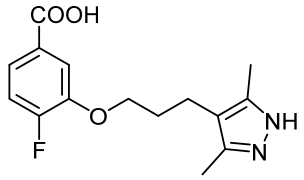
CLAIMS:

1. Use of compound 1, having the formula:



or a pharmaceutically acceptable salt thereof, in the preparation of a medicament for the treatment of transthyretin (TTR) amyloidosis in a subject in need thereof, wherein the treatment comprises a therapeutically effective amount of Compound 1 or the pharmaceutically acceptable salt thereof to be administered to a subject, wherein said therapeutically effective amount is a total daily dosage of about 10 mg to 2,000 mg.

2. The use of claim **1**, wherein said therapeutically effective amount is a total daily dosage of about:

(a) 10 mg to 50 mg;

(b) 50 mg to 300 mg;

(c) 50 mg to 150 mg;

(d) 150 mg to 800 mg;

(e) 800 mg to 1600 mg;

(f) 800 mg; or

(g) 1600 mg.

3. The use of claim **1** or **2**, wherein Compound 1 is in HCl salt form.

4. The use of any one of claims **1** to **3**, wherein said Compound 1, or the pharmaceutically acceptable salt thereof, is to be administered orally.

5. The use of any one of claims **1** to **4**, wherein Compound 1, or the pharmaceutically acceptable salt thereof, is to be administered:

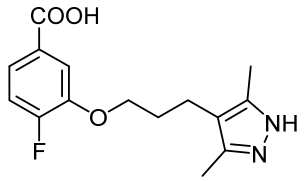
(a) once daily;

(b) twice daily;

(c) three times daily; or

(d) four times daily.

6. Use of compound 1, having the formula:



or a pharmaceutically acceptable salt thereof, in the preparation of a medicament for the treatment of transthyretin (TTR) amyloidosis in a subject in need thereof, wherein the treatment comprises a therapeutically effective amount of Compound 1, or the pharmaceutically acceptable salt thereof to be administered to a subject in need thereof, wherein the therapeutically effective amount maintains a trough blood plasma concentration of Compound 1 of at least 5 μM.

7. The use of claim **6**, wherein the therapeutically effective amount maintains a trough blood plasma concentration of Compound 1 of:

(a) at least 6 μM;

(b) at least 7.5 μM;

(c) at least 8 μM;

(d) from 5 to 30 μM;

(e) from 5 to 25 μM;

(f) from 6 to 20 μM;

(g) from 7.5 to 15 μM; or

(h) from 7.5 to 10 μM.

8. The use of any one of claims **1** to **7**, wherein said TTR amyloidosis is a disease or condition selected from the group consisting of familial amyloid polyneuropathy,

familial amyloid cardiomyopathy, senile systemic amyloidosis, central amyloidosis, ocular amyloidosis, Leptomeningeal amyloidosis, oculoleptomeningeal amyloidosis, vitreous amyloidosis, gastrointestinal amyloidosis, neuropathic amyloidosis, non-neuropathic amyloidosis, non-hereditary amyloidosis, reactive/secondary amyloidosis, cerebral amyloidosis.

9. The use of claim **8**, wherein said TTR amyloidosis is Leptomeningeal amyloidosis, optionally wherein:

(a) said Leptomeningeal amyloidosis is characterized by a TTR protein that comprises an aspartic acid to glycine mutation at position 18 (D18G);

(b) said Leptomeningeal amyloidosis is characterized by a TTR protein that comprises a glycine to arginine mutation at position 53 (G53R);

(c) said Leptomeningeal amyloidosis is characterized by a TTR protein that comprises a tyrosine to cysteine mutation at position 114 (Y114C); or

(d) said Leptomeningeal amyloidosis is characterized by a TTR protein that comprises a threonine to proline mutation at position 49 (T49P).

10. The use of any one of claims **1** to **7**, wherein said TTR amyloidosis is transthyretin amyloidosis (ATTR) cardiomyopathy or transthyretin amyloidosis (ATTR) polyneuropathy, optionally wherein:

(a) said TTR amyloidosis is characterized by a TTR protein that comprises a threonine to alanine mutation at position 60 (T60A);

(b) said TTR amyloidosis is characterized by a TTR protein that comprises a proline to serine mutation at position 24 (P24S);

(c) said TTR amyloidosis is characterized by a TTR protein that comprises an aspartic acid to alanine mutation at position 38 (D38A); or

(d) said TTR amyloidosis is characterized by a TTR protein that comprises a leucine to histidine mutation at position 58 (L58H).

11. The use of any one of claims **1** to **7**, wherein said TTR amyloidosis is transthyretin amyloidosis (ATTR) cardiomyopathy, optionally wherein said ATTR cardiomyopathy is wild-type ATTR cardiomyopathy (ATTRwt-CM) or familial ATTR cardiomyopathy (ATTRm-CM).

12. Use of claim **11**, wherein said ATTRm-CM is characterized by a TTR protein that comprises a valine to isoleucine mutation at position 122 (V122I) or a threonine to proline mutation at position 49 (T49P).

13. The use of any one of claims **1** to **7**, wherein said TTR amyloidosis is transthyretin amyloidosis (ATTR) polyneuropathy, optionally wherein said ATTR polyneuropathy is wild-type ATTR polyneuropathy (ATTRwt-PN) or familial ATTR polyneuropathy (ATTRm-PN).

14. The use of claim **13**, wherein said ATTRm-PN is characterized by a TTR protein that comprises a valine to methionine mutation at position 30 (V30M), a phenylalanine to leucine mutation at position 64 (F64L), or a tyrosine to cysteine mutation at position 114 (Y114C).

15. The use of claim **11** or **12**, wherein the therapeutically effective amount of Compound 1, or the pharmaceutically acceptable salt thereof, to be administered to a subject with ATTR cardiomyopathy:

(a) improves, stabilizes or delays worsening in New York Heart Association (NYHA) functional classification of subjects relative to a placebo, optionally wherein the Compound 1 or the pharmaceutically acceptable salt thereof to be administered reduces the New York Heart Association (NYHA) functional classification of the subject;

(b) improves, stabilizes or delays worsening in the Kansas City Cardiomyopathy Questionnaire (KCCQ) classification of subjects relative to a placebo, optionally wherein subjects have an average improvement of at least one level in the Kansas City Cardiomyopathy Questionnaire (KCCQ) classification of subjects;

(c) improves, stabilizes or delays worsening in the EuroQoL-5 Dimensions (EQ-5D-5L) classification of subjects relative to a placebo, optionally wherein subjects have an average improvement of at least five points in the EuroQoL-5 Dimensions (EQ-5D-5L) utility score;

(d) improves the subject's performance in a six minute walk test relative to a placebo, optionally wherein the subject walks at least 25 m further than a baseline distance measured prior to treatment with Compound 1, or the pharmaceutically acceptable salt thereof;

(e) decreases serum blood levels of brain natriuretic peptide (BNP) relative to a placebo, optionally wherein serum blood levels of BNP of said subject decreases at least 10% as compared to a baseline level of BNP in said subject prior to treatment with Compound 1, or the pharmaceutically acceptable salt thereof;

(f) decreases serum blood levels of N-terminal pro-brain natriuretic peptide (N-terminal pro-BNP) relative to a placebo, optionally wherein serum blood levels of N-terminal pro-BNP of said subject decreases at least 10% as compared to a baseline level of N-terminal pro-BNP in said subject prior to treatment with Compound 1, or the pharmaceutically acceptable salt thereof;

(g) decreases the frequency of cardiovascular-related hospitalizations as compared to subjects not receiving treatment;

(h) decreases mortality as compared to subjects not receiving treatment.

16. The use of claim **13** or **14**, wherein the therapeutically effective amount of Compound 1, or the pharmaceutically acceptable salt thereof, to be administered to a subject with ATTR polyneuropathy:

(a) improves the Neuropathy Impairment Score (NIS) in said subject relative to a placebo, optionally wherein the NIS score is reduced by at least 10 % as compared to a baseline level measured prior to treatment with Compound 1, or the pharmaceutically acceptable salt thereof;

(b) improves the modified Neuropathy Impairment Score+7 (mNIS+7) in said subject relative to a placebo, optionally wherein the mNIS+7 score is reduced by at least 10 % as compared to a baseline level measured prior to treatment with Compound 1, or the pharmaceutically acceptable salt thereof;

(c) improves the subject's Norfolk Quality of Life Diabetic Neuropathy (QOL-DN) questionnaire score relative to a placebo, optionally wherein the

Norfolk QOL-DN score is improved by at least 10 % as compared to a baseline level measured prior to treatment with Compound 1, or the pharmacuetically acceptable salt thereof;

(d) improves the subject's composite autonomic symptom score (COMPASS-31) score relative to a placebo, optionally wherein the COMPASS-31 score is improved by at least 0.5 points as compared to a baseline level measured prior to treatment with Compound 1, or the pharmaceutically acceptable salt thereof;

(e) improves the modified BMI (mBMI) of the subject relative to a placebo; or

(f) improves the 10-meter walk test speed of the subject relative to a placebo.

17. The use of claims **1** to **16**, wherein:

(a) Compound 1, or the pharmaceutically acceptable salt thereof, is to be administered chronically;

(b) Compound 1, or the pharmaceutically acceptable salt thereof, is to be administered for 28 days;

(c) Compound 1, or the pharmaceutically acceptable salt thereof, is to be administered for 56 days;

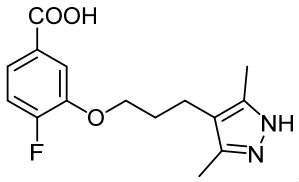
(d) Compound 1, or the pharmaceutically acceptable salt thereof, is to be administered for 84 days;

(e) Compound 1, or the pharmaceutically acceptable salt thereof, is to be administered for 18 months; or

(f) Compound 1, or the pharmaceutically acceptable salt thereof, is to be administered for 30 months.

18. The use of claims **1** to **17**, wherein the subject is to be receiveing a diuretic therapy agent, optionally wherein the diuretic therapy agent is furosemide or torsemide.

19. A single unit dosage comprising about 10-1,000 mg of Compound 1, having the formula:



, or a pharmaceutically acceptable salt thereof.

20. The single unit dosage form of claim **19**, wherein:

(a) the single unit dosage form comprises (i) about 200 mg of Compound 1 or (ii) about 400 mg of Compound 1;

(b) the single unit dosage form (i) is a tablet or (ii) is a capsule; and/or

(c) Compound 1 is in HCl salt form.

21. A kit comprising one or more unit dosages of claim **19** or **20**.

22. The kit of claim **21**, further comprising a label with instructions for administering Compound 1.