September 2025

Corporate Presentation

SOLID BIOSCIENCES

Forward Looking Statement

This presentation contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding future expectations, plans and prospects for the company; the ability to successfully achieve and execute on the company’s goals, priorities and achieve key clinical milestones; the company’s SGT-003, SGT-212 and SGT-501 programs, including expectations for additional CTA filings, site activations, expanded clinical development, production of additional SGT-003 GMP batches, initiation and enrollment in clinical trials, dosing, and availability of clinical trial data; the cash runway of the company and the sufficiency of the Company’s cash, cash equivalents, and available-for-sale securities to fund its operations; and other statements containing the words “anticipate,” “believe,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “should,” “target,” “would,” “working” and similar expressions. Any forward-looking statements are based on management’s current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the company’s ability to advance SGT-003, SGT-212, SGT-501, SGT-601, SGT-401 and other programs and platform technologies on the timelines expected or at all; obtain and maintain necessary and desirable approvals from the FDA and other regulatory authorities; replicate in clinical trials positive results found in preclinical studies and early-stage clinical trials of the company’s product candidates; obtain, maintain or protect intellectual property rights related to its product candidates; compete successfully with other companies that are seeking to develop Duchenne, Friedrich’s ataxia, and other neuromuscular and cardiac treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-003, SGT-212, SGT-501, SGT-601, SGT-401 and other candidates, achieve its other business objectives and continue as a going concern. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the company’s actual results to differ from those contained in the forward-looking statements, see the “Risk Factors” section, as well as discussions of potential risks, uncertainties and other important factors, in the company’s most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this presentation represent the company’s views as of the date hereof and should not be relied upon as representing the company’s views as of any date subsequent to the date hereof. The company anticipates that subsequent events and developments will cause the company's views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so.

This presentation contains estimates and other statistical data made by independent parties and by us relating to market size and other data about our industry. This data involves a number of assumptions and limitations, and you are cautioned not to give undue weight to such data and estimates. In addition, projections, assumptions and estimates of our future performance and the future performance of the markets in which we operate are necessarily subject to a high degree of uncertainty and risk.

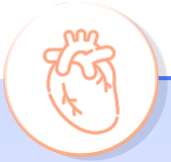
2025 Marks a Transformational Year for Solid Biosciences

With three clinical-stage programs, Solid is poised to become an industry-leading neuromuscular and cardiac precision genetic medicines company

Q1 2025

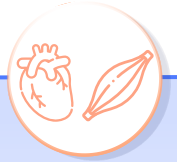
- January: **IND clearance announced for SGT-212**, first-in-class dual route gene therapy for Friedreich's ataxia
- February: First-in-human, **potential best-in-class data** from Phase 1/2 INSPIRE DUCHENNE trial of SGT-003

Mid 2025



- July: **SGT-501 IND clearance & Fast Track designation announced**, first-in-class cardiac gene therapy for CPVT
- July: **Evolution into diversified clinical-stage neuromuscular & cardiac gene therapy company**

Q4 2025



- ❑ **SGT-003:** FDA meeting to discuss regulatory pathways + additional clinical data expected
- ❑ **SGT-501:** Phase 1b initiation of dosing expected
- ❑ **SGT-212:** Phase 1b initiation of dosing expected

Solid is Led by an Experienced Management Team With Significant Industry Expertise

Bo Cumbo
President and CEO

AAVANTIBIO · SAREPTA THERAPEUTICS · VERTEX · GILEAD

Kevin Tan
Chief Financial Officer

Selecta Biosciences · SAREPTA THERAPEUTICS · CPP Investments · MACQUARIE

Jessie Hanrahan, Ph.D.
Chief Regulatory & Preclinical Operations Officer

AAVANTIBIO · bluebirdbio · SANOFI GENZYME · Boston Scientific

Ty Howton, J.D.
Chief Operating Officer

AAVANTIBIO · SAREPTA THERAPEUTICS · VERTEX · Genentech

Gabriel Brooks, M.D.
Chief Medical Officer

Pfizer · 4DMT · GE HealthCare · GILEAD

Paul Herzich
Chief Technology Officer

AAVANTIBIO · NOVARTIS · bridgebio · LogicBio THERAPEUTICS

Shuli Kulak, M.D.
Head of Corporate Strategy & Business Development

AAVANTIBIO · Lonza · BIOMARIN · Deep Science Ventures

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Clinical Stage Genetic Medicines Company Targeting Neuromuscular and Cardiac Diseases

Program	Indication	Research / Discovery	Preclinical	Phase 1/2	Milestone (anticipated)	Worldwide Rights
Neuromuscular						
SGT-003	Duchenne muscular dystrophy				FDA Meeting to Discuss Regulatory Pathways	✓
SGT-212	Friedreich's ataxia (FA)				First Patient Dosed Q4 2025	✓
Cardiac						
SGT-501	RYR2-Mediated CPVT				First Patient Dosed Q4 2025	✓
	CASQ2-Mediated CPVT					✓
SGT-601	TNNT2 DCM					✓
SGT-401	BAG3-Mediated DCM					✓
SGT-701	RBM20 DCM					✓
Mayo Clinic Collaboration	Six Undisclosed Targets					✓
Platform						
Capsid Library[1]	Cardiac & NM				Cardiac Capsid Selection Q4 2025	✓

Notes: 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 Pinnacle™ 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. 1. Cardiac Capsid Library currently in NHPs, Mice and Pigs;

Anticipated Near-Term Milestones

Program	Milestone	Anticipated Timing
Neuromuscular		
SGT-003 for Duchenne	Initial 3 patient Phase 1/2 data (safety, microdystrophin expression & biomarker data)	✓
	Participant enrollment and clinical site expansion	Ongoing
	Anticipated meeting with FDA to discuss regulatory pathways	Q4 2025
SGT-212 for Friedreich's ataxia	IND cleared by FDA	✓
	Phase 1b trial initiation	Q4 2025
Cardiac		
SGT-501 for CPVT	RYR2 IND cleared by FDA; CTA approved by Health Canada	✓
	Phase 1b trial initiation	Q4 2025
SGT-601 for TNNT2	IND-enabling studies	Ongoing
Capsids		
AAV-SLB101	First-in-human data (SGT-003)	✓
Capsid Library (multiple capsids)	Complete rounds of NHP, mouse, and pig studies	Cardiac capsid selection Q4 2025
Pipeline		
Multiple Pipeline Assets	BAG3 & RBM20 preclinical studies, Mayo Clinic collaboration preclinical work	Ongoing

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Neuromuscular Lead Program

Duchenne Muscular Dystrophy (Duchenne)

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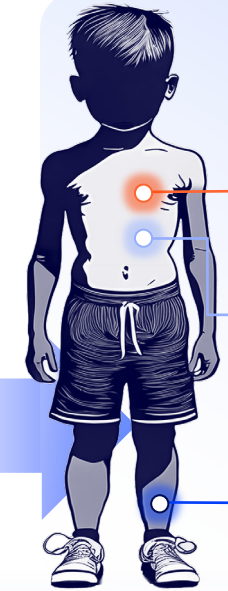
Duchenne is a Disease of Impaired Muscle Integrity & Dysfunction[1-4]

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 muscle integrity decline **predict negative outcomes in certain organs**, such as the heart[5-7]

In Duchenne, muscle fiber regeneration becomes impaired, leading to deterioration of muscle integrity resulting in difficulties with mobility, thoracic scoliosis, respiratory failure, and cardiac failure[4]



- 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 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.

SGT-003: Next-Generation Duchenne Gene Therapy

Targeted delivery, refined CMC, high expression & differentiated biomarker activity are key components of next-generation profile[1]

Next-Generation Design

Microdystrophin Construct Design
Hinge selection, nNOS binding domain

Advanced Capsid Engineering
Integrin-binding, improved muscle tropism, decreased liver targeting

Safety & Tolerability Profile
To date, no SAEs observed[2]; use of steroids alone for immunomodulatory regimen

High Protein Expression

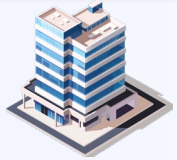
Microdystrophin Protein Expression
Western blot + mass spectrometry

Microdystrophin Positive Fibers
Immunofluorescence

Dystrophin-Associated Protein Complex Restoration
Beta sarcoglycan positive fibers

nNOS Membrane Localization
nNOS activity-positive fibers

Broad Biomarker Activity



Muscle Integrity
Biomarker analysis

Muscle Damage
Biomarker analysis

Muscle Maturation & Stem Cell Preservation
eMHC

Cardiac Function
LVEF, troponin

Contribute to a **differentiated Duchenne gene therapy** investigational product profile

[1]Initial INSPIRE DUCHENNE Phase 1/2 data on file as of February 11, 2025. Solid Biosciences.
[2]Safety data on file as of August 12, 2025. Solid Biosciences.

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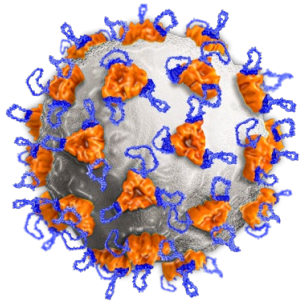
SGT-003: Next-Generation Duchenne Gene Therapy Candidate Optimized to Transduce and Preserve Muscle

SGT-003 MICRODYSTROPHIN TRANSGENE

Unique inclusion of nNOS-binding domain designed with the goal of preventing activity-induced ischemia and associated muscle injury[1,2]

SGT-003 AAV-SLB101 CAPSID



Rationally designed capsid targeting integrin receptors which are upregulated in dystrophic muscle[3]

SGT-003 was designed to enhance cardiac and skeletal muscle transduction of an optimized transgene while reducing liver targeting[1]

αSYN=alpha-syntrophin; ABD1=actin-binding domain 1; DGBD=dystroglycan binding domain; H=hinge; R=spectrin-like repeat; nNOS=neuronal nitric oxide synthase.

1. Lai Y, et al. J Clin Invest. 2009;119(3):624-635. 2. Ramos JN, et al. Mol Ther. 2019;27(3):623-635. 3. Hong A.V., et al. *Nature Communications.* 2024;15:7965.

SGT-003 is an investigational product that has not been approved by the FDA. No conclusions regarding safety and efficacy can be made.

AAV-SLB101 Capsid Demonstrated Robust Transduction & High VCN After Treatment With SGT-003 (N=3)

Lower dose than approved first-generation Duchenne gene therapy – with no use of eculizumab or other intensive immunomodulation

Next-generation microdystrophin gene therapy candidate, SGT-003[1]

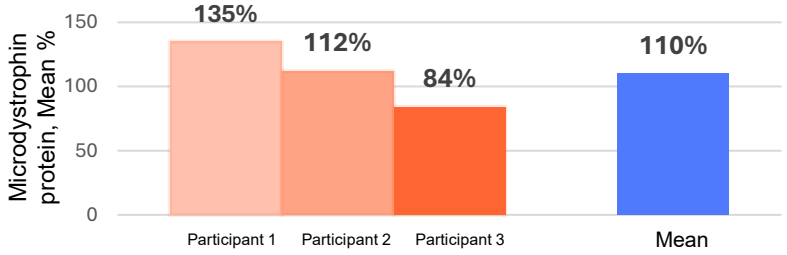
Patient	Dose	Copies/Nucleus (VCN)
1	1.0×10^{14} vg/kg	19.8
2		28.6
3		7.6
Mean		18.7

VCN=vector copy number.
1. Data on file as of February 11, 2025. Solid Biosciences.

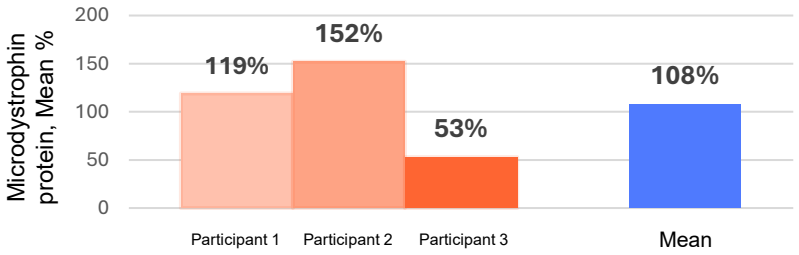
Significant Next Generation Microdystrophin Expression at Day 90

Comprehensive orthogonal measurements showed significant microdystrophin expression

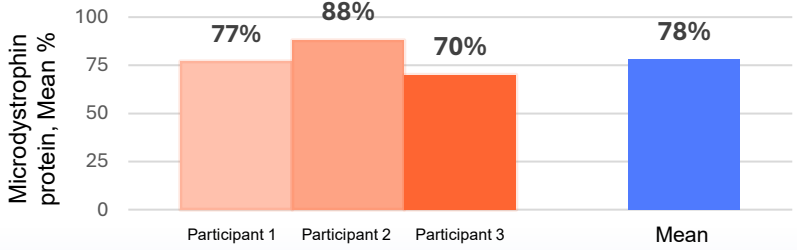
Microdystrophin Expression Measured by Western Blot[1]

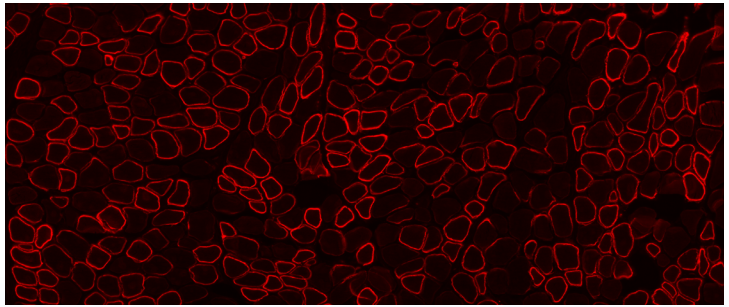


Microdystrophin Expression Measured by MS[1]



Dystrophin Positive Fibers Measured by IF[1]





Mean microdystrophin expression (n=3) measured by western blot[1] was observed to be >2x greater for SGT-003 (Day 90) than approved first-generation Duchenne gene therapy (Weeks 12 & 64)[2-4]

Baseline Western blot and mass spectrometry (MS) were both 0% mean normal dystrophin. Baseline dystrophin positive fibers were 1.5% measured by IF.
1. Data on file as of February 11, 2025. Solid Biosciences. 2. Mendell JR, et al. *Nat Med.* 2025 Jan;31(1):332-341. 3. Sarepta Therapeutics EMBARK Part 2 conference call, January 27, 2025. 4. The comparison presented represent cross-trial comparisons and do not represent data from a head-to-head clinical trial. Differences in study design, patient populations, treatment protocols and endpoints may impact direct comparisons. Participant #2 representative images shown.

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SGT-003 Microdystrophin was Observed to Bind and Restore Key Elements of Dystrophin-Associated Protein Complex

% Positive Fibers (IF) – Microdystrophin[1]

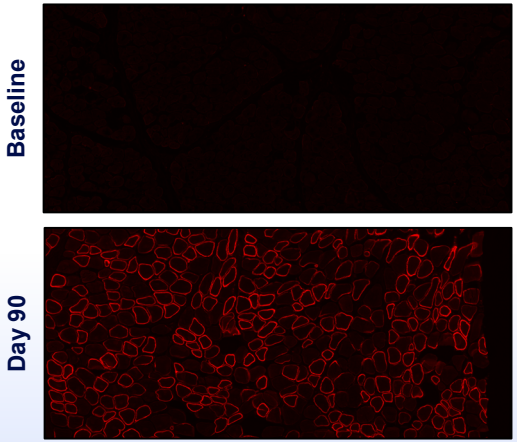
Patient	1	2	3	Mean
Day 90 Values	77%	88%	70%	78%
Baseline Values	0.8%	2.3%	1.3%	1.5%
Change From Baseline (Fold Change)	96x	38x	53x	53x

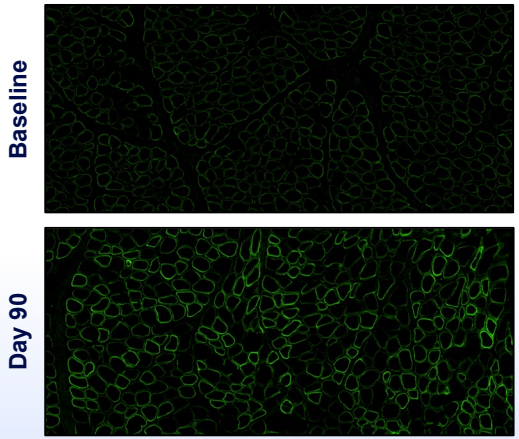
% Positive Fibers - Beta Sarcoglycan[1]

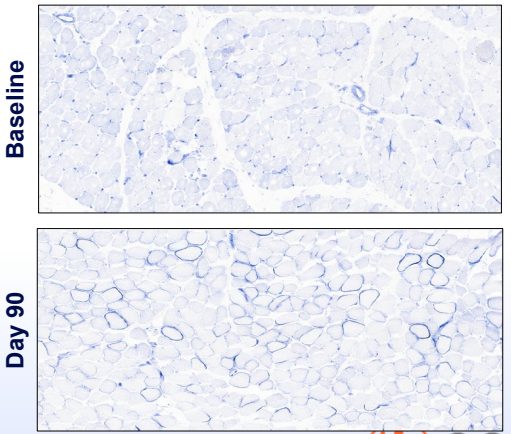
Patient	1	2	3	Mean
Day 90 Values	60%	88%	63%	70%
Baseline Values	0%	2.5%	1.5%	1.3%
Change From Baseline (Fold Change)	∞	34x	41x	52x

% Positive Fibers - nNOS Activity[1]

Patient	1	2	3	Mean
Day 90 Values	48%	53%	25%	42%
Baseline Values	0%	1.5%	0.5%	0.7%
Change From Baseline (Fold Change)	∞	34x	49x	62x







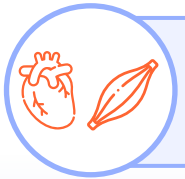
1. Data on file as of February 11, 2025. Solid Biosciences. Dystrophin positive fibers are not adjusted for fat and fibrosis; these are absolute numbers. Participant #2 representative images shown.

Solid's Microdystrophin Gene Therapy Demonstrated nNOS Co-Localization and Activity in DMD Patients

42% of muscle fibers showed appropriately localized nNOS expression/activity at Day 90 (mean: N=3 participants)[1]

nNOS Plays an Important Role in Muscle Health, Adaptation, and Performance[2,3]

- Prevents muscle wasting[2]
- Protects against fibrosis[4]
- Protects against oxidative stress[2]
- Supports muscle repair and recovery[2]
- Promotes anti-inflammatory effects[5]
- Supports cellular repair and regeneration[6]
- Improves blood flow[2,7]
- Improves stamina[2,3]
- Regulates muscle contraction[2]



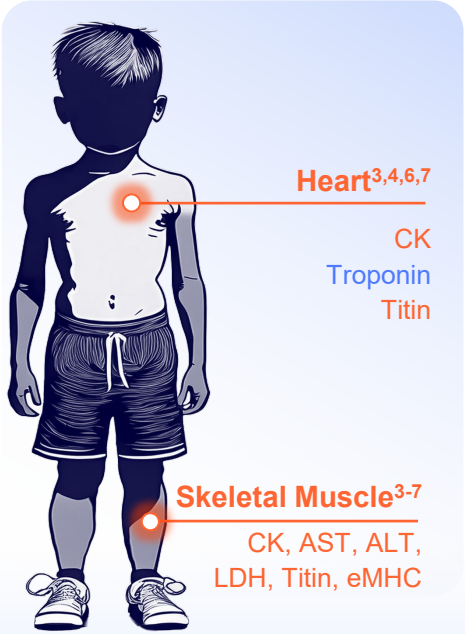
Restoring properly localized nNOS activity at the muscle membrane is essential to more fully protect cardiac and skeletal muscle

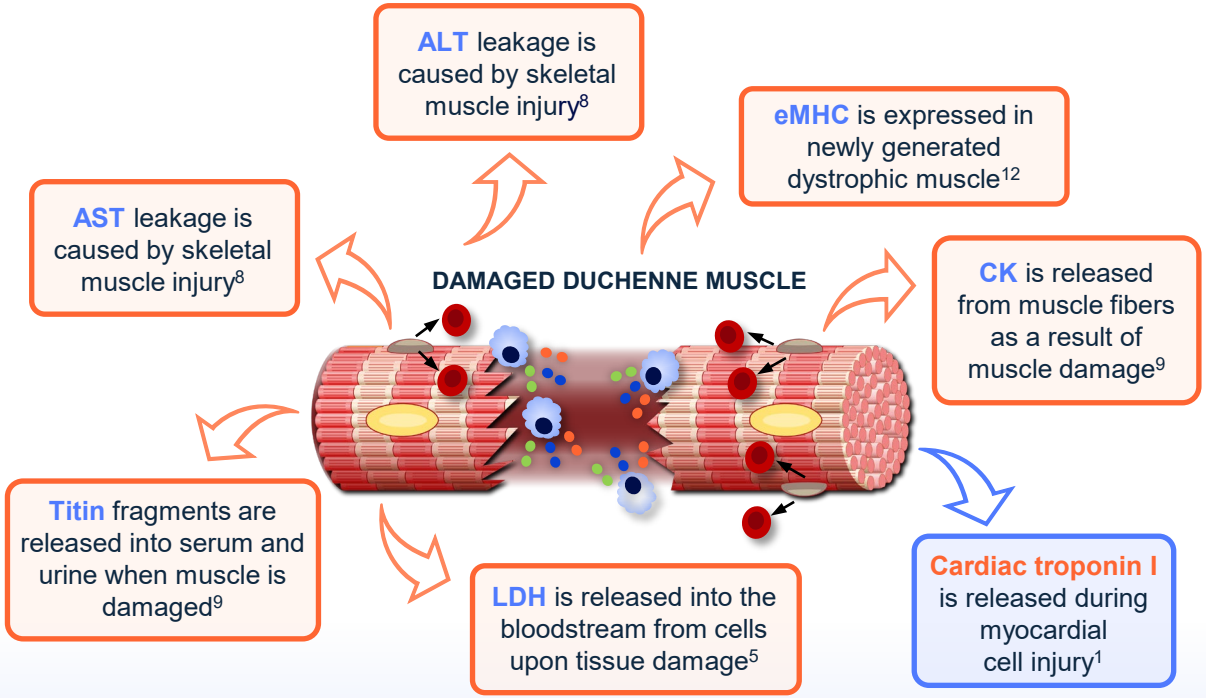
Loss of nNOS at the sarcolemma leads to impaired NO-mediated vasodilation, functional ischemia, and muscle fatigue and breakdown[1,2]

NO=nitric oxide; SR=sarcoplasmic reticulum.

1. Data on file as of February 11, 2025. Solid Biosciences. 2. Zhao J, et al. *Mol Med*. 2019;25(1):31. 3. Kobayashi YM, et al. *Nature*. 2008;456(7221):511-515. 4. Wehling-Henricks M, Tidball JG. *PLoS One*. 2011;6(10):e25071. 5. Nguyen HX, Tidball JG. *J Physiol*. 2003;550(Pt 2):347-356. 6. Buono R, et al. *Stem Cells*. 2012; 30(2):197-209. 7. Stamler JS, et al. *Physiol Rev*. 2001;81(1):209-237.

Comprehensive Biomarker Assessment of Muscle Integrity, Resilience, & Preservation





1. Hiramuki Y, et al. *Sci Rep*. 2025;15(1):1778. 2. Siddique Ahmed Khan M, et al. *Int J Sci Res*. 2016;5(11):156-157. 3. Aujla RS, et al. *StatPearls* [Internet]. 2024. 4. US Department of Veterans Affairs. Accessed December 13, 2024. https://www.hepatitis.va.gov/hcv/patient/diagnosis/labtests-AST.asp 5. Farhana A, Lappin SL. *StatPearls* [Internet]. 2023. 6. Park KC, et al. *Cardiovasc Res*. 2017;113(14):1708-1718. 7. ElSaygh J, et al. *Cardiol Rev*. February 9, 2024. Online ahead of print. 8. Aulbach AD, Amuzie, CJ. A Comprehensive Guide to Toxicology in Nonclinical Drug Development (Second Edition). 2017. 9. Oshida N, et al. *Sci Rep*. 2019;9(1):19498. 10. Kim EY, et al. *Ann Rehabil Med*. 2017;41(2):306-312. 11. Voleti S, et al. *Pediatr Cardiol*. 2020;41(6):1173-1179. 12. Guiraud S, et al. *Hum Mol Genet*. 2019;28(2):307-319.

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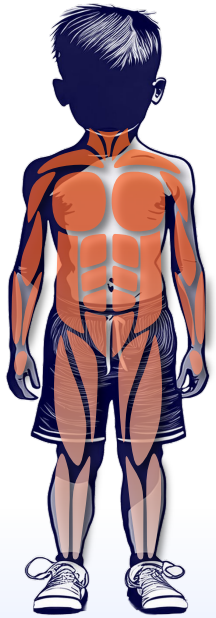
Reductions in Biomarkers of Muscle Breakdown Showed Comprehensive Improvements in Muscle Health After Treatment With SGT-003[1-3]

7 simultaneous biomarker reductions observed at Day 90 (mean: N=3 participants)[1-4]

Improved muscle integrity, as indicated by a coordinated biomarker profile, may support a **slowing of disease progression and better long-term clinical outcomes**[1-3]

Monitoring of multiple Duchenne biomarkers offers a powerful approach for **assessing disease trajectory**, with long-term assessment to **establish treatment effectiveness**[4]



Muscle Integrity

- Serum CK (-57%)[5]
- Serum LDH (-60%)[5]
- Serum AST (-45%)[5]
- Serum ALT (-54%)[5]
- Serum Titin (-42%)[5]
- Histologic eMHC (-59%)[5]
- Serum Troponin (-36%)[a,5]

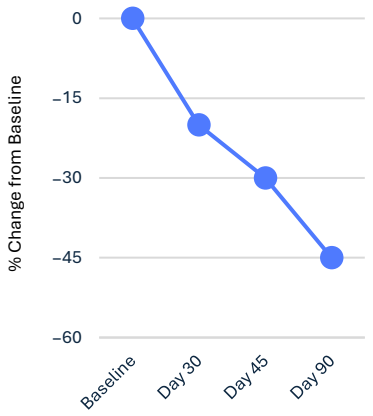
[a]Serum troponin data from Participant 3, only, at Day 90: Participant 3 had elevated troponin levels at baseline. Troponin levels for Participants 1 & 2 were 0 at baseline.

1. Siddique Ahmed Khan M, et al. *Int J Sci Res*. 2016;5(11):156-157. 2. Voleti S, et al. *Pediatr Cardiol*. 2020;41(6):1173-1179. 3. Oshida N, et al. *Sci Rep*. 2019;9(1):19498. 4. FDA-NIH Biomarker Working Group. Last updated January 25, 2021. Accessed December 12, 2024. https://health.uconn.edu/pepper-center/wp-content/uploads/sites/272/2023/12/BEST-Biomarkers-EndpointS-and-other-Tools-Resource.pdf. 5. Reductions represent mean of N=3 participants at Day 90. Data on file as of February 11, 2025. Solid Biosciences.

Improvements in Markers of Muscle Injury[1]

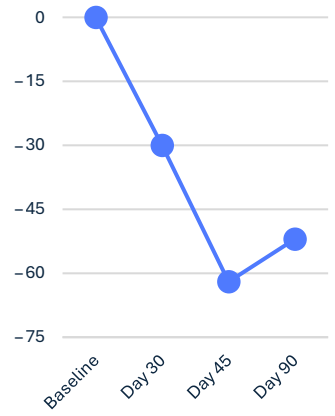
AST, **ALT**, **CK**, and **LDH** are released from muscle into circulation in Duchenne due to tissue damage and muscle injury[2-4]

SERUM AST (IU/L)[1,a]



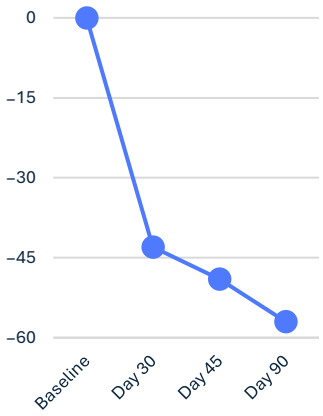
Serum AST (−45%)[1]

SERUM ALT (IU/L)[1,a]



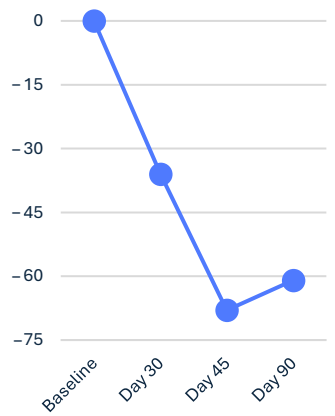
Serum ALT (−54%)[1]

SERUM CK (IU/L)[1,a]



Serum CK (−57%)[1]

SERUM LDH (IU/L)[1,a]



Serum LDH (−60%)[1]

ALT=alanine aminotransferase; AST=aspartate aminotransferase; CK=creatine kinase; LDH=lactate dehydrogenase.
[a]Mean (n=3) change from baseline results shown.

1. Data on file. Solid Biosciences. 2025. 2. Aulbach AD, Amuzie CJ. *A Comprehensive Guide to Toxicology in Nonclinical Drug Development.* 2nd ed. 2017. 3. Kim EY, et al. *Ann Rehabil Med.* 2017;41(2):306-312. 4. Farhana A, Lappin SL. *StatPearls* [Internet]. 2023.

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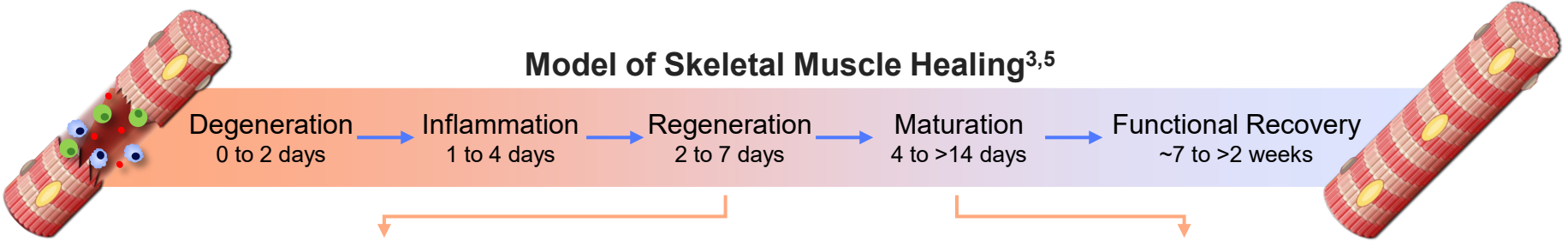
eMHC is Elevated in Dystrophic Muscles Primarily Due to Muscle Breakdown and Regeneration[1-3]

As muscle fibers deteriorate, muscle stem cells are activated to replace damaged fibers. During this regenerative process, newly formed muscle fibers express embryonic myosin heavy chain (eMHC)[3,4]



- During this regenerative process, the newly formed muscle fibers express **eMHC**[1,5]
- High eMHC protein levels in muscle biopsies indicate **active muscle regeneration**[6]
- in DMD, muscle regeneration **cannot keep up with muscle breakdown**, leading to progressive weakness despite ongoing repair attempts[2,4,5]

- **Muscle fibers often remain in an immature state**, leading to **persistent eMHC expression**, due to **constant muscle injury and incomplete repair** in DMD[2-4]

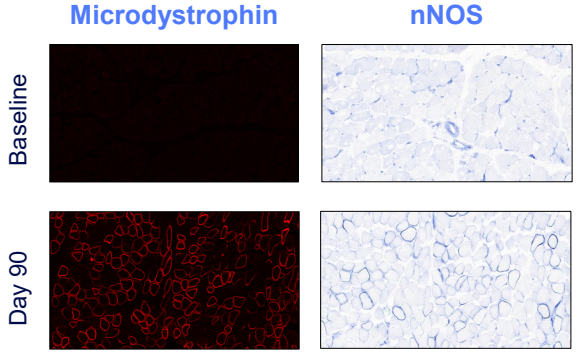
eMHC expression is significantly upregulated in dystrophic muscle fibers, making it a biomarker for disease progression and response to therapies[1-3]

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. Sewry CA, et al. *Neuromuscul Disord*. 2021;31(5):371-384.

SGT-003 was Observed to Reduce Muscle Breakdown & Increase Muscle Preservation

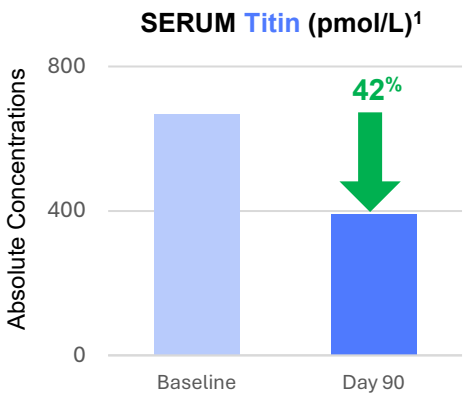
Robust microdystrophin expression and nNOS co-localization led to reduced markers of muscle loss and dystrophic regeneration at Day 90 (mean: N=3 patients)

MICRODYSTROPHIN EXPRESSION & nNOS ACTIVITY



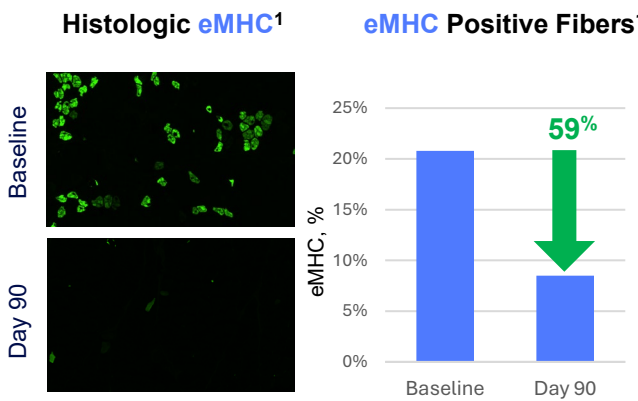
SGT-003 demonstrated robust microdystrophin expression and localized nNOS appropriately to the sarcolemma

REDUCTION IN MUSCLE BREAKDOWN



Titin fragments are decomposed and released into serum and urine when muscle is damaged.[2] Increased titin concentrations are associated with Duchenne muscle breakdown

INCREASED MUSCLE PRESERVATION

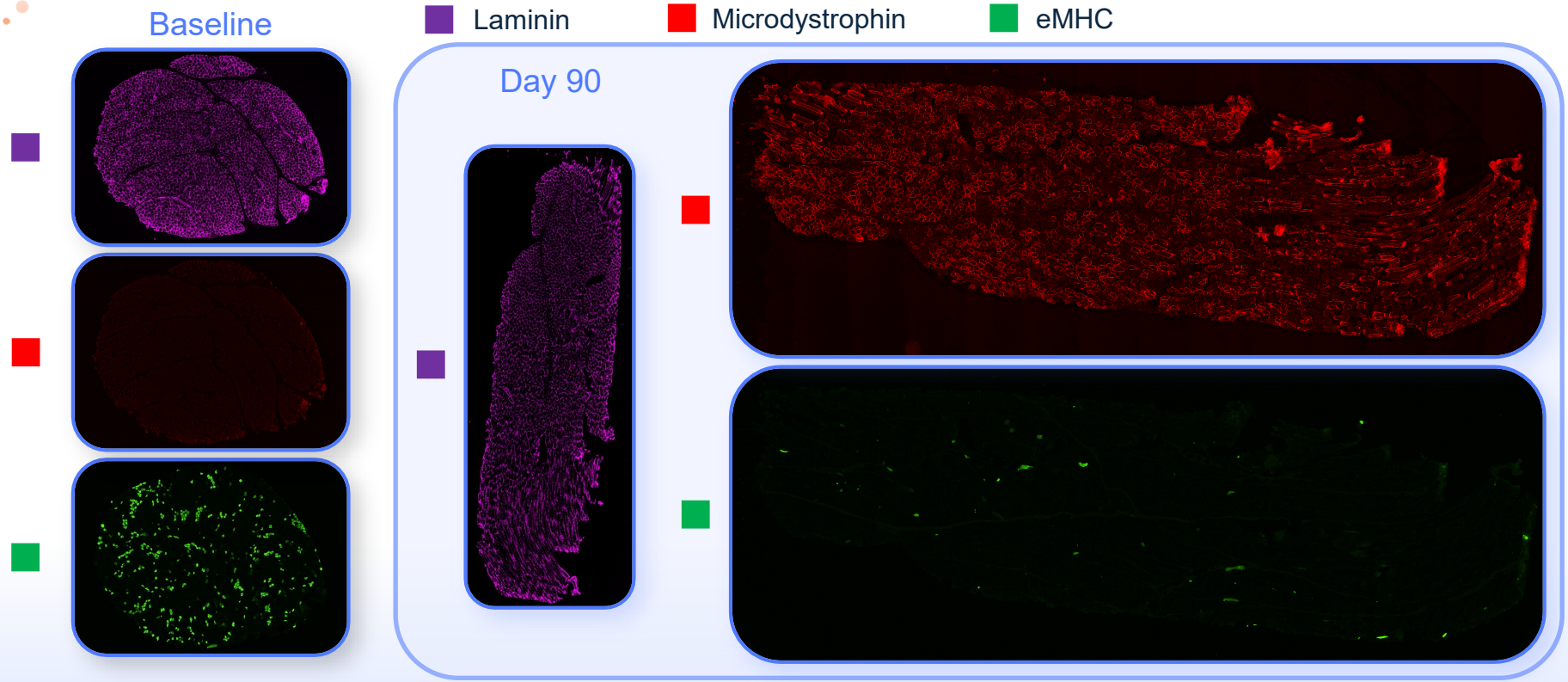


eMHC is expressed in newly generated dystrophic muscle.[3] The presence of elevated eMHC levels indicate active muscle breakdown, incomplete repair, and impaired muscle preservation and resilience

Treatment with SGT-003 resulted in robust microdystrophin expression, proper localization of nNOS, and reductions in biomarkers of muscle injury, breakdown and dystrophic regeneration

1. Data on file as of February 11, 2025. Solid Biosciences. 2. Oshida N, et al. *Sci Rep*. 2019;9(1):19498. 3. Guiraud S, et al. *Hum Mol Genet*. 2019;28(2):307-319.

Full Slide Scans of Muscle Biopsy Sections Showed Uniform Improvements in eMHC, a Marker of Muscle Breakdown and Dystrophic Regeneration[a]



[a]Participant 2 representative images are shown. Laminin staining is used to demarcate muscle membranes
Data on file. Solid Biosciences. 2025.

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

Cardiomyopathy is a leading cause of death in Duchenne muscular dystrophy[2]

INCIDENCE OF DUCHENNE-RELATED CARDIOMYOPATHY OCCURS EARLY IN LIFE[3]

AGE 0

Cardiac disease is underway long before overt cardiac dysfunction appears[4]

AGE 6

25% evidence of cardiomyopathy by **age 6 years**[3]

AGE 10

59% evidence of cardiomyopathy by **age 10 years**[3]

AGE 18

98% evidence of cardiomyopathy by **age 18 years**[3]

40% of these patients will progress to **heart failure**[3]

Cardiac tissue has limited regenerative capacity: by age 25, only ~1% of cardiomyocytes will turn over annually[5]

Early troponin elevation is predictive of severe cardiac disease in neuromuscular diseases[6-9]

A hs-cTnI level >7.6 ng/L is correlated with a 3-fold increased risk of cardiac disease[10]

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[11]

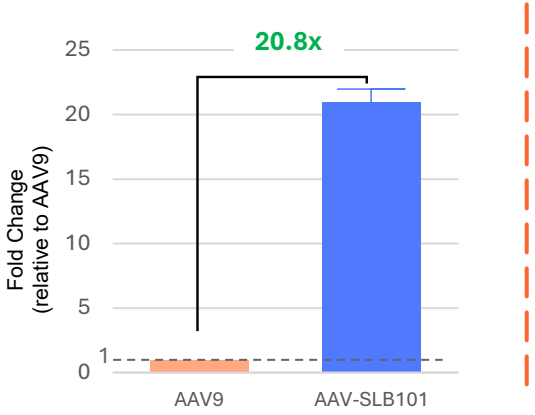
hs-cTn=high-sensitivity cardiac troponin.

1. Schultz TI, et al. *JACC Basic Transl Sci.* 2022;7(6):608-625. 2. Meyers TA, et al. *Int J Mol Sci.* 2019;20(17):4098. 3. Gandhi S, et al. *Cells.* 2024;13(14):1168. 4. James J, et al. *Neuromuscul Disord.* 2011;21(7):462-467. 5. Parmacek MS, Epstein JA. *N Engl J Med.* 2009;361(1):86-88. 6. Sheybani A, et al. *Pediatr Res.* 2022;92(6):1613-1620. 7. Voleti S, et al. *Pediatr Cardiol.* 2020;41(6):1173-1179. 8. Wagner KR, et al. *Biomark Med.* 2021;15(15):1389-1396. 9. Saunders JT, et al. *Circulation.* 2011;123(13):1367-1376. 10. Spurney CF, et al. *Open Heart.* 2021;8(1):e001592. 11. D'Amario D, et al. *Heart.* 2017;103(22):1770-1779.

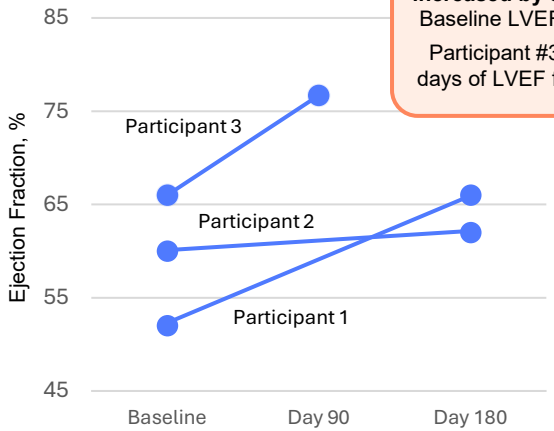
SOLID BIOSCIENCES

AAV-SLB101: 20.8x Greater Cardiomyocyte Transduction vs. AAV9 & Early Cardiac Markers After SGT-003 Dosing

Non-Clinical: Luciferase Expression in iCell Cardiomyocytes[1,a]



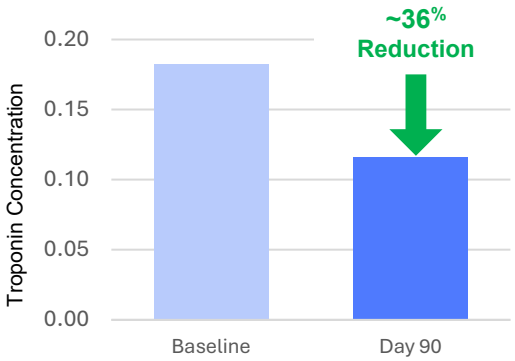
SGT-003 Clinical Trial LVEF(%)[1,b]



In the 2 participants at Day 180, **mean baseline cardiac function increased by 8%** from a Baseline LVEF of 56%.

Participant #3 has 90 days of LVEF follow up.

SERUM Troponin (ng/mL)
Participant #3 (only)[c]



Lower LVEF has been shown to correlate with a higher 5-year probability of death.[2] Reducing troponin concentrations by >25% could result in 5-fold greater reduction in the risk of cardiac events[2]

LVEF=left ventricle ejection fraction.
1. Data on file as of February 11, 2025. Solid Biosciences. 2. Soslow JH, et al. *Circ Heart Fail*. 2023;16(8):e010040.
[a]iCell Cardiomyocytes are derived from human, induced pluripotent stem cells (iPSCs).
[b]Participant 3 has yet to reach the Day 180 follow-up as of February 12, 2025. All three participants demonstrate LVEF above baseline at all follow up timepoints.
[c]Serum troponin data only from Participant 3 at Day 90: Participant 3 had elevated troponin levels at baseline. Troponin levels for Participants 1 & 2 were 0 at baseline.

SGT-003 Tolerability and Safety Profile to Date Supports Steroid-Only Immunomodulatory Regimen

SGT-003 is the only Duchenne gene therapy to utilize a steroid-only immunomodulatory regimen

Immunomodulation Regimens With Use of Duchenne Gene Therapies

	SGT-003 (Solid Biosciences)[1]	SRP-9001 (Sarepta Therapeutics)[2,3]	RGX-202 (REGENXBIO)[4,5]
Corticosteroids (High-dose, pre-taper period)	~30 Days	~60 Days	~60 Days
Sirolimus		(non-ambulatory[a])	
Eculizumab			
Duration of Regimen	**~30 Days[b]**	**~60 Days[b,3]**	**~84 Days[4]**

Corticosteroids are generally well tolerated. Adverse events are mitigated through limiting dose and duration[6]

For illustrative purposes only: no head-to-head clinical trial has been conducted evaluating SGT-003 with SRP-9001, RGX-202 or other products. No cross-trial comparisons can be drawn.
[a] Potential addition of sirolimus to immunosuppression regimen in non-ambulatory patients. Sarepta Conference Call. July 2025. [b] Durations represent time from dosing to beginning of steroid taper.

1. Data on file. Solid Biosciences. 2025. 2. Mendell JR, et al. Presented at: Academy of Managed Care Pharmacy Meeting; March 21-24, 2023. San Antonio, TX. 3. Sarepta Therapeutics, Inc. 2024. https://www.elevidys.com/downloads/Elevidys_Treatment_Guide_For_Caregivers.pdf 4. REGENXBIO. June 2025. https://ir.regenxbio.com/static-files/eee6445c-ae47-4551-9bcd-67c11ac769f9. 5. REGENXBIO. CureDuchenne Webinar August 2025. https://cureduchenne.org/general/regenxbio-affinity-duchenne/. 6. Yasir M, et al. *StatPearls* [Internet]. Last updated July 3, 2023. https://www.ncbi.nlm.nih.gov/books/NBK531462/.

INSPIRE DUCHENNE Interim Safety Summary – August 2025 Update

SGT-003 has been well tolerated in participants dosed to date with no treatment-emergent SAEs reported

- Glucocorticoids alone were used for immunosuppression
- No need for any additional immunosuppressive agents

- No treatment-emergent SAEs reported
- One Grade 1 AESI of elevated liver enzymes noted during steroid taper period. As of August 12, 2025, participant has shown no clinical manifestations and has been responsive to steroid therapy

SELECT PATIENT CHARACTERISTICS:

- Age range: 5 to 10 years
- Weight range: 18.9 to 39.7 kg

SGT-003 TEAEs Safety data cutoff: August 12, 2025		**Participants (N=15)** **n (%)**
SAEs		0 (0.00%)
AESIs	Hepatotoxicity (defined based only on lab criteria)	1 (6.7%)[1] Grade 1
	Thrombotic microangiopathy	0 (0.00%)
	Myocarditis	0 (0.00%)
	Myositis	0 (0.00%)

The most common treatment-related adverse events (AEs) reported: nausea (n=15), vomiting (n=14), thrombocytopenia/platelet count decreased (n=10), decreased appetite (n=9), and headache (n=6).

AE=adverse event; AESI=adverse event of special interest; GGT=gamma-glutamyl transferase; GLDH=glutamate dehydrogenase; SAE=serious adverse event; TEAE=treatment-emergent adverse event.
1. CTCAE Grade 1 hypertransaminaseamia; no bilirubin elevation; no events of Drug Induced Liver Injury.
Data on file. Solid Biosciences. 2025.

SOLID BIOSCIENCES

Neuromuscular Pipeline Program

Friedreich's Ataxia (FA)

SOLID BIOSCIENCES

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/.

Introducing SGT-212: The Only Dual Administration Approach to Address Both Neurologic and Cardiac Manifestations of FA

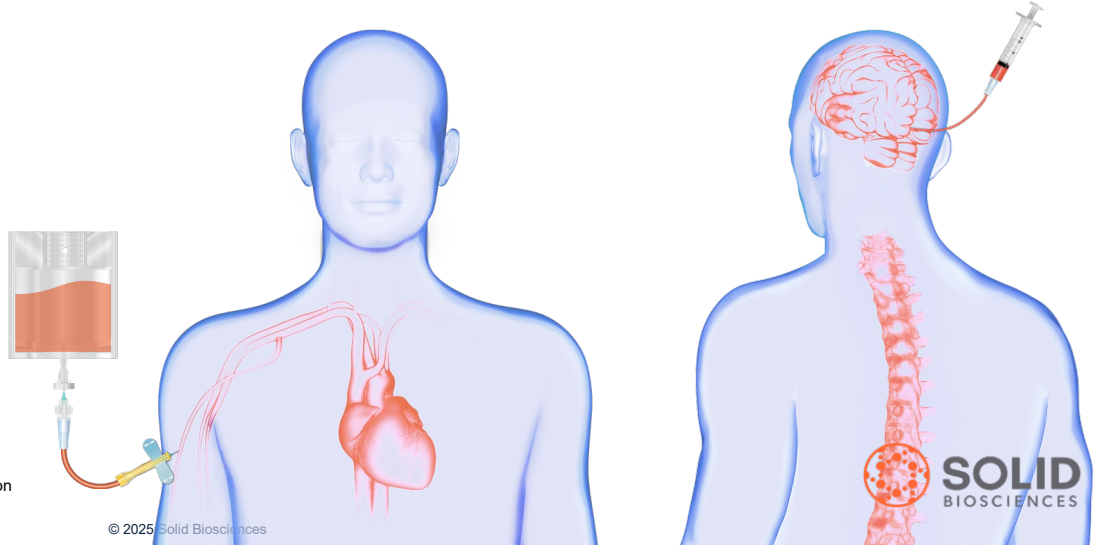
SGT-212
is the only FA gene therapy in development designed to directly address the neurologic and cardiac manifestations of FA

Intravenous (IV) Administration

- Focused on treating largest cause of mortality in Friedreich's ataxia: cardiomyopathy
- Potential to treat other disease-relevant organ systems

Direct Dentate Nuclei (IDN) Infusions*

- Removes challenges of crossing blood-brain barrier to address most disease-critical brain structure with potential to treat ataxia and dysarthria
- Direct administration using convection-enhanced delivery, which utilizes a catheter to deliver therapy using bulk flow
- MRI imaging during infusion, plus the use of gadolinium, will provide confirmation of delivery



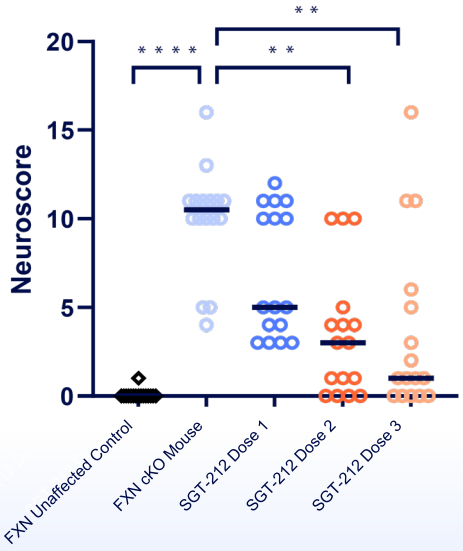
*Administration simplified for illustrative purposes. Actual SGT-212 IDN administration will use FDA-approved delivery catheter system.

SGT-212 Systemic Administration Resulted in Significant Neurological and Neuromotor Function Improvements

Neuronal proof-of-concept achieved in disease-relevant knockout mouse model (nKO)

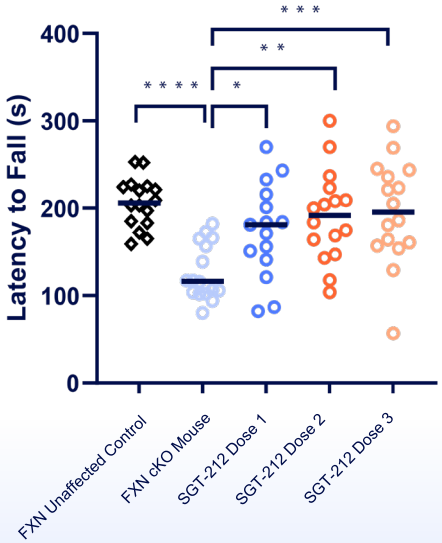
Neurological Assessment Score

Neuroscore[1] (Day 56)



Neuromotor Function Assessment

RotaRod[2] (Day 60)



Asterisks=statistical significance; FXN = Frataxin

1. The neurological score assessment was used to assess the severity of ataxia. 2. The RotaRod test evaluates coordination and balance by measuring the time to fall for mice running on a spinning rod that progressively accelerates – a decreased latency to fall indicates neuromotor impairment.

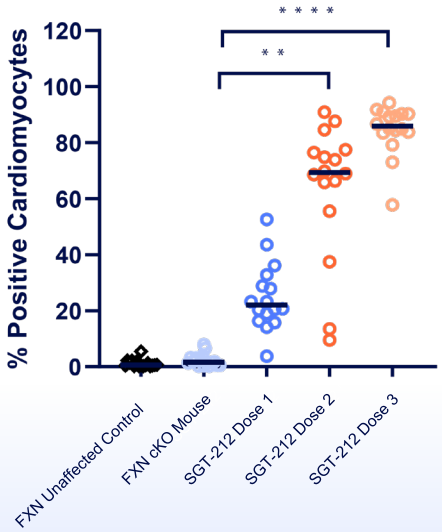
Data on file. Solid Biosciences 2024.

SGT-212 Systemic Administration Demonstrated Cardiac FXN Expression, Activity and Resolution of Cardiomyopathy Phenotype

Cardiac proof-of-concept achieved in disease-relevant knockout mouse model (cKO)

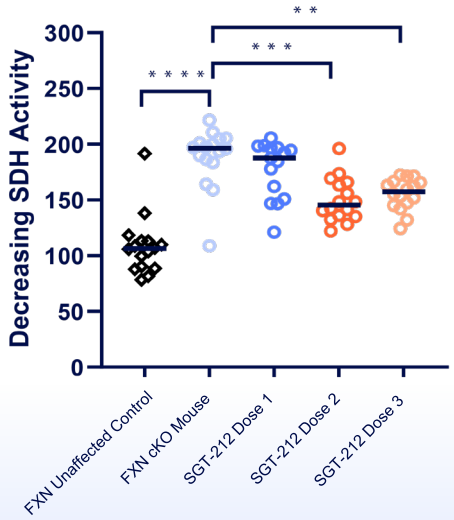
FXN Cardiac Expression

In Situ Hybridization in Heart



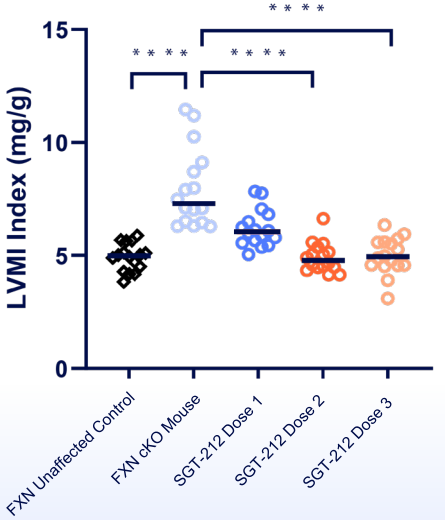
Mitochondrial Function

Succinate Dehydrogenase (SDH)



Indicator of Cardiac Structure

Left Ventricular Mass Index (Day 30)*

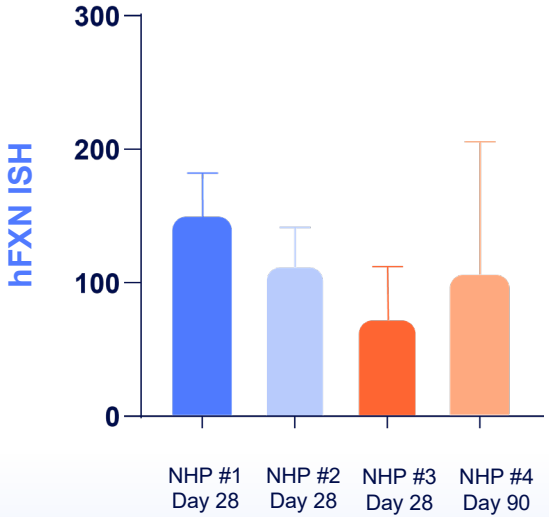


Asterisks=statistical significance
*Research has indicated that increased LVMI is correlated with increased risk of all-cause mortality (Pousset F, et al. 2015)
Data on file. Solid Biosciences 2024.

IDN Administration of SGT-212 Resulted in Robust FXN Expression in the Cerebellum in NHPs at Clinically Relevant Dose

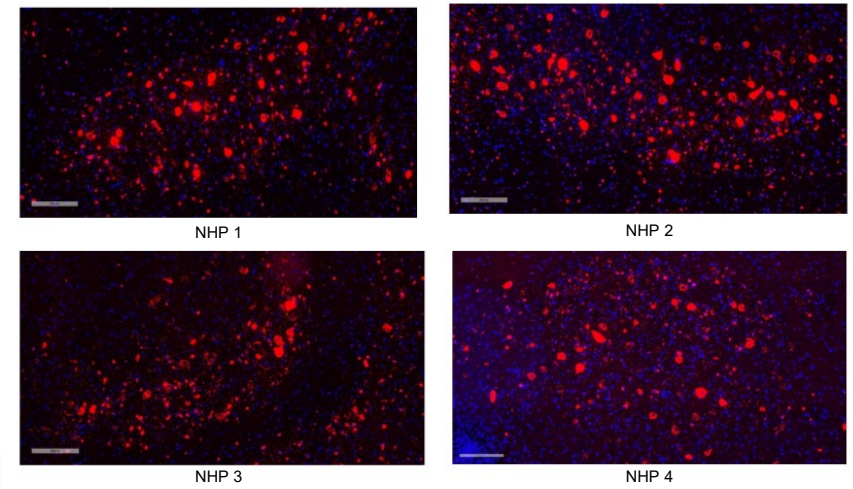
hFXN Expression in Dentate Nuclei (Cerebellum)

In Situ Hybridization*



hFXN Properly Localized to Dentate Nuclei (Cerebellum)

In Situ Hybridization



Human frataxin (hFXN)

*Data represent an average.
Data on file. Solid Biosciences 2024.

Solid has Built Robust Understanding and Expertise in FA Through Extensive Preclinical Work in NHPs

Substantial in-house preclinical work and preclinical studies by collaborators have been conducted across multiple candidates, routes of administration & dose levels

Overall NHP Studies Performed

9 NHP studies conducted in total across 4 different development candidates

n=120+ NHPs tested

Range of dose levels tested across 4 routes of administration (IV, IT, IV & IT, IV & IDN)

Follow-up time as long as 365 days post dose (including SGT-212)

SGT-212 NHP Tox Study Findings

- Dose-dependent & long-term biodistribution in NHP tissues was associated with corresponding transgene expression in the heart, dentate nucleus, and DRG
- The precision MRI-guided IDN injection procedure was safe and well tolerated by NHPs
- The proposed clinical IDN and IV dose levels demonstrated no treatment-related findings (both in CNS and non-CNS)
- The proposed clinical IDN and IV dose levels elicited therapeutically relevant levels of FXN expression

IV = Intravenous; IT = Intrathecal; IDN = Intradentate Nuclei; FXN = Frataxin; DRG = Dorsal Root Ganglion
Data on file. Solid Biosciences 2024.

Clinical Trial Design: SGT-212 Phase 1b Study

First-in-Human, Open-Label, Multi-Center Study to Enroll a Minimum of 6 Participants

Dosing expected to initiate Q4 2025

Objective

Primary Objective

- To evaluate the safety and tolerability of IDN infusion and systemic IV infusion of SGT-212 gene therapy in participants with FA

Exploratory Objectives

- To evaluate the effect of SGT-212 on:
  - Frataxin protein expression
  - Motor function and disability
  - Cardiac function
  - Speech function

Design

Design

Study includes **3 cohorts** based ambulatory status:

- Cohort 1: Non-Ambulatory Participants
- Cohort 2: Ambulatory Participants
- Cohort 3: Ambulatory and Non-Ambulatory Participants (dose refinement or dose expansion)

All participants are adults with FA with documented cardiac hypertrophy

SGT-212 delivered by: magnetic resonance imaging (MRI) guided bilateral infusion to the dentate nuclei (DN) and intravenous (IV) infusion

Endpoints

Primary Endpoint

Incidence and severity of TEAEs from baseline to month 12

Exploratory Endpoints

Change from baseline frataxin protein expression in the blood, cardiac and skeletal muscle starting at day 90

Change from baseline starting at 18 months in key functional tests (e.g. mFARS, 9-hole peg test, timed 25-foot walk, among others)

Change from baseline starting at 12 months in left ventricular structure and function

Cardiac Lead Program

Catecholaminergic Polymorphic Ventricular Tachycardia (CPVT)

SOLID BIOSCIENCES

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

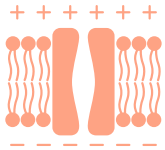
AAV-based delivery of a genetic payload to the heart intended to achieve expression of wild-type CASQ2 protein using a cardiac-selective promoter and an optimized transient transfection manufacturing process

RYR2=Ryanodine receptor, CASQ2= Calsequestrin 2

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

SOLID BIOSCIENCES

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

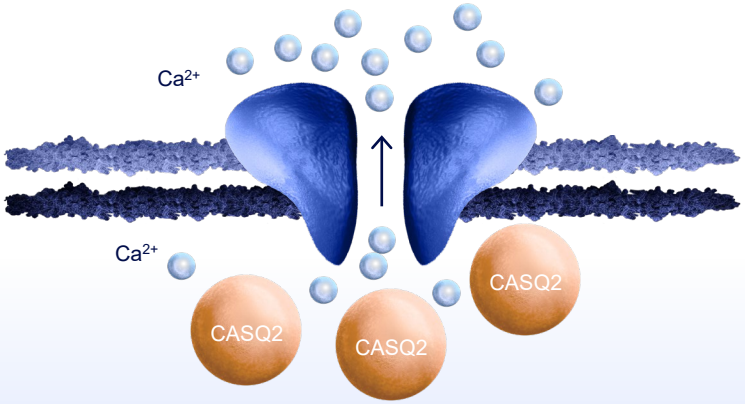
- Beta blockers
- Flecainide
- Implantable Cardioverter Defibrillators
- Left Cardiac Sympathetic Denervation

Rationale for CASQ2 Augmentation in RYR2 CPVT

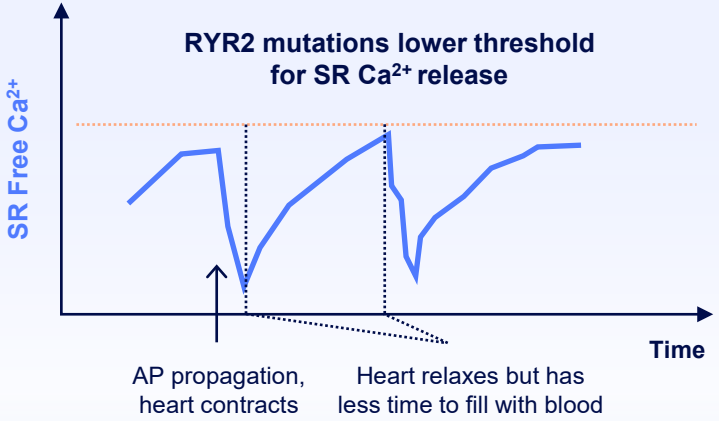
In RYR2 pathogenic mutations, normal CASQ2 levels are insufficient to maintain RYR2 in a closed conformation during diastole in high calcium flux states (such as with adrenaline)

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



Arrhythmia



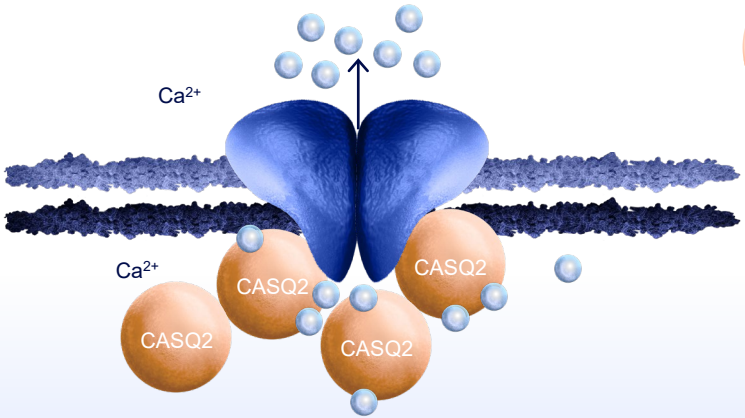
RyR, Ryanodine Receptor; SR, sarcoplasmic reticulum; AP, action potential; Ca^{2+}, calcium
Sources: Proprietary data from Priori Lab

Rationale for CASQ2 Augmentation in RYR2 CPVT (cont.)

Cardiac delivery of SGT-501 is intended to increase CASQ2, thus enhancing Ca^{2+} buffering and counteracting Ca^{2+} sensitivity caused by RYR2 pathogenic mutations

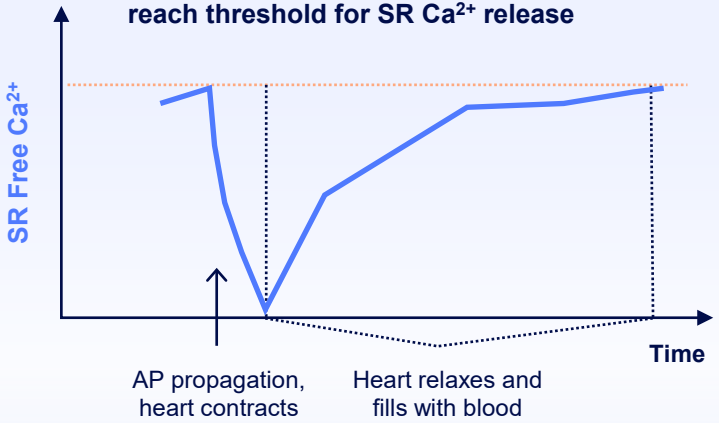
RYR2 Mutation-Related CPVT + Increased CASQ2 expression

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



Normal Rhythm

Increased CASQ2 increases time to reach threshold for SR Ca^{2+} release



RyR, Ryanodine Receptor; SR, sarcoplasmic reticulum; AP, action potential; Ca^{2+}, calcium
Sources: Proprietary data from Priori Lab

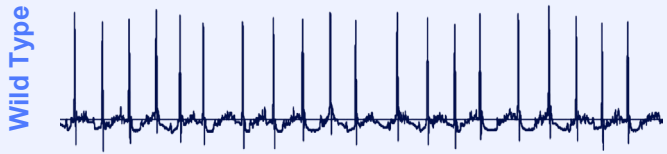
RYR2 CPVT Transgenic Mouse Model Used To Support Proof-of-Concept For AAV Gene Delivery of Human CASQ2

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

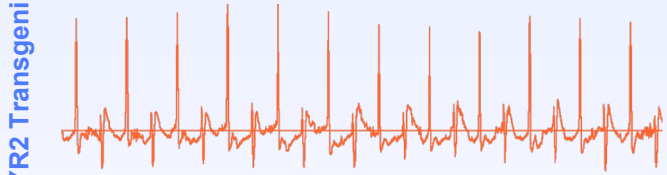
WT Mice
Dosed With Vehicle
IP dose epinephrine & caffeine

RYR2 Transgenic Mice
Dosed With Vehicle
IP dose epinephrine & caffeine

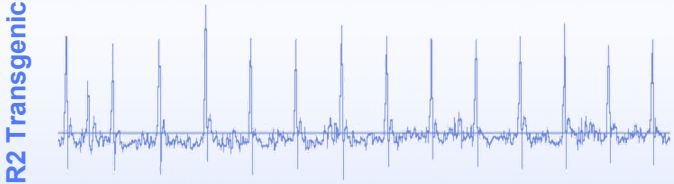
RYR2 Transgenic Mice
Dosed With SGT-501
IP dose epinephrine & caffeine



Normal heart rhythm **in WT background strain animals**



Polymorphic and/or bidirectional arrhythmic morphology **in transgenic animals**



Normal heart rhythm **seen after β-adrenergic challenge in mice treated with SGT-501**

WT=Wild Type
Data on file. Solid Biosciences 2025.

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

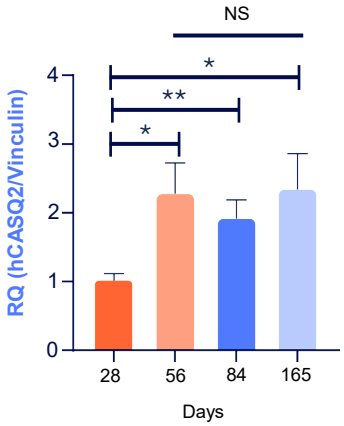
Clinically relevant expression levels continued through month 6 indicating potential durability and stability of expression

hCASQ2 Protein Expression

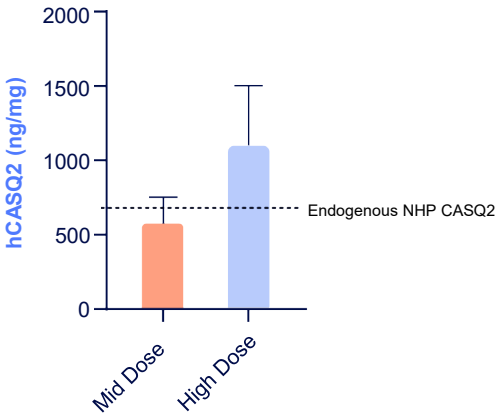
Mouse Kinetics: Expression significantly increased until Day 56, followed by continued stable expression through Day 165

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

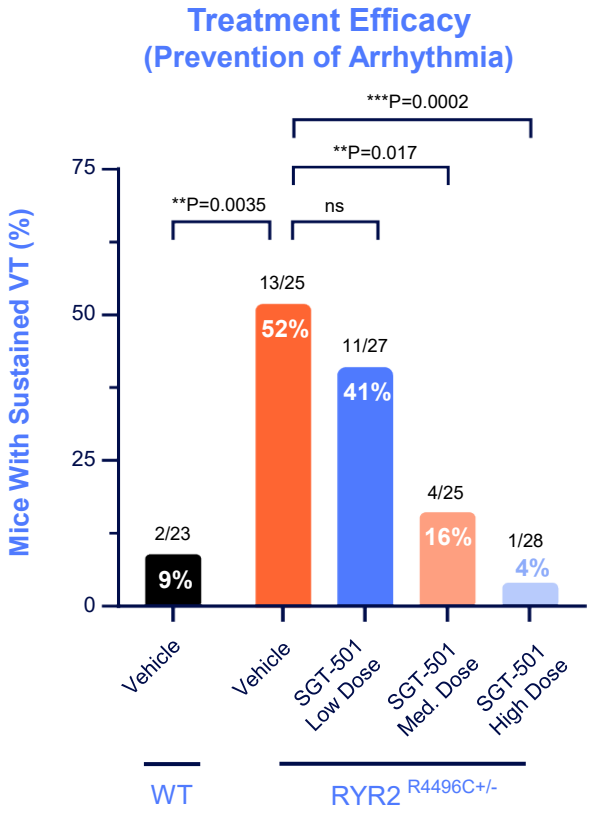
SGT-501 Demonstrated Protection From Sustained VT & Arrhythmia

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 (epinephrine + caffeine) in an RYR2 transgenic mouse model of CPVT

Treatment efficacy was normalized to background model penetrance of 52%



Data on file. Solid Biosciences 2025.

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.

Clinical Trial Design: SGT-501 Phase 1b Study

First-in-Human, Open-Label, Multi-Center Study to Enroll a Minimum of 6 Participants

Dosing expected to initiate Q4 2025

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 background beta-blocker and/or flecainide

Endpoints

Primary Endpoint

Incidence of TEAEs through Day 360

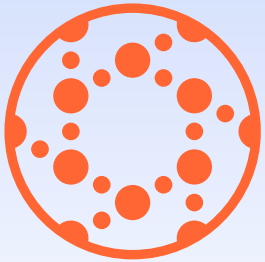
Secondary Endpoints

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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