Wainua™ (eplontersen) – New orphan drug approval

- On December 21, 2023, AstraZeneca and Ionis announced the FDA approval of Wainua (eplontersen), for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTR-PN) in adults.

- hATTR-PN is a progressive systemic disease caused by genetic mutations, resulting in misfolded TTR protein and accumulation as amyloid fibrils in the peripheral nerves. As the TTR protein fibrils accumulate, more tissue damage occurs and the disease worsens. Worldwide, there are about 40,000 patients with hATTR-PN.

- Wainua is a ligand-conjugated antisense oligonucleotide designed to reduce the production of TTR protein.

- The efficacy of Wainua was established in a randomized, open-label study in adult patients with hATTR-PN. Patients were randomized to receive either Wainua once every 4 weeks or Tegsedi® (inotersen) once per week. Tegsedi is Ionis' other antisense oligonucleotide for hATTR-PN. Efficacy assessments were based on a comparison of the Wainua arm with an external placebo group in another study composed of a comparable population of adult patients with hATTR-PN. The efficacy endpoints were the change from baseline to week 35 in the modified Neuropathy Impairment Scale+7 (mNIS+7) composite score and the change from baseline to week 35 in the Norfolk Quality of Life-Diabetic Neuropathy (QoL-DN) total score.

    - The least-squares (LS) mean change in the mNIS+7 score was 0.2 with Wainua vs. 9.2 with placebo (treatment difference of -9.0, 95% CI: -13.5, -4.5; $p < 0.001$).
    - The LS mean change in the Norfolk QoL-DN score was -3.1 with Wainua vs. 8.7 with placebo (treatment difference of -11.8, 95% CI: -16.8, -6.8; $p < 0.001$).

- A warning and precaution for Wainua is reduced serum vitamin A levels and recommended supplementation.

- The most common adverse reactions (≥ 9%) with Wainua use were vitamin A decreased and vomiting.

- The recommended dose of Wainua is 45 mg administered by subcutaneous injection once monthly.

    - Prior to initiation, patients and/or caregivers should be trained on proper preparation and administration of Wainua.

- AstraZeneca and Ionis plans to launch Wainua in January 2024. Wainua will be available as a 45 mg/0.8 mL single-dose autoinjector.

At Optum, we help create a healthier world, one insight, one connection, one person at a time. All Optum trademarks and logos are owned by Optum, Inc., in the U.S. and other jurisdictions. All other trademarks are the property of their respective owners. This document contains information that is considered proprietary to Optum Rx and should not be reproduced without the express written consent of Optum Rx. RxNews® is published by the Optum Rx Clinical Services Department.

© 2023 Optum, Inc. All rights reserved. ORX6547968A-TEMPLATE_220208