Pfizer Completes Enrollment of Phase 2 Study of Domagrozumab (PF-06252616) in Duchenne Muscular Dystrophy

NEW YORK, N.Y. May 30 – Pfizer Inc. (NYSE:PFE) announced today the completion of patient enrollment in a multicenter Phase 2 clinical trial of the investigational compound, domagrozumab (PF-06252616), in boys with Duchenne muscular dystrophy (DMD). The trial enrolled 121 patients and seeks to evaluate the safety, tolerability and efficacy of PF-06252616 in boys aged 6 to less than 16 years old diagnosed with DMD regardless of genotype. The study is conducted over two years, after which participants may be eligible to continue on an open-label extension study.

“We would like to extend our heartfelt gratitude to all the boys and families who expressed interest in the study, underwent screening for the study, and to those who ultimately participated,” said Greg LaRosa, Ph.D., Senior Vice President and Chief Scientific Officer of Pfizer’s Rare Disease Research Unit. “DMD is a devastating disease, and taking this challenge on requires a collaborative approach, including the essential support from the advocacy community in both guiding trial design and implementation, as well as providing ongoing resources to patients and caregivers.”

Domagrozumab is an experimental, infused, anti-myostatin monoclonal antibody. Myostatin is a naturally occurring protein in muscles that helps control muscle growth; it is believed that blocking the activity of myostatin may have potential therapeutic application in treating muscle wasting diseases such as DMD.

Domagrozumab was granted Orphan Drug designation in July 2012 and Fast Track Designation in November 2012 by the U.S. Food and Drug Administration. The European Medicines Agency granted the investigational candidate Orphan Medical Product designation in February 2013.
Pfizer is also advancing a pre-clinical asset for DMD using recombinant Adeno-Associated Virus (rAAV) vector-based gene therapy, which entered Pfizer’s portfolio with the 2016 acquisition of Bamboo Therapeutics, Inc. Gene therapy is an emerging area of medical research focused on highly specialized, transformative treatments addressing the root cause of diseases caused by genetic mutation.

**About Duchenne Muscular Dystrophy**

Duchenne muscular dystrophy (DMD) is a genetic disorder characterized by progressive muscle degeneration and weakness. DMD is caused by an absence of dystrophin, a protein that helps keep muscle cells intact. Symptom onset is in early childhood, usually between ages of 3 and 5. The disease primarily affects boys, but in rare cases it can affect girls. Muscle weakness can begin as early as age 3, first affecting the muscles of the hips, pelvic area, thighs and shoulders, and later the skeletal (voluntary) muscles in the arms, legs and trunk. The calves often are enlarged. By the early teens, the heart and respiratory muscles are also affected.

**Pfizer and Rare Disease**

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide,\(^i\) 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.\(^ii\)

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 and meet the needs of patient families.

**About 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 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 @PfizerNews, LinkedIn, YouTube and like us on Facebook at Facebook.com/Pfizer.

DISCLOSURE NOTICE: The information contained in this release is as of May 30, 2017. 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 product candidate, domagrozumab (PF-06252616), including its 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 the ability to meet anticipated clinical study commencement and completion dates as well as the possibility of unfavorable study results, including unfavorable new clinical data and additional analyses of existing clinical data; risks associated with initial data, including the risk that the final results of the Phase 2 study for domagrozumab and/or additional clinical trials may be different from (including less favorable than) the initial data results and may not support further clinical development; whether and when new drug applications may be filed in any jurisdictions for domagrozumab; whether and when any such applications may be approved by regulatory authorities, which will depend on the assessment by such regulatory authorities of the benefit-risk profile suggested by the totality of the efficacy and safety information submitted; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of domagrozumab; 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, 2016 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.
