Pfizer Logo

European Commission Approves VYNDAQEL®, the First Treatment in the EU for Transthyretin Amyloid Cardiomyopathy (ATTR-CM)

Tuesday, February 18, 2020 - 06:45am

—VYNDAQEL is the only EC-approved medicine proven to reduce mortality and frequency of cardiovascular-related hospitalizations in adults with wild-type or hereditary ATTR-CM—

—VYNDAQEL is the first approved medicine in the EU to treat both ATTR-CM and stage 1 symptomatic transthyretin amyloid polyneuropathy (ATTR-PN)—

NEW YORK--(BUSINESS WIRE)-- Pfizer Inc. (NYSE: PFE) announced today that the European Commission (EC) has approved VYNDAQEL® (tafamidis), a once-daily 61 mg oral capsule, for the treatment of wild-type or hereditary transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR-CM). VYNDAQEL is the first and only treatment approved in the European Union (EU) for patients with ATTR-CM. Prior to this approval, treatment options for patients with ATTR-CM were restricted to symptom management, and, in rare cases, heart (or heart and liver) transplant.

“Until today, there were no approved medicines to treat patients with ATTR-CM in the EU. Today’s approval represents incredible progress for these patients and reflects our steadfast commitment to delivering breakthrough medicines to rare disease patients,” said Paul Levesque, Global President, Pfizer Rare Disease. “Additionally, with today’s milestone, VYNDAQEL is now the first treatment to have two formulations approved in the EU to treat manifestations of transthyretin amyloidosis: one for cardiomyopathy, and one for stage 1 polyneuropathy.”

ATTR-CM is a rare, underdiagnosed and 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. Once diagnosed, the median life expectancy in patients with ATTR-CM, dependent on sub-type, is approximately two to 3.5 years.

“Before today, the European transthyretin amyloidosis community had a dire need for new therapeutic options that can improve outcomes for patients with cardiomyopathy,” said Thibaud Damy, MD, coordinator of the French Referral Centers for Cardiac Amyloidosis and past president of the French Heart Failure and Cardiomyopathy group, French Society of Cardiology. “VYNDAQEL represents a major advance for patients, as it can significantly reduce all-cause mortality and the frequency of cardiovascular-related hospitalizations in patients with wild-type or hereditary ATTR-CM.”

The EC approval of VYNDAQEL is based on results from the Phase 3 ATTR-ACT study, the first and only completed global, double-blind, randomized, placebo-controlled clinical trial to investigate a pharmacologic

therapy for the treatment of ATTR-CM. The study compared patients who received an oral daily dose of 20 mg or 80 mg of tafamidis meglumine compared to those who received placebo.

In the primary analysis of the study, VYNDAQEL (tafamidis meglumine) demonstrated a significant reduction in the hierarchical combination of all-cause mortality and frequency of cardiovascular-related hospitalisations compared to placebo over a 30-month period in patients with wild-type or hereditary ATTR-CM ($p=0.0006$). Additionally, individual components of the primary analysis demonstrated a relative reduction in the risk of all-cause mortality and frequency of cardiovascular-related hospitalization of 30% ($p=0.026$) and 32% ($p<0.0001$), respectively, with VYNDAQEL versus placebo.

VYNDAQEL also had significant and consistent treatment effects compared to placebo on functional capacity and health status first observed at six months and continuing through 30 months. Specifically, VYNDAQEL reduced the decline in performance on the six-minute walk test ($p<0.0001$) and reduced the decline in health status as measured by the Kansas City Cardiomyopathy Questionnaire – Overall Summary score ($p<0.0001$).

VYNDAQEL was well tolerated in this study, with an observed safety profile comparable to placebo. The frequency of adverse events in patients treated with VYNDAQEL was generally similar and comparable to placebo. The approval is also based on findings from an evaluation of the free acid form of tafamidis 61 mg, which demonstrated that one 61 mg capsule of tafamidis free acid corresponds to an 80 mg tafamidis meglumine dose (4 x 20 mg capsules). The safety of the 61 mg dose was not evaluated in ATTR-ACT. The tafamidis 61 mg capsule was developed for patient convenience to enable a single capsule for daily administration.

In 2011, the tafamidis meglumine 20 mg capsule formulation of VYNDAQEL was approved in the EU for transthyretin amyloidosis in adult patients with stage 1 symptomatic polyneuropathy (ATTR-PN) to delay peripheral neurologic impairment.

About ATTR Amyloidosis

ATTR amyloidosis is rare, progressive disease characterized by the abnormal buildup of amyloid deposits composed of misfolded transthyretin protein in the body’s organs and tissues. ATTR amyloidosis can impact numerous organs and tissues in the body, including the peripheral nervous system, and organs such as the heart, kidneys, gastrointestinal tract and eyes. ATTR-CM and ATTR-PN are two presentations of the disease.

ATTR-CM affects the heart and leads to restrictive cardiomyopathy and progressive heart failure. There are two sub-types of ATTR-CM: hereditary, which is caused by a mutation in the transthyretin gene and can occur in people as early as their 50s and 60s; or the wild-type form which is associated with aging, and is thought to be more common, usually affecting men after age 60. Often ATTR-CM is diagnosed only after symptoms have become severe.

ATTR-PN results from a genetic mutation of the transthyretin gene causing amyloid fibrils to form in the peripheral and autonomic nerves. ATTR-PN typically occurs during active adult years with onset as early as the 30s in some patients, followed by disease progression that may reach the terminal stage in approximately 10 years on average from disease onset.

About VYNDAQEL (tafamidis 61 mg) and VYNDAQEL (tafamidis meglumine 20 mg)

VYNDAQEL (tafamidis 61 mg) and VYNDAQEL (tafamidis meglumine 20 mg) are oral transthyretin stabilizers that selectively bind to transthyretin, stabilizing the tetramer of the transthyretin transport protein and slowing the formation of amyloid.

The tafamidis 61 mg capsule corresponds to an 80 mg tafamidis meglumine dose (4x 20mg capsules) and was developed for patient convenience to enable a single capsule for daily administration. VYNDAQEL 61 mg and VYNDAQEL 20 mg are not substitutable on a per milligram basis.

Tafamidis was granted Orphan Drug Designation for ATTR-CM in both the EU and US in 2012 and in Japan in 2018. Tafamidis was approved for the treatment of ATTR-CM in Japan under SAKIGAKE designation in March 2019, in the United States in May 2019, in the United Arab Emirates in November 2019, in Brazil in December 2019 and in Canada in January 2020.

VYNDAQEL (tafamidis meglumine) 20 mg was first approved in 2011 in the EU for the treatment of transthyretin amyloid polyneuropathy (ATTR-PN), in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment. Currently, it is approved for ATTR-PN in over 40 countries, including Japan, countries in Europe, Brazil, Mexico, Argentina, Israel, Russia, and South Korea. VYNDAQEL is not approved for ATTR-PN in the US.

VYNDAQEL (tafamidis), a once-daily 61 mg oral capsule, was granted marketing authorization for patients with ATTR-CM in the EU in February 2020.

VYNDAQEL® (tafamidis meglumine) and VYNDAMAX™ (tafamidis) From the U.S. Important Safety Information

Adverse Reactions

In studies in patients with ATTR-CM the frequency of adverse events in patients treated with VYNDAQEL was similar to placebo.

Specific Populations

Pregnancy: Based on findings from animal studies, VYNDAQEL and VYNDAMAX may cause fetal harm when administered to a pregnant woman.

Lactation: There are no available data on the presence of tafamidis in human milk, the effect on the breastfed infant, or the effect on milk production. Tafamidis is present in rat milk. When a drug is present in animal milk, it is likely the drug will be present in human milk. Breastfeeding is not recommended during treatment with VYNDAQEL and VYNDAMAX.

The full prescribing information for VYNDAQEL and VYNDAMAX can be found here.

Pfizer Rare Disease

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs. The Pfizer focus on rare disease builds on more than two decades of experience, a dedicated research unit focusing on rare disease, and a global portfolio of multiple medicines within a number of disease areas of focus, including hematology, neuroscience, and inherited metabolic disorders.

Pfizer Rare Disease combines pioneering science and deep understanding of how diseases work with insights from innovative strategic collaborations with academic researchers, patients, and other companies to deliver transformative treatments and solutions. We innovate every day leveraging our global footprint to accelerate the development and delivery of groundbreaking medicines and the hope of cures.

Click here to learn more about our Rare Disease portfolio and how we empower patients, engage communities in our clinical development programs, and support programs that heighten disease awareness.

Pfizer Inc.: Breakthroughs that change patients' lives

At Pfizer, we apply science and our global resources to bring therapies to people that extend and significantly improve their lives. We strive to set the standard for quality, safety and value in the discovery, development and manufacture of health care products, including innovative medicines and vaccines. Every day, Pfizer colleagues work across developed and emerging markets to advance wellness, prevention, treatments and cures that challenge the most feared diseases of our time. Consistent with our responsibility as one of the world's premier innovative biopharmaceutical companies, we collaborate with health care providers, governments and local communities to support and expand access to reliable, affordable health care around the world. For more than 150 years, we have worked to make a difference for all who rely on us. We routinely post information that may be important to investors on our website at www.pfizer.com. In addition, to learn more, please visit us on www.pfizer.com and follow us on Twitter at @Pfizer and @Pfizer_News, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

DISCLOSURE NOTICE: The information contained in this release is as of February 18, 2020. Pfizer assumes no obligation to update forward-looking statements contained in this release as the result of new information or future events or developments.

This release contains forward-looking information about VYNDAQEL (tafamidis), an approval for VYNDAQEL by the European Commission for the treatment of wild-type or hereditary transthyretin amyloidosis in adult patients with cardiomyopathy and Pfizer's rare disease portfolio, including their potential benefits, that involves substantial risks and uncertainties that could cause actual results to differ materially from those expressed or implied by such statements. Risks and uncertainties include, among other things, uncertainties regarding the commercial success of VYNDAQEL; the uncertainties inherent in research and development, including the ability to meet anticipated clinical endpoints, commencement and/or completion dates for our clinical trials, regulatory submission dates, regulatory approval dates and/or launch dates, as well as the possibility of unfavorable new clinical data and further analyses of existing clinical data; the risk that clinical trial data are subject to differing interpretations and assessments by regulatory authorities; whether regulatory authorities will be satisfied with the design of and results from our clinical studies; whether and when any new or supplemental drug applications may be filed in any other jurisdictions for VYNDAQEL; whether and when regulatory authorities in any other jurisdictions where applications for VYNDAQEL may be pending or filed for the treatment of wild-type or hereditary transthyretin amyloidosis or any other potential indications for VYNDAQEL may approve any such applications, which will depend on myriad factors, including making a determination as to whether the product's benefits outweigh its known risks and determination of the product's efficacy, and, if approved, whether VYNDAQEL will be commercially successful; decisions by regulatory authorities impacting labeling, manufacturing processes, safety and/or other matters that could affect the availability or commercial potential of VYNDAQEL, including for the treatment of wild-type or hereditary transthyretin amyloidosis in adult patients with cardiomyopathy (ATTR-CM); and competitive developments.

A further description of risks and uncertainties can be found in Pfizer's Annual Report on Form 10-K for the fiscal year ended December 31, 2018 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information and Factors That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the U.S. Securities and Exchange Commission and available at www.sec.gov and www.pfizer.com.

View source version on businesswire.com: https://www.businesswire.com/news/home/20200218005225/en/

Media Relations:
Lisa O’Neill
0044 7929 339560
Lisa.O'Neill@pfizer.com

Steve Danehy
212-733-1538
Steven.Danehy@pfizer.com

Investors:
Chuck Triano
212-733-3901
Charles.E.Triano@pfizer.com

Source: Pfizer Inc.