PHASE 2 STUDY IN BOYS 6 - <10 YEARS OF AGE WITH DUCHENNE MUSCULAR DYSTROPHY

(ClinicalTrials.gov Identifier: NCT02310763)

What is the study?

A multi-center clinical study evaluating the safety & tolerability, efficacy, pharmacokinetics (what the body does to a drug) and pharmacodynamics (what a drug does to a body) of the new investigational compound, PF-06252616, in approximately 105 boys diagnosed with Duchenne Muscular Dystrophy (DMD) who are able to walk and climb stairs. This study is designed to support future potential regulatory filings.

What is PF-06252616?

PF-06252616 is an experimental anti-myostatin monoclonal antibody. Myostatin acts in the body to help regulate muscle growth by inhibiting (blocking) muscle differentiation and growth. Blocking the activity of myostatin may have therapeutic application in treating muscle wasting diseases, such as DMD. Based on the proposed mechanism of action of PF-06252616, there is the potential to increase muscle mass and function in boys with DMD who have evidence of reduced muscle mass. Preclinical evidence of increased muscle size and function has been demonstrated in mice and nonhuman primates. The Phase 1 study of PF-06252616 in healthy adults is in the process of being reported.

Who will be considered eligible for the study?

Boys who are diagnosed with DMD *and* who are age 6 years to <10 years *and* who are able to walk. The diagnosis of DMD must be confirmed in the subject's medical history *and* by genetic testing obtained during routine medical care. Genetic testing is not performed as part of this study. Eligible participants must be on glucocorticosteroids for a minimum of 6 months prior to signing informed consent to join the study. Eligible participants must also able to perform the 4 stair climb in $\geq$ 0.33 stairs/second < 1.6 stairs/second. Additional screening evaluations will be conducted at the study site to confirm eligibility.

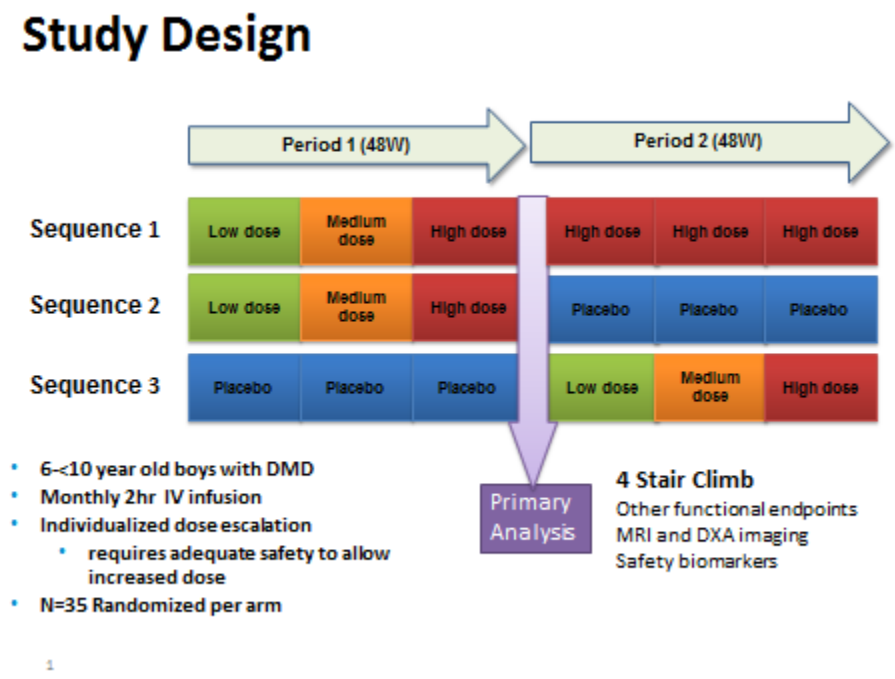
What is the design of the study and will there be a placebo arm?

The study will have two periods and each period will run for 48 weeks. At the beginning of the study, boys will be randomized to one of three groups. The first group will receive study drug in period 1 and remain on study drug in period 2. The second group will also receive study drug in period 1 but in period 2, they will switch to placebo. The third group will receive placebo in period 1 and then study drug in period 2. The study will be blinded so the participants, their families and investigators won't

know which group they are enrolled in. The primary analysis for the study will be based on the data collected from period 1. This design will allow the investigators to see both the effect of starting and stopping the drug as well as constant exposure over a long period of time to the same dose. It's likely that the drug will actually take 2/3 of the second period of the trial to wash out in the group that is transitioned to placebo so participants in that group may still get some drug exposure during the placebo period of the study.

The drug will be given as a 2 hour IV infusion every 4 weeks. Within each subject, the dose level may increase every 16 weeks depending on how well tolerated and safe it is. If any safety issues are observed at a given dose level, the dose may either not increase, decrease or dosing may be terminated.

As well as monthly clinic visits, some of which will require attendance on two consecutive days, the study involves a number of MRI scans, and frequent testing of strength, walking and stair climbing ability. All reasonable travel and accommodations will be provided or costs reimbursed.



What happens when the study ends?

If safety and efficacy are demonstrated in this Phase 2 study, subjects who complete this study may be invited to participate in an open-label study.

Where is the study being conducted?

Confirmed study sites include Cincinnati (OH) Children's Hospital Medical Center (Dr. Brenda Wong) and Kennedy Krieger Institute Center for Genetic Muscle Disorders at Johns Hopkins in Baltimore, MD (Dr.

Kathryn Wagner). Additional centers are also expected to become active sites participating in the study: US (MO, IA, CA, WA, MN, CO, TX, NC, UT, MA and FL), Canada, Europe and Japan, with the goal of opening all these sites by April 2015.

Potential subjects will not be prioritized according to their proximity to a study center. It is recognized that rolling enrollment presents a challenge to the community, but in an effort to preserve the consistency and integrity of the clinical study data, transfer among sites will not be allowed.

Where can I go for further information about the study?

The most up-to-date information will be posted on ClinicalTrials.gov, with an opportunity to contact Pfizer for additional information. Please note that you will need the ClinicalTrails.gov identification number for this study when you request information about this study. More information will become available as the study advances.