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Regulatory submission of Tafamidis by Pfizer accepted by USFDA

17 January 2019 | News

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Pfizer has announced that the US Food and Drug Administration (FDA) accepted for filing the company's New Drug Applications (NDAs) for tafamidis for the treatment of transthyretin amyloid cardiomyopathy (ATTR-CM).

Pfizer has submitted two NDAs based on two forms of tafamidis: meglumine salt and free acid. Tafamidis is the only product to complete a Phase 3 trial evaluating its efficacy, safety, and tolerability in patients with ATTR-CM, a rare, fatal, and underdiagnosed condition.

The tafamidis meglumine form (20 mg capsule) has been granted Priority Review. The FDA grants Priority Review to medicines that may offer significant advances in treatment or may provide a treatment where no adequate therapy exists.

The tafamidis free acid form (61 mg capsule) will be under Standard Review. This form is bioequivalent to the 80 mg tafamidis meglumine dose, which was administered as four 20 mg capsules in the pivotal trial; it was developed for patient convenience to enable a single capsule for daily administration. The target PDUFA action date for a decision by the FDA is in November 2019.

Brenda Cooperstone MD, Senior Vice President and Chief Development Officer, Rare Disease, Pfizer Global Product Development said, "The diagnosis of ATTR-CM is often delayed, primarily because disease awareness is low and patients often present with symptoms similar to more common causes of heart failure. In fact, we believe less than one percent of patients living with this disease are currently diagnosed. The FDA's filing acceptance is an encouraging step toward our goal of further raising awareness and providing a treatment option for ATTR-CM patients who are in desperate need of an approved pharmacologic therapy. We look forward to working with the FDA to bring the first treatment for this deadly disease to patients."

The submission is based on findings from the pivotal Phase 3 Transthyretin Amyloid Cardiomyopathy (ATTR-ACT) study, which evaluated the efficacy, safety, and tolerability of tafamidis meglumine compared to placebo for the treatment of patients with ATTR-CM.

ATTR-CM is a rare and progressive disease caused by destabilization of a transport protein called transthyretin, which is composed of four identical subunits (a tetramer). In ATTR-CM, heart failure occurs when unstable tetramers dissociate, resulting in misfolded proteins that aggregate into amyloid fibrils and deposit predominantly in the heart.

Tafamidis is an oral, investigational product being evaluated as a potential treatment for ATTR-CM. Tafamidis is a small molecule that selectively binds at specific sites on the transthyretin tetramer to prevent destabilization of the transthyretin transport protein and formation of amyloid that causes ATTR-CM. It is not approved for any use in the United States.