

Preliminary clinical cardiac findings using POLARIS-101™, a next-generation capsid with high cardiac tropism, in the SGT-003 program

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INTRODUCTION

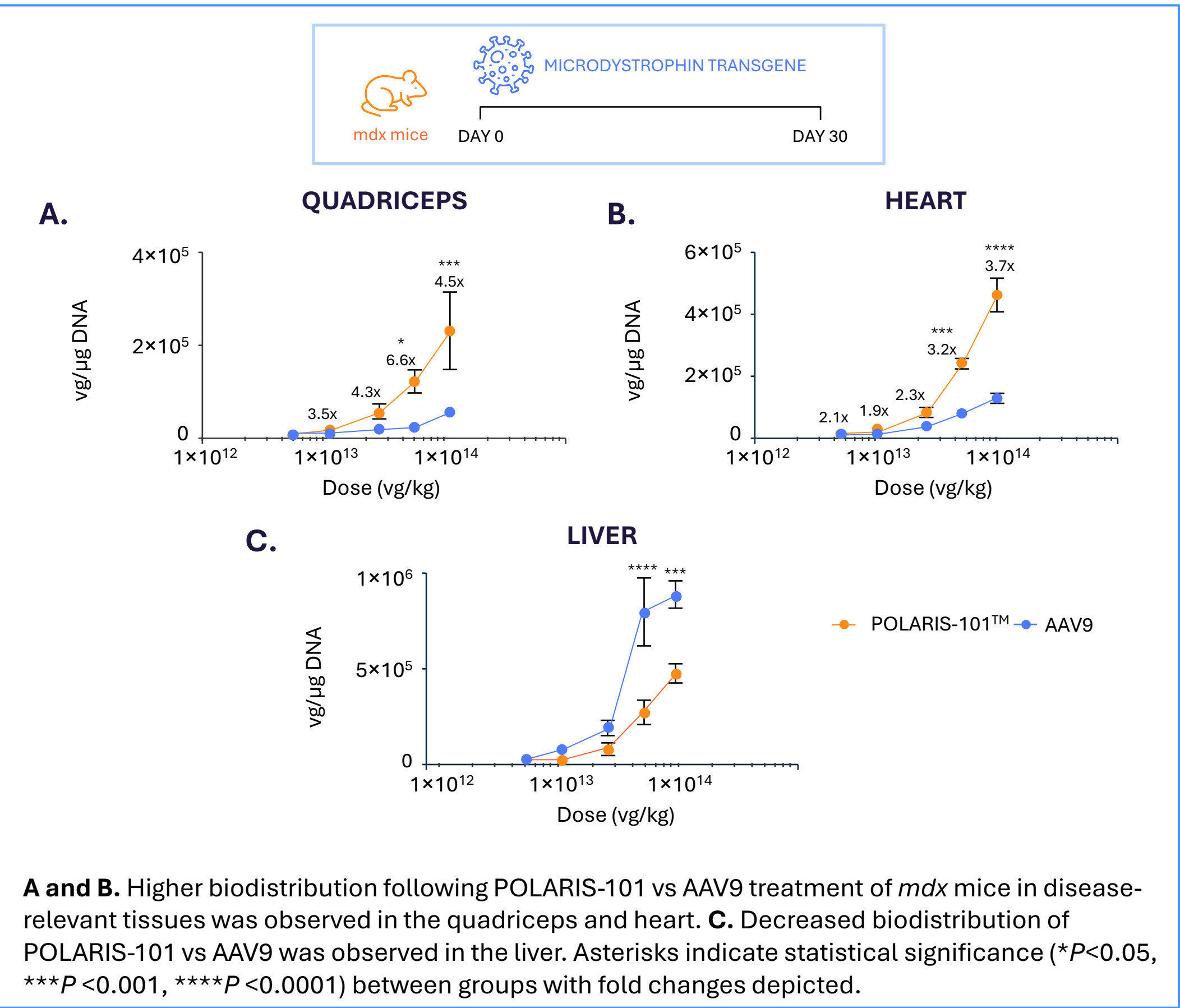
- Duchenne muscular dystrophy is a rare, X-linked recessive disease caused by the loss of functional dystrophin resulting in progressive, severe skeletal myopathy and cardiomyopathy, which is a leading cause of death
- Overt cardiomyopathy typically manifests late in the second decade
- However, intermittent and asymptomatic troponin I elevations and subtle declines in systolic function can be observed even before the age of 10
- This underscores the urgent need for therapies that not only restore dystrophin in skeletal muscle but also in the heart to preserve cardiac function
- POLARIS-101™ (formerly known as AAV-SLB101), Solid Biosciences' proprietary capsid, was rationally designed to improve cardiac and skeletal muscle tropism and is utilized in SGT-003, Solid's next-generation, investigational microdystrophin gene therapy for Duchenne
- POLARIS-101 demonstrated increased muscle tropism and decreased liver uptake in both healthy animals and disease models (wild type and *mdx* mice, non-human primates, and human myotubes and iPSC-derived cardiac myocytes) when compared to first generation vectors (AAV9 and AAVrh74)
- In the *mdx* mouse model of Duchenne and in non-human primates, POLARIS-101 -mediated transgene expression was several-fold higher than AAVrh74 in multiple muscle groups, and specifically four times higher in the heart

METHODS

- INSPIRE DUCHENNE is an ongoing open-label Phase 1/2 study evaluating a single IV dose of SGT-003 (1E14 vg/kg) in participants with Duchenne; primary endpoints include TEAEs through Day 360 and Day 90 microdystrophin expression, with secondary endpoints including evaluations of microdystrophin durability (Day 360) and function assessments
- SGT-003 transduction and microdystrophin expression were evaluated in muscle biopsies collected from INSPIRE DUCHENNE study participants
- Cardiac function and biomarkers of cardiac injury, were monitored

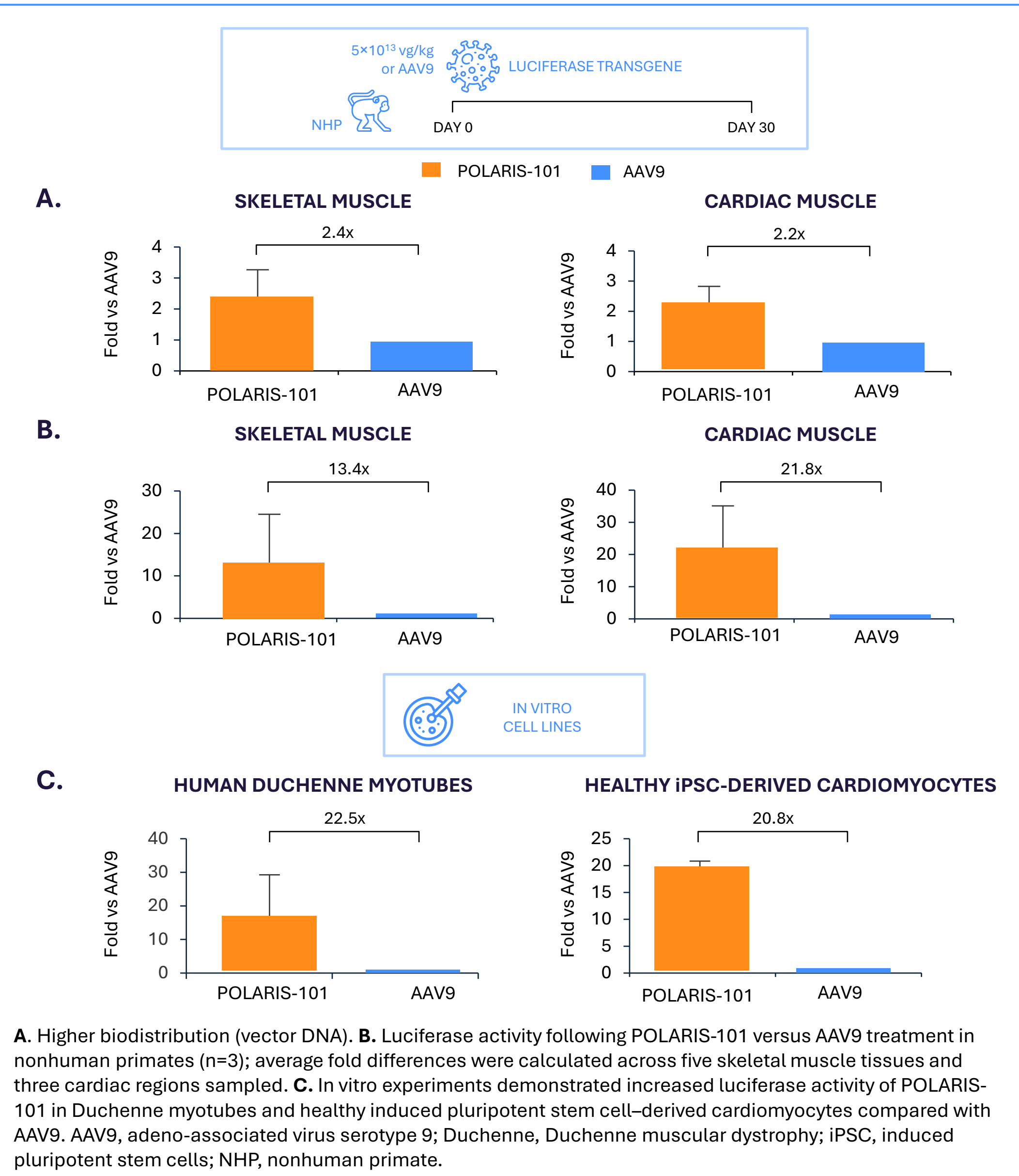
RESULTS

Figure 1. Head-to-Head Comparison in *mdx* Mouse Model of Duchenne



POLARIS-101™ (AAV-SLB101) TRANSDUCTION EFFICIENCY IN THE HEART IS HIGH BASED ON DATA FROM HUMAN CELL LINES, MICE, AND NHPs

Figure 2. Transduction Efficiency of POLARIS-101 Compared to AAV9 in Non-human Primates (NHPs) and Human Cell Lines



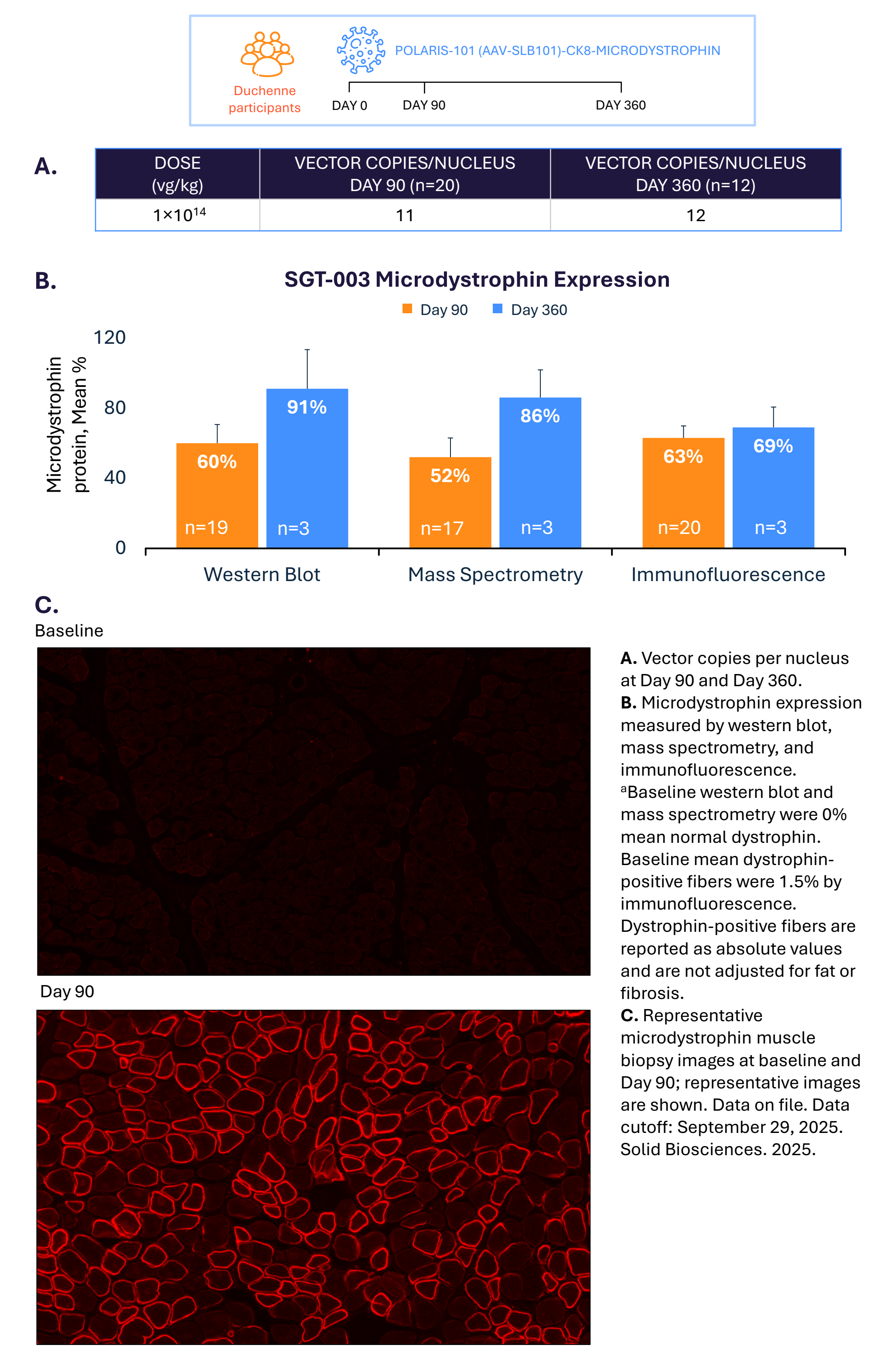
REFERENCES
1. Harmelink M, et al. Presented at ASGCT, May 11-15, 2026, Boston, MA. Data cutoff February 23, 2026. 2. Shieh P, et al. Presented at the International Congress on Neuromuscular Diseases, July 7-11, Florence, Italy. Data cutoff of May 29, 2026. 3. Romanowicz J, et al. J Am Soc Echocardiogr. 2023;36(3):310-323.

ACKNOWLEDGMENTS

This study was sponsored by Solid Biosciences Inc. (Charlestown, MA, USA).

COMPREHENSIVE ORTHOGONAL MEASUREMENTS SHOWED CONSISTENT MICRODYSTROPHIN EXPRESSION^a

Figure 3. Data from INSPIRE DUCHENNE Clinical Trial of SGT-003¹



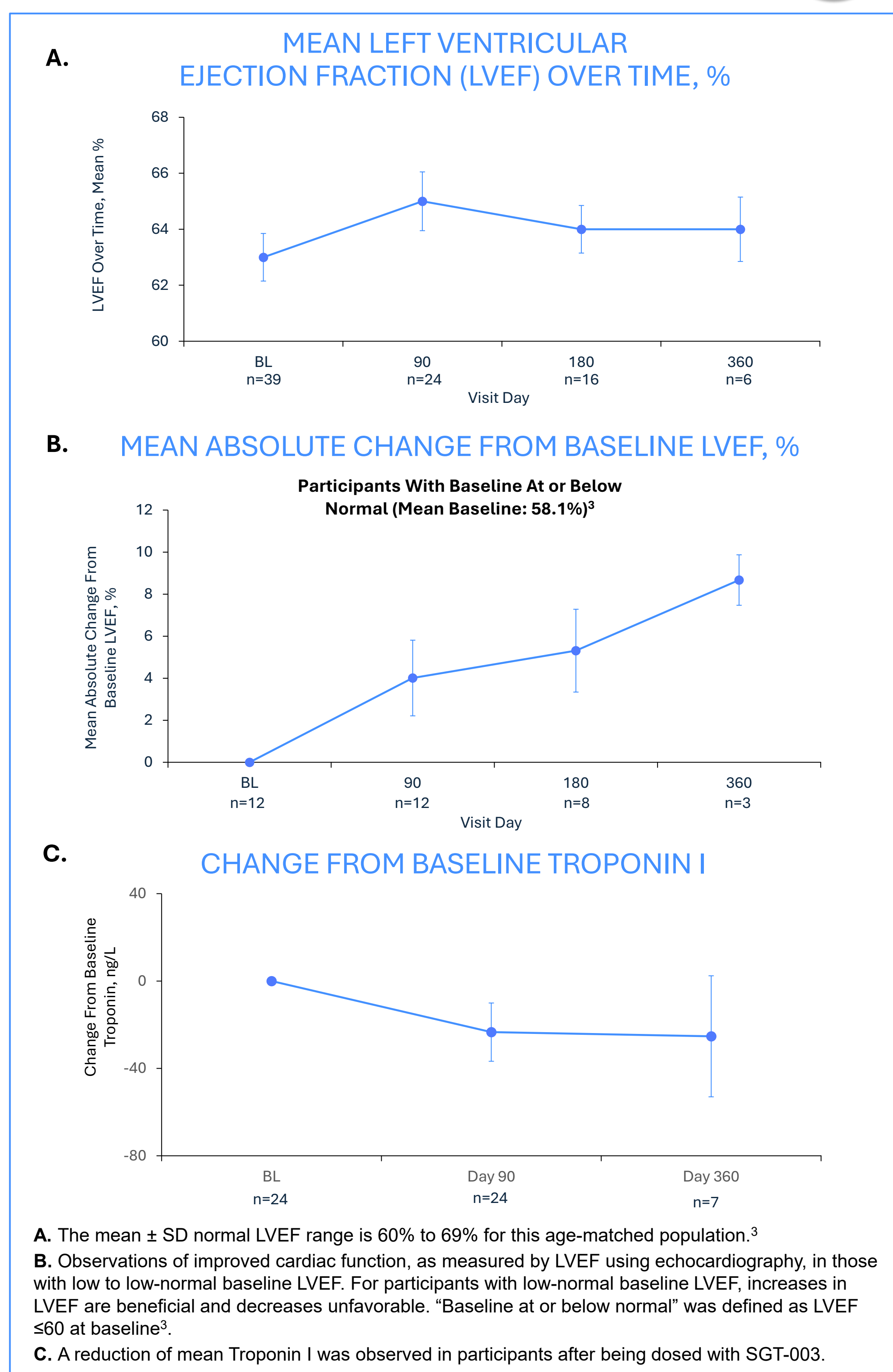
SGT-003 HAS BEEN GENERALLY WELL TOLERATED

Table 1. Safety Summary, INSPIRE DUCHENNE Clinical Study of SGT-003: POLARIS-101 (AAV-SLB101)-CK8-Microdystrophin²

SGT-003 Participants With Treatment-Related SAEs		n (%)
Serious Adverse Events (SAEs)		2 (4.0)*
*One (n=1) participant with a possibly related transient adverse event of inflammation treated with two doses of antibiotics. One (n=1) previously reported immune mediated myositis. Both events have resolved.		
SGT-003 Participants With Treatment-Related AEs		n (%)
Most Common Adverse Events (AEs)	Vomiting	32 (64.0)
	Nausea	27 (54.0)
	Decreased Appetite	15 (30.0)
	Thrombocytopenia	13 (26.0)
	Abdominal Pain	9 (18.0)

Data cutoff of May 29, 2026. Solid Biosciences.

Figure 4. Signals of SGT-003 Cardiac Treatment Effect from INSPIRE DUCHENNE¹



CONCLUSIONS

- Early findings in the Phase 1/2 INSPIRE DUCHENNE (NCT06138639) study investigating SGT-003 indicate a positive safety profile, high levels of microdystrophin expression, and potentially favorable changes in cardiac biomarkers
- The mean levels of troponin I were observed to have declined post-treatment with SGT-003.
- Improvements in systolic function, as measured by LVEF using echocardiography, were also observed and were largely driven by patients with low-normal systolic function at baseline
- These findings suggest that SGT-003 may not only slow skeletal muscle decline but may also mitigate the progressive cardiomyopathy that has increasingly becoming a leading cause of death for patients with Duchenne
- Together, these findings underscore the potential for POLARIS-101 (AAV-SLB101) as a targeted gene therapy vector for neuromuscular and cardiac indications, such as Duchenne.