October 30, 2025

**Dear Duchenne Community,**

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

REGENXBIO recently announced the completion of enrollment in the pivotal (Phase III) portion of the AFFINITY DUCHENNE® study of RGX-202*. **While the pivotal (Phase III) study is completely enrolled, REGENXBIO continues to enroll ambulatory participants aged 1 and above in the confirmatory portion of the study.**

For more information on our announcement, here is a link to the press release:

[REGENXBIO RGX-202 press release October 30, 2025]

For more information on the AFFINITY DUCHENNE® study, talk with your doctor or visit any of these resources:

- Visit the study on clinicaltrials.gov
- Visit AFFINITY DUCHENNE® study page www.regenxbiodmdtrials.com
- Reach out to a patient navigator to help verify whether your child may be eligible at https://mytomorrows.com/trials/affinity-duchenne/en-us/

REGENXBIO has full supply of RGX-202 ready and available for the confirmatory study. RGX-202 is manufactured in-house at our headquarters in Rockville, MD, with leading product purity levels in Duchenne gene therapy. Our Manufacturing Innovation Center allows us to closely manage product quality and move faster to meet the needs of patients and families.

Data from the AFFINITY DUCHENNE study will support a Biologics License Application (BLA) filing in the U.S. using the FDA's **accelerated approval pathway**. REGENXBIO is planning to submit a BLA in mid-2026.

The **FDA's accelerated approval pathway** helps make promising new treatments for serious conditions available sooner. Normally, it can take many years to fully study a new drug and understand how it helps people feel, function, or live longer. With accelerated approval, the FDA can approve therapy based on early evidence that suggests it's likely to have real benefits for patients. This pathway helps speed access to potential new treatments while studies continue to confirm their long-term effects.

We would like to 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.

**Sending our best to you and your families,**
**The REGENXBIO Team**

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