

# RGX-202

REGENXBIO's investigational gene therapy for the treatment of Duchenne Muscular Dystrophy (DMD)

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PPMD End Duchenne Tour - Detroit - 21Aug2022

# Forward-looking Statements

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# REGENXBIO

Seeking to improve lives through the curative potential of gene therapy

- **Leader in AAV Gene Therapy**  
with exclusive rights to the NAV<sup>®</sup> Technology Platform
- **End-to-End Capabilities**  
from research through clinical development and commercial-ready manufacturing
- **Broad Internal Pipeline**  
across multiple therapeutic areas including neuromuscular
- **Proprietary AAV Manufacturing**  
NAVXpress<sup>™</sup> platform and internal manufacturing facilities

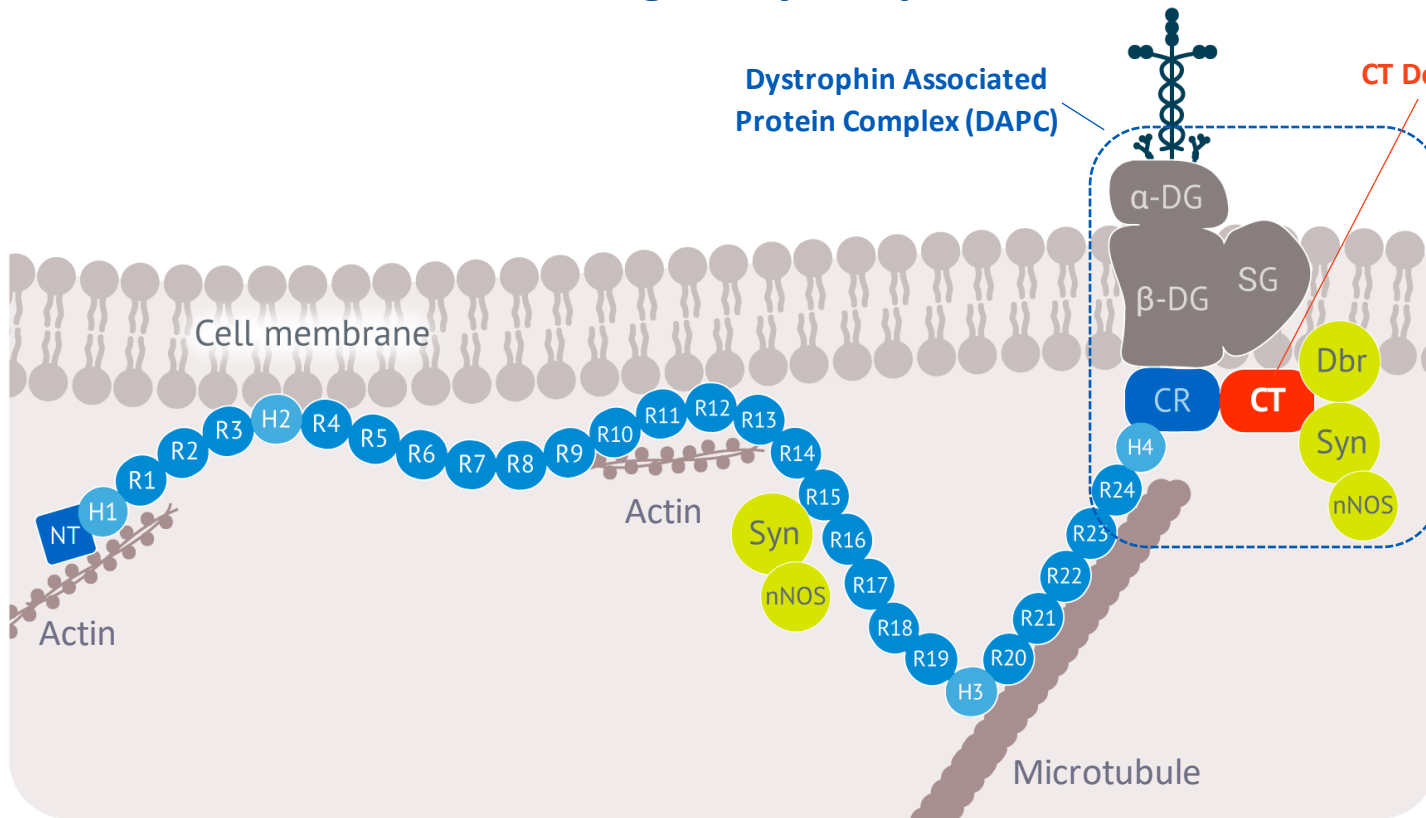
# REGENXBIO's Investigational Gene Therapy for Duchenne

SEEKING TO  
ADVANCE A NOVEL  
GENE THERAPY TO  
IMPROVE THE LIVES  
OF BOYS WITH  
DUCHENNE

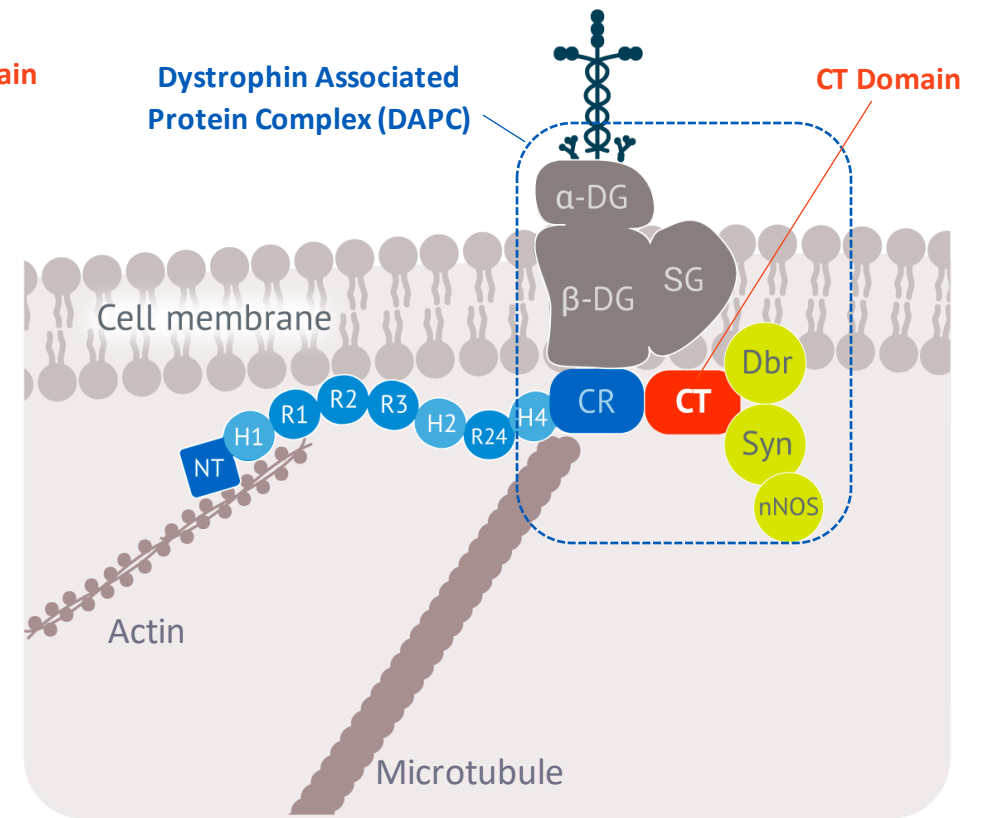
- > RGX-202 is an optimized microdystrophin gene therapy with unique features
- > Partnering with Duchenne community to guide development
- > Preclinical studies completed providing evidence of potential benefit
- > U.S. FDA IND clearance to evaluate RGX-202 in a Phase I/II study (AFFINITY DUCHENNE™)
- > Plan to initiate dosing in first half of 2023

# RGX-202 Microdystrophin Designed to Retain Key Elements of Full-Length Dystrophin, including the C-Terminal (CT) Domain

## Full Length-Dystrophin



## RGX-202 Microdystrophin

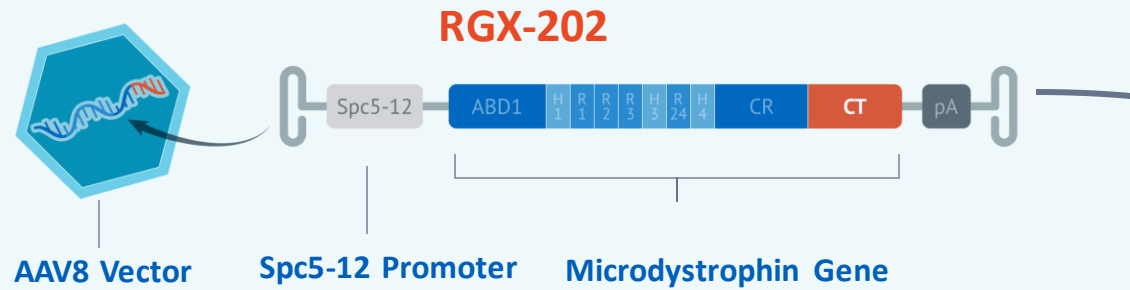


### Abbreviations

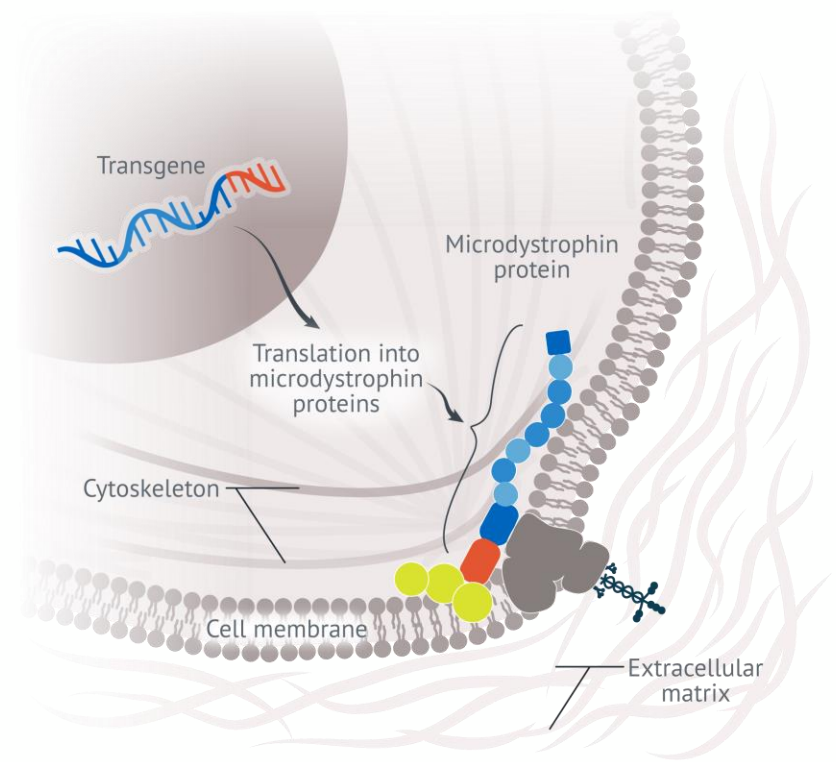
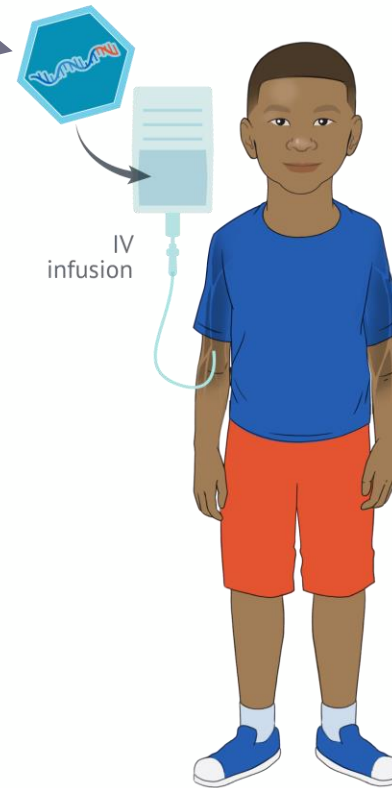
Syn: Syntrophin; Dbr: Dystrobrevin; CR: Cystein rich domain; nNOS: Neuronal nitric oxide synthase; DG: Dystroglycan; H: hinge; R: repeat



# Optimizing an AAV gene therapy to address lack of functional dystrophin

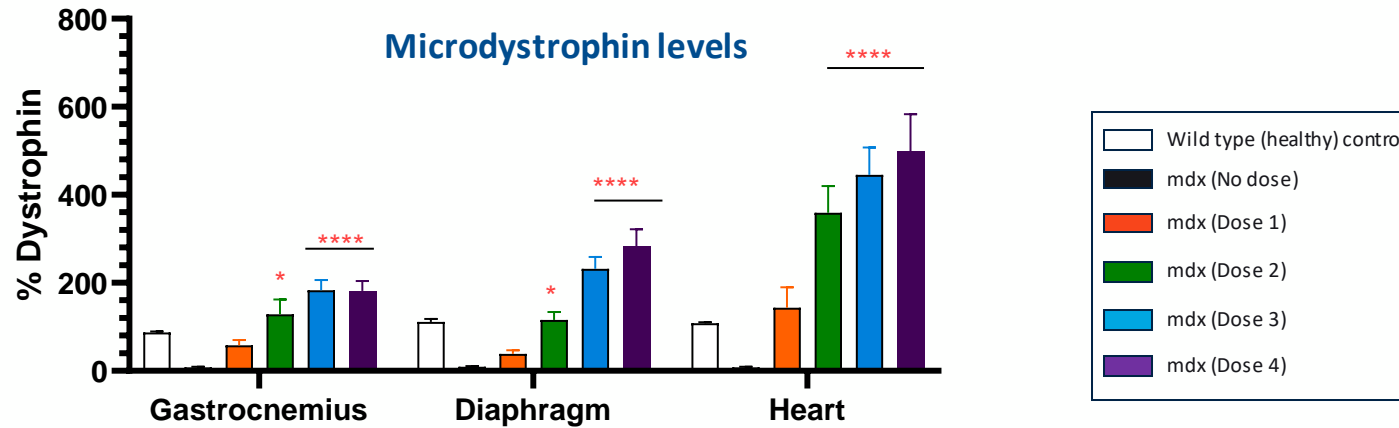


- ✓ **AAV8 Vector** delivers the transgene to skeletal and heart muscles in the body
- ✓ **Promoter (Spc5-12)** drives expression of protein in muscles
- ✓ **Microdystrophin Gene** encodes a novel protein designed to retain key elements of full-length dystrophin including the CT Domain

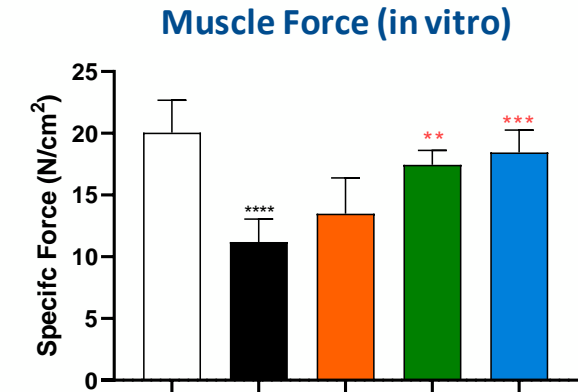


# RGX-202 Preclinical Evidence in DMD animal model (*mdx* mice)

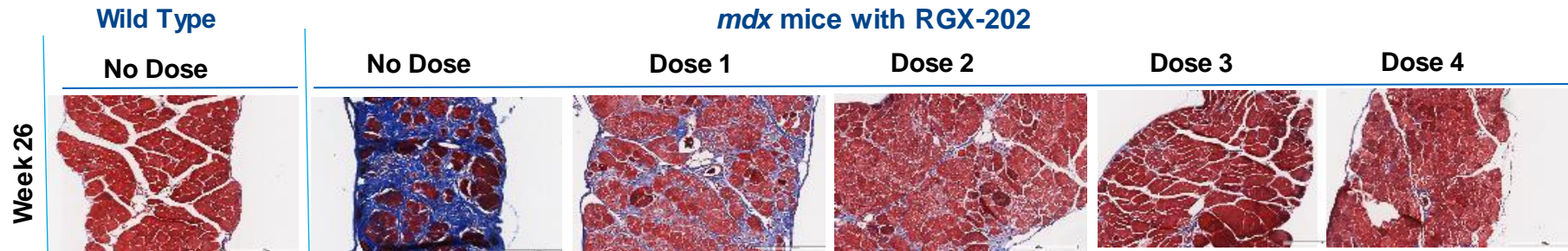
## Robust microdystrophin expression in skeletal and cardiac muscle



## Significant increase in muscle function and strength



## Improvement in muscle (fibrosis)



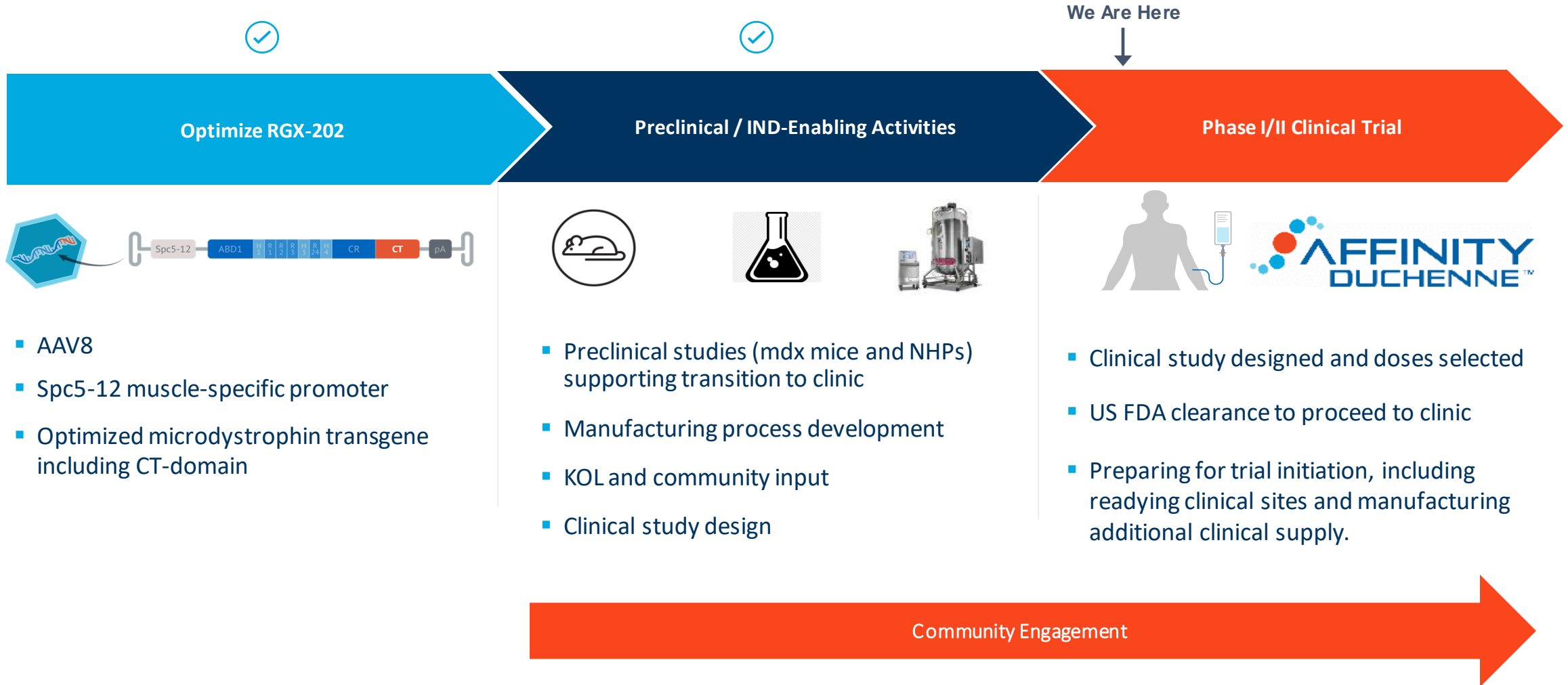
Data Sources:

Kim S. et al., A Novel AAV8 Vector for Microdystrophin Gene Therapy OF Duchenne Muscular Dystrophin: Preclinical Studies in *MDX* mouse, Poster Presented at ASGCT 2021 Annual Meeting, May 11-14, 2021

Kim S. et al., A Novel AAV8 Vector for Microdystrophin Gene Therapy OF Duchenne Muscular Dystrophin: Preclinical Studies in *MDX* mouse, Poster Presented at WMS 2021 Annual Meeting, Sep 20-24, 2021

# Phase I/II (AFFINITY DUCHENNE™) Start-Up Underway

Anticipate initiation of dosing in the first half of 2023





# AFFINITY DUCHENNE™ Phase I/II Clinical Trial Overview

Open-label, multi-center study to evaluate the safety, tolerability, and efficacy of a one-time dose of RGX-202 in boys with Duchenne



## Key Eligibility Criteria

Boys aged 4 to 11 years

Ambulatory function (can complete timed function tests)

Specific gene mutations

No antibodies against the gene therapy (AAV8 capsid)

Additional criteria will apply



## Key Outcome Measures

Safety assessment

Microdystrophin protein levels in muscle measured by biopsy

Skeletal and heart muscle measured by MRI

Strength and function measured by North Star Ambulatory Assessment (NSAA) and timed function tests

Information on the trial will be available on [clinicaltrials.gov](https://clinicaltrials.gov) in the future

# SUMMARY

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# Thank You

To learn more about REGENXBIO, please visit:

[www.regenxbio.com](http://www.regenxbio.com)

Please stay in touch with us by email:

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