

## Vutrisiran: AL Amyloidosis or Leptomeningeal Amyloidosis

The following information is provided in response to your unsolicited inquiry. It is intended to provide you with a review of the available scientific literature and to assist you in forming your own conclusions in order to make healthcare decisions. This document is not for further dissemination or publication without authorization.

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### SUMMARY

- The use of vutrisiran in patients with primary amyloidosis (AL amyloidosis) or leptomeningeal amyloidosis has not been evaluated in clinical studies.
  - In the phase 3 HELIOS-A and HELIOS-B studies, patients were excluded if they had known primary amyloidosis (AL amyloidosis) or leptomeningeal amyloidosis.<sup>1-4</sup>

### AMVUTTRA PRESCRIBING INFORMATION – RELEVANT CONTENT

The **INDICATIONS AND USAGE** section provides the following information<sup>5</sup>:

#### ***Polyneuropathy of Hereditary Transthyretin-mediated Amyloidosis***

*AMVUTTRA is indicated for the treatment of the polyneuropathy of hereditary transthyretin-mediated amyloidosis (hATTR-PN) in adults.*

#### ***Cardiomyopathy of Wild-type or Hereditary Transthyretin-mediated Amyloidosis***

*AMVUTTRA is indicated 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.*

### ABBREVIATIONS

AL = amyloid light chain; ATTR-CM = transthyretin amyloidosis with cardiomyopathy; hATTR-PN = hereditary transthyretin amyloidosis with polyneuropathy.

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### REFERENCES

1. Adams D, Tournev IL, Taylor MS, et al. Efficacy and safety of vutrisiran for patients with hereditary transthyretin-mediated amyloidosis with polyneuropathy: a randomized clinical trial. *Amyloid*. 2023;30(1):18-26. doi:10.1080/13506129.2022.2091985
2. Alnylam Pharmaceuticals. Data on file. MED-ALL-TTRSC02-2300015.
3. Fontana M, Berk JL, Gillmore JD, et al. Vutrisiran in patients with transthyretin amyloidosis with cardiomyopathy. *N Engl J Med*. 2025;392(1):33-44. doi:10.1056/NEJMoa2409134

4. Protocol for: Fontana M, Berk JL, Gillmore JD, et al. Vutrisiran in patients with transthyretin amyloidosis with cardiomyopathy. *N Engl J Med*. 2025;392(1):33-44. doi:10.1056/NEJMoa2409134
5. AMVUTTRA (vutrisiran) Prescribing Information. Cambridge, MA: Alnylam Pharmaceuticals, Inc.