Dear Duchenne Community,

We would like 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 Tuesday, March 5th announcing additional interim safety and biomarker data in the Phase I/II AFFINITY DUCHENNE® trial of RGX-202 in Duchenne patients ages 4 to 11 years old. This update included positive initial data from the first patient dosed at dose level 2, who was 12 years old at the time of dosing.

Results were presented at the 2024 MDA Clinical & Scientific Conference by Aravindhan Veerapandiyan, M.D., trial investigator at Arkansas Children's Hospital.

RGX-202 DATA SUMMARY

- RGX-202 remains well tolerated in 5 patients at both dose levels, with no serious adverse events
- Robust RGX-202 microdystrophin expression was demonstrated at both dose levels
- Notably, the first patient dosed at dose level 2 showed 75.7% microdystrophin expression vs normal control at 3 months after dosing

RGX-202 is investigational and is not approved for use by any regulatory agency. RGX-202 is a potential, one-time AAV gene therapy for the treatment of Duchenne that includes an optimized transgene for a novel microdystrophin. REGENXBIO's microdystrophin retains the functional elements of the C-Terminal (CT) domain found in naturally occurring dystrophin. Presence of the CT domain has been shown in preclinical studies to recruit several key proteins to the muscle cell membrane, leading to improved muscle resistance to contraction-induced muscle damage in dystrophic mice.

REGENXBIO is on track to initiate a pivotal trial in the second half of 2024. The Company plans to use RGX-202 microdystrophin expression as a surrogate endpoint to support a Biologics License Application (BLA) filing using the FDA's accelerated approval pathway.

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

REGENXBIO RGX-202 press release 5 March 2024

We would like to thank the patients, their families and clinicians who have participated in our clinical trials. 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