Pfizer Announces Positive Topline Results From Phase 3 ATTR-ACT Study Of Tafamidis In Patients With Transthyretin Cardiomyopathy

Thursday, March 29, 2018 - 08:47am

Tafamidis Demonstrated a Statistically Significant Reduction in the Combination of All-cause Mortality and Frequency of Cardiovascular-related Hospitalizations in Global Trial
Currently, There are No Approved Pharmacological Medications Specifically Indicated for Treating Transthyretin Cardiomyopathy

Pfizer Inc. (NYSE:PFE) announced today that the Tafamidis Phase 3 Transthyretin Cardiomyopathy (ATTR-ACT) study evaluating tafamidis for the treatment of transthyretin cardiomyopathy met its primary endpoint, demonstrating a statistically significant reduction in the combination of all-cause mortality and frequency of cardiovascular-related hospitalizations compared to placebo at 30 months. The preliminary safety data showed that tafamidis was generally well tolerated in this population and no new safety signals were identified.

The ATTR-ACT study was designed to assess clinically meaningful outcomes for the use of tafamidis as a treatment for patients with transthyretin cardiomyopathy, a rare, fatal, and underdiagnosed condition associated with progressive heart failure.1,2 The average life expectancy for people with transthyretin cardiomyopathy is 3 to 5 years from diagnosis.3 The prevalence of transthyretin cardiomyopathy is presently unknown; however, it is estimated that less than 1% of people with the disease are diagnosed. Currently, there are no approved pharmacological medications specifically indicated for treating transthyretin cardiomyopathy.4

“These topline results are important for people with transthyretin cardiomyopathy and bring us one step closer to realizing the potential for a new treatment for those in desperate need,” said Brenda Cooperstone MD, senior vice president and chief development officer, Rare Disease, Pfizer Global Product Development. “Pfizer Rare Disease has been at the forefront of improving the understanding of transthyretin cardiomyopathy, and we thank the patients who participated in the trial and their families, as well as the physicians and investigational sites that contributed to this important study. We look forward to sharing the detailed results of the study with the cardiovascular community and discussing these data with health authorities to determine an appropriate regulatory path forward.”

“Our findings offer real hope for people with transthyretin cardiomyopathy and their families,” said Mat Maurer MD, Arnold and Arlene Goldstein professor of Cardiology, Columbia University Vagelos College of Physicians and Surgeons. “As health care professionals, all we can do right now is manage symptoms of the disease, as there are no approved pharmacological treatment options at this time. The need for medicines that treat transthyretin cardiomyopathy is critical.”

In 2011, tafamidis was granted orphan drug designation for transthyretin cardiomyopathy in both the EU and US. In June 2017, the US Food and Drug Administration (FDA) granted Fast Track designation to tafamidis for transthyretin cardiomyopathy; additionally, in March 2018, the Ministry of Labor Health and Welfare in Japan granted SAKIGAKE designation to tafamidis for this indication.

About the ATTR-ACT Study1 ATTR-ACT is a Phase 3 international, multicenter, double-blind, placebo-controlled, randomized, 3-arm clinical study in 441 patients that investigated the efficacy, safety, and tolerability of an oral daily dose of 20 mg or 80 mg tafamidis meglumine capsules compared to placebo. The study included both patients with the variant, or hereditary, form of the disease, and those with the wild-type form, which is not hereditary and may occur as people age. The primary analysis of the study, which compared tafamidis to placebo, was the hierarchical combination of all-cause mortality and frequency of cardiovascular-related hospitalizations over a 30-month period in patients with transthyretin cardiomyopathy.

For more information on the ATTR-ACT study, go to www.clinicaltrials.gov. These results are preliminary topline data and are subject to further analysis. The full data and detailed results will be submitted for presentation at an upcoming scientific congress as well as for publication in a peer-reviewed journal.

Tafamidis is an investigational treatment for transthyretin cardiomyopathy and is not approved for this indication.

Pfizer Rare Disease Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide,5 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.

Working together for a healthier world® 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. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer health care 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 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 March 29, 2018. 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 a potential indication for tafamidis for the treatment of transthyretin cardiomyopathy (the “Potential Indication”) 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, the uncertainties inherent in research and development, including, without limitation, the ability to meet anticipated clinical trial commencement and completion dates and regulatory submission dates, as well as the possibility of unfavorable clinical trial results, including unfavorable new clinical data and additional analyses of existing clinical data; the risk that clinical trial data are subject to differing interpretations, and, even when we view data as sufficient to support the safety and/or effectiveness of a product candidate, regulatory authorities may not share our views and may require additional data or may deny approval altogether; 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 jurisdictions for tafamidis for the Potential Indication; whether and when regulatory authorities in any such jurisdictions where applications for tafamidis may be pending (including the application pending with the FDA for the potential treatment of transthyretin familial amyloid polyneuropathy, for which the company received a complete response letter in 2012) or filed may approve any such applications, which will depend on the assessment by such regulatory authority of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted, and, if approved, whether tafamidis will be commercially successful; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of tafamidis, including for the Potential Indication; 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, 2017 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.

References _____ 1Data on file. Pfizer Inc. New York, NY.

2THAOS - Transthyretin Amyloidosis Outcomes Survey. Disease Background - transthyretin amyloidosis. https://www.thaos.net/Physicians/DiseaseBackground.cfm.

Accessed March 13, 2018.

3Connors LH, Doros G, Sam F, Badiee A, Seldin DC, Skinner M. Clinical features and survival in senile systemic amyloidosis: comparison to familial transthyretin cardiomyopathy. *Amyloid*. 2011;18(sup1):157-159. doi:10.3109/13506129.2011.574354059

4Ando Y, Coelho T, Berk JL, et al. Guideline of transthyretin-related hereditary amyloidosis for clinicians. *Orphanet Journal of Rare Diseases*. 2013;8(1):31. doi:10.1186/1750-1172-8-31.

5Pfizer Inc. Rare disease. http://www.pfizer.com/health-and-wellness/health-topics/rare-diseases/areas-of-focus. Accessed March 9, 2018.

Pfizer Inc. Media Relations: Neha Wadhwa, 212-733-2835 Neha.Wadhwa@pfizer.com or Investors: Chuck Triano, 212-733-3901 Charles.E.Triano@pfizer.com