Bristol-Myers Squibb / Roche

Duchenne Muscular Dystrophy

Anti-Myostatin Adnectin Program

[BMS-986089 / RO7239361 (RG6206)]

Parent Project Muscular Dystrophy's 2017 Connect Conference

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Disclosure and Disclaimer

- Cliff Bechtold (M.S.) is a full-time employee of Bristol-Myers Squibb, manufacturer of BMS-986089 / RO7239361 (RG6206).
- BMS-986089 / RO7239361 (RG6206) is currently in development as a treatment for Duchenne Muscular Dystrophy; it is not approved for sale in any country.

What is BMS-986089 / RO7239361 (RG6206)?

- BMS-986089 / RO7239361 (RG6206) is an investigational protein that is designed to bind to myostatin.
- Myostatin is a protein produced primarily in skeletal muscle cells that prevents muscle cell growth and differentiation.
- Animals lacking myostatin or animals treated with substances that block the activity of myostatin have larger and stronger muscles.

Phase 1 Study Results

- In a Phase 1 study using single and multiple ascending (increasing) doses in normal healthy adults, weekly subcutaneous (under the skin) doses of up to 180 mg BMS-986089 / RO7239361 (RG6206) were safe and well tolerated.
- The most common adverse effects were mild injection site erythema (redness of the skin), rash and injection site reaction.
- Increases in thigh muscle volume and total lean body mass in healthy adults were observed following multiple dosing.
- The extent and duration of free myostatin reduction increased with dose.
- These data support further study of BMS-986089 / RO7239361 (RG6206).

Ongoing Research Study - CN001-006

- An ongoing research study, CN001-006, is evaluating the safety, tolerability and pharmacokinetics of multiple dose ranging subcutaneous doses of BMS-986089 / RO7239361 (RG6206) in ambulatory boys with Duchenne Muscular Dystrophy. This study is taking place in the US and in Canada and is now closed to enrollment.

CN001-016 – New Study - Phase 2/3

- CN001-016 is a global randomized (participants are assigned at random, by chance alone), double-blind (where neither the participants nor the study team know who is getting the study drug versus placebo), placebo-controlled study to assess the effectiveness, safety, and tolerability of BMS-986089 / RO7239361 (RG6206) in ambulatory boys with Duchenne Muscular Dystrophy.
- CN001-016 has **now started to enroll in the US**. There are multiple study sites open and/or planned in the following states: CA, FL, GA, IL, KS, MD, MO, NY, OH, PA, Washington DC, and Canada (locations to be confirmed).
- Global recruitment will begin shortly.

CN001-016 - Duration and Inclusion Criteria

- The duration of the double-blind period of the study, where a participant may receive the investigation drug or placebo (2:1 ratio, twice as many participants will receive the investigation drug compared to those that receive placebo) is planned for 48 weeks.
- The double-blind period will be followed by an open-label period where active study drug is given to all participants for 48 weeks.
- The study will plan to enroll ambulatory boys 6 to 11 years of age inclusive. Key inclusion criteria include the ability to climb 4 stairs in 8 seconds or less at screening and boys must be receiving corticosteroids for at least 6 months.
- Note that all **DMD mutations are eligible** however full eligibility requirements for this study would be assessed at a participating study site.
- Eligible participants may receive, at no cost, transportation services with a travel concierge service to cover the costs of study-required travel.
- For more information visit **BMStrialDMD.com** or visit clinicaltrials.gov (NCT03039686).