Dear Duchenne Community, **18Nov2024**

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

REGENXBIO issued a press release on November 18th announcing that the pivotal phase of the AFFINITY DUCHENNE® study of RGX-202 is enrolling and the first patient has been dosed.

Key eligibility criteria include:

- Ambulatory boys aged 1 and above and weighing 10 kg or greater at screening
- Genetically confirmed DMD (mutations in exons 18 and above)
- No pre-existing antibodies to the gene therapy (AAV8 capsid)

REGENXBIO also shared positive safety and efficacy data from the Phase I/II portion of the study, including functional (outcomes) results.

- Functional data were reported for three patients in dose level 1 (12 months after dosing) and two patients in dose level 2 / pivotal dose (9 months after dosing). Improvements in function were demonstrated at both dose levels on the North Start Ambulatory Assessment (NSAA) and timed function tests.
- Participants who received RGX-202 exceeded available natural history controls and established benchmarks for clinical outcomes.
- New biomarker data confirm consistent, high expression and transduction of RGX-202 microdystrophin in the muscle and localized to the sarcolemma (muscle cell membrane), which means RGX-202 is getting to the right place.
- As of November 1st, RGX-202 was well tolerated with no serious adverse events (SAEs) or AEs of special interest (AESIs) reported. Common drug-related AEs included nausea, vomiting and fatigue. All resolved and are typically anticipated with gene therapy administration.

REGENXBIO has reached alignment with the U.S. Food and Drug Administration (FDA) on this pivotal program and expects to file a Biologics Licensing Agreement (BLA) to the FDA in 2026 using the accelerated approval pathway.

AFFINITY DUCHENNE is a multi-center, open label research study of RGX-202. For more information on the study, including participating sites, visit clinicaltrials.gov - AFFINITY DUCHENNE NCT05693142

RGX-202 is investigational and not approved for use by any regulatory agency. RGX-202 is a potential, one-time gene therapy for the treatment of Duchenne that includes an optimized transgene for a novel microdystrophin. REGENXBIO's differentiated RGX-202 microdystrophin includes the C-Terminal (CT) domain found in naturally occurring dystrophin. In preclinical studies, the CT domain has been shown to protect the muscle from contraction-induced stress and improve its ability to repair itself, which could lead to improved durability.

We are pleased to be moving into the pivotal stage of this program based on the strength of our efficacy, biomarker and safety data from the Phase I/II portion of the trial, and the need for new treatments for patients with Duchenne.

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

REGENXBIO RGX-202 press release November 18 2024

We are grateful to the patient community for their ongoing support for this program. We will continue to provide updates on this program through the patient advocacy groups. We would like to thank the patients, their families and clinicians who have taken part in our clinical trial. Your participation and dedication help guide our work and advance important research for Duchenne.

If you have questions, you may email us any time at duchenne@regenxbio.com.

Wishing you and your family peace during the holidays,

The REGENXBIO Team