

Update on the INSPIRE DUCHENNE Phase 1/2 Study of SGT-003 Microdystrophin Gene Therapy

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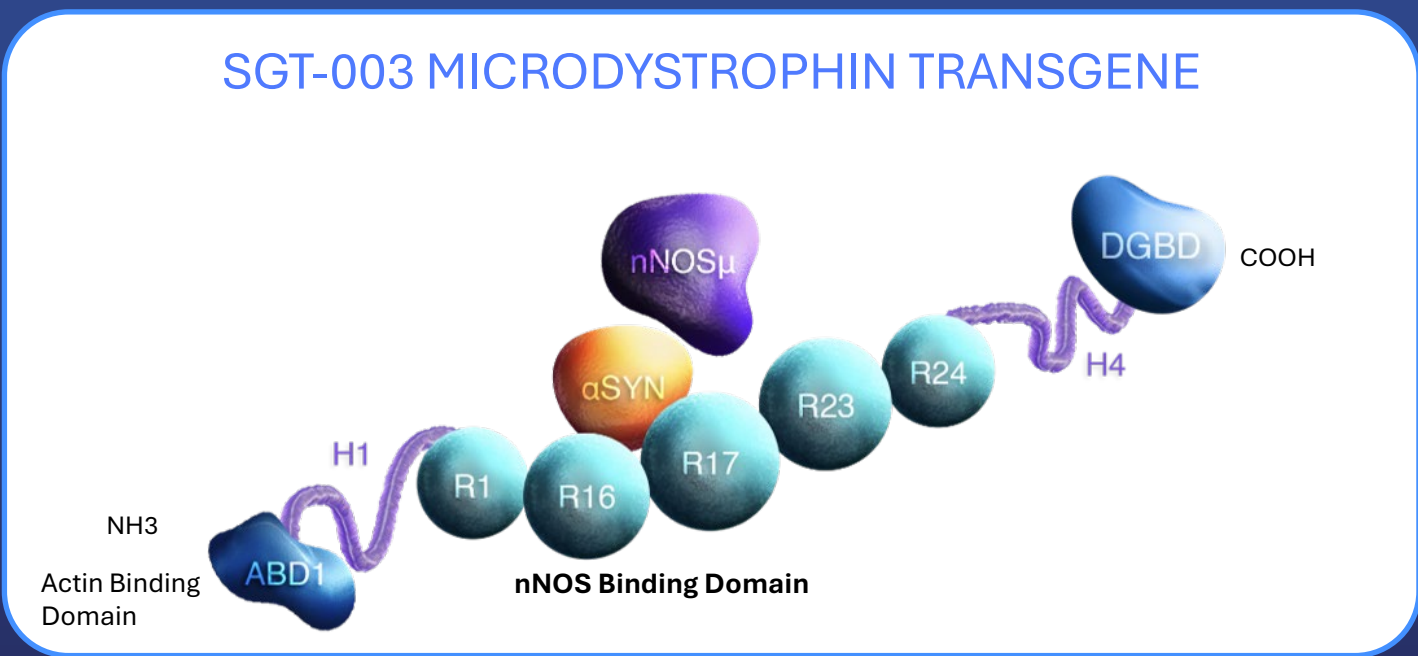
INTRODUCTION

Background

- Duchenne muscular dystrophy (Duchenne) is a chronic, progressive, and ultimately fatal X-linked recessive neuromuscular disorder caused by the lack of functional dystrophin protein¹
 - Dystrophin is required for maintaining muscle integrity and function²⁻⁴
 - Deterioration of muscle integrity leads to muscle fiber breakdown, resulting in progressive functional decline
- Shortened, functional microdystrophin transgenes can be packaged into adeno-associated viruses (AAVs) to replace dystrophin⁵
 - Microdystrophins vary based on their unique compositions⁶

SGT-003 Overview

- SGT-003 is Solid's next-generation AAV-microdystrophin gene therapy with key features:
 - Microdystrophin transgene: unique inclusion of the R16/R17 neuronal nitric oxide synthase (nNOS)-binding domain with the goal of preventing activity-induced ischemia and associated muscle injury⁷



- POLARIS-101™ capsid (formerly known as AAV-SLB101): rationally designed muscle-tropic capsid that leads to higher levels in muscle and reduced levels in the liver⁸
- Full/empty capsid ratio: SGT-003 manufacturing uses a transient transfection process, focusing on maintaining high % full capsid ratios to improve purity

INSPIRE DUCHENNE (NCT06138639) Study Design

- Phase 1/2, open-label study evaluating the safety, tolerability, and efficacy of a single intravenous (IV) infusion of SGT-003 at 1E14 vg/kg
- Actively enrolling participants 0 to < 12 years of age with a confirmed diagnosis of Duchenne
- Uses a steroid-only regimen for prophylactic immunomodulation
- Primary safety endpoint: incidence of treatment-emergent adverse events (TEAEs) through Day 360
- Primary efficacy endpoint: change from baseline of microdystrophin protein levels at Day 90
- Secondary endpoints include:
 - Microdystrophin expression: Protein levels at Day 360 and distribution at Day 90 and Day 360
 - Motor function: Time-to-Rise, 10-Meter Walk/Run, North Star Ambulatory Assessment, 4-Stair Climb, 6-Minute Walk Test, Stride Velocity 95th Centile

RESULTS

SGT-003 HAS BEEN GENERALLY WELL TOLERATED

- No drug-induced liver injury, thrombotic microangiopathy, atypical hemolytic uremic syndrome, or myocarditis have been observed to date

Table 1. INSPIRE DUCHENNE Enrollment⁹

Cohorts	Eligible age range (years)	Ages at enrollment (years)	Weights for dosing (kg)	Participants enrolled (n)
1-3	0 to <12	6 mo. to 10 years	6.5 to 39.7	50/53*

*Participants 51-53 were dosed after the safety data cutoff of May 29, 2026. Solid Biosciences. 2026.

Table 2. INSPIRE DUCHENNE Safety Data¹⁰

SGT-003 Participants With Treatment-Related SAEs	n (%)
Serious Adverse Events (SAEs)	2 (4.0)*

*One (n=1) participant with a possibly related transient adverse event of inflammation treated with two doses of antibiotics. One (n=1) previously reported immune mediated myositis. Both events have resolved.

SGT-003 Participants With Treatment-Related AEs	n (%)
Vomiting	32 (64.0)
Nausea	27 (54.0)
Decreased Appetite	15 (30.0)
Thrombocytopenia	13 (26.0)
Abdominal Pain	9 (18.0)

Data cutoff of May 29, 2026. Solid Biosciences.

REFERENCES

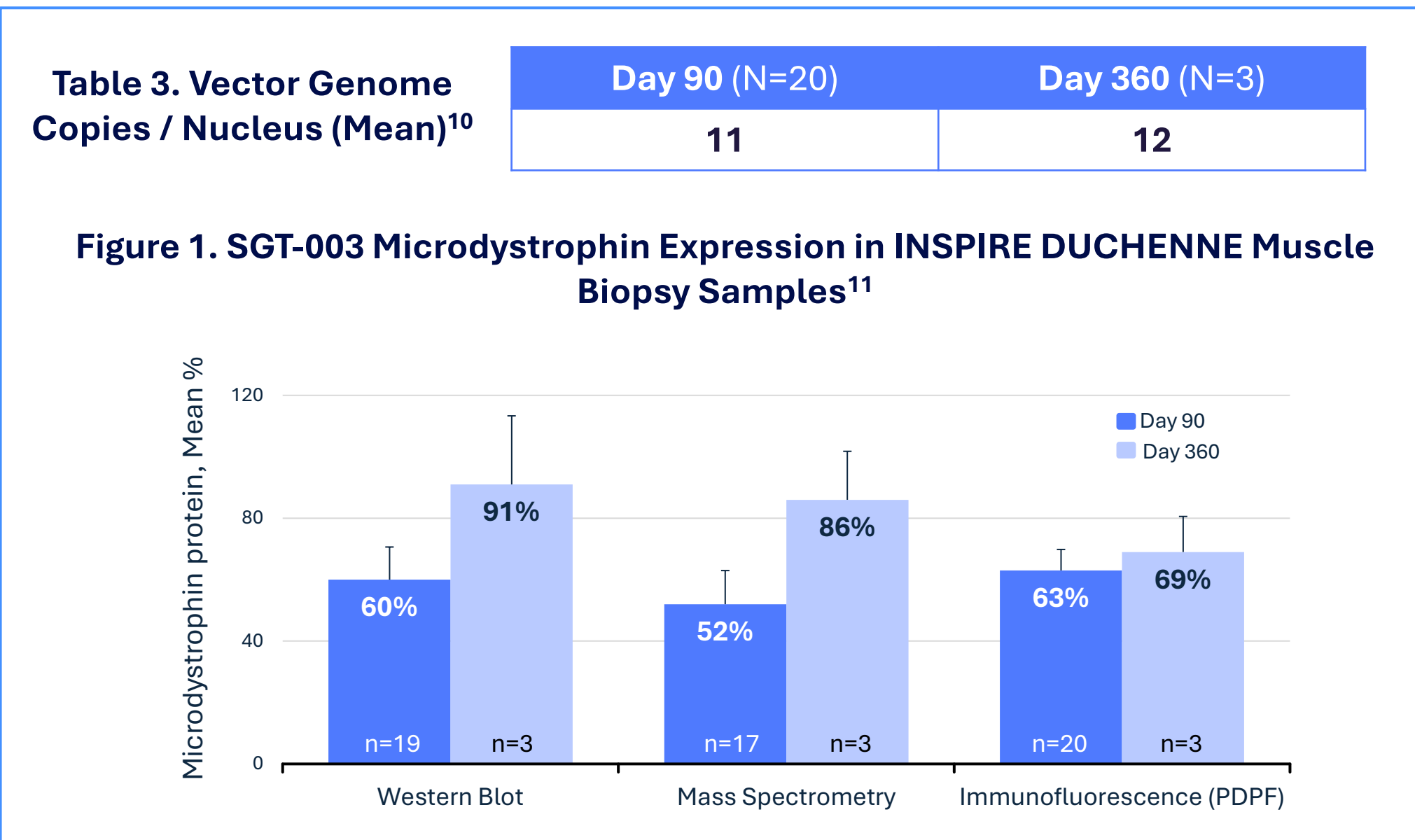
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ACKNOWLEDGMENTS

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RESULTS Cont'd

SGT-003 DEMONSTRATED ROBUST TRANSDUCTION & MICRODYSTROPHIN EXPRESSION



PDPF: percent dystrophin positive fibers

Figure 1. Values are means ± standard error of the mean. Western blot and mass spectrometry baselines were 0% mean normal dystrophin, immunofluorescence is based on a manual count. Western blot, mass spectrometry and immunofluorescence assays are conducted by multiple external vendors; at the time of this analysis, one western blot sample (from participant 20) and three mass spectrometry samples (from participants 15,16 and 20) had not been received

SGT-003 RESTORES KEY ELEMENTS OF THE DAPC

Figure 2. Immunofluorescence Evaluation of Dystrophin Associated Protein Complex (DAPC) Restoration in INSPIRE DUCHENNE Muscle Biopsy Samples¹¹

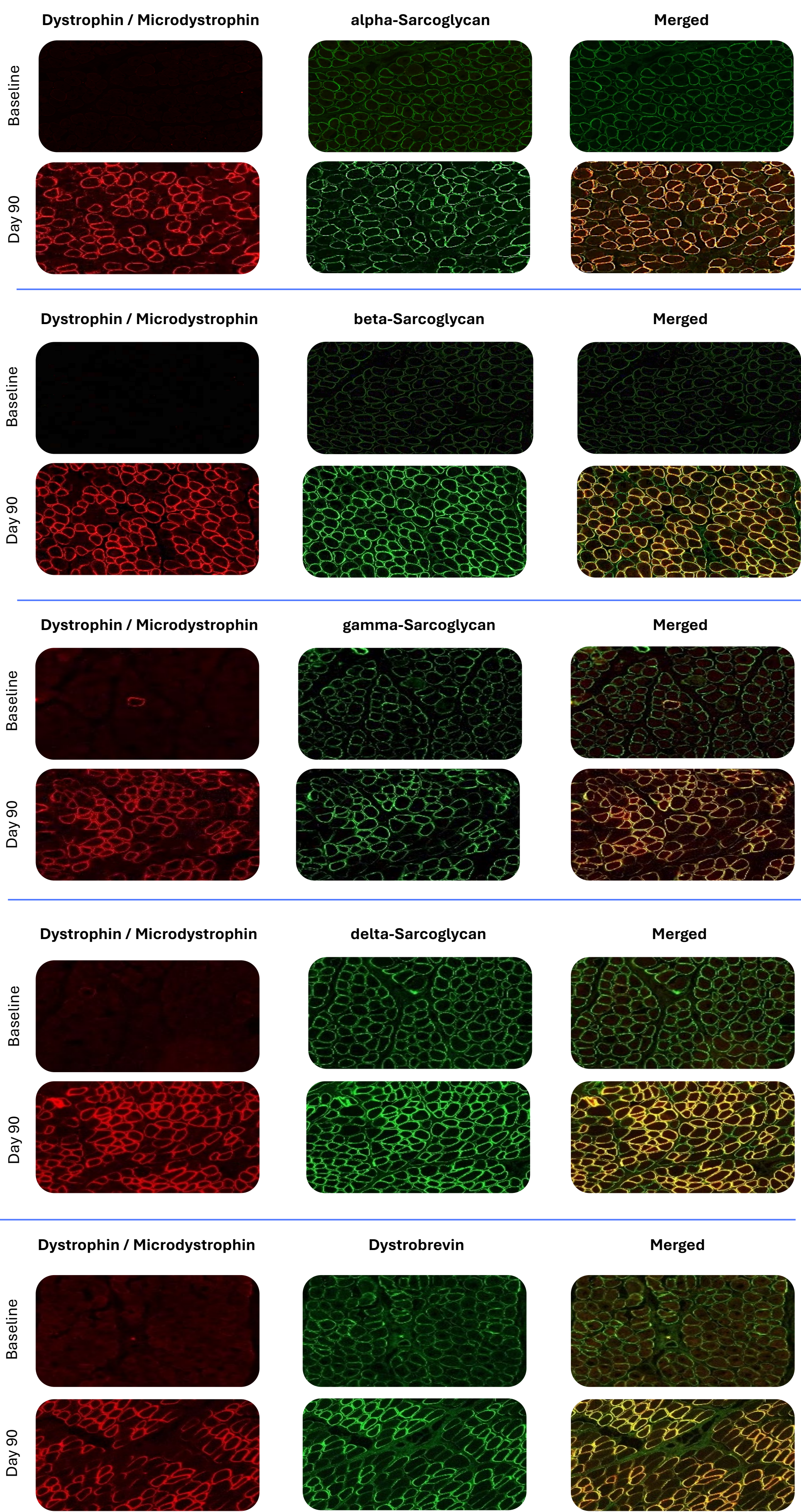
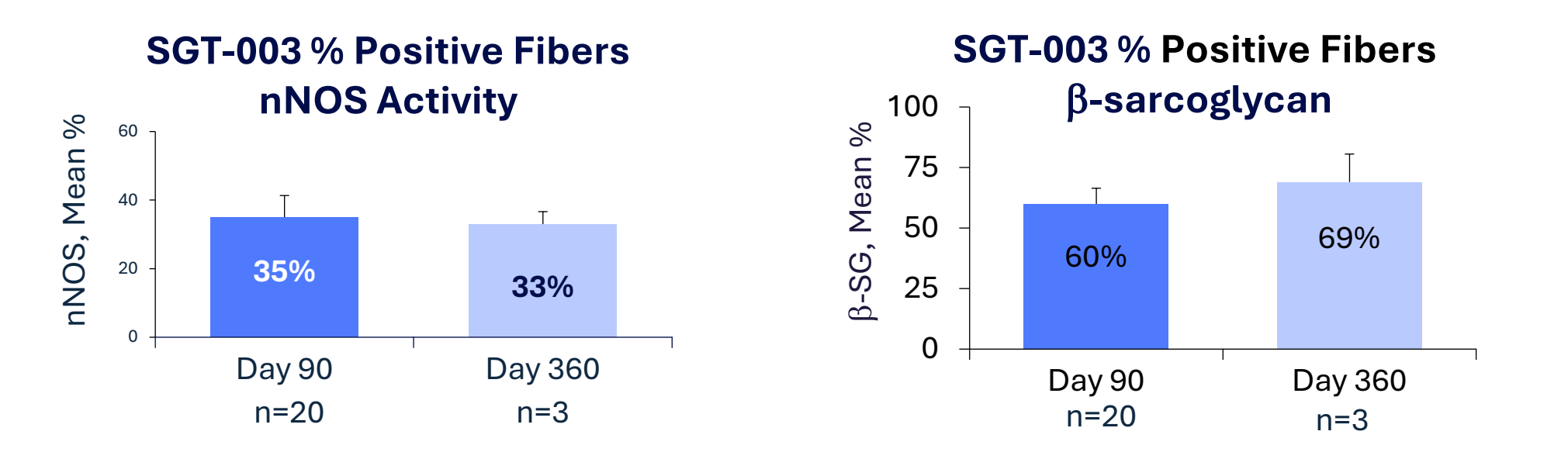


Figure 2. Muscle biopsies showed restored membrane localization of the DAPC, corresponding with microdystrophin expression. Representative images are shown from the same participant.

Figure 3. Quantification of nNOS & β-sarcoglycan Sarcolemmal Restoration in INSPIRE DUCHENNE Muscle Biopsy Samples¹¹



nNOS: neuronal nitric oxide synthase

Figure 3. Beta-sarcoglycan and nNOS values were based on a manual count.

SGT-003 IMPROVES MUSCLE HEALTH

Figure 4. Reduced Chronic Regenerative Demand Observed Post-SGT-003 Treatment¹¹

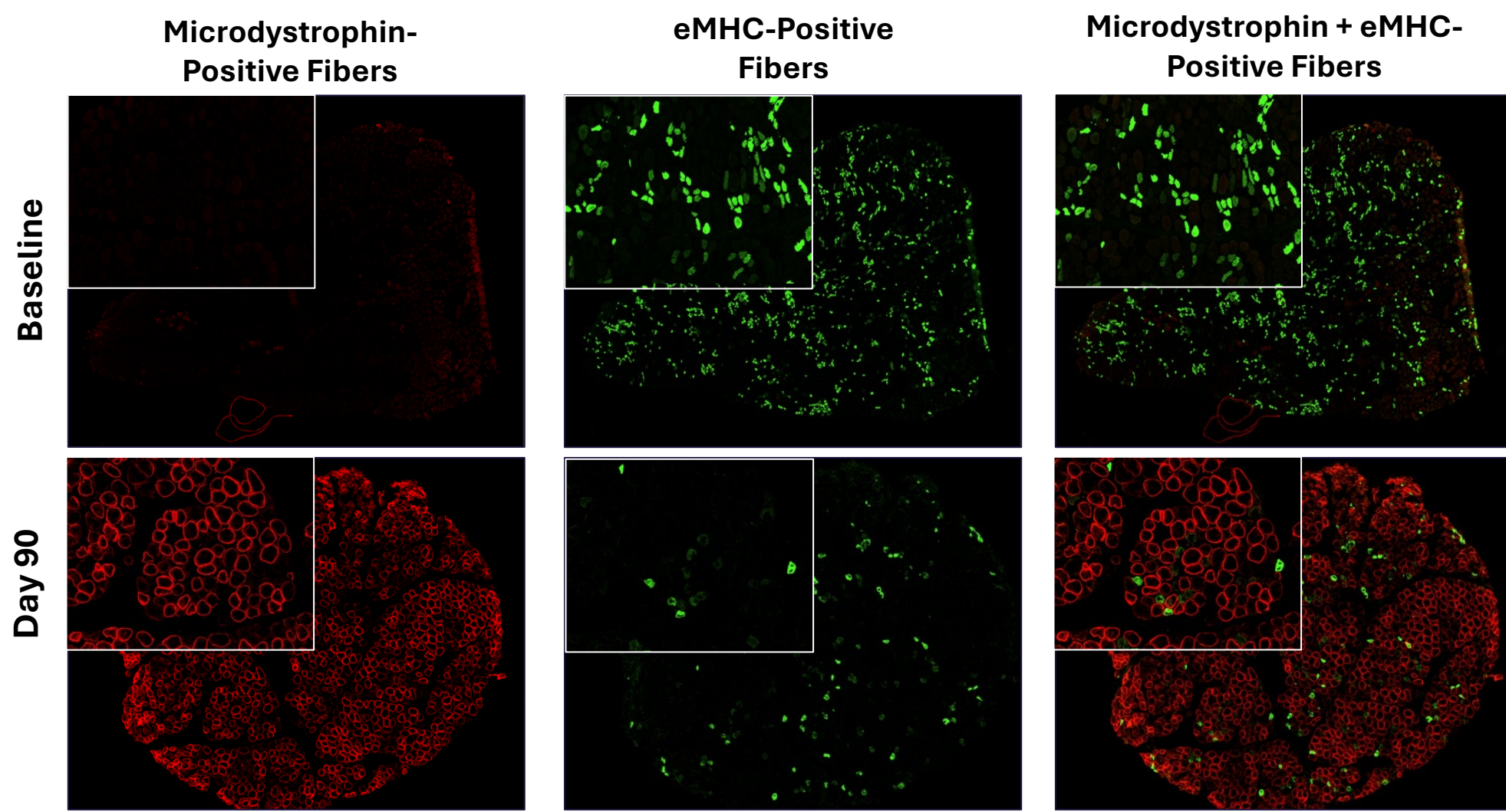


Figure 4. The reduction in eMHC suggests reduced need for muscle repair and preservation of muscle architecture.

SGT-003 SHOWS IMPROVED MUSCLE INTEGRITY

Figure 5. Reductions in Markers of Disrupted Muscle Integrity After SGT-003¹¹

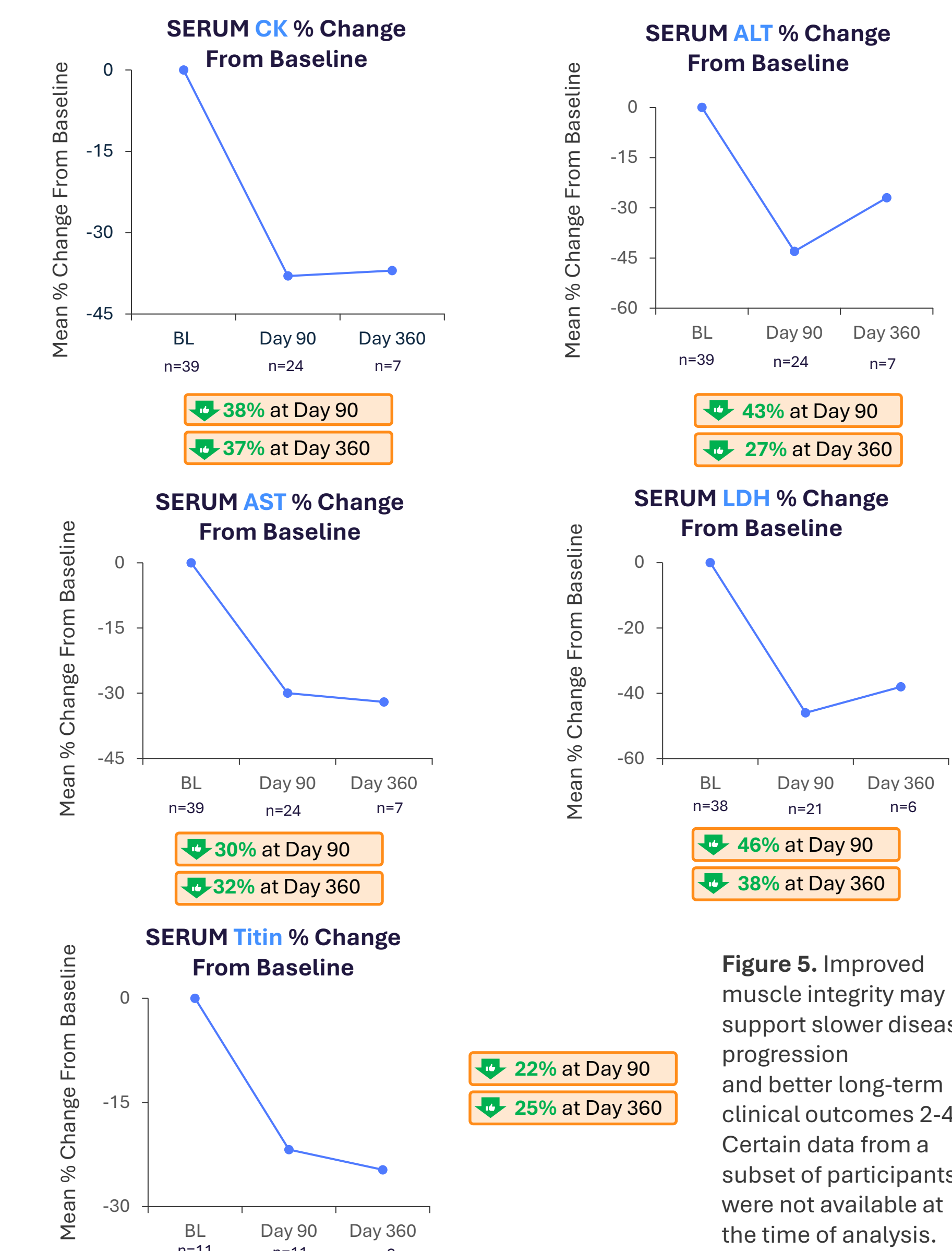


Figure 5. Improved muscle integrity may support slower disease progression and better long-term clinical outcomes 2-4. Certain data from a subset of participants were not available at the time of analysis.

STABLE TO IMPROVED CARDIAC FUNCTION OBSERVED AFTER SGT-003 TREATMENT

Figure 6. Observations of Cardiac Function and Cardiac Injury Markers Following SGT-003 Treatment¹¹

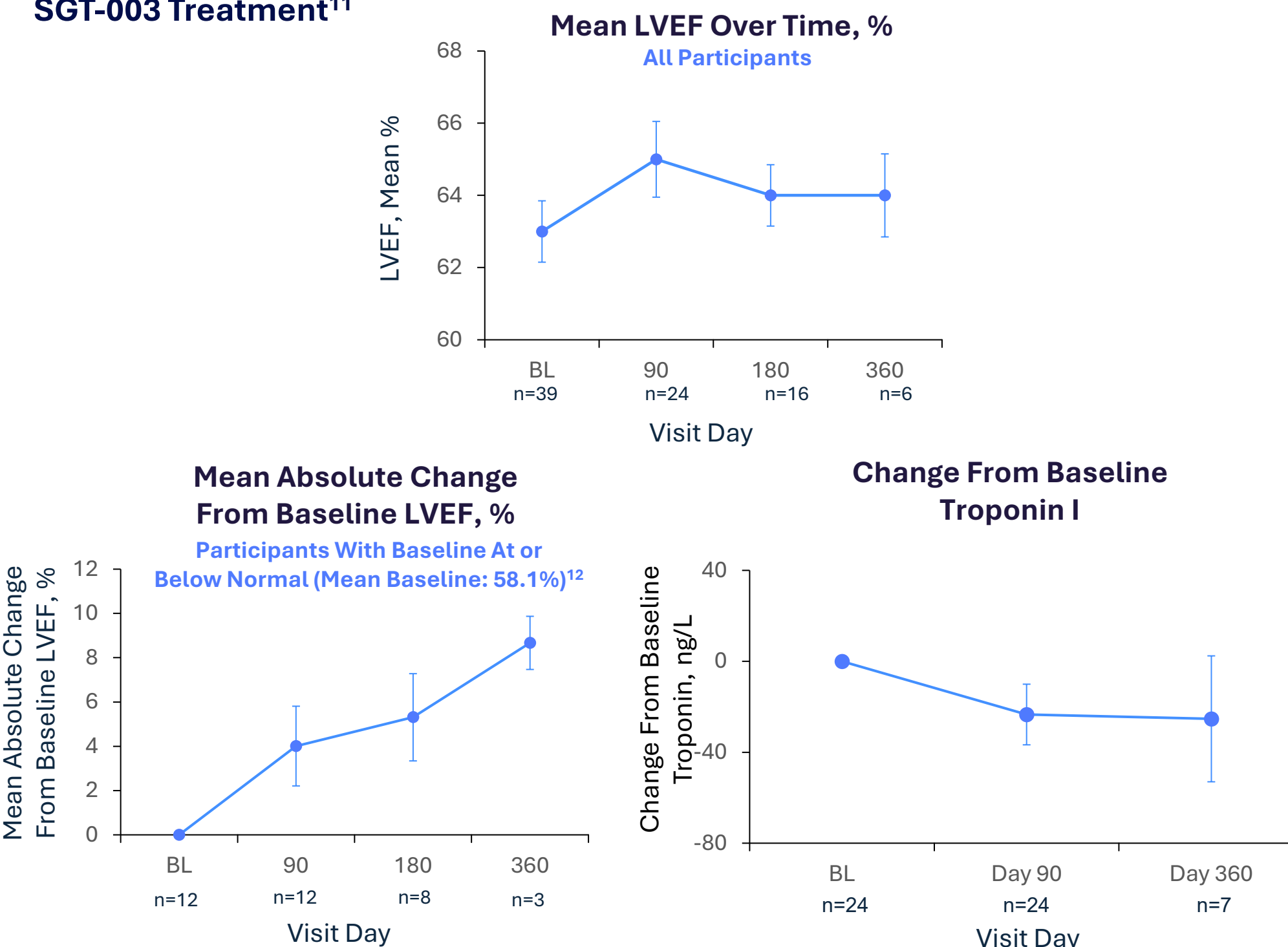


Figure 6. Observations of improved cardiac function are driven by participants with low-normal baseline LVEF¹³. Changes in troponin I levels were driven by participants with elevated troponin at baseline

CONCLUSIONS

- SGT-003 has been generally well tolerated in the 53 participants dosed in the INSPIRE DUCHENNE study as of August 4, 2026, using a steroid-only prophylactic immunomodulation regimen
- Robust transduction and microdystrophin expression have been observed in muscle biopsy samples collected at Day 90 and Day 360 after treatment with SGT-003
- Corresponding restoration of key elements of the DAPC has been observed in muscle biopsy samples
- Improvements in markers of muscle integrity have been evident as early as Day 90 and persist to at least Day 360 after treatment with SGT-003
- Stable-to-improved cardiac function has been observed post treatment with SGT-003 as measured by LVEF