CardioAbstracts: HELIOS-B

CardioAbstracts is a live and on-demand webinar series featuring Q&A with leading clinicians, session recordings, and downloadable resources. On May 6, 2026, we hosted a 45-minute seminar (https://hfsa.org/cardioabstracts-helios-b) moderated by Marianna Fontana, MD, PhD, FRCP; with panelists Farooq Hameed Sheikh, MD, FACC; and Pablo Garcia-Pavia, MD, PhD. The session covered the following studies:

LINK TO WEBINAR

HELIOS-B: 12-Month Results from the Open-Label Extension Period of Vutrisiran in Patients with Transthyretin Amyloidosis with Cardiomyopathy

P. Garcia-Pavia, R. Witteles, C. Morbach, D. Delgado, H. Kitaoka, M. S. Maurer,P. Van der Meer, A. Yilmaz, V. Algalarrondo, S. Solomon, F. Cappelli, K. L. Boyle, S. A. Eraly, L. Yang, J. Vest, M. Fontana

A total of 478 patients completed the Helios B double-blind study. Of those participants, 97 percent chose to continue into a two-year open-label extension study. During this next stage, they received Vutrisiran at a dose of 25 milligrams every three months, allowing researchers to further observe its long-term effects and safety.

The data showed that long-term vutrisiran treatment significantly reduced all-cause mortality and recurrent cardiovascular events, with risk reductions of 37% overall and 42% in the monotherapy population. Improvements were also seen in KCCQ and NYHA Classification scores, while NT-proBNP and Troponin I levels remained stable. Safety findings through 1 year were consistent with the double-blind period, suggesting long-term vutrisiran was well tolerated.

LESSON LEARNED:

Treatment with vutrisiran was associated with continued reductions in all-cause mortality and cardiovascular events compared with placebo over one year in the extension study. Overall, these results support vutrisiran's potential to become a standard of care for newly diagnosed and progressing patients.

Effects of Vutrisiran on Measures of Cardiac Structure, Function, and Amyloid Burden by Cardiovascular Magnetic Resonance from the HELIOS-B Trial

Y. Razvi, A. Sheikh, R. Patel, A. Achten, J. Mansell, B. Claggett, A. Martinez-Naharro, L. Venneri, P. N. Hawkins, J. Moon, J. Vest, S. Bansilal, S. Eraly, E. Aldinc, S. D. Solomon, J. D. Gillmore, M. Fontana

This study retrospectively identified 43 UK National Amyloidosis Centre patients participating in the HELIOS-B trial who underwent serial multiparametric cardiovascular magnetic resonance as part of their routine clinical care.

Pooled analysis showed that treatment with vutrisiran was associated with statistically significant and directionally favorable changes in multiple measures of cardiac structure and function compared to placebo.

LESSON LEARNED:

Over three years, vutrisiran showed favorable effects on cardiac structure, function, and amyloid burden versus placebo, supporting the idea that suppressing transthyretin production may promote amyloid regression and cardiac remodeling.

This program was funded by an independent grant from Alnylam.

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Vutrisiran Improved Outcomes Versus Placebo in Patients with Transthyretin Amyloidosis with Cardiomyopathy and Severe Chronic Kidney Disease: Post Hoc Analysis of HELIOS-B

F. H. Sheikh, J. Dang, M. Fontana, V. Audard, P. G.-Pavia, M. Khouri, A. Jobbe-Duval, Y. Brailovsky, H. Zheng, S. Early, C. Moffitt, A. Yilmaz

This study evaluated the impact of vutrisiran on renal function, efficacy, and safety in patients who progressed to CKD Stage 4 or greater during HELIOS-B. The post hoc analysis assessed the proportion of patients with a ≥40% decline in eGFR from baseline. Outcomes were evaluated in the overall and monotherapy groups and in the subgroup receiving tafamidis at baseline.

The data showed that fewer patients experienced worsening renal function with treatment versus placebo in HELIOS-B. Treatment was also associated with trends towards improvement in eGFR versus placebo over time. In the overall population, vutrisiran reduced the risk of ACM by 57% during the double-blind period plus 6 months of the open-label extension study, and the risk of recurrent CV events by 51% during the double-blind period.

LESSON LEARNED:

Vutrisiran may attenuate eGFR decline and improve clinical outcomes compared with placebo in patients with ATTR-CM and advanced CKD. This treatment was associated with preservation of renal function, with fewer patients experiencing ≥40% reductions in eGFR versus placebo. Overall, these data support the use of vutrisiran as an effective and well-tolerated treatment option for patients with ATTR-CM and compromised renal function.

Evidence of Fewer Gastrointestinal Events in ATTR-CM-Patients Treated with Vutrisiran Compared with Placebo: Analysis from HELIOS-B

M. Urey, Q. M.Bui, L. Obici, S. Early, E. Aldinc, C. Morbach

This analysis evaluated whether treatment with vutrisiran, compared with placebo, was associated with a lower incidence of gastrointestinal events among patients with ATTR-CM in the HELIOS-B study.

The analysis showed a 42% lower rate of GI events with vutrisiran versus placebo in the overall population, with consistent results in the monotherapy and baseline tafamidis groups. In the monotherapy group, GI events were reduced by 37%. while diarrhea, nausea, and vomiting decreased by over 50% across all study populations.

LESSON LEARNED:

These findings suggest that vutrisiran may offer a treatment benefit for GI manifestations potentially linked to ATTR. However, since adverse events were self-reported and not adjudicated, interpretation of the results may be limited.

This program was funded by an independent grant from Alnylam.