ORION

BIOTECH RESEARCH

One label expansion. The evidence behind it.

ALNY / AMVUTTRA / ATTR cardiomyopathy

EVENT 20 MAR 2025 | REVIEWED 09 SEP 2026

A manually curated historical example, prepared with AI assistance and checked against linked primary sources. Evidence cutoff: 20 March 2025. This is not a live alert or an automatically generated account report.

01 / WHAT CHANGED

JUNE 2022 / EARLIER LABEL

Hereditary ATTR polyneuropathy

The original adult indication covered polyneuropathy caused by hereditary ATTR amyloidosis. FDA original label

MARCH 2025 / EXPANDED LABEL

ATTR cardiomyopathy added

Adult wild-type or hereditary ATTR-CM: reducing cardiovascular death, CV hospitalizations and urgent heart-failure visits. FDA label

An indication expansion for an existing product. Alnylam announced the approval on 20 March 2025.

02 / CLINICAL EVIDENCE TO INSPECT

0.72

Hazard ratio
95% CI 0.55-0.93

HELIOS-B / March 2025 FDA label

Randomized, double-blind, placebo-controlled.
654 participants: 326 AMVUTTRA / 328 placebo.
Primary composite: all-cause mortality and recurrent cardiovascular events.
Follow-up: 33-36 months. p = 0.01.

Source-reported modified Andersen-Gill estimates, not ORION calculations. Section 14.2 / Table 4.

03 / KEEP THE OPEN QUESTIONS

A placebo comparison does not rank active competitors. The FDA label reports 40% baseline tafamidis use, a predominantly male population and a vitamin-A reduction warning. Review the full label. Approval alone does not establish uptake, access, revenue or share-price performance.

Keep the source boundaries visible.

Review notes / primary sources / the next research question

04 / TWO DIFFERENCES WORTH KEEPING

Publication versus label. The original trial abstract reports 655 randomized participants and a 0.56 lower confidence limit. The FDA label uses 654 and 0.55. This brief preserves the FDA figures; it does not establish the reason for the difference. Original trial abstract.

Later registry context. The retrieved ClinicalTrials.gov record was updated on 12 January 2026; results were first posted on 21 October 2025. Both are after this event. The record is linked for study identity and later context, not event-day evidence. No event-day ORION capture is claimed. NCT04153149.

NEXT RESEARCH QUESTION

Which later primary disclosures establish uptake and access after the label expansion?

Keep that follow-up separate from this historical record. A question, not a forecast.

05 / OPEN THE ORIGINAL SOURCES

S1 FDA original prescribing information
Revised June 2022 (month precision) / Retrieved 9 Sep 2026

S2 FDA prescribing information, supplement 006
Revised March 2025 (month precision); Table 4, PDF page 13 / Retrieved 9 Sep 2026

S3 Alnylam approval announcement
Published 20 March 2025 / Retrieved 9 Sep 2026

S4 Original HELIOS-B paper: PubMed abstract
Published online 30 August 2024; DOI 10.1056/NEJMoa2409134 / Retrieved 9 Sep 2026

S5 ClinicalTrials.gov: NCT04153149
Updated 12 January 2026; later context only / Retrieved 9 Sep 2026

Exact publication and retrieval times were not established. Source URLs and hashes of preserved files are in the companion metadata. Selected-source review, not a complete clinical, commercial or investment assessment. No investment or treatment recommendation. No endorsement by Alnylam, FDA or the investigators is implied.