Late Breaking Abstract #6188

Deramiocel Significantly Slows Upper Limb Functional Decline in Duchenne Muscular Dystrophy: Skeletal Muscle Outcomes from the Phase 3 HOPE - 3 Trial

Craig McDonald[1], Susan Iannaccone[2], **Aravindhan Veerapandiyan[3]**, Arun S. Varadhachary[4], Leigh Ramos-Platt[5], Kathryn Gambetta[6], Russell Butterfield[7], Mindy Leffler[8], Kati Maharry[9], Nathaniel J. Hogan[9], Susan Apkon[10], Cuixia Tian[11], Katherine Mathews[12], Nancy Bass[13], Chamindra Laverty[14], Han Phan[15], Partha Ghosh[16], Seth Perlman[17], Rebecca Scharf[18], Kathryn Mosher[19], Saunder Bernes[20], Jane Emerson[21], Edward Smith[15], Michael Binks[9], Mark Awadalla[9], Linda Marban[9]

[1]UC Davis Dept. of PM&R, [2]Department of Pediatrics, UT Southwestern, [3]Arkansas Children's Hospital/UAMS, [4]Washington University in St Louis, [5]USC/Children's Hospital of Los Angeles, [6]Ann & Robert H. Lurie Children's Hospital of Chicago, [7]University of Utah, [8]Consultant, [10]Children's Hospital Colorado, [11]Cincinnati Children's Hospital Medical Center, [12]University of Iowa, Dept of Pediatrics, [13]Children's Wisconsin, [14]UC San Diego, [15]Rare Disease Research LLC, [16]Boston Children's Hospital, [17]Seattle Children's Hospital, [18]University of Virginia Children's Hospital, [19]Akron Children's Hospital, [20]Phoenix Children's Hospital, [21]University of Missouri School of Medicine, [9]Capricor, Inc.

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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, 2025, as filed with the Securities and Exchange Commission on March 17, 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.

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. Veerapandiyan has received clinical trial research grants from Capricor, Inc.

Dr. Veerapandiyan has consulted for Biogen, Novartis, Sarepta Therapeutics, PTC Therapeutics, Scholar Rock, Pfizer, Catalyst, Lupin, Entrada Therapeutics, Avidity, Solid, Regenxbio, Insmed, Keros, Precisionbio, Grünenthal, Mesoblast and Italfarmaco

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Introduction to Deramiocel

Cardiosphere derived cells (CDCs) are cardiac tissue derived progenitor 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 clinically and statistically meaningful benefit in both musculoskeletal (PUL v2.0) and cardiac domains (LVEF)

- HOPE-2- randomized, double blind, PBO controlled[4]
- HOPE-2 open label extension[5]

A Phase 3 study was initiated in 2022 and the primary 12-month analysis of musculoskeletal endpoints will be presented today

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).

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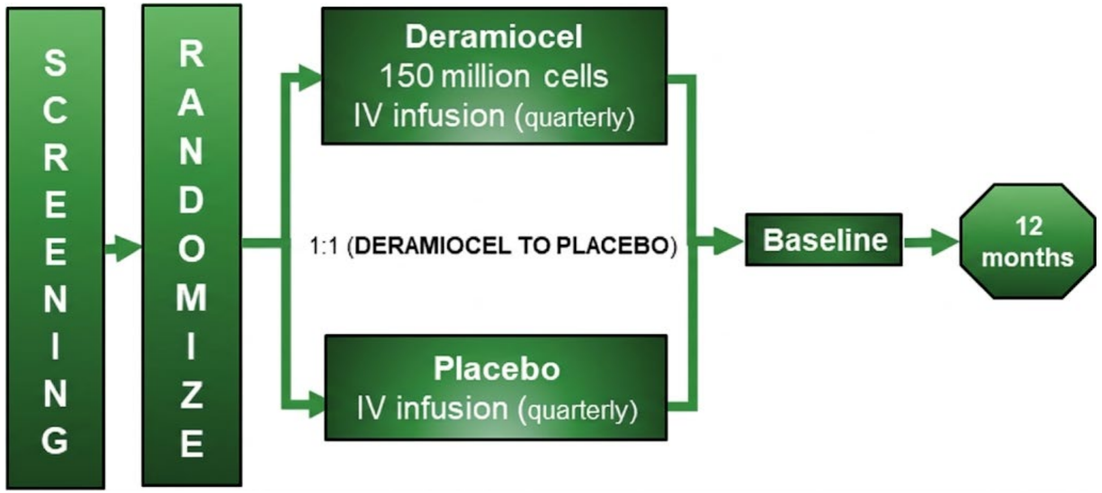
HOPE-3 Pivotal Phase 3 Trial

Study Design



Key Entry Criteria

- Ages ≥ 10 who are late or non-ambulatory
- PUL v2.0 Entry Item Score ≥ 2
- LVEF ≥ 35%
- All patients on background stable corticosteroid therapy

HOPE-3

DUCHENNE CLINICAL TRIAL

Design & Endpoints

- **Randomized** (1:1), double-blind, placebo-controlled study
- **N = 106** subjects enrolled at 20 trial sites in USA

- **Primary efficacy endpoint**[1]: skeletal muscle assessment
  - **Performance of Upper Limb** (PUL v2.0)

- **Key secondary endpoint**[1]: cardiac assessment
  - Left ventricular fraction (LVEF)

- **Other secondary endpoints**: **mid-level PUL v2.0**[1], GST[1], LGE[1] and **Duchenne Video Assessment (DVA): Eat 10 bites**

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ClinicalTrials.gov ID: NCT05126758

[1]Type-I error controlled

Background to PUL v2.0 and DVA endpoints

Performance of upper limb (PUL) v2.0

Functional scale designed to assess upper limb function in both ambulant and non-ambulant patients[1]

Entry Item: begins with a "starter" task that determines the functional starting point (0-6)

Total score follows the proximal to distal progression in DMD and is comprised of 22 items in 3 domains: shoulder level, elbow and distal level (scoring 12,17 and 13 points respectively). Assessed by trained PT.

Most items are scored on a 0–2 scale:

2: Performed without compensation;

1: Performed with compensation;

0: Unable to perform.

The maximum score is 42 and is normally achieved by age 5 with well defined progression of loss[2].

Duchenne Video Assessment (DVA): Eat 10 Bites

A valid and reliable tool used to capture change in the severity of upper limb functional impairment over time during performance of clinically meaningful activities of daily living[3]

Video-based home assessment with centralized observer evaluation of how the degree of difficulty, compensatory movement, endurance, and task efficiency evolve relative to a prior performance

Eat 10 Bites task is a central component of the DVA due to its high sensitivity to incremental changes and direct relevance to patient independence

DVA-certified physical therapists (PTs) assess the compensations using scorecards[4], were blinded to date order and treatment allocation

References:

1. Mayhew A *et al. Dev Med Child Neurol* 2013; **55**: 1038–45.
2. Pane M *et al. J Neuromuscular Dis* 2023; **10**: 567–74.
3. Contesse M *et al*. *Muscle Nerve* 2021; **64**: 180–9.
4. Contesse MG*et al. PLoS ONE* 2022; **17**: e0266845.

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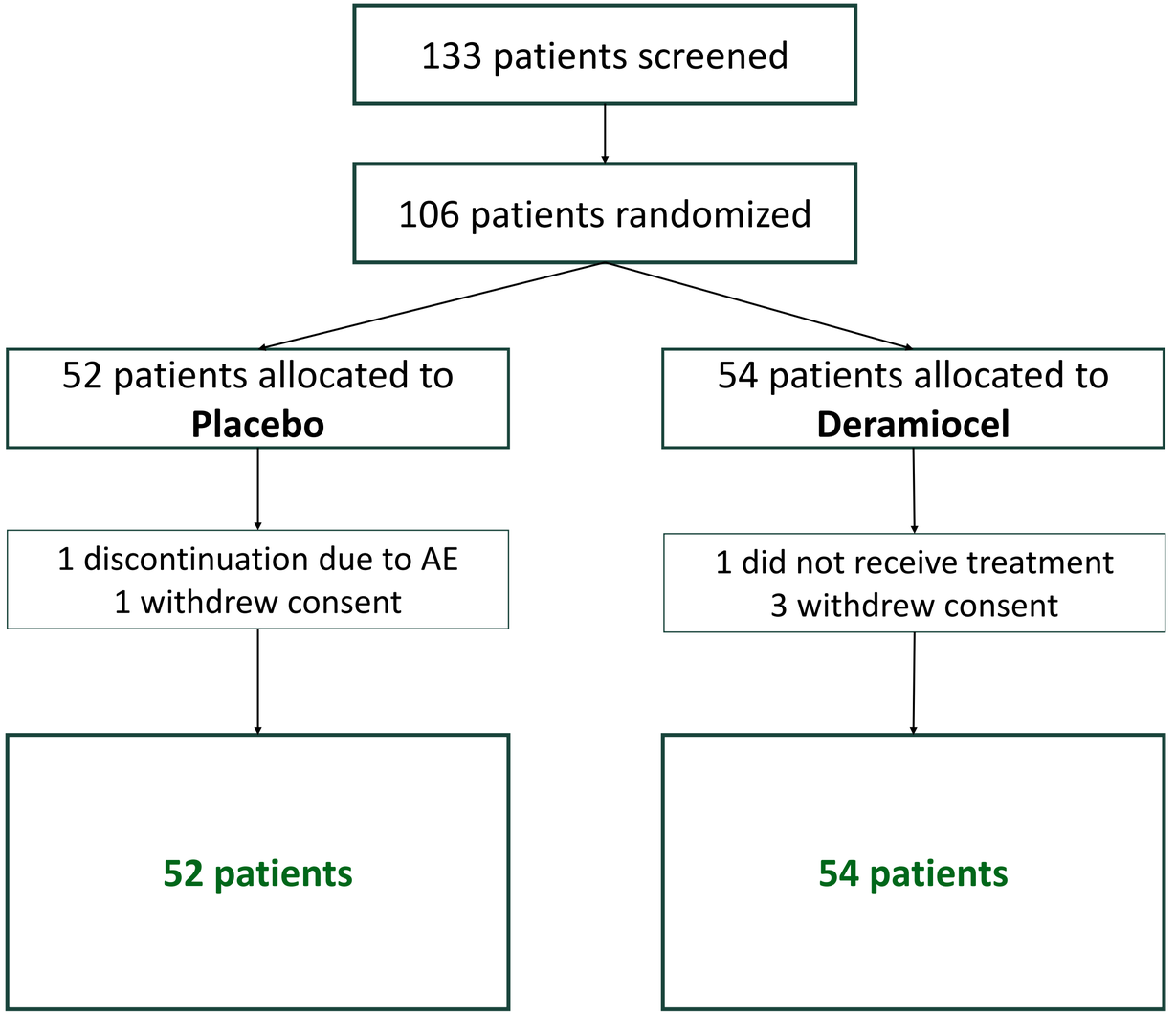
HOPE-3 Pivotal Phase 3 Trial

Patient disposition



OUTCOME MEASURES

Primary PUL v2.0 (ITT=106)

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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)
PUL v2.0 baseline score	**26.5 (6.69)**	**27.6 (7.36)**	**27.1 (7.03)**
Exon-skipping co-medication	**12 (23.1)**	**14 (25.9)**	**26 (24.5)**
Ambulatory status			
Non-ambulatory	44 (84.6)	46 (85.2)	90 (84.9)
Ambulatory	8 (15.4)	8 (14.8)	16 (15.1)

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

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)

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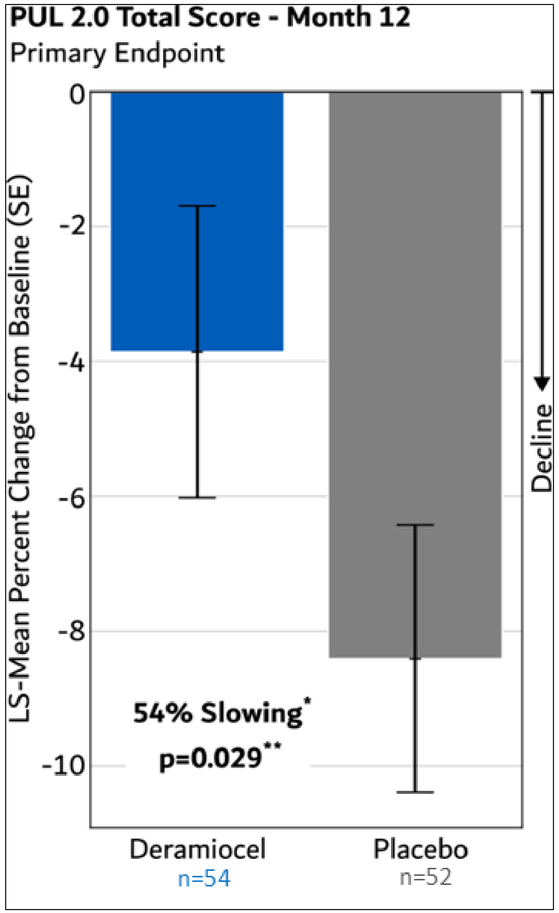
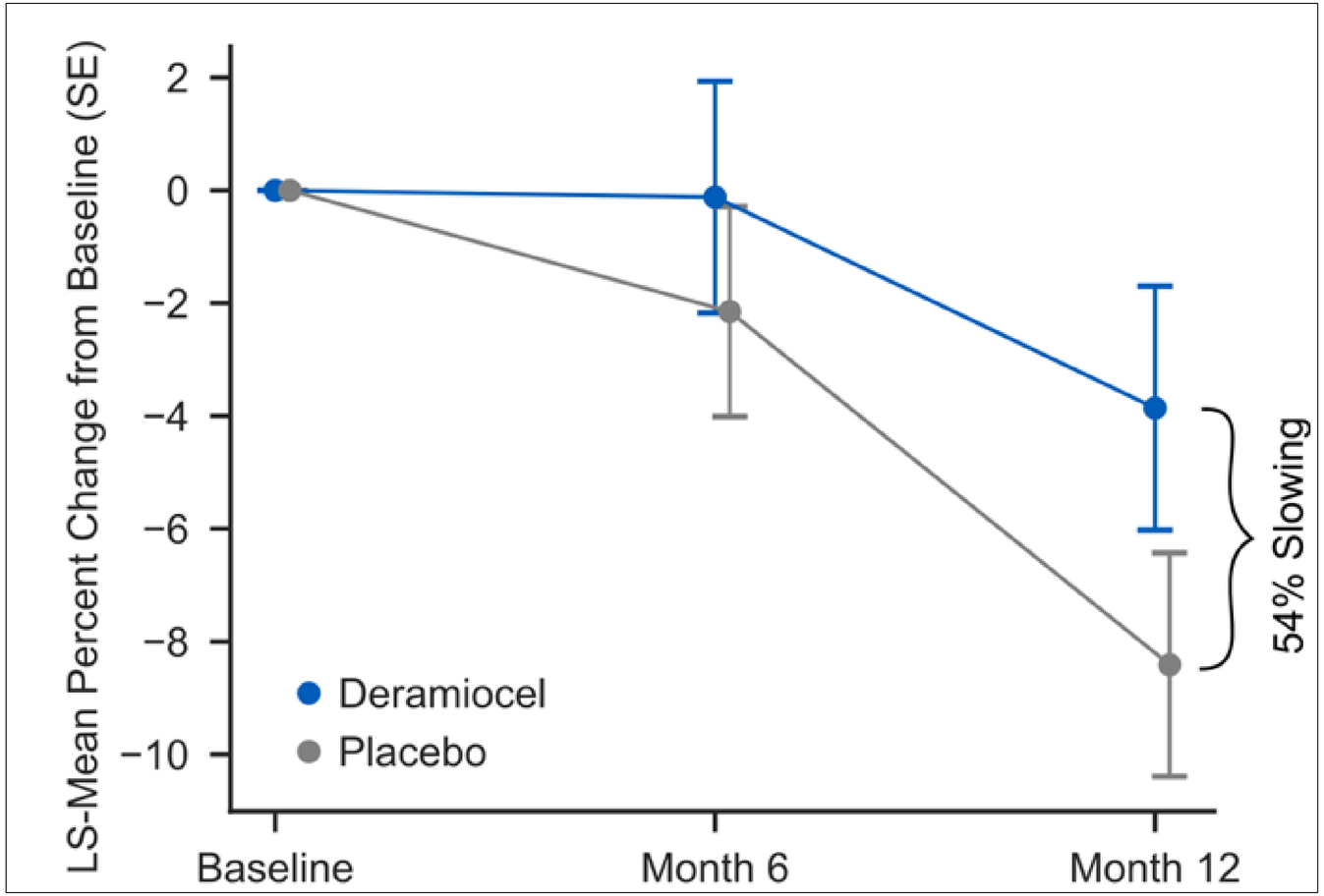
Primary Endpoint: PUL v2.0 at 12 Months

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

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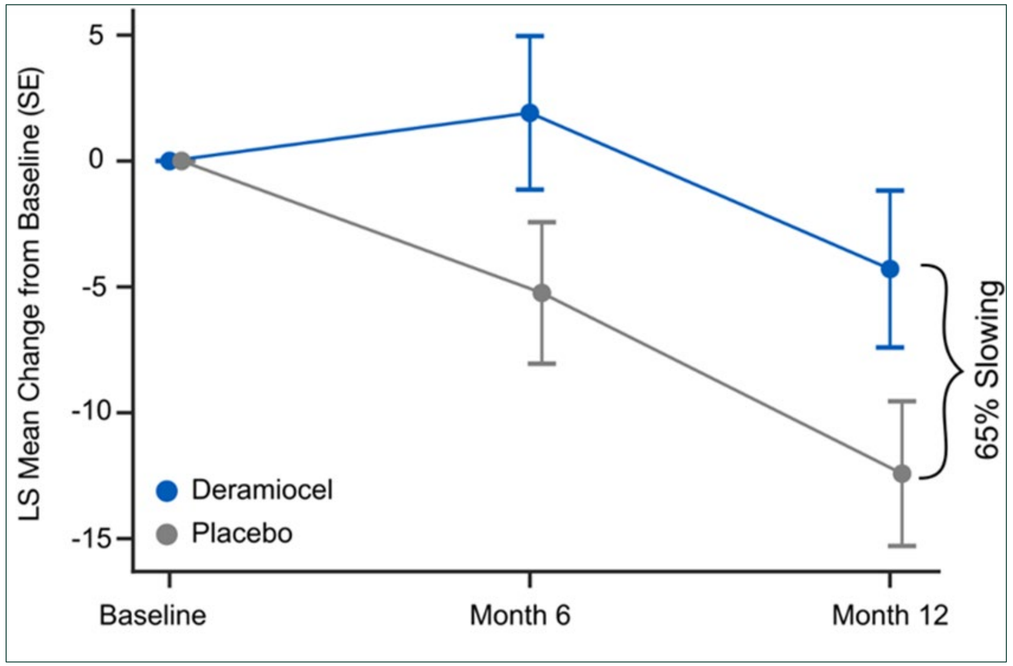
Mid-Level PUL v2.0 vs. DVA: Eat 10 Bites

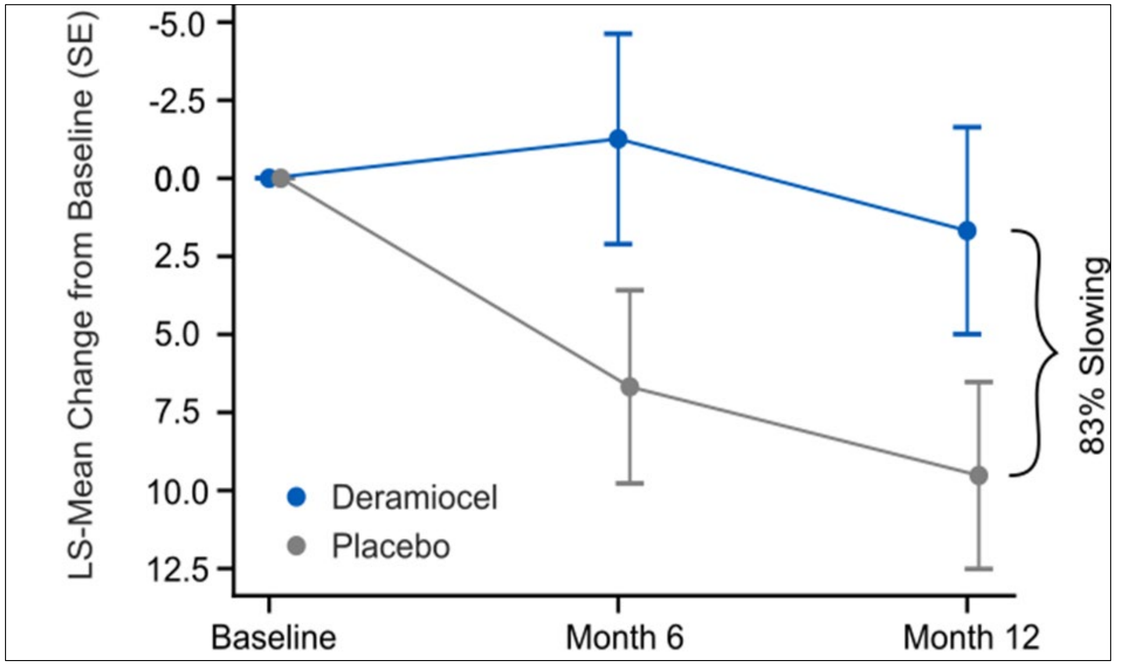
Similar Statistically Significant Benefits in Hand to Mouth Function across multiple analyses

Mid-Level PUL v2.0



Disease Progression

Duchenne Video Assessment (DVA): Eat 10 bites



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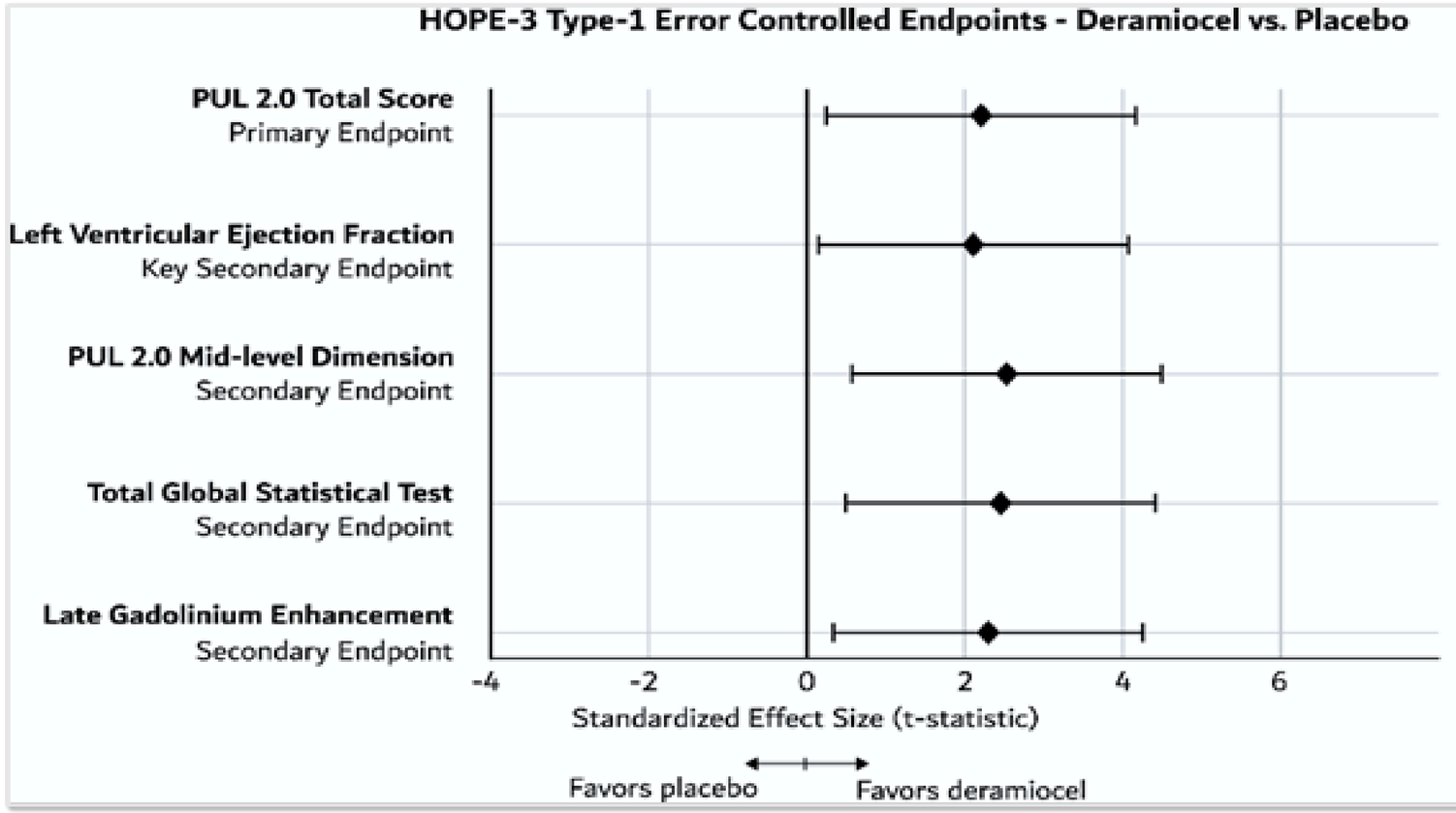
In addition to upper limb function, Deramiocel provides treatment benefits on cardiac function and fibrosis

Overview of All Type-I Error-Controlled Endpoints (Primary and Secondary)



	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

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Conclusions

- **Deramiocel is an allogeneic cell therapy, which has acceptable tolerability and safety**
- **HOPE-3 study met all its primary and secondary (type-1 error controlled) endpoints**
- **Upper Limb Function – Deramiocel slowed disease progression by ~50-80%**
  - Performance of Upper Limb (PUL v2.0) Total Score – Primary endpoint
  - Performance of Upper Limb (PUL v2.0) Mid level score – Secondary endpoint, Type-1 error controlled
  - Eat 10 Bites – Duchenne Video Assessment – Secondary endpoint

PDUFA Date: August 22, 2026

Efficacy on Cardiac Function

– Deramiocel preserved LVEF and slowed LGE (cardiac fibrosis)

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

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

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CureDuchenne

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