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## A Phase 1b/2 study of RG6206 (BMS-986089) in boys with Duchenne muscular dystrophy: 72-week update

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### **Purpose:**

To assess safety, tolerability, PK and PD of anti-myostatin adnectin RG6206 (BMS-986089) in a Phase 1b/2 study (NCT02515669) in ambulatory boys with Duchenne muscular dystrophy (DMD).

### **Methods:**

During a 24-week double-blind phase, 43 boys with DMD, aged 5–10 years, were randomized to receive weekly subcutaneous injections of RG6206 (4–50 mg) or placebo (3:1 respectively). All participants then received RG6206 during a 48-week open-label phase.

The primary endpoint was safety and tolerability over 24 weeks. Secondary endpoints included; PK, anti-drug antibody levels and serum myostatin levels. Dual energy X-ray absorptiometry imaging measured lean body mass (LBM). Informed consent was obtained from patients and their families, and approval was obtained from relevant ethics committees and institutional review boards.

### **Results:**

The most common AEs were mild-to-moderate injection site reactions that resolved without change to treatment. No clinically significant changes in lab values or vital signs were observed.

We will report on change in LBM in boys who received RG6206 for 72 weeks and in patients with DMD from the Cincinnati Children's Hospital (analyses conducted by the Collaborative Trajectory Analysis Project) who had not received treatment.

### **Conclusions:**

No drug-related safety findings leading to withdrawal were identified. In total 41 boys are enrolled in a 228-week open-label extension. A Phase 2/3 study is recruiting (NCT03039686).

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**Açıklama [CO(1)]:** Note to authors:

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