vector to the patient, optionally wherein the anti-cholestatic agent is to be administered to the patient in one or more doses that commence within about four weeks, within about three weeks, within about two weeks, or within about one week of administration of the viral vector to the patient.

5. The use of claim 4, wherein the anti-cholestatic agent is to be administered to the patient in one or more doses that commence on the same day as administration of the viral vector to the patient.

6. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for treating XLMTM in a human patient in need thereof and who has been previously administered an anti-cholestatic agent.

7. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for reducing stiffness and/or joint contractures in a human patient diagnosed as having XLMTM and who has been previously administered an anti-cholestatic agent.

8. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for increasing diaphragm and/or respiratory muscle progression in a human patient diagnosed as having XLMTM and who has been previously administered an anti-cholestatic agent.

9. The use of any one of claims 1-8, wherein:
(i) the viral vector is to be administered to the patient in an amount of less than about 3×10^{14} vg/kg;
(ii) the viral vector is to be administered to the patient in an amount of from about 3×10^{13} vg/kg to about 2.3×10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of from about 8×10^{13} vg/kg to about 1.8×10^{14} vg/kg, from about 1×10^{14} vg/kg to about 1.6×10^{14} vg/kg, from about 1.1×10^{14} vg/kg to about 1.5×10^{14} vg/kg, or from about 1.2×10^{14} vg/kg to about 1.4×10^{14} vg/kg;
(iii) the viral vector is to be administered to the patient in an amount of about 1.3×10^{14} vg/kg;
(iv) the patient is five years old or younger at the time of administration of the viral vector;
(v) the patient is from about 1 month old to about 5 years old at the time of administration of the viral vector; or
(vi) the use further comprises monitoring the patient for development of cholestasis, hyperbilirubinemia, or one or more symptoms thereof.

10. The use of claim 9, wherein:
(i) the viral vector is to be administered to the patient in an amount of less than about 2.5×10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of less than about 2×10^{14} vg/kg, less than about 1.5×10^{14} vg/kg, or less than about 1.4×10^{14} vg/kg;
(ii) the patient is four years old or younger at the time of administration of the viral vector, optionally wherein the patient is three years old or younger, two years old or younger, one year old or younger, or six months old or younger; or
(iii) the patient is monitored for the development of cholestasis, hyperbilirubinemia, or one or more symptoms thereof by evaluating a parameter in a blood sample obtained from the patient, wherein a finding that the parameter is above a reference level identifies the patient as having cholestasis, hyperbilirubinemia, or one or more symptoms thereof.

11. The use of claim 10, wherein:
(i) the parameter comprises the level of a serum bile acid in the blood sample; or
(ii) the parameter comprises one or more results of a liver function test.

12. The use of claim 11, wherein:
(i) the serum bile acid is cholic acid, chenodeoxycholic acid, deoxycholic acid, or ursodeoxycholic acid; or
(ii) the parameter comprises the level of aspartate aminotransferase or alanine aminotransferase in the blood sample.

13. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for treating XLMTM in a human patient in need thereof, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient the viral vector in an amount of less than about 3×10^{14} vg/kg,

(b) monitoring the patient for development of cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and, if the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

14. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for reducing stiffness and/or joint contractures in a human patient diagnosed as having XLMTM, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient the viral vector in an amount of less than about 3×10^{14} vg/kg,

(b) monitoring the patient for development of cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and, if the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

15. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for increasing diaphragm and/or respiratory muscle progression in a human patient diagnosed as having XLMTM, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient the viral vector in an amount of less than about 3×10^{14} vg/kg,

(b) monitoring the patient for development of cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and, if the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

16. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for treating XLMTM in a human patient in need thereof, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient the viral vector in an amount of less than about 3×10^{14} vg/kg,

(b) determining that the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

17. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for reducing stiffness and/or joint contractures in a human patient diagnosed as having

XLMTM, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient the viral vector in an amount of less than about 3 x 10^{14} vg/kg,

(b) determining that the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

18. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for increasing diaphragm and/or respiratory muscle progression in a human patient diagnosed as having XLMTM, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient the viral vector in an amount of less than about 3 x 10^{14} vg/kg,

(b) determining that the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

19. The use of any one of claims 13-18, wherein:
(i) the viral vector is to be administered to the patient in an amount of less than about 2.5 x 10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of less than about 2 x 10^{14} vg/kg, less than about 1.5 x 10^{14} vg/kg, or less than about 1.4 x 10^{14} vg/kg;
(ii) the viral vector is to be administered to the patient in an amount of from about 3 x 10^{13} vg/kg to about 2.3 x 10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of from about 8 x 10^{13} vg/kg to about 1.8 x 10^{14} vg/kg, from about 1 x 10^{14} vg/kg to about 1.6 x 10^{14} vg/kg, from about 1.1 x 10^{14} vg/kg to about 1.5 x 10^{14} vg/kg, or from about 1.2 x 10^{14} vg/kg to about 1.4 x 10^{14} vg/kg;
(iii) the viral vector is to be administered to the patient in an amount of about 1.3 x 10^{14} vg/kg;
(iv) the patient is five years old or younger at the time of administration of the viral vector; or
(v) the patient is from about 1 month old to about 5 years old at the time of administration of the viral vector.

20. The use of claim 15, wherein the patient is four years old or younger at the time of administration of the viral vector, optionally wherein the patient is three years old or younger, two years old or younger, one year old or younger, or six months old or younger.

21. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for treating XLMTM in a human patient in need thereof that is five years old or younger, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient a therapeutically effective amount of the viral vector,

(b) monitoring the patient for development of cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and, if the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

22. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for reducing stiffness and/or joint contractures in a human patient diagnosed as having XLMTM, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient a therapeutically effective amount of the viral vector,

(b) monitoring the patient for development of cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and, if the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

23. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for increasing diaphragm and/or respiratory muscle progression in a human patient diagnosed as having XLMTM, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient a therapeutically effective amount of the viral vector,

(b) monitoring the patient for development of cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and, if the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

24. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for treating XLMTM in a human patient in need thereof that is five years old or younger, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient a therapeutically effective amount of the viral vector,

(b) determining that the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

25. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for reducing stiffness and/or joint contractures in a human patient diagnosed as having XLMTM, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient a therapeutically effective amount of the viral vector,

(b) determining that the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

26. Use of a viral vector comprising a transgene encoding MTM1 in the manufacture of a medicament for increasing diaphragm and/or respiratory muscle progression in a human patient diagnosed as having XLMTM, wherein the viral vector is to be administered to the patient in accordance with a method comprising:

(a) administering to the patient a therapeutically effective amount of the viral vector,

(b) determining that the patient exhibits cholestasis, hyperbilirubinemia, or one or more symptoms thereof, and

(c) administering to the patient an anti-cholestatic agent.

27. The use of any one of claims 21-26, wherein:

(i) the patient is four years old or younger at the time of administration of the viral vector, optionally wherein the patient is three years old or younger, two years old or younger, one year old or younger, or six months old or younger;

(ii) the patient is from about 1 month old to about 5 years old at the time of administration of the viral vector;

(iii) the viral vector is to be administered to the patient in an amount of less than about 3×10^{14} vg/kg;

(iv) the viral vector is to be administered to the patient in an amount of from about 3×10^{13} vg/kg to about 2.3×10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of from about 8×10^{13} vg/kg to about 1.8×10^{14} vg/kg, from about 1×10^{14} vg/kg to about 1.6×10^{14} vg/kg, from about 1.1×10^{14} vg/kg to about 1.5×10^{14} vg/kg, or from about 1.2×10^{14} vg/kg to about 1.4×10^{14} vg/kg; or

(v) the viral vector is to be administered to the patient in an amount of about 1.3×10^{14} vg/kg.

28. The use of claim 27, wherein the viral vector is to be administered to the patient in an amount of less than about 2.5×10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of less than about 2×10^{14} vg/kg, less than about 1.5×10^{14} vg/kg, or less than about 1.4×10^{14} vg/kg.

29. Use of an anti-cholestatic agent in the manufacture of a medicament for treating or preventing cholestasis or hyperbilirubinemia in a human patient that has XLMTM and who has been previously administered a viral vector comprising a transgene encoding MTM1 in an amount of less than about 3×10^{14} vg/kg.

30. The use of claim 29, wherein:

(i) the viral vector is to be administered to the patient in an amount of less than about 2.5 x 10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of less than about 2 x 10^{14} vg/kg, less than about 1.5 x 10^{14} vg/kg, or less than about 1.4 x 10^{14} vg/kg;
(ii) the viral vector is to be administered to the patient in an amount of from about 3 x 10^{13} vg/kg to about 2.3 x 10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of from about 8 x 10^{13} vg/kg to about 1.8 x 10^{14} vg/kg, from about 1 x 10^{14} vg/kg to about 1.6 x 10^{14} vg/kg, from about 1.1 x 10^{14} vg/kg to about 1.5 x 10^{14} vg/kg, or from about 1.2 x 10^{14} vg/kg to about 1.4 x 10^{14} vg/kg;
(iii) the viral vector is to be administered to the patient in an amount of about 1.3 x 10^{14} vg/kg;
(iv) the patient is five years old or younger at the time of administration of the viral vector; or
(v) the patient is from about 1 month old to about 5 years old at the time of administration of the viral vector.

31. The use of claim 30, wherein the patient is four years old or younger at the time of administration of the viral vector, optionally wherein the patient is three years old or younger, two years old or younger, one year old or younger, or six months old or younger.

32. Use of an anti-cholestatic agent in the manufacture of a medicament for treating or preventing cholestasis or hyperbilirubinemia in a human patient that has XLMTM, has been previously administered a viral vector comprising a transgene encoding MTM1, and that was five years old or younger at the time of administration of the viral vector.

33. The use of claim 32, wherein:
(i) the patient was four years old or younger at the time of administration of the viral vector, optionally wherein the patient was three years old or younger, two years old or younger, one year old or younger, or six months old or younger;
(ii) the patient was from about 1 month old to about 5 years old at the time of administration of the viral vector;
(iii) the viral vector is to be administered to the patient in an amount of less than about 3 x 10^{14} vg/kg;
(iv) the viral vector is to be administered to the patient in an amount of from about 3 x 10^{13} vg/kg to about 2.3 x 10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient in an amount of from about 8 x 10^{13} vg/kg to about 1.8 x 10^{14} vg/kg, from about 1 x 10^{14} vg/kg to about 1.6 x 10^{14} vg/kg, from about 1.1 x 10^{14} vg/kg to about 1.5 x 10^{14} vg/kg, or from about 1.2 x 10^{14} vg/kg to about 1.4 x 10^{14} vg/kg; or
(v) the viral vector is to be administered to the patient in an amount of about 1.3 x 10^{14} vg/kg.

34. The use of claim 33, wherein the viral vector is to be administered to the patient in an amount of less than about 2.5 x 10^{14} vg/kg, optionally wherein the viral vector is to be administered to the patient

in an amount of less than about 2 x 10^{14} vg/kg, less than about 1.5 x 10^{14} vg/kg, or less than about 1.4 x 10^{14} vg/kg.

35. The use of any one of claims 1-31, 33, and 34, wherein:
(i) the viral vector is to be administered to the patient in a single dose comprising the amount;
(ii) the viral vector is to be administered to the patient in two or more doses that, together, comprise the amount; or
(iii) the viral vector is to be administered to the patient in two or more doses that each, individually, comprise the amount.

36. The use of claim 35, wherein:
(i) the two or more doses are separated from one another by one year or more; or
(ii) the two or more doses are to be administered to the patient within about 12 months of one another.

37. The use of any one of claims 1-36, wherein the viral vector is selected from the group consisting of adeno-associated virus (AAV), adenovirus, lentivirus, retrovirus, poxvirus, baculovirus, herpes simplex virus, vaccinia virus, and a synthetic virus.

38. The use of claim 37, wherein the viral vector is an AAV.

39. The use of claim 38, wherein:
(i) the AAV is an AAV1, AAV2, AAV3, AAV4, AAV5, AAV6, AAV7, AAV8, AAV9, AAVrh10, or AAVrh74 serotype; or
(ii) the viral vector is a pseudotyped AAV.

40. The use of claim 39, wherein the pseudotyped AAV is AAV2/8 or AAV2/9, optionally wherein the pseudotyped AAV is AAV2/8.

41. The use of any one of claims 1-40, wherein the transgene encoding MTM1 is operably linked to a muscle specific promoter.

42. The use of claim 41, wherein the muscle specific promotor is a desmin promoter, a muscle creatine kinase promoter, a myosin light chain promoter, a myosin heavy chain promoter, a cardiac troponin C promoter, a troponin I promoter, a myoD gene family promoter, an actin alpha promoter, an actin beta promoter, an actin gamma promoter, or a promoter within intron 1 of ocular paired like homeodomain 3.

43. The use of claim 42, wherein the muscle specific promoter is a desmin promoter.

44. The use of any one of claims 1-43, wherein:

(i) the viral vector is resamirigene bilparvovec; or
(ii) the viral vector is to be administered to the patient by way of intravenous, intramuscular, intradermal, or subcutaneous administration.

45. The use of any one of claims 1-44, wherein the anti-cholestatic agent is selected from the group consisting of a bile acid, a farnesoid X receptor (FXR) ligand, a fibroblast growth factor 19 (FGF-19) mimetic, a Takeda-G-protein-receptor-5 (TGR5) agonist, a peroxisome proliferator-activated receptor (PPAR) agonist, a PPAR-alpha agonist, a PPAR-delta agonist, a dual PPAR-alpha and PPAR-delta agonist, an apical sodium-dependent bile acid transporter (ASBT) inhibitor, an immunomodulatory drug, an antifibrotic therapy, and a nicotinamide adenine dinucleotide phosphate oxidase (NOX) inhibitor.

46. The use of claim 45, wherein:
(i) the FXR ligand is obeticholic acid, cilofexor, tropifexor, tretinoin, or EDP-305;
(ii) the FGF-19 mimetic is aldafermin;
(iii) the TGR5 agonist is INT-777 or INT-767;
(iv) the PPAR agonist is bezafibrate, seladelpar, or elafibrinor;
(v) the PPAR-alpha agonist is fenofibrate;
(vi) the PPAR-delta agonist is seladelpar;
(vii) the dual PPAR-alpha and PPAR-delta agonist is elafibranor;
(viii) the ASBT inhibitor is odevixibat, maralixibat, or linerixibat;
(ix) the immunomodulatory drug is rituximab, abatacept, ustekinumab, infliximab, baricitinib, or FFP-104;
(x) the antifibrotic therapy is a vitamin D receptor agonist or simtuzumab; or
(xi) the NOX inhibitor is setanaxib.

47. The use of claim 45, wherein the bile acid is ursodeoxycholic acid, nor-ursodeoxycholic acid, or a pharmaceutically acceptable salt thereof.

48. The use of claim 47, wherein the bile acid is ursodiol.

49. The use of any one of claims 45, 47, and 48, wherein:
(i) the bile acid is to be administered to the patient in a single dose; or
(ii) the bile acid is to be administered to the patient in a plurality of doses.

50. The use of claim 49, wherein the bile acid is to be administered to the patient in an amount of from about 5 mg/kg/dose to about 20 mg/kg/dose, optionally wherein the bile acid is to be administered to the patient in an amount of from about 6 mg/kg/dose to about 19 mg/kg/dose, from about 7 mg/kg/dose to about 18 mg/kg/dose, from about 8mg/kg/dose to about 17 mg/kg/dose, from about 10 mg/kg/dose to about 15 mg/kg/dose, or from about 12 mg/kg/dose to about 13 mg/kg/dose.

51. The use of claim 50, wherein the bile acid is to be administered to the patient in an amount of from about 5 mg/kg/dose to about 11 mg/kg/dose, optionally wherein the bile acid is to be administered to the patient in an amount of from about 6 mg/kg/dose to about 10 mg/kg/dose, or from about 7 mg/kg/dose to about 9 mg/kg/dose.

52. The use of claim 51, wherein the bile acid is to be administered to the patient in one or more doses per day, week, or month.

53. The use of claim 52, wherein the bile acid is to be administered to the patient in one or more doses per day, optionally wherein the bile acid is to be administered to the patient in one dose per day, in two doses per day, three doses per day, four doses per day, or five doses per day.

54. The use of claim 53, wherein the bile acid is to be administered to the patient in one dose per day.

55. The use of any one of claims 45-54, wherein:
(i) the bile acid is to be administered to the patient in an amount of from about 5 mg/kg/day to about 40 mg/kg/day, optionally wherein (i) the bile acid is to be administered to the patient in an amount of from about 6 mg/kg/day to about 39 mg/kg/day, from about 8 mg/kg/day to about 37 mg/kg/day, from about 13 mg/kg/day to about 32 mg/kg/day, or from about 20 mg/kg/day to about 25 mg/kg/day, or (ii) the bile acid is to be administered to the patient in an amount of from about 17 mg/kg/day to about 23 mg/kg/day, from about 18 mg/kg/day to about 22 mg/kg/day, or from about 19 mg/kg/day to about 21 mg/kg/day;
(ii) the bile acid is to be administered to the patient in an amount of 20 mg/kg/day;
(iii) the bile acid is to be administered to the patient by way of a unit dosage form comprising 250 mg of the bile acid;
(iv) the bile acid is to be administered to the patient by way of a unit dosage form comprising 500 mg of the bile acid; or
(v) the bile acid is to be administered to the patient by way of enteral administration.

56. The use of any one of claims 1-55, wherein:
(i) the patient does not have a history of cholestasis or hyperbilirubinemia;
(ii) the patient was born at greater than or equal to 35 weeks of gestational age and is or was from term age to about 5 years old at the time of administration of the viral vector;
(iii) the patient is male; or
(iv) the patient requires mechanical ventilatory support, optionally wherein mechanical ventilatory support comprises invasive mechanical ventilatory support and noninvasive mechanical ventilatory support.

57. The use of claim 56, wherein the patient does not have a history of any underlying liver disease.

58. The use of any one of claims 9-57, wherein:
(i) the patient is determined to exhibit cholestasis or one or more symptoms thereof by a finding that the patient exhibits a serum total bile acids level that is greater than 14 µmol/L;
(ii) the patient is determined to exhibit cholestasis or one or more symptoms thereof by a finding that the patient exhibits one or more parameters in a blood test that is increased or decreased relative to a reference level;
(iii) the one or more parameters comprises the level of gamma-glutamyl transferase, alkaline phosphatase, aspartate aminotransferase, and/or alanine aminotransferase;
(iv) the patient is determined to exhibit hyperbilirubinemia or one or more symptoms thereof by a finding that the patient exhibits a bilirubin level that is greater than 1 mg/dL in a bilirubin test; or
(v) the patient is determined to exhibit cholestasis, hyperbilirubinemia, or one or more symptoms thereof by a finding that the patient exhibits a parameter in blood test that is increased relative to a reference level.

59. The use of claim 58, wherein:
(i) the blood test is a liver function test;
(ii) upon administering the viral vector to the patient, the patient displays a bilirubin level that is greater than 1 mg/dL in a bilirubin test by about 3 weeks after administration of the viral vector to the patient;
(iii) the bilirubin level comprises a direct bilirubin level or a total bilirubin level;
(iv) the parameter comprises the level of a serum bile acid;
(v) the parameter comprises one or more results of a liver function test; or
(vi) the one or more parameters comprises the level of gamma-glutamyl transferase, alkaline phosphatase, aspartate aminotransferase, and/or alanine aminotransferase.

60. The use of claim 59, wherein:
(i) the serum bile acid is cholic acid, chenodeoxycholic acid, deoxycholic acid, or ursodeoxycholic acid; or
(ii) the parameter comprises the level of aspartate aminotransferase or alanine aminotransferase.

61. A kit comprising a viral vector comprising a transgene encoding MTM1 and a package insert, wherein the package insert instructs a user of the kit to administer the viral vector to a patient having XLMTM in accordance with the use of any one of claims 1-31 and 35-60.

62. A kit comprising an anti-cholestatic agent and a package insert, wherein the package insert instructs a user of the kit to administer the anti-cholestatic agent to a patient to treat or prevent cholestasis or hyperbilirubinemia in accordance with the use of any one of claims 32-60.