ASGCT Abstract #329

Phase-3 efficacy in Duchenne muscular dystrophy in HOPE-3 confirms skeletal and cardiac muscle functional benefit and supports the clinical translation of anti-fibrotic activity for deramiocel, an allogeneic cardiac derived cell therapy

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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, revenue and reimbursement estimates, projected terms of definitive agreements, our financial position, our possible uses of existing cash and investment resources, and statements regarding our litigation with Nippon Shinyaku Co., Ltd. and NS Pharma, Inc., including the nature of the dispute, our expectations regarding any legal proceedings, and our ability to commercialize Deramiocel independent of our existing distribution agreement 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, 2025, as filed with the Securities and Exchange Commission on March 17, 2026 and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2026, as filed with the Securities and Exchange Commission on May 13, 2026. 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.

Deramiocel and the StealthX™ vaccine are investigational candidates and have not been approved for commercial use in any indication.

Disclosures

- **Dr. Soslow has received consulting income from Capricor Therapeutics**

Duchenne Cardiomyopathy (DMD-CM)

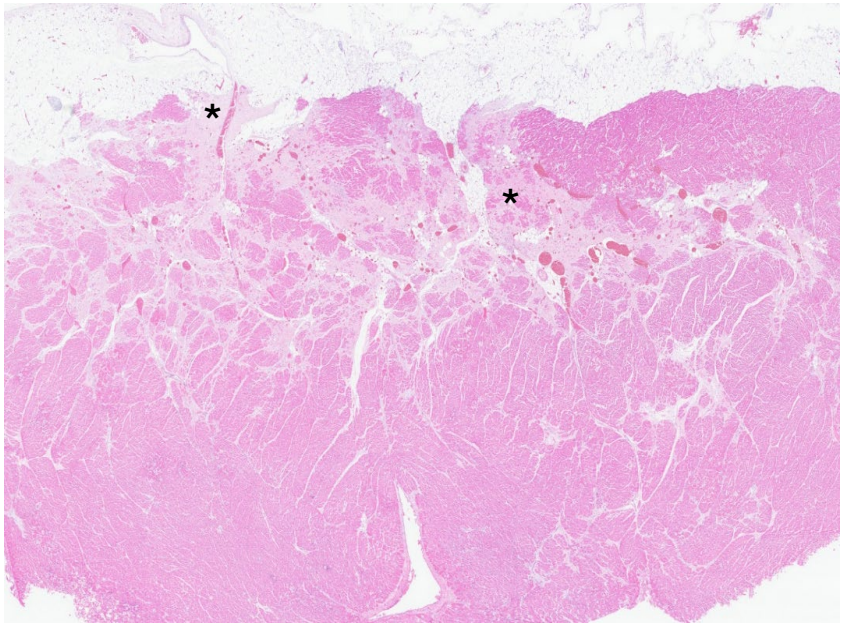
- **DMD is a devastating X-linked disease that manifests in progressive skeletal muscle weakness and cardiomyopathy**
- **The DMD gene encodes for dystrophin protein, which stabilizes the sarcolemma during muscle contraction**
  - **Absence of dystrophin results in inflammation, fibrosis, and eventual cardiomyocyte death with fibrofatty replacement**
- **Untreated, patients lose ambulation by 10-12 years of age and die from respiratory failure in the 2nd-3rd decade**
- **Supportive care measures have extended life expectancy to a median of 28 y/o and unmasked the cardiovascular phenotype**
- ***Cardiomyopathy is now the leading cause of death in DMD***

DMD-CM: Cardiac Histology

Progressive Fibrofatty Replacement of Left Ventricular Myocardium

LV myocardium samples — muscle (pink) progressively replaced by fibrofatty tissue with age.

PRE-TEEN



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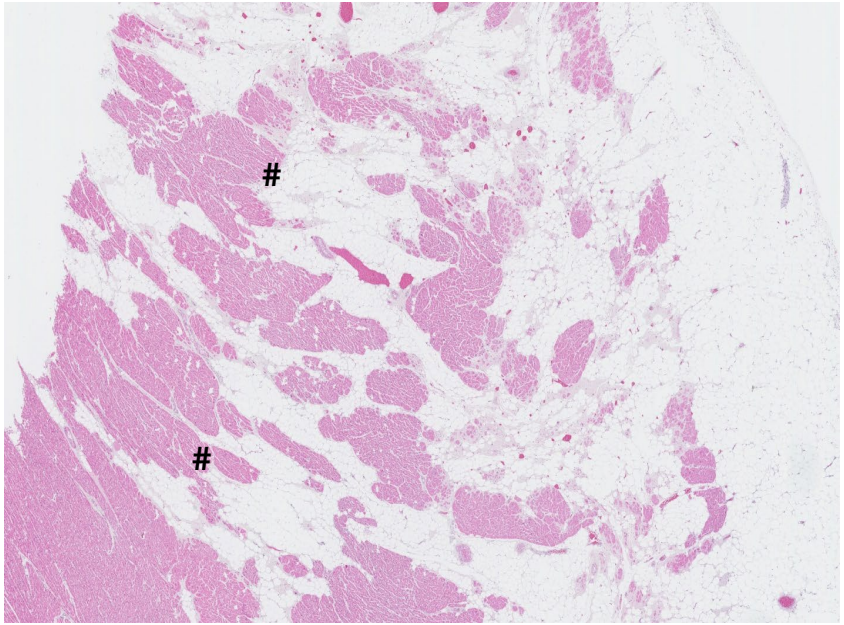


* ***Focal fibrofatty infiltration***

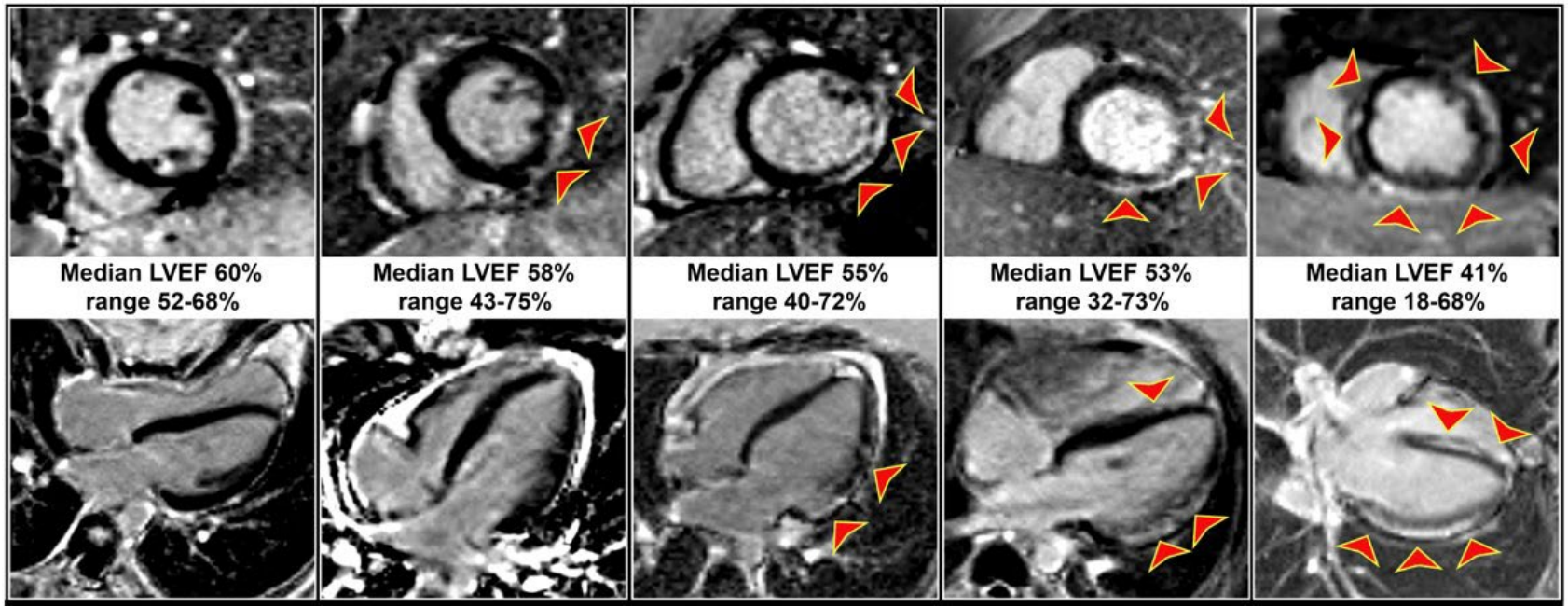
\# ***Extensive fibrofatty replacement***

Breaux et al., 2024

DMD-CM: CMR

Fibrosis is a Clear Pathogenic Feature of DMD

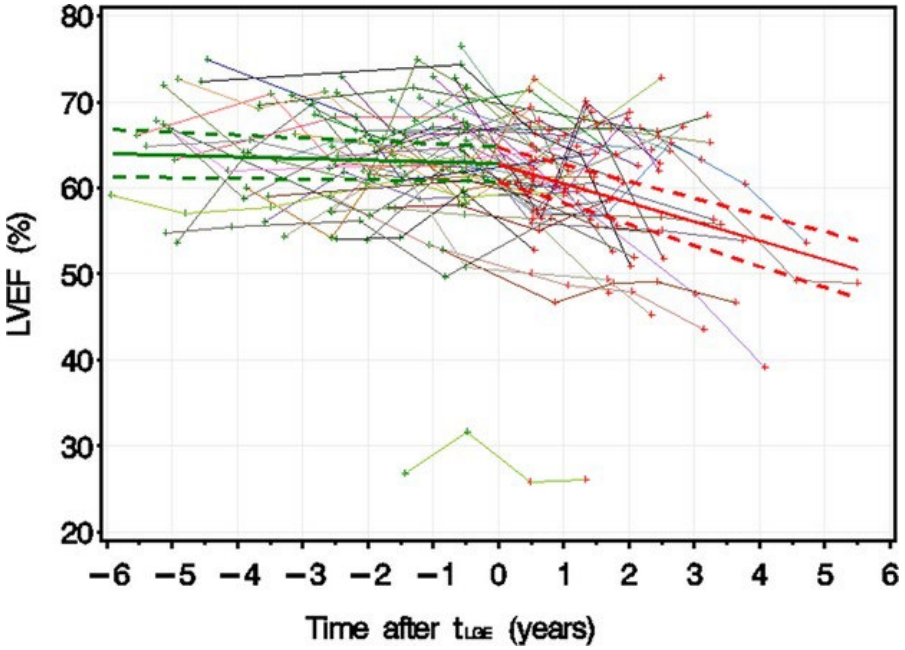
LGE burden increases as LVEF declines — visible on cardiac MRI across the full range of clinical severity.



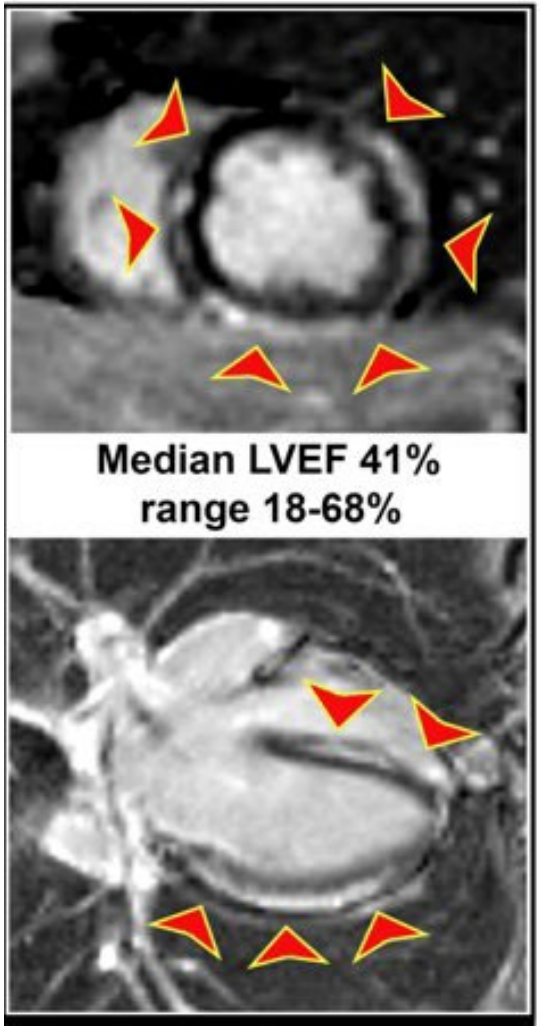
Top row: short-axis views · Bottom row: long-axis views · Red arrowheads mark LGE (scar). Groups span median LVEF 60% → 41%.

DMD-CM: Longitudinal CMR

Systolic Function (LVEF) Declines After Onset of Scar



Tandon et al., 2015 · LVEF as measured by LGE

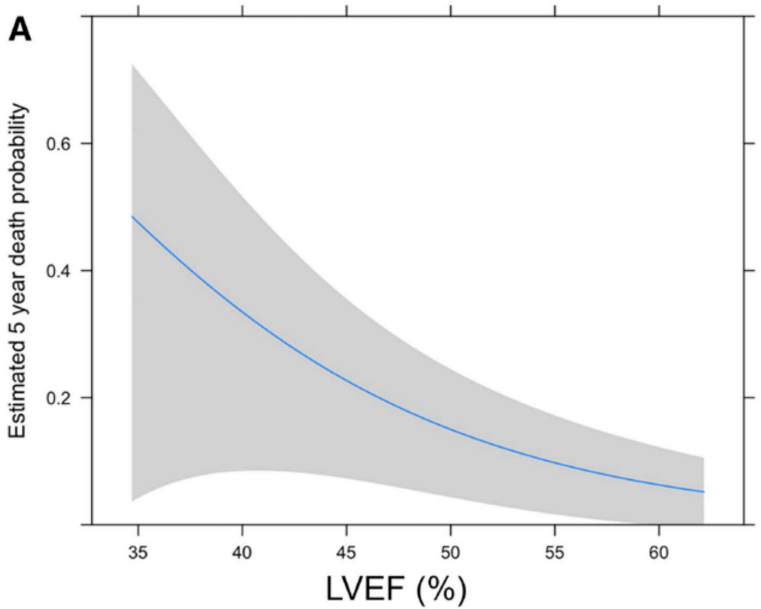


DMD-CM: CMR Natural History Model

3% reduction in LVEF carries 1.32 HR for mortality

Table 4. Model for All-Cause Mortality Based on First Available Visit That Included a Cardiac MRI

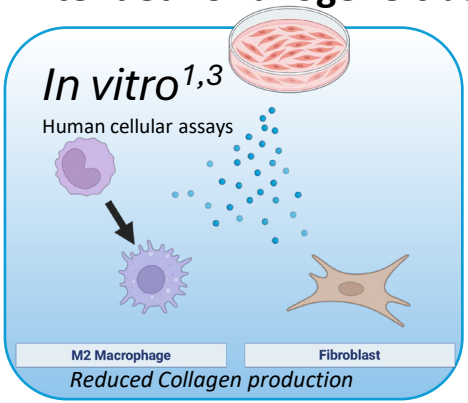
Measure	N	Number of events	Hazard ratio, confidence interval	*P* value
LV ejection fraction per 3% decrease	78	15	1.32 (1.12–1.55)	<0.001
LV end diastolic volume indexed per 4 mL/m^2 increase	77	14	1.20 (1.09–1.32)	<0.001
LV end systolic volume indexed per 2 mL/m^2 increase	77	14	1.12 (1.06–1.19)	<0.001

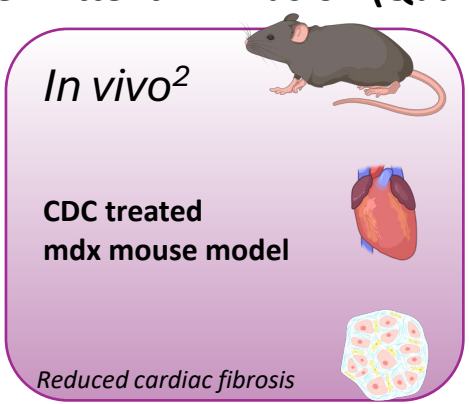


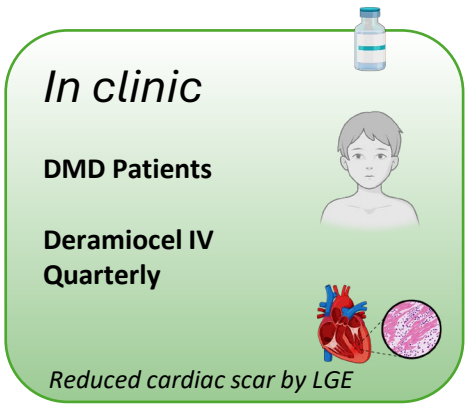
Soslow, J.H. *et al.* Cardiovascular Measures of All-Cause Mortality in Duchenne Muscular Dystrophy. *Circ.: Hear. Fail.* **16,** e010040 (2023).

CDCs and Deramiocel

- **Cardiosphere derived cells (CDCs) are stromal cells selectively cultured out of cardiac tissue explants with regenerative properties[1].**
- **CDCs are not stem cells and do not engraft. They exert their anti-fibrotic and anti-inflammatory effects through the release of exosomes.**
- **Deramiocel is a GMP manufactured product composed of a human CDC cell suspension and intended for allogeneic use by intermittent IV infusion (Quarterly)**







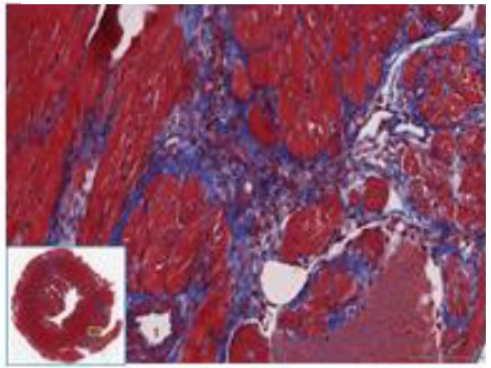
References:
1. Marbán, E. *Circ. Res.* **135**, 877–885 (2024).
2. Rogers, R. G. *et al. JCI Insight* **4**, e125754 (2019), Rogers, R. G. *et al. Stem Cell Rep.* **20**, 102468 (2025).
3. Chimenti, I. *et al. Circ. Res.* **106**, 971–980 (2010), Walravens, A.-S. *et al. Sci. Rep.* **11**, 8666 (2021).

Cardiosphere Derived Cells (CDCs)

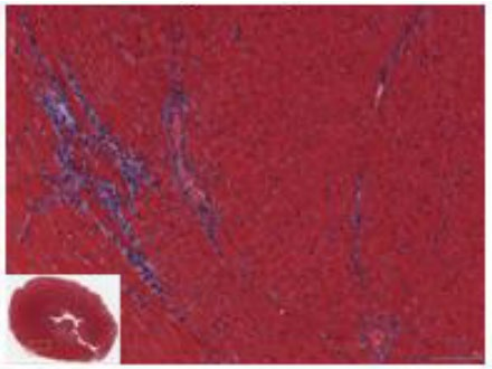
Inhibition of cardiac fibrosis in mdx mouse model of DMD

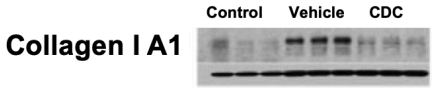
Capricor

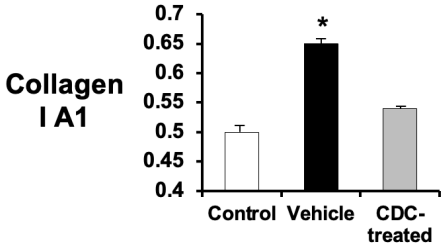
***mdx* Heart (vehicle)**

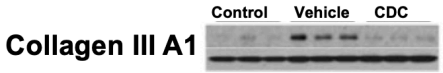


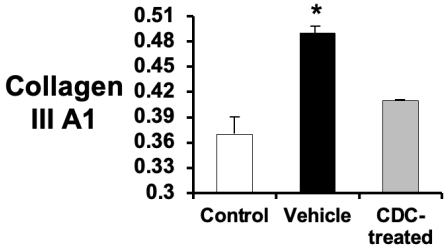
***mdx* Heart (CDC-treated)**









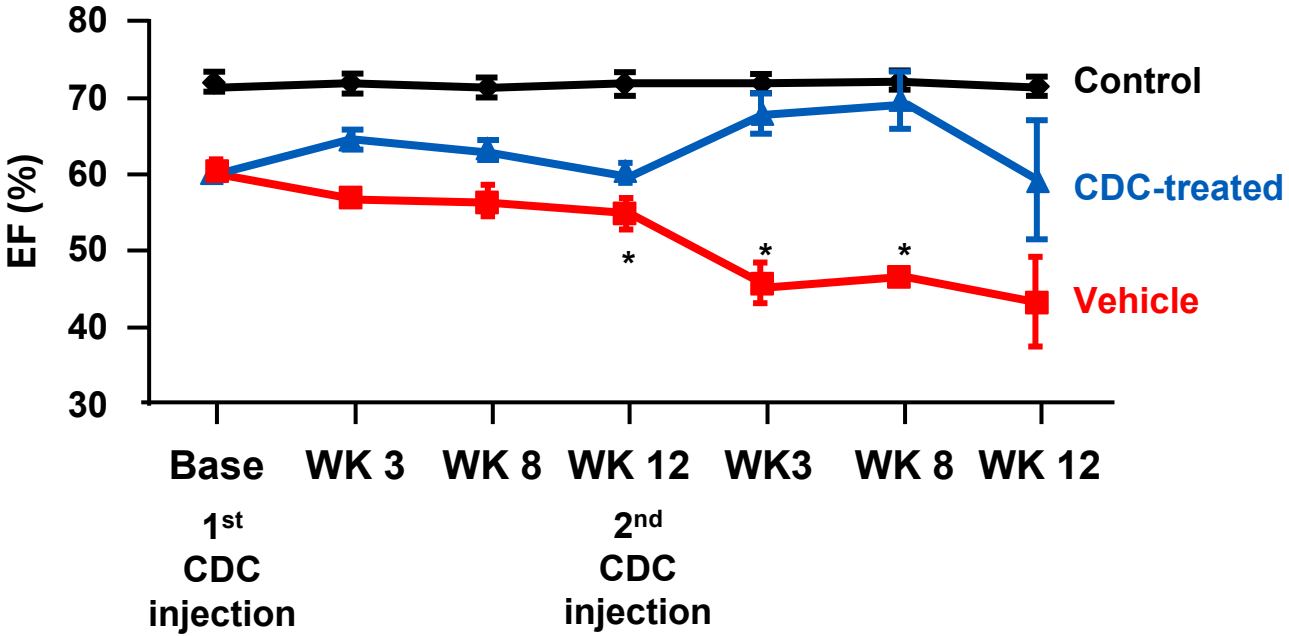


Rogers, R. G. *et al. JCI Insight* **4**, e125754 (2019).
Aminzadeh et al., *Stem Cell Rep.* **10**, 942–955 (2018)

* = p < 0.05 vs control and CDC-treated

Cardiosphere Derived Cells (CDCs)

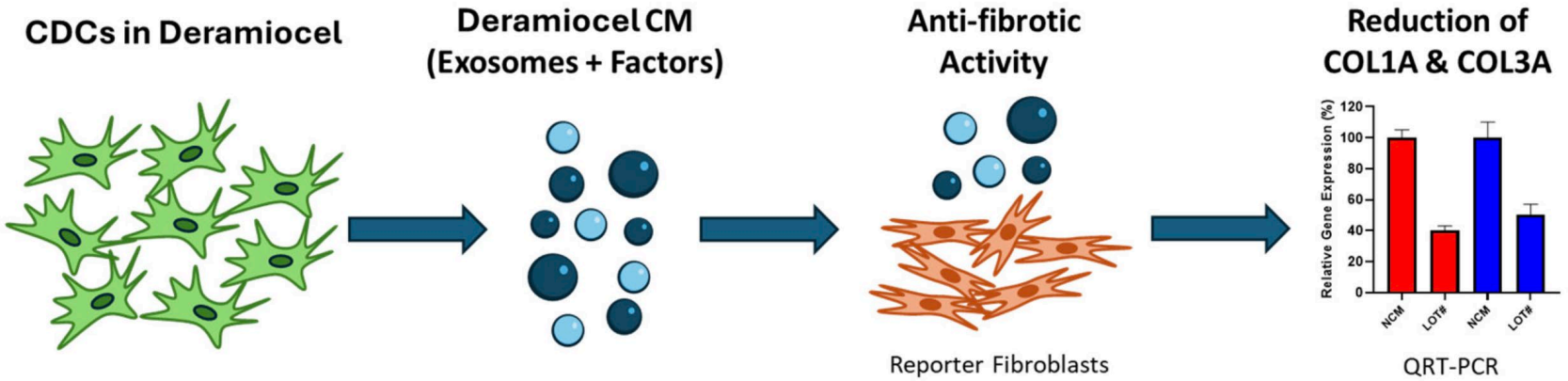
Improvement in LVEF in mdx mouse model of DMD



Aminzadeh et al., *Stem Cell Rep*. **10,** 942–955 (2018); * = **p < 0.05 vs control and CDC-treated**

Deramiocel (CDCs)

In vitro anti-fibrotic potency confirmed with each lot



Li, Y. *et al.* *Biomedicines* **13**, 2652 (2025).

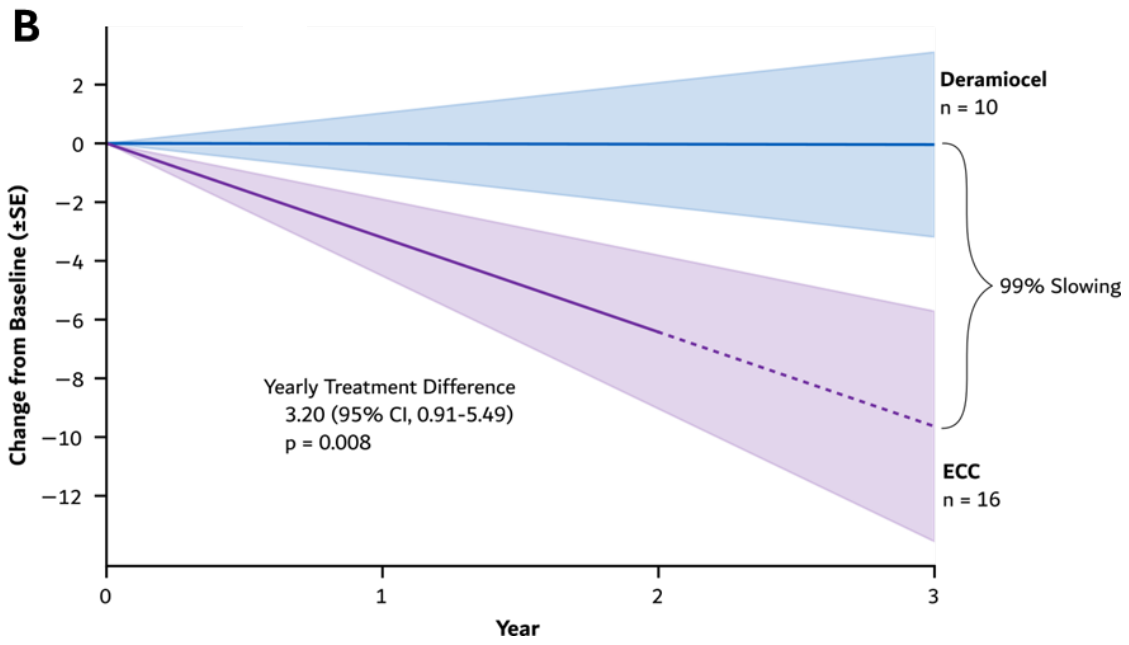
Deramiocel in DMD (HOPE Studies)

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)[1]
- HOPE-2 open label extension (n=16)[2]

HOPE-2-OLE (n=10 with CMR)

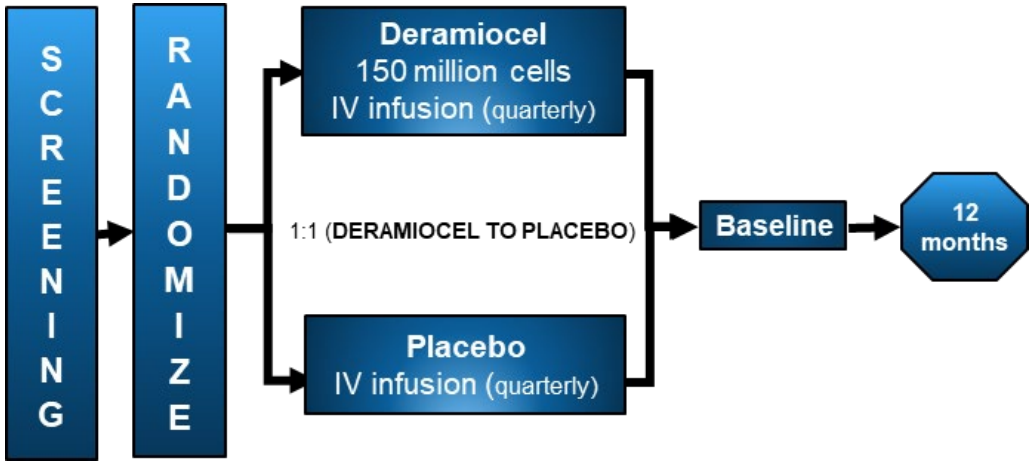
LVEF comparison with matched external control group[3]



References: .1. McDonald, C. M. *et al. Lancet* **399,** 1049–1058 (2022).
2. McDonald, C. *et al. Neuromuscular Disorders* **43,** Supplement 1 (2024)
3. McDonald, C et al, *Submitted for publication*

HOPE-3 Pivotal Phase 3 Trial

Study Design



Key Entry Criteria

- Ages ≥ 10 who are late or non-ambulatory
- LVEF ≥ 35%
- All patients on background stable corticosteroid therapy

HOPE-3
DUCHENNE CLINICAL TRIAL

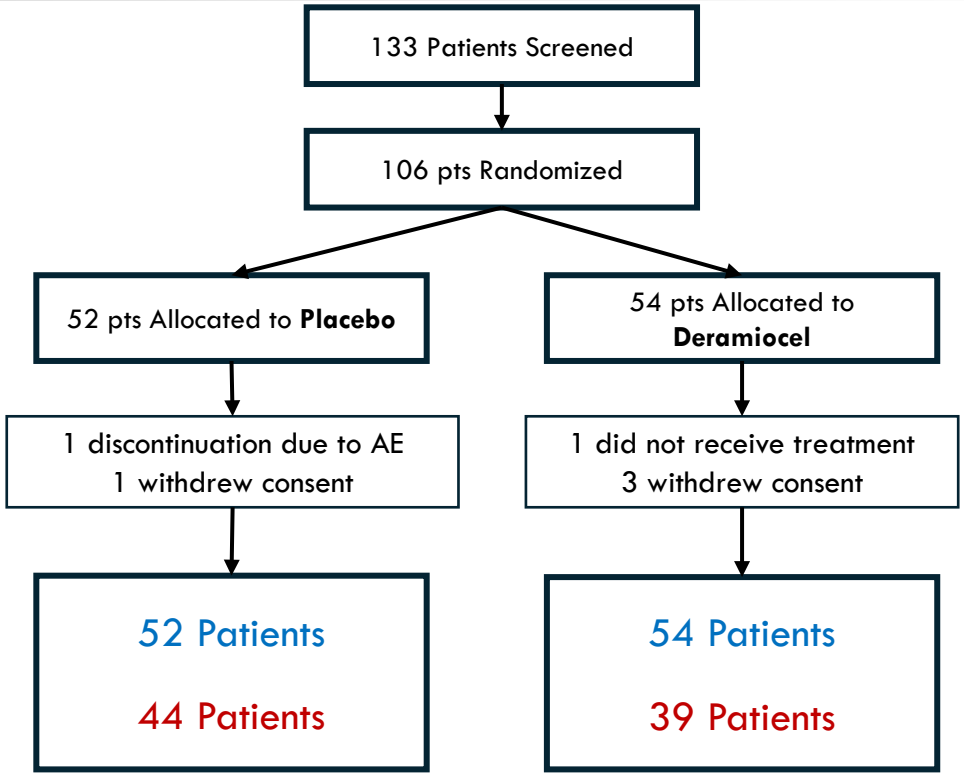
Design & Endpoints

- 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
- **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

HOPE-3 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
Ambulatory status			
Non-ambulatory	44 (84.6)	46 (85.2)	90 (84.9)
Ambulatory	8 (15.4)	8 (14.8)	16 (15.1)

Cardiac ITT Population	Placebo (N=44)	Deramiocel (N=39)	Overall N=83)
Median Age, (range) (Years)	14(10-22)	16 (10-22)	15(10-22)
LVEF (%) at Baseline (Mean (SD))	59.6 (5.96)	54.3 (7.60)	57.1 (7.24)
Median (Range)	59 (48-74)	55 (36-69)	57.5 (36.5-74)
Cardiomyopathy, n (%)	32 (72.7)	32 (82.1)	64 (77.1)
Baseline Cardiac Medication Type, n (%)			
ACE/ARB/ARNI	40 (90.9)	37 (94.9)	77 (92.8)
Beta Blockers	17 (38.6)	17 (43.6)	34 (41.0)
SGLT2i	2 (4.5)	3 (7.7)	3 (3.6)
MRA	29 (65.9)	21 (53.8)	50 (60.2)
No Cardiac Medication	3 (6.8)	2 (5.1)	5 (6.0)

[1]One subject enrolled but dropped out prior to baseline assessment (n=105)

HOPE-3 Safety Results

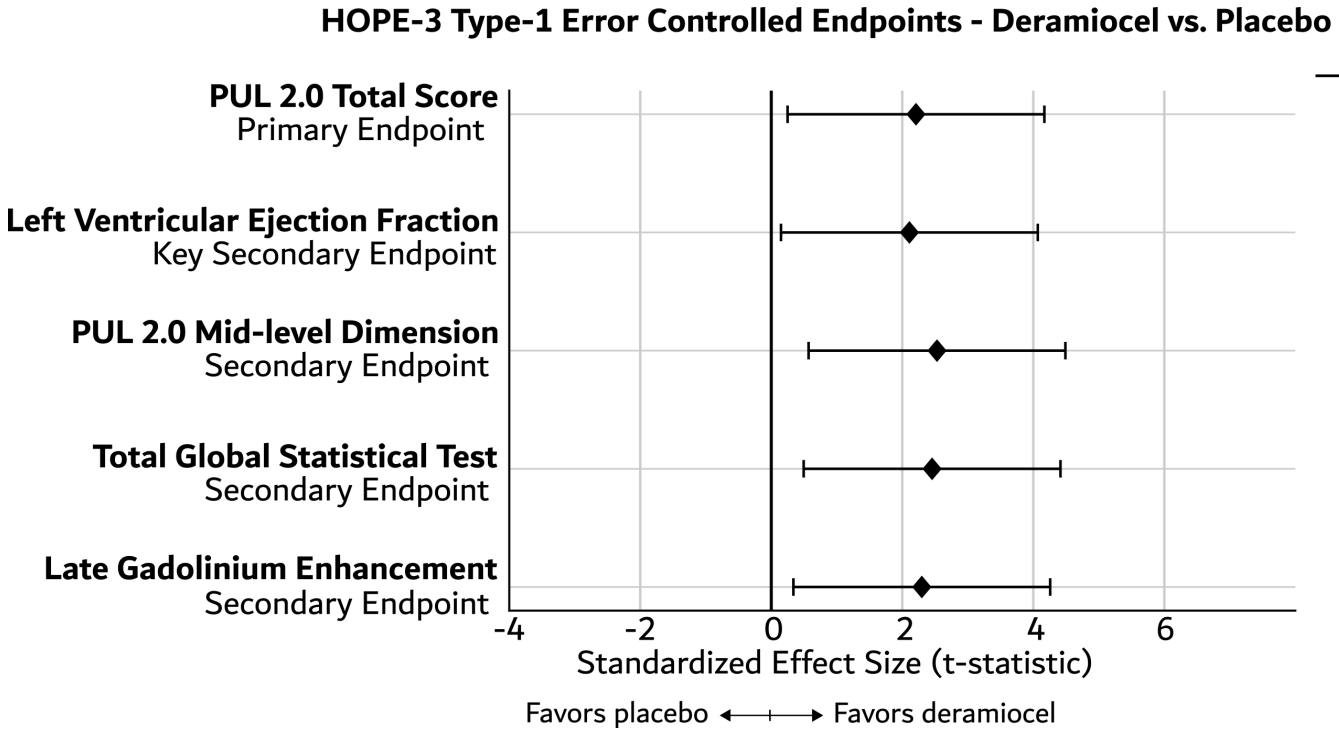
Deramiocel profile is comparable to placebo (which includes DMSO)

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)

HOPE-3 Overview of All Type-I Error-Controlled Endpoints

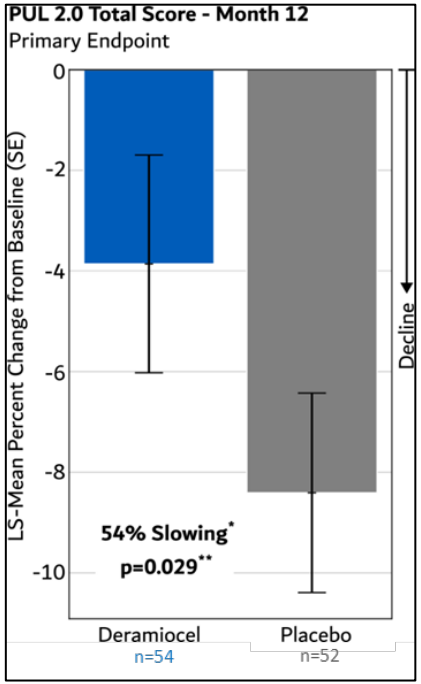
Deramiocel vs. Placebo

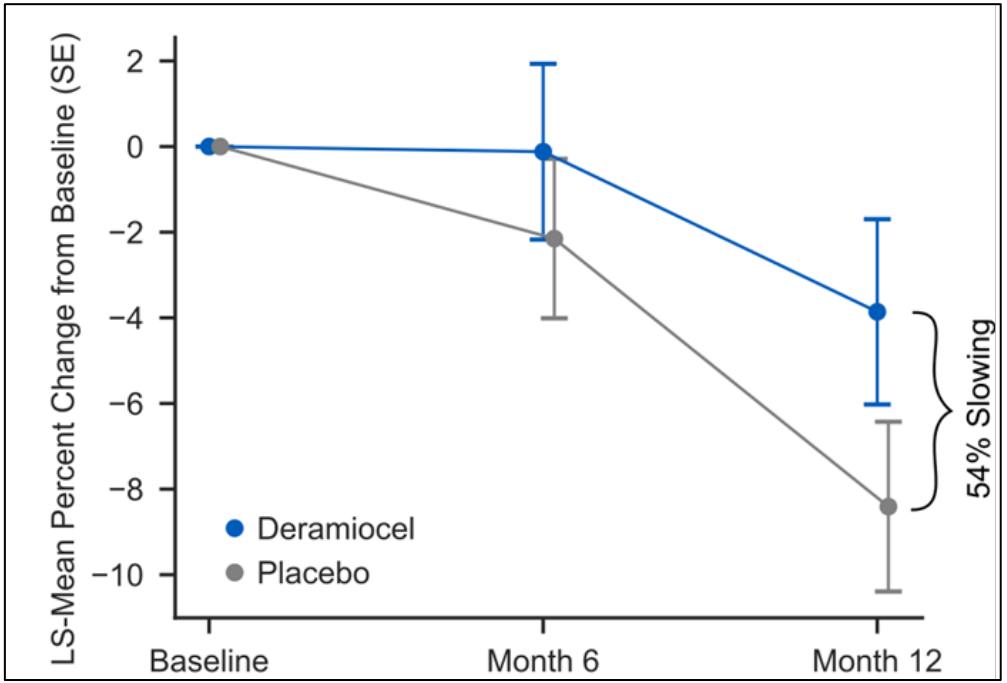


	n	Treatment Difference	p-value
PUL 2.0 Total Score Primary Endpoint	106	1.2 pts	0.029
Left Ventricular Ejection Fraction Key Secondary Endpoint	83	2.4 % pts	0.041
PUL 2.0 Mid-level Dimension Secondary Endpoint	106	1.0 pts	0.008
Total Global Statistical Test Secondary Endpoint	103	2.5 (t-statistic)	0.017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

HOPE-3 Primary Endpoint: PUL 2.0 at 12 Months

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

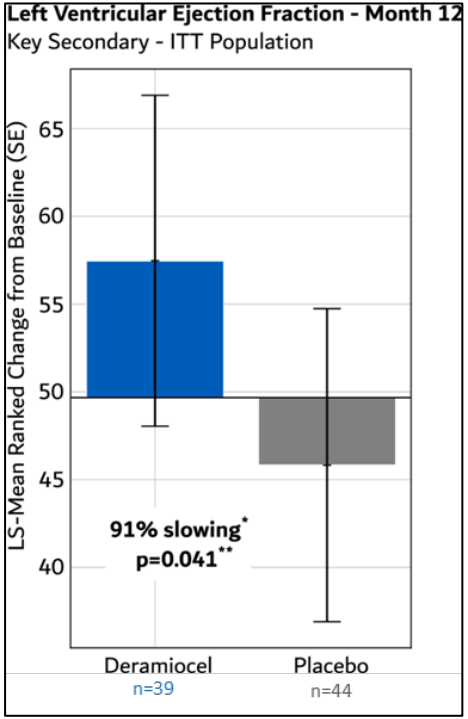


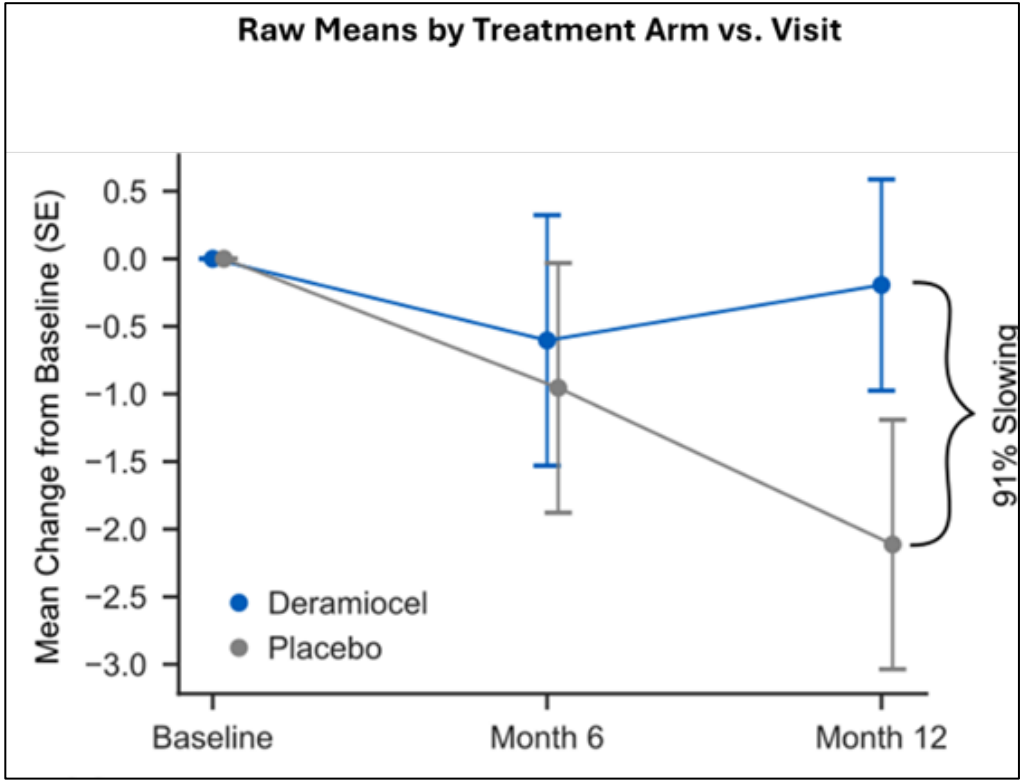


*LS-Mean difference = 4.55 percentage point (1.2 -point difference on the PUL scale)
** Based on prespecified repeated measures model using percent change from BL

HOPE-3 Key Secondary Endpoint: LVEF at 12 Months

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



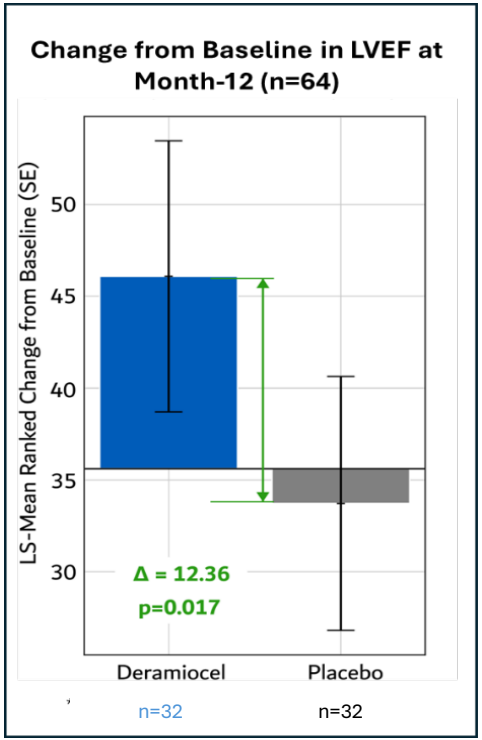


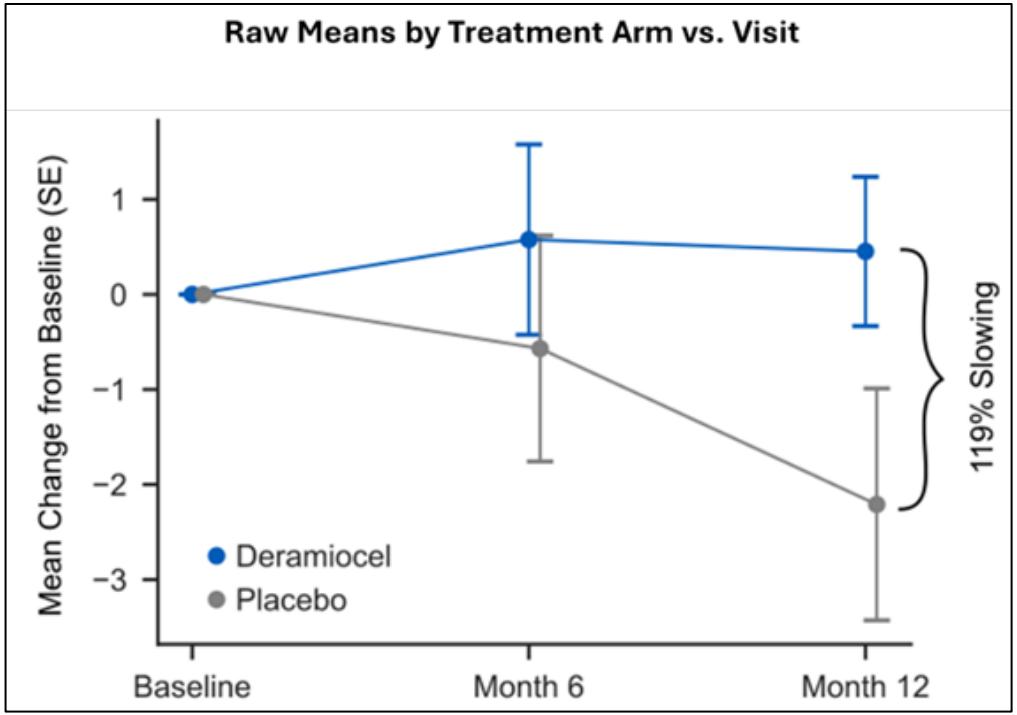
* LS-mean difference = 11.65 ranks (2.4 percentage point difference in LVEF)
** Based on prespecified rank ANCOVA model
LVEF: n reflects the number of patients in the ITT population with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months (n=83)

HOPE-3 Secondary Endpoint[1]: LVEF at 12 Months

Patients with Cardiomyopathy[2] at Baseline

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



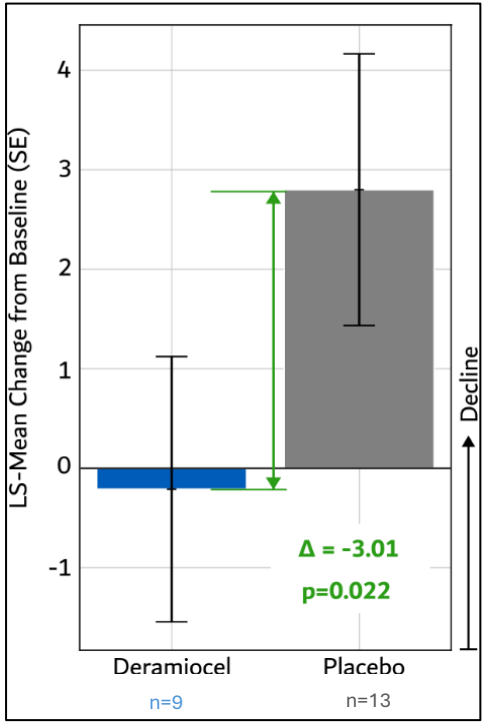


* LS-mean difference = 12.36 ranks (3.3 percentage point difference in LVEF)
** Based on prespecified rank ANCOVA model
LVEF: n reflects the number of patients in the ITT population with centrally reviewed and evaluable cardiac MRI LVEF assessments at baseline and 12 months (n=64)

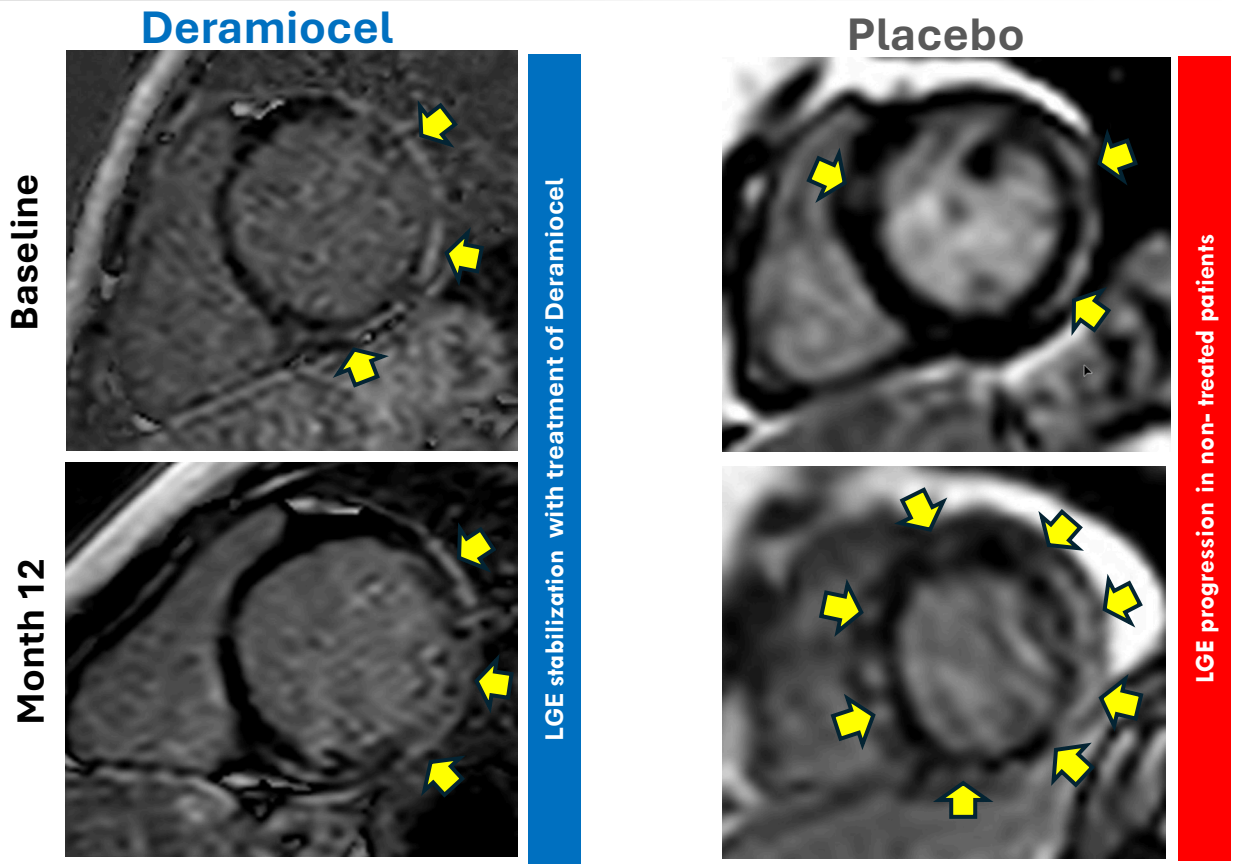
1 – Prespecified endpoint not TYPE-I error controlled
2 – Cardiomyopathy is defined as having clinical diagnosis, LVEF<55%, or LGE >0 myocardial segments

HOPE-3 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)



Conclusions

- **Deramiocel is an allogeneic cell therapy delivered intravenously every 3 months with an acceptable tolerability and safety profile**
- **The HOPE-3 Phase 3 study met all primary and secondary (type-1 error controlled) endpoints**
- **Anti-fibrotic activity was confirmed by stabilization of LGE in DMD-CM**
- **Stabilization of left ventricular ejection fraction (LVEF) seen in HOPE-3 is reasonably likely to translate into mortality benefit[1]**
- **Deramiocel BLA for the potential treatment of DMD under FDA review**
  - **Target PDUFA date: August 22, 2026**

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

Acknowledgements

A Huge Thank You!

To all the patients and families who participated in the HOPE-3 Study and…

- Muscular Dystrophy Association
- Parent Project Muscular Dystrophy
- CureDuchenne
- Michael Taylor, M.D., Ph.D. (Austin Childrens)
- Kan Hor, M.D. (Nationwide Childrens)
- HOPE-3 Investigators (20 sites) and Coordinators