ON BEHALF OF THE INSPIRE DUCHENNE STUDY TEAM

Update on the INSPIRE DUCHENNE Phase 1/2 Study of the Next-Generation Microdystrophin Gene Therapy Candidate SGT-003 for Duchenne Muscular Dystrophy

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INSPIRE DUCHENNE

SGT-003 is an investigational product that has not been approved in any region. No conclusions regarding safety and efficacy can be made.

Disclosures

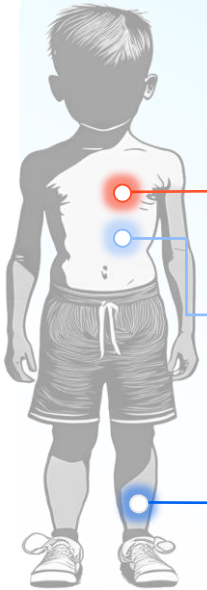
- Clinical Trial Support: Sarepta, Dyne, Avidity, Ultragenyx, Solid
- Advisory Boards: Armatus, Encoded, Insmed, Dyne, Solid
- Past Royalties: Astellas

Duchenne Muscular Dystrophy (Duchenne): Background

Duchenne is an X-linked recessive neuromuscular disorder caused by a lack of functional dystrophin[1]

- Dystrophin is required for maintaining muscle integrity and function[2-4]
  - Deterioration of muscle integrity leads to loss of essential membrane proteins and muscle fiber breakdown and leakage, resulting in progressive functional decline

- Shortened, functional "microdystrophin" transgenes can be packaged into AAVs to replace dystrophin[5]
  - Microdystrophins vary based on their unique compositions[6]



- Decreased heart function
- Cardiomyopathy

HEART FAILURE

- Weak diaphragm

RESPIRATORY FAILURE

- Loss of muscle mass
- Inflammation
- Fibrosis

LOSS OF AMBULATION

The impact of treatments on muscle integrity is key for patients with Duchenne[7]

AAV: adeno-associated virus.

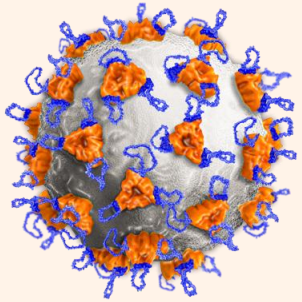
1. Duan D, et al. *Nat Rev Dis Primers*. 2021;7(1):13. 2. Sheybani A, et al. *Pediatr Res*. 2022;92(6):1613-1620. 3. Voleti S, et al. *Pediatr Cardiol*. 2020;41(6):1173-1179. 4. Wagner KR, et al. *Biomark Med*. 2021;15(15):1389-1396. 5. Crudele JM, et al. *Hum Mol Genet*. 2019;28(R1):R102-R107. 6. Ramos JN, et al. *Mol Ther*. 2019;27(3):623-635. 7. Escobar-Huertas JF, et al. *Cytoskeleton (Hoboken)*. 2024;81(6-7):269-286.

SGT-003 is a Next-Generation Candidate Optimized for Robust Function and Transduction in Skeletal and Cardiac Muscle

SGT-003 MICRODYSTROPHIN TRANSGENE

- Unique inclusion of the nNOS-binding domain with the goal of preventing activity-induced ischemia and associated muscle injury[1]
- nNOS is important for normalizing NO production, improving calcium homeostasis and enabling normal contraction-relaxation cycling in the heart[2,3]

POLARIS-101™ CAPSID (AAV-SLB101)



- Rationally designed capsid engineered to include an RGD motif inserted into the VR-VIII loop of AAV9, resulting in up to 60 copies of the peptide displayed across the capsid surface[4,5]
- RGD–integrin–mediated uptake preferentially targets skeletal and cardiac muscle due to integrin upregulation, especially in diseased tissue, limiting off-target uptake

nNOS: neuronal nitric oxide synthase; NO: nitric oxide.

1. Data on file. Solid Biosciences. 2026. 2. Zhang YH, et al. *J Physiol*. 2014;592(Pt 15):3189-3200. 3. Ziolo MT, et al. *J Mol Cell Cardiol*. 2012;45(5):625-632. 4. DiMattia MA, et al. *J Virol*. 2012;86(12):6947-6958. 5. Drouin LM, Agbandje-McKenna M. *Future Virol*. 2013;8(12):1183-1199.

INSPIRE DUCHENNE: Study Overview

- Single-dose level (1.0E14 vg/kg), open-label, Phase 1/2 study
- Actively enrolling: US, Canada, Italy, and the UK
- Patients < 12 years of age with a confirmed diagnosis of Duchenne
- Potential for further enrollment of older and non-ambulatory patients (Cohorts 4 and 5)
- Prophylactic prednisone regimen alone used as immunomodulation

Primary Endpoints:

- Incidence of treatment-emergent adverse events through Day 360
- Change from baseline of microdystrophin protein levels at Day 90

Secondary Endpoints:

- Microdystrophin protein levels and distribution at Days 90 and 360
- TTR, 10MWR, 4SC, NSAA, 6MWT, SV95C at Days 360 and 540

Exploratory Endpoints:

- % predicted FVC, PEF, FEV1; Bayley-4; PODCI

KEY ELIGIBILITY CRITERIA

Age:	Cohort 1: Aged 4 to <7 years Cohort 2: Aged 7 to <12 years Cohort 3: Aged 0 to <4 years
***DMD* Genetic Variant Exclusions:**	Any deletion in exons 1 to 11, 42 to 45, or 57-69, inclusive
Ambulation:	Cohorts 1, 2: Required Cohort 3: N/A
Additional Function:	Cohorts 1, 3: N/A Cohort 2: TTR and 10MWR criteria
Antibodies:	Negative for AAV9 antibodies
Prior Treatments:	No history of gene therapy ≥12-week washout from exon-skipping therapies, vamorolone, and/or givinostat
Steroid Regimen:	Cohorts 1, 2: On a stable dose of daily oral steroids (prednisone/deflazacort) for ≥12 weeks Cohort 3: N/A

4SC: 4-stair climb; 6MWT: 6-minute walk test; 10MWR: 10-meter walk/run; FEV1: forced expiratory volume in 1 second; FVC: forced vital capacity; IV: intravenous; N/A: not applicable; NSAA: North Star Ambulatory Assessment; PEF: peak expiratory flow; PODCI: pediatric outcomes data collection instrument; SV95C: stride velocity 95th centile; TTR: time to rise.
INSPIRE DUCHENNE: NCT06138639. Data on file. Solid Biosciences.

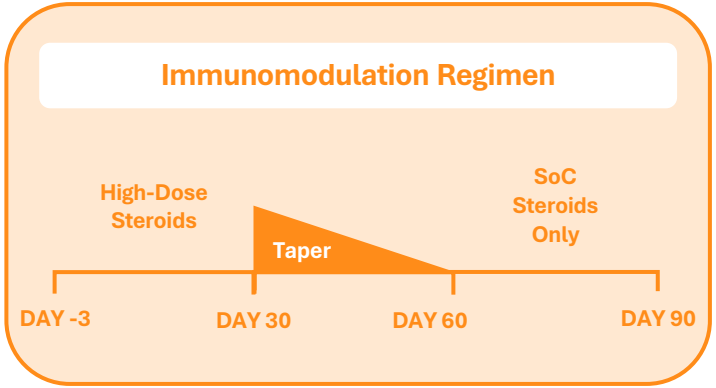
As of May 4, 2026

INSPIRE DUCHENNE Safety Data for 46 Participants

Cohorts	Eligible Age Range (years)	Ages at Enrollment (years)	Weights for Dosing (kg)	Participants Enrolled (n)
1-3	0 to <12	1 to 10	9.9 to 39.7	46

SGT-003 Participants With Treatment-Related SAEs	n (%)
Serious Adverse Events (SAEs)	1 (2.2)[1]

SGT-003 Participants With Treatment-Related AEs		n (%)
Most Common Treatment-Related Adverse Events (AEs)	Vomiting	31 (67.4)
	Nausea	27 (58.7)
	Decreased appetite	15 (32.6)
	Thrombocytopenia	13 (28.3)
	Abdominal Pain	8 (17.4)



SGT-003 uses a steroid-only prophylactic immunomodulatory regimen supported by rigorous safety monitoring

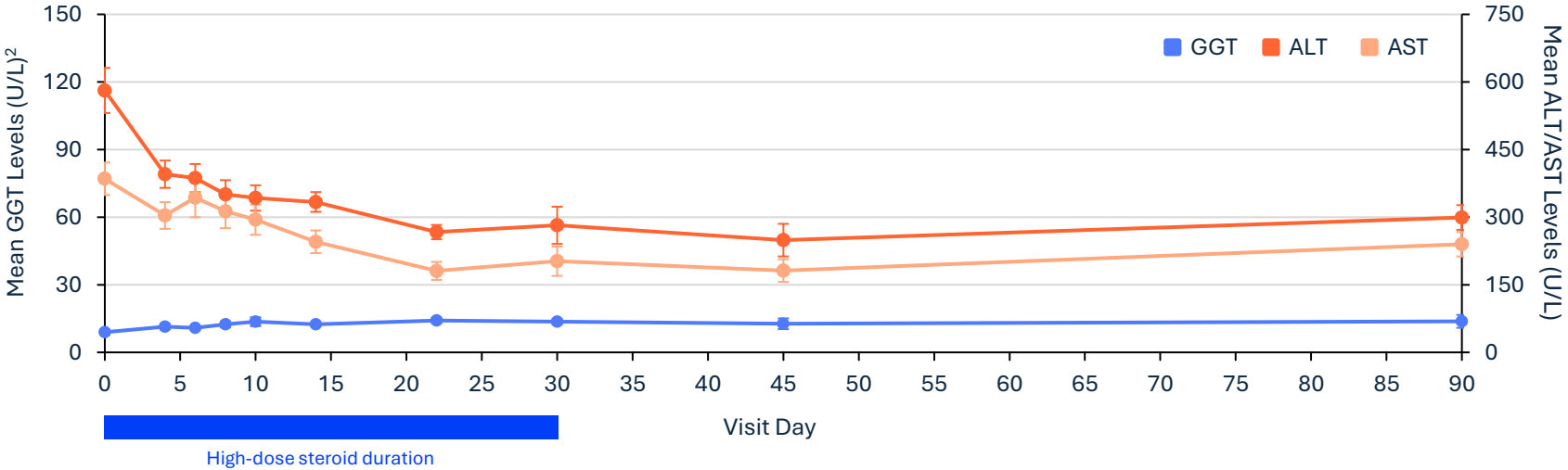
AE: adverse event; SAE: serious adverse event
1. One (n=1) previously reported Common Terminology Criteria for Adverse Events Grade 3 SAE of immune-mediated myositis. The myositis was not associated with muscle pain or weakness and has resolved. Data on file as of May 4, 2026. Solid Biosciences. 2026.

As of February 23, 2026

Liver Enzymes Declined or Remained Stable Following SGT-003 Administration

SGT-003 Clinical Trial Liver Biomarkers[1]

(n=24 [includes only participants with data to Day 90])



Stable GGT with declining ALT/AST suggests preserved liver function and improving muscle integrity

ALT: alanine aminotransferase; AST; aspartate aminotransferase; GGT: gamma-glutamyltransferase

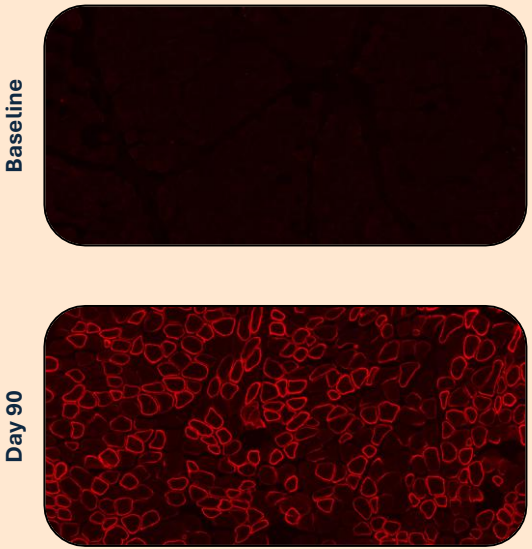
1. Values are means ± standard error of the mean. 2. Error bars for GGT data are present but obscured by their associated data points due to the small range for standard error.

Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026.

As of February 23, 2026

SGT-003 Demonstrated Robust Microdystrophin Transduction & Expression

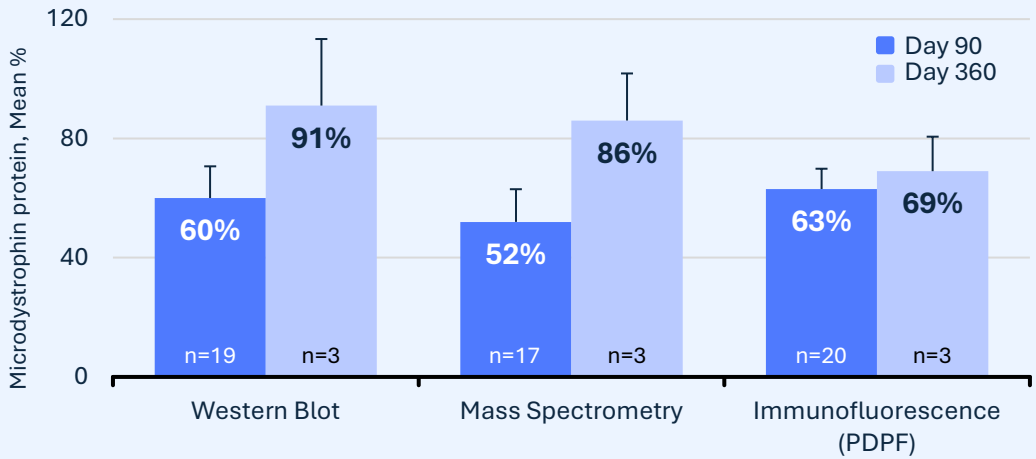
EXAMPLE SGT-003 MUSCLE BIOPSY MICRODYSTROPHIN STAINING



VECTOR GENOME COPIES/NUCLEUS (MEAN)

Day 90 (N=20)	Day 360 (N=3)
11	12

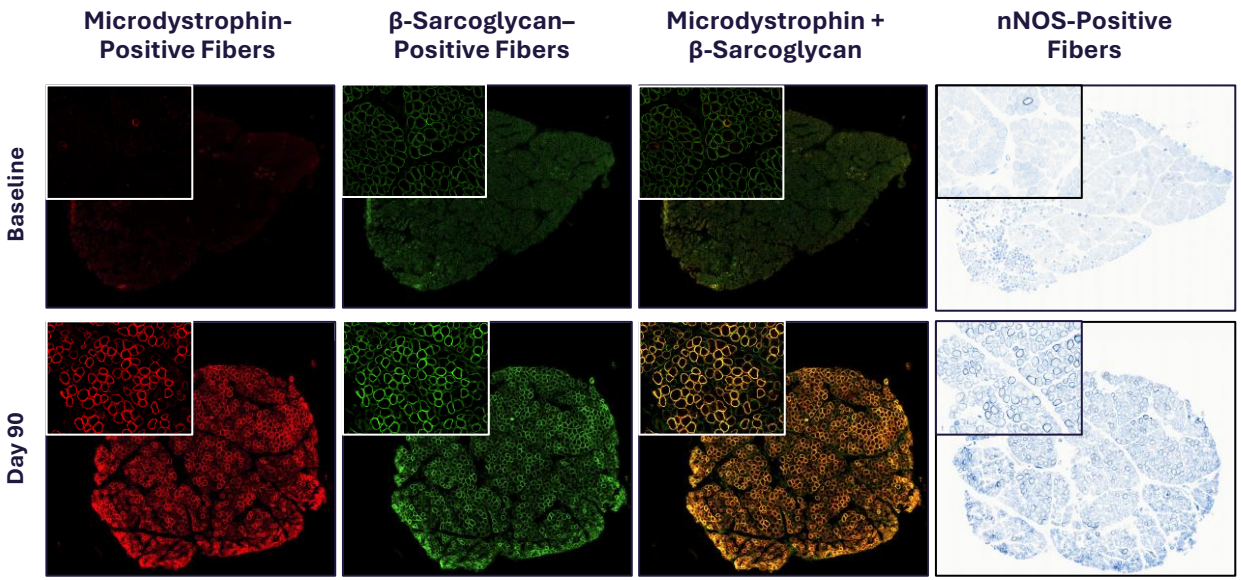
SGT-003 MICRODYSTROPHIN EXPRESSION[1,2,3]

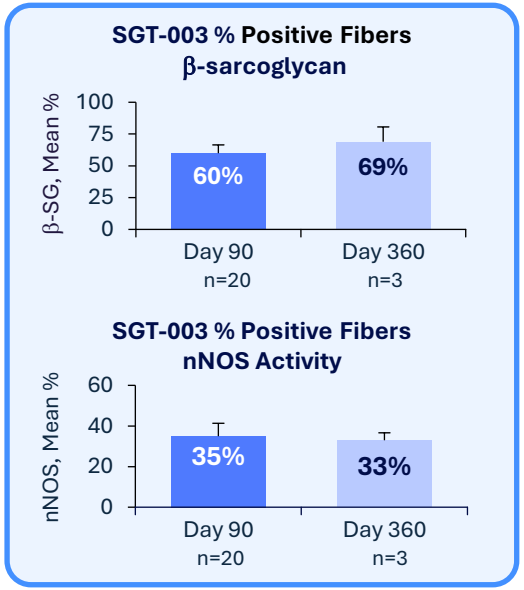


PDPF: percent dystrophin positive fibers. 1. Values are means ± standard error of the mean. 2. Western blot and mass spectrometry baselines were 0% mean normal dystrophin, immunofluorescence is based on a manual count. 3. Western blot, mass spectrometry and immunofluorescence assays are conducted by multiple external vendors; at the time of this analysis, complete results from one western blot sample (from participant 20) and three mass spectrometry samples (from participants 15,16 and 20) had not been received. Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026.

As of February 23, 2026

Restoration of Key Elements of the DAPC Observed After Treatment with SGT-003



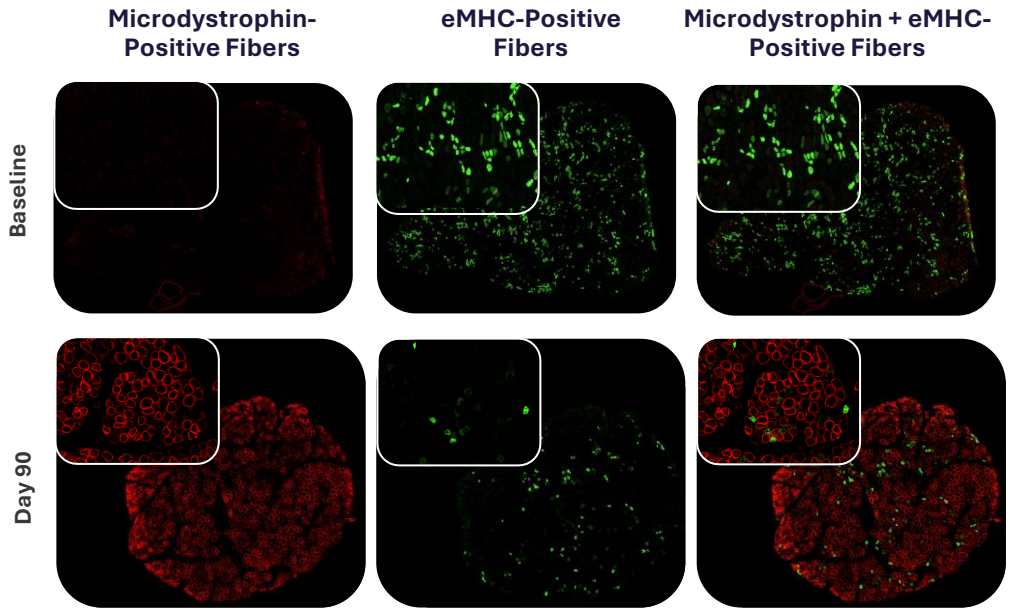


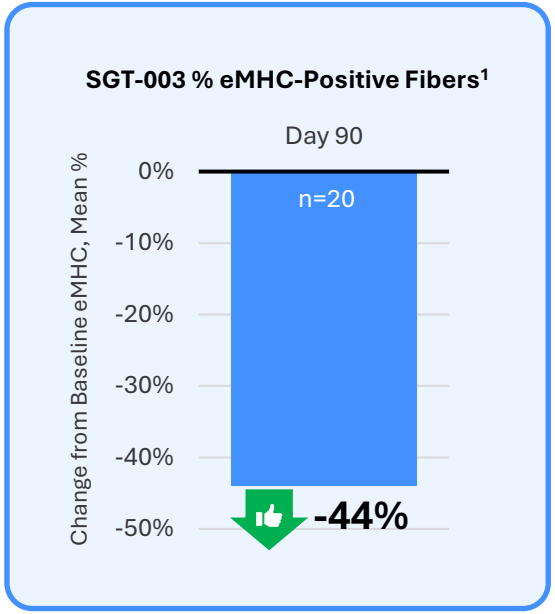
nNOS: neuronal nitric oxide synthase

Beta-sarcoglycan and nNOS were based on a manual count; Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026. Representative images shown.

As of February 23, 2026

Improved Muscle Health Observed Post-SGT-003 Treatment





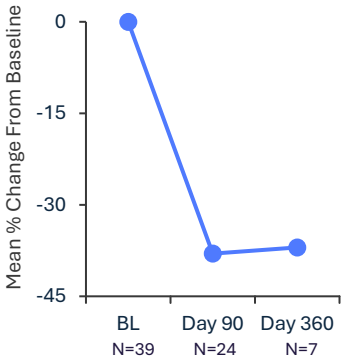
The reduction in eMHC positive fibers suggests reduced need for muscle repair and preservation of muscle architecture

eMHC: embryonic myosin heavy chain
Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026. Representative images shown.

As of February 23, 2026

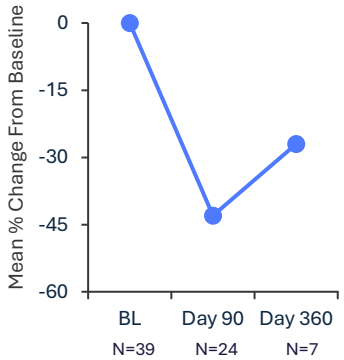
Improved Muscle Integrity Observed After SGT-003 Treatment

SERUM CK % Change From Baseline[1]



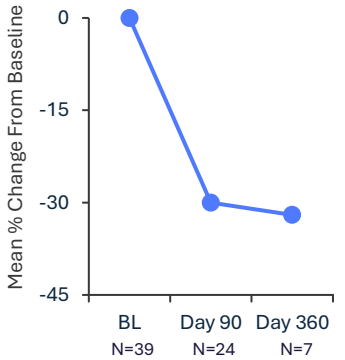
- 38% at Day 90
- 37% at Day 360

SERUM ALT % Change From Baseline[1]



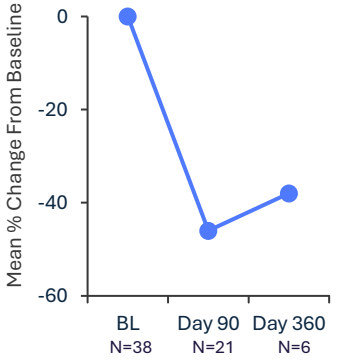
- 43% at Day 90
- 27% at Day 360

SERUM AST % Change From Baseline[1]



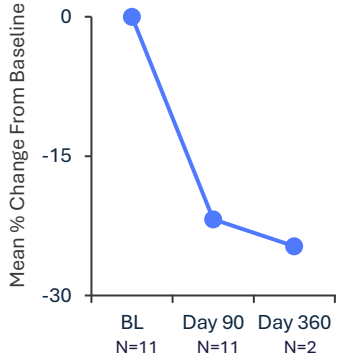
- 30% at Day 90
- 32% at Day 360

SERUM LDH % Change From Baseline[1,2]



- 46% at Day 90
- 38% at Day 360

SERUM Titin % Change From Baseline[1,2]



- 22% at Day 90
- 25% at Day 360

Improved muscle integrity may support slower disease progression and better long-term clinical outcomes[2-4]

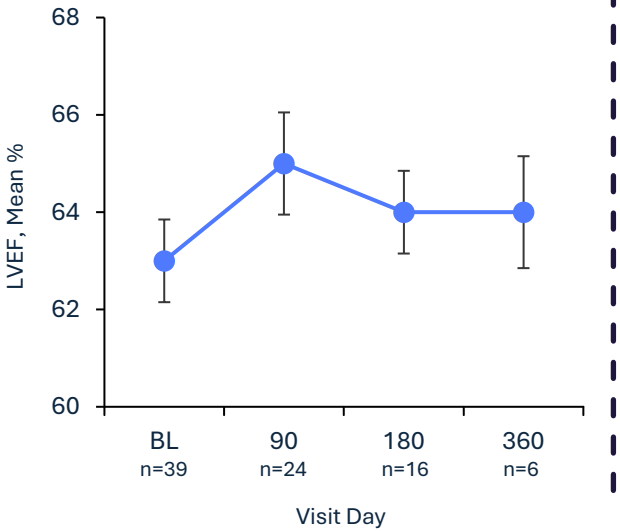
1. Data on file and available at time of analysis. Data cutoff February 23, 2026. Solid Biosciences. 2026. 2. Siddique Ahmed Khan M, et al. *Int J Sci Res*. 2016;5(11):156-157. 3. Voleti S, et al. *Pediatr Cardiol*. 2020;41(6):1173-1179. 4. Oshida N, et al. *Sci Rep*. 2019;9(1):19498. 2. Certain data from a subset of participants were not available at the time of analysis.

As of February 23, 2026

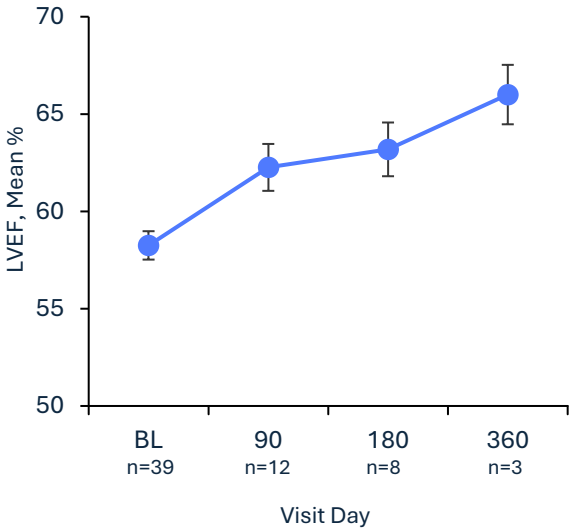
Stable-to-Improved Cardiac Function Observed Post-Treatment

Observations of improved cardiac function are driven by participants with low-normal baseline LVEF[1,2]

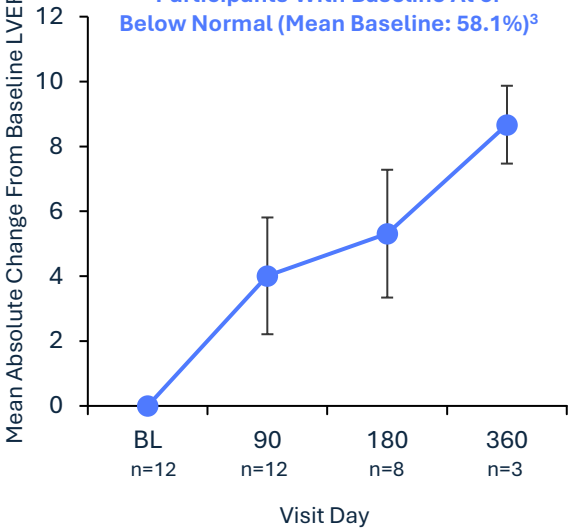
Mean LVEF Over Time, %
All Participants



Mean LVEF Over Time, %
Participants With Baseline At or Below Normal (Mean Baseline: 58.1%)[3]



Mean Absolute Change From Baseline LVEF, %
Participants With Baseline At or Below Normal (Mean Baseline: 58.1%)[3]

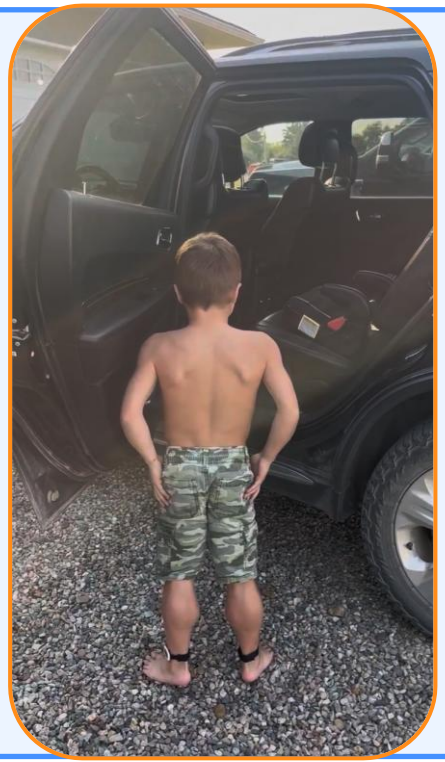


1. Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026. 2. For participants with low-normal baseline LVEF, increases in LVEF are beneficial and decreases unfavorable.[4,5] 3. "Baseline at or below normal" was defined as LVEF ≤60 at baseline.

Participant A: Example of Home-Based Activity Monitoring Analysis to Provide Initial Assessments of Changes in Motor Function

Baseline to Day 360 After SGT-003 Dosing: Getting Into A Car (Choice Activity)

Functional Assessments Will be Analyzed Following Guidance From Regulators



Baseline

Day 360

This video depicts an individual participant treated with SGT-003, Solid Biosciences' investigational Duchenne gene therapy. Individual results may vary and may not be representative of all participants.

Participant B: Example of Home-Based Activity Monitoring Analysis to Provide Initial Assessments of Changes in Motor Function

Baseline to Day 180 After SGT-003 Dosing: Home-Based Stair Climb

Functional Assessments Will be Analyzed Following Guidance From Regulators

Baseline

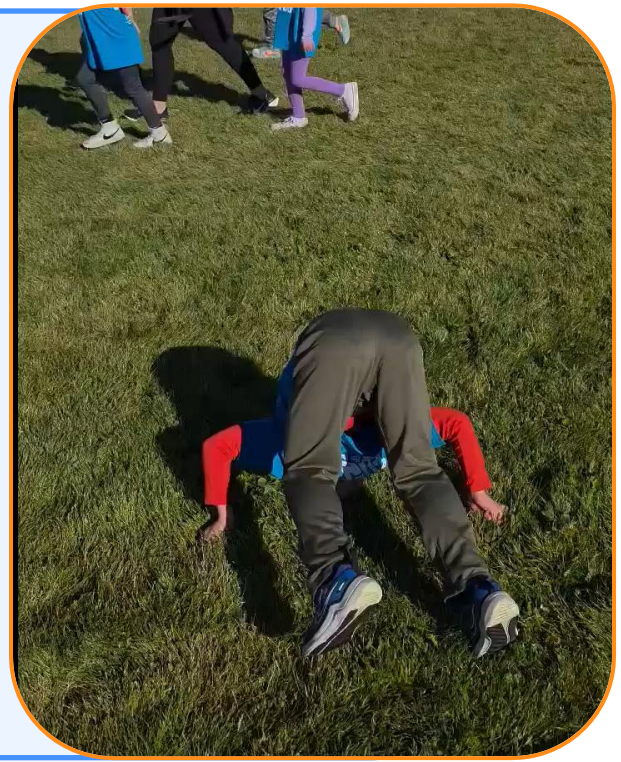
Day 180

This video depicts an individual participant treated with SGT-003, Solid Biosciences' investigational Duchenne gene therapy. Individual results may vary and may not be representative of all participants.

Participant B: Example of Patient-Chosen Activity to Provide Initial Assessments of Changes in Motor Function

Head Stand – Day 180 After SGT-003 Dosing

Functional Assessments Will be Analyzed Following Guidance From Regulators

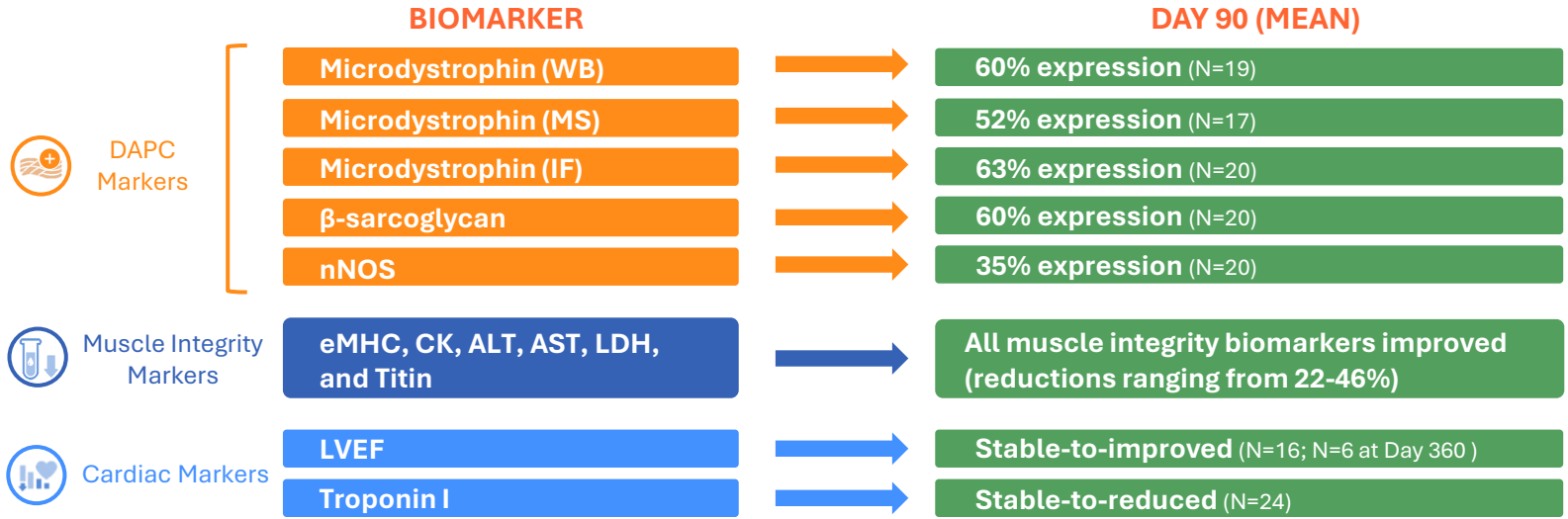


Day 180

This video depicts an individual participant treated with SGT-003, Solid Biosciences' investigational Duchenne gene therapy. Individual results may vary and may not be representative of all participants.

As of February 23, 2026

SGT-003: Encouraging Clinical and Safety Profile



- SGT-003 is administered at a 1.0E14 vg/kg dose
- Robust microdystrophin expression observed after treatment with SGT-003
- SGT-003 uses a lower-burden, steroid-only immunomodulation regimen and has been generally well tolerated in 46 participants dosed to date[1]

1. Data cutoff May 4, 2026. Data on file. Solid Biosciences. 2026.

Acknowledgments

INSPIRE DUCHENNE

Scan the QR code to navigate to the study posting (NCT06138639)

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