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Drug reduces deaths and hospitalizations from underdiagnosed form of heart failure

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A phase three clinical trial has shown that a drug called tafamidis significantly reduces deaths and hospitalizations in patients with transthyretin amyloid cardiomyopathy (ATTR-CM), a progressive form of heart failure that may be more common than doctors realize.

The findings were published in the *New England Journal of Medicine* by the trial's co-chair Mathew S. Maurer, MD, a heart failure specialist at Columbia University Vagelos College of Physicians and Surgeons and NewYork-Presbyterian, and colleagues.

The takeaway

If tafamidis receives FDA approval for transthyretin amyloid cardiomyopathy, it would be the first medical therapy for this life-threatening disease.

Compared to a placebo, the drug reduced deaths by 30 percent, reduced cardiovascular-related hospitalizations by 32 percent, and slowed the decline in quality of life among the 441 patients enrolled in the 2½-year study.

Background

Once diagnosed, patients with ATTR-CM only live on average three to five more years.

"ATTR-CM is considered to be a rare disease, but it is underdiagnosed," Maurer says. "Until recently, cardiologists rarely tested for ATTR-CM, because diagnosis required a heart muscle biopsy and there has been no treatment for the disease. But now that we can detect the disease with noninvasive imaging, we're finding more cases."

ATTR-CM occurs when a protein called transthyretin becomes unstable and clumps together and forms sticky amyloid in heart muscle. (Amyloid deposits also occur in Alzheimer's disease, but those plaques develop through a different mechanism and cannot be treated with the drug tested in this study.)

The disease is most common in men over the age of 60 and is caused by heritable genetic mutations or age-related changes in the regulation of transthyretin.

Tafamidis acts by stabilizing transthyretin, preventing its dissociation and ability to form amyloid.

What the study means for patients

"Based on this study, tafamidis may offer the first treatment for patients with this type of heart disease," Maurer says. "Right now, the best we can do is manage the symptoms of ATTR-CM."

The drug, the study found, also slowed decline in heart function and quality of life without causing more adverse effects.

A different drug, patisiran, was recently approved by the FDA to treat nerve damage—but not heart disease—caused by transthyretin.

What's next

The U.S. Food and Drug Administration will consider whether to approve tafamidis for the treatment of transthyretin amyloid cardiomyopathy.

Patients can receive tafamidis at certain sites through an early access program established by Pfizer. NewYork-Presbyterian/Columbia University Irving Medical Center is the program's first site and is now accepting and enrolling patients.

Caveats

"Tafamidis prevents progression of the disease, and like other preventive drugs, it should be given as early as possible," Maurer says. "We'll need to diagnose people with ATTR-CM earlier for this drug to have the biggest benefit. Currently, patients are diagnosed with advanced disease, and we need to change that."

The study, "Tafamidis Treatment for Patients with Transthyretin Amyloid Cardiomyopathy," was published August 27 in the *New England Journal of Medicine*.

Provided by Columbia University