August 2026
Corporate Presentation

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Clinical Stage Genetic Medicines Company Targeting Neuromuscular and Cardiac Diseases

Program	Indication	Research / Discovery	Preclinical	Phase 1/2	Phase 3	Worldwide Rights
Neuromuscular[1]						
SGT-003	Duchenne	INSPIRE DUCHENNE IMPACT DUCHENNE				✓
SGT-212	FA	FALCON				✓
Cardiac						
SGT-501	CPVT (RYR2)	ARTEMIS				✓
	CPVT (CASQ2)					✓
SGT-601	DCM (TNNT2)					✓
SGT-401	DCM (BAG3)					✓
SGT-701	DCM (RBM20)					✓
Mayo Clinic Collaboration	6 Undisclosed Targets					✓
Platform						
Capsid Library[2]	Cardiac & Neuromuscular					✓

1. In 2020, Solid entered into a collaboration agreement with Ultragenyx for the development of UX810, a next-generation Duchenne construct comprised of Solid's proprietary nNOS microdystrophin and Ultragenyx's PinnacleTM PCL manufacturing platform for use with AAV8 and Clade E variants thereof. Solid has the option to co-fund collaboration programs in return for a profit share or increased royalty payments at proof-of-concept. 2. Cardiac Capsid Library currently in NHPs, mice and pigs.

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SGT-003

Duchenne Muscular Dystrophy

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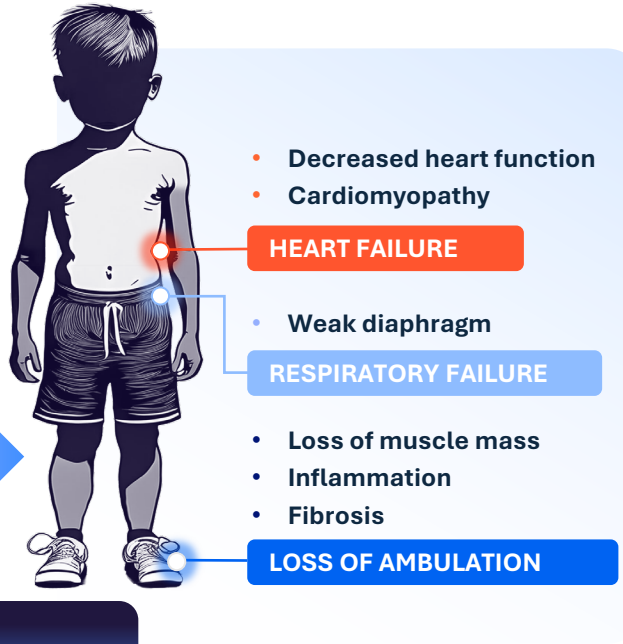
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Duchenne is a Disease of Impaired Muscle Integrity and Dysfunction[1-4]

Muscle integrity is the ability of muscle tissue to remain structurally and functionally whole[1-3]

- Muscle integrity underlies **strength** and **mobility**[1-3]
- Preservation of muscle integrity is critical for **normal muscle function**[1-3]
- Early signals of decline in muscle integrity **predict negative outcomes in certain organs**, such as the heart[5-7]

In Duchenne, muscle integrity deteriorates over time, resulting in difficulties with mobility, thoracic scoliosis, respiratory failure, and cardiac failure[4]



The impact of treatments on muscle integrity for patients with Duchenne is key to determining efficacy[4]

1. Michele DE. *FEBS J.* 2022;289(21):6460-6462. 2. Coronado-Zarco R, de León AO. *J Frailty Sarcopenia Falls*. 2023;8(4):254-260. 3. Collins KH, et al. *Front Physiol*. 2018;9:112. 4. Escobar-Huertas JF, et al. *Cytoskeleton (Hoboken)*. 2024;81(6-7):269-286. 5. Sheybani A, et al. *Pediatr Res*. 2022;92(6):1613-1620. 6. Voleti S, et al. *Pediatr Cardiol*. 2020;41(6):1173-1179. 7. Wagner KR, et al. *Biomark Med*. 2021;15(15):1389-1396.

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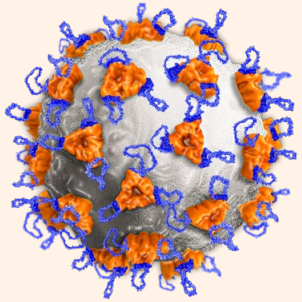
SGT-003 is Optimized for Robust Function and Transduction in Skeletal and Cardiac Muscle

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.

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Current INSPIRE DUCHENNE Participant Disposition by Data Type[1]

	Day 90[4]	Day 360[4]
Participants who have been dosed and have reported safety data[2]	N=50/53	
Participants with biopsy data/DAPC markers[3]	n=20	n=3
Participants with serum biomarker data/muscle integrity markers[5]	n=24	n=7
Participants with cardiac data/markers[6]	n=24	n=6

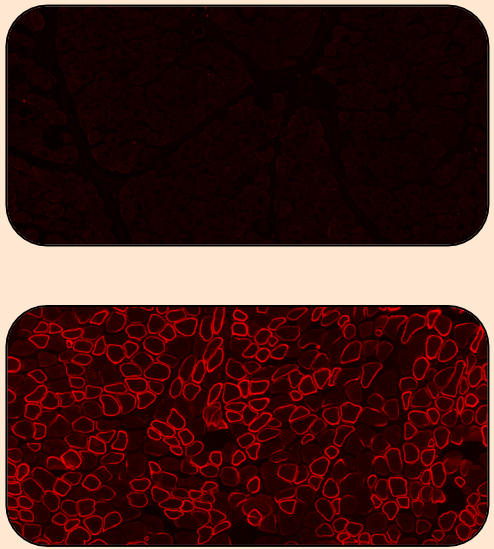
1. Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026. 2. Participants 51-53 were dosed after the safety data cutoff of May 29, 2026. 3. DAPC markers include microdystrophin, β-sarcoglycan, nNOS activity, and eMHC. 4. Certain data from a subset of participants were not available at the time of analysis. 5. Muscle integrity markers include CK, ALT, AST, LDH, and Titin. 6. Cardiac markers include LVEF and troponin.

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SGT-003 Demonstrated Robust Microdystrophin Transduction & Expression

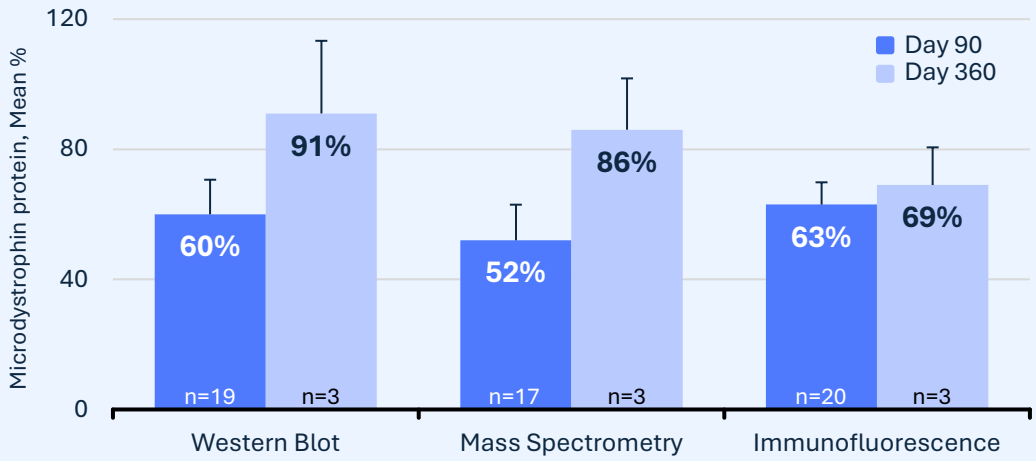
EXAMPLE SGT-003 MICRODYSTROPHIN BIOPSY



VECTOR GENOME COPIES (MEAN)

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

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

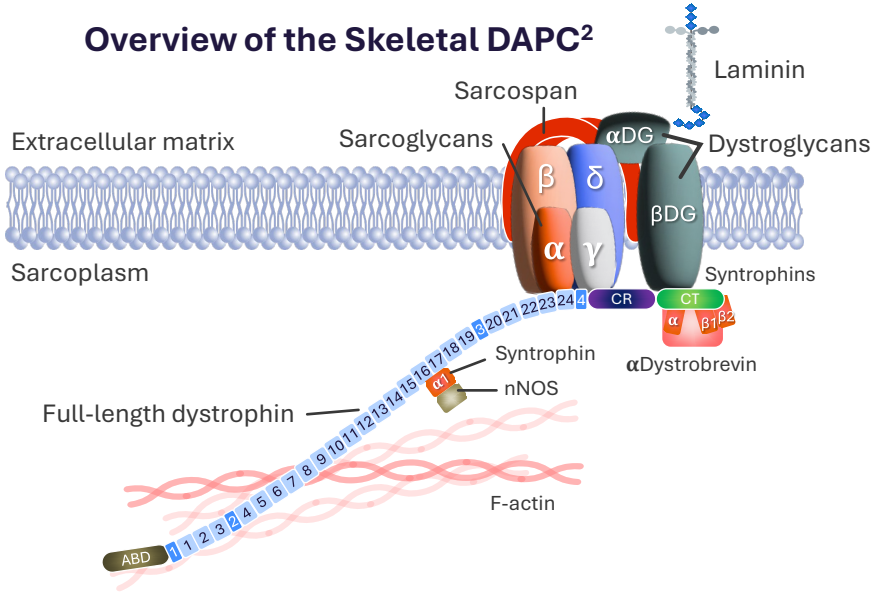


1. Values are means ± standard error of the mean. 2. Western blot and mass spectrometry baselines were 0% mean normal dystrophin, immunofluorescence is based on a manual count. 3. Western blot, mass spectrometry and immunofluorescence assays are conducted by multiple external vendors; at the time of this analysis, 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.

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Assessment of the Dystrophin-Associated Protein Complex (DAPC) Informs Treatment Impact on Muscle Integrity[1]

Dystrophin is a key component of the DAPC, which is an essential structure for muscle integrity[1]



The DAPC consists of 3 main functional domains[1]

- Extracellular: α-dystroglycan, laminin-binding
- Transmembrane: β-dystroglycan, **sarcoglycans**, sarcospan
- Intracellular: **dystrophin**, syntrophins, dystrobrevin, **nNOS**

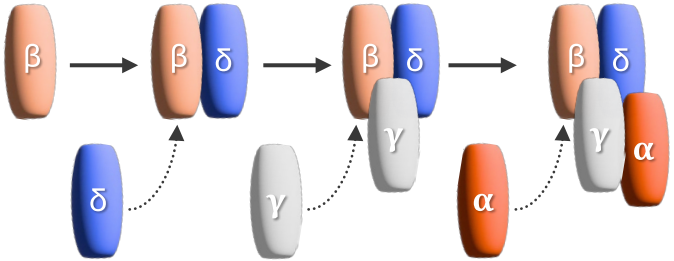
In Duchenne, the absence of dystrophin destabilizes the entire DAPC, triggering a cascade of structural, signaling, and metabolic defects that impair muscle integrity[3,4]

DAPC=dystrophin-associated protein complex. 1. Gao QQ, McNally EM. *Compr Physiol*. 20151;5(3):1223-1239. 2. Wilson DGS, et al. *Commun Biol*. 2022;5(1):1022. 3. Hack AA, Groh ME, McNally EM. *Microsc Res Tech*. 2000;48(3-4):167-80. 4. Hack AA, et al. *Proc Natl Acad Sci USA*. 1999;96(19):10723-8.

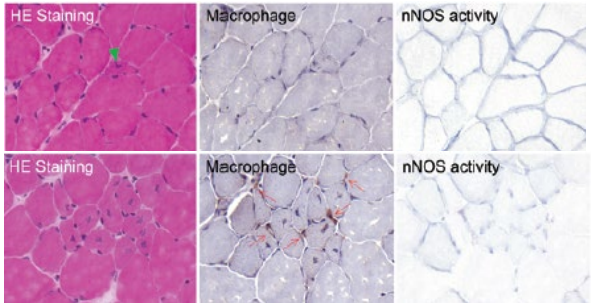
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β-sarcoglycan and nNOS are Necessary for Proper DAPC Function

SARCOGLYCAN COMPLEX FORMATION[1]



MICRODYSTROPHIN CONTRUCTS WITHOUT R16/R17 CANNOT RECRUIT nNOS[4]



- β-sarcoglycan tightly associates with δ-sarcoglycan to form a functional core that recruits γ- and α-sarcoglycan[2]
- Disruption of the β/δ core interferes with association of the sarcoglycan complex to the plasma membrane[3]

- Lack of nNOS at the sarcolemma leads to impaired NO-mediated vasodilation, functional ischemia, muscle fatigue and breakdown[5]
- Restoration of properly localized nNOS helps prevent muscle wasting, regulate muscle contraction, protect against oxidative stress, and is essential to more fully protect cardiac and skeletal muscle[5]

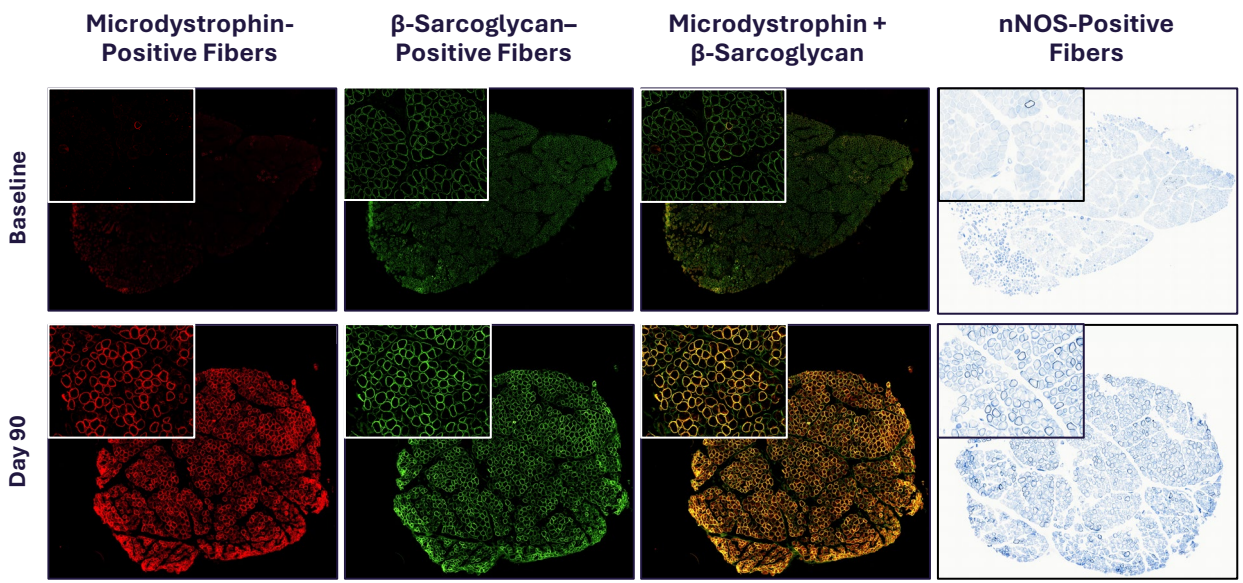
α=alpha; β=beta; δ=delta; γ=gamma.

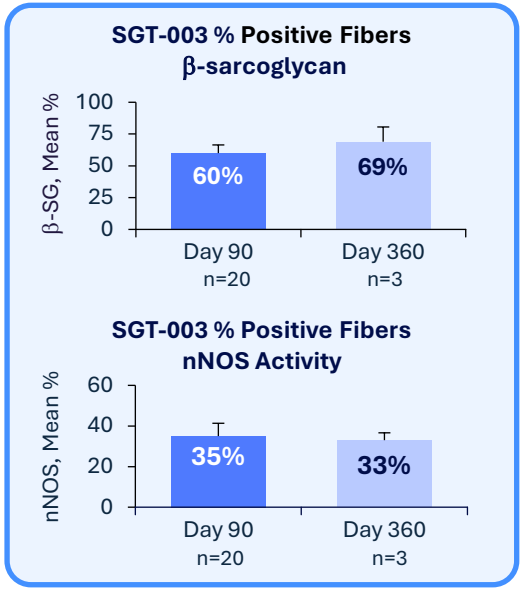
1. Gao QQ, McNally EM. *Compr Physiol*. 2015;5(3):1223-39. 2. Tarakci H, Berger J. *Front Biosci (Landmark Ed)*. 2016;21(4):744-756. 3. Shi W, et al. *Muscle Nerve*. 2004;29(3):409-419. 4. Lai Y, et al. *J Clin Invest*. 2009;119(3):624-35. Staining captured after 8 days of intensive treadmill running (6-month-old male mice). 5. Buono R, et al. *Stem Cells*. 2012; 30(2):197-209.

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





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

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INSPIRE DUCHENNE Clinical Progress: 53 Participants Dosed[1]

Safety Profile[2]

- SGT-003 has been generally well tolerated
- 2 treatment-related SAEs (4.0%) resolved

Clinical Profile[3] Microdystrophin (Means)

Vector Genome Copies
11 (Day 90; N=20)
12 (Day 360; N=3)

Microdystrophin (WB)
60% expression (Day 90; N=19)
91% expression (Day 360; N=3)

Microdystrophin (MS)
52% expression (Day 90; N=17)
86% expression (Day 360; N=3)

Microdystrophin (IF)
63% expression (Day 90; N=20)
69% expression (Day 360; N=3)

Clinical Profile[3] DAPC Restoration (Means)

β-sarcoglycan
60% positive fibers (Day 90; N=20)
69% positive fibers (Day 360; N=3)

nNOS
35% positive fibers (Day 90; N=20)
33% positive fibers (Day 360; N=3)

1. N=53 dosed as of August 4, 2026. 2. Participants 51-53 were dosed after the safety data cutoff of May 29, 2026.
3. Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026.

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Monitoring of Muscle Integrity Offers a Powerful Approach to Assess Disease Trajectory and Treatment Effectiveness

Disease Features	How It's Measured	What Improvements Demonstrate
1 Sarcolemmal damage[1]	CK[1], AST and ALT[2]	Membrane stability[1]
2 Contractile scaffold damage[3]	Titin[3]	Sarcomere preservation[3]
3 Tissue damage	LDH[4] Troponin I[5]	Reduced tissue injury[4,6]
4 Muscle necrosis	Functional endpoints LVEF[6]	Reduced cellular damage[2,5]
5 Muscle regeneration	eMHC[7-9]	Muscle fiber stability and satellite cell preservation[7-9]

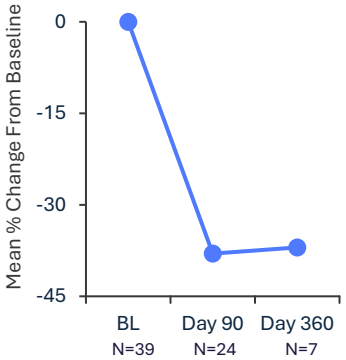
1. Fortunato F, Ferlini A. *J Neuromuscul Dis*. 2023;10(6):987-1002. 2. Aulbach AD, Amuzie CJ. A Comprehensive Guide to Toxicology in Nonclinical Drug Development (Second Edition). 2017. 3. Herzog W. *Biophys Rev*. 2018;10(4):1187-1199. 4. Farhana A, Lappin SL. *StatPearls* [Internet]. 2023. 5. Sheybani A, et al. *Pediatr Res*. 2022;92(6):1613-1620. 6. Romanowicz J, et al. *J Am Soc Echocardiogr*. 2023;36(3):310-323. 7. Guiraud S, et al. *Hum Mol Genet*. 2019;28(2):307-319. 8. Dubuisson N, et al. *Int J Mol Sci*. 2022;23(24):16080. 9. Cardone N, et al. *Acta Neuropathol Commun*. 2023;11(1):167.

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Improved Muscle Integrity and Resilience Observed After SGT-003 Treatment

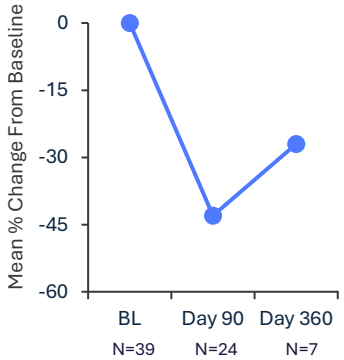
SERUM CK % Change From Baseline[1]



38% at Day 90

37% at Day 360

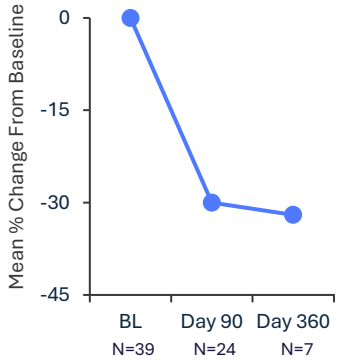
SERUM ALT % Change From Baseline[1]



43% at Day 90

27% at Day 360

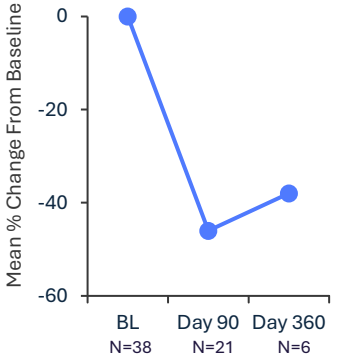
SERUM AST % Change From Baseline[1]



30% at Day 90

32% at Day 360

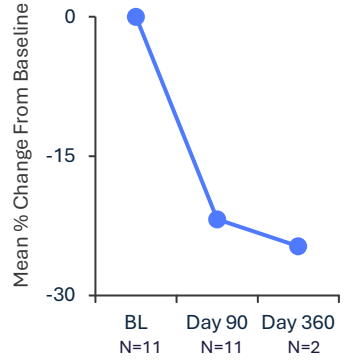
SERUM LDH % Change From Baseline[1,2]



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]

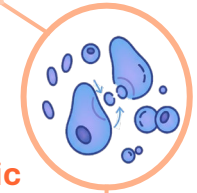
1. Data on file and available at time of analysis. Data cutoff February 23, 2026. Solid Biosciences. 2026. 2. Siddique Ahmed Khan M, et al. *Int J Sci Res*. 2016;5(11):156-157. 3. Voleti S, et al. *Pediatr Cardiol*. 2020;41(6):1173-1179. 4. Oshida N, et al. *Sci Rep*. 2019;9(1):19498. 2. Certain data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

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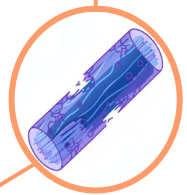
Loss of Dystrophin Creates a Vicious Cycle of Dystrophic Regeneration That Drives eMHC Expression[1-3]



Muscle fiber damage and degeneration[3,4]



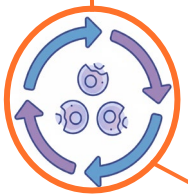
Satellite cell activation and proliferation; **eMHC is expressed[2-4]**



Newly formed muscle fibers[3,4]



Continuous, incomplete muscle repair[2,4,5]



Satellite cell exhaustion[6]



Progressive fibrosis and muscle failure[6]

Cycle of Dystrophic Regeneration in Duchenne

Over time, the chronic cycle of muscle damage and repair will lead to **muscle weakness**

Muscle regeneration is futile: **new fibers will be dystrophic**

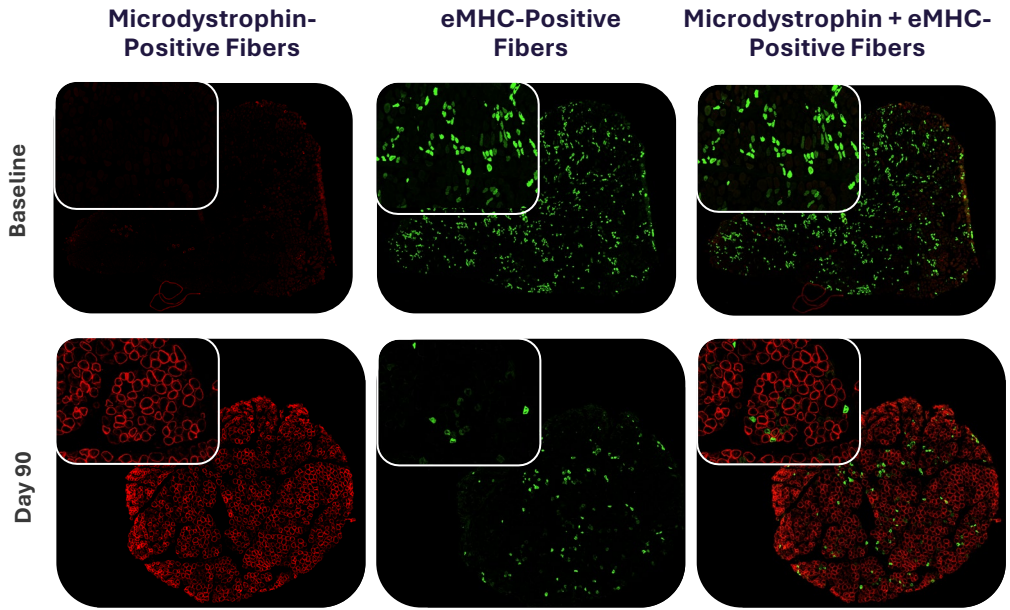
eMHC=embryonic myosin heavy chain.

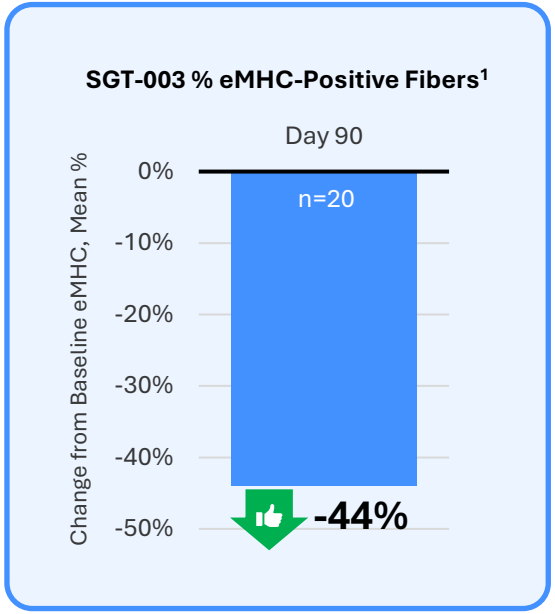
1. Schiaffino S, et al. *Skelet Muscle*. 2015;5:22. 2. Guiraud S, et al. *Hum Mol Genet*. 2019;28(2):307-319. 3. Dubuisson N, et al. *Int J Mol Sci*. 2022;23(24):16080. 4. Cardone N, et al. *Acta Neuropathol Commun*. 2023;11(1):167. 5. Forcina L, et al. *Cells*. 2020;9(5):1297. 6. Abdel-Sama E, et al. *Acta Myol*. 2009;28(3):94-100.

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Reduced Chronic Regenerative Demand Observed Post-SGT-003 Treatment





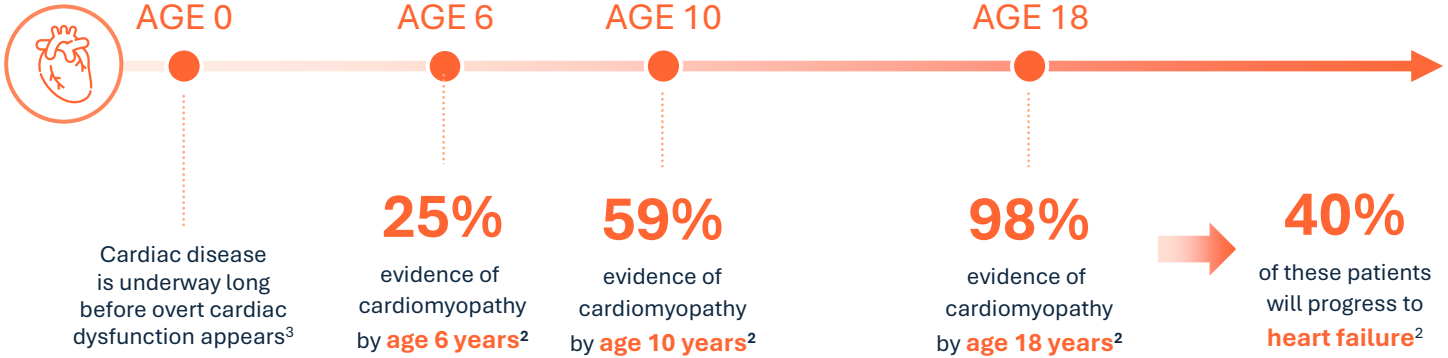
The reduction in eMHC suggests reduced need for muscle repair and preservation of muscle architecture

Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026. Representative images shown.

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Loss of Dystrophin Leads to Progressive Degeneration of Cardiac Muscle[1]

INCIDENCE OF DUCHENNE-RELATED CARDIOMYOPATHY OCCURS EARLY IN LIFE[2]



Cardiac tissue has limited regenerative capacity: only ~1% of cardiomyocytes turn over annually[4]

Cardiomyopathy is a leading cause of death in Duchenne[5]

1. Schultz TI, et al. *JACC Basic Transl Sci.* 2022;7(6):608-625. 2. Gandhi S, et al. *Cells.* 2024;13(14):1168. 3. James J, et al. *Neuromuscul Disord.* 2011;21(7):462-467. 4. Parmacek MS, Epstein JA. *N Engl J Med.* 2009;361(1):86-88. 5. Meyers TA, et al. *Int J Mol Sci.* 2019;20(17):4098.

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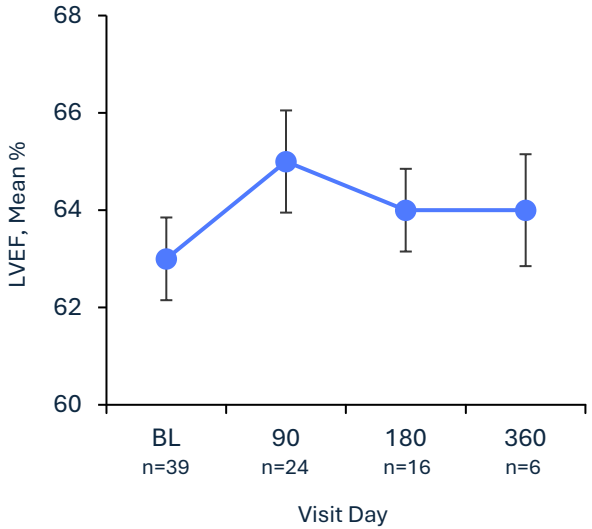
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Stable-to-Improved Cardiac Function Observed Post-Treatment

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

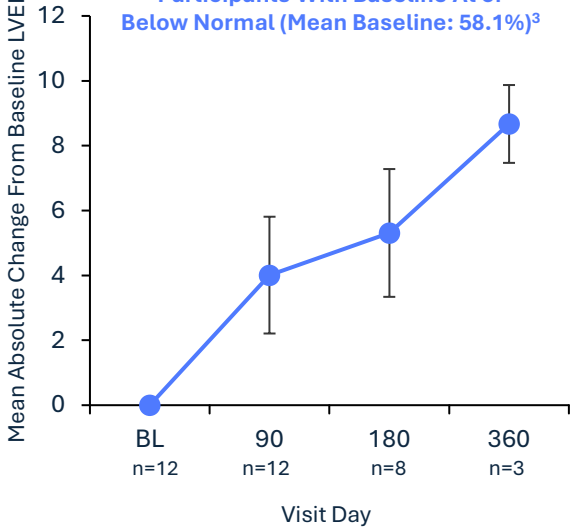
Mean LVEF Over Time, %

All Participants



Mean Absolute Change From Baseline LVEF, %

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



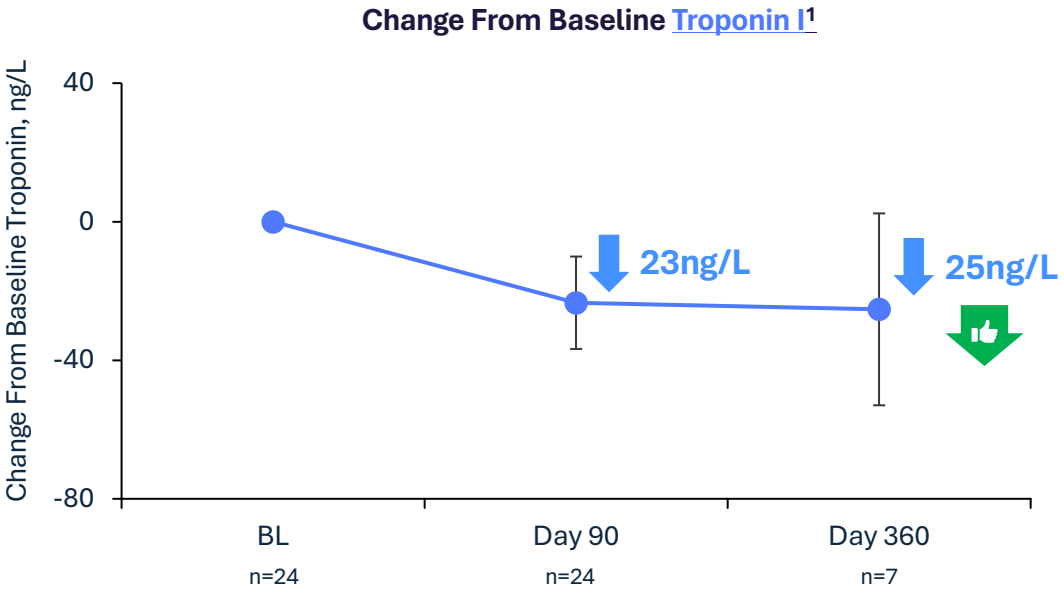
1. Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026. 2. For participants with low-normal baseline LVEF, increases in LVEF are beneficial and decreases unfavorable. 3. "Baseline at or below normal" was defined as LVEF ≤60 at baseline: Romanowicz J, et al. *J Am Soc Echocardiogr*. 2023;36(3):310-323.

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As of February 23, 2026

Stable-to-Improved Troponin I Levels Observed Following SGT-003 Treatment

Changes in troponin I levels were driven by participants with elevated troponin at baseline[1]



Early troponin elevation is predictive of severe cardiac disease in neuromuscular diseases[2-5]

In muscular dystrophies, a hs-cTnI level >7.6 ng/L is correlated with a 3-fold increased risk of cardiac disease[6]

Early detection of changes in the heart using troponin inform interventions to slow disease progression, improve quality of life, and lower the risk of severe cardiomyopathy[7]

1. Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026. 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. Saunders JT, et al. *Circulation.* 2011;123(13):1367-1376. 6. Spurney CF, et al. *Open Heart.* 2021;8(1):e001592. 7. D'Amario D, et al. *Heart.* 2017;103(22):1770-1779.

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INSPIRE DUCHENNE Clinical Progress: 53 Participants Dosed[1]

Safety Profile[2]

- SGT-003 has been generally well tolerated
- 2 treatment-related SAEs (4.0%) resolved

Clinical Profile[3] Cardiac Health (Means)

LVEF
Stable-to-improved
(Day 180; N=16) (Day 360; N=6)

Troponin I
Stable-to-improved
(Day 90; N=24) (Day 360; N=7)

Clinical Profile[3] Muscle Integrity (Means)

CK
38% reduction (Day 90; N=24)
37% reduction (Day 360; N=7)

LDH
46% reduction (Day 90; N=21)
38% reduction (Day 360; N=6)

ALT
43% reduction (Day 90; N=24)
27% reduction (Day 360; N=7)

Titin
22% reduction (Day 90; N=11)
25% reduction (Day 360; N=2)

AST
30% reduction (Day 90; N=24)
32% reduction (Day 360; N=7)

eMHC
44% reduction (Day 90; N=20)

1. N=53 dosed as of August 4, 2026. 2. Participants 51-53 were dosed after the safety data cutoff of May 29, 2026.
3. Data on file. Data cutoff February 23, 2026. Solid Biosciences. 2026.

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SGT-003 Uses a Low-Burden, Steroid-Only Immunomodulatory Regimen

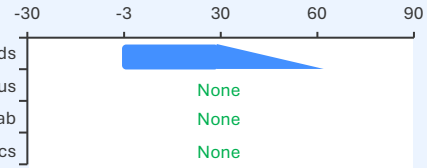
Prophylactic Immunomodulation Regimens With Use of Duchenne Gene Therapies

	SGT-003[1]	Other Therapies
Corticosteroids	DURATION: ~33 DAYS	DURATION: ~60 DAYS
Eculizumab	NO USE OF ECULIZUMAB	**Increased risk of meningococcal disease (according to eculizumab's boxed warning)[2]**
Sirolimus	NO USE OF SIROLIMUS	**Increased risk of cytopenia, impaired wound healing, and lung toxicity (according to sirolimus' label)[3]**

SGT-003 uses a low-burden prophylactic immunomodulatory regimen supported by rigorous safety monitoring

SGT-003[1]

Duration (Days) in Relation to Dosing Day 0



Pulse steroids	
Sirolimus	None
Eculizumab	None
Prophylactic antibiotics	None
Increased Risk Exposure Due to Immune Regimen	
Metabolic/osteopathic risk	✓
Opportunistic infection	None
Meningococcal infection	None
Progressive multifocal leukoencephalopathy	None
Lymphoma risk	None
Poor wound healing/myositis	None
Renal injury	None
Observed Adverse Events	
Drug-induced liver injury	None
Myocarditis/cardiomyopathy	None
Risk of gene therapy–associated death	None
Resource Requirements	
Required sirolimus toxicity monitoring	None
REMs prescribing requirement for eculizumab	None
Significant demand for healthcare resources	None
Vaccination status management	None

1. Data on file as of February 23, 2026. Solid Biosciences. 2026. 2. Soliris. Prescribing information. Alexion Pharmaceuticals Inc; 2025. 3. Rapamune. Prescribing information. Pfizer Inc; 2024.

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Utilizing ALT, AST and GGT as Signals of Both Liver and Muscle Health

ALT and AST[1-3]

ALT and AST are routinely considered markers of liver health, but in Duchenne, are substantially increased due to release from damaged skeletal muscle.



AST and ALT, leaked from the liver, are the **most sensitive markers of acute hepatocellular injury**



ALT and AST, highly concentrated in skeletal muscle cells, **leak into the serum** when the absence of dystrophin leads to **membrane damage**

GGT[1]

GGT, which is not expressed in skeletal muscle, helps distinguish whether elevated transaminases originate from the liver or from dystrophic muscle.



GGT is found in liver and biliary epithelial cells and acts as a **marker of hepatobiliary disease**



Normal GGT in the presence of elevated ALT and AST points to **muscle damage rather than hepatic injury**

1. Aulbach AD, Amuzie, CJ. A Comprehensive Guide to Toxicology in Nonclinical Drug Development (Second Edition). 2017. 2. Liver Fellow Network. Accessed July 21, 2025. https://www.aasld.org/liver-fellow-network/core-series/back-basics/how-approach-elevated-liver-enzymes 3. Khalid A, et al. Cureus. 2025;17(9):e92233.

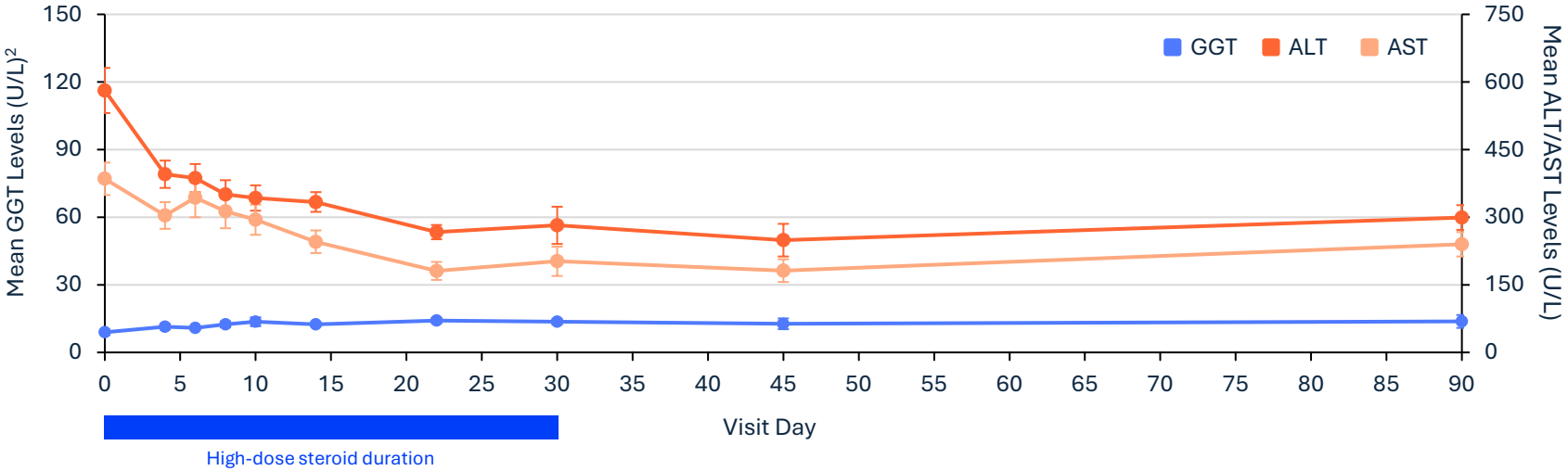
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As of February 23, 2026

Liver Enzymes Declined or Remained Stable in the Peri-Dosing Period 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

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.

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As of May 29, 2026

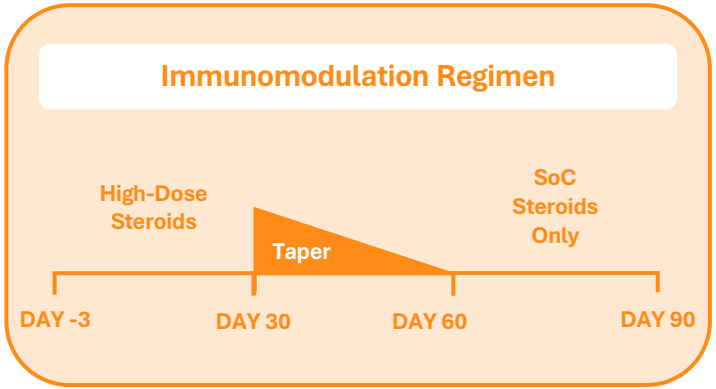
INSPIRE DUCHENNE Interim Safety Data for 50 Participants

Cohorts	Eligible Age Range (years)	Ages at Enrollment (years)	Weights for Dosing (kg)	Participants Enrolled (n)
1-3	0 to <12	6 mo – 10 years	6.5 to 39.7	50/53[1]

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

2. 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 (%)
Most Common Adverse Events (AEs)	Vomiting	32 (64.0)
	Nausea	27 (54.0)
	Decreased appetite	15 (30.0)
	Thrombocytopenia	13 (26.0)
	Abdominal Pain	9 (18.0)



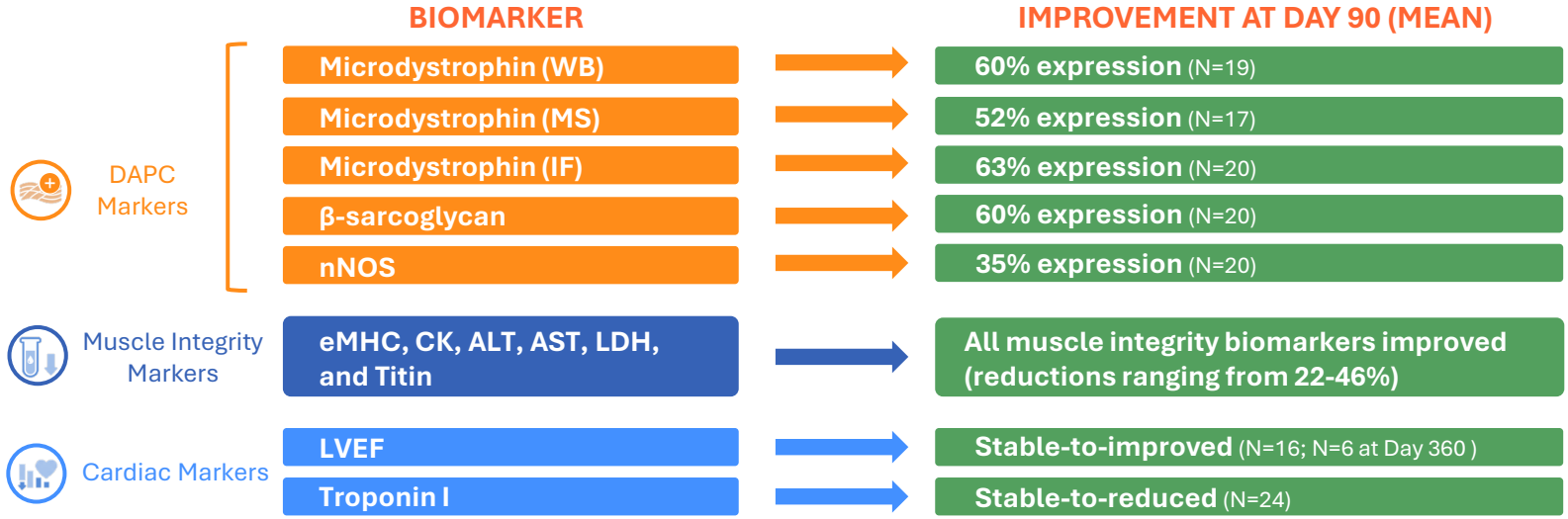
No drug-induced liver injury, thrombotic microangiopathy, atypical hemolytic uremic syndrome, or myocarditis have been observed to date

AE: adverse event; SAE: serious adverse event.
1. Participants 51-53 were dosed after the safety data cutoff of May 29, 2026. Solid Biosciences. 2026.

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As of February 23, 2026

SGT-003: Promising Clinical and Safety Profile



SAFETY SUMMARY

- SGT-003 has been generally well tolerated[1]
- No drug-induced liver injury, thrombotic microangiopathy, atypical hemolytic uremic syndrome, or myocarditis have been observed to date

1. Safety data on file as of May 29, 2026.
All other biopsy and biomarker data on file as of February 23, 2026. Solid Biosciences. 2026.

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SGT-212

Friedreich's Ataxia

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Friedreich's Ataxia (FA): A Progressive Genetic Neuromuscular Disease with High Unmet Medical Need

Affected Population

ESTIMATED

~5,000-7,000



patients in the US[1]

25,000



in EU[2]

PREVALENCE

1:40,000

people[3]

Cause

FA is a monogenic disease resulting from a deficiency of the frataxin (FXN) protein, which is important for mitochondrial function.

Postulated Mechanism: Decreased levels of FXN lead to less efficient energy production and buildup of toxic byproducts, resulting in oxidative stress that damages cells in the central nervous system and heart

Clinical Presentation and Unmet Need

Signs & Symptoms

- FA is a multisystem disease that affects motor control and coordination
- Most have loss of vision and hearing, slurred speech, muscle weakness
- The majority of patients with FA develop cardiac complications, most commonly presenting as hypertrophic cardiomyopathy and arrhythmia
- Cardiac complications are the primary cause of death

Age of Onset & Mortality

- Average onset of disease is between ages 10 and 15
- Average lifespan < 40 years



Solid Approach

Dual route of administration – IV and IDN – to deliver AAV-based gene therapy directly to the heart and cerebellum to restore functional expression of FXN in the heart and central nervous system

1. Koeppen AH. J Neurol Sci. 2011. 2. European Medicines Agency. Public summary of opinion on orphan designation: Omaveloxolone for treatment of Friedreich's ataxia. https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu3182037. 3. Friedreich's Ataxia - Symptoms, Causes, Treatment | NORD. 2023. https://rarediseases.org/rare-diseases/friedreichs-ataxia/.

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mFARS: Quantifying Neurological Function in Friedreich's Ataxia

93

0

	Maximum Score	Key Functions Assessed	Clinical Relevance
Bulbar Function	5	Speech clarity, cough effectiveness	Communication airway protection; stable marker
Upright Stability	36	Balance, stance, gait, ambulation	Highly sensitive to progression; loss of ambulation
Upper Limb Coordination	36	Fine motor skills, dexterity	Daily living tasks (eating, dressing, writing)
Lower Limb Coordination	16	Leg movement, control	Mobility, transfers, standing

- While mFARS is primarily driven by mobility & limb movements, the **bulbar domain reflects high-consequence clinical outcomes**
- In FA patients[1]:
  - ~1/3 exhibit unsafe swallowing patterns
  - Aspiration may occur silently in the absence of an effective cough
- **Bulbar dysfunction becomes increasingly clinically relevant in advanced FA**[2]
- **Even modest improvements** (decrease) in bulbar score post-dose **may signal meaningful functional impact**

The maximum mFARS score is 93 points, with a higher score indicative of more severe disease

mFARS=modified Friedreich's Ataxia Rating Scale. mFARS is administered by trained clinicians using standardized protocols.
1. Astudillo et al., CHEST 2024; 2. Borel et al., Ann Clin Transl Neurol 2019.

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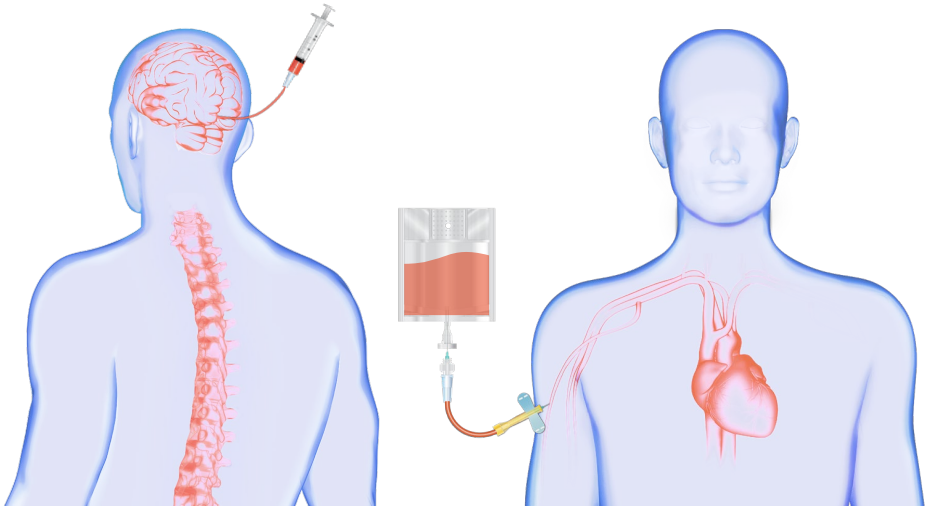
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SGT-212 Leverages Precision Technology to Address Quality of Life and Cardiac Mortality

SGT-212 is the only dual route gene therapy (IDN & IV) approach for the treatment of Friedreich's ataxia – designed to address the neurologic, systemic and cardiac manifestations of the disease

SGT-212:

- *Capsid*: AAVhu68
- *Dosing Cadence*: IDN first, followed by IV
- Ubiquitous promoter



Slowing or halting disease progression is the most meaningful treatment impact for the FA community[1]

Treatment approaches that address both quality of life *and* mortality/lifespan represent the future **standard for FA disease management**

IDN=intradentate nuclei; IV=intravenous

1. Friedreich's Ataxia Research Alliance (FARA) *Voice of the Patient* Survey, 2017.

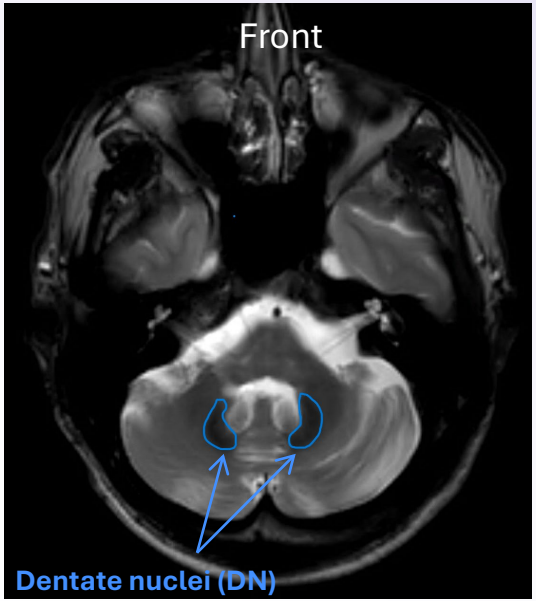
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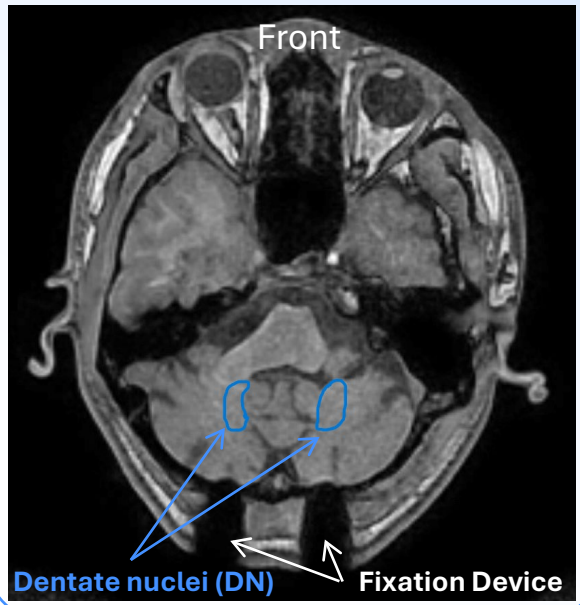
SGT-212: Phase 1b FALCON Trial Participant Dosing

Precision anatomic targeting to the dentate nuclei enables direct engagement of the critical disease driver

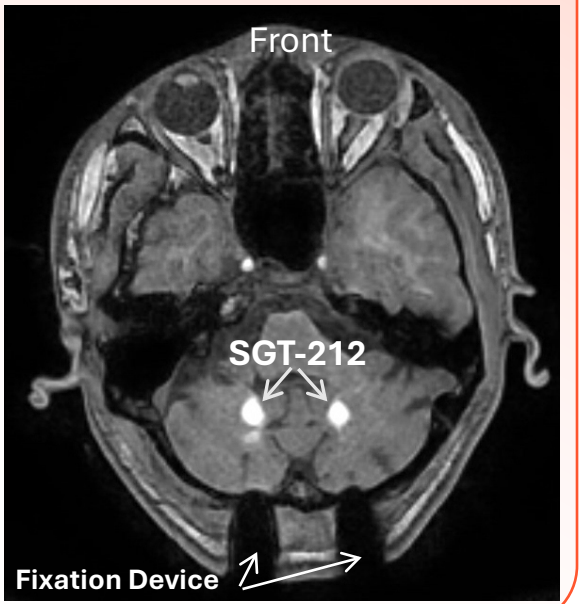
Baseline MRI



Pre-Dose With SGT-212



Post-Dose With SGT-212



Intra-procedural MRI-imaging confirmed precise delivery of SGT-212 into the dentate nuclei

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FALCON Phase 1b Trial Design

A first-in-human, open-label, multi-center trial designed (NCT07180355) to evaluate the safety and tolerability of contemporaneous IDN and systemic IV infusion of SGT-212 gene therapy in participants with FA

Cohorts

Study to enroll approximately 10 participants aged 18-40 years with FA and documented cardiac hypertrophy

1. **Non-ambulatory**
2. **Ambulatory**
3. **Ambulatory & Non-Ambulatory** *(Optional)*

Study Endpoints

Primary Endpoint

Incidence and severity of TEAEs from baseline to month 12

Secondary & Exploratory Endpoints

Change from baseline in serum biomarkers

Change from baseline cardiac frataxin expression at Day 90 and Month 18

Change from baseline in assessments measuring key aspects of the disease, such as neuromuscular function, fatigue and speech

Change from baseline cardiac structure and function

FALCON Active Clinical Sites

- The Ohio State University (OSU)
- The Children's Hospital of Philadelphia (CHOP)
- The University of California, Los Angeles (UCLA)

FALCON Enrollment

- Two participants dosed
- SGT-212 has been well tolerated with no treatment-related serious adverse events (TRSAEs) observed as of August 4, 2026

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SGT-501

Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)

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Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT): a Fatal Disorder in a Young Population

Affected Population

~33,000



people[2]

1:10,000

people[2]

Cause

CASQ2 & RYR2 proteins: Regulate cardiac calcium (Ca^{2+}), important for electrical conduction and cardiac contraction / relaxation

Postulated Mechanism: Mutations in RYR2 or CASQ2 genes disrupt Ca^{2+} release into the cytoplasm triggering abnormal contraction and relaxation leading to arrhythmias

Clinical Presentation and Unmet Need

Signs & Symptoms

- Most commonly presents as syncope events or cardiac arrest
- Quality of life severely impacted. Risk of spontaneous arrhythmias and/or sudden death
- Poor Prognosis: Historically up to 50% mortality by age 35[1]

Age of Onset

- Typically identified in younger patients (mean onset between 7-9 y/o)[1]

Standard of Care

- Treatment landscape has not changed in decades: approved treatments – beta blockers and flecainide – do not address the underlying cause of disease, require strict compliance, and have challenging side effects



Solid Approach

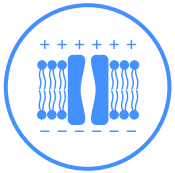
AAV-based delivery of a genetic payload to the heart intended to achieve expression of wild-type CASQ2 protein using a cardiac-selective promoter and an optimized transient transfection manufacturing process

RYR2=Ryanodine receptor, CASQ2= Calsequestrin 2

1. Abbas, et al. *Arrhythm Electrophysiol Rev*. 2022. 2. Priori, et al. *JACC Focus Seminar* 2021

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CPVT Represents High Unmet Need With No Approved Therapies That Treat Underlying Cause of Disease



CPVT is a channelopathy; a genetic mutation affects specific ion channels in cardiomyocytes



Mutations in RYR2 (calcium channel) and CASQ2 (calcium-binding protein) are the most common causes of CPVT



Altered calcium ion channels impact electrical conduction and cardiac contraction – can lead to fatal arrhythmia

Standard CPVT treatments are used off-label, require strict compliance, and have challenging side effects that are life-limiting

- Beta blockers
- Flecainide
- Implantable Cardioverter Defibrillators
- Left Cardiac Sympathetic Denervation

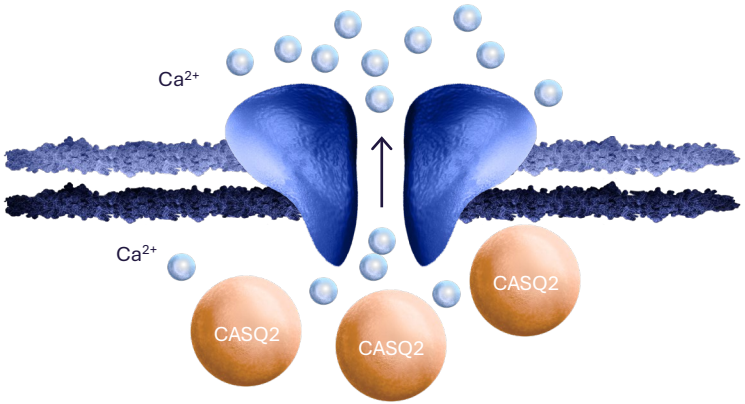
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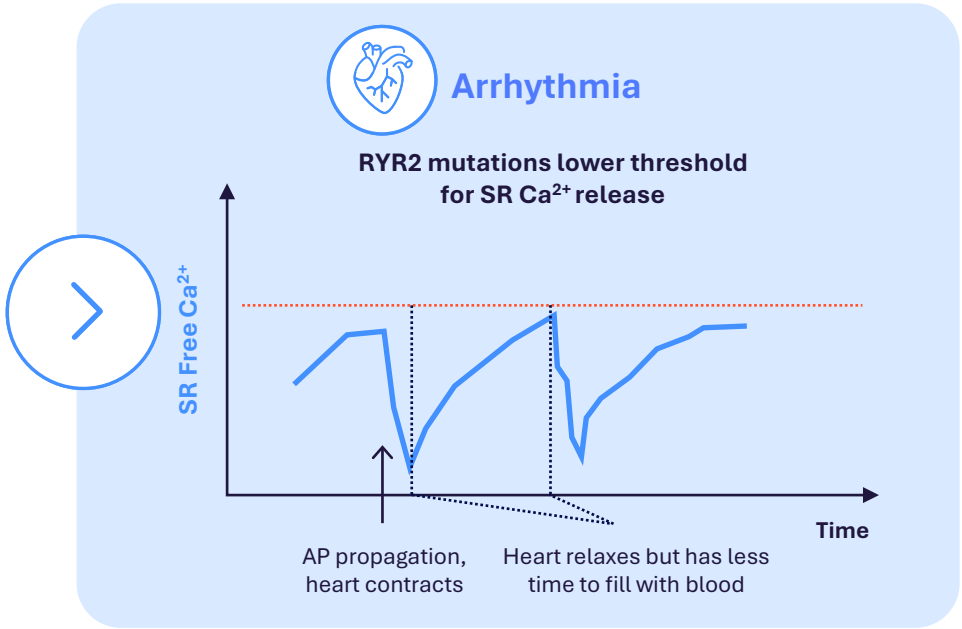
Rationale for CASQ2 Augmentation in RYR2 CPVT

In RYR2 pathogenic mutations, normal CASQ2 levels are insufficient to maintain RYR2 in a closed conformation during diastole in high calcium flux states (such as with adrenaline)

RYR2 Mutation-Related CPVT

Mutations in RYR2 make the channel more sensitive to SR Ca^{2+} levels. This can result in abnormal release of Ca^{2+} in diastole that can lead to delayed afterdepolarizations (DAD) and resultant ventricular arrhythmia





RyR, Ryanodine Receptor; SR, sarcoplasmic reticulum; AP, action potential; Ca2+, calcium

Sources: Proprietary data from Priori Lab

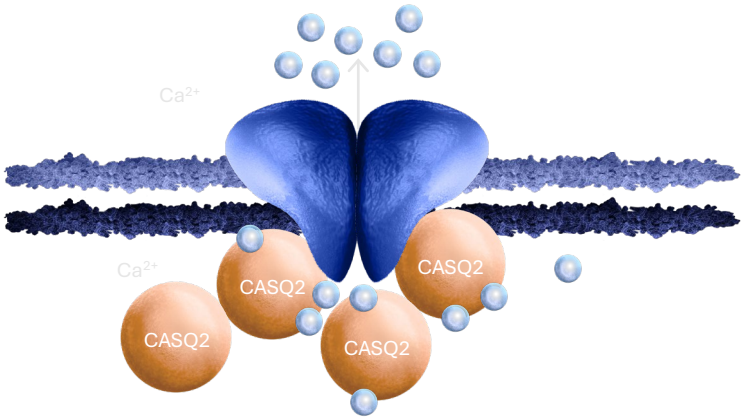
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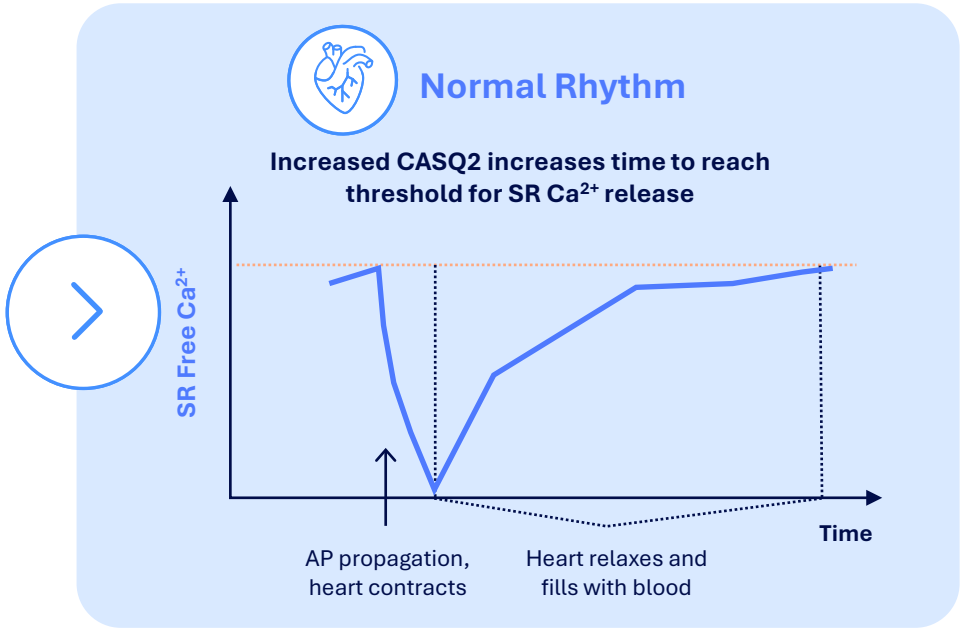
Rationale for CASQ2 Augmentation in RYR2 CPVT (cont.)

Cardiac delivery of SGT-501 is intended to increase CASQ2, thus enhancing Ca2+ buffering and counteracting Ca2+ sensitivity caused by RYR2 pathogenic mutations

RYR2 Mutation-Related CPVT + Increased CASQ2 expression

Increased CASQ2 enhances Ca^{2+} buffering within the SR and helps stabilize RYR2 in the closed state in diastole, reducing or eliminating the probability of delayed afterdepolarizations (DAD) and resultant ventricular arrhythmia





RyR, Ryanodine Receptor; SR, sarcoplasmic reticulum; AP, action potential; Ca2+, calcium

Sources: Proprietary data from Priori Lab

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RYR2 CPVT Transgenic Mouse Model Used To Support Proof-of-Concept For AAV Gene Delivery of Human CASQ^2

ECG response to β-adrenergic stimulation in WT and RYR2 transgenic mice 85 days post vehicle or SGT-501 treatment



WT Mice
Dosed With Vehicle
IP dose epinephrine & caffeine

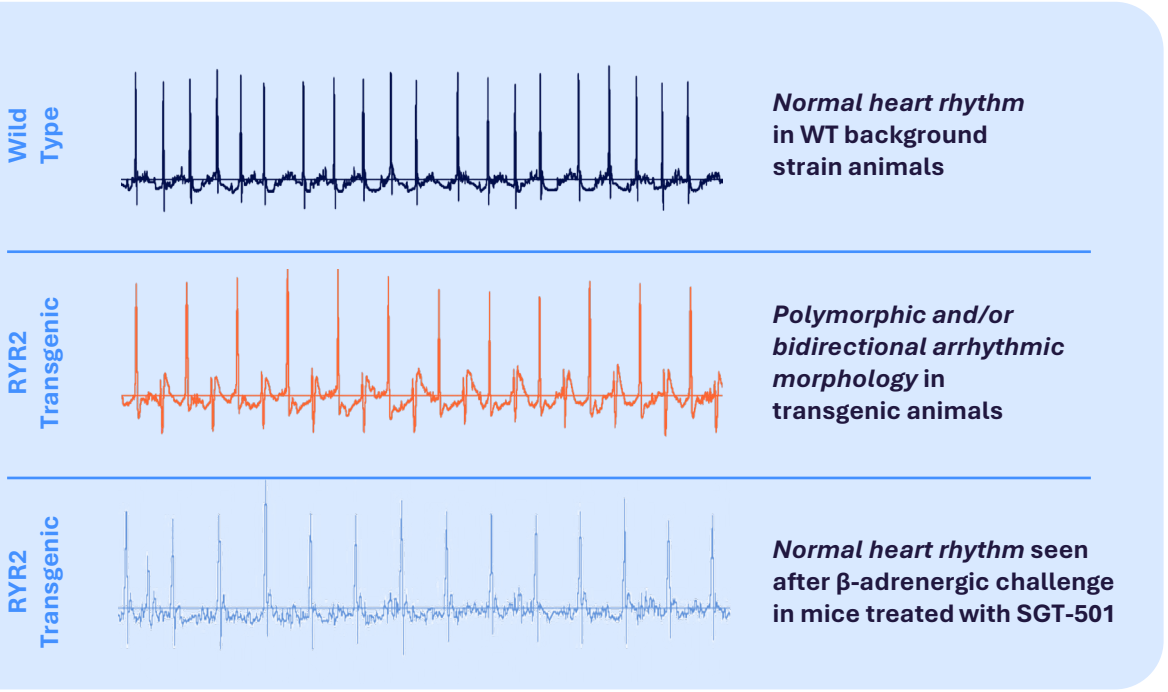


RYR2 Transgenic Mice
Dosed With Vehicle
IP dose epinephrine & caffeine



RYR2 Transgenic Mice
Dosed With SGT-501
IP dose epinephrine & caffeine





WT=Wild Type. Phenotype penetrance in mouse model of disease: ~50%.
Data on file. Solid Biosciences 2025.

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SGT-501 Elicited Steady Cardiac Protein Expression in Mice and NHPs

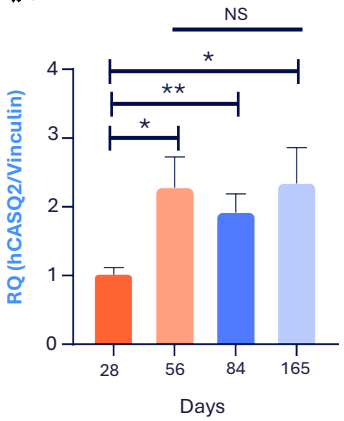
Clinically relevant expression levels continued through month 6 indicating potential durability and stability of expression

hCASQ2 Protein Expression

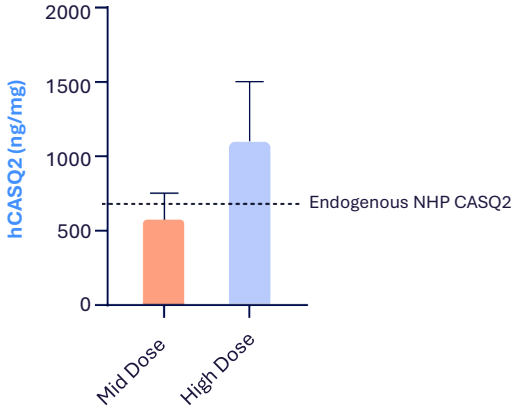
Mouse Kinetics: Expression increased until Day 56, followed by continued durable expression through Day 165

NHP Kinetics: hCASQ2 expression levels were similar between 3- and 6-months post SGT-501 administration. hCASQ2 protein was increased 1.7-fold and 2.3-fold in the Mid- and High-Dose groups compared to endogenous NHP CASQ2 levels, respectively

hCASQ2 Kinetics



6-month hCASQ2



Human CASQ2 expression levels achieved in the High-Dose group were similar to CASQ2 levels measured in human heart samples (1,147 ± 486 ng/mg tissue protein). Data represented as mean ±SEM

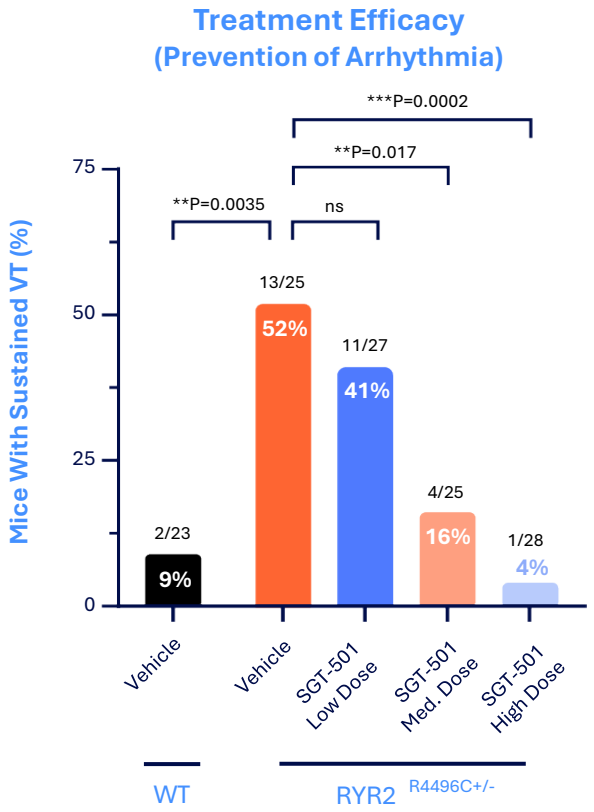
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SGT-501 Demonstrated Protection From Sustained VT & Arrhythmia

SGT-501 demonstrated dose-responsive reduction in adrenaline-mediated VT in RYR2 adult mice

Proof-of-Concept Study Efficacy

SGT-501 treatment resulted in dose-responsive prevention of arrhythmia upon β-adrenergic challenge in an RYR2 transgenic mouse model of CPVT



Data on file. Solid Biosciences 2025.

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SGT-501 was Well Tolerated in NHP GLP Toxicology Study

NHP GLP Tox Study

- 3- and 6-month timepoints
- 6 treatment groups across 3 dose levels
- Evaluated single and triple immunosuppression regimens
- N = 4/group

Findings

- SGT-501 was well tolerated at each evaluated dose level: no adverse effects were observed on hematology or serum clinical chemistry in NHPs after treatment.
- SGT-501 IV administration of SGT-501 resulted in vector biodistribution in NHP cardiac tissue, providing confidence in potential for increased cardiac human CASQ2 expression in CPVT patients.
- Human CASQ2 transgene protein expression was detected only in the heart.

Data on file. Solid Biosciences 2025.

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ARTEMIS Clinical Trial Design: SGT-501 Phase 1b Study

First-in-human, open-label, multi-center study designed to evaluate the safety and tolerability of a single IV infusion of SGT-501 gene therapy in participants with CPVT

Objective

Primary Objective

- To evaluate the safety and tolerability of a single IV infusion of SGT-501 gene therapy in participants with CPVT

Secondary Objectives

- To evaluate the efficacy of SGT-501 by:
  - Assessing arrhythmia burden during exercise
  - Assessing arrhythmia burden over time

Design

Design

Study includes up to **3 cohorts** based on age and on dose level

- Cohort 1: Participants ≥ 18, Dose Level 1
- Cohort 2[1]: Participants ≥ 18, Dose Level 2[2]
- Cohort 3: Participants ≥ 7 to < 18 years of age, dosed level at or below dose(s) assessed in adults[2]

All participants must have a history of life-threatening ventricular arrhythmic event with documented prior history of a VAS score of ≥ 2, and must be on a stable dose of beta-blocker and/or flecainide

Endpoints

Primary Endpoint

Incidence of TEAEs through Day 360

Secondary Endpoints

Change from baseline of VAS on exercise treadmill test at Day 180

Exploratory Endpoints

Change from baseline in the incidence of ventricular arrhythmia at Day 180 with ECG patch

TEAEs=treatment-emergent adverse events
1. Optional for dose exploration; 2. Based on DSMB recommendation

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1. Expected timing, subject to participant enrollment

Anticipated Near-Term Milestones

Program		Milestone	Anticipated Timing
Neuromuscular			
SGT-003 for Duchenne		FDA alignment on: 1) Phase 3 randomized, double-blind, placebo-controlled trial design, 2) that an unmet need remains in Duchenne, 3) and recognition that SGT-003 microdystrophin is novel	✓
	INSPIRE DUCHENNE (Phase 1/2)	Reported updated interim participant Phase 1/2 data (safety, microdystrophin expression & biomarker data)	✓
		Expected meeting with FDA to discuss INSPIRE DUCHENNE SGT-003 data package & to seek guidance on a potential accelerated approval pathway	Late Q4 2026
	IMPACT DUCHENNE (Phase 3)	First participant dosed	✓
SGT-212 for FA	FALCON (Phase 1b)	First participant dosed	✓
		Initial data expected[1]	Q1 2027
Cardiac			
SGT-501 for CPVT	ARTEMIS (Phase 1b)	Phase 1b activation of first clinical trial site	✓
		Dosing of first participant	H2 2026
		Initial safety data expected[1]	H1 2027
SGT-601 for TNNT2		IND-enabling nonclinical package complete	✓
Capsids			
POLARIS-101™ (formerly known as AAV-SLB101)		50+ agreements including licenses executed with corporations, institutions and academic labs	✓
Capsid Library (multiple capsids)		Translational studies underway to enable next-generation cardiac and skeletal muscle capsid selection	H2 2026
Pipeline			
Multiple Pipeline Assets		Mayo Clinic collaboration research/discovery work	Ongoing

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