



# Pfizer Initiates Phase 2 Study of PF-06252616 in Duchenne Muscular Dystrophy

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Pfizer Inc. (NYSE:PFE) announced today enrollment of the first patient in a multicenter Phase II clinical trial of the investigational compound PF-06252616 in boys with Duchenne muscular dystrophy (DMD), a genetic disorder characterized by progressive muscle degeneration and weakness. PF-06252616 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.

“DMD is a devastating and debilitating disease impacting approximately 1 in 3,500 male births worldwide with no current treatment options,” said Kevin Lee, Ph.D., senior vice president and chief scientific officer of Pfizer’s Rare Disease Research Unit. “We are pleased to be taking this important next step in the development of PF-06252616 as an investigational therapy for DMD in the hopes of potentially bringing a much-needed therapy to individuals and families with this devastating disease.”

The phase 2 clinical trial will evaluate the safety, tolerability and efficacy of PF-06252616 in boys aged 6 to <10 years old diagnosed with DMD regardless of genotype. Based on the proposed mechanism of action of PF-06252616, Pfizer is exploring whether there is the potential to increase muscle mass and function in boys with DMD who are weak and have lost muscle.

“We are enthusiastic about the potential for myostatin inhibitors to stimulate increases in muscle mass and strength for people living with Duchenne muscular dystrophy. This approach could potentially add an important angle in our fight against this disease and we are pleased to see the time and great care that Pfizer has expended on its development,” said Dr. Sharon Hesterlee, vice president of Research for Parent Project

Muscular Dystrophy (PPMD). “Our Duchenne community is proud to have the extraordinary passion and expertise of the Pfizer team fighting with us.”

PF-06252616 was granted Orphan Drug designation in July 2012 and Fast Track Designation in November 2012 by the U.S. Food and Drug Administration (FDA). The FDA’s Fast Track Designation is a process designed to facilitate the development and expedite the review of new drugs and biologics intended to treat or prevent serious conditions and that address an unmet medical need.<sup>ii</sup> The European Medicines Agency (EMA) granted the investigational candidate Orphan Medical Product designation in February 2013.

### 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 the 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.<sup>iii</sup>

### 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 22 medicines approved worldwide that treat rare diseases in the areas of hematology, neuroscience, inherited metabolic disorders, pulmonology, and oncology.

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, Pfizer has worked to make a difference for all who rely on us. To learn more, please visit us at [www.pfizer.com](http://www.pfizer.com).

DISCLOSURE NOTICE: The information contained in this release is as of December 17, 2014. 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, 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. Such 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; whether and when new drug applications may be filed in any jurisdictions for PF-06252616; 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 PF-06252616; 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, 2013 and in its subsequent reports on Form 10-Q, including in the sections thereof captioned "Risk Factors" and "Forward-Looking Information That May Affect Future Results", as well as in its subsequent reports on Form 8-K, all of which are filed with the SEC and available at [www.sec.gov](http://www.sec.gov) and [www.pfizer.com](http://www.pfizer.com).

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i National Institutes of Health. National Human Genome Research Institute. Learning About Duchenne Muscular Dystrophy. Available on <http://www.genome.gov/19518854>. Accessed on December 4, 2014

ii United States Food and Drug Administration. Fast Track, Breakthrough Therapy, Accelerated Approval, and Priority Review. Available at <http://www.fda.gov/ForPatients/Approvals/Fast/ucm405399.htm>. Accessed on

December 15, 2014.

iii Muscular Dystrophy Association. Duchenne Muscular Disease Overview. Available at <http://mda.org/disease/duchenne-muscular-dystrophy/overview>. Accessed on December 15, 2014.

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