Claims:

1. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of treating a neurodegenerative disease or one or more symptoms associated with a neurodegenerative disease in a subject in need thereof, wherein the neurodegenerative disease is not cerebellar ataxia or Niemann-Pick type C disease.

2. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 1, wherein the neurodegenerative disease is associated with lysosomal dysfunction.

3. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 2, wherein the neurodegenerative disease associated with lysosomal dysfunction is chosen from alcoholism, Alzheimer's disease, amyotrophic lateral sclerosis (ALS), Canavan disease, frontotemporal lobar degeneration, Huntington's disease, multiple system atrophy (MSA-P/MSA-C), multiple sclerosis, narcolepsy, Parkinson's Disease, Smith Lemli Opitz Syndrome (SLOS) (an inborn error of cholesterol synthesis), Tangier disease, Pelizaeus-Merzbacher Disease, Pick's disease, frontotemporal dementia with parkinsonism, prion diseases, progressive supranuclear palsy, spinal muscular atrophy, and neurodegenerative lysosomal storage disorders (LSDs).

4. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 3, wherein the neurodegenerative disease associated with lysosomal dysfunction is chosen from ALS, MSA-P, MSA-C, frontotemporal dementia with parkinsonism, progressive supranuclear palsy, and Alzheimer's disease.

5. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 1, wherein the neurodegenerative disease is chosen from Alexander's disease, Alper's disease, cerebral palsy, Cockayne syndrome, corticobasal degeneration, HIV-associated dementia, Kennedy's disease, neuroborreliosis, primary lateral sclerosis, , Refsum's disease, Schilder's disease, subacute combined degeneration of spinal cord secondary to pernicious anaemia, hereditary motor and sensory neuropathy with proximal dominance, Wobbly Hedgehog Syndrome (WHS), progressive muscular atrophy (Duchenne-Aran muscular atrophy), progressive bulbar palsy, pseudobulbar palsy, HIV-associated neurocognitive disorders (HAND), Vascular Parkinsonism, lower body Parkinson's syndrome, and cerebellar downbeat nystagmus.

6. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 1, wherein the neurodegenerative disease is a Motor Neuron Disease.

7. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of treating a neurodegenerative disease in a subject in need thereof, wherein the subject is asymptomatic.

8. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 7, wherein the subject has been found to have a genetic and/or biochemical marker of the neurodegenerative disease.

9. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of delaying onset of a neurodegenerative disease or one or more symptoms of a neurodegenerative disease that would otherwise be expected to manifest according to typical disease progression.

10. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of treating a neurodegenerative disease or one or more symptoms associated with a neurodegenerative disease in a subject in need thereof, wherein the method comprises administering a therapeutically effective amount of the acetyl-leucine to the subject in need thereof for a duration chosen from at least about 3 months, at least about 6 months, at least about 1 year, at least about 2 years, and at least about 5 years.

11. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of delaying progression of a neurodegenerative disease or one or more symptoms associated with a neurodegenerative disease over time as compared to typical disease progression, wherein the method comprises administering a therapeutically effective amount of the acetyl-leucine to the subject in need thereof for a duration chosen from at least about 3 months, at least about 6 months, at least about 1 year, at least about 2 years, and at least about 5 years.

12. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of reversing progression of a neurodegenerative disease or one or more symptoms associated with a neurodegenerative disease over time, wherein the method comprises administering a therapeutically effective amount of the acetyl-leucine to the subject in need thereof for a duration chosen from at least about 3 months, at least about 6 months, at least about 1 year, at least about 2 years, and at least about 5 years.

13. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of improving in a subject in need thereof a biochemical marker of a neurodegenerative disease over time, wherein the method comprises administering a therapeutically effective amount of the acetyl-leucine to the subject in need thereof for a duration chosen from at least about 3 months, at least about 6 months, at least about 1 year, at least about 2 years, and at least about 5 years.

14. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 13, wherein the biochemical marker is increased lysosomal volume.

15. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 1-14, wherein the method comprises initiating administration of a therapeutically effective amount of acetyl-leucine to the subject in need thereof when the subject is asymptomatic.

16. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 15, wherein the initial administration occurs after the subject has been found to have a genetic and/or biochemical marker of the neurodegenerative disease.

17. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 1-9, wherein the method comprises administering a therapeutically effective amount of the acetyl-leucine to the subject in need thereof for a duration chosen from at least about 3 months, at least about 6 months, at least about 1 year, at least about 2 years, and at least about 5 years.

18. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 1-17, wherein the acetyl-leucine is acetyl-DL-leucine.

19. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 1-17, wherein the acetyl-leucine has an enantiomeric excess of the L-enantiomer or the D-enantiomer.

20. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 1-9, wherein the method comprises administering the acetyl-leucine to the subject in need thereof at a therapeutically effect amount of from about 1 g to about 15 g per day, from about 1 g to about 10 g per day, from about 1.5 g to about 7 g per day, from about 4 g to about 6 g per day, or from about 4 g to about 5 g per day.

21. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 10-17, wherein the therapeutically effective amount of acetyl-leucine is from about 1 g to about 15 g per day, from about 1 g to about 10 g per day, from about 1.5 g to about 7 g per day, from about 4 g to about 6 g per day, or from about 4 g to about 5 g per day.

22. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 7-14, wherein the neurodegenerative disease is associated with lysosomal dysfunction.

23. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 22, wherein the neurodegenerative disease associated with lysosomal dysfunction is chosen from alcoholism, Alzheimer's disease, amyotrophic lateral sclerosis (ALS), Canavan disease, frontotemporal lobar degeneration, Huntington's disease, multiple system atrophy (MSA-P/MSA-C), multiple sclerosis, narcolepsy, Parkinson's Disease, Smith Lemli Opitz Syndrome (SLOS) (an inborn error of cholesterol synthesis), Tangier disease, Pelizaeus-Merzbacher Disease, Pick's disease, frontotemporal dementia with parkinsonism, prion diseases, progressive supranuclear palsy, spinal muscular atrophy, neurodegenerative lysosomal storage disorders (LSDs), Spinocerebellar Ataxia (SCA) 1, Spinocerebellar Ataxia (SCA) 2, Spinocerebellar Ataxia (SCA) 3 (Machado-Joseph disease), Spinocerebellar Ataxia (SCA) 6, Spinocerebellar Ataxia (SCA) 7, Spinocerebellar Ataxia (SCA) 17, dentatorubral-pallidoluysian atrophy, Autosomal Recessive Spastic Ataxia of Charlevoix-Saguenay (ARSACS), autosomal recessive cerebellar ataxia type 1 (Recessive Ataxia of Beauce (RAB), SYNE-1 mutation), autosomal recessive cerebellar ataxia type 2 (spinocerebellar ataxia autosomal recessive 9, SCAR9), ataxia with vitamin E deficiency (AVED), ataxia telangiectasia (Louis Barr disease), Freidreich's ataxia (FRDA), and ataxia with coenzyme Q10 deficiency.

24. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to claim 23, wherein the neurodegenerative disease associated with lysosomal dysfunction is chosen from ALS, MSA-P, MSA-C, frontotemporal dementia with parkinsonism, progressive supranuclear palsy, Alzheimer's disease, SCA 1, and ataxia telangiectasia.

25. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 7-14, wherein the neurodegenerative disease is chosen from Alexander's disease, Alper's disease, cerebral palsy, Cockayne syndrome, corticobasal degeneration, HIV-associated dementia, Kennedy's disease,

neuroborreliosis, primary lateral sclerosis, , Refsum's disease, Schilder's disease, subacute combined degeneration of spinal cord secondary to pernicious anaemia, hereditary motor and sensory neuropathy with proximal dominance, Wobbly Hedgehog Syndrome (WHS), progressive muscular atrophy (Duchenne-Aran muscular atrophy), progressive bulbar palsy, pseudobulbar palsy, HIV-associated neurocognitive disorders (HAND), Vascular Parkinsonism, lower body Parkinson's syndrome, cerebellar ataxia, and cerebellar downbeat nystagmus.

26. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 7-14, wherein the neurodegenerative disease is a Motor Neuron Disease.

27. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of reducing the severity of a neurodegenerative disease or reducing the severity of or eliminating one or more existing symptoms associated with a neurodegenerative disease in a subject in need thereof, wherein the neurodegenerative disease is not cerebellar ataxia or Niemann-Pick Type C.

28. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method of providing neuroprotection in a subject having, suspected of having, or at risk of having a neurodegenerative disease, wherein the method comprises administering a therapeutically effective amount of the acetyl-leucine to the subject for a duration chosen from at least about 3 months, at least about 6 months, at least about 1 year, at least about 2 years, and at least about 5 years.

29. Acetyl-leucine, or a pharmaceutically acceptable salt thereof, for use in a method according to any of claims 7, 9-13, and 28, wherein the neurodegenerative disease is chosen from ALS, MSA-P, MSA-C, frontotemporal dementia with parkinsonism, progressive supranuclear palsy, Alzheimer's disease, SCA 1, ataxia telangiectasia, cerebellar downbeat nystagmus, SCA 28, ataxia with oculomotor apraxia type 4 (AOA4), and corticobasal degeneration.