January 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 and SGT-212 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 company’s expectations for submission of an IND for SGT-501 and to submit additional INDs by the end of 2026; 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.

Solid Biosciences: Pioneering the Next Generation of Precision Genetic Medicines with Multiple Clinical Stage Assets in 2025

Focused on developing robust platform-enabling technologies, Solid is uniting experts in science, technology and patient care to advance neuromuscular and cardiac therapies

Neuromuscular

Next-generation neuromuscular pipeline targeting degenerative diseases – **both programs now have FDA IND clearance**

- Duchenne Muscular Dystrophy
- Friedreich's Ataxia (FA)

FDA IND CLEARANCE ANNOUNCED JAN 2025

Cardiac

Cutting edge gene therapy platform – **pipeline expansion underway through Mayo Clinic collaboration**

- Inherited Arrhythmia Disorders & Channelopathies
- Dilated Cardiomyopathies (DCM)
- Hypertrophic Cardiomyopathies (HCM)

Delivery

Delivery is the bedrock of gene therapy, and Solid is building an innovative technology library across the key components:

- Capsids
- Genetic Regulators (Promoters, UTRs, Introns)
- Immunomodulation (NAb^{+}, Redosing)
- CMC Purity

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 (DMD)				FIH Data Q1 2025 [1]	✓
SGT-212	Friedreich's Ataxia (FA)				First Patient Dosed 2H 2025	✓
Cardiac						
SGT-501	RYR2-Mediated CPVT				IND 1H 2025	✓
	CASQ2-Mediated CPVT					✓
SGT-601	TNNT2 DCM				IND 2H 2026	✓
SGT-401	BAG3-Mediated DCM					✓
SGT-701	RBM20 DCM					✓
Mayo Clinic Collaboration	Six Undisclosed Targets					✓
Platform						
Capsid Library [2]	Cardiac & NM				FIH Data Q1 2025 [3]	✓

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. Initial safety, expression and biomarker data for first 3 patients dosed; 2. Cardiac Capsid Library currently in NHPs, Mice and Pigs; 3. AAV-SLB101

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January 2025 Corporate Update

INSPIRE DUCHENNE trial ongoing with 4 patients dosed safely to date *

Patient Dosing Ongoing In SGT-003 Phase 1/2 INSPIRE DUCHENNE Trial

INSPIRE DUCHENNE Trial Intended to Support Accelerated Approval

Friedreich's ataxia FDA IND Clearance Received for SGT-212

Cardiac Gene Therapy Pipeline Primed for Transformative Expansion

Strong Progress In Capsid Library Out-licensing

First-in-human evaluation of SGT-003 for treatment of Duchenne muscular dystrophy; initial data expected Q1 2025

* Data reported on January 1, 2025

January 2025 Corporate Update

INSPIRE DUCHENNE expanded globally with intent to support accelerated approval in U.S.

- Patient Dosing Ongoing In SGT-003 Phase 1/2 INSPIRE DUCHENNE Trial
- **INSPIRE DUCHENNE Trial Intended to Support Accelerated Approval**
- Friedreich's ataxia FDA IND Clearance Received for SGT-212
- Cardiac Gene Therapy Pipeline Primed for Transformative Expansion
- Strong Progress In Capsid Library Out-licensing

INSPIRE DUCHENNE trial protocol expanded to enroll approximately 43 participants and broaden age cohorts to 4 to < 7 and 7 to < 12 years of age

January 2025 Corporate Update

SGT-212: the first full-length frataxin replacement gene therapy designed to treat the CNS, neuromuscular and cardiac manifestations of Friedreich's ataxia

- Patient Dosing Ongoing In SGT-003 Phase 1/2 INSPIRE DUCHENNE Trial
- INSPIRE DUCHENNE Trial Intended to Support Accelerated Approval
- **Friedreich's ataxia FDA IND Clearance Received for SGT-212**
- Cardiac Gene Therapy Pipeline Primed for Transformative Expansion
- Strong Progress In Capsid Library Out-licensing

FDA IND clearance for SGT-212 – 1st dual route of administration – targeting CNS, neuromuscular and cardiac manifestations of FA announced January 2025

January 2025 Corporate Update

In-house development coupled with external collaborations expected to build dominant cardiac precision genetic medicine pipeline by 2030

- Patient Dosing Ongoing In SGT-003 Phase 1/2 INSPIRE DUCHENNE Trial
- INSPIRE DUCHENNE Trial Intended to Support Accelerated Approval
- Friedreich's ataxia FDA IND Clearance Received for SGT-212
- **Cardiac Gene Therapy Pipeline Primed for Transformative Expansion**
- Strong Progress In Capsid Library Out-licensing

Cardiac pipeline positioned to evolve over next five years with multiple INDs:

- **CPVT**: 1H 2025 *
- **TNNT2**: 2H 2026 *
- **Mayo Clinic Collaboration**: 2027-2030 *

* Expected IND submission windows

January 2025 Corporate Update

Multiple capsid and promoter libraries and the integration of AI for future technology generation all expected to establish Solid as industry leader in gene therapy innovation

- Patient Dosing Ongoing In SGT-003 Phase 1/2 INSPIRE DUCHENNE Trial
- INSPIRE DUCHENNE Trial Intended to Support Accelerated Approval
- Friedreich's ataxia FDA IND Clearance Received for SGT-212
- Cardiac Gene Therapy Pipeline Primed for Transformative Expansion
- **Strong Progress In Capsid Library Out-licensing**

AAV-SLB101 (proprietary capsid in SGT-003) has been licensed to 15 academic labs and corporations

Solid is building additional cardiac and neuromuscular capsid and promoter libraries with **final capsid selection targeted for Q4 2025**

Neuromuscular Lead Program

Duchenne Muscular Dystrophy (Duchenne)

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INSPIRE DUCHENNE Clinical Trial Update

Patient dosing initiated Q2 2024

- Six (6) clinical sites in North America (5 in the U.S., 1 in Canada) have been activated as of December 2024
- U.K. MHRA authorized the INSPIRE DUCHENNE clinical trial application (CTA) in November 2024, with expected site activation in 2H 2025
- First international participant expected to be dosed in January 2025
- Four (4) participants have been dosed as of January 1, 2025 – dosing has been well tolerated in all patients to date, with no serious adverse events (SAEs) observed
- Initial safety, expression and biomarker data expected in Q1 2025
- Solid has designed the revised INSPIRE DUCHENNE protocol to pursue accelerated approval in the U.S.

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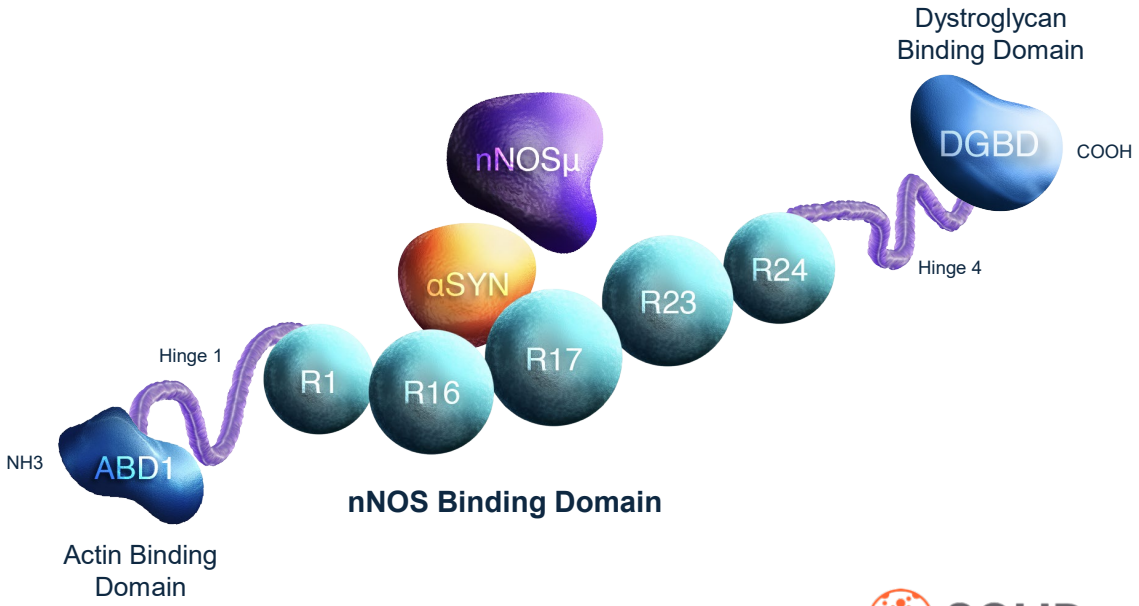
SGT-003 Utilizes an Optimized Transgene, Next Generation Capsid and Improved Manufacturing Process

Next-generation construct has shown promising results in preclinical testing

Transgene | Capsid | Manufacturing Process

Solid's microdystrophin uniquely includes the nNOS binding domain – loss of sarcolemmal nNOS impairs nitric oxide-mediated vasodilation and can lead to functional ischemia[2,3]

- nNOS may lead to reduction of exercise-induced fatigue associated with Duchenne[2,3]
- Microdystrophin inclusion of R16/R17 facilitates binding of α-Syntrophin, which mediates binding of nNOS in skeletal muscle[4]
- α-Syntrophin is the only dystrophin-associated syntrophin that binds nNOS; constructs without R16/R17 domain cannot recruit nNOS[4]

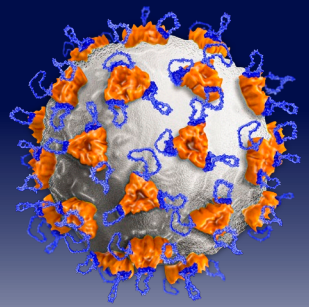


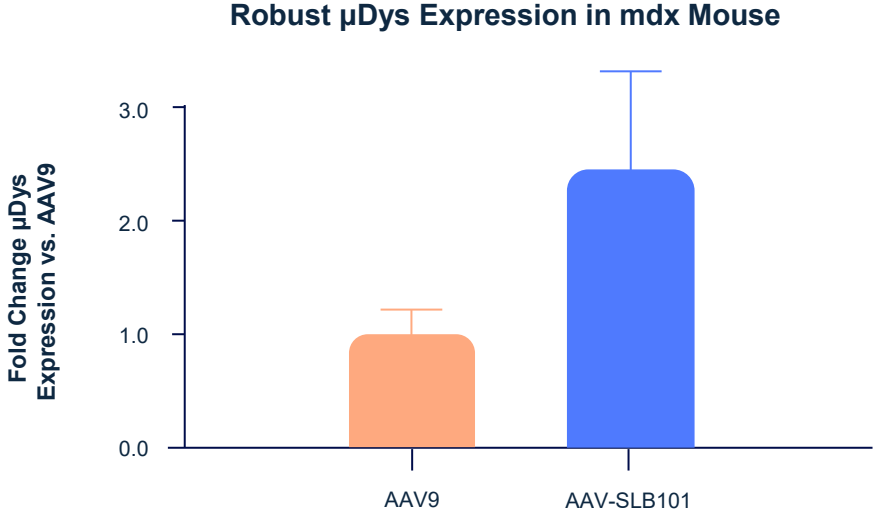
SGT-003 Utilizes an Optimized Transgene, Next Generation Capsid and Improved Manufacturing Process

Next-generation construct has shown promising results in preclinical testing

Transgene | **Capsid** | Manufacturing Process

AAV-SLB101 is a rationally designed capsid with the goal of improving skeletal muscle tropism





Preclinical Data Indicate AAV-SLB101 Capsid Elicited Rapid Transduction & Expression Within 1 Week of Dosing

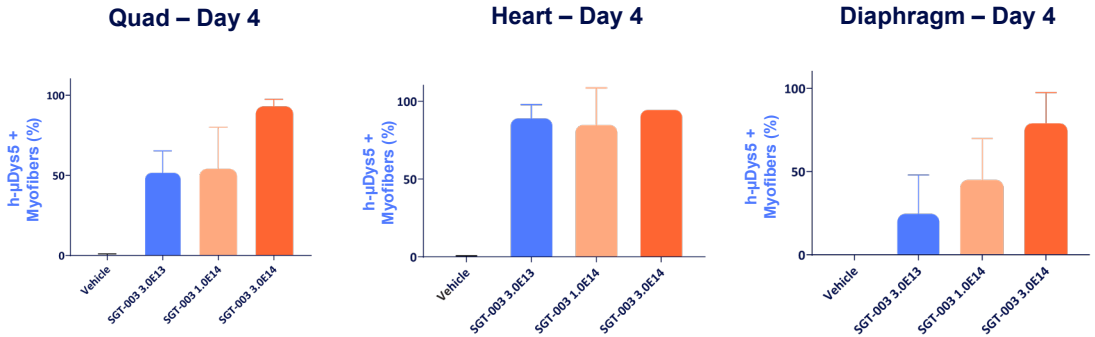
After IgG antibody levels are reduced below a certain threshold, potential redosing opportunities may exist for capsids capable of rapid transduction

Transgene | **Capsid** | Manufacturing Process

- Microdystrophin expression observed within 4 days of dosing in mice
- Additional work underway to evaluate potential redosing in Duchenne, with opportunity to expand addressable market

SLB101: Rapid Transduction & Expression by Day 4

SGT-003



Data on file. Solid Biosciences. 2025.

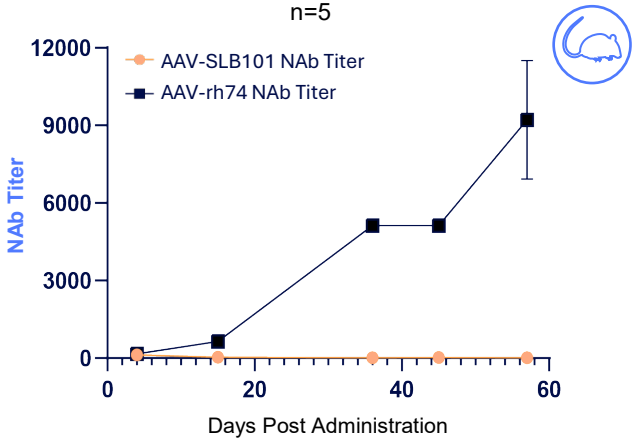
Preclinical Studies Show Low Cross-Reactivity Between AAV-SLB101 and AAVrh74 Neutralizing Antibody Titers in NHPs and Mice

After IgG antibody levels are reduced below a certain threshold, potential redosing opportunities may exist for capsids capable of rapid transduction

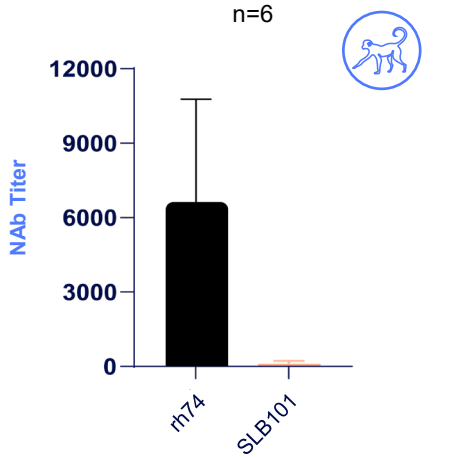
Transgene | **Capsid** | Manufacturing Process

- SLB101 NAb titer levels were evaluated longitudinally following treatment with rh74 in mice
- Low SLB101 NAb titer levels detected in mice treated with rh74 out to day 60
- Low cross reactivity between rh74 & SLB101 titers also detected in NHP sera samples

Longitudinal Assessment of SLB101 Nabs



Day 30 Assessment of SLB101 NAbs

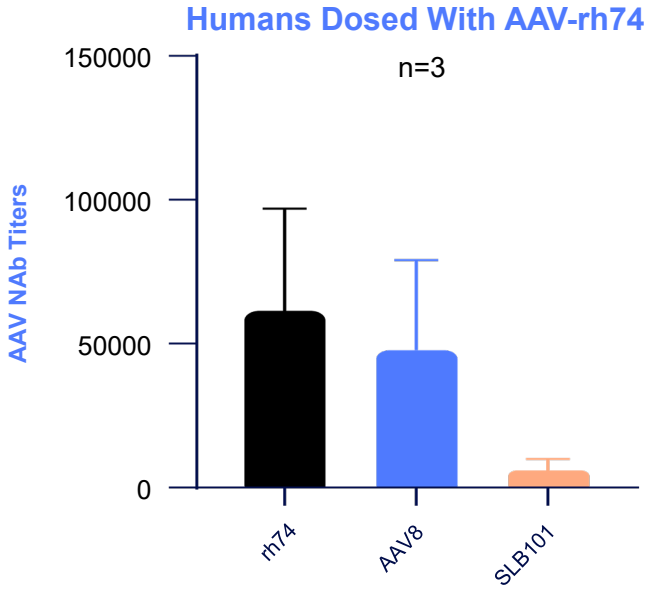


Data on file. Solid Biosciences. 2025.

Low Cross-Reactivity Replicated in Human Sera Samples From Individuals Previously Dosed With AAVrh74 Capsid Indicate Redosing Potential

Findings indicate potential for redosing with SLB101 after treatment with rh74 capsid

Transgene | **Capsid** | Manufacturing Process



Data on file. Solid Biosciences. 2025.

Low cross reactivity between rh74 and SLB101 titers combined with SLB101's ability to induce rapid transduction and expression provide the potential opportunity for redosing Duchenne patients previously treated with rh74

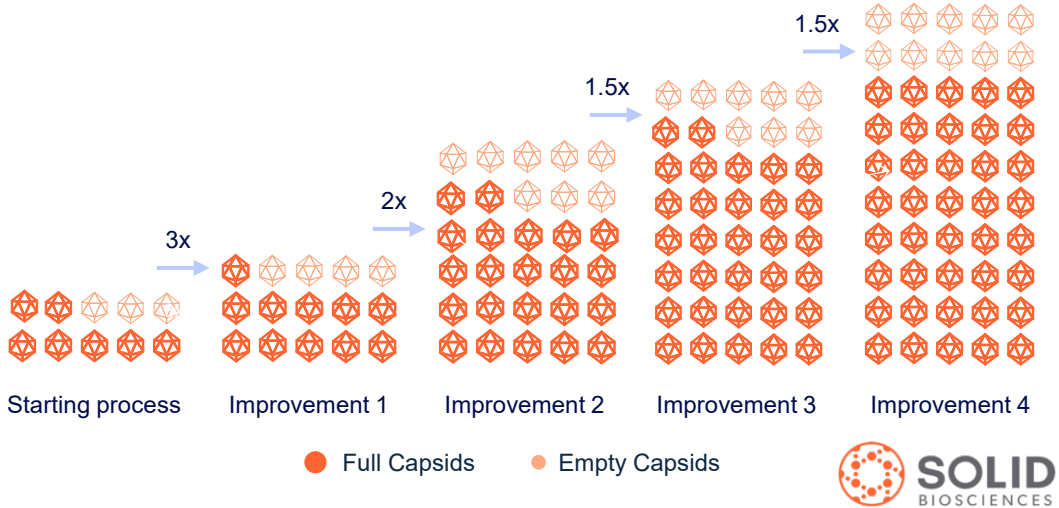
SGT-003 Utilizes an Optimized Transgene, Next Generation Capsid and Improved Manufacturing Process

Next-generation construct has shown promising results in preclinical testing

Transgene | Capsid | **Manufacturing Process**

Current yields and full to empty ratios have potential to significantly **reduce COGS** and **enhance safety and efficacy** for Duchenne and other gene therapies

Full/Empty and Yield Improvements



Initial INSPIRE DUCHENNE Safety and Tolerability Data Consistent With GLP Toxicology NHP Study

NHP GLP TOX Study Findings

- Well tolerated in both groups throughout study
- No early mortality events, no unscheduled take downs
- No pathology findings: organ weight changes, macroscopic or microscopic
- Liver enzyme levels comparable to vehicle at clinical dose level (1E14 vg/kg)
- NHPs dosed at 3x clinical dose level

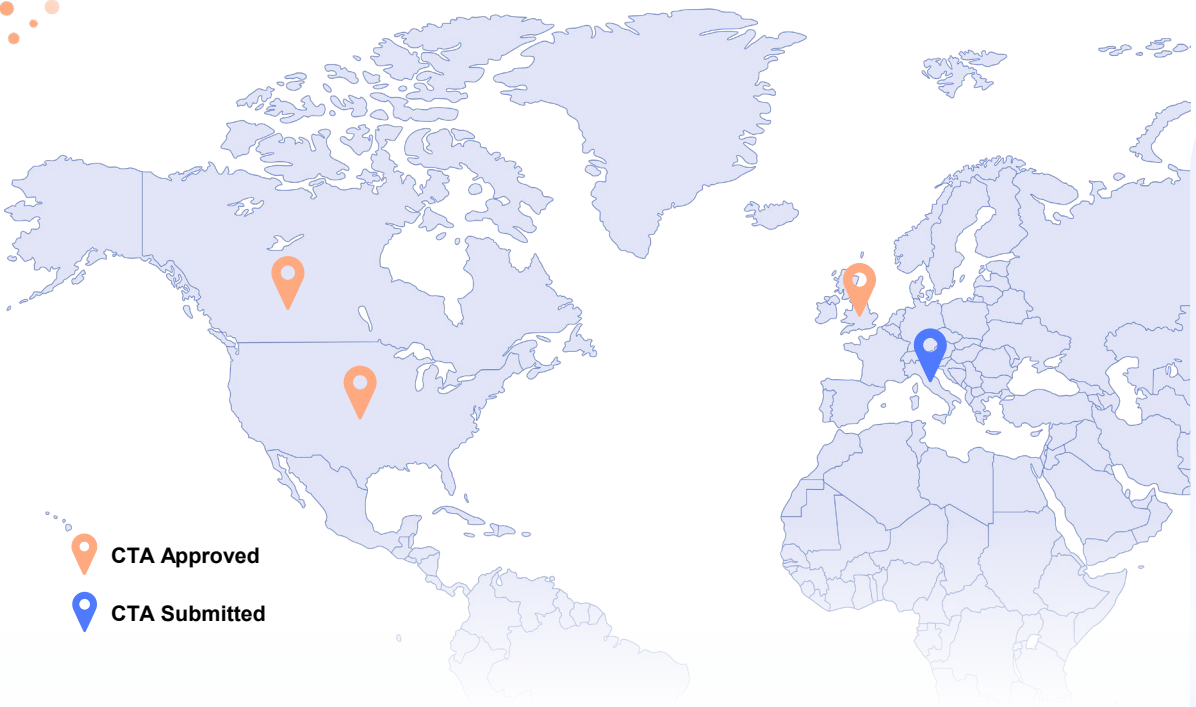
INSPIRE DUCHENNE Initial Safety

Well tolerated in all patients (n=4) dosed as of Jan. 1, 2025

No observed adverse events of special interest (AESIs)

Immunosuppression achieved with use of steroids alone

INSPIRE DUCHENNE SGT-003 Ongoing Phase 1/2 Trial Dosing: Distinct U.S. and Global Regulatory Pathways Anticipated



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has designed comprehensive pivotal programs for parallel **pursue accelerated approval in the US and global regulatory approvals**

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INSPIRE DUCHENNE Clinical Trial Design: Ongoing SGT-003 Phase 1/2 Study

First-in-Human Open-Label, Single-Dose Study

Protocol Updated September 2024 to Enroll an Anticipated 43 Patients

Objective

Primary Objective

- To investigate the **safety and tolerability** of a single intravenous 1E14vg/kg dose of SGT-003

Secondary Objective

- To investigate the **efficacy** of a single intravenous 1E14vg/kg dose of SGT-003

Design

Design

Study includes **2 cohorts** based on age at the time of signing the informed consent:

- Cohort 1: Ambulatory participants aged 4 to < 7
- Cohort 2: Ambulatory participants aged 7 to < 12

All participants must have a genetically confirmed Duchenne diagnosis with a documented dystrophin gene mutation.

Participants must be on a stable dose of at least **0.5 mg/kg/day** of oral daily prednisone or **0.75 mg/kg/day** deflazacort for ≥12 weeks prior to entering the study

Endpoints

Primary Endpoint

- Incidence of treatment-emergent adverse events (AEs) through Day 360

Secondary Endpoints

- Change from baseline of microdystrophin protein levels at Day 90 and Day 360
- Change from baseline in the NSAA score at Day 540
- Change from baseline in stride velocity 95th centile (SV95C) at Day 540

INSPIRE DUCHENNE

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Neuromuscular Pipeline Program

Friedreich's Ataxia (FA)

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

Affected Population

ESTIMATED

~5,000

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: A Revolutionary Dual Administration Approach to Address Both Neurologic and Cardiac Manifestations of FA

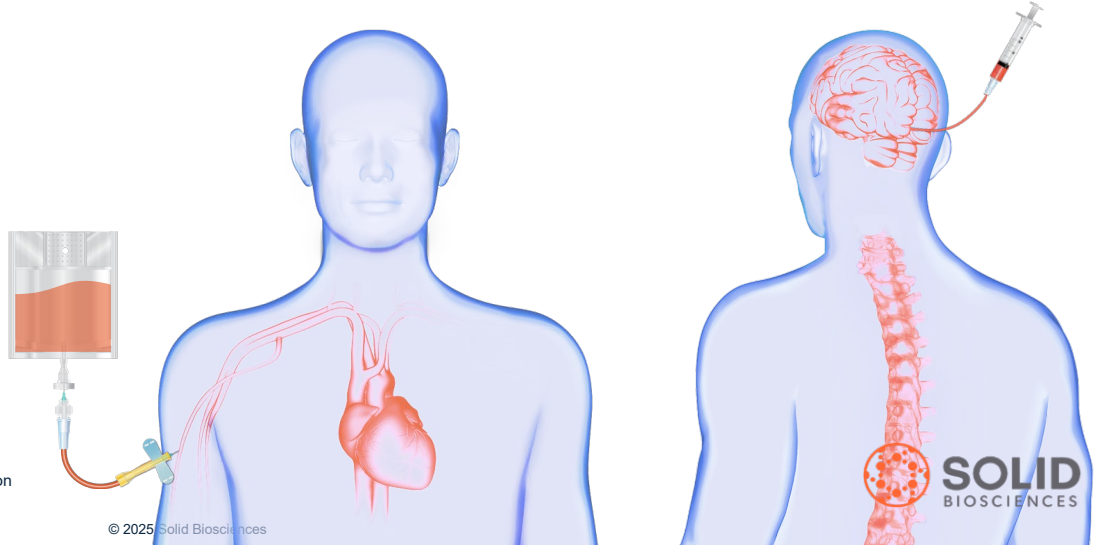
SGT-212
is the only FA gene therapy in development designed to directly address the neurologic and cardiac manifestations of FA

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



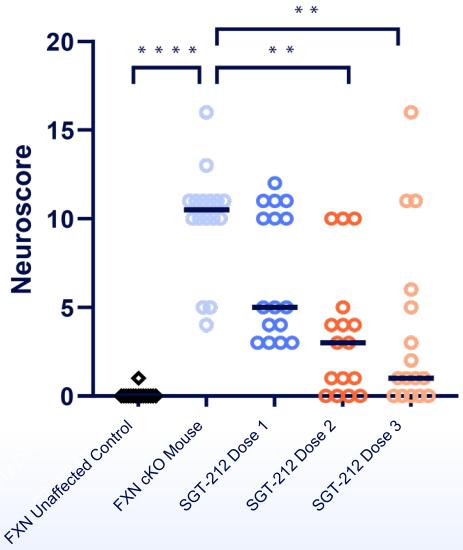
*Administration simplified for illustrative purposes. Actual SGT-212 IDN administration will use FDA-approved, MRI-guided delivery system.

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

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

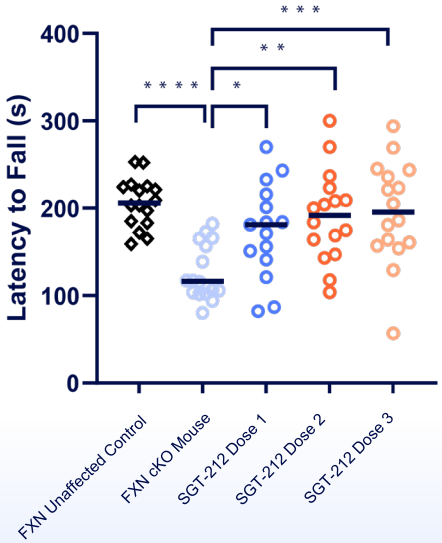
Neurological Assessment Score

Neuroscore[1] (Day 56)



Neuromotor Function Assessment

RotaRod[2] (Day 60)



FXN = Frataxin

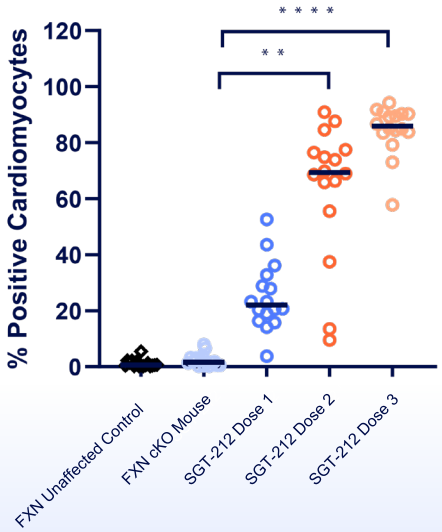
1. The neurological score assessment was used to assess the severity of ataxia. 2. The RotaRod test evaluates coordination and balance by measuring the time to fall for mice running on a spinning rod that progressively accelerates – a decreased latency to fall indicates neuromotor impairment.
Data on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

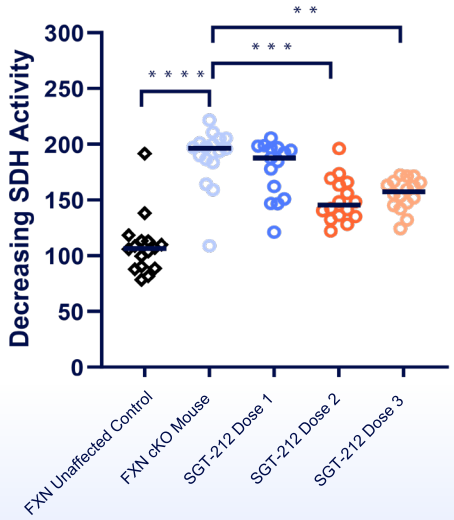
FXN Cardiac Expression

In Situ Hybridization in Heart



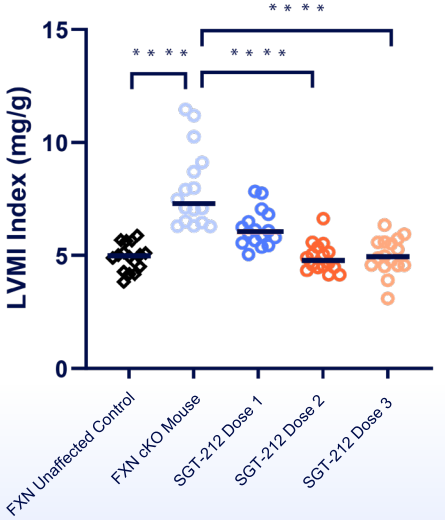
Mitochondrial Function

Succinate Dehydrogenase (SDH)



Indicator of Cardiac Structure

Left Ventricular Mass Index (Day 30)*



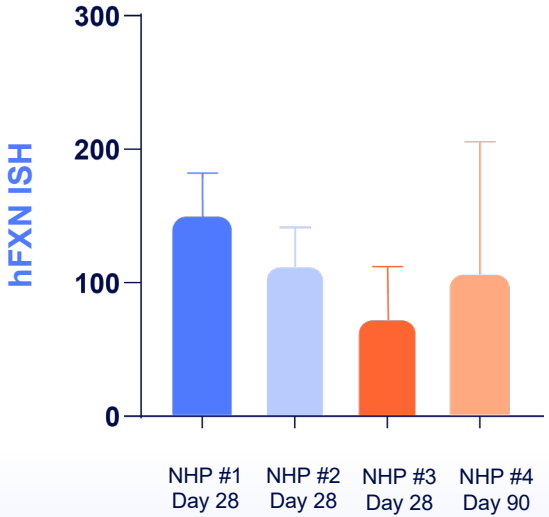
*Research has indicated that increased LVMI is correlated with increased risk of all-cause mortality (Pousset F, et al. 2015)

Data on file. Solid Biosciences 2024.

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

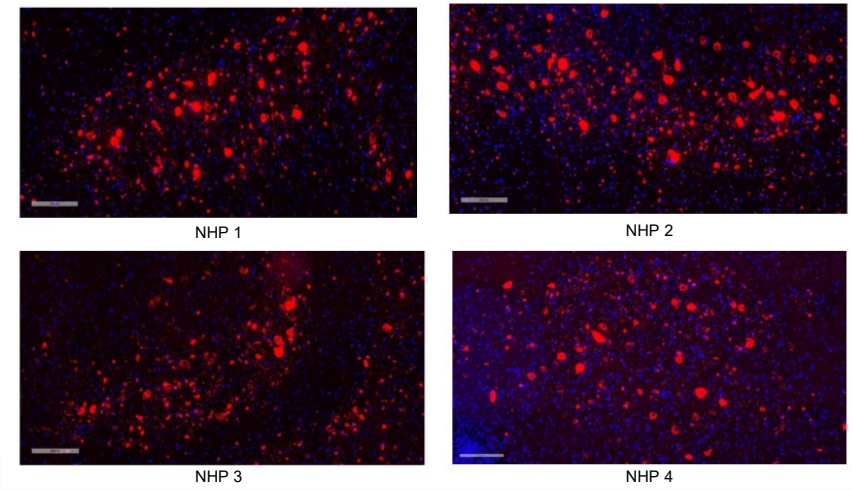
hFXN Expression in Dentate Nuclei (Cerebellum)

In Situ Hybridization



hFXN Properly Localized to Dentate Nuclei (Cerebellum)

In Situ Hybridization



Human frataxin (hFXN)

Data 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.

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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 H2 2025

Objective

Primary Objective

- To evaluate the safety and tolerability of IDN infusion and systemic IV infusion of SGT-212 gene therapy in subjects 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)

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

Affected Population

~33,000

1:10,000

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[2]

Age of Onset

- Typically identified in younger patients (mean onset between 7-9 y/o)[2]

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 to achieve safe expression of wild-type CASQ2 protein using a cardiac-selective promoter and an optimized transient transfection manufacturing process

RYR2=Ryanodine receptor, CASQ2= Calsequestrin 2

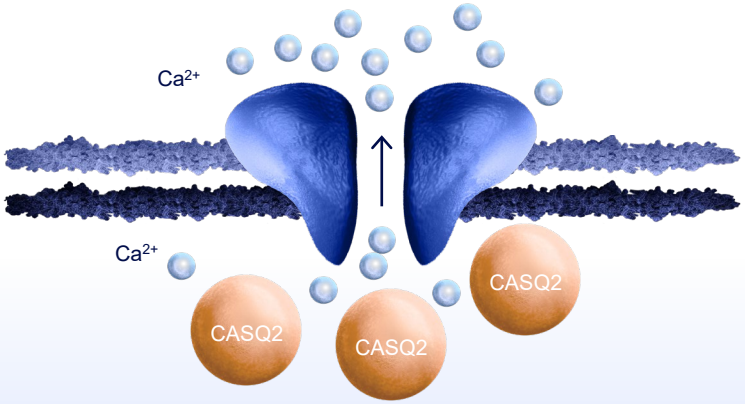
1. Napolitano, et al. 2022 2. Abbas, et al. 2022

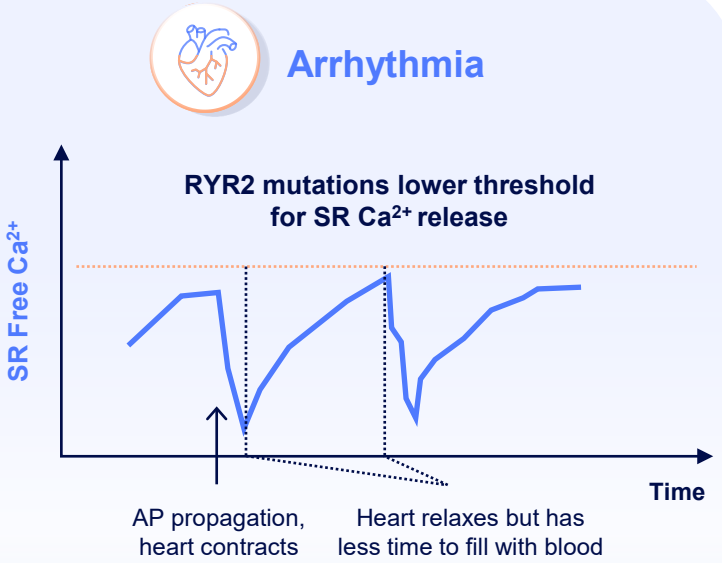
Rationale for CASQ2 Augmentation in RYR2 CPVT

In RYR2 pathogenic mutations, normal CASQ2 levels are insufficient to buffer Ca^{2+} contributing to delayed afterdepolarizations (DAD)

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





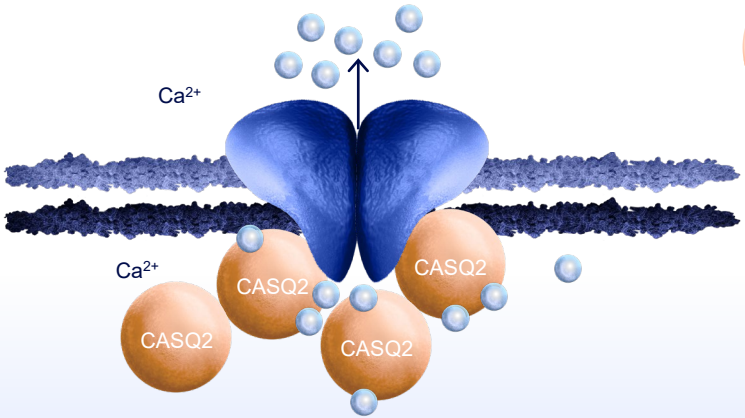
RyR, Ryanodine Receptor; SR, sarcoplasmic reticulum; AP, action potential; Ca^{2+}, calcium
Sources: Proprietary data from Priori Lab

Rationale for CASQ2 Augmentation in RYR2 CPVT (cont.)

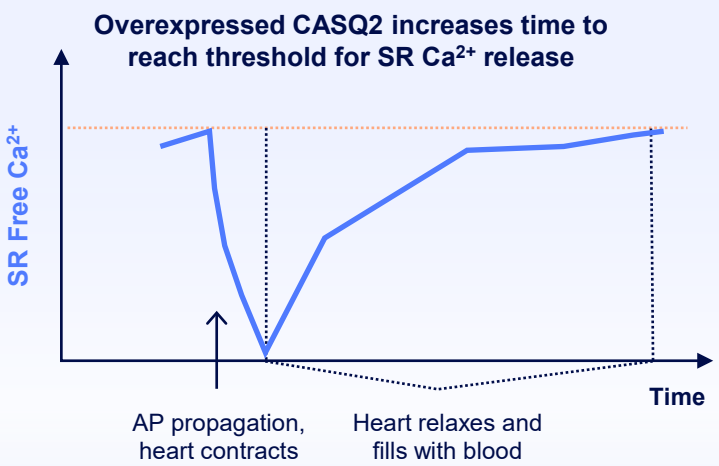
Cardiac delivery of SGT-501 is intended to increase CASQ2, thus enhancing Ca^{2+} buffering and counteracting Ca^{2+} sensitivity caused by RYR2 pathogenic mutations

RYR2 Mutation-Related CPVT + Overexpressed CASQ2

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



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

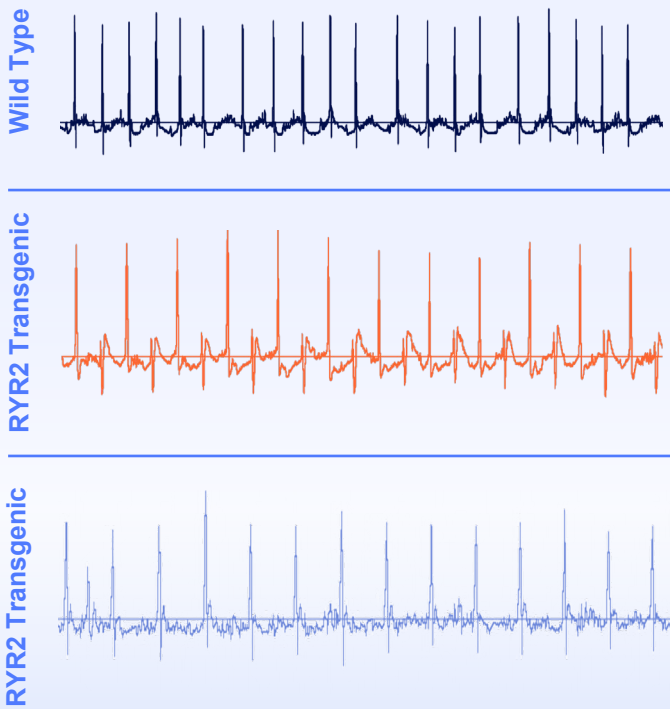
RYR2 CPVT Transgenic Mouse Model Used To Support Proof of Concept For AAV Gene Delivery of Human CASQ2

ECGs from WT and RYR2 transgenic mice 85 days after dosing with vehicle or SGT-501 and following challenge with β-adrenergic agents

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**

Data on file. Solid Biosciences 2025.

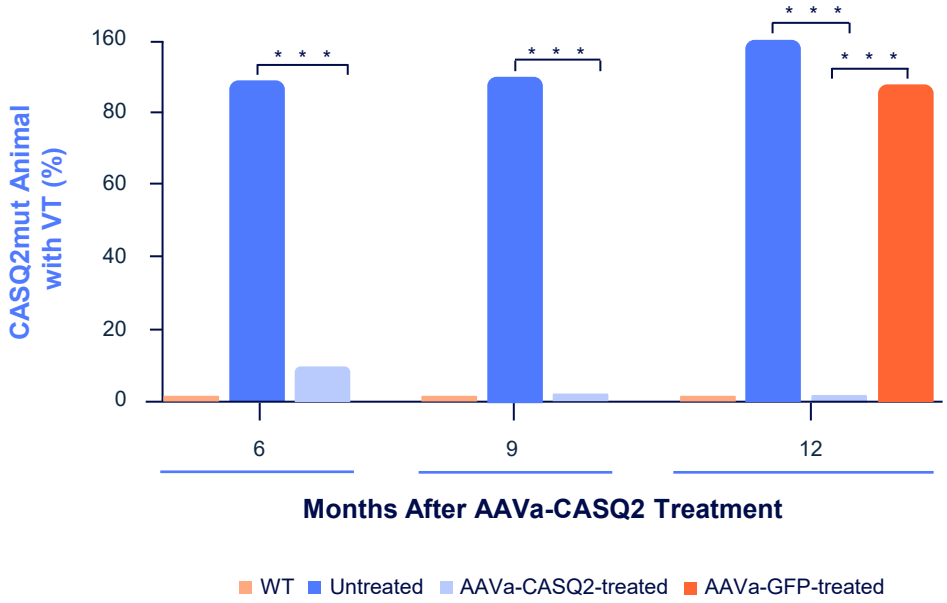
AAV-CASQ2 Treatment Eliminated Arrhythmias in CASQ2-Mutant Mouse Model

Data suggests CASQ2 augmentation was well tolerated & highly protective in CPVT-relevant transgenic mouse models

CASQ2-Related CPVT Mouse

Significantly fewer **CASQ2 mutant mice** experienced arrhythmias 6-12 months after AAVa-CASQ2 gene therapy[1]

40-50% transduction, achieved in both neonates and adult mice, prevented development of VT upon β-adrenergic challenge[1]



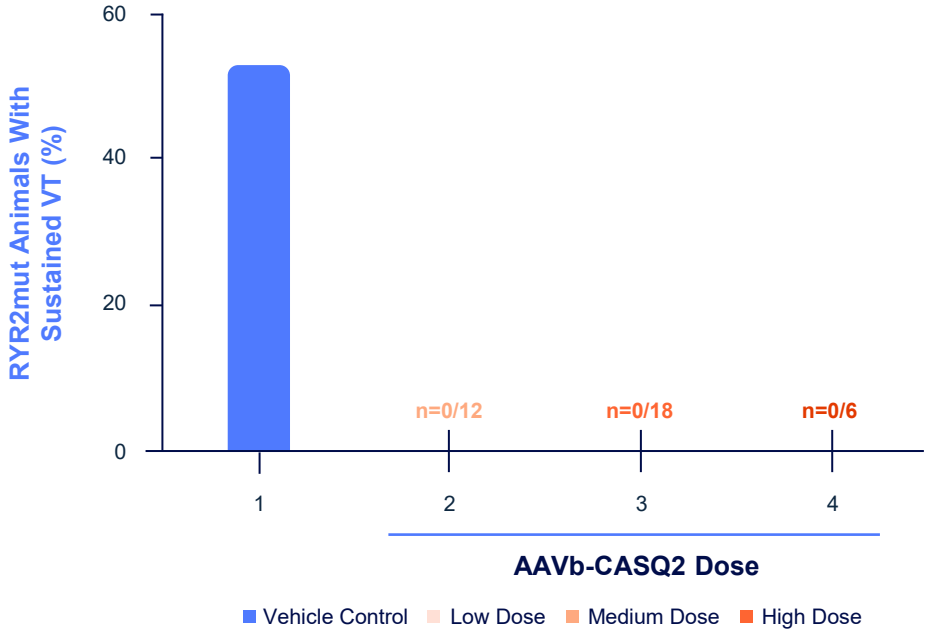
***P<0.001, AAVa-CASQ2–treated vs untreated and AAVa-CASQ2–treated vs AAVa-GFP–treated,
VT, Ventricular Tachycardia
1. Denegri, et al. 2014

AAV-CASQ2 Treatment Eliminated Arrhythmias in RYR2 Mouse Model

Data suggests CASQ2 augmentation was well tolerated & highly protective in CPVT-relevant juvenile transgenic mouse models

RYR2-Related CPVT Mouse

At 12 weeks old, **none** of the **RYR2 mutant mice** treated with AAVb-CASQ2 gene therapy exhibited arrhythmias[1]



1. Priori Lab, unpublished data

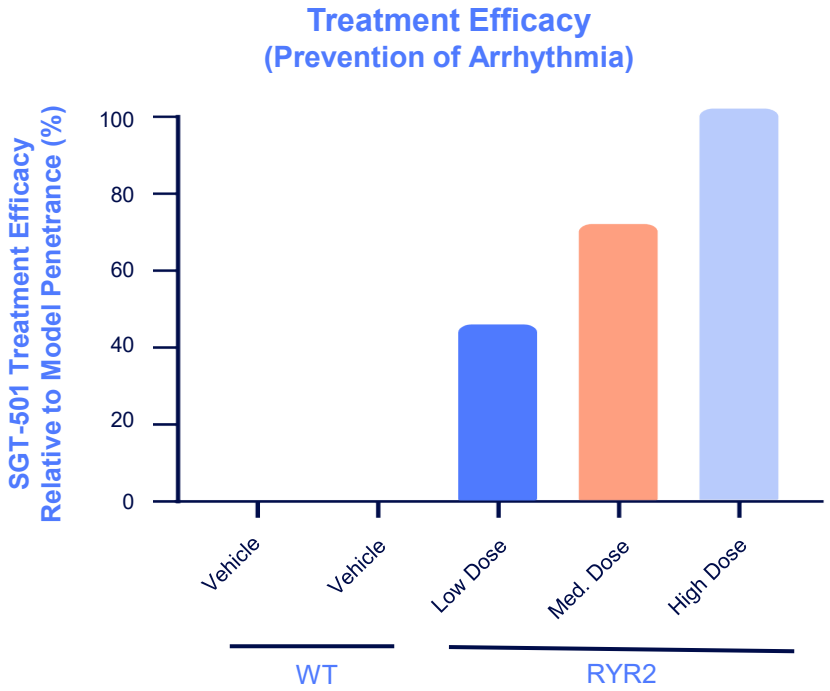
SGT-501 Demonstrated Protection From Sustained VT & Arrhythmia

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 40-99% efficacy rates upon β-adrenergic challenge 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 Elicited Steady Cardiac Protein Expression Though Month 6

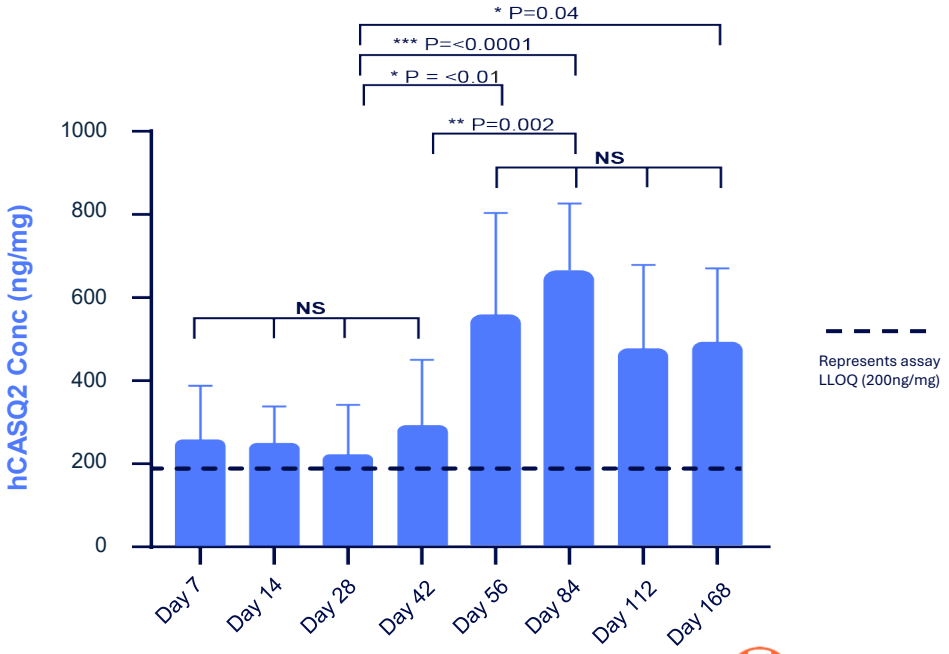
Robust expression levels continued through month 6 indicating potential durability and stability of expression

hCASQ2 Protein Expression Trends

Expression significantly increased at Day 56, peaking at Day 84, followed by continued expression through Day 168, suggesting durable and stable expression profile

At peak expression, hCASQ2 is overexpressed 2-3x over endogenous levels in WT mice

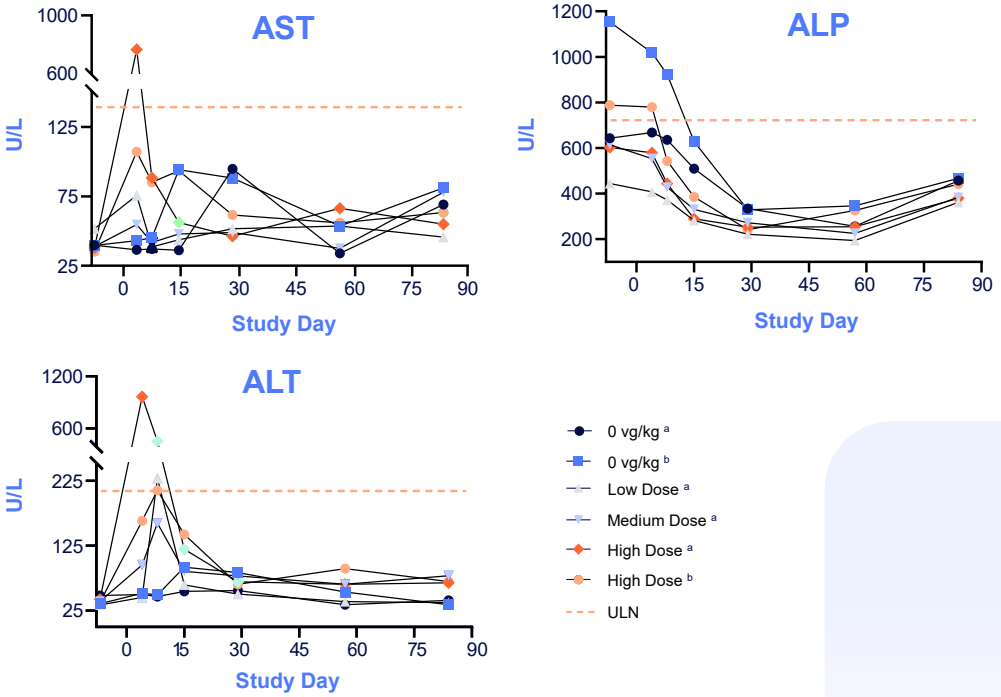
hCASQ2 6-Month Expression



Two-Way ANOVA with Tukey's Multiple Comparisons; N=8 per group.
Data on file. Solid Biosciences 2025.

SGT-501 NHP GLP Toxicology Study Underway

Toxicology studies assessing low-, medium-, and high-dose treatment groups



Data presented as group mean; [a] Single Immunosuppression; [b] Triple Immunosuppression
Data on file as of December 31, 2024. Solid Biosciences.
In-life portion of 6-month NHP GLP toxicology study to be completed in Q1 2025.

Observations

Preliminary liver clinical chemistry shows safe response. Minimal enzyme elevations were seen at Day 4 and resolved by Day 8-15 without intervention, while animals maintained normal clinical signs.

Cardiac Pipeline Program

TNNT2 – Thin Filament Cardiomyopathy

TNNT2-Related Dilated Cardiomyopathy (DCM): A Fatal Disorder With High Unmet Need

Affected Population

ESTIMATED

>27,000

patients in the US

PREVALENCE

~3%

DCM Cases[1]

Cause

Mutations in the TNNT2 gene coding for cardiac troponin T protein, which functions to regulate cardiac muscle contraction, leads to dilated cardiomyopathy (DCM)

Postulated Mechanism: Cardiac troponin T is part of the troponin protein complex that binds actin and regulates cardiac muscle contraction in response to changes in intracellular calcium concentration

Clinical Presentation and Unmet Need

Signs & Symptoms

- Patients typically present with symptoms of heart failure including dyspnea, fatigue and chest pain, or arrhythmia
- Poor long-term prognosis: up to 50% mortality or heart transplant within 10 years

Age of Onset

- DCM caused by mutations in TNNT2 is typically diagnosed between ages 20-50 (~50% onset by age 30)[2], with low diagnosis rates; though estimated US prevalence is ~27,000, only ~5,000 patients are diagnosed

Standard of Care

- No approved therapies address underlying cause of disease

Solid Approach

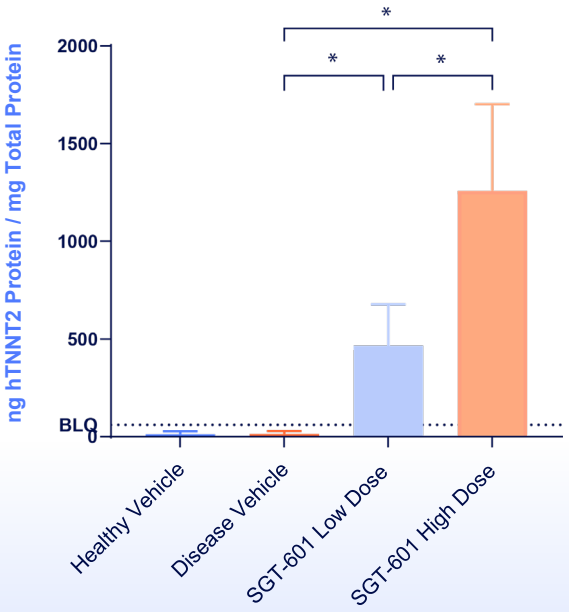
AAV-SLB101 delivered human TNNT2 transgene with a cardiac-selective promoter utilizing transient transfection manufacturing process

1. McNally et al. 2017. 2. Hershberger et al. 2009

Robust, Dose-Dependent, Cardiac-Selective Expression of Human TNNT2 Confirmed in Heart

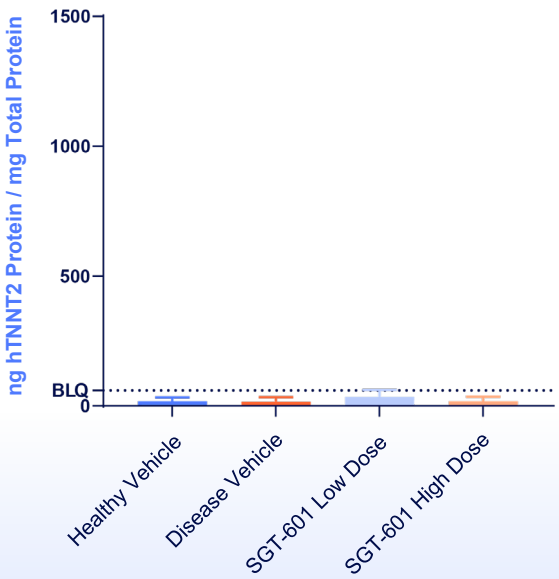
Human TNNT2 Protein

Heart



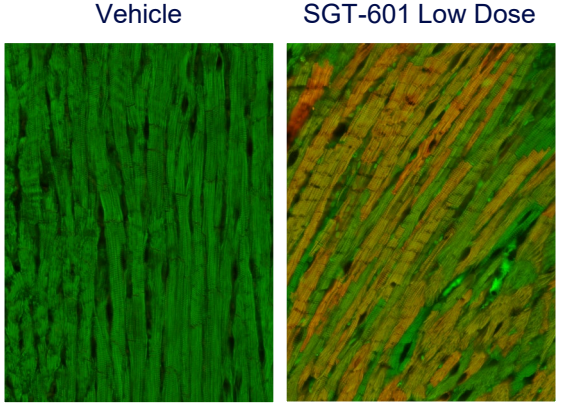
Human TNNT2 Protein

Gastrocnemius



Human TNNT2 Protein Correctly Localized to the Heart Cell Sarcomere

Heart



Homozygous R141W KI mice:

Mouse TNNT2
Human TNNT2

BLQ = Below level of quantitation
Data on file. Solid Biosciences 2025.

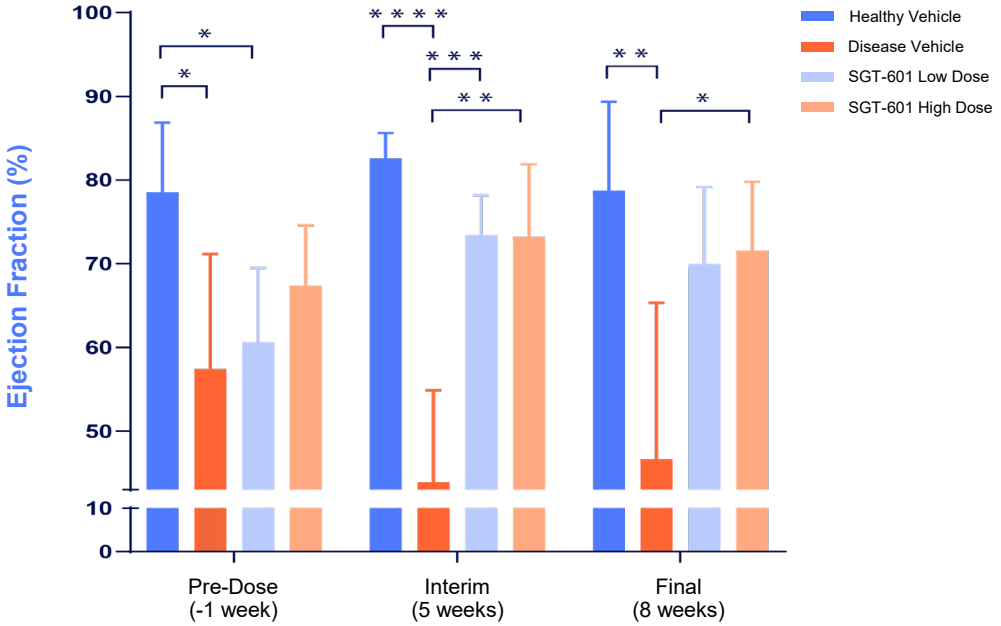
Initial Efficacy Study Suggests Stability of Cardiac Function Over Time After Treatment With SGT-601

Exploratory Efficacy

SGT-601 treatment resulted in restoration of ejection fraction function and stabilization of disease at low and high doses

Mouse data support continued development of AAV-SLB101 gene delivery of human TNNT2

Ejection Fraction Response to SGT-601 Treatment



Data on file. Solid Biosciences 2025.

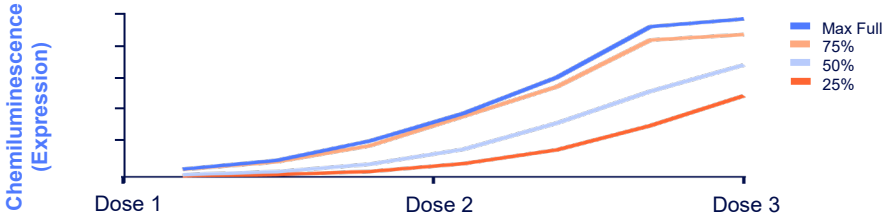
Delivery Platform

Full/Empty Capsid Ratios Can Impact Transduction and Expression of AAV Products

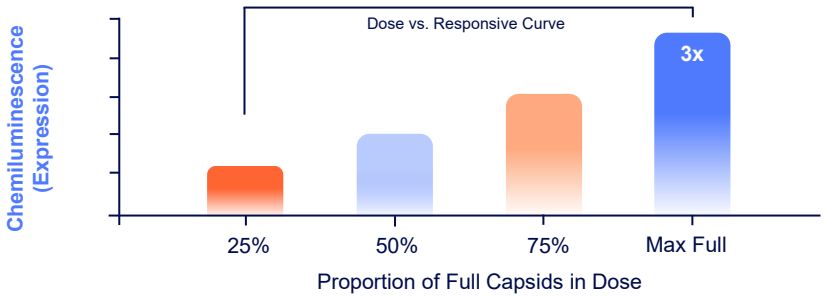
~3-fold difference in chemiluminescence (expression) based on percent full/empty ratios

- Transducing C2C12 cells with an AAV luciferase construct allowed chemiluminescence to act as a readout of expression
- Keeping ddPCR titer constant and serially diluting with empty capsids demonstrated that expression was impacted at constant dose
- **Maximizing the percentage of full capsids has the potential to improve both expression and safety of an AAV product**

AAV-SLB101 DS Protein Expression vs Percent Full Capsids (Titer Match Load)[a]



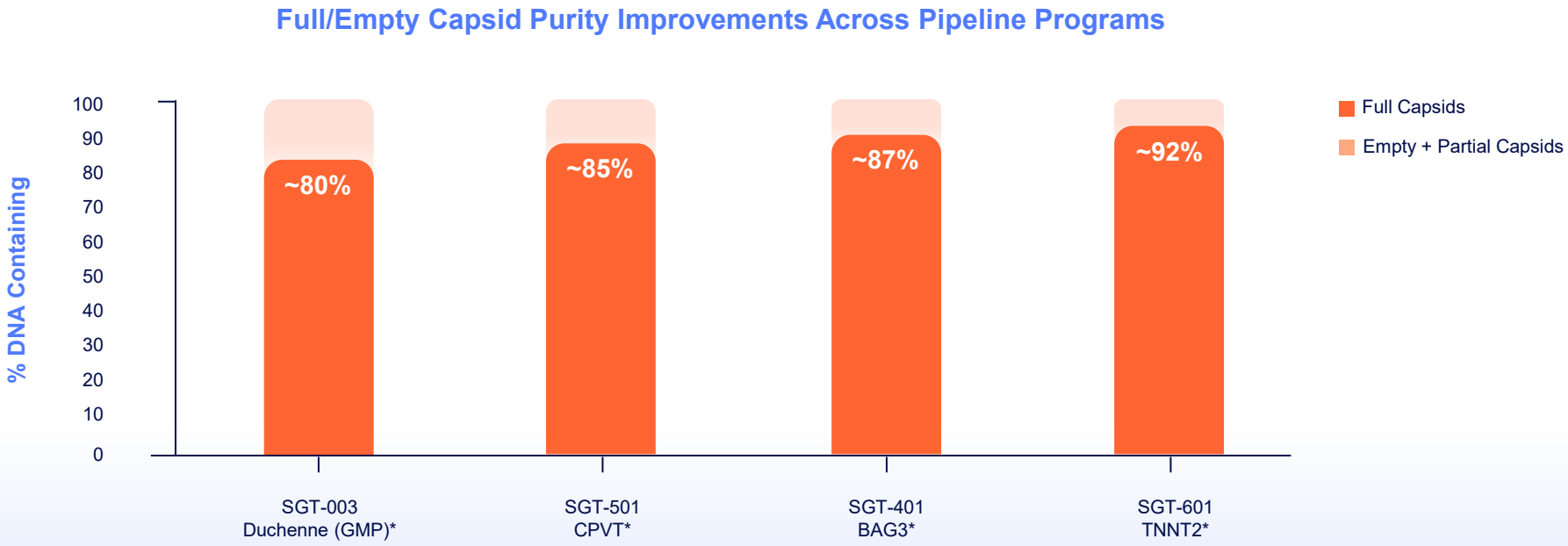
AAV DS Protein Expression vs Percent Full Capsids



ddPCR=Droplet Digital PCR.
[a]AAV-luciferase diluted with empty AAV capsids to yield theoretical 25%-100% full capsids with a series of dilutions based off initial gene of interest titer. N=3 per sample.
Data on file. Solid Biosciences. 2024.

Solid's Manufacturing Constructs Have Potential to Set Industry Standards for Full / Empty Capsid Purity

Further improvements continue in full / empty ratios



*Data on file. SGT-003 GMP scale currently at 1000L, SGT-501 GMP scale currently at 500L, SGT-401 and SGT-601 currently at 2L scale in process development (PD).

Anticipated Near-Term Milestones

Program	Milestone (anticipated)	Timing
Neuromuscular		
SGT-003 for Duchenne	INSPIRE DUCHENNE Phase 1/2 patient dosing commenced	✓
	Submit multiple CTAs for global trial (already authorized in Canada & UK)	Ongoing
	Initial 3 patient Phase 1/2 data (safety, microdystrophin expression & biomarker data)[1]	Q1 2025
SGT-212 for Friedreich's Ataxia	IND cleared by FDA	✓
	Phase 1/2 study initiation	2H 2025
Cardiac		
SGT-501 for CPVT	IND-enabling, GLP toxicology NHP studies	Ongoing
	Planned submission of RYR2 IND	1H 2025
SGT-601 for TNNT2	IND submission	2H 2026
Capsids		
AAV-SLB101	First-in-human data	Q1 2025
Capsid Library (multiple capsids)	Complete rounds of NHP, mouse, and pig studies	Ongoing
Pipeline		
Multiple Pipeline Assets	BAG3 preclinical studies, Mayo Clinic collaboration preclinical work	Ongoing

1. Initial 3 patient safety, expression and biomarker data