FDA approves treatments for heart failure caused by rare disease

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(HealthDay)—Vyndaqel (tafamidis meglumine) and Vyndamax

(tafamidis) capsules have been approved to treat adults with cardiomyopathy caused by transthyretin mediated amyloidosis (ATTR-CM), the U.S. Food and Drug Administration announced today.

Recommended dosage is four 20-mg capsules of Vyndaqel once daily or a single 61-mg capsule of Vyndamax once daily, according to the manufacturer. The two drugs, which are the first approved treatment for ATTR-CM, have the same moiety of tafamidis but are not substitutable on a per-milligram basis, the FDA said.

Approval was based on a clinical trial of 441 patients with ATTR-CM who were randomly assigned to receive Vyndaqel or placebo. Patients receiving Vyndaqel had a higher survival rate at an average of 30 months and a reduction in cardiovascular-related hospitalizations compared with patients receiving placebo.

Although patient numbers in the clinical trials were small, researchers did not identify any drug-related side effects. The manufacturer's prescribing information for Vyndaqel lists potential side effects of diarrhea, urinary tract infection, vaginal infection, and abdominal pain. Because of the risk for fetal harm with tafamidis, the FDA recommends providers discuss pregnancy planning and prevention with women.

Approval was granted to FoldRx, a subsidiary of Pfizer.

More information: More Information