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FDA Advisory Committee Finds Data Support Effectiveness of Tafamidis Meglumine, Pfizer's Novel Investigational Treatment for Transthyretin Familial Amyloid Polyneuropathy (TTR-FAP)

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(BUSINESS WIRE)--Pfizer Inc. (NYSE:PFE) announced today that the United States Food and Drug Administration's (FDA) Peripheral and Central Nervous System Drugs Advisory Committee voted on Pfizer's clinical data package for tafamidis meglumine. Tafamidis is a novel, investigational, oral therapy for the treatment of Transthyretin Familial Amyloid Polyneuropathy (TTR-FAP) in adult patients with symptomatic polyneuropathy to delay neurologic impairment. The Advisory Committee did not find substantial evidence of efficacy on a clinical endpoint. The Committee then voted 13-4 that the data provide substantial evidence of efficacy for a surrogate endpoint that is reasonably likely to predict a clinical benefit. This recommendation will be taken into consideration by the FDA when making its decision on Pfizer's New Drug Application (NDA) for tafamidis as a treatment for TTR-FAP, a rare and fatal neurodegenerative disease.

"TTR-FAP is an irreversible and devastating disease with no FDA-approved treatment in the U.S.," said Dr. Yvonne Greenstreet, senior vice president and head of Medicines Development Group for Pfizer's Specialty Care Business Unit. "The panel's assessment represents a positive step forward in our goal to provide this much-needed medicine to patients suffering from this rare and fatal disease. Pfizer will continue to work with the FDA as the Agency finalizes its review of our NDA for tafamidis."

TTR-FAP is a rare, progressive and fatal neurodegenerative disease that affects approximately 8,000 patients worldwide.[1,2,3] Because it is a hereditary disease, family members may also be at risk for developing the disease.[4] In the U.S., a non-endemic region, the incidence is estimated to be about 1 in 100,000, impacting approximately 3,000 people.[5,6] There is currently no FDA-approved treatment in the U.S. designed specifically to treat TTR-FAP. If approved by the FDA, tafamidis would be the first and only medication in the U.S. indicated to treat patients with this debilitating genetic disease.

The FDA has granted the tafamidis NDA both an orphan drug and a priority review designation. The Orphan Drug Designation program provides orphan status to drugs and biologics that are defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases and disorders that affect fewer than 200,000 people in the U.S. Priority review designation is granted to drugs that have the potential to offer significant improvement in treatment or provide a treatment where no adequate therapy exists.

About Transthyretin Familial Amyloid Polyneuropathy (TTR-FAP)

Transthyretin Familial Amyloid Polyneuropathy (TTR-FAP) is a rare and fatal neurodegenerative disease, primarily caused by a genetic mutation of the transthyretin (TTR) gene.[1,2] In TTR-FAP, TTR destabilization leads to misfolded proteins that form amyloid fibrils in the peripheral and autonomic nerves, as well as other organs including the GI tract, kidneys and heart.[7,8] Patients with TTR-FAP experience significantly diminished quality of life due to symptoms including polyneuropathy characterized by sensory loss, pain and weakness in the lower limbs; as well as severe impairment of the autonomic nervous system commonly manifesting as erectile dysfunction, alternating diarrhea and constipation, unintentional weight loss, orthostatic hypotension, urinary incontinence, urinary retention and delayed gastric emptying.[4,7,8] As the disease progresses, patients often lose the ability to walk, needing wheelchair assistance, and eventually become bedridden and unable to care for themselves.[9] TTR-FAP typically occurs during active adult years with onset as early as the 30s, followed by disease progression that may reach the terminal stage in approximately 10 years on average.[8,10]

About Tafamidis

Tafamidis is a novel, selective stabilizer of the TTR protein approved in November 2011 by the European Commission (the trade name in the European Union is VYNDAQEL®). It is indicated in the European Union for the treatment of TTR amyloidosis in adult patients with stage 1 symptomatic polyneuropathy to delay peripheral neurologic impairment.

About Pfizer's Specialty Care Business

Pfizer's Specialty Care Business Unit is the world's largest specialty pharmaceuticals business, with a commitment to the eradication, remission, and relief of serious diseases. Pfizer's Specialty Care Business Unit brings together the best scientific minds to challenge the most feared diseases of our time, and we seek solutions to prevent and relieve suffering of patients with serious diseases, regardless of prevalence. Pfizer is an established global leader in rare diseases, offering marketed products treating 18 orphan indications in the U.S. to address the unique needs of small patient populations affected by uncommon and often life-threatening conditions. We are on the front lines of discovering innovative medicines and delivering hope through continued focus on research, development and commercialization of orphan medicines.

Pfizer Inc: Working together for a healthier world™

At Pfizer, we apply science and our global resources to improve health and well-being at every stage of life. We strive to set the standard for quality, safety and value in the discovery, development and manufacturing of medicines for people and animals. Our diversified global health care portfolio includes human and animal biologic and small molecule medicines and vaccines, as well as nutritional products and many of the world's best-known consumer products. 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 the world's leading biopharmaceutical company, we also 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, Pfizer has worked to make a difference for all who rely on us. To learn more about our commitments, please visit us at www.pfizer.com.

DISCLOSURE NOTICE: *The information contained in this release is as of May 24, 2012. 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 that involves substantial risks and uncertainties about a product in development, tafamidis, including its potential benefits, that is under review by the FDA. Such risks and uncertainties include, among other things, the uncertainties inherent in research and development; whether and when the FDA and regulatory authorities in other jurisdictions in which applications may be filed will approve applications for tafamidis as well as their decisions regarding labeling and other matters that could affect its availability or commercial potential; 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, 2011 and in its reports on Form 10-Q and Form 8-K.

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