June 24, 2026

Dear Duchenne Community,

We are writing to share an important update on RGX-202[1], REGENXBIO's investigational gene therapy for Duchenne muscular dystrophy. This morning, we announced that we dosed the last participant in the confirmatory study of RGX-202. Data from the pivotal and confirmatory portions of the AFFINITY DUCHENNE® trial will support the Biologics License Application (BLA). This update completes the registration and development process needed to begin preparing for RGX-202 BLA submission.

REGENXBIO is committed to bringing RGX-202 to patients as quickly as possible, and we plan to begin a BLA submission in the third quarter this year with the goal of seeking FDA approval under the accelerated approval pathway in the second half of 2027.

You can read more about this update in our press release

In May, REGENXBIO shared positive topline data from the pivotal portion of the AFFINITY DUCHENNE trial[2], which met several of the requirements for accelerated approval, including:

- Met primary endpoint - microdystrophin expression exceeded the predefined threshold in 93% of patients at Week 12
- Demonstrated statistically significant correlation between microdystrophin expression at Week 12 (n=30) and functional improvement at one year (NSAA, n=9)
- RGX-202 was well tolerated in the pivotal trial (n=31)

We are working to expand the RGX-202 clinical program globally and will share more with the community as we advance those plans.

We extend our sincere thanks to the patients, families and investigators who participate in our clinical trials. Your participation helps 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
Executive Director, PatientAdvocacy

*About the Biologics License Application (BLA)**: A BLA is the formal request submitted to the FDA by a company seeking approval of a new therapy.*

*About the FDA's Accelerated Approval (AA) pathway**: As the DMD community may be aware, the FDA's accelerated approval pathway is designed to help bring potentially important new therapies for serious conditions to patients sooner. Because it can take many years to fully understand a treatment's impact on how patients feel, function, or survive, the pathway allows the FDA to consider surrogate or intermediate clinical endpoints that are reasonably likely to predict clinical benefit. This approach can provide earlier access to potentially life-changing therapies while additional studies are conducted to confirm the expected benefit.*

A confirmatory study is designed to verify the predicted clinical benefit seen in previous studies and may be required by the FDA for gene therapies as part of the accelerated approval pathway.

[1] 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.
[2] Results based on data available as of April 16, 2026.