Dear Duchenne Community,

We are writing to provide an update on RGX-202*, REGENXBIO's investigational gene therapy for the treatment of Duchenne muscular dystrophy (Duchenne).

REGENXBIO issued a press release on June 5th sharing new positive interim data from the Phase 1/2 portion of the AFFINITY DUCHENNE® study of RGX-202. AFFINITY DUCHENNE is a Phase 1/2/3 study currently enrolling patients. New data include safety, biomarker, and functional (outcomes) interim results. (REGENXBIO RGX-202 press release - June 5, 2025)

RGX-202 Phase 1/2 AFFINITY DUCHENNE Data Update – as of May 7, 2025

Safety

- RGX-202 was well tolerated in 13 study participants across both dose levels with no serious adverse events (SAEs) and no AEs of special interest (AESI) reported. Common drug-related AEs included nausea, vomiting and fatigue, all typically anticipated with administration of a gene therapy.

Function

- Interim functional results were reported for study patients approximately 6 to 12 years of age at dosing who received dose level 2 (our pivotal dose) of RGX-202 and reached their 9-month (n=5) or 12-month (n=4 of the 5) assessments.
- RGX-202 continues to demonstrate evidence of positively impacting the course of Duchenne.
- Dose level 2 patients demonstrated improved performance at 9 and 12 months on the North Star Ambulatory Assessment (NSAA) and timed function tests (Time to Stand, 10 Meter Walk-run, Time to Climb), exceeding available external natural history controls that were matched for both age and baseline function.

Biomarker

- Biomarker data from the Phase 1/2 study continues to support consistent, robust expression and transduction of RGX-202 microdystrophin.

Program Status

The AFFINITY DUCHENNE trial is enrolling eligible participants in the Phase 3 (pivotal) portion of the trial with study centers in the U.S. and Canada. The trial is expected to enroll approximately 30 patients to support an anticipated U.S. Biologics License Application (BLA) submission using the FDA's accelerated approval pathway in mid-2026. After the last participant is dosed, enrollment into the confirmatory study is expected to be initiated.

For more information

To learn more about RGX-202 and the AFFINITY DUCHENNE trial, visit https://regenxbiodmdtrials.com/. You may also visit clinicaltrials.gov https://clinicaltrials.gov/study/NCT05693142.

We appreciate the patients, families, clinicians and advocacy organizations who have supported our program and look forward to sharing additional updates.

Warm regards,

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*RGX-202 is investigational and not approved for use by any regulatory agency.

This letter contains forward-looking statements, which are subject to risks and uncertainties. Actual outcomes may differ. Please refer to our SEC filings for more information.