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Pfizer gets USFDA approval for Vyndaqel® and Vyndamax

07 May 2019 | News

First and only medicines approved for patients with either wild-type or hereditary transthyretin amyloid cardiomyopathy

Pfizer Inc. announced that the U.S. Food and Drug Administration (FDA) has approved both VYNDAQEL® (tafamidis meglumine) and VYNDAMAX™ (tafamidis) for the treatment of the cardiomyopathy of wild-type or hereditary transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular mortality and cardiovascular-related hospitalization.

VYNDAQEL and VYNDAMAX are two oral formulations of the first-in-class transthyretin stabilizer tafamidis, and the first and only medicines approved by the FDA to treat ATTR-CM.

Transthyretin amyloid cardiomyopathy is a rare, life-threatening disease characterized by the buildup of abnormal deposits of misfolded protein called amyloid in the heart and is defined by restrictive cardiomyopathy and progressive heart failure. Previously, there were no medicines approved to treat ATTR-CM; the only available options included symptom management, and, in rare cases, heart (or heart and liver) transplant. It is estimated that the prevalence of ATTR-CM is approximately 100,000 people in the U.S and only one to two percent of those patients are diagnosed today.

"The approvals of VYNDAQEL and VYNDAMAX are a testament to the significant research and development investment in our innovative cardiovascular outcomes trial, ATTR-ACT. We are proud to bring these medicines to ATTR-CM patients who are in dire need of treatment," said Brenda Cooperstone, MD, Senior Vice President and Chief Development Officer, Rare Disease, Pfizer Global Product Development. "VYNDAQEL and VYNDAMAX reduce cardiovascular mortality and the frequency of cardiovascular-related hospital stays in patients with wild-type or hereditary forms of this rare disease, giving them a chance for more time with their loved ones."

"ATTR-CM is not only fatal, but also significantly underdiagnosed, with some patients cycling through multiple doctors and a myriad of tests over a period of years while the disease progresses," said Isabelle Lousada, Founder and CEO, Amyloidosis Research Consortium. "ATTR-CM is a rare disease for which more education and awareness is needed. The approval of these medicines represents an important advance for patients; however, it is equally important that we work as a community to recognize the critical importance of early diagnosis."

The FDA approval was based on data from the pivotal Phase 3 Transthyretin Amyloidosis Cardiomyopathy Clinical Trial (ATTR-ACT), the first global, double-blind, randomized, placebo-controlled clinical study to investigate a pharmacological therapy for the treatment of this disease.