Amvuttra® (vutrisiran) – New indication

- On March 20, 2025, Alnylam announced the FDA approval of Amvuttra (vutrisiran), for the **treatment of the cardiomyopathy of wild-type or hereditary transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular mortality, cardiovascular hospitalizations and urgent heart failure visits**.

- Amvuttra is also approved for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTRPN) in adults.

- The approval of Amvuttra for the new indication was based on a randomized, double-blind, placebo-controlled study (HELIOS-B) in 654 adult patients with wild-type or hereditary ATTR-CM. Patients were randomized to receive Amvuttra or placebo. The primary endpoint was the composite outcome of all-cause mortality and recurrent cardiovascular (CV) events (CV hospitalizations and urgent heart failure visits) during the double-blind treatment period of up to 36 months, evaluated in the overall population and in the monotherapy population (defined as patients not receiving tafamidis at study baseline).

    — Amvuttra led to significant reduction in the risk of all-cause mortality and recurrent CV events compared to placebo in the overall and monotherapy population of 28% (p = 0.01) and 33% (p = 0.02), respectively.
    — Both components of the primary composite endpoint individually contributed to the treatment effect in the overall and monotherapy population.

- The recommended dose of Amvuttra is 25 mg administered by subcutaneous injection once every 3 months.

    — Amvuttra should be administered by a healthcare professional.