Dear Duchenne Community,

We are pleased to share interim clinical data from our Phase I/II AFFINITY DUCHENNE™ study of RGX-202, our investigational gene therapy for the treatment of Duchenne muscular dystrophy (Duchenne). REGENXBIO shared interim clinical data at the World Muscle Society Annual Conference, October 3-7, 2023, in Charleston, South Carolina. Please find the news release here and a summary below.

Data are from **three patients dosed with RGX-202 at Dose Level 1**.

SAFTEY DATA

- As of September 28, 2023 (the data cut date), RGX-202 was reported to be well tolerated with no serious adverse events among the three patients.
- At the time of the data cut date, the follow-up time ranged from 3 weeks to more than five months after gene therapy administration.
- Two patients who have reached or surpassed the three-month follow-up have completed the immunosuppression regimen per study protocol.

BIOMARKER DATA

- Initial biomarker data from two boys who completed 3-month study assessments showed readily detectable RGX-202 microdystrophin levels from bicep muscle biopsies taken at 3 months after RGX-202 administration.
- A reduction from baseline in serum creatinine kinase (CK) levels was also observed at ten weeks. Elevated CK levels are associated with muscle injury and are uniformly elevated in boys with Duchenne.

PROGRAM NEXT STEPS

- REGENXBIO expects to dose boys at the next dose level (Dose Level 2) in the AFFINITY DUCHENNE study by the end of 2023.
- In 2024, the company expects to share initial strength and functional assessment data for both Dose Levels 1 and 2 in the AFFINITY DUCHENNE study, and to make a pivotal dose determination and initiate a pivotal program for RGX-202.

ABOUT AFFINITY DUCHENNE

The Phase I/II AFFINITY DUCHENNE trial is a multicenter, open-label dose escalation and dose expansion clinical study to evaluate the safety, tolerability, and clinical efficacy of a one-time intravenous (IV) dose of RGX-202 in boys with Duchenne. RGX-202 uses the NAV® AAV8 vector to deliver a transgene for a novel microdystrophin that includes the functional elements of the C-terminal (CT) domain as well as a muscle-specific promoter. The trial continues to recruit, and you can find more information, including eligibility, on clinicaltrials.gov: **AFFINITY DUCHENNE (NCT05693142).**

We are looking forward to sharing information with the Duchenne community in a **webinar hosted by Parent Project Muscular Dystrophy (PPMD) on October 11, 2023, at 1pm ET.**

Warm regards,

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