REGENXBIO

AFFINITY DUCHENNE® Trial of RGX-202
Interim Data Update

March 5, 2024

Forward-looking Statements

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Agenda

- **Welcome**
- **AFFINITY DUCHENNE® Phase I/II trial of RGX-202 for the treatment of Duchenne**
  - New interim results
  - Clinic and caregiver videos
- **Q&A**

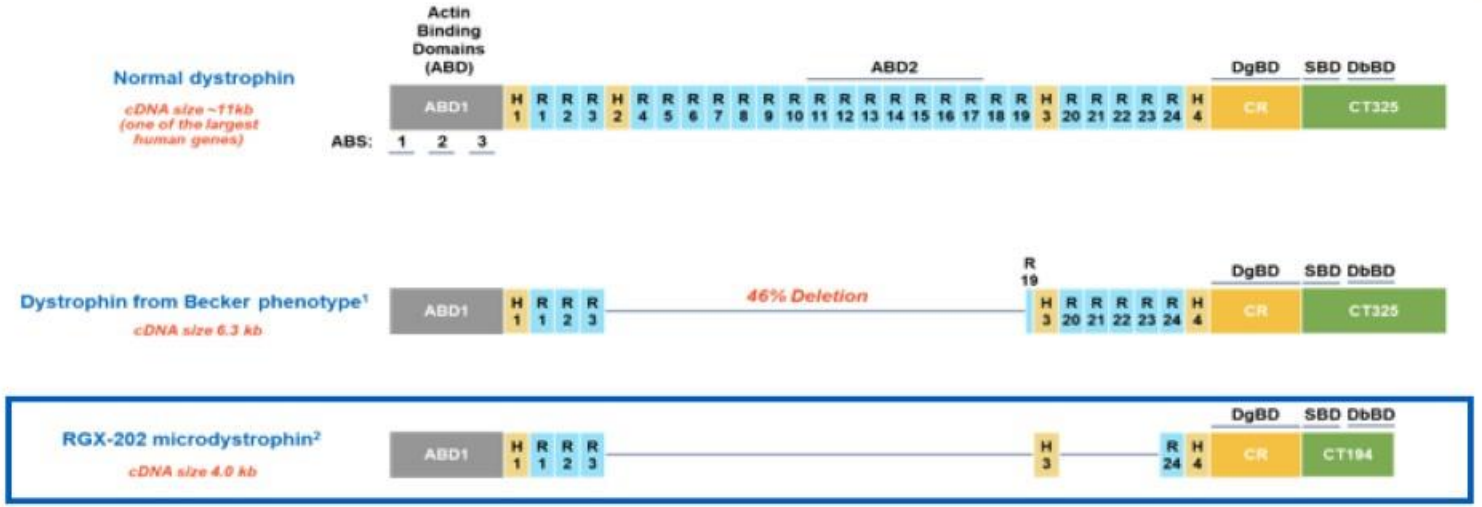
Ken Mills
President and CEO
REGENXBIO Inc.

Steve Pakola, M.D.
Chief Medical Officer
REGENXBIO Inc.

Aravindhan Veerapandiyan, M.D.
Arkansas
Children's Hospital

RGX-202 is Novel Among Current class of AAV- microdystrophins

RGX-202 expresses a new, differentiated microdystrophin with important biology that is the most similar to a natural shortened dystrophin that protects muscles from degenerating



RGX-202 is the only microdystrophin designed to deliver a transgene that includes the functional elements of the C-Terminal (CT) domain found in naturally occurring dystrophin.

1. Harper (2002) Nat Med
2. Wang (2000) PNAS

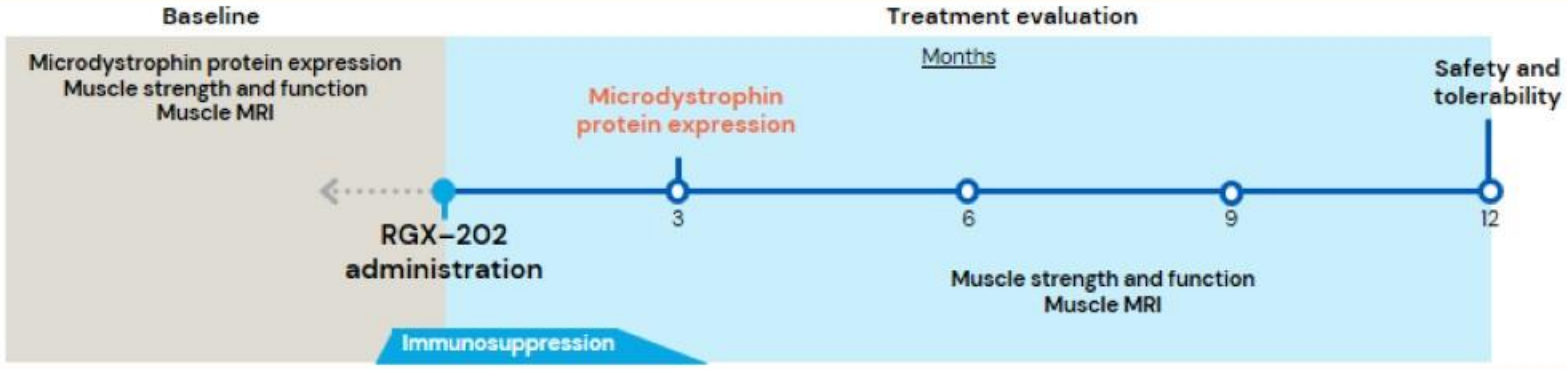
RGX-202 Study Overview

Key Eligibility Criteria

- **Boys aged 4 to 11 years** at screening
- **Genetically confirmed DMD** (mutations in exons 18 and above)
- **100-meter walk:** able to perform without assistive devices
- **No pre-existing antibodies** to the gene therapy (AAV8 capsid)

Study Plan

Administration and Assessments Timeline



RGX-202 has been well tolerated in 5 patients at both dose levels with no serious adverse events.
Age at dosing: 4 years 4 months – 12 years; post-administration follow up: 1-11 months

Phase I/II AFFINITY DUCHENNE Trial of RGX-202

Interim Clinical Data

Interim Data: Dose Level 1

Dose Level 1

- Robust RGX-202 microdystrophin expression observed
- Serum CK levels meaningfully decreased, representative of improvement in muscle disease

Patient	Age at Dosing	RGX-202 Microdystrophin Western blot (Jess method) (% Normal Control)	CK Levels, week 10 (% reduction from baseline)
Dose Level 1 (1x1014 GC/kg)			
1	4 yrs 4 mos	38.8	-43
2	10 yrs 5 mos	11.1	-44
3	6 yrs 6 mos	83.4	-93

Microdystrophin expression adjusted for muscle content
Control was level of wild-type (normal) dystrophin in normal muscle
Muscle biopsies are collected from bicep at baseline and 3 months post RGX-202 administration
Elevated CK levels are correlated with muscle injury and are uniformly elevated in patients with Duchenne
Data cut date of February 23, 2024

Interim Data: Dose Level 2, 1st Patient

Robust RGX-202 microdystrophin expression was observed at three months in 12-year-old, with comparable results obtained via Western Blot and LC-MS

RGX-202 Microdystrophin Expression

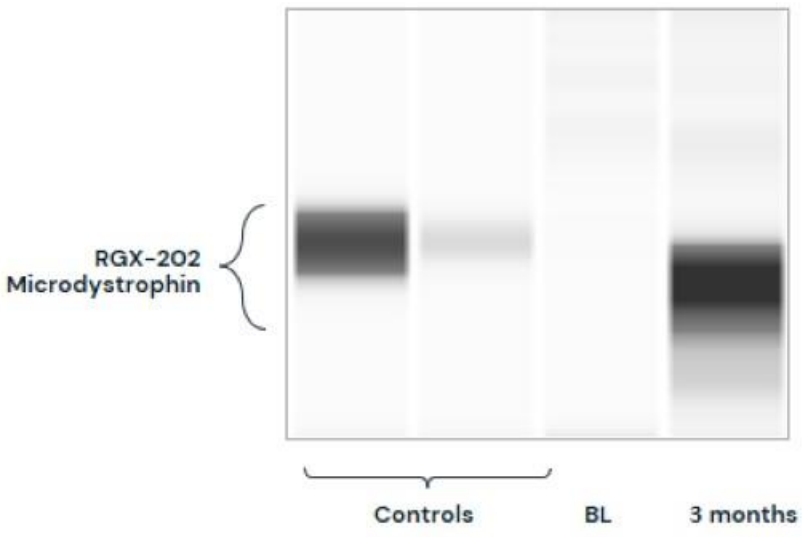
RGX-202 Microdystrophin (% Normal Control)	Patient 4 (12 yrs 0 mo 24.3 kg)
Western blot (Jess method)	75.7

CK levels

	Avg Baseline	Week 10
CK Levels (U/L)	13,131	2,983
% Reduction	-77	

Elevated CK levels are associated with muscle injury and are uniformly elevated in patients with Duchenne

Western Blot (Jess)



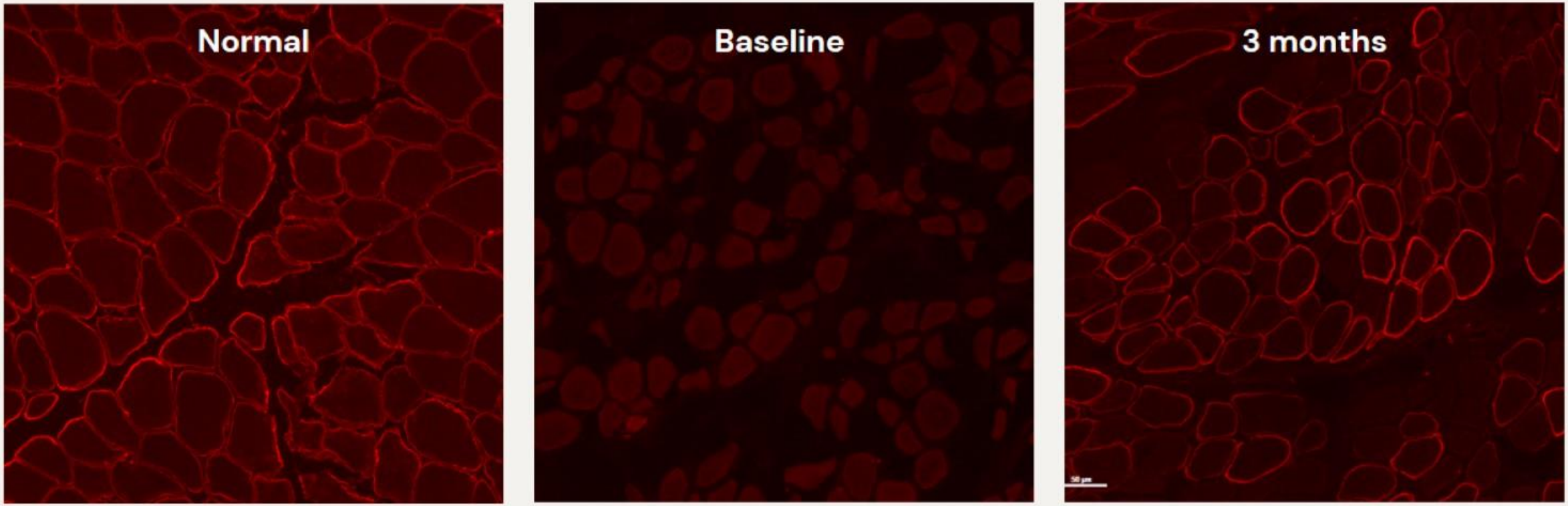
LC-MS

RGX-202 Microdystrophin Expression at 3 months

Robust RGX-202 microdystrophin expression was demonstrated at both dose levels

Age range at screening	Dose Level 1 (n = 3)	Dose Level 2 (n = 2)
	% RGX-202 microdystrophin	
4 to 5 years	38.8	
6 to 7 years	83.4	
≥ 8 years	11.1	**75.7**

RGX-202 microdystrophin is localized to the sarcolemma



Representative IF images from healthy control, patient 1 at baseline, and participant 1 three months post RGX-202 administration stained with antibody against dystrophin and RGX-202 microdystrophin

AFFINITY DUCHENNE: Summary

RGX-202 has been well-tolerated at both dose levels with no SAEs

Robust RGX-202 microdystrophin expression was observed at both dose levels in all ages

Encouraging observations of early improvements in daily activities associated with strength and function provided by caregivers

REGENXBIO to initiate pivotal trial in second half of 2024 using RGX-202 microdystrophin expression as a surrogate endpoint likely to predict clinical benefit

Q&A

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Accelerating Rare Disease Treatments in 2024

RGX-202 for treatment of Duchenne