WAINZUA® ▼ (eplontersen) is indicated for the treatment of hereditary transthyretin-mediated amyloidosis (ATTRv amyloidosis) in adult patients with Stage 1 and 2 polyneuropathy.[1]

Prescribing Information and adverse event reporting statement can be found on the last page.

WAINZUA®
eplontersen 45 mg
injection for subcutaneous use

This is a promotional material developed by AstraZeneca UK and intended for healthcare professionals in the UK.

Exploring the true burden of ATTRv amyloidosis

ATTRv amyloidosis is a debilitating, progressive, and potentially life-threatening disease[2–4]

Some patients may have to make **dramatic lifestyle changes** (e.g. ability to work or personal wellbeing) owing to **multisystemic symptoms and organ dysfunction**[5]

Early and effective intervention is needed to address the significant impairment of QoL among patients with ATTRv amyloidosis and their caregivers[5–8]

Impact on patients:

LOSS OF INDEPENDENCE

64% (9/14) reported **difficulty dressing, bathing, and using the bathroom***[9]

LOSS OF PHYSICAL FUNCTION

79% (11/14) reported **difficulty gripping or holding objects, walking, or standing***[9]

LOSS OF AUTONOMIC GI FUNCTION

63% (696/1114) reported **GI symptoms**, which had a **negative impact on nutritional status†**[10]

LOSS OF LIFE

Median survival is **5–15 years from diagnosis** of ATTRv-PN[11]

"I had pain in my legs and back... I could not walk with the pain"

Patient with ATTRv amyloidosis[5]

"I couldn't use a towel after showering because it felt like sandpaper"

Patient with ATTRv amyloidosis[5]

Impact on caregivers such as family and friends:

DAILY LIFE

~6h on practical care and ~4h on emotional support‡§[12]

EMPLOYMENT

Over half (56%; 20/36) of caregivers changed their employment status‡¶[12]

MENTAL HEALTH

Significantly higher HADS anxiety and depression scores vs. matched controls‡**[12]

To improve treatment experience and adherence, it is crucial to consider each patient's priorities and preferences, based on their individual challenges and QoL expectations[13–16]

*Based on a qualitative, non-interventional study in the US and Canada, investigating the symptoms and impact of ATTRv amyloidosis on patients' function and well-being (N=14);[9]

†Based on the Transthyretin Amyloidosis Outcomes Survey (THAOS), a global, multicentre, longitudinal, observational survey that analysed data from 1579 patients with ATTRv amyloidosis and 160 patients with ATTRwt amyloidosis from 17 countries (largest contributors: Portugal, US, Italy, France, Germany, Brazil, Sweden, and Japan); Proportion of patients with GI symptoms (63%) pertains to patients with a *TTR* mutation;[10]

‡UK cross-sectional online survey assessing the caregiver burden of ATTRv amyloidosis in 36 caregivers and matched controls (N=72) using measures of HRQoL (EQ-5D-3L), anxiety and depression (HADS), and caregiver and patient characteristics questions. A companion general population survey was designed to collect data to compare with the caregiver data where relevant;[12]

§Practical care: median=3.0, SD = 6.2; emotional support: median=2.0, SD=6.0[12]

¶Stopped working, reduced hours, changed jobs for flexibility, or other reasons;[12]

**HADS anxiety score: median=9.3 vs. median=6.1, t=-3.25, $p<0.01$;[12] HADS depression score: median=7.6 vs. median=4.4, t=-3.39, $p<0.01$.[12]

Abbreviations and references can be found on the last page.

AstraZeneca

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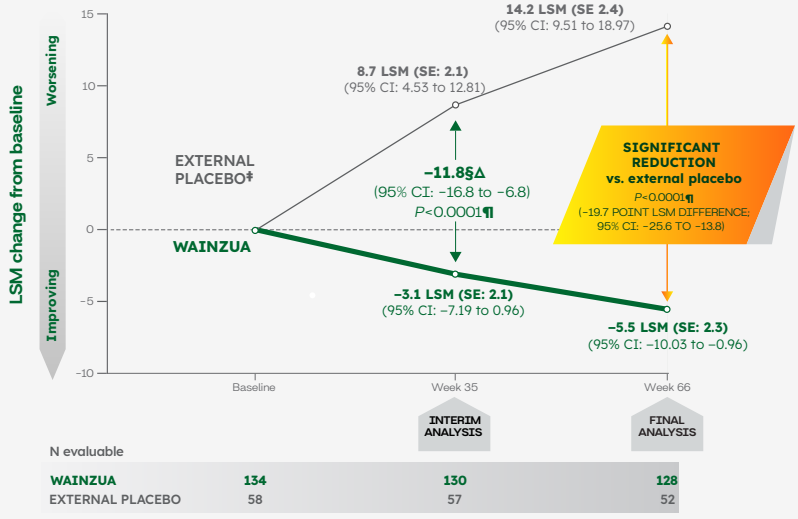
WAINZUA®
eplontersen 45 mg
injection for subcutaneous use

WAINZUA is the first and only *TTR* gene silencer in a once-monthly pre-filled pen which delivers significant efficacy[1,17]

WAINZUA significantly improves quality of life vs. external placebo*[1,17]

Change in Norfolk QoL-DN total score from baseline vs. external placebo through Week 66*[†1,17]



N evaluable			
WAINZUA	134	130	128
EXTERNAL PLACEBO	58	57	52

- Reduces neuropathy impairment vs. external placebo up to Week 66*[1,17]
- Consistently suppresses TTR vs. external placebo up to Week 65*[1,17]
- Helps prevent nutritional decline** (secondary endpoint) vs. external placebo up to Week 65*[17]
- Generally well-tolerated[1,17]

The most frequent adverse reactions during treatment with WAINZUA observed in ≥5% of patients were vitamin A decreased, injection site reactions, vomiting, and proteinuria[1]

Vitamin A deficiency is noted as a special warning in the WAINZUA SmPC. Serum vitamin A levels below the lower limit of normal should be corrected and any symptoms or signs related to vitamin A deficiency should be evaluated prior to initiation of treatment with WAINZUA. **Please refer to the SmPC for further details prior to prescribing.**

Offer patients flexibility in administration and prescribe once-monthly WAINZUA for your eligible patients[1]

EMPOWERING
Home delivery available to suit patients' lifestyles[††]

CONVENIENT
Once-monthly dosing via a subcutaneous injection[1]

FLEXIBLE AND PORTABLE
Patients can either self-administer or have their caregiver administer in their preferred environment,[1] or have a nurse administer at home via the Homecare Service (available only to eligible patients at healthcare organisations that have formally signed up to it).
[††] Store WAINZUA in a refrigerator at 2°C to 8°C. If needed, it can remain in its original carton below 30°C for up to 6 weeks[‡‡1]

Provide holistic support to your patients beyond treatment with the Acapella Patient Support Programme, developed and funded by AstraZeneca UK

To find out more about WAINZUA, click or type the following website address into your web browser
https://www.myastrazeneca.co.uk/wainzua.html

*NEURO-TTRansform was an open-label, single-group, Phase III trial that enrolled 168 patients with Coutinho Stage 1 or 2 ATTRv-PN (144 assigned to eplontersen and 24 treated with inotersen as a reference group) and included 60 patients categorised as external placebo controls from NEURO-TTR, an inotersen trial. At Week 65, the LSM reduction in serum TTR protein levels from baseline was −81.7% with WAINZUA vs. −11.2% with external placebo (difference, −70.4% [95% CI: −75.2% to −65.7%]; *P*<0.0001). At Week 66, the mNIS+7 score showed that the neuropathy impairment from baseline vs. external placebo had significantly reduced (0.3 point LSM increase vs. 25.1 point increase; difference, −24.8 [95% CI: −31.0 to −18.6]; *P*<0.0001) and there was significantly improved quality of life from baseline vs. external placebo as measured by Norfolk QoL-DN (−5.5 point LSM decrease vs. 14.2 point increase; difference, −19.7 [95% CI: −25.6 to −13.8]; *P*<0.0001);[1,17] †The Norfolk QoL-DN score is a patient-reported quality-of-life assessment, measuring physical function/large-fibre neuropathy symptoms, activities of daily living symptoms, small-fibre neuropathy, and autonomic neuropathy. The Norfolk QoL-DN total score has a range of −4 to 136, and a higher score indicates poorer QoL;[1] ‡External placebo group from another randomised controlled trial (NEURO-TTR);[17]

§Treatment difference presents results from formal Week 35 interim analysis based on MI ANCOVA adjusted by propensity score weights with fixed categorical effects for treatment, disease stage, Val30Met mutation, previous treatment, and fixed covariates for the baseline. Only data up to Week 35 are included in the Week 35 interim analysis. The Week 66 LSM treatment difference (WAINZUA vs. external placebo) is based on MMRM adjusted by propensity score weights with fixed categorical effects for treatment, time, treatment-by-time interaction, disease stage, Val30Met mutation, previous treatment, and fixed covariates for the baseline and the baseline-by-time interaction. Analysis based on data collected up to 52 days after last dose of study treatment;[1] ¶P-value at Week 66 not formally tested due to statistically significant results at Week 35;[1]

**mBMI is a validated measure that accounts for volume overload by including the serum albumin levels, and is defined as body mass index (BMI) × albumin, with higher scores indicating better nutritional status;[18]

††The first injection administered by the patient or caregiver should be performed under the guidance of an appropriately qualified HCP. Patients and/or caregivers should be trained in the subcutaneous administration of WAINZUA.[1] The WAINZUA Homecare Service, provided by HealthNet Homecare, is a patient support service developed and funded by AstraZeneca UK that allows patients to arrange the delivery of their medication for when it suits them. The service is available only to eligible patients at healthcare organisations that have formally signed up to it;

‡‡If not used within 6 weeks, it should be discarded. Store in the original package to protect from light. Do not freeze. Do not expose to heat.[1]

Abbreviations and references can be found on the last page.

Abbreviations and references

ANCOVA=analysis of covariance; **ATTRv-(PN)**=hereditary transthyretin amyloidosis (with polyneuropathy); **ATTRwt**=wild-type transthyretin amyloidosis; **CI**=confidence interval; **EQ-5D**=European Quality of Life 5 Dimensions 3 Level; **GI**=gastrointestinal; **HADS**=hospital anxiety and depression scale; **HCP**=healthcare professional; **(HR)QoL**=(health-related) quality of life; **LSM**=least square means; **mBMI**=modified body mass index; **mNIS**=modified neuropathy impairment score; **MMRM**=mixed model for repeated measures; **QoL**=quality of life; **SD**=standard deviation; **SE**=standard error; **SmPC**=Summary of Product Characteristics; **t**=t-test; **TTR**=transthyretin.

1. WAINZUA® (eplontersen) Summary of Product Characteristics; **2.** Ando Y, et al. *Orphanet J Rare Dis.* 2013;8(1):31; **3.** Nativi-Nicolau JN, et al. *Heart Fail Rev.* 2022;27(3):785–793; **4.** Adams D, et al. *J Neurol.* 2021;268(6):2109–2122; **5.** Rintell D, et al. *Orphanet J Rare Dis.* 2021;16(1):70; **6.** Bolte FJ, et al. *Orphanet J Rare Dis.* 2020;15(1):287; **7.** Lane M and Polydefkis M. *Neurol Ther.* 2024;13(6):1527–1533; **8.** Inês M, et al. *Orphanet J Rare Dis.* 2020;15(1):67; **9.** Lovley A, et al. *J Patient Rep Outcomes.* 2021;5(1):3; **10.** Wixner J, et al. *Orphanet J Rare Dis.* 2014;9:61; **11.** Hawkins PN, et al. *Ann Med.* 2015;47(8):625–638; **12.** Acaster S and Nestler-Parr S. *Orphanet J Rare Dis.* 2023;18(1):17; **13.** Obici L, et al. *BMJ Open.* 2023;13(9):e073130; **14.** NHS. Personalised Care. Available at: https://www.england.nhs.uk/personalisedcare/health-literacy/. Accessed March 2026; **15.** Ward J, et al. *Res Social Adm Pharm.* 2020;16(1):108–110; **16.** National Institute for Health and Care Excellence (NICE). Shared decision making. Guideline NG197. Available at: https://www.nice.org.uk/guidance/ng197/chapter/recommendations. Accessed March 2026; **17.** Coelho T, et al. *JAMA.* 2023;330(15):1448–1458 (including Supplementary Materials); **18.** Dongiglio F, et al. *Cardiogenetics.* 2022;12(2):185–197.

Prescribing Information

WAINZUA®▼ (eplontersen) 45 MG SOLUTION FOR INJECTION IN PRE-FILLED PEN

Consult Summary of Product Characteristics (SmPC) before prescribing.

Indication: For the treatment of hereditary transthyretin-mediated amyloidosis (ATTRv amyloidosis) in adult patients with Stage 1 and 2 polyneuropathy.

Presentation: Pre-filled pen containing 45 mg eplontersen in 0.8 mL.

Dosage and Administration: Treatment should be prescribed and supervised by physicians experienced in the diagnosis and treatment of amyloidosis. The recommended dose is 45 mg of eplontersen by subcutaneous injection administered monthly. If a dose is missed, the dose should be administered as soon as possible. Thereafter, the patient can resume dosing at monthly intervals from date of last dose. A patient or their caregiver may administer Wainzua after being trained in subcutaneous injection technique. The pre-filled pen should be removed from refrigerated storage at least 30 minutes before use and allowed to reach room temperature prior to injection. Self-administer as a subcutaneous injection into the upper thigh or abdomen. If a healthcare professional or caregiver administers the injection, the upper arm can also be used. A patient should not self-inject in the arm. It should not be injected into skin which is tender, bruised, erythematous, or hard, into scars or damaged skin or area around the navel area. **Elderly population:** No dosing adjustment is required for patients ≥65 years of age. **Renal impairment:** No dose adjustment is necessary in patients with mild to moderate renal impairment (estimated glomerular filtration rate [eGFR] ≥45 to <90 ml/min/1.73 m^2). Eplontersen has not been studied in patients with eGFR <45 ml/min/1.73 m^2 or end-stage renal disease and should only be used in these patients if clinical benefit outweighs the potential risk. **Hepatic impairment:** No dosing adjustment is necessary in patients with mild hepatic impairment. Wainzua has not been studied in patients with moderate to severe hepatic impairment, the clinical benefit must outweigh the potential risk if used in these patients. **Patients undergoing liver transplant:** The safety and efficacy of Wainzua in patients undergoing liver transplant has not been established. **Paediatric population**: The safety and efficacy of Wainzua in children and adolescents under 18 years of age has not been established.

Contraindications: Hypersensitivity to the active substance or excipients.

Warnings and Precautions: **Vitamin A deficiency:** Wainzua is expected to reduce serum vitamin A, and any symptoms or signs related to vitamin A deficiency should be evaluated prior to initiation of treatment. Patients receiving Wainzua should take oral supplementation of approximately, but not exceeding, 2500 IU (female) to 3000 IU (male) of vitamin A per day to reduce the potential risk of ocular symptoms due to vitamin A deficiency. Vitamin A levels below the lower limit of normal should be corrected. Before initiating treatment with Wainzua, exclude pregnancy and advise patients on effective contraception. Discontinue Wainzua and vitamin A supplementation if a women intends to become pregnant, or in the event of an unplanned pregnancy. Due to the long half-life of eplontersen, a vitamin A deficiency may develop after cessation of treatment. No recommendation can be given whether to continue or discontinue vitamin A supplementation during the first trimester of an unplanned pregnancy. **Sodium Content:** Wainzua is considered essentially 'sodium-free'.

Drug Interactions: Wainzua is not expected to cause or be affected by drug-drug interactions.

Pregnancy and Lactation: Wainzua should not be used during pregnancy. Wainzua should not be initiated in pregnant women or women intending to become pregnant. There is no data for the use of Wainzua in pregnant women. Wainzua will reduce the plasma levels of vitamin A, which is crucial for normal foetal development. It is not known whether vitamin A supplementation will be sufficient to reduce the risk to the foetus. **Lactation:** A risk to the breastfed child cannot be excluded. A decision must be made whether to discontinue breastfeeding or to discontinue/abstain from Wainzua therapy, taking into account the benefit of breastfeeding for the child and the benefit of therapy for the woman.

Ability to Drive and Use Machines: Wainzua has no or negligible influence on the ability to drive and use machines.

Undesirable Events: Consult SmPC for full list of side effects. **Very Common (≥1/10):** Vitamin A decreased. **Common (≥1/100 to <1/10):** Vomiting, proteinuria, cataracts, injection site reactions (erythema, pain, pruritus).

Legal Category: POM.

Marketing Authorisation Number: Wainzua 45 mg solution for injection in pre-filled pen PLGB 17901/0377.

Presentation & Basic NHS Cost: Wainzua 45 mg solution for injection in pre-filled syringe 1 x single use pre-filled pen: £31,954.12

Business Responsible for Sale and Supply/Further Information: AstraZeneca UK Limited, 2 Pancras Square, 8th Floor, London, N1C 4AG, UK.

WAINZUA is a registered trademark of the AstraZeneca group of companies.

Date of preparation 01/2025.

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Adverse events should be reported. Reporting forms and information can be found at https://yellowcard.mhra.gov.uk/ or search for MHRA Yellow Card in the Google Play or Apple App Store. Adverse events should also be reported to AstraZeneca by visiting *https://contactazmedical.astrazeneca.com* or by calling 0800 783 0033.