Capricor Therapeutics™

Deramiocel – HOPE-3 Phase 3 Study Results

Parent Project Muscular Dystrophy

Deramiocel Regulatory Update

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Target PDUFA Date

August 22, 2026

Deramiocel - Mechanism of Action

- **Deramiocel** is a cellular therapy under review by the FDA for approval for the treatment of **cardiomyopathy** in patients with DMD

- **Deramiocel** is administered by IV infusion every 3 months
- **Deramiocel** is a suspension of allogeneic Cardiosphere Derived Cells (CDCs) which are **not stem cells and do not engraft**

- **Deramiocel** mechanism of action is via exosome release which have anti-fibrotic, anti-inflammatory, and immunomodulatory activities
- **Deramiocel** has been administered to ~125 DMD patients with over 800 infusions
- **Deramiocel** granted Orphan Drug, RMAT and Rare Pediatric Disease designations

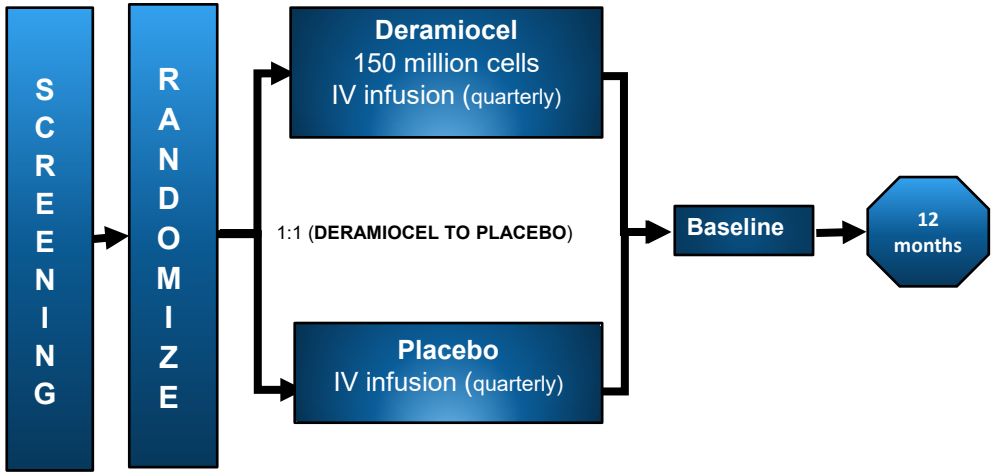
HOPE-3 Pivotal Phase 3 Trial

Study Design

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: 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
- **Announced positive topline results**: **Dec. 2025**



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

HOPE-3 Safety Results

Favorable Safety Profile

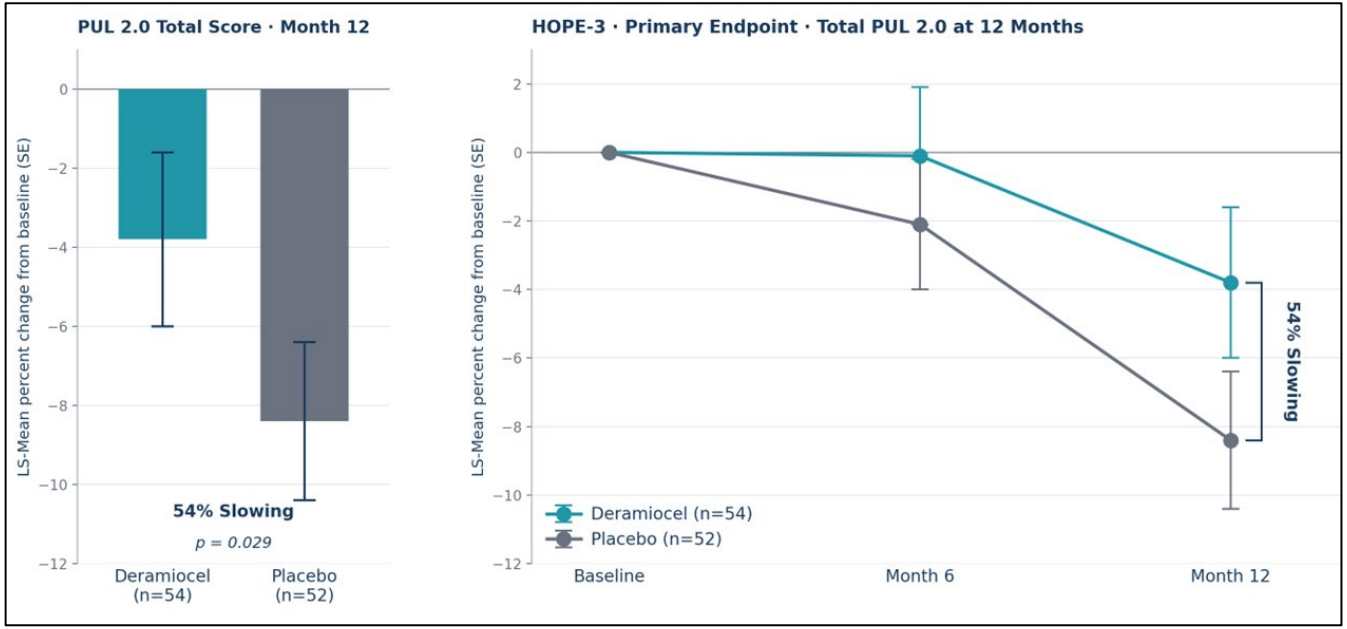
Overview	Placebo (n=52)	Deramiocel (n=53)	Overall (n=105)
Any TEAEs	43 (82.7)	50 (94.3)	93 (88.6)
TEAEs related to IP or administration procedure	19 (36.5)	44 (83.0)	63 (60.0)
TEAEs related to IP	16 (30.8)	44 (83.0)	60 (57.1)
TEAEs related to administration procedure	9 (17.3)	23 (43.4)	32 (30.5)
TEAEs RELATED TO IP / PROCEDURE — BY MAXIMUM SEVERITY, n (%)			
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
SERIOUS EVENTS & DEATHS, n (%)			
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 procedure	1 (1.9)	1 (1.9)	2 (1.9)

[1] Safety population (n=105)

HOPE-3 Primary Endpoint

Total PUL 2.0 at 12-Months

Mean Percent Change from Baseline



Δ 1.2 pts

54% slowing of disease

p = 0.029

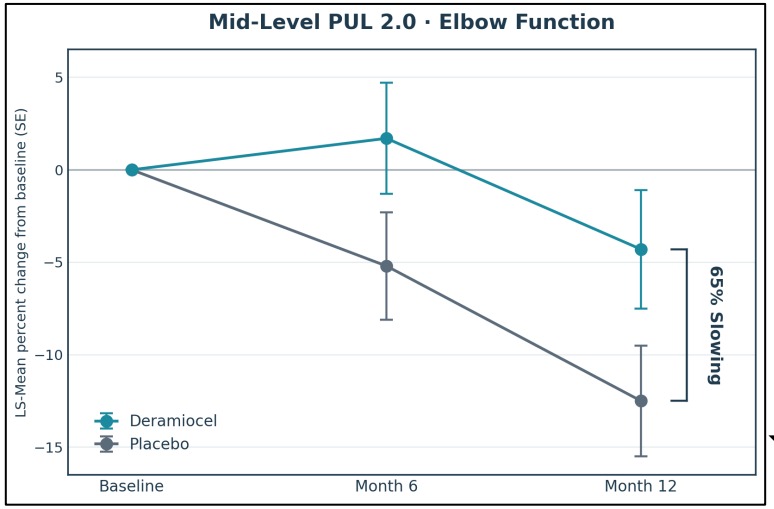
LS-mean difference 4.55 pp

Prespecified repeated-measures model using percent change from baseline. LS-mean difference = 4.55 percentage points, corresponding to a 1.2-point difference on the PUL 2.0 scale.

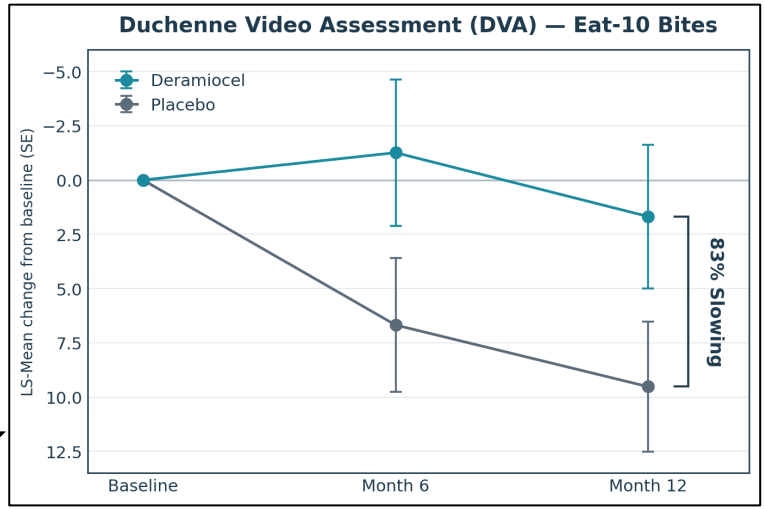
Mid-Level PUL 2.0 vs. DVA Eat-10-Bites

Statistically significant hand-to-mouth benefit observed via two independent measurement approaches.

Mean Percent Change from Baseline



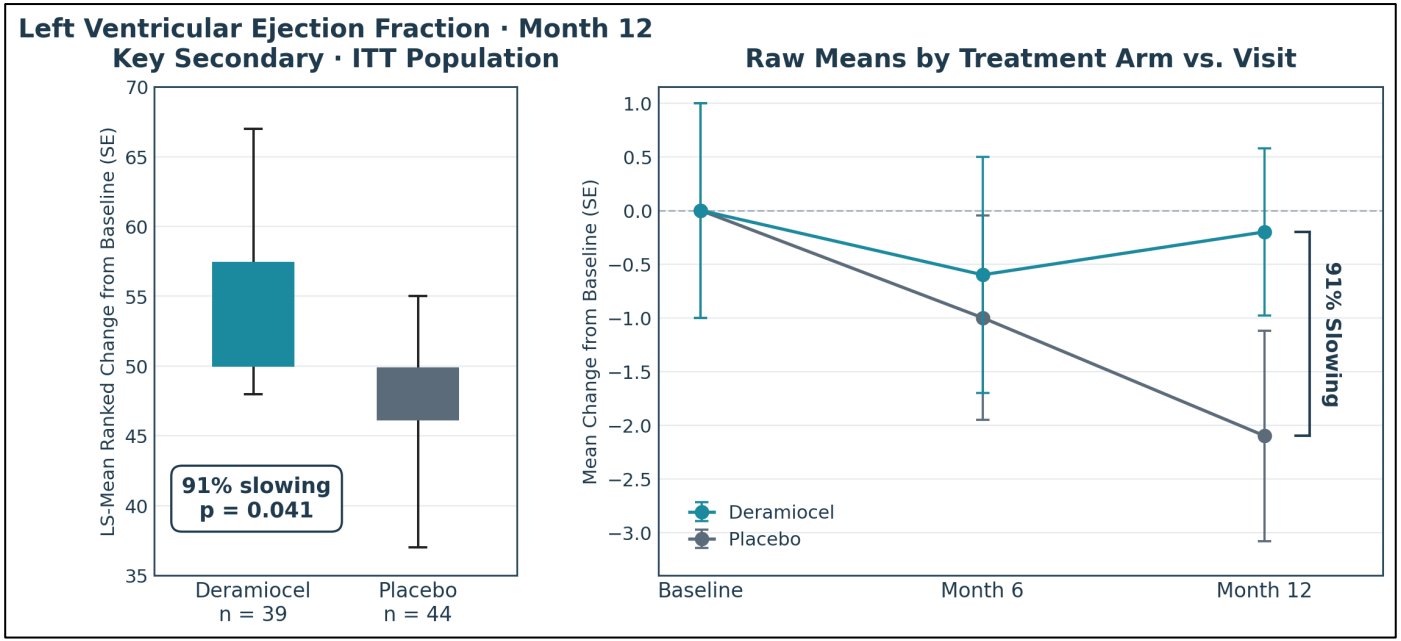
Disease Progression



- Two different tests agree: Mid-level PUL, done by a clinician, and the DVA Eat-10 Bites video.
- Both capture the same meaningful change for patients; confirming the result holds up across independent measures.

HOPE-3 Key Secondary Endpoint

Left Ventricular Ejection Fraction at 12-Months



Δ 2.4

LVEF percentage pts.

p = 0.041

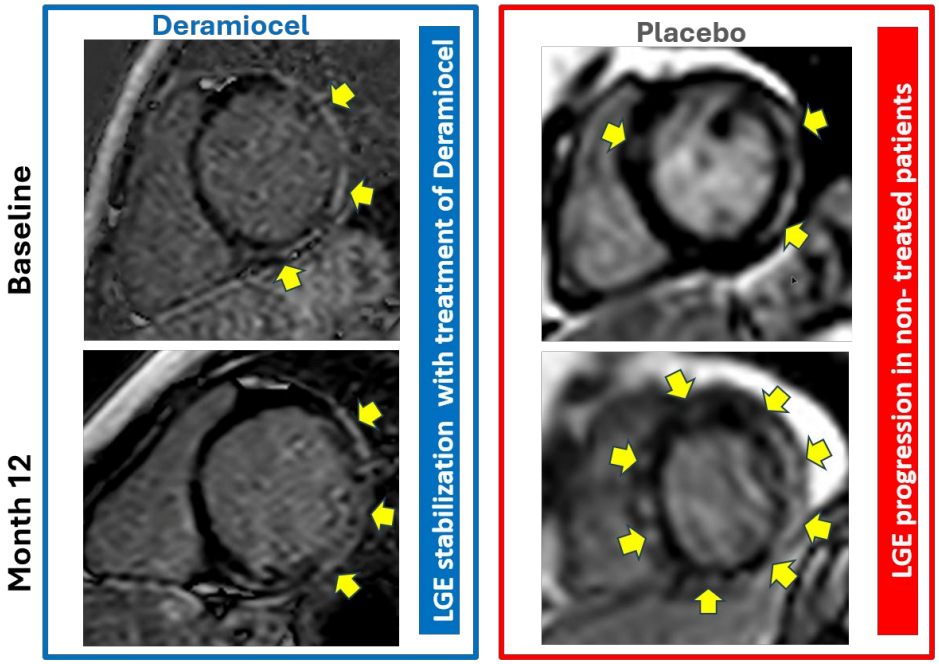
LS-mean diff. 11.65 ranks

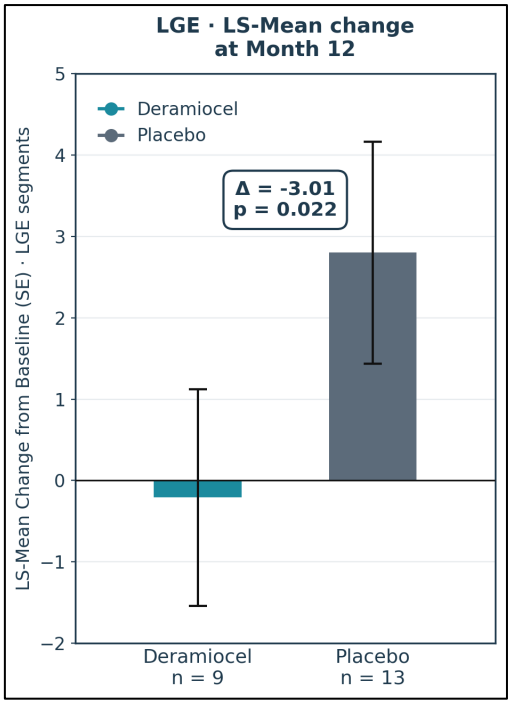
91% slowing of disease

LS-mean difference = 11.65 ranks, corresponding to a 2.4 percentage-point difference on LVEF. Based on prespecified rank ANCOVA model. ITT population with centrally reviewed and evaluable cardiac MRI LVEF at baseline and Month 12 (n=83).

Secondary: Late Gadolinium Enhancement

Progression of Heart Muscle Scarring





Δ 3.0

Mean Change Segments

p = 0.022

- **LGE stabilized with Deramiocel**
- **Non-treated patients progressed by 3 segments on average**

Conclusions- Skeletal Muscle

Allogeneic cell therapy
IV every 3 months · acceptable tolerability & safety

HOPE-3 met ALL endpoints
Primary + secondary (type-1 error controlled)

Upper Limb Function

Slowed disease progression in skeletal muscle function

- **54% slowing** PUL 2.0 Total Score (p=0.029)
- **65% slowing** PUL 2.0 Mid-level score (p=0.008)
- **83% slowing** Eat-10 Bites — Duchenne Video Assessment (p=0.018)

▶ **Protects activities of daily living** - transferring, turning, eating independently

Conclusions – Cardiac Function

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Cardiac Function

Stabilized LVEF and LGE (fibrosis) progression

- **91% slowing** LVEF — all evaluable patients (p=0.041)
- **>100% slowing** LVEF — known cardiomyopathy (p=0.017)
- **Reduced** rate of LGE segment progression (p=0.022)

▶ **Preserved 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

PPMD

[1]Soslow, J. H. et al. Circ.: Heart Fail. 16, e010040 (2023).

Acknowledgements

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A Huge Thank You!

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

PATIENT ADVOCACY ORGANIZATIONS

- Parent Project Muscular Dystrophy
- Muscular Dystrophy Association
- CureDuchenne

LEAD COLLABORATORS

- **Chet Villa, M.D.** *Cincinnati Children's*
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All the HOPE-3 Investigators and Study Staff