March 24, 2025

Dear Duchenne Community,

We are pleased to share updates on the Phase 1/2/3 AFFINITY DUCHENNE® trial of RGX-202, REGENXBIO's investigational gene therapy for the treatment of Duchenne muscular dystrophy.

The Phase 3 (pivotal) portion of this trial is open and enrolling ambulatory boys aged 1 and above with a confirmed diagnosis of Duchenne. To learn more, visit **regenxbiodmdtrials.com** or scan the QR code below**.**

New Data from the Phase 1/2/3 AFFINITY DUCHENNE® trial

At the MDA annual scientific conference held last week, Dr. Carolina Tesi-Rocha from Stanford Children's Hospital and an investigator in the AFFINITY DUCHENNE® trial presented new biomarker data from the Phase 1/2 portion of the trial. This included the first microdystrophin data from our cohort for younger patients, aged 1-3.

- RGX-202 has been well tolerated in 12 patients across both dose levels, with no serious adverse events or adverse events of special interest, and no myocarditis or myositis reported[1, 2].
    - The most common drug-related AEs reported are nausea, vomiting and fatigue, all resolved
    - The AFFINITY DUCHENNE trial includes a comprehensive, short-course immune suppression regimen to help mitigate the potential of adverse events.

- New biomarker data continues to support consistent robust expression, transduction and appropriate localization at the sarcolemma (muscle cell membrane) of the RGX-202 microdystrophin across all ages.
    - In a patient aged 3 years old, RGX-202 microdystrophin expression was measured at 12 weeks to be 122.3% compared to normal control.
    - In a patient aged 7 years old, RGX-202 microdystrophin expression was measured at 12 weeks to be 31.5% compared to normal control.

- As we previously shared in November 2024, the first five boys dosed in the Phase 1/2 portion of the AFFINITY DUCHENNE trial showed functional improvements that exceeded available external natural history controls measured at 9 months (dose level 2) and 12 months (dose level 1).

[1] *Data cut-off February 21, 2025*

[2] *AEs of special interest include central or peripheral neurotoxicity, drug-induced liver injury and thrombocytopenia*

Learn more in our press release - **REGENXBIO RGX-202 biomarker press release 19Mar2025**

We plan to share additional Phase 1/2 data, including additional functional data, in the first half of this year. We plan to submit a Biologics License Application (BLA) under the U.S. FDA's accelerated approval pathway in mid-2026.

Clinical Trial Website

Please visit our clinical trial website for AFFINITY DUCHENNE - created as a resource for families, patients and healthcare providers. To access, visit: **regenxbiodmdtrials.com** or scan the QR code below**.**

The website provides information on RGX-202 and the AFFINITY DUCHENNE trial, including key eligibility criteria, what to expect from participation, and study locations. Information on the AFFINITY BEYOND antibody testing program is also available.

We thank the Duchenne community, including trial participants and their families, for their continued support as we work to advance RGX-202 as a potential new gene therapy for Duchenne. Our team remains committed to helping the community, and we are pleased to share these updates with you. If you have questions, please contact the patient advocacy team at **duchenne@regenxbio.com**

With warm regards from the Team at REGENXBIO,

Steve Pakola, MD
Chief Medical Officer

Vivian Fernandez
Executive Director, Patient Advocacy


AFFINITY DUCHENNE
RGX-202: An Investigational, One-Time Gene Therapy Treatment for Duchenne Muscular Dystrophy
AFFINITY DUCHENNE® Study
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