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Pfizer Showcases 12 New Data Presentations On Transthyretin Amyloidosis, Increasing Understanding Of A Rare Disease

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Findings Presented at International Symposium on Amyloidosis Provide Insights into Initial Symptoms and Signs, Underscore Importance of Early Diagnosis and Treatment

NEW YORK, NY, July 5 - Pfizer Inc. (NYSE:PFE) announced today new research findings that can help clinicians better understand and identify the rare, neurodegenerative disease, transthyretin familial amyloid polyneuropathy (TTR-FAP), and transthyretin cardiomyopathy (TTR-CM), a related type of amyloidosis that causes progressive heart failure. The findings are among 12 Pfizer presentations at the 15th International Symposium on Amyloidosis (ISA) in Uppsala, Sweden being held July 3-7. More information about the presentations is available at http://www.pfizer.com/news/press_kits/TTR_Amyloidosis

The 12 Pfizer oral and poster presentations include insights into TTR-FAP early signs, symptoms [1,2] and associated risk factors, all of which health care providers must recognize for earlier diagnosis and treatment. Findings are also being presented regarding disease epidemiology,[3,4,5] progression[6,7] and burden among patients and caregivers.8,9 TTR-FAP, which often takes years to diagnose, is a genetic, progressive, and irreversible disease that significantly impairs quality of life and is estimated to affect about 10,000 people worldwide.[10] When left untreated, people with TTR-FAP die, on average, within 10 years of symptom onset.[11,12]

The Pfizer presentations that highlight TTR-CM[13,14,15] include the the first detailed description of the rationale and design of the Transthyretin Amyloid Cardiomyopathy Tafamidis* Clinical Trial (ATTR-ACT), a Phase 3 clinical trial.[14]

"The data presented here are critical to advancing the understanding of the spectrum of this debilitating disease," said Kevin W. Williams, M.D., J.D., M.P.H., chief medical officer, Rare Disease, Pfizer Innovative Health. "As part of Pfizer's dedication to the rare disease community, we remain committed to being at the forefront of raising awareness of TTR-FAP and TTR-CM to help both patients and their loved ones."

About TTR-FAP

TTR-FAP is caused by a mutation in the gene for the protein transthyretin (TTR), resulting in the production of unstable TTR proteins that can accumulate as amyloid deposits in nerves and other organs, interfering with normal function.[16,17] Patients with TTR-FAP experience considerable burden of illness early in the course of disease and this burden increases with disease progression. As TTR-FAP symptoms progress, patients require a considerable amount of assistance, are unable to care for themselves, and may become bedridden or require hospitalization.[18,19]

About TTR-CM

TTR-CM, a type of cardiac amyloidosis, is a rare and fatal condition that leads to progressive heart failure. The average life expectancy for people with TTR-CM is three to five years from diagnosis. Currently, there are no approved medications specifically for TTR-CM. In 2013, Pfizer initiated the ATTR-ACT Study, the first Phase 3 clinical trial in TTR-CM, to evaluate the safety and efficacy of tafamidis in a global, double-blind, randomized, placebo-controlled study. The study completed enrollment in August 2015.[20]

Pfizer and Rare Diseases

Rare diseases are among the most serious of all illnesses and impact millions of patients worldwide, representing an opportunity to apply our knowledge and expertise to help make a significant impact in addressing unmet medical needs. The Pfizer focus on rare diseases builds on more than a decade of experience and a global portfolio of more than 20 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders, pulmonology and oncology.

As a leader in the TTR-FAP community, Pfizer Inc. has been at the forefront of educational initiatives to raise awareness of this rare disease among health care professionals and to facilitate dialogue between patients, their families and their physicians. These efforts have contributed to a global increase in diagnosis rates and treatment.[20]

Pfizer Inc.: 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 healthcare products. Our global portfolio includes medicines and vaccines as well as many of the world's best-known consumer healthcare 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, Pfizer has worked to make a difference for all who rely on us. For more information, please visit us at www.pfizer.com. In addition, to learn more,

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* Tafamidis is an investigational agent not approved in the United States.

1 Coelho T, Keohane D, Sultan M, Carlsson M, Ong M-L. Transition from asymptomatic to symptomatic transthyretin familial amyloid polyneuropathy: an analysis from the Transthyretin Amyloidosis Outcomes Survey. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

2 Waddington Cruz M, Schmidt H, Botteman MF, Carter JA, Chopra AS, Stewart M, Hopps M, Fallet S, Amass L. Epidemiological and clinical characteristics of persons with transthyretin familial amyloid polyneuropathy: A global synthesis of 532 cases. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

3 Waddington Cruz M, Barroso F, González-Duarte A, Mundayat R, Ong M-L, on behalf of the THAOS Investigators. The demographic, genetic, and clinical characteristics of Latin American subjects enrolled in the Transthyretin Amyloidosis Outcomes Survey. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

4 Waddington Cruz M, Foguel D, Berensztejn AC, Pedrosa RC, Mundayat R, Ong M-L, on behalf of THAOS Investigators. The demographic, genetic, and clinical characteristics of Brazilian subjects enrolled in the Transthyretin Amyloidosis Outcomes Survey. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

5 Schmidt H, Waddington Cruz M, Botteman MF, Carter JA, Chopra A, Stewart M, Hopps M, Fallet S, Amass L. Global epidemiology of transthyretin familial amyloid polyneuropathy: A Systematic review. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

6 Li, H, Gundapaneni B, Schwartz J, Keohane D, Amass L. Impact of baseline neurologic score on disease progression in transthyretin familial amyloid polyneuropathy. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

7 Coelho T, Waddington Cruz M, Fallet S, Carlsson M, Ong M-L. The natural history of transthyretin familial polyneuropathy: An analysis from the Transthyretin Amyloidosis Outcomes Survey. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

8 Stewart M , Shaffer S, Murphy B, Loftus J, Alvir J, Cicchetti M, Lenderking WR. Characterizing the high disease burden of transthyretin amyloidosis for patients and caregivers. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

9 Conceição I, Stewart M, Sultan M, Mundayat R, Ong M-L, Keohane D. A health-related quality of life comparison of symptomatic transthyretin amyloidosis subjects and asymptomatic carriers from the Transthyretin Amyloidosis Outcome Survey. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

10 Plante´-Bordeneuve V, Update in the diagnosis and management of transthyretin familial amyloid polyneuropathy. Neurology. 2014:261 :1227-1233. doi:10.1007/s00415-014-7373-0.

11 Plante´-Bordeneuve V, Ferreira A, Lalu T, et al. Diagnostic pitfalls in sporadic transthyretin familial amyloid polyneuropathy (TTR-FAP). Neurology. 2007;69:693-698.

12 Coelho T, Maia LM, Martins da Silva A, et al. Long-term effects of tafamidis for

the treatment of
transthyretin familial amyloid polyneuropathy. J Neurol. 2013.doi: 10.1007/s00415-013-7051-7.

13 Rapezzi C, Waddington Cruz M, Lorenzini M, Maurer MS, Kristen AV, Damy T, Coelho T, Yu C-R, Ong M- L, on behalf of THAOS Investigators. Male gender is a risk factor for myocardial involvement in transthyretin-related amyloidosis: A study based on the Transthyretin Amyloidosis Outcomes Survey. Oral presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

14 Maurer M, Elliott P, Merlini G, Shah SJ, Waddington Cruz M, Flynn A, Gundapaneni B, Hahn C, Riley S, Schwartz J, Sultan MB, Rapezzi C, on behalf of the 1028 Study Investigators. Rationale and design of the
Phase 3 Transthyretin Amyloid Cardiomyopathy Tafamidis clinical trial. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

15 Garan AR, Li H, Ebede B, Chyou J, Dizon J, Maurer M. The Prognostic Value of Ventricular Arrhythmias
In Transthyretin Cardiac Amyloidosis. Poster presentation at: 15th International Symposium on Amyloidosis, July 3-7, 2016, Uppsala, Sweden.

16 Benson MD, Kincaid JC. The molecular biology and clinical features of amyloid neuropathy. Muscle
Nerve. 2007;36:411-423.

17 Hou X, Aguilar M-I, Small DH. Transthyretin and familial amyloidotic polyneuropathy: recent progress in understanding the molecular mechanism of neurodegeneration. FEBS J. 2007;274:1637-1650.

18 Suhr OB, Svendsen IH, Andersson R, Danielsson A, Holmgren G, Ranløv PJ. Hereditary transthyretin
amyloidosis from a Scandinavian perspective. J Intern Med. 2003 Sep;254(3):225-35.

19 Jonsèn E, Athlin E, Suhr O. Familial amyloidotic patients' experience of the disease and of liver transplantation. J Adv Nurs. 1998;27:52-58.

20 Data on file. Pfizer Inc, New York, NY