



# Pfizer Terminates Domagrozumab (PF-06252616) Clinical Studies for the Treatment of Duchenne Muscular Dystrophy

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NEW YORK--(BUSINESS WIRE)--Pfizer Inc. (NYSE: PFE) announced today that it is terminating two ongoing clinical studies evaluating domagrozumab (PF-06252616) for the treatment of Duchenne muscular dystrophy (DMD): a Phase 2 safety and efficacy study (B5161002) and an open-label extension study (B5161004). The Phase 2 study (B5161002), did not meet its primary efficacy endpoint, which was to demonstrate a difference in the mean change from baseline in 4 Stair Climb (in seconds) following one year of treatment with domagrozumab as compared to placebo in patients with DMD. Further evaluation of the totality of evidence including secondary endpoints did not support a significant treatment effect. The decision comes after a thorough review of data available at the time of the primary analysis, which evaluated all study participants after one year of treatment, as well as those participants who were in the trial beyond one year. The studies were not terminated for safety reasons. Pfizer will continue to review the data to better understand any insights they may provide, and will share results with the scientific and patient community.

“We are disappointed by these results and while we are not progressing with the studies, the data will contribute to a greater understanding of this disease and we will evaluate the total data set to see if there is a place for this medicine in muscular diseases,” said Seng Cheng, PhD, Senior Vice President and Chief Scientific Officer, Pfizer Rare Disease Research Unit. “We are extremely grateful to all those involved with this trial, especially the boys who participated, and their families.”

Pfizer is continuing research in DMD and rare neuromuscular diseases, with the goal of bringing therapies to patients with unmet needs. The company's continued partnership with advocacy associations and the community is critical to finding innovative therapies for these diseases. Pfizer has one ongoing clinical trial in DMD with a gene therapy, PF-06939926, which is an investigational, recombinant AAV9 capsid carrying a truncated or shortened version of the human dystrophin gene (mini-dystrophin) under the control of a human muscle specific promotor.<sup>1</sup>

### About the Domagrozumab Clinical Studies<sup>1</sup>

The Phase 2 double-blind, placebo-controlled, multicenter clinical trial investigated the efficacy and safety of domagrozumab, administered in monthly IV doses, in 121 boys aged 6 to 15 with DMD, regardless of underlying mutation. It was designed as a two-year, placebo-controlled study (with the primary analysis after one year); all subjects used background corticosteroid therapy. The open-label extension study was designed to evaluate long-term safety and efficacy of domagrozumab.

### About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a rare, serious, debilitating childhood genetic disease characterized by progressive muscle degeneration and weakness and significantly shortened life expectancy. It is the most common form of muscular dystrophy worldwide and primarily affects boys, with incidence of 1 in every 3500 to 5000 live male births each year.<sup>2,3</sup> Children with DMD typically present with signs of weakness, including late walking, trouble getting up, and difficulty running or climbing stairs, usually manifesting in early childhood between the ages of 1 and 4 years.<sup>4</sup> The progressive muscle degeneration leads to a loss of the ability to walk in the early teenage years, on average. Weakness of respiratory muscles ultimately leads to use of mechanical ventilatory support, and weakness in cardiac muscle involvement results in cardiomyopathy.

### Pfizer Rare Disease

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide,<sup>5</sup> representing an opportunity to apply our knowledge and expertise to help make a significant impact on addressing unmet medical needs.<sup>1</sup> 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, neuromuscular, and inherited metabolic disorders.<sup>1</sup>

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](http://www.pfizer.com). In addition, to learn more, please visit us on [www.pfizer.com](http://www.pfizer.com) and follow us on Twitter at @Pfizer and @Pfizer\_News, LinkedIn, YouTube and like us on Facebook at [Facebook.com/Pfizer](https://www.facebook.com/Pfizer).

DISCLOSURE NOTICE: The information contained in this release is as of August 30, 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 Pfizer's rare disease portfolio, 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 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 drug applications may be filed in any jurisdictions for any rare disease product candidates; whether and when regulatory authorities in any such jurisdictions 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 such product candidates will be commercially successful; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of any rare disease product candidates; 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](http://www.sec.gov) and [www.pfizer.com](http://www.pfizer.com).

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

2 National Institutes of Health. National Human Genome Research Institute. Learning About Duchenne Muscular Dystrophy. Available on <https://www.genome.gov/19518854/>. Accessed August 21, 2018.

3 Mendell JR, Shilling C, Leslie ND, Flanigan KM, al-Dahhak R, Gastier-Foster J, et al. Evidence-based path to newborn screening for Duchenne muscular dystrophy. *Annals of Neurology*. 2012;71(3):304-13.

4 NIH. Duchenne muscular dystrophy. <https://rarediseases.info.nih.gov/diseases/6291/duchenne-muscular-dystrophy>. Accessed August 21, 2018.

5 Pfizer Inc. Rare disease. <https://www.pfizer.com/health-wellness/disease-conditions/rare-diseases/areas-of-focus>. Accessed August 9, 2018.

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