## Dear Duchenne Community,

We are excited to share updates about RGX-202, our investigational gene therapy for the treatment of Duchenne muscular dystrophy (Duchenne) and our trials: the AFFINITY DUCHENNE™ Phase I/II trial of RGX-202 and AFFINITY BEYOND™, our antibody assessment study in boys with Duchenne.

### AFFINITY DUCHENNE

On January 23, 2023, REGENXBIO announced that AFFINITY DUCHENNE, our Phase I/II first-in-human trial of RGX-202, has been initiated in the U.S. and is recruiting participants for the study. You can now find more information about the trial on clinicaltrials.gov: **AFFINITY DUCHENNE (NCT05693142)**

The AFFINITY DUCHENNE trial is an open-label, multi-center clinical trial designed to evaluate the safety, tolerability, and clinical efficacy of a one-time intravenous dose of RGX-202 in boys with Duchenne. RGX-202 uses REGENXBIO's proprietary NAV® AAV8 vector to deliver a transgene for a novel microdystrophin, a shortened and functional version of dystrophin protein.

**The trial may enroll up to 18 participants who meet the following key eligibility criteria:**

- Boys aged 4 to 11 years
- *DMD* gene mutations in exons 18 and above
- Have a clinical picture consistent with typical DMD
- Ambulatory and able to walk 100 meters independently without assistive devices, as assessed at screening
- Able to complete the time to stand (TTS) per protocol-specific criteria
- Participant has been on a stable dose of systemic glucocorticoids, according to standard of care, for at least 12 weeks prior to screening
- No pre-existing antibodies to the gene therapy (AAV8 capsid)
- Has not received any investigational or commercial gene therapy product over his lifetime

### AFFINITY BEYOND

The AFFINITY BEYOND observational study is non-interventional and consists of a phone/video interview and a home health visit with bloodwork. Information collected in this study may be used to identify potential participants for DMD investigational gene therapy clinical trials. The objective of this study is to understand the prevalence of anti-AAV8 and anti-AAV9 antibodies in boys with Duchenne and to help identify participants who may be eligible for REGENXBIO's investigational gene therapy clinical trials. You can find more information about the trial on clinicaltrials.gov: **AFFINITY BEYOND (NCT05683379)**

**The study may enroll up to 200 US participants. Key eligibility criteria includes:**

- Boys aged 0 to 11 years
- Diagnosis of Duchenne muscular dystrophy
- Has not received any investigational or commercial gene therapy product over his lifetime

We look forward to hosting a community webinar in the future to provide additional information on AFFINITY DUCHENNE and AFFINITY BEYOND.

**Wishing you and your families a happy and healthy New Year,**

**Warm regards,**

**Duchenne team at REGENXBIO**
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