

A Phase 1b/2 study of the anti-myostatin adnectin RG6206 (BMS-986089) in ambulatory boys with Duchenne muscular dystrophy: A 72-week treatment update

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Myostatin is produced by muscle cells to limit muscle growth. Studies have shown that blocking myostatin can lead to an increase in muscle size. RG6206 (BMS-986089) is an investigational agent that inhibits myostatin activity and is currently under investigation as a treatment for Duchenne muscular dystrophy (DMD).

This Phase 1b/2 study (NCT02515669) tested the safety, tolerability and pharmacokinetics (how a drug is metabolized by the body) of RG6206 in boys with DMD. It also assessed if RG6206 had an effect on the levels of myostatin in blood and if treatment had effects on lean body mass (LBM). Forty-three boys with DMD, able to walk and aged 5–10 years, were randomly assigned to receive either weekly subcutaneous injections (under the skin) of RG6206 or placebo (treatment-placebo ratio 3:1) during a 24-week double-blind phase of the study, when neither the participants nor the investigators knew who received treatment with RG6206. Afterwards, all participants received open-label RG6206 for 48 weeks, when both the participants and the investigators knew who received treatment with RG6206.

No drug-related safety findings leading to any boy leaving the study were observed. During this study, treatment with RG6206 resulted in a decrease in the levels of free myostatin in serum from blood.

We will report on the effect of treatment with RG6206 on LBM and LBM index in boys with DMD who received treatment for 72 weeks compared with boys with DMD in the cTAP who had not received any treatment.

Forty-one boys with DMD are now enrolled in a 228-week study extension. A Phase 2/3 study is recruiting (NCT03039686).

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