CLAIMS

1. A nucleic acid comprising nucleotides 7 to 2248 of SEQ ID NO: 11.

2. A recombinant adeno-associated virus particle (rAAV) comprising the nucleic acid of claim 1, wherein the rAAV is infectious.

3. The rAAV particle of claim 2, wherein the AAV is AAV-1.

4. A composition comprising the rAAV particle of claim 2 or 3 and a pharmaceutically acceptable carrier.

5. The composition of claim 4, wherein the composition is formulated to treat a muscle wasting disorder or neuropathy in a subject in need thereof or is formulated to stimulate muscle growth in a subject in need thereof.

6. A composition comprising a nucleic acid encoding a NT-3 polypeptide suitable for use in treating a muscle wasting disorder or neuropathy in a human subject or improving muscle strength in a human subject or stimulating muscle growth in a human subject suffering from a muscle wasting disorder, wherein:

a) the nucleic acid comprises the nucleotide sequence of claim 1,

b) the nucleic acid is expressed by the rAAV of claim 2 or 3;

c) the nucleic acid is expressed by a recombinant adeno-associated virus (rAAV) scAAV1 comprising the nucleic acid of claim 1, and the rAAV is administered at a dose that results in sustained expression of a low concentration of NT-3 polypeptide,

d) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the composition is formulated for an intramuscular route of administration and the dose of the rAAV is $1.5x10^{12}$ vg/kg to $6.5x10^{12}$ vg/kg,

e) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the

composition is formulated for an intramuscular route of administration and the dose of the rAAV is $2x10^{12}$ vg/kg to $6x10^{12}$ vg/kg,

f) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the composition is formulated for an intramuscular route of administration and the dose of the rAAV is $2x10^{12}$ vg/kg,

g) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the composition is formulated for an intramuscular route of administration and the dose of the rAAV is $4x10^{12}$ vg/kg,

h) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the composition is formulated for an intramuscular route of administration and the dose of the rAAV administered is $6x10^{12}$ vg/kg,

i) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the composition is formulated for an intramuscular injection at a concentration of $2x10^{13}$ vg/ml administered using 3 to 6 injections per muscle of 0.5 to 1 ml, or

j) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the composition is formulated for an intramuscular injection at a concentration of $2x10^{13}$ vg/ml administered using multiple injections at a total volume of 5 to 14 ml.

7. The composition of claim 6, wherein the composition is formulated for intramuscular injection.

8. The composition of claim 6 or 7, wherein the improvement in the muscle strength is measured as a decrease in composite score on the CMT Pediatric Scale (CMTPeds) or as a decrease in disease progression over a two year time period.

9. Use of a nucleic acid encoding the NT-3 polypeptide for the manufacture of a medicament for treating a muscle wasting disorder or neuropathy in a human subject or

improving muscle strength or stimulating muscle growth in a human subject suffering from a muscle wasting disorder, wherein:

a) the nucleic acid comprises the nucleotide sequence of claim 1,

b) the nucleic acid is expressed by the rAAV of claim 2 or 3;

c) the nucleic acid is expressed by a recombinant adeno-associated virus (rAAV) scAAV1 comprising the nucleic acid of claim 1, and the rAAV is at a dose that results in sustained expression of a low concentration of NT-3 polypeptide,

d) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the medicament is formulated for an intramuscular route of administration and the dose of the rAAV is $1.5x10^{12}$ vg/kg to $6.5x10^{12}$ vg/kg,

e) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the medicament is formulated for an intramuscular route of administration and the dose of the rAAV is $2x10^{12}$ vg/kg to $6x10^{12}$ vg/kg,

f) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the medicament is formulated for an intramuscular route of administration and the dose of the rAAV is $2x10^{12}$ vg/kg,

g) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the medicament is formulated for an intramuscular route of administration and the dose of the rAAV is $4x10^{12}$ vg/kg,

h) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the medicament is formulated for an intramuscular route of administration and the dose of the rAAV administered is $6x10^{12}$ vg/kg,

i) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the medicament is formulated for an intramuscular injection at a concentration of $2x10^{13}$ vg/ml administered using 3 to 6 injections per muscle of 0.5 to 1 ml, or

j) the nucleic acid encoding the NT-3 polypeptide is the recombinant adeno-associated virus (rAAV) scAAV1.tMCK.NTF3 comprising nucleotides 7-2248 of SEQ ID NO: 11, the medicament is formulated for an intramuscular injection at a concentration of $2x10^{13}$ vg/ml administered using multiple injections at a total volume of 5 to 14 ml.

10. The use of claim 9, wherein the medicament is formulated for an intramuscular injection.

11. The use of claim 9 or 10, wherein the improvement in the muscle strength is measured as a decrease in composite score on the CMT Pediatric Scale (CMTPeds) or as a decrease in disease progression over a two year time period.

12. The composition of any one of claims 6-8, or use of any one of claims 9-11, wherein the ~~subject is suffering from~~ muscle wasting disorder or neuropathy is Charcot-Marie-Tooth (CMT) neuropathy.

13. The composition of any one of claims 6-8, or use of ~~claim 12~~ any one of claims 9-11, wherein the subject has one of the genetic variants provided in Table 1, the genetic variant Val30Met in the transthyretin (TTR) gene, the genetic variant Ile107Val in the transthyretin (TTR) gene or the genetic variant Ser77Tyr in the transthyretin (TTR) gene.

14. The composition of any one of claims 6-8 ~~or 12-13~~, or use of any one of claims 9-~~13~~11, wherein the ~~subject is suffering from~~ muscle wasting disorder or neuropathy is a transthyretin amyloid neuropathy, an acquired neuropathy caused by cancer, diabetes mellitus, human immunodeficiency virus (HIV) infection, thyroid disorder, hypothyroidism, hypoglycemia, uremia, renal insufficiency, hepatic dysfunction, hepatic failure, polycythemia, connective tissue disorders, lyme disease, celiac disease, leprosy, porphyria, Sjogren's syndrome, poliomyelitis, acromegaly, disorders of lipid/glycolipid metabolism, West Nile syndrome, amyloidosis, mitochondrial disorders, dysproteinemic disorders, monoclonal gammapathy of undetermined significance (MGUS), POEMS syndrome, nutritional/vitamin deficiency, vitamin B_{12} deficiency, vitamin E deficiency, copper deficiency, hereditary myopathy, peripheral neuropathy, toxic neuropathy, autoimmune peripheral polyneuropathy, acute inflammatory demyelinating polyradiculoneuropathy (AIDP), chronic inflammatory demyelinating

polyradiculoneuropahty (CIDP), vasculitic mononeuritis multiplex, paraneuropathy, idiopathic ganglionitis, amyotrophic lateral sclerosis, multifocal motor conduction lock neuropathy, or lower motor neuron syndrome, neuromuscular disease, muscular atrophy, drug-induced myopathy, sarcopenia, cachexia, type II muscle fiber atrophy, age-related muscular atrophy or an acquired autoimmune primary muscle disorder.