Capricor Therapeutics™

2026 MDA CLINICAL & SCIENTIFIC CONFERENCE

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Confirmation of musculoskeletal and cardiac benefit in DMD from deramiocel, an allogeneic cell therapy, in the Phase 3 HOPE-3 study

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Forward Looking Statements

Statements in this presentation regarding the efficacy, safety, and intended utilization of Capricor's product candidates; the initiation, conduct, size, timing and results of clinical trials; the pace of enrollment of clinical trials; plans regarding regulatory filings, future research and clinical trials; regulatory developments involving products, including future interactions with regulatory authorities and the ability to obtain regulatory approvals or otherwise bring products to market; manufacturing capabilities; dates for regulatory meetings; the potential that required regulatory inspections may be delayed or not be successful which would delay or prevent product approval; the ability to achieve product milestones and to receive milestone payments from commercial partners; and any other statements about Capricor's management team's future expectations, beliefs, goals, plans or prospects constitute forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Any statements that are not statements of historical fact (including statements containing the words "believes," "plans," "could," "anticipates," "expects," "estimates," "should," "target," "will," "would" and similar expressions) should also be considered to be forward-looking statements. There are a number of important factors that could cause actual results or events to differ materially from those indicated by such forward-looking statements. More information about these and other risks that may impact Capricor's business is set forth in Capricor's Annual Report on Form 10-K for the year ended December 31, 2024, as filed with the Securities and Exchange Commission on March 26, 2025, and in our Quarterly Report on Form 10-Q for the quarter ended September 30, 2025, as filed with the Securities and Exchange Commission on November 10, 2025. All forward-looking statements in this presentation are based on information available to Capricor as of the date hereof, and Capricor assumes no obligation to update these forward-looking statements.

Capricor has entered into an agreement for the exclusive commercialization and distribution of Deramiocel for DMD in the United States and Japan with Nippon Shinyaku Co., Ltd. (U.S. subsidiary: NS Pharma, Inc.), subject to regulatory approval. Deramiocel and the StealthX™ vaccine are investigational candidates and have not been approved for commercial use in any indication.

Disclosures

- **Dr. McDonald has received consulting income and clinical trial research grants from Capricor Therapeutics**

Regulatory Update

Target PDUFA Date

22 August 2026

Introduction to Deramiocel

- **Cardiosphere derived cells (CDCs) are cardiac tissue derived cells which:**
  - Have anti-fibrotic, immunomodulatory and anti-inflammatory activities in human in vitro assays[1]
  - In murine *mdx* models improve both skeletal and cardiac muscle function and reduce cardiac fibrosis[2]
  - Have activities mediated by CDC derived extracellular vesicles and soluble factors[3]

- **Deramiocel is a human GMP manufactured product composed of a CDC cell suspension and intended for allogeneic use by intermittent IV infusion (Quarterly)**

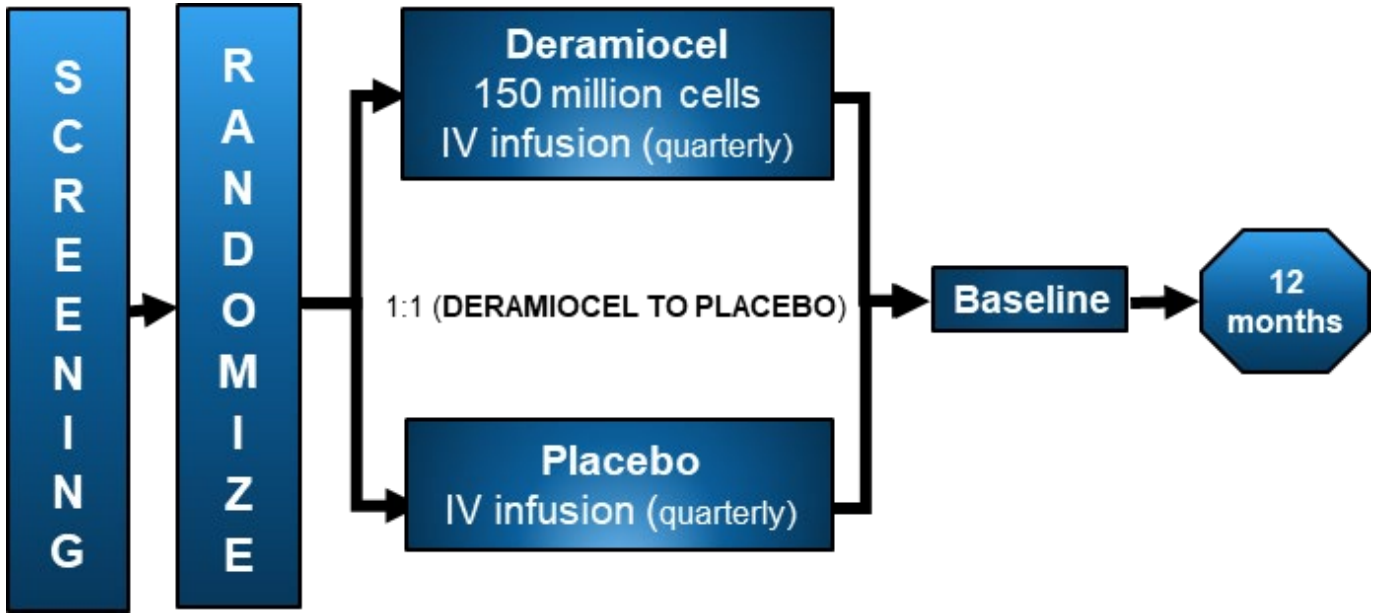
- **Phase 2 studies of deramiocel in DMD suggest a durable clinically and statistically significant benefit in both musculoskeletal and cardiac domains**
  - HOPE-2- randomized, double blind, PBO controlled (n=20)[4]
  - HOPE-2 open label extension (n=16)[5]

- **A Phase 3 study was initiated in 2022 and the primary 12month analysis will be presented today**
  - HOPE-3– randomized, double blind, PBO controlled (n=106)

References: 1. Marbán, E. *Circ. Res.* **135**, 877–885 (2024).
2. Rogers, R. G. *et al. JCI Insight* **4**, e125754 (2019), Rogers, R. G. *et al. Stem Cell Rep.* **20**, 102468 (2025).
3,Chimenti, I. *et al. Circ. Res.* **106**, 971–980 (2010), Walravens, A.-S. *et al. Sci. Rep.* **11**, 8666 (2021).
4.McDonald, C. M. *et al. Lancet* **399**, 1049–1058 (2022).
5. McDonald, C. *et al. Neuromuscular Disorders* **43**, Supplement 1 (2024).

HOPE-3 Pivotal Phase 3 Trial

Study Design



Key Entry Criteria

- Ages ≥ 10 who are late or non-ambulatory
- PUL 2.0 Entry Item Score ≥ 2
- LVEF ≥ 35%
- All patients on background stable corticosteroid therapy

HOPE-3
DUCHENNE CLINICAL TRIAL

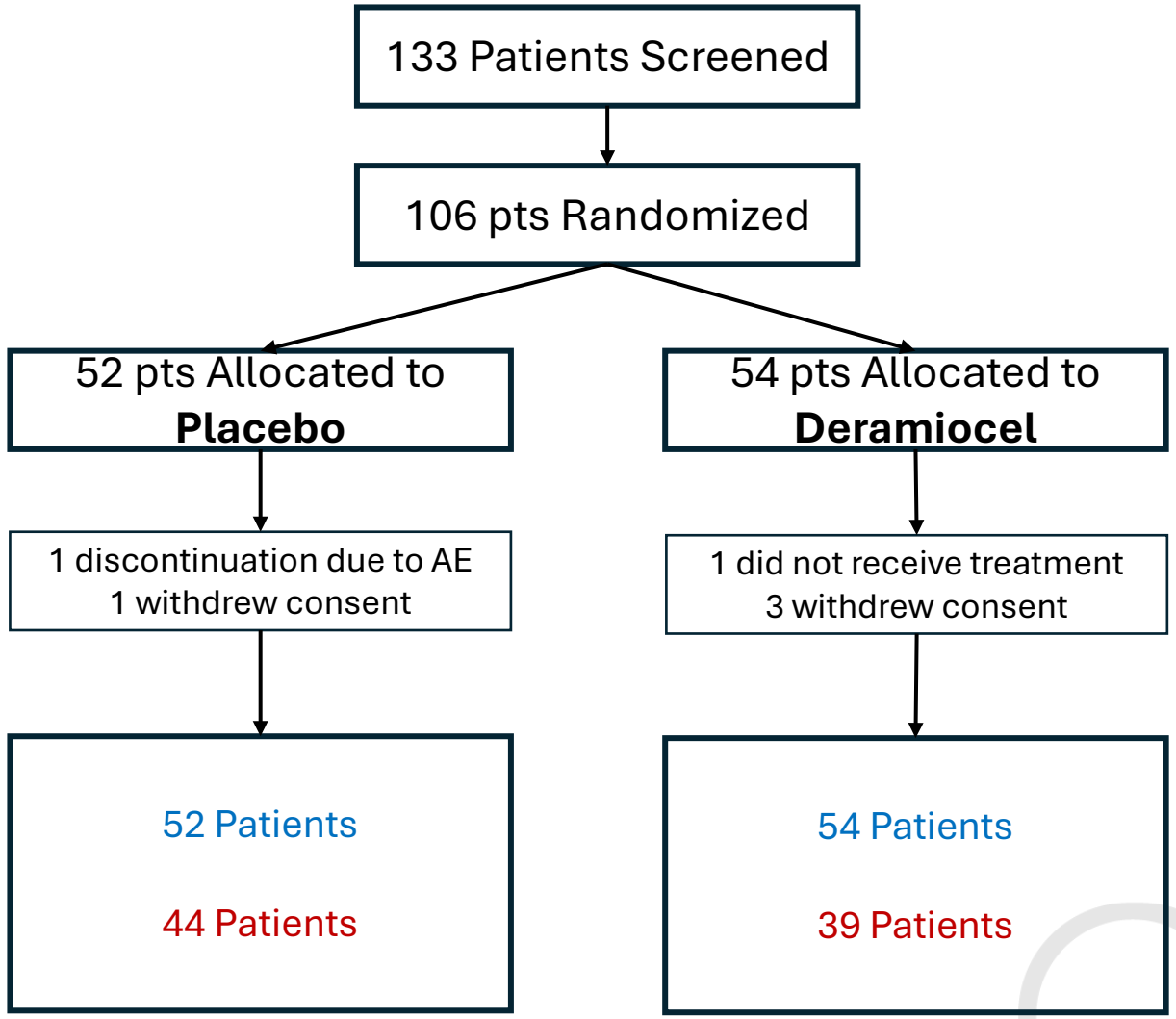
Design & Endpoints

- Phase 3: randomized (1:1), double-blind, placebo-controlled study
- **N = 106 subjects randomized**
- Conducted in the United States at 20 clinical sites
- **Primary efficacy endpoint[1]**: PUL v2.0
  skeletal muscle assessment
- **Key secondary endpoint[1]**: left ventricular fraction (LVEF)
  cardiac assessment
- **Other secondary endpoints[1]**: mid-level PUL v.2.0, GST and LGE

ClinicalTrials.gov ID: NCT05126758
[1]Type-I error controlled

HOPE-3 Pivotal Phase 3 Trial

Patient disposition



OUTCOME MEASURES

Primary PUL 2.0 (ITT=106)

Key Secondary LVEF* (ITT=83)

*Population for LVEF required centrally analyzable baseline and 12-month images

Population Demographics

Well balanced treatment groups

Baseline Demographics	Placebo (n=52)	Deramiocel (n=54)	Overall (n=106)[1]
Age (years)			
N	52	54	106
Mean (SD)	14.6 (2.95)	15.4 (3.10)	15.0 (3.04)
Median	14	15	15
Min, Max	10, 22	10, 22	10, 22
PUL v2.0 entry item score			
2,3	23 (44.2)	25 (46.3)	48 (45.3)
4,5,6	29 (55.8)	29 (53.7)	58 (54.7)
Diagnosed cardiomyopathy[2]			
No	14 (26.9)	13 (24.1)	27 (25.5)
Yes	38 (73.1)	41 (75.9)	79 (74.5)
Baseline LVEF%			
n	46	45	91
Mean (SD)	59.303 (6.108)	55.345 (7.743)	57.346 (7.206)
Median	59.309	55.892	57.532
Min, Max	47.395, 73.981	36.537, 71.112	36.537, 73.981
Ambulatory status			
Non-ambulatory	44 (84.6)	46 (85.2)	90 (84.9)
Ambulatory	8 (15.4)	8 (14.8)	16 (15.1)

[1]One subject enrolled but dropped out prior to baseline assessment (n=105)
[2]Updated as of Feb. 2026; subgroup: 64 of 79 patients with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months

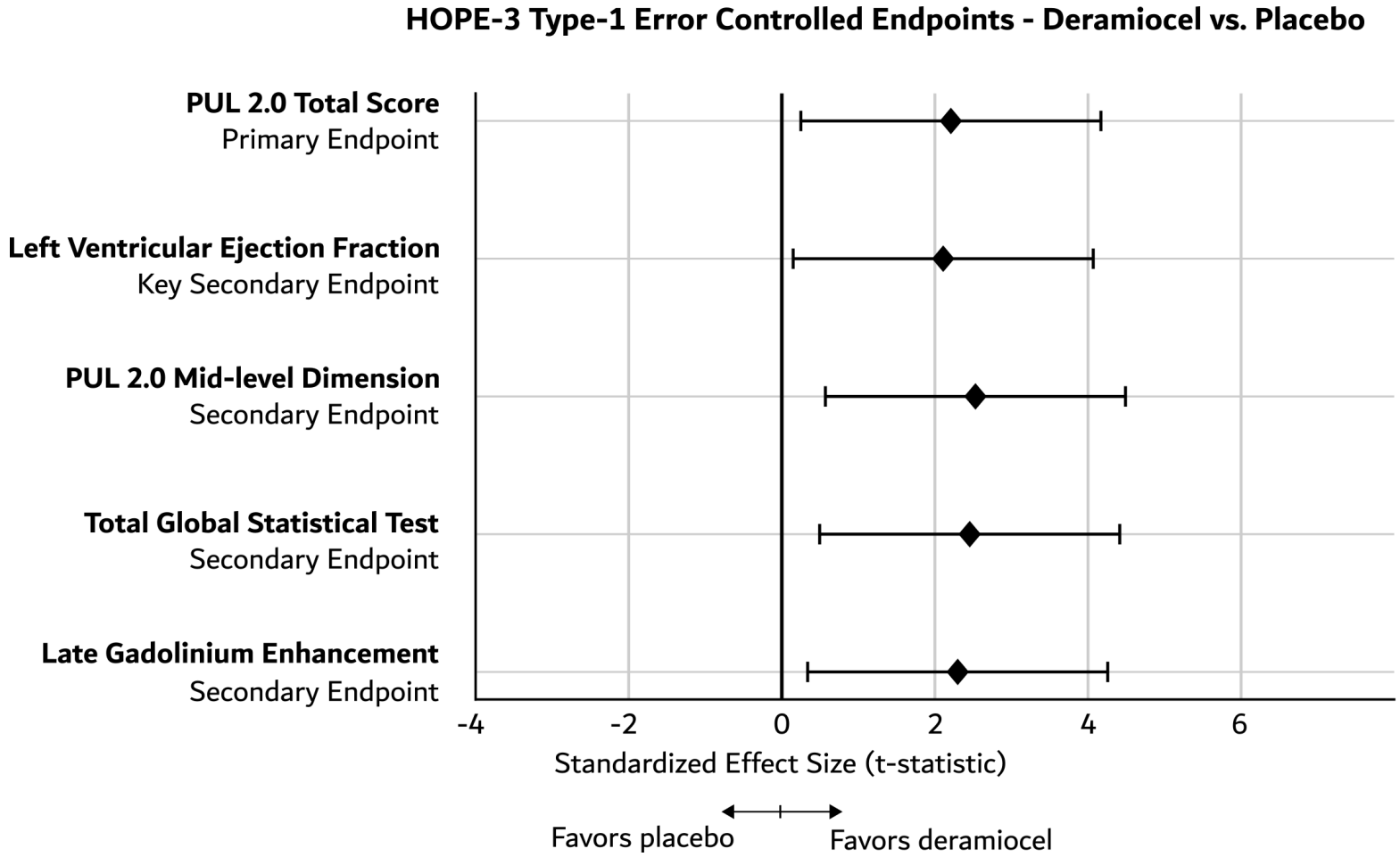
Safety Results

Deramiocel profile is comparable to placebo (which includes DMSO)

Overview	Placebo (n=52), n (%)	Deramiocel (n=53), n (%)	Overall (n=105[1]), n (%)
Any TEAEs	43 (82.7)	50 (94.3)	93 (88.6)
TEAEs related to IP or administration procedure	19 (36.5)	44 (83.0)	63 (60.0)
TEAEs related to IP	16 (30.8)	44 (83.0)	60 (57.1)
TEAEs related to administration procedure	9 (17.3)	23 (43.4)	32 (30.5)
TEAEs related to IP or administration procedure by maximum severity			
Mild (grade 1)	15 (28.8)	19 (35.8)	34 (32.4)
Moderate (grade 2)	3 (5.8)	25 (47.2)	28 (26.7)
Severe (grade 3)	0	0	0
Life-threatening (grade 4)	1 (1.9)	0	1 (1.0)
Fatal (grade 5)	0	0	0
TEAEs leading to death	0	0	0
Any serious TEAEs	5 (9.6)	1 (1.9)	6 (5.7)
Serious TEAEs related to IP or administration procedure	1 (1.9)	1 (1.9)	2 (1.9)

[1] Safety population (n=105)

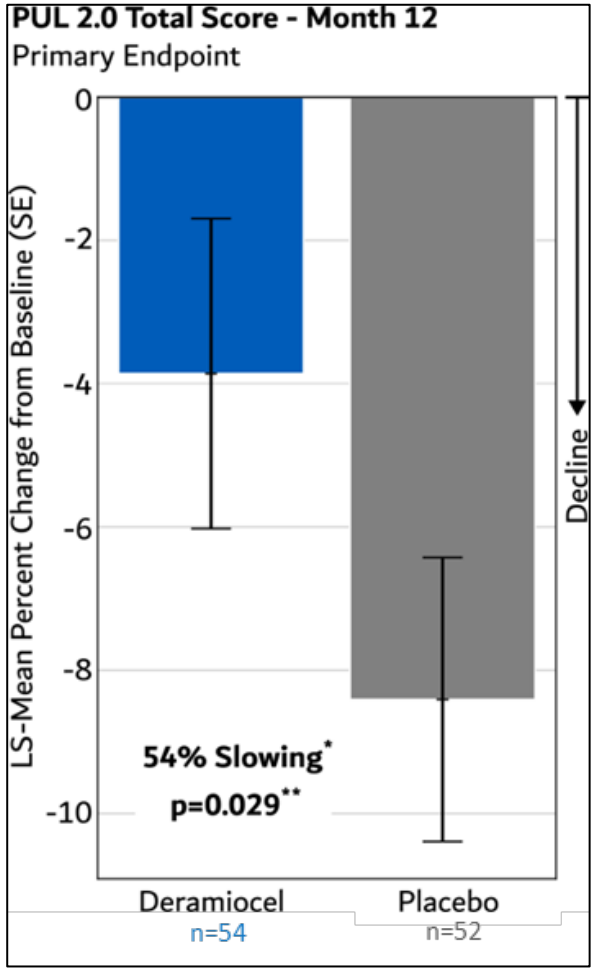
Overview of All Type-I Error-Controlled Endpoints

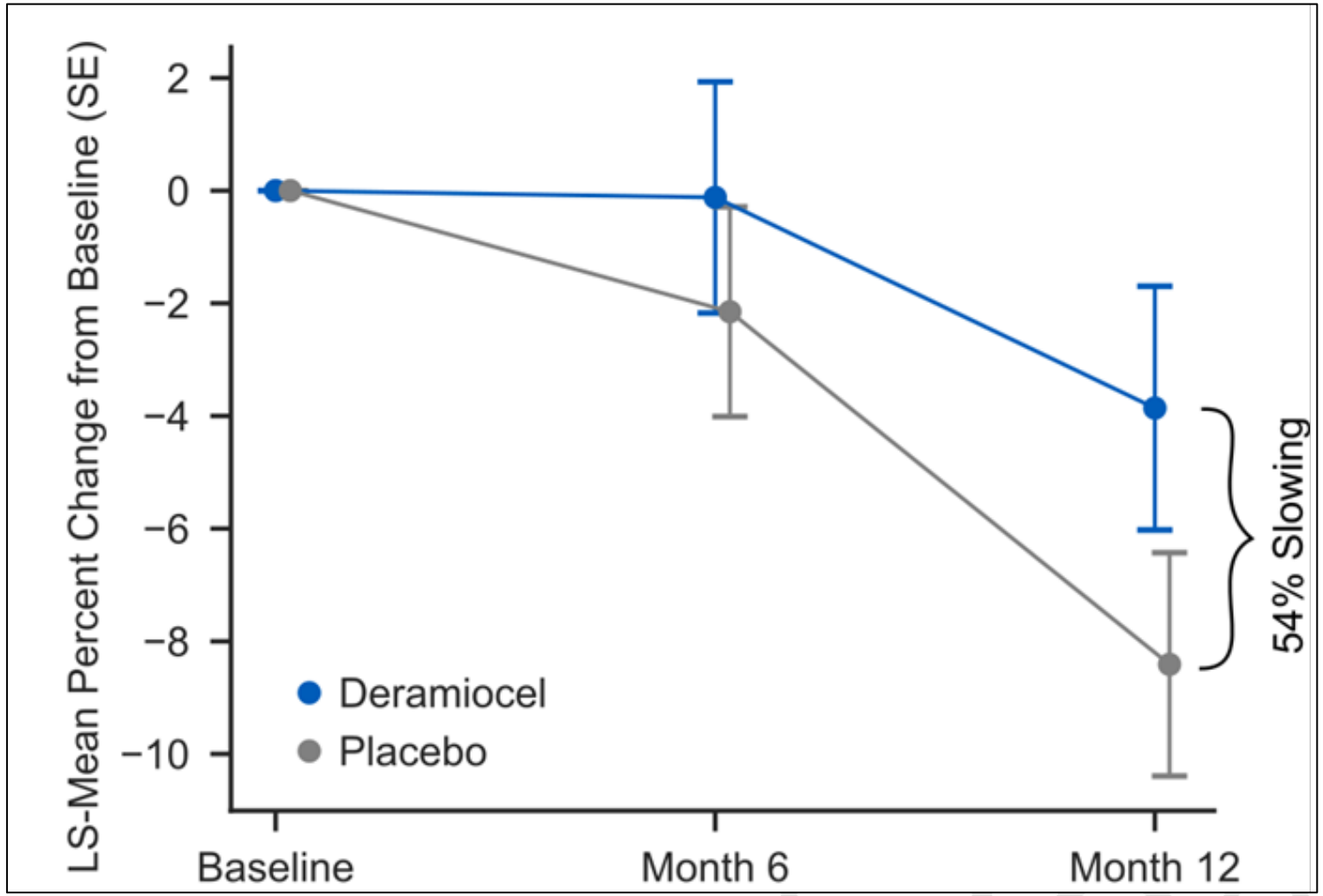


	n	Treatment Difference	p-value
PUL 2.0 Total Score Primary Endpoint	106	1.2 pts	0.029
Left Ventricular Ejection Fraction Key Secondary Endpoint	83	2.4 % pts	0.041
PUL 2.0 Mid-level Dimension Secondary Endpoint	106	1.0 pts	0.008
Total Global Statistical Test Secondary Endpoint	103	2.5 (t-statistic)	0.017
Late Gadolinium Enhancement Secondary Endpoint	22	-3.0 segments	0.022

Primary Endpoint: PUL 2.0 at 12 Months

Deramiocel treatment difference = 1.2 points (54% slowing); p=0.029

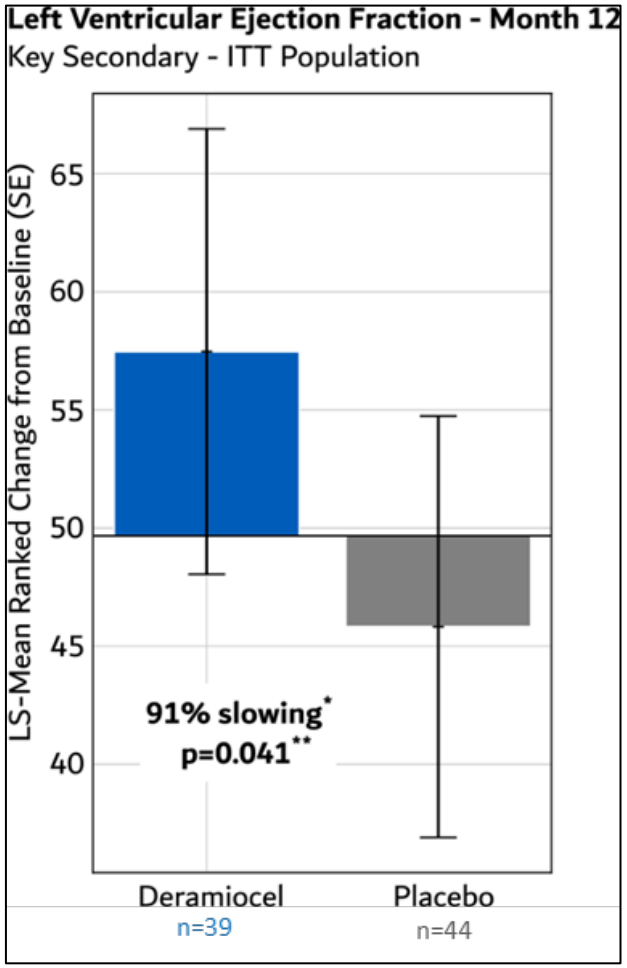


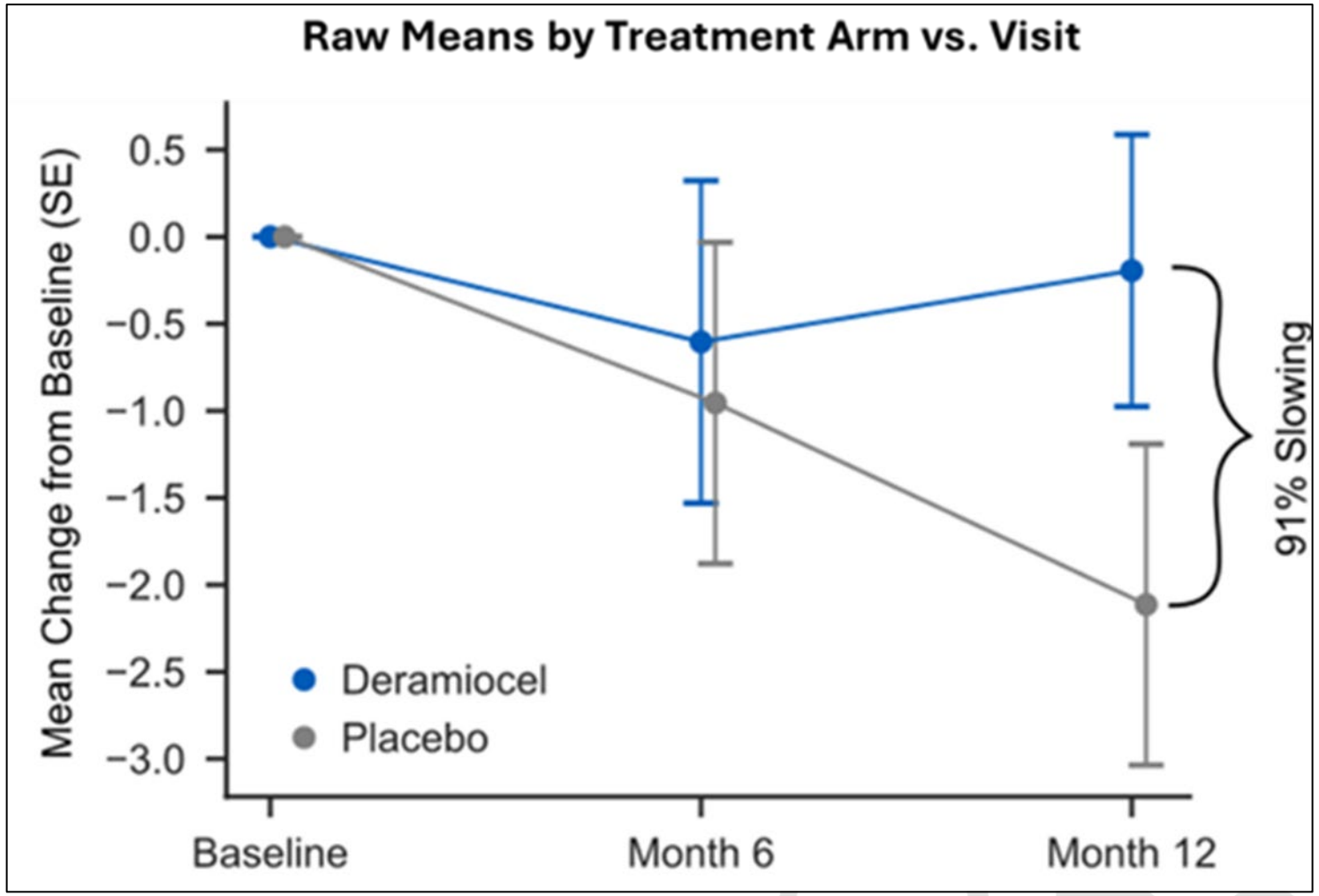


*LS-Mean difference = 4.55 percentage point (1.2 -point difference on the PUL scale)
** Based on prespecified repeated measures model using percent change from BL

Key Secondary Endpoint: LVEF at 12 Months

Deramiocel treatment difference = 2.4% pts on LVEF (91% slowing); p=0.041 for ranked comparison



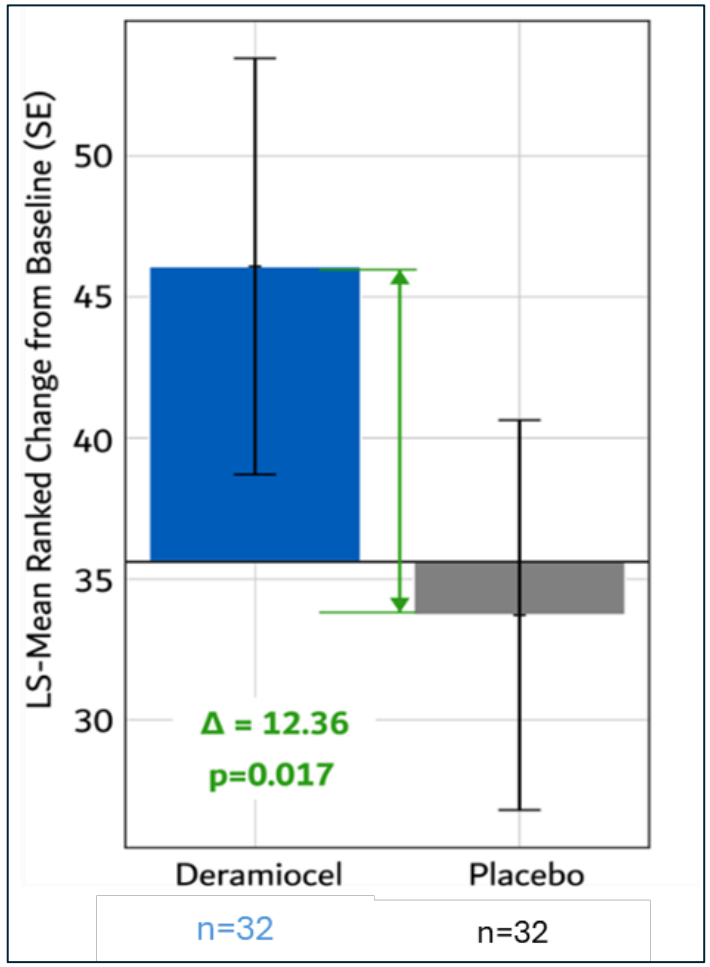


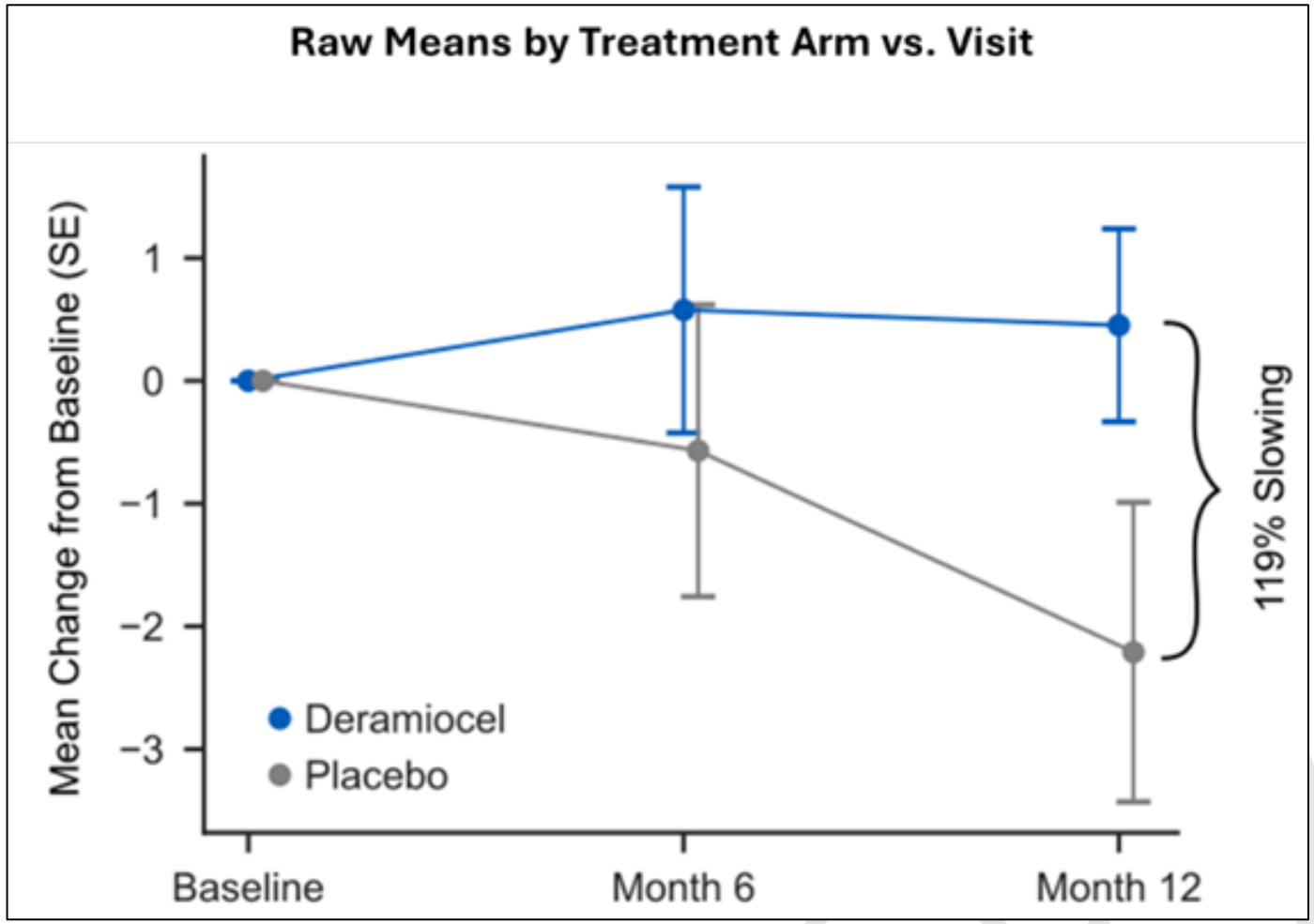
* LS-mean difference = 11.65 ranks (2.4 percentage point difference in LVEF)
** Based on prespecified rank ANCOVA model
LVEF: n reflects the number of patients in the ITT population with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months (n=83)

Secondary Endpoint[1]: LVEF at 12 Months

Patients with Cardiomyopathy[2] at Baseline

Deramiocel treatment difference = 3.3% pts on LVEF (>100% slowing); p=0.017 for ranked comparison



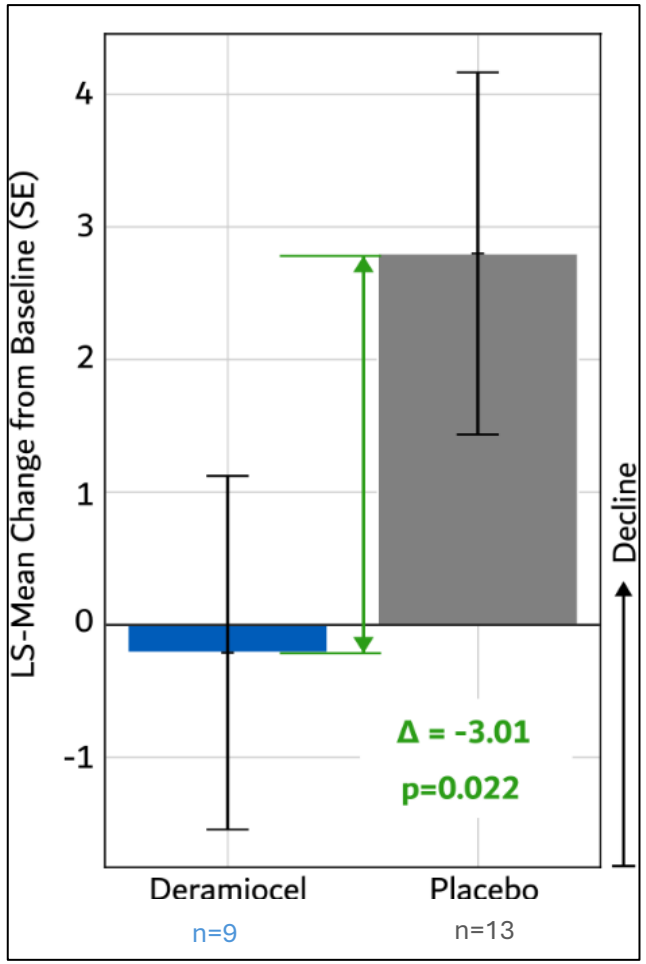


* LS-mean difference = 12.36 ranks (3.3 percentage point difference in LVEF)
** Based on prespecified rank ANCOVA model
LVEF: n reflects the number of patients in the ITT population with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months (n=64)

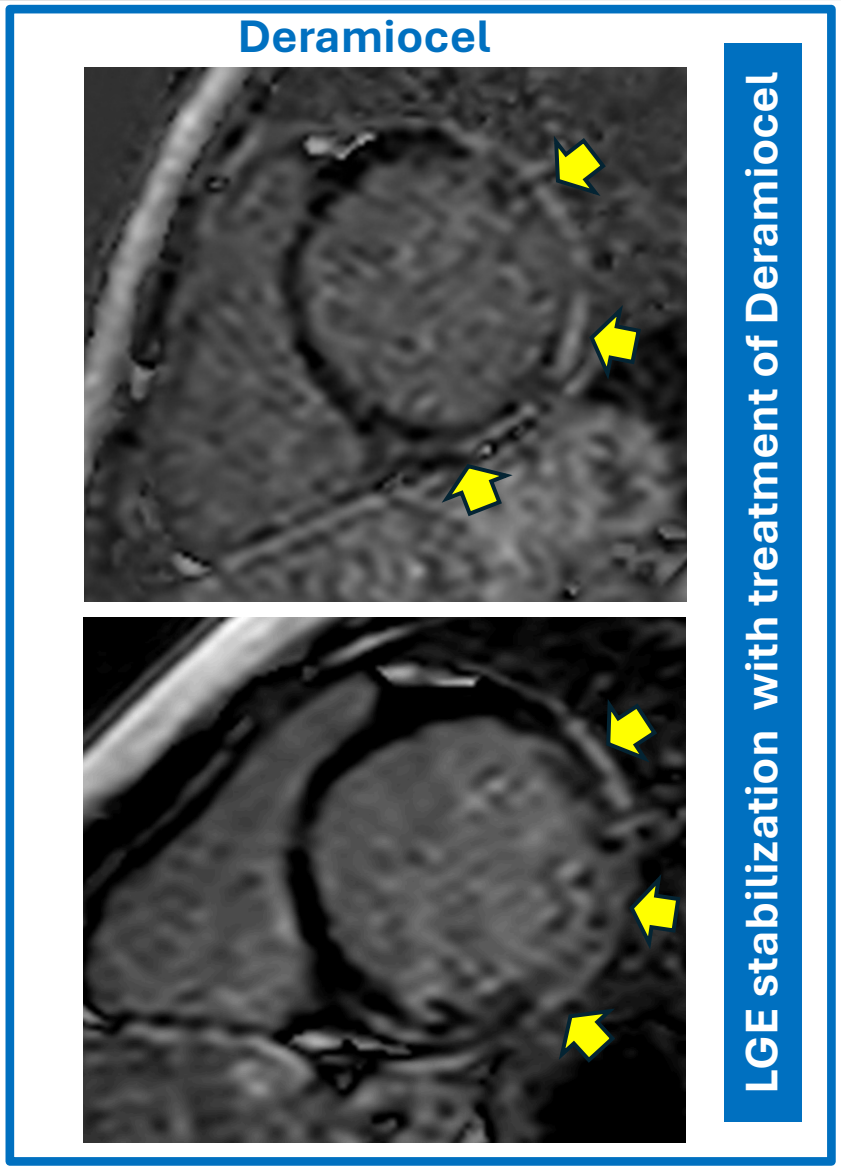
1 – Prespecified endpoint not TYPE-I error controlled
2 – Cardiomyopathy is defined as having clinical diagnosis, LVEF<55%, or LGE >0 myocardial segments

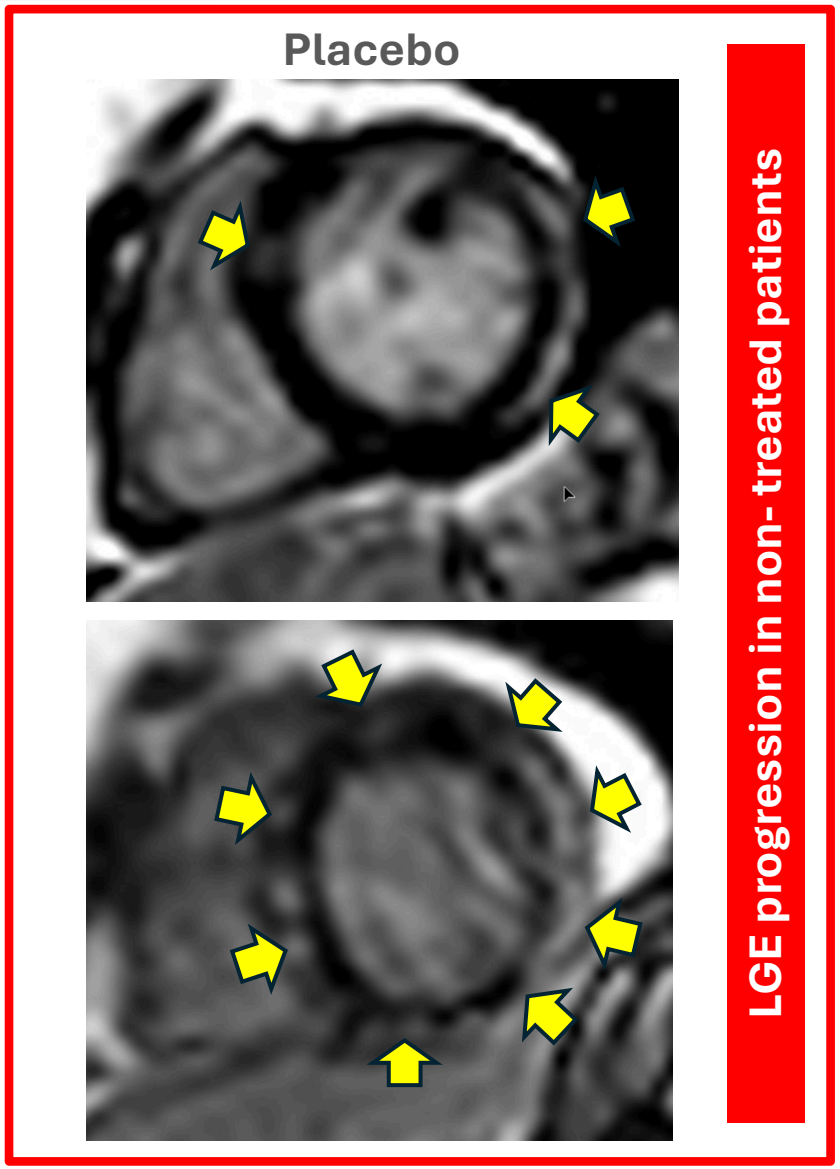
Secondary Endpoint: Late Gadolinium Enhancement

Deramiocel treatment difference = Δ 3 Segments; p=0.022; LS Mean Change at 12 Months



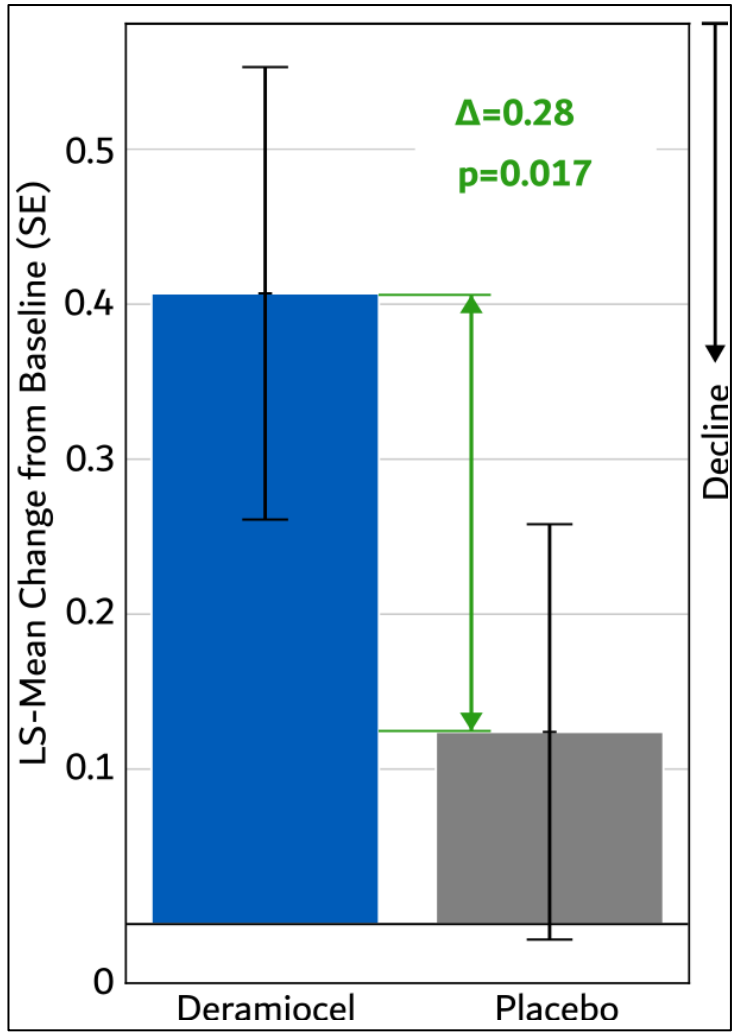
n reflects the number of patients in the ITT population with centrally reviewed and evaluable cardiac MRI LGE images at baseline and 12 months (n=22)





Secondary Endpoint: Global Statistical Test

Deramiocel treatment difference = Δ 0.28; p=0.017; LS Mean Change at 12 Months



GST is a patient-Level composite outcome to incorporate clinically relevant components of:

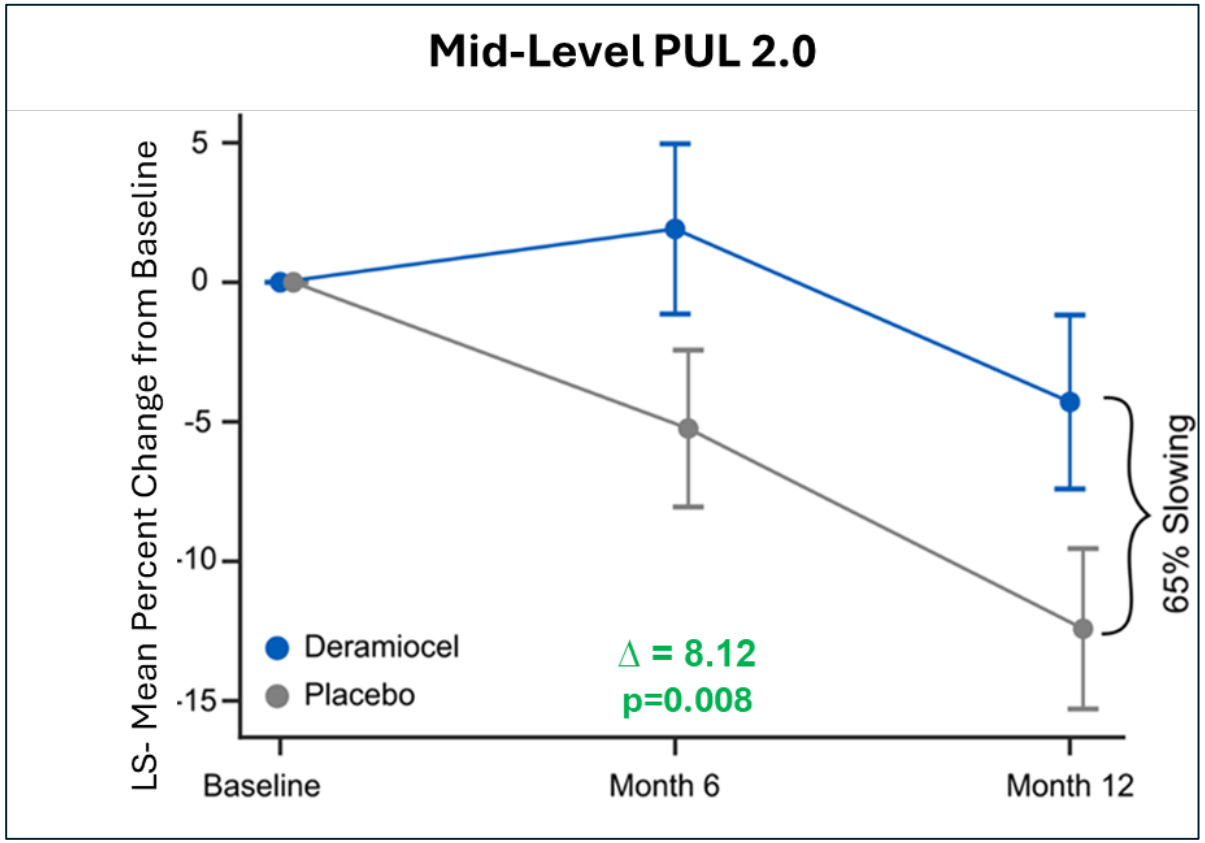
- **Performance of Upper Limb 2.0 Total Score (PUL 2.0)**
- **Left Ventricular Ejection Fraction (LVEF)**
- **Patient Global Impression – Severity (PGI-S)**

Represents how a patient *Feels* (PGI-S), *Functions* (PUL 2.0), and *Survives* (LVEF).

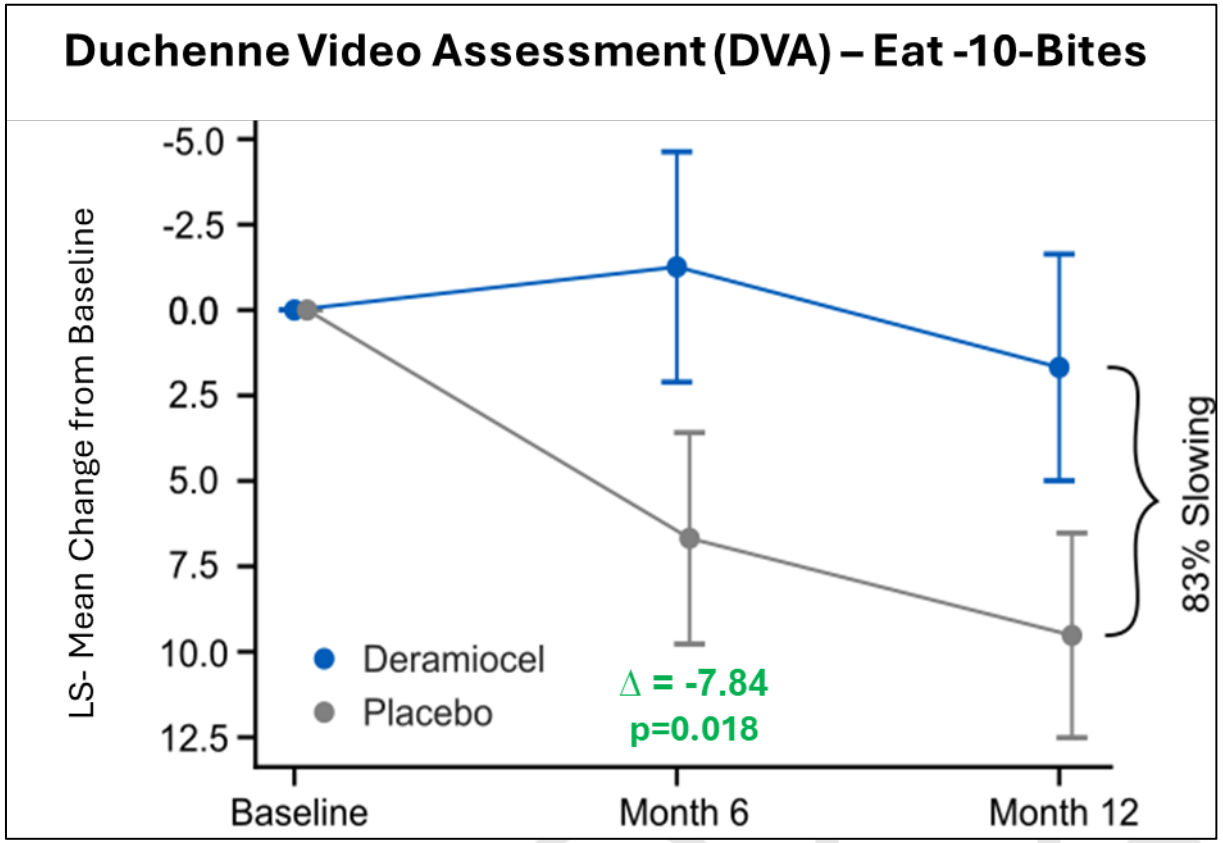
*using and ANCOVA model. Treatment difference is a Z-score.

Mid-Level PUL 2.0 vs. DVA Eat-10 Bites

Similar Statistically Significant Benefits in Hand-to-Mouth Function across multiple analyses



Disease Progression



Conclusions - 1

- **Deramiocel is an allogeneic cell therapy delivered intravenously every 3 months and has an acceptable tolerability and safety profile**
- **The HOPE-3 Phase 3 study met <u>all</u> its primary and secondary (type-1 error controlled) endpoints**

<u>Upper Limb Function</u> – deramiocel slowed disease progression by 50-80%

1) Performance of Upper Limb (PUL2.0) Total Score
2) Performance of Upper Limb (PUL2.0) Mid level score
3) Eat 10 Bites – Duchenne Video Assessment

➢ **Likely to protect from the loss of performance of activities of daily living (ADL) – transferring, turning, eating independently**

1. Soslow, J. H. *et al. Circ.: Hear. Fail.* **16**, e010040 (2023).

Conclusions - 2

<u>Cardiac Function</u> – deramiocel preserved LVEF and slowed LGE (cardiac fibrosis)

1) LVEF slowing of 91% in all evaluable patients
2) LVEF slowing of >100% in known cardiomyopathy patients
3) Reduced rate of LGE segment progression

➢ **Preservation of cardiac function is likely to translate into survival benefit[1]**

HOPE-3 confirms musculoskeletal and cardiac functional benefit in DMD and demonstrates anti-fibrotic activity

1. Soslow, J. H. *et al. Circ.: Hear. Fail.* **16**, e010040 (2023).

Acknowledgements

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- Michael Taylor, M.D., Ph.D. (Austin Childrens)
- Kan Hor, M.D. (Nationwide Childrens)
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Questions