March 11, 2026

Dear Duchenne Community,

We are pleased to share updates on the Phase I/II AFFINITY DUCHENNE® trial of RGX-202*, REGENXBIO's investigational gene therapy for Duchenne muscular dystrophy.

REGENXBIO recently shared new positive interim data from the Phase I/II AFFINITY DUCHENNE® trial of RGX-202. At the Muscular Dystrophy Association (MDA) scientific conference this week, Dr. Carolina Tesi-Rocha, an investigator in the AFFINITY DUCHENNE trial from Stanford Children's Hospital presented new safety, functional and biomarker data, as well as cardiac MRI measures.

You can read more about this update in our press release.

Data Summary - Phase I/II AFFINITY DUCHENNE® Trial of RGX-202 (data cut: January 5, 2026)

Safety

- RGX-202 continued to be well tolerated, with no serious adverse events (SAEs) and no adverse events of special interest (AESIs) in the Phase I/II study.
    - There was no evidence of liver injury across multiple measures (including GGT, total bilirubin) in the pivotal dose Phase I/II patients as of the data cut date.
    - A mean reduction in creatine kinase (CK) was seen in the phase I/II pivotal dose patients at one year following gene therapy treatment, and supported by mean reductions in ALT, AST, and LDH.
- The AFFINITY DUCHENNE trial includes a comprehensive, short-course immune suppression protocol to manage potential side effects.

Functional Data

- The data update included functional measures from seven study participants that received RGX-202 at the same dose (the pivotal dose) being evaluated in the Phase III trial of RGX-202.
- RGX-202 continued to show positive impacts on the trajectory of disease at one year.
    - These pivotal dose participants demonstrated improved performance on North Star Ambulatory Assessment (NSAA) and timed function tests (Time to Stand, 10 Meter Walk-run, Time to Climb), exceeding expected disease trajectory and external controls. Notably, five of the seven pivotal dose participants included in this update were at least 8 years old at dosing, an age when functional decline is expected.

Cardiac Function

- Pivotal dose participants showed cardiac stability one year after treatment, as measured by cardiac MRI.

Biomarker Data

- Biomarker data from the Phase I/II study continues to support consistent, high expression and transduction of RGX-202 microdystrophin.

- *RGX-202 is investigational and is not approved for use by any regulatory agency. Its safety and efficacy have not been established and continues to be evaluated.

REGENXBIO continues to enroll approximately 30 participants aged 1 and older in the confirmatory portion of the AFFINITY DUCHENNE Phase III study. To learn more, visit regenxbiodmdtrials.com or scan the QR code below.

We thank the patients, their families and clinicians who are participating in our clinical program. Your participation helps guide our work and advance research for Duchenne. If you have questions, you may email us any time at duchenne@regenxbio.com.

With warm regards from the Team at REGENXBIO,

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