CLINICAL TRIAL PROTOCOL

Label
A Phase 2b Efficacy and Safety Study of PTC124 in Subjects with Nonsense Mutation Mediated Duchenne and Becker Muscular Dystrophy

Name of the disease(s), group(s) of diseases concerned by the clinical trial
Duchenne and Becker Muscular Dystrophy

Name of drug(s) or medical product(s) concerned by the clinical trial
PTC124 Ataluren

Start date of the trial
February 2008

End date of the trial
August 2010

Phase number of the trial
Phase II

This study is a Phase 2b, multicenter, randomized, double-blind, placebo-controlled, doseranging, efficacy and safety study, designed to document the clinical benefit of PTC124 when administered as therapy of patients with DMD/BMD due to a nonsense mutation (premature stop codon) in the dystrophin gene. It is planned that ~165 boys who are at least 5 years of age and can walk at least 75 meters (80 yards) will be enrolled. Study subjects will be enrolled at sites in North America, Europe, Israel, and Australia. They will be randomized in a 1:1:1 ratio to either a higher dose of PTC124, a lower dose of PTC124, or placebo. Subjects will receive study drug 3 times per day (at breakfast, lunch, and dinner) for 48 weeks. Subjects will be evaluated at clinic visits every 6 weeks. Additional safety laboratory testing, which may be performed at the investigational site or at an accredited local laboratory or clinic, is required every 3 weeks for the first 24 weeks of the study. At the completion of blinded treatment, all compliant participants will be eligible to receive open-label PTC124 in a separate extension study.