May 14, 2026

Dear Duchenne Community,

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

This morning, REGENXBIO shared positive topline and interim functional and safety data from the pivotal Phase III portion of the trial. You can read more about this update in our press release.

RGX-202 is designed to deliver a novel microdystrophin protein that includes the C-Terminal (CT) domain found in naturally occurring dystrophin. The pivotal Phase III portion of the AFFINITY DUCHENNE study evaluated RGX-202 in 31 participants.

The trial met its primary endpoint, with 93% of participants achieving greater than 10% microdystrophin expression at Week 12. In 9 participants who reached one year post-dosing, functional improvement (changes from baseline) were demonstrated on the NSAA and timed function tests. RGX-202 was also well tolerated to date.

Additionally, we were encouraged to see that among the 9 participants with one year of follow-up, microdystrophin expression levels were statistically significantly correlated with NSAA change from baseline.

These AFFINITY DUCHENNE data support our plans to file a Biologics License Application in the U.S. using the FDA's accelerated approval pathway in 2027.

To learn more about our work, visit **regenxbiodmdtrials.com** or scan the QR code below.

We extend our sincere thanks to the patients, families and investigators who are participating in our clinical trial. Your participation continues to guide our work and help advance research for Duchenne. If you have questions, please email us at **duchenne@regenxbio.com.**

Sending our best wishes on behalf of the Team at REGENXBIO,

Naz Dastgir, DO
Executive Director, Clinical Development

Vivian Fernandez
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*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.