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Pfizer Doses First Patient Using Investigational Mini-Dystrophin Gene Therapy for the Treatment of Duchenne Muscular Dystrophy

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Pfizer Inc. has initiated a Phase 1b clinical trial for its mini-dystrophin gene therapy candidate, PF-06939926, in boys with Duchenne muscular dystrophy (DMD). The first boy received an infusion of the mini-dystrophin gene on March 22nd, administered under the supervision of principal investigator, Edward Smith, MD, Associate Professor of Pediatrics and Neurology at Duke University Medical Center. Screening and enrollment of patients is expected to continue at up to four clinical research sites in the United States. Early data from this trial are expected in the first half of 2019, once all patients have been evaluated for one full year post-treatment.

“On behalf of the community of individuals and families living with Duchenne muscular dystrophy, we applaud the important step Pfizer has taken to advance a potentially transformational treatment option for boys stricken with this terrible disease,” said Debra Miller, CEO and Founder of Cure Duchenne. “The momentum we are seeing in the field of gene therapy emphasizes the maturing opportunity to advance the science. Today, there are very limited treatment options for our boys. Through collaboration and ongoing dialogue with companies like Pfizer, we hope to succeed in finding therapies that could dramatically change the outcomes for those with DMD.”

The multi-center, open-label, non-randomized, ascending dose study of a single intravenous infusion of PF-06939926 will enroll approximately 12 ambulatory boys aged 5 to 12 years with DMD. In addition to evaluating safety and tolerability, the study will evaluate measurements of dystrophin expression and distribution, as well as assessments of muscle strength, quality and function. As part of the screening process, potential candidates for treatment will be tested to confirm a negative result for antibodies against the adeno-associated virus serotype 9 (AAV9) capsid and for a T-cell (immune) response to dystrophin.

“Investment in this trial represents the culmination of years of research on behalf of patients by scientists at Pfizer and academic medical centers, along with the support of the DMD patient advocacy community, in the important quest to advance a program that could potentially change the trajectory of this debilitating disease,” said Greg LaRosa, PhD, Senior Vice President and Chief Scientific Officer of Pfizer’s Rare Disease Research Unit. “We’ve listened to the patient community and we know there is a dire need for treatment options; with this in mind, we have built on important scientific advances to design a therapy with the potential to deliver the mini-dystrophin gene to the body and address the underlying cause of DMD, regardless of mutation. This trial will assess the safety of this approach to gene therapy and could provide valuable data demonstrating its potential impact to slow down or stop the progression of DMD.”

Pfizer’s Advancement of Gene Therapy

This clinical trial is Pfizer's first recombinant AAV-based gene therapy program to enter the clinic stemming from Pfizer's 2016 acquisition of Bamboo Therapeutics. Pfizer also made a recent $100 million expansion of its Sanford, North Carolina gene therapy commercial-scale manufacturing facility.

Over the last several years, Pfizer has invested to create end-to-end capabilities to design novel AAV vectors and to build capacity to manufacture gene therapy products. Pfizer is collaborating with Sangamo Therapeutics to advance gene therapy programs with an ongoing Phase 1/2 trial in Hemophilia A, and a pre-clinical program in amyotrophic lateral sclerosis (ALS). Pfizer is also collaborating with Spark Therapeutics to advance a Hemophilia B gene therapy program currently in a Phase 1/2 clinical trial.

About the Investigational Gene Therapy, PF-06939926

DMD is caused by an absence of dystrophin, a protein that helps keep muscle cells intact. In the absence of dystrophin, muscle cells deteriorate. PF-06939926 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. The AAV9 capsid was chosen as the delivery mechanism because of its potential to target muscle tissue.

PF-06939926 was granted Orphan Drug and Pediatric Rare Disease Designations by the FDA and Orphan Medical Product Designation by the European Medicines Agency in May 2017.

More information about the current clinical trial can be found at www.clinicaltrials.gov (NCT03362502) and by contacting the Pfizer ClinicalTrials.Gov call center at 1-800-718-1021 or via email at ClinicalTrials.gov_Inquiries@pfizer.com.

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a serious genetic disease characterized by progressive muscle degeneration and weakness. Symptoms usually manifest in early childhood between the ages of 3 and 5. The disease primarily affects boys. 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. By the early teens, patients typically lose their ability to walk and the heart and respiratory muscles are also affected, ultimately resulting in premature death. DMD is the most common form of muscular dystrophy worldwide with incidence of 1 in every 3500 to 5000 live male births.1 2

Pfizer and Rare Disease

Rare disease includes some of the most serious of all illnesses and impacts millions of patients worldwide,3 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, neuromuscular, and inherited metabolic disorders.

In addition, Pfizer has remained committed to DMD with its other ongoing clinical program, involving domagrozumab, an investigational, infused, anti-myostatin monoclonal antibody currently in a Phase 2 trial.

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 @Pfizer_News, LinkedIn, Instagram, YouTube and like us on Facebook at Facebook.com/Pfizer.

DISCLOSURE NOTICE: The information contained in this release is as of April 12, 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 PF-06939926, a mini-dystrophin gene therapy candidate, for the potential treatment of Duchenne muscular dystrophy and the potential of gene therapy, 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 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; 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 applications may be filed with regulatory authorities for any potential gene therapies; whether and when regulatory authorities may approve any such applications, 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, and, if approved, whether any such gene therapies will be commercially successful; decisions by regulatory authorities regarding labeling and other matters that could affect the availability or commercial potential of any such gene therapies; 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.

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1 National Institutes of Health. National Human Genome Research Institute. Learning About Duchenne Muscular Dystrophy Available on https://www.genome.gov/19518854/ Accessed March 29, 2018.
2 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. [PubMed]
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Rare Disease: Facts and Statistics. http://globalgenes.org/rare-diseases-facts-statistics. Accessed March 28, 2018.

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