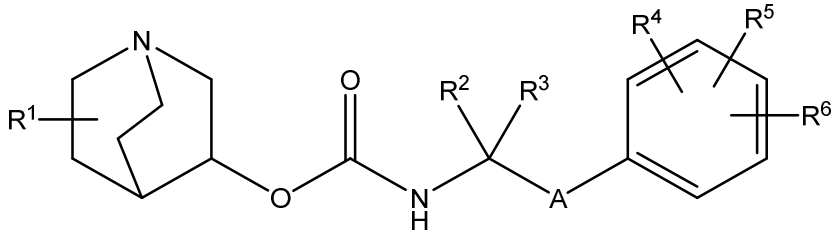
CLAIMS

1. A compound for use in a method for treating or preventing supranuclear gaze palsies (e.g., associated with a lysosome storage disease), in a subject in need thereof, wherein the compound is a compound of formula (I),



(I)

or a pharmaceutically acceptable salt or prodrug thereof, wherein:

R^1 is selected from hydrogen, halogen (e.g., fluorine), cyano, nitro, hydroxy, thio, amino, C_{1-6}-alkyl (e.g., methyl or ethyl), C_{2-6}-alkenyl, C_{2-6}-alkynyl, C_{1-6}-alkyloxy, C_{2-6}-alkenyloxy, and C_{2-6}-alkynyloxy, wherein said alkyl, alkenyl, alkynyl, alkyloxy, alkenyloxy, or alkynyloxy is optionally substituted with one or more (e.g., 1, 2 or 3) groups selected from halogen, cyano, nitro, hydroxy, thio or amino;

R^2 and R^3 are independently selected from C_{1-3}-alkyl, optionally substituted by one or more (*e.g.* 1, 2 or 3) halogens, or R^2 and R^3 together form a cyclopropyl or cyclobutyl group, optionally substituted by one or more (*e.g.* 1 or 2) halogens;

R^4, R^5 and R^6 are each independently selected from hydrogen, halogen, nitro, hydroxy, thio, amino, C_{1-6}-alkyl, and C_{1-6}-alkyloxy, wherein said alkyl or alkyloxy is optionally substituted by one or more (*e.g.* 1, 2 or 3) groups selected from halogen, hydroxy, cyano, and C_{1-6}-alkyloxy; and

A is a 5- or 6-membered aryl or heteroaryl group (e.g., phenyl or thiazolyl), optionally substituted with 1, 2 or 3 groups independently selected from halogen, hydroxy, thio, amino, nitro, C_{1-6}alkoxy and C_{1-6}alkyl.

2. The compound for use of claim 1, wherein:

R^1 is selected from hydrogen, fluorine, methyl and ethyl, wherein said methyl or ethyl is optionally substituted by 1 or 2 groups selected from halogen, hydroxy, thio or amino;

R^2 and R^3 are each independently selected from methyl and ethyl groups, optionally substituted with one or more fluorines;

R^4 is selected from a halogen (e.g., fluorine), C_{1-3}-alkyl (e.g., methyl) and C_{1-3}-alkyloxy (e.g., methoxy or ethoxy), wherein said alkyl or alkyoxy is optionally substituted by one or more (e.g., 1, 2 or 3) groups selected from a halogen and C_{1-3}-alkyloxy (e.g., methoxy or ethoxy);

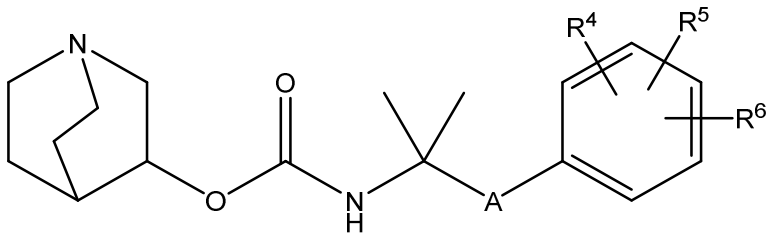
R^5 and R^6 are each hydrogen; R^4 is fluorine or 2-methoxyethoxy, and R^5 and R^6 are hydrogen;

R^4 is positioned at the 4-position of the phenyl ring to which it is attached (i.e., *para* to the A substituent);

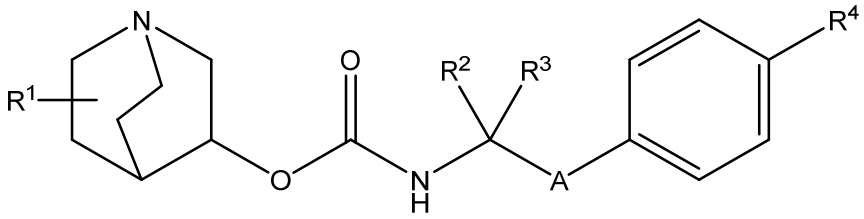
A is phenyl, optionally substituted with 1, 2 or 3 groups independently selected from halogen, hydroxy, thio, amino, nitro, C_{1-6}alkoxy and C_{1-6}alkyl (e.g., methyl), optionally wherein the two groups attached to the A substituent are positioned in a 1,3- or a 1,4- relationship to each other (i.e., meta or para); and/or

A is a 5-membered heteroaryl group which contains 1 or 2 heteroatoms selected from N and S, optionally wherein the two groups attached to the A substituent are positioned in a 1,3- relationship to each other (i.e., meta).

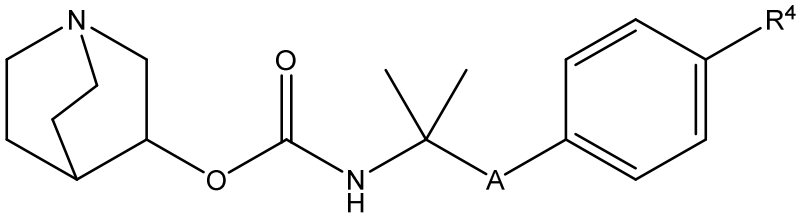
3. The compound for use of claim 1 or claim 2, wherein said compound is a compound of formula (II), (III), (IV), (V), (VI), (VII), (VIII), (IX) or (XI),



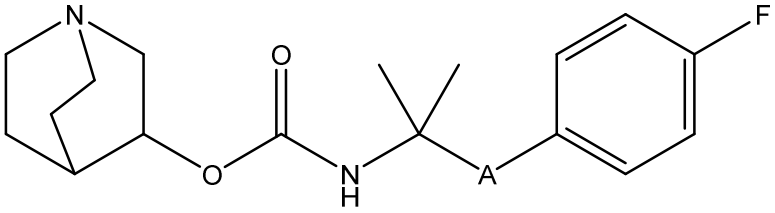
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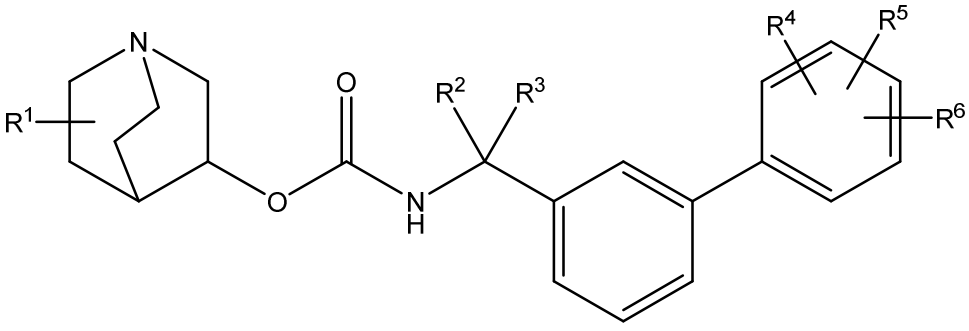
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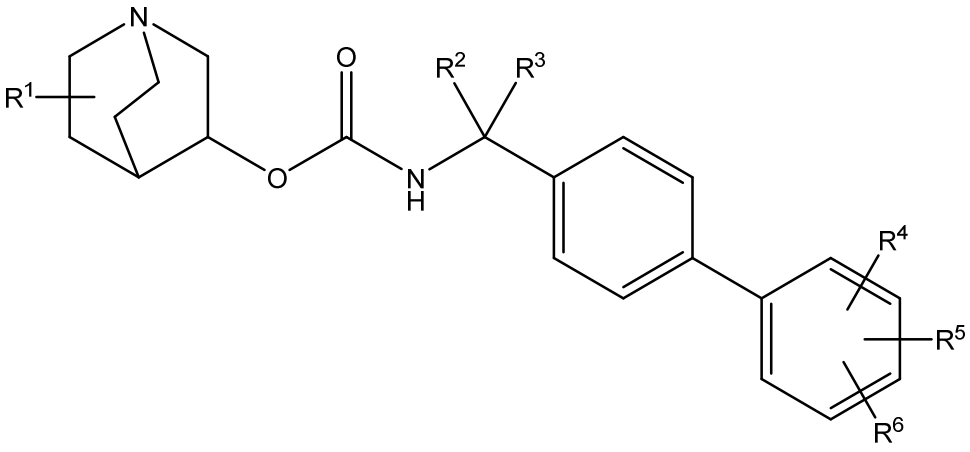
(IV)



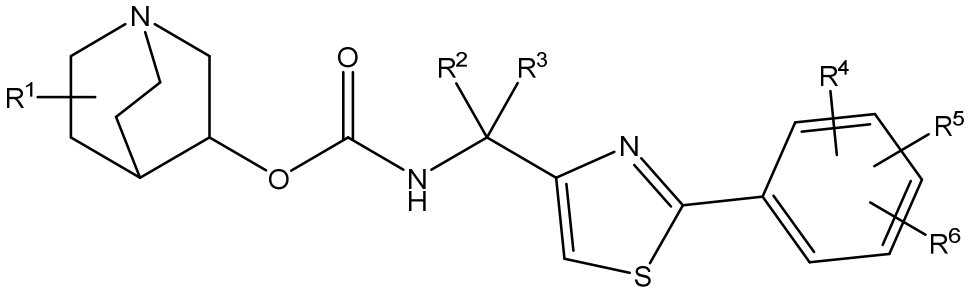
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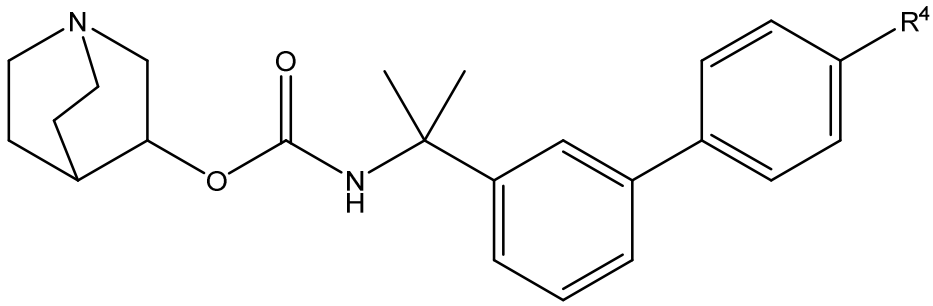
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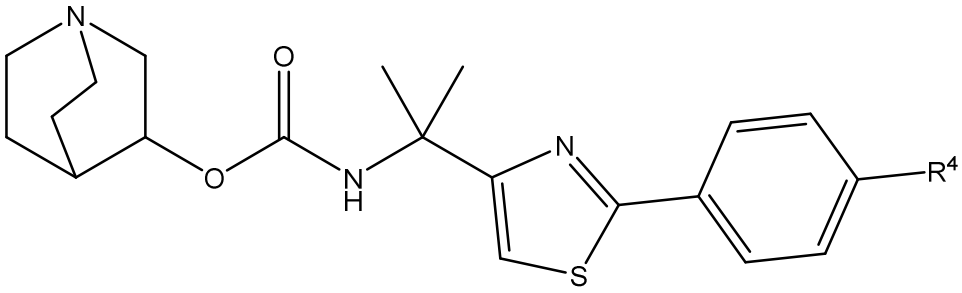
(VII)



(VIII)



(IX)



(XI)

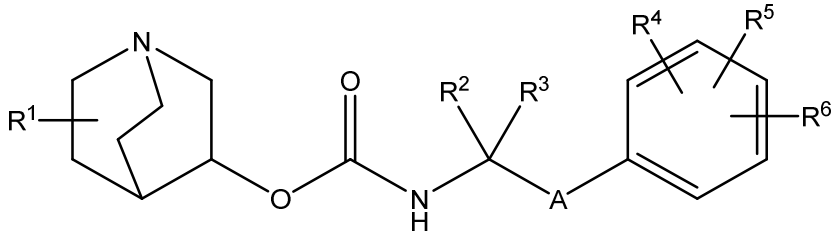
or a pharmaceutically acceptable salt or prodrug thereof.

4. The compound for use of claim 3, wherein said compound is a compound of formula (IX) or (XI) and wherein R^4 is fluorine.

5. The compound for use of claim 1, wherein said compound is selected from: quinuclidin-3-yl (2-(4'-fluoro-[1,1'-biphenyl]-3-yl)propan-2-yl)carbamate; (*S*)-quinuclidin-3-yl (2-(2-(4-fluorophenyl)thiazol-4-yl)propan-2-yl)carbamate; (*S*)-quinuclidin-3-yl (2-(4'-(2-methoxyethoxy)-[1,1'-biphenyl]-4-yl)propan-2-yl)carbamate; and the pharmaceutically acceptable salts and prodrugs thereof.

6. The compound for use of any one of claims 1 to 5, wherein the supranuclear gaze palsy is a conjugate gaze palsy.

7. A compound for use in a method for treating or preventing cognitive dysfunction and/or gait abnormalities, including ataxia, associated with a lysosome storage disease, in a subject in need thereof, wherein the compound is a compound of formula (I),



(I)

or a pharmaceutically acceptable salt or prodrug thereof, wherein:

R^1 is selected from hydrogen, halogen (e.g., fluorine), cyano, nitro, hydroxy, thio, amino, C_{1-6}-alkyl (e.g., methyl or ethyl), C_{2-6}-alkenyl, C_{2-6}-alkynyl, C_{1-6}-alkyloxy, C_{2-6}-alkenyloxy, and C_{2-6}-alkynyloxy, wherein said alkyl, alkenyl, alkynyl, alkyloxy, alkenyloxy, or alkynyloxy is optionally substituted with one or more (e.g., 1, 2 or 3) groups selected from halogen, cyano, nitro, hydroxy, thio or amino;

R^2 and R^3 are independently selected from C_{1-3}-alkyl, optionally substituted by one or more (*e.g.* 1, 2 or 3) halogens, or R^2 and R^3 together form a cyclopropyl or cyclobutyl group, optionally substituted by one or more (*e.g.* 1 or 2) halogens;

R^4, R^5 and R^6 are each independently selected from hydrogen, halogen, nitro, hydroxy, thio, amino, C_{1-6}-alkyl, and C_{1-6}-alkyloxy, wherein said alkyl or alkyloxy is

optionally substituted by one or more (*e.g.* 1, 2 or 3) groups selected from halogen, hydroxy, cyano, and C_{1-6}-alkyloxy; and
A is a 5- or 6-membered aryl or heteroaryl group (e.g., phenyl or thiazolyl), optionally substituted with 1, 2 or 3 groups independently selected from halogen, hydroxy, thio, amino, nitro, C_{1-6}alkoxy and C_{1-6}alkyl.

8. The compound for use of claim 7, wherein:

R^1 is selected from hydrogen, fluorine, methyl and ethyl, wherein said methyl or ethyl is optionally substituted by 1 or 2 groups selected from halogen, hydroxy, thio or amino;

R^2 and R^3 are each independently selected from methyl and ethyl groups, optionally substituted with one or more fluorines;

R^4 is selected from a halogen (e.g., fluorine), C_{1-3}-alkyl (e.g., methyl) and C_{1-3}-alkyloxy (e.g., methoxy or ethoxy), wherein said alkyl or alkyoxy is optionally substituted by one or more (e.g., 1, 2 or 3) groups selected from a halogen and C_{1-3}-alkyloxy (e.g., methoxy or ethoxy);

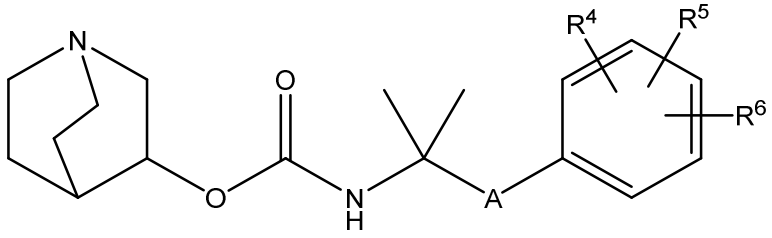
R^5 and R^6 are each hydrogen; R^4 is fluorine or 2-methoxyethoxy, and R^5 and R^6 are hydrogen;

R^4 is positioned at the 4-position of the phenyl ring to which it is attached (i.e., *para* to the A substituent);

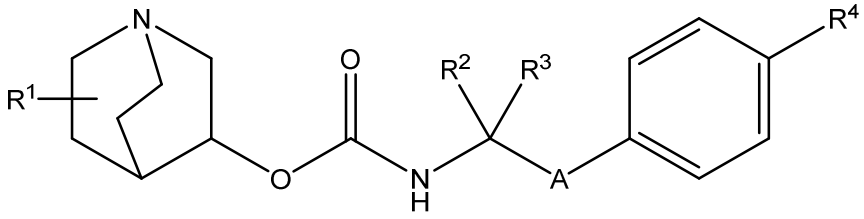
A is phenyl, optionally substituted with 1, 2 or 3 groups independently selected from halogen, hydroxy, thio, amino, nitro, C_{1-6}alkoxy and C_{1-6}alkyl (e.g., methyl), optionally wherein the two groups attached to the A substituent are positioned in a 1,3- or a 1,4- relationship to each other (i.e., meta or para); and/or

A is a 5-membered heteroaryl group which contains 1 or 2 heteroatoms selected from N and S, optionally wherein the two groups attached to the A substituent are positioned in a 1,3- relationship to each other (i.e., meta).

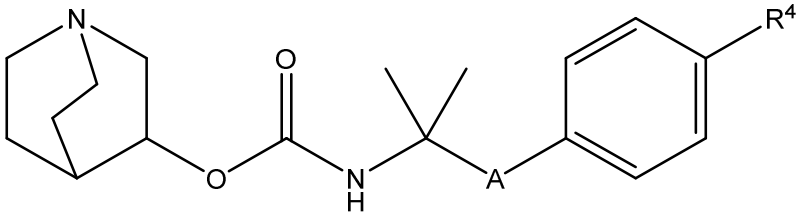
9. The compound for use of claim 7 or claim 8, wherein said compound is a compound of formula (II), (III), (IV), (V), (VI), (VII), (VIII), (IX) or (XI),



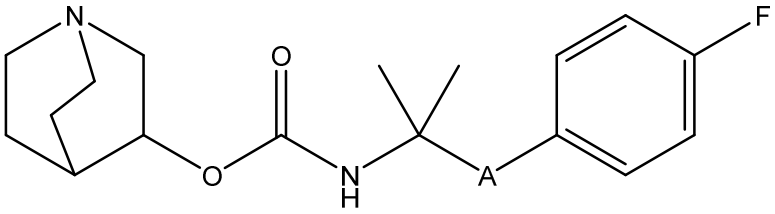
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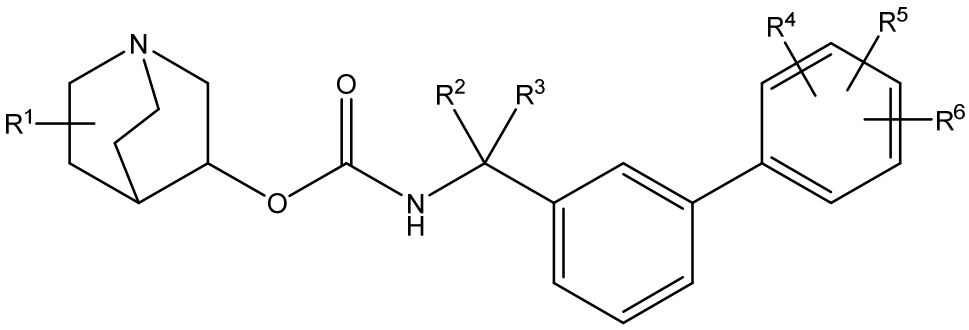
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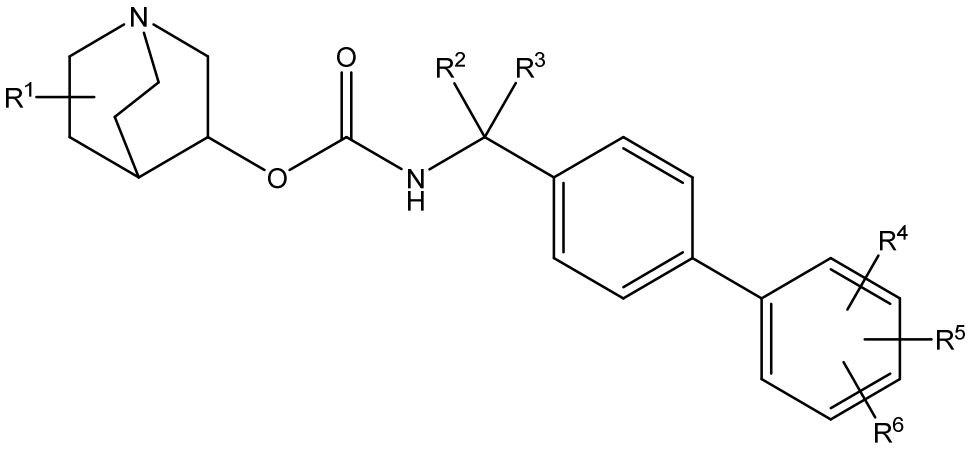
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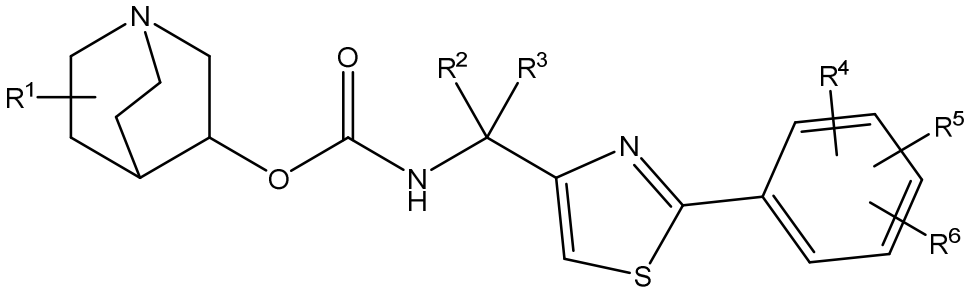
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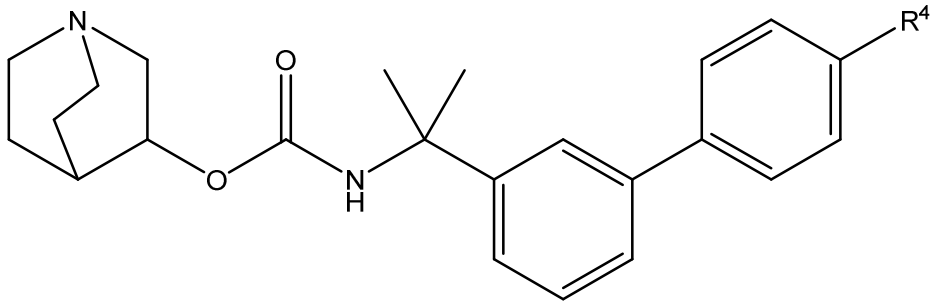
(VI)



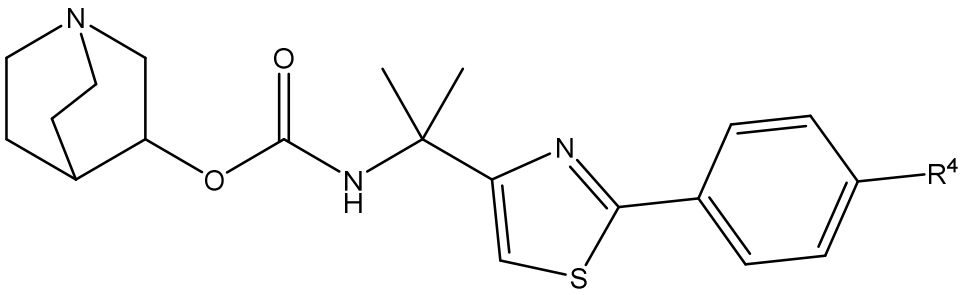
(VII)



(VIII)



(IX)



(XI)

or a pharmaceutically acceptable salt or prodrug thereof.

10. The compound for use of claim 9, wherein said compound is a compound of formula (IX) or (XI) and wherein R^4 is fluorine.

11. The compound for use of claim 7, wherein said compound is selected from: quinuclidin-3-yl (2-(4'-fluoro-[1,1'-biphenyl]-3-yl)propan-2-yl)carbamate; (*S*)-quinuclidin-3-yl (2-(2-(4-fluorophenyl)thiazol-4-yl)propan-2-yl)carbamate; (*S*)-quinuclidin-3-yl (2-(4'-(2-methoxyethoxy)-[1,1'-biphenyl]-4-yl)propan-2-yl)carbamate; and the pharmaceutically acceptable salts and prodrugs thereof.

12. The compound for use of any one of claims 7 to 11, wherein the ataxia is a cerebellar ataxia, optionally wherein the ataxia shows symptoms selected from gait instability, asthenia, asynergy, delayed reaction time, dyschronometria, dysarthria, dysphagia, hypotonia, dysmetria, hypometria, hypermetria, dysdiadochokinesia, speech slurring, voice tremor, ataxic respiration, postural instability, and combinations thereof, for example, wherein the primary ataxic deficit is a gait instability.

13. The compound for use of claim 12, wherein the subject has a baseline ataxia of at least 0.5 on the Scale for Assessment and Rating of Ataxia (SARA) scale at the initiation of therapy according to the method, e.g., a baseline SARA score of at least 1, or at least 2, or at least 3, or at least 4, or at least 5, or at least 10 or at least 20.

14. The compound for use of any one of claims 7 to 13, wherein the cognitive dysfunction is a dementia, e.g., Alzheimer's disease or Parkinson's disease, optionally wherein the dementia shows signs of defects in visual search speed, scanning speed of processing, mental flexibility and/or executive functioning.

15. The compound for use of any one of claims 7 to 14, wherein the method is effective to provide a reduction on the SARA ataxia scale of at least 0.5, e.g., a SARA score reduction of at least 1, or at least 2, or at least 3, or at least 5, or at least 10; or wherein the method is effective to reduce the SARA score to between 0.00 and 3.00, or between 0.00 and 2.00, or between 0.00 and 1.50, or between 0.00 and 1.00, or between 0.00 and 0.50.

16. The compound for use of any one of claims 7 to 15, wherein the method is effective to improve cognitive ability or reduce cognitive deficits, e.g., as measured by a reduction in the time taken to complete the trail-making test (TMT), TMT-A and/or TMT-B, a reduction in the difference between TMT-A time and TMT-B time (TMT-A – TMT-B), for example, a reduction of at least 10%, or at least 20%, or at least 30%, or at least 40%, or at least 50% (e.g., wherein TMT-A decreases by 5-20%, and/or TMT-B decreases by 25-30%, and/or [TMT-A – TMT-B] decreases by 25-30%).

17. The compound for use of any one of claims 1 to 16, wherein the subject has Gaucher disease Type 3, Niemann-Pick disease Type C, or a GM2-gangliosidosis (e.g., Tay-Sachs disease, Sandhoff disease, or GM2 gangliosidosis AB variant).

18. The compound for use of any one of claims 1 to 16, wherein the subject is diagnosed with a mutation in a gene selected from: the gene *GBA1*; the genes *NPC1* and/or *NPC2*; the gene *HEXA* (encoding hexosaminidase A) and/or the gene *HEXB* (encoding hexosaminidase B) and/or the gene *GM2A* (encoding the GM2 ganglioside activator protein).

19. The compound for use of any one of claims 1 to 18, wherein said compound, or pharmaceutically acceptable salt or prodrug thereof, is administered by systemic administration, *e.g.* via a non-parenteral route, optionally wherein said compound, or pharmaceutically acceptable salt or prodrug thereof, is administered orally; and/or wherein the subject is administered a daily dose of about 1 mg to about 50 mg of the compound, e.g., from 5 to 50 mg, or from 10 to 40 mg, or from 10 to 30 mg, or from 10 to 20 mg, or from 20 to 30 mg, or from 30 to 40 mg, or from 40 to 50 mg, or from 5 to 25 mg, or from 20 to 50 mg, or from 5 to 15 mg, or from 15 to 30 mg, or about 15 mg.

20. The compound for use of any one of claims 1 to 19, wherein the compound is in a pharmaceutically acceptable acid addition salt form selected from the hydrochloride, hydrobromide, hydroiodide, nitrate, sulfate, bisulfate, phosphate, acid phosphate, acetate, lactate, citrate, acid citrate, tartrate, bitartrate, succinate, hydroxysuccinate, malate, maleate, fumarate, gluconate, saccharate, benzoate, methanesulfonate, and pamoate.

21. The compound for use of any one of claims 1 to 20, wherein the compound is (S)-quinuclidin-3-yl (2-(2-(4-fluorophenyl)thiazol-4-yl)propan-2-yl)carbamate in malate salt form.

22. The compound for use of any one of claims 1 to 21, wherein the subject is diagnosed with Parkinson's disease.

23. The compound for use of any one of claim 1-22, wherein the subject undergoes concurrent treatment with enzyme replacement therapy (ERT), e.g., using a glucocerebrosidase (e.g., imiglucerase, velaglucerase, or taliglucerase).