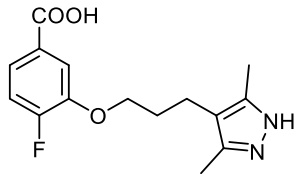
CLAIMS:

1. Use of Compound 1, having the formula:



(Compound 1)

in HCl salt form, in the preparation of a medicament for the treatment of transthyretin (TTR) amyloidosis in a subject in need thereof, wherein the medicament is to be administered at a total daily dosage of about 1,600 mg of the HCl salt form of Compound 1, wherein the medicament is to be administered twice daily.

2. The use of claim **1**, wherein said medicament is to be administered orally.

3. The use of claim **1** or **2**, 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, and cerebral amyloidosis.

4. The use of any one of claims **1** to **3**, wherein said TTR amyloidosis is transthyretin amyloidosis (ATTR) cardiomyopathy.

5. The use of claim **4**, wherein said ATTR cardiomyopathy is wild-type ATTR cardiomyopathy (ATTRwt-CM) or familial ATTR cardiomyopathy (ATTRm-CM).

6. Use of claim **5**, 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).

7. The use of claim **1** or **2**, wherein said TTR amyloidosis is transthyretin amyloidosis (ATTR) polyneuropathy.

8. The use of claim **7**, wherein said ATTR polyneuropathy is wild-type ATTR polyneuropathy (ATTRwt-PN) or familial ATTR polyneuropathy (ATTRm-PN).

9. The use of claim **8**, 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).

10. The use of any one of claims **4** to **6**, wherein the medicament 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 medicament 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 the medicament;

(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 the medicament;

(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 the medicament;

(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.

11. The use of any one of claims **7** to **9**, wherein the medicament 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 the medicament;

(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 the medicament;

(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 the medicament;

(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 the medicament;

(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.

12. The use of any one of claims **1** to **11**, wherein:

(a) the medicament is to be administered chronically;

(b) the medicament is to be administered for 28 days;

(c) the medicament is to be administered for 56 days;

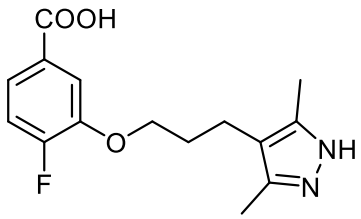
(d) the medicament is to be administered for 84 days;

(e) the medicament is to be administered for 18 months; or

(f) the medicament is to be administered for 30 months.

13. The use of any one of claims **1** to **12**, wherein the subject is to be receiving a diuretic therapy agent, optionally wherein the diuretic therapy agent is furosemide or torsemide.

14. Use of Compound 1, having the formula:



(Compound 1)

in HCl salt form, in the preparation of a medicament for the treatment of transthyretin amyloidosis (ATTR) cardiomyopathy in a subject in need thereof, wherein the medicament is to be administered at a total daily dosage of about 1,600 mg of the HCl salt form of Compound 1, wherein the medicament is to be administered twice daily; and

wherein the administration of the medicament:

(1) reduces the decline in a six-minute walk test compared to subjects not receiving treatment; or

(2) improves the six-minute walk test performance of the subject compared to a baseline distance measured prior to treatment with the medicament.

15. The method of claim **14**, wherein administration of the medicament reduces the decline in a six-minute walk test compared to subjects not receiving treatment.

16. The method of claim **14**, wherein administration of the medicament improves the six-minute walk test performance of the subject compared to a baseline distance measured prior to treatment with the medicament.

17. The method of claim **14**, wherein the subject maintains about the same six minute walking distance as prior to treatment.

18. The method of claim **14**, wherein the mean change in six minute walk test from baseline between the subject receiving the medicament and subjects not receiving treatment is at least 10 meters.

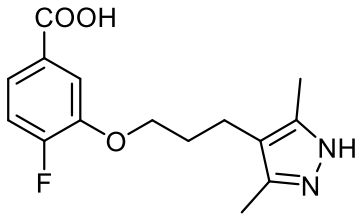
19. The method of claim **14**, wherein the mean change in six minute walk test from baseline between the subject receiving the medicament and subjects not receiving treatment is at least 20 meters.

20. The method of claim **14**, wherein the mean change in six minute walk test from baseline between the subject receiving the medicament and subjects not receiving treatment is at least 30 meters.

21. The method of claim **14**, wherein the subject walked at least 25 meters further than a baseline distance measured prior to treatment with the medicament.

22. The method of claim **14**, wherein the subject walked at least 30 meters further than a baseline distance measured prior to treatment with the medicament.

23. Use of Compound 1, having the formula:



(Compound 1)

in HCl salt form, in the preparation of a medicament for slowing the progression of transthyretin amyloidosis (ATTR) cardiomyopathy in a subject in need thereof, wherein the medicament is to be administered at a total daily dosage of about 1,600 mg of the HCl salt form of Compound 1, wherein the medicament is to be administered twice

daily, and wherein the administration of the medicament reduces the decline in a six-minute walk test performance of the subject compared to subjects not receiving treatment.