HELIOS-B

CLINICAL TRIAL SUMMARY

Presenters	Alnylam Pharmaceuticals
Objectives	To evaluate the efficacy and safety of vutrisiran 25 mg administered subcutaneously (SC) once every 3 months (q3M) compared to placebo in patients with ATTR amyloidosis with cardiomyopathy

Source: https://clinicaltrials.gov/study/NCT04153149

TRIAL DESIGN

Phase 3, Randomized, Double-blind, Placebo-controlled, Multicenter Trial

SAMPLE SIZE

655 patients underwent randomization; 326 were assigned to receive vutrisiran and 329 to receive placebo

INCLUSION CRITERIA

- Has a documented diagnosis of transthyretin (ATTR) amyloidosis with cardiomyopathy, classified as either hereditary ATTR (hATTR) amyloidosis with cardiomyopathy or wild-type ATTR (wtATTR) amyloidosis with cardiomyopathy meeting pre-specified diagnostic criteria
- Has medical history of heart failure (HF) with at least 1 prior hospitalization for HF OR clinical evidence of HF

METHODOLOGY

- Researchers assigned patients with ATTR-CM in a 1:1 ratio to receive vutrisiran (25 mg) or placebo every 12 weeks for up to 36 months.

- The primary end point was a composite of death from any cause and recurrent cardiovascular events.

- Secondary end points included death from any cause, the change from baseline in the distance covered on the 6-minute walk test, and the change from baseline in the Kansas City Cardiomyopathy Questionnaire–Overall Summary (KCCQ-OS) score.

RESULTS

Vutrisiran treatment led to a lower risk of death from any cause and recurrent cardiovascular events than placebo (hazard ratio in the overall population, 0.72; hazard ratio in the monotherapy population, 0.67) and a lower risk of death from any cause through 42 months (hazard ratio in the overall population, 0.65).

Among the patients in the overall population, 125 in the vutrisiran group and 159 in the placebo group had at least one primary end-point event.

In the overall population, treatment with vutrisiran resulted in less decline in the distance covered on the 6-minute walk test than placebo & less of a decline in the KCCQ-OS score. Similar benefits were observed in the monotherapy population.

The incidence of adverse events was similar in the two groups; serious adverse events occurred in 62% of the patients in the vutrisiran group and in 67% of those in the placebo group.

CONCLUSION

Among patients with ATTR-CM, treatment with vutrisiran led to a lower risk of death from any cause and cardiovascular events than placebo and preserved functional capacity and quality of life.