Patisiran can preserve functional capacity in transthyretin amyloidosis, according to study

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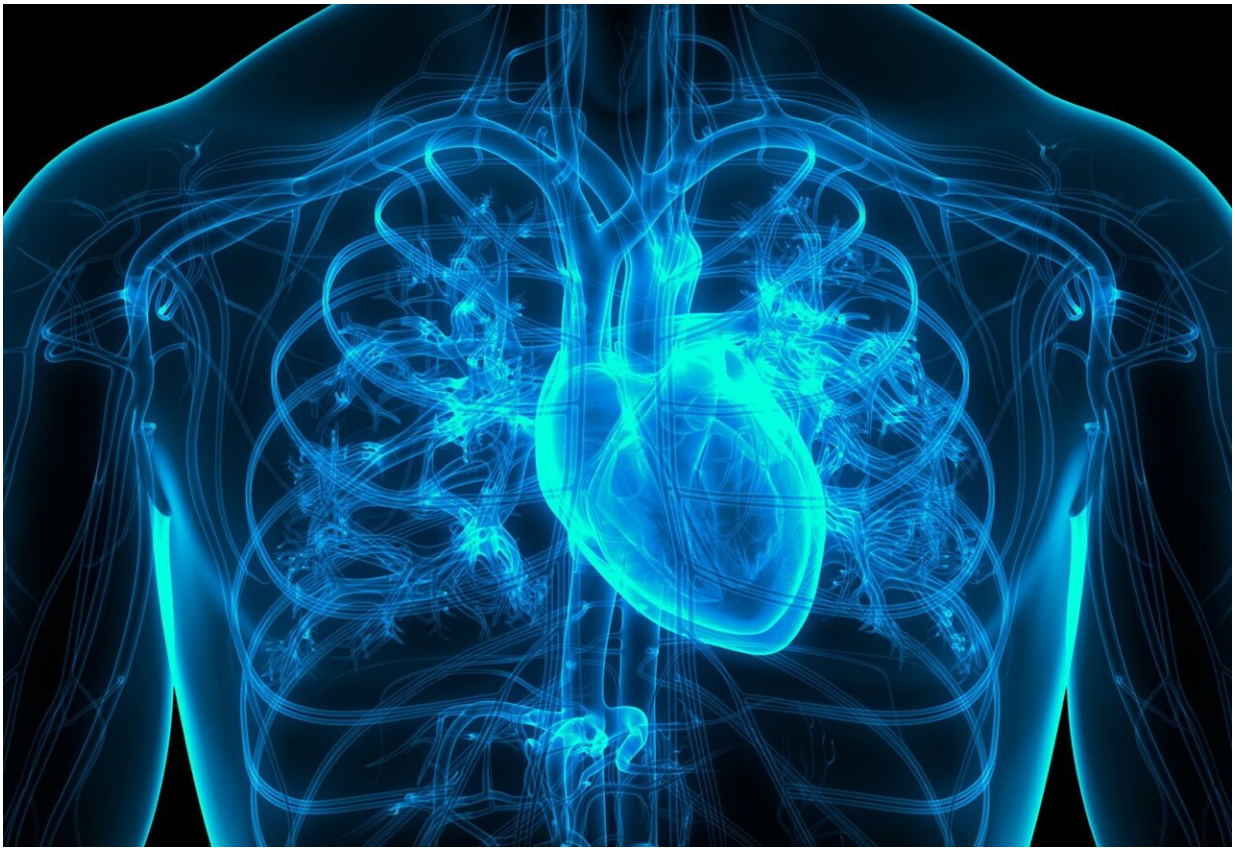

For patients with transthyretin amyloidosis (ATTR amyloidosis), administration of an RNA interference therapeutic agent, patisiran, over 12 months results in preserved functional capacity, according to a study

published in the Oct. 26 issue of the *New England Journal of Medicine*.

Mathew S. Maurer, M.D., from the Columbia University Irving Medical Center in New York City, and colleagues conducted a phase 3 randomized trial involving patients with hereditary ATTR cardiac amyloidosis who were assigned to receive patisiran or placebo once every three weeks for 12 months (181 and 179 patients, respectively).

The researchers found that the decline in the six-minute walk distance was lower in the patisiran group than the placebo group at month 12 (Hodges-Lehmann estimate of median difference, 14.69 m); the Kansas City Cardiomyopathy Questionnaire-Overall Summary score increased and decreased in the patisiran and placebo groups, respectively (least-squares mean difference, 3.7 points).

For the second secondary end point (composite of death from any cause, cardiovascular events, and change from baseline in the six-minute walk distance over 12 months), no significant benefits were observed. Compared with those receiving placebo, patients in the patisiran group more often had infusion-related reactions, arthralgia, and muscle spasms.

"Studies of longer duration are needed to determine whether the benefits observed with the reduction of transthyretin mediated by RNA interference are associated with a reduction in mortality and improved hospitalization outcomes in patients with ATTR cardiac amyloidosis," the authors write.

More information: Mathew S. Maurer et al, Patisiran Treatment in Patients with Transthyretin Cardiac Amyloidosis, *New England Journal of Medicine* (2023). DOI: 10.1056/NEJMoa2300757

Giampaolo Merlini, A Step Forward in Solving Amyloidosis, *New England Journal of Medicine* (2023). DOI: 10.1056/NEJMe2309308