SGT-003

A Next Generation Microdystrophin Gene Therapy for Duchenne Muscular Dystrophy

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Chief Medical Officer

Acknowledgements

- Parent Project Muscular Dystrophy
- Patients and Families That Participated in or Enabled the IGNITE DMD Study
- The DMD Community for Partnership and Motivation for Advancing to Next Generation Gene Therapy

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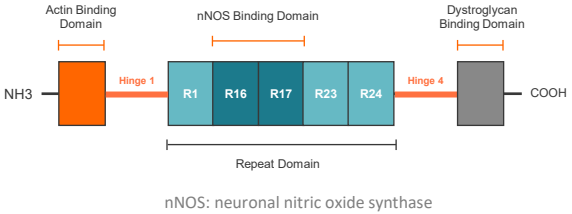
Forward Looking Statements

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 priorities and achieve key clinical milestones; the company’s next-generation Duchenne muscular dystrophy program, SGT-003, including expectations for initial dosing in its clinical study, INSPIRE DUCHENNE, reading out data from the study and the ability to translate pre-clinical study results in human studies; the company’s preclinical programs, including expectations for filing INDs, process development activities, and the company’s future development of preclinical and capsid programs; 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 ability to recognize the anticipated benefits of Solid’s acquisition of AavantiBio; the company’s ability to advance SGT-003, its preclinical programs and capsid libraries on the timelines expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; replicate in clinical trials positive results found in preclinical studies 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 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 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.

SGT-003: A Next Generation AAV Microdystrophin Gene Therapy for Duchenne

Unique Microdystrophin Transgene

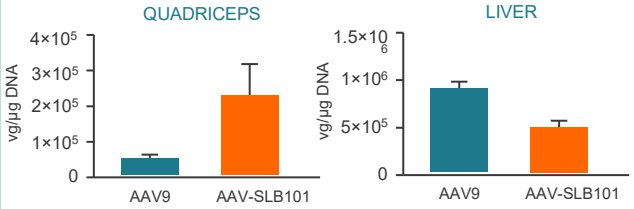
Prevented exercise-induced fatigue and muscle injury by stabilizing the membrane and recruiting nNOS[1,2]



nNOS: neuronal nitric oxide synthase

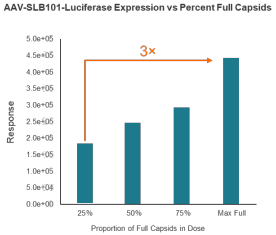
Novel Muscle-Tropic AAV-SLB101 Capsid

Enhanced muscle biodistribution and transduction[3]
Reduced levels in the liver



Optimized Manufacturing Process

Improved full/empty capsid ratio, intended to enhance safety and efficacy[3]

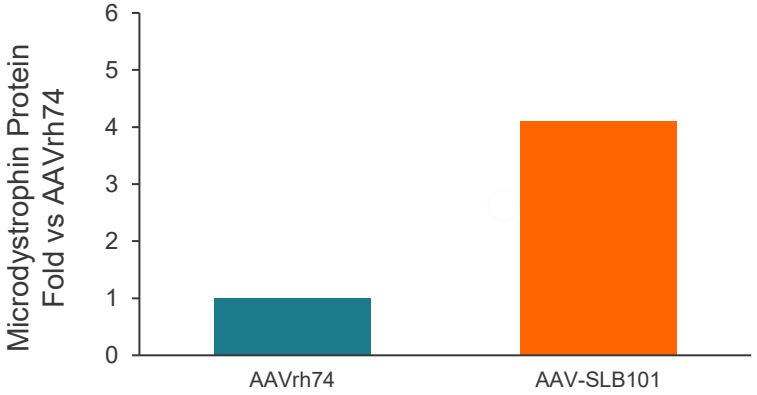


1. Ramos JN, et al. Mol Ther. 2019;27(3):623-635. 2. Koeks. WMS. 2019 3. Data on file, Solid Biosciences, 2024

AAV-SLB101 Exhibited Superior Protein Expression vs AAVrh74 in NHPs

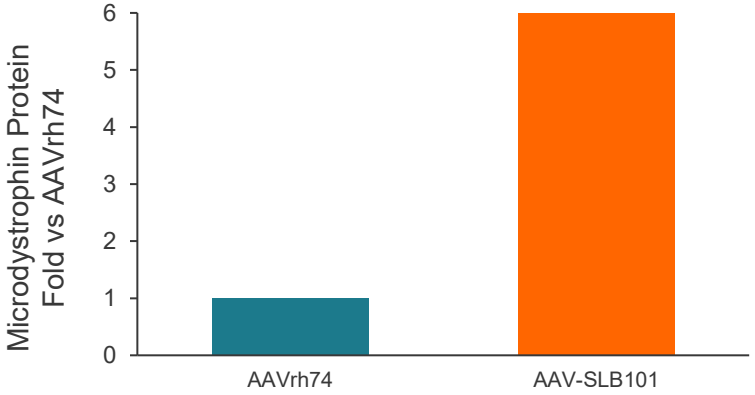
Heart

CK8-Microdystrophin Protein (LC/MS)
in NHP at 1.0E14 vg/kg



Quadriceps

CK8-Microdystrophin Protein (LC/MS)
in NHP at 1.0E14 vg/kg



Superior transgene expression of AAV-SLB101 in NHP muscle tissues supports AAV-SLB101-based therapeutic candidate SGT-003

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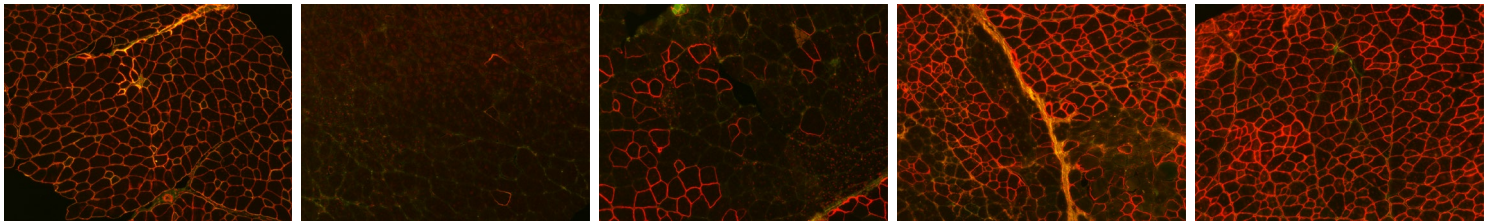
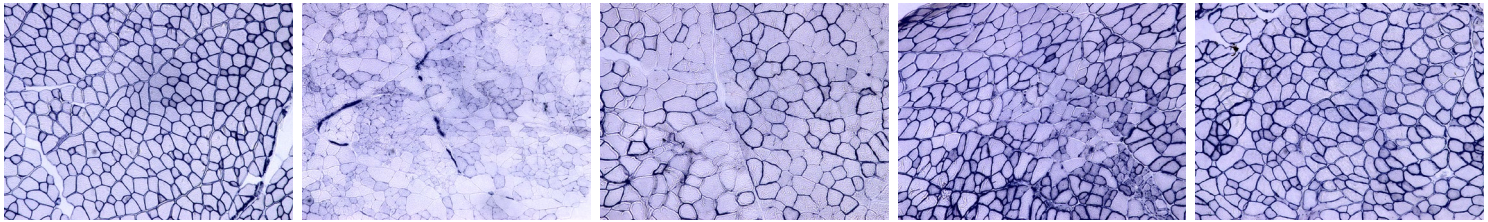
SGT-003 Treatment Led to Robust Membrane-Localized Microdystrophin Expression and nNOS Restoration in *mdx* Mice

MICRODYSTROPHIN

WT- Vehicle | *mdx*- Vehicle | *mdx*- 2.0E12 vg/kg | *mdx*- 6.0E12 vg/kg | *mdx*- 3.0E13 vg/kg



nNOS ACTIVITY



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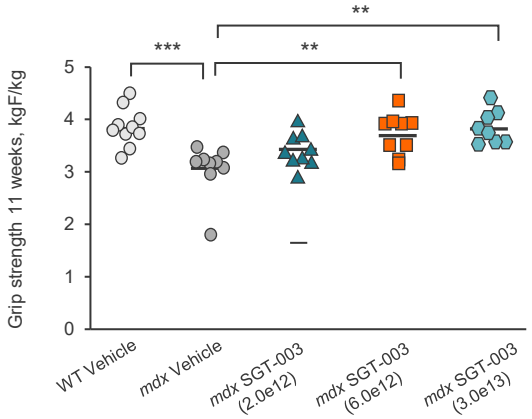
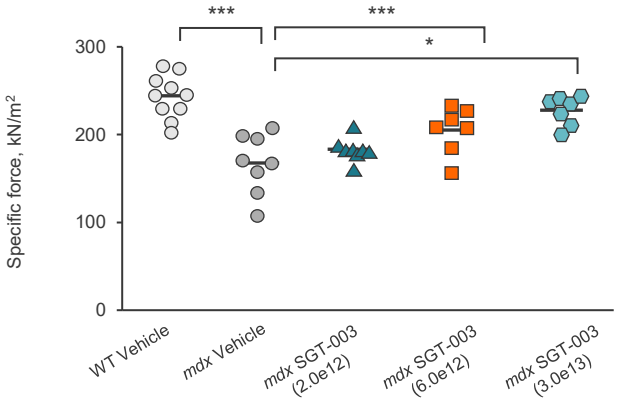
SGT-003 Treated *mdx* Mice Demonstrated Rescued Muscle Function

All functional efficacy measures were significantly improved compared to vehicle at doses of ≥6.0E12 (vg/kg)

GRIP STRENGTH[a]



MUSCLE FORCE[a]



[a]Asterisks indicate statistical significance between the indicated groups.
Data on file. Solid Biosciences. 2024.

INSPIRE DUCHENNE

SGT-003 IND Cleared by the US FDA and Has Advanced to the Clinic

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INSPIRE DUCHENNE – *Active and Openly Enrolling in the U.S.*

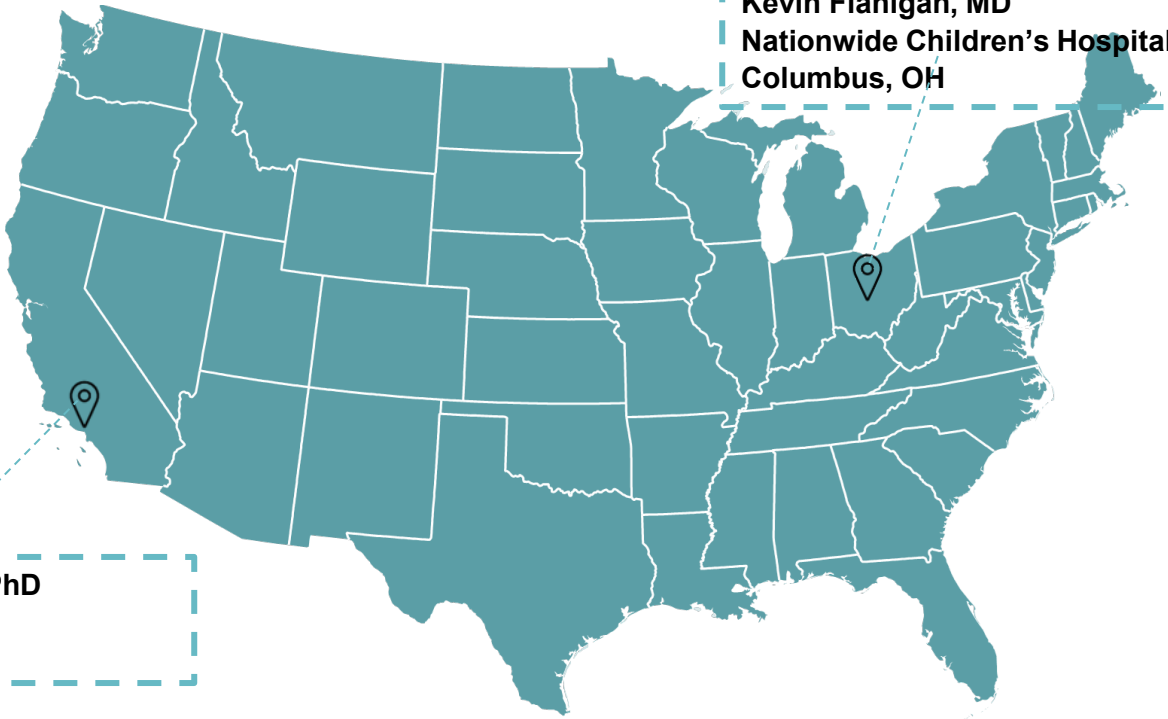
First-in-human study of SGT-003: a next generation AAV microdystrophin gene therapy for Duchenne

- **Multicenter, Open-label, Phase 1/2 Study**
  - Investigating the safety, tolerability, and efficacy of a single IV infusion of SGT-003 at 1E14 vg/kg
  - Using a prophylactic immunomodulatory regimen of 2 mg/kg/day prednisone from Day -1 to Day 30

- **Key Eligibility Criteria**
  - Age: 4 to < 8 years
  - DMD Mutation: Excluding those with any deletion mutation in exons 1 to 11 or 42 to 45, inclusive
  - Antibodies: Negative for AAV antibodies
  - Steroid Regimen: On a stable dose of daily oral steroids for ≥12 weeks prior to entering the study

- **Study Assessments**
  - Safety: Frequent visits in first 30 days and less frequent visits out to 5 years post-dosing
  - Microdystrophin Expression: Needle biopsies of muscle at Screening, Day 90, and Day 360
  - Function: Motor function, pulmonary function, patient reported outcomes, activity, and video assessments

Initiating with 2 Clinical Sites in the United States



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Thank You!

Learn More about INSPIRE DUCHENNE and Reach Out for Additional Information

ClinicalTrials.gov Posting

- A Study of SGT-003 Gene Therapy in Duchenne Muscular Dystrophy (INSPIRE DUCHENNE)
- ClinicalTrials.gov ID: NCT06138639
- https://www.clinicaltrials.gov/study/NCT06138639

Solid INSPIRE DUCHENNE Study Team

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Scan this QR code to navigate to the study posting

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