



Deramiocele Program Update

Parent Project Muscular Dystrophy JOIN THE FIGHT. END DUCHENNE.

Capricor Therapeutics, Inc. • Nasdaq: CAPR
October 7, 2026 • PPMD Webinar

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; results of securities litigation; 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 Deramiocecel 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 June 30, 2026, as filed with the Securities and Exchange Commission on August 14, 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.

Deramiocecel and Capricor's StealthX™ exosome therapeutics are investigational and have not been approved for commercial use in any indication.

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CLINICIANS/KOLS

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National Principal Investigator, HOPE-2 and HOPE-3

Professor of Pediatrics; Chair, Department of Physical Medicine and Rehabilitation; Director of Rehabilitation Services and of the Neuromuscular Disease Clinics, UC Davis Health

Chet Villa, M.D.

Professor of Pediatrics

The Heart Institute, Cincinnati Children's Hospital Medical Center

NEW FDA DECISION DATE (PDUFA)

Nov. 22, 2026

Extended 3 months from Aug. 22, 2026
after FDA classified our submission as a
major amendment

✓ BLA remains under active review



PHASE 3 HOPE-3 RESULTS

4.55%

better preserved arm function
(PUL 2.0) vs. placebo at 12 months

✓ Primary endpoint met ($p=0.029$)

One of the largest DMD datasets



REFINED INDICATION FOR

Ages 9+

Seeking approval for upper
limb impairment in DMD

Deramiocele BLA Amendment Data Package

How our application changed, and the new data FDA is now reviewing

ORIGINAL FILING

**DMD-associated
cardiomyopathy**

FILED WITH

HOPE-2 (Phase 2) RCT
12-month results
CRL issued July 2025

HOPE-3 (Phase 3) RCT
12-month results



REFINED INDICATION

**DMD upper limb
impairment**
Ages 9 and older



NEW DATA ADDED

HOPE-3
Robustness and sensitivity analyses

HOPE-3 OLE
24-month skeletal muscle and cardiac
data

HOPE-2 + HOPE-3
Integrated summary of efficacy (ISE)

THE LANCET

Articles

Deramiocele heart-derived cellular therapy in advanced Duchenne muscular dystrophy (HOPE-3): a phase 3, randomised, double-blind, placebo-controlled trial

Craig M McDonald, Chet Villa, Jonathan H Soslow, Kati Maharry, Nathaniel Hogan, Michael Binks, Kevin C Berth, Kristi A Elliott, Michael Taylor, Kan N Hor, James Signorovich, Erik K Henricson, Han C Phan, Susan Apkon, Partha S Ghosh, Cuixia Tian, Aravindhan Veerapandian, Leigh Ramos-Platt, Katheryn Gambetta, Saunder M Bernes, Arun S Varadhachary, Susan T Iannaccone, Chamindra G Laverty, Seth J Perlman, Nancy E Bass, Kathryn Mosher, Russell J Butterfield, Katherine D Mathews, Rebecca J Scharf, Edward C Smith, Jane Anne Emerson, Eugenio Mercuri, Mark S Awadalla, Linda Marbán*, Eduardo Marbán*, on behalf of the HOPE-3 Investigators†



McDonald, et al. *The Lancet*, July 2026

Consistent Benefits Shown Across the HOPE Studies



~1 point per year PUL 2.0 treatment benefit, consistent in every HOPE study

	HOPE-3	HOPE-3 OLE	HOPE-2	HOPE-2 OLE	ISE
Duration	12 months	12 months	12 months	36 months	12 months
Comparison	Deramiocelel vs. placebo	Delayed-start vs. placebo	Deramiocelel vs. placebo	Deramiocelel vs. ECP	Deramiocelel vs. placebo
Sample size (treated/control)	n=54/52	n=49 (paired)	n=8/12	n=13/30	n=62/64
PUL 2.0 Total • annual effect	1.1	1.6	1.8	1.2	1.3
PUL 2.0 Mid-level • annual effect	0.9	0.9	0.9	N/A	0.9

Effect = annual difference in PUL 2.0 score (points). N/A: HOPE-2 OLE external control did not include PUL mid-level. Sources: Gillman et al., 2025.



Robust
Holds across all analysis methods



Clinically meaningful
FDA acknowledged 1 point in 12 months is meaningful



Matters to patients
Linked to maintaining quality of life



HOPE-3 OLE: 2-Year Results

Craig McDonald, M.D.

National Principal Investigator, UC Davis Health

HOPE-3 OLE Overview

HOPE-3 OLE Efficacy Results

HOPE-3 OLE Safety Summary

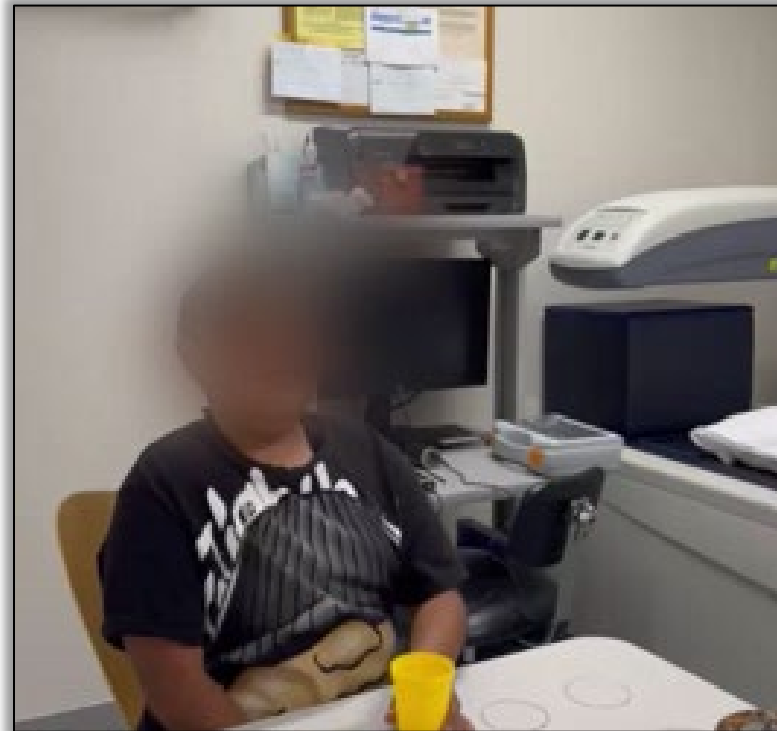
PUL 2.0: 1-Point Change is Clinically Meaningful

Disease Progression

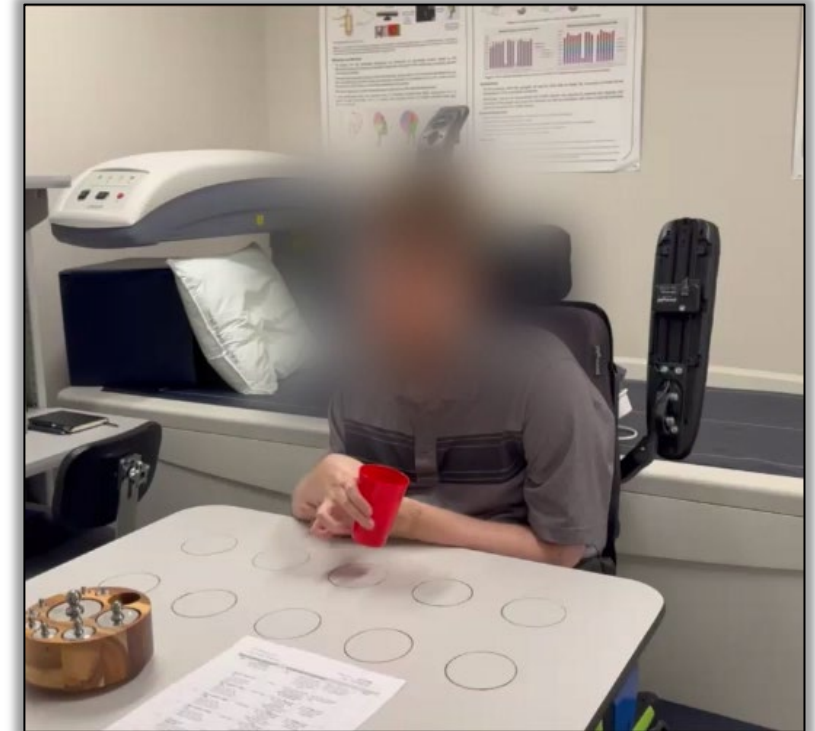


PUL 2.0 Score	
2	Normal Ability
1	With difficulty / compensation
0	Unable to perform

Bringing Hand to Mouth
PUL 2.0 Score = 2
(Normal)

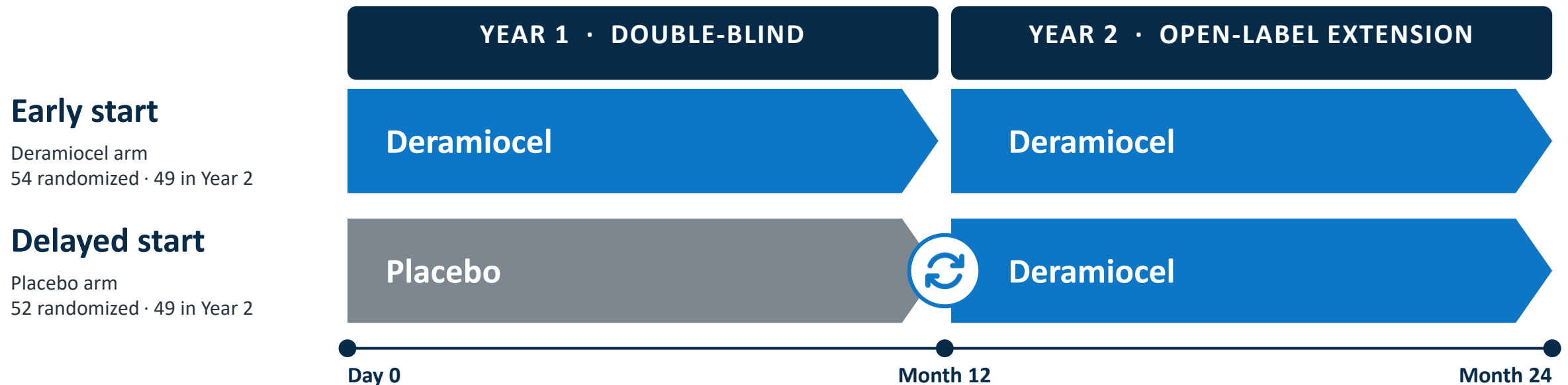


Bringing Hand to Mouth
PUL 2.0 Score = 1
(Difficulty/Compensation)



HOPE-3 OLE: Study Design

Year 1 compared DeramioceI with placebo. In Year 2, all patients received DeramioceI



PATIENTS

98

entered Year 2
49 per group

82

completed 2 years
40 early · 42 delayed

CARDIAC MRI (cMRI) SCANS

85

MRI at Month 12
40 early · 45 delayed

69

MRI at Month 24
31 early · 38 delayed

HOPE-3 OLE: Baseline Characteristics

A later-stage, mostly non-ambulatory population; groups were similar at the start

~15 years

average age at randomization

~84%

non-ambulatory in both groups
(not walking independently)

Characteristic	Deramiciocel (early start, N=49)	Placebo (delayed start, N=49)
Age at randomization, years, mean (SD)	15.3 (3.11)	14.4 (2.93)
Age at DMD diagnosis, years, mean (SD)	4.6 (2.60)	4.3 (2.22)
Non-ambulatory, n (%)	41 (83.7)	41 (83.7)
PUL 2.0 entry item score 2 to 3, n (%)	22 (44.9)	20 (40.8)
PUL 2.0 entry item score 4 to 6, n (%)	27 (55.1)	29 (59.2)
PUL 2.0 Total Score, mean (SD)	27.71 (7.48)	26.73 (6.81)
Slow progressor, n (%) [*]	1 (2.0)	5 (10.2)
Systemic corticosteroid, n (%)	49 (100.0)	48 (98.0) [†]

Patients entering the OLE (N=98), by original randomization assignment; values recorded at the double-blind baseline (Day 0). All patients were male.

^{*}Any of: PUL 2.0 entry item score of 6 at both screening visits; exon 44 skipping amenability; deletion of exons 3 to 7; or a mutation associated with Becker muscular dystrophy or a mild dystrophinopathy phenotype (Kesari et al. 2008).

[†]One placebo patient counted as untreated in the corticosteroid row was on deflazacort per the eligibility record.

HOPE-3 Primary Endpoint: PUL 2.0 Total Score

Patients on Deramiocelel lost less arm function over 12 months than those on placebo

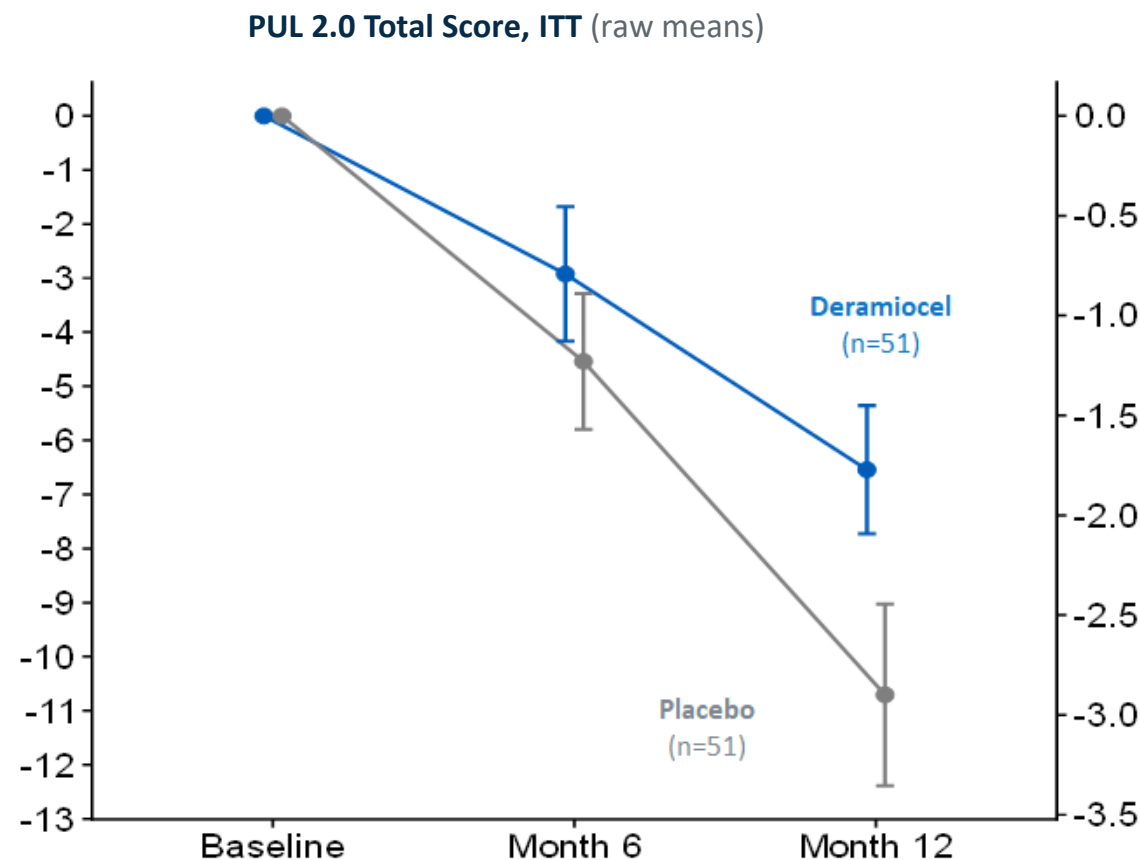
PRIMARY ENDPOINT

4.55%

Less decline vs. placebo
(percent change)

$p = 0.029$

Mean **Percent**
Change (SE)



SENSITIVITY ANALYSIS

1.1 points

Less decline vs. placebo
(absolute change)

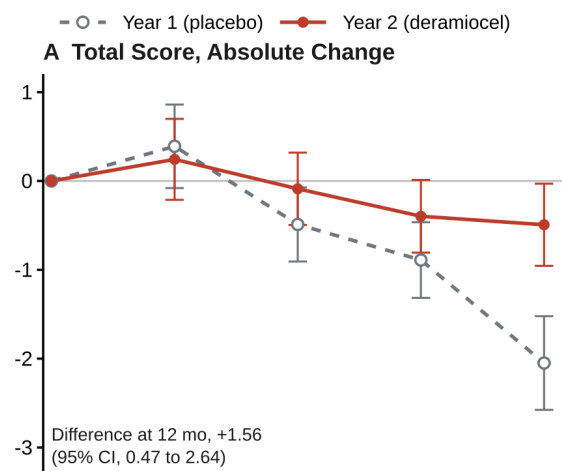
$p = 0.050$

Mean **Absolute**
Change (SE)

HOPE-3 OLE: PUL 2.0 Results

The benefit follows the treatment, not the passage of time

Delayed start: placebo year vs. first treated year



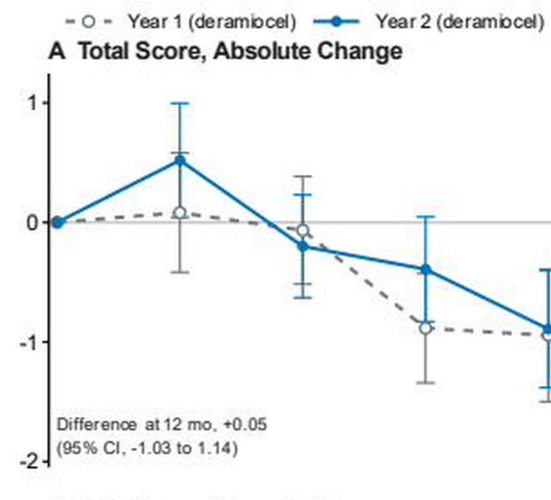
Difference at 12 mo

+1.56

(95% CI 0.47 to 2.64)
✓ Treated year better

Also favored on mid-level (+0.88) and percent change (+6.84, +8.47); all CIs exclude zero

Early start: treated year 1 vs. treated year 2



Difference at 12 mo

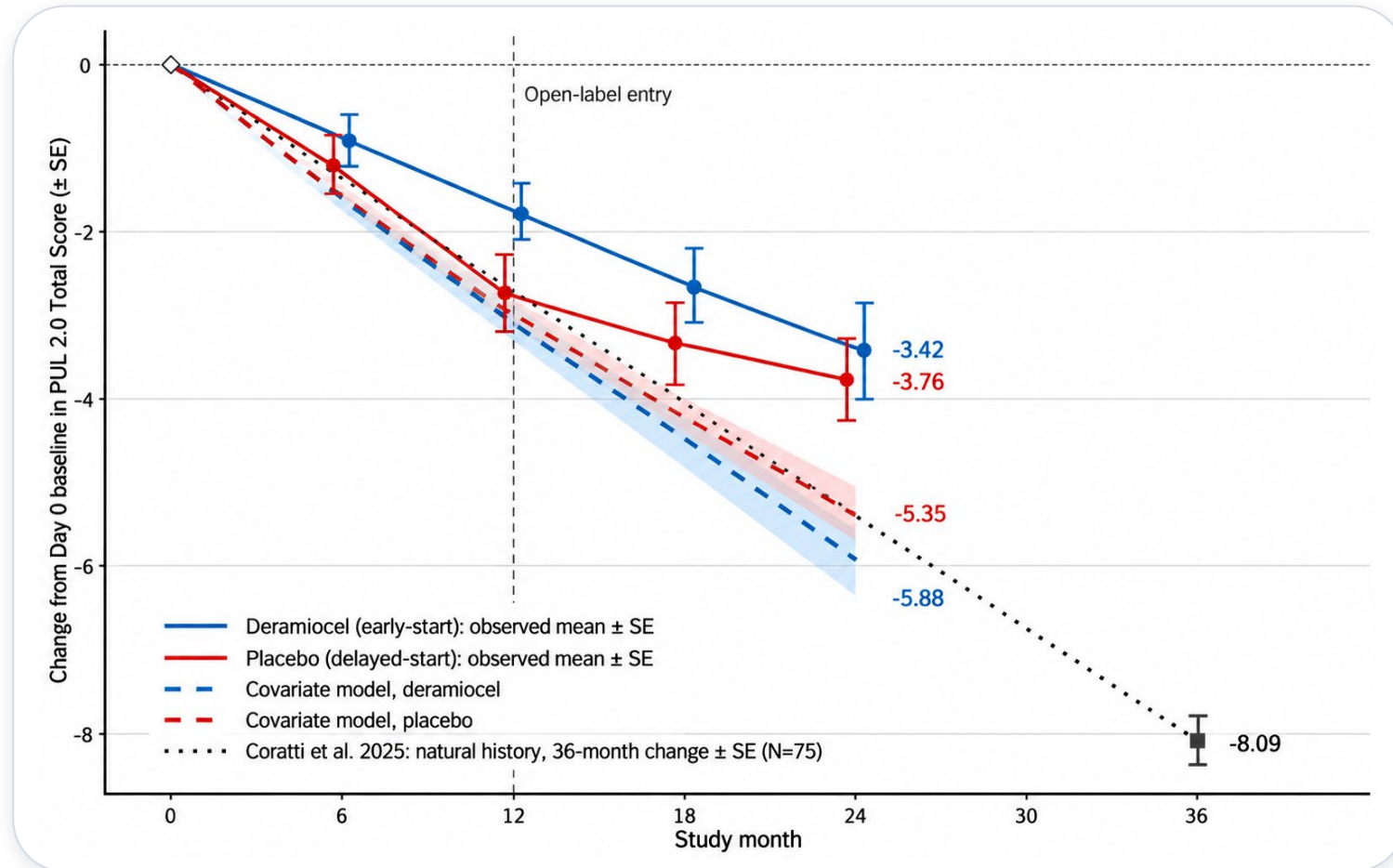
+0.05

(95% CI -1.03 to 1.14)
✓ No difference, as expected

Not randomized; each patient is their own comparator. Change from each period's own anchor (Day 0 or Month 12), months 0–12. Cross-phase; ITT; error bars ± 1 SE.

HOPE-3 OLE: Two Years vs. Expected Decline

At 2 years, patients on Deramiciel declined less than models and natural history predicted



KEY TAKEAWAYS

- **Early start patients**
 - -3.42 vs. -5.88 points after 2 years on Deramiciel
- **Delayed start patients:**
 - -3.76 vs. -5.35 points after switching at Month 12
- Decline slowed once placebo patients started Deramiciel
- **Open-label, model-based comparison: supportive of the randomized HOPE-3 results**

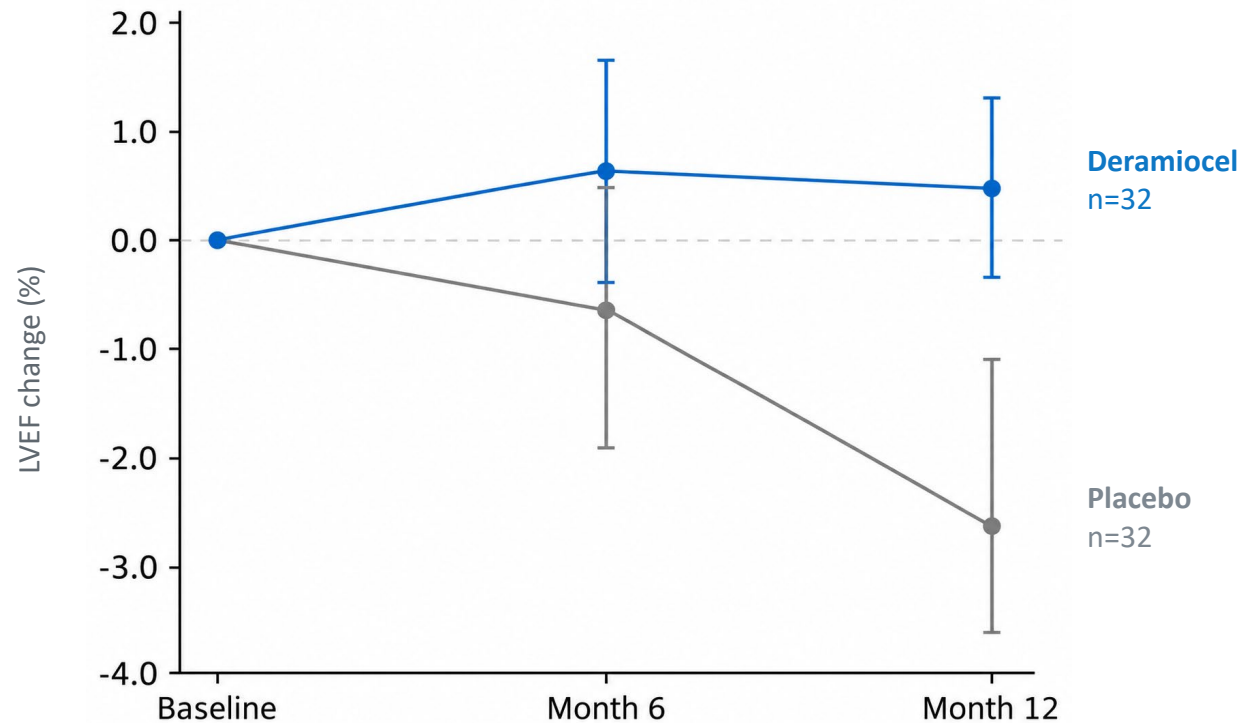
Open-label extension compared with covariate prediction models and published natural history; supportive and exploratory. % less decline = $(\text{predicted} - \text{observed}) \div \text{predicted}$ at Month 24.
References: Coratti G, et al. J Neuromuscul Dis. 2025;13(3):293-303. Michaëls M, et al. Neuromuscul Disord. 2025;53:105540. Nicks EH, et al. MDA 2026, Poster 152M.

HOPE-3: Heart Function (LVEF: 1-Year Results)

PATIENTS WITH CARDIOMYOPATHY

Heart pumping function held steady on Deramiocelel and declined on placebo

Change in LVEF from baseline (raw means, $\pm$ SE)



DIFFERENCE VS. PLACEBO

2.8%

in LVEF at 12 months (n=64)

p = 0.017 ranked ANCOVA **p = 0.026** Wilcoxon

HOPE-3 OLE: LVEF vs. Matched Natural History

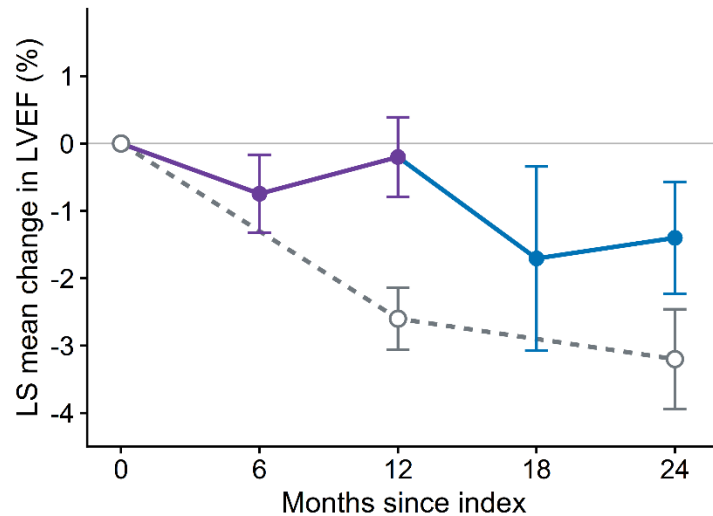
—●— First year on Deramiciel (both arms)

—●— Second year (early start only)

—○— Matched external natural history

ITT

Treated n=73 · External n=103



Month 12

+2.4 pp

(0.9, 3.9)

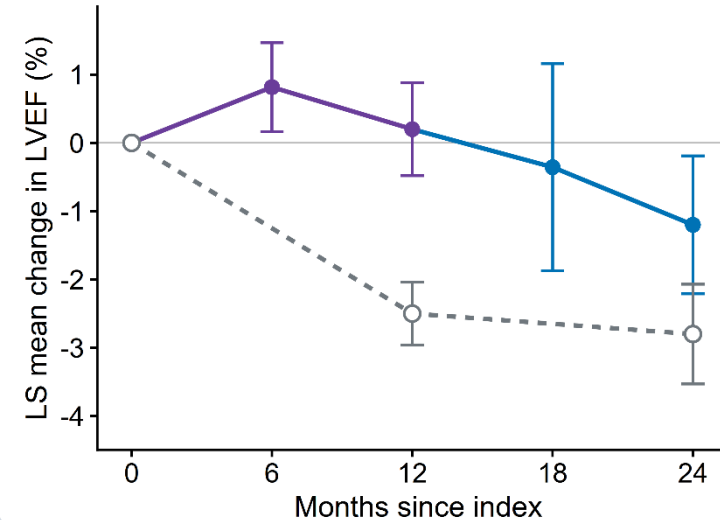
Month 24

+1.8 pp

(-0.4, 4.0)

Cardiomyopathy (sub-population)

Treated n=56 · External n=100



Month 12

+2.7 pp

(1.1, 4.4)

Month 24

+1.7 pp

(-0.8, 4.2)

Heart function preserved

LVEF stayed near baseline on Deramiciel; matched patients declined

Matching: DMDCCC natural history; propensity score on age, LVEF, LVEDVi and LVESVi at index; nearest neighbor 1:1 to 1:5, caliper 0.25 SD. All treated: 73 of 84 matched to 103. Cardiomyopathy: 56 of 63 matched to 100; Differences are treated minus external, pp (95% CI). Panel y-axes differ.

WHAT AN INFUSION INVOLVES

1 Before

Oral prednisone and antihistamines to help prevent reactions

2 During

IV infusion, about 60 minutes

3 After

Approximately 2 hours of observation by the care team following infusion

Generally well-tolerated

Side effects were balanced between groups and mostly mild, short-lived and manageable.

Mild-to-moderate allergic-type (hypersensitivity) reactions; pre-medication with high-dose oral steroids and H1/H2 blockers largely reduces them.

No known long-term safety concerns

Long-term follow-up of up to ~60 months in HOPE-2 OLE and 24 months in HOPE-3 OLE.



HOPE-3: Robustness & Sensitivity Analyses

Nathaniel Hogan, Ph.D.

Biostatistics, Capricor Therapeutics

600+ robustness analyses

Integrated HOPE-2 & HOPE-3 efficacy (ISE)

Within-patient comparisons

Two Additional Deliverables Provided to the FDA

Robustness Report (HOPE-3)

- Aim to address FDA concerns regarding fragility
- Aim to address issues related to analysis plan choices
- Illustrates a robust primary result in the HOPE-3 trial

Integrated Summary of Efficacy (HOPE-2 & HOPE-3)

- Efficacy results of combined HOPE-2 and HOPE-3 data
- Further confirms lack of fragility of Deramiocele to treat DMD

HOPE-3: Robustness of the Primary Endpoint

600+ analyses

performed to test the robustness of the primary result

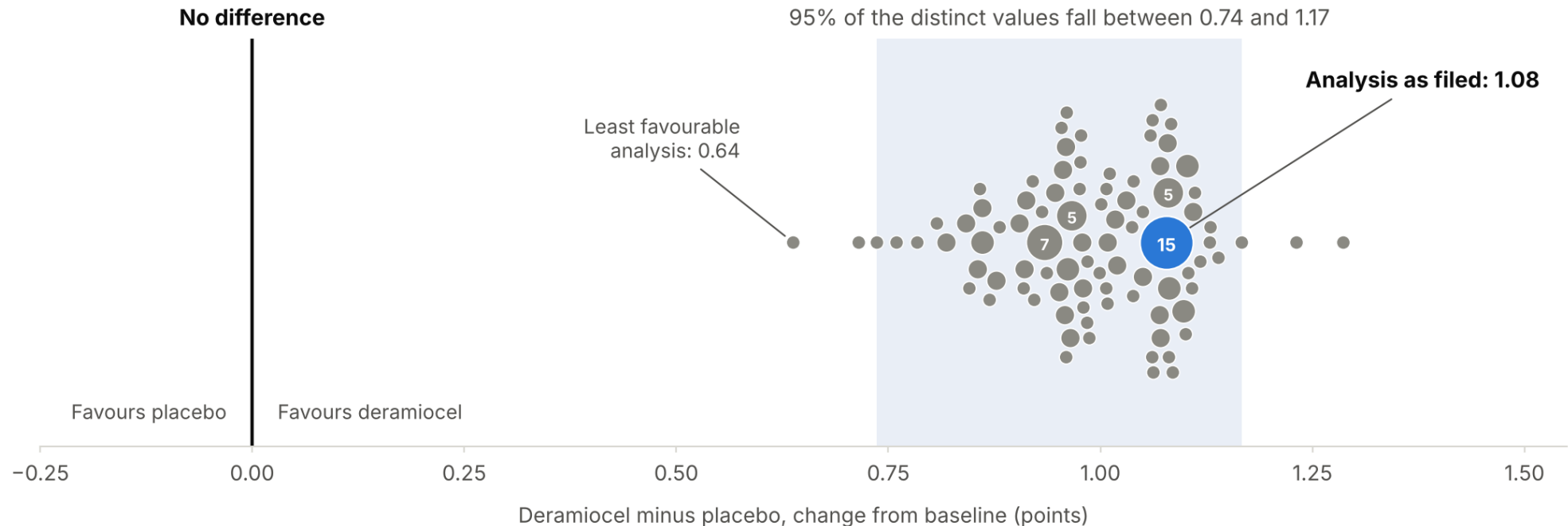
~1.0 point benefit

consistent treatment effect across analyses

Real effect

Shows lack of fragility: every analysis favored Deramiocele

B Absolute change: 89 distinct values from 153 analyses



Each marker is one analysis result; marker size reflects the number of analyses giving that treatment estimate. Shaded band: 95% of distinct values.

Integrated Summary of Efficacy (ISE)

HOPE-2 and HOPE-3 combined into one integrated analysis

Both placebo-controlled trials used the same dosing, administration and assessments

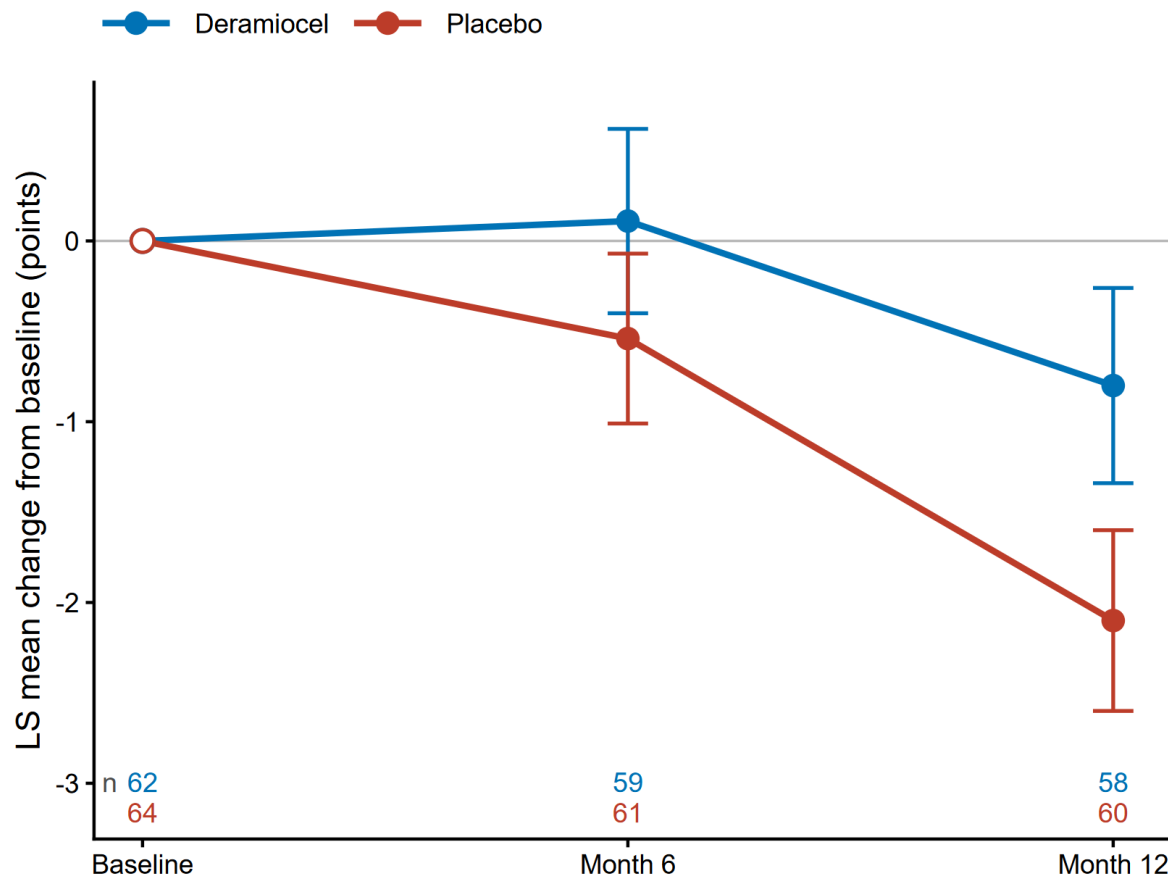


PARTICIPANTS CONTRIBUTING TO EACH ENDPOINT

Study	PUL 2.0 Total Score, MMRM		LVEF, ANCOVA		LVEF, cardiomyopathy at baseline	
	Deramioceol	Placebo	Deramioceol	Placebo	Deramioceol	Placebo
HOPE-2	8	12	6	6	3	3
HOPE-3	54	52	39	44	32	32
Total	62	64	45	50	35	35

ISE: PUL 2.0 Total Score at Month 12

PUL 2.0 Total, LS mean change from baseline (points, $\pm$ SE)



Month 12 treatment difference

Deramiciel vs placebo, pooled HOPE-2 & HOPE-3

ABSOLUTE CHANGE

1.29 PUL points

$p = 0.0087$ · 95% CI 0.33 to 2.25

PERCENT CHANGE

5.27 percentage points

$p = 0.0051$ · 95% CI 1.61 to 8.93

ISE: PUL 2.0 at Month 12, Sensitivity Analyses

Sensitivity analyses aimed to address AdCom concerns

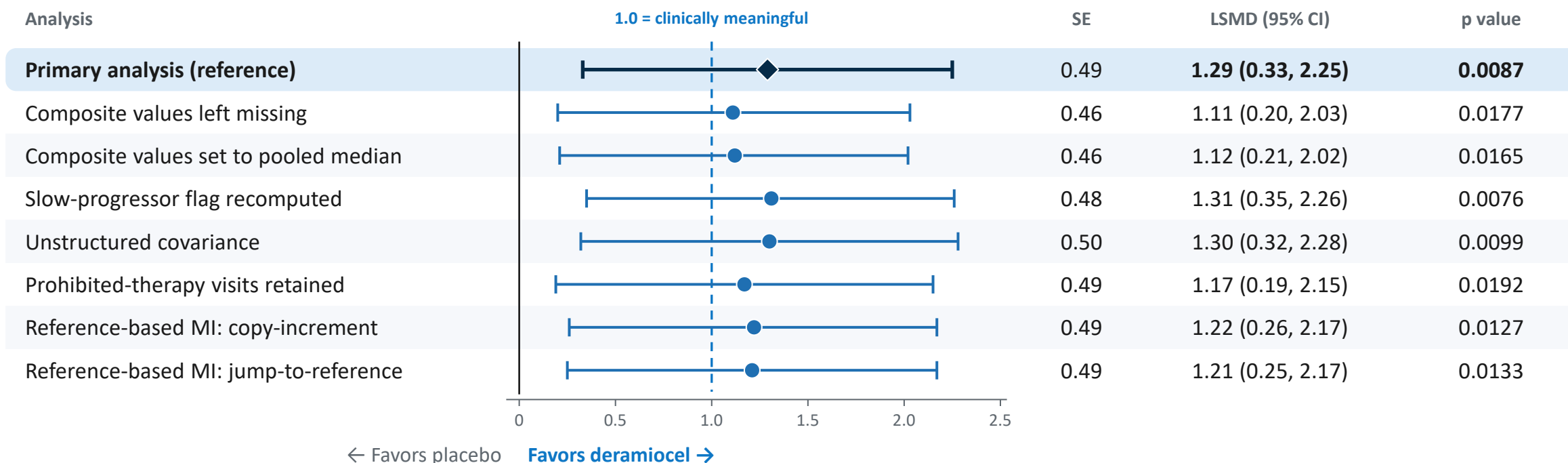
1.11–1.31

point estimate range
across all 7 analyses
(primary 1.29)

8 of 8

95% confidence intervals
exclude zero

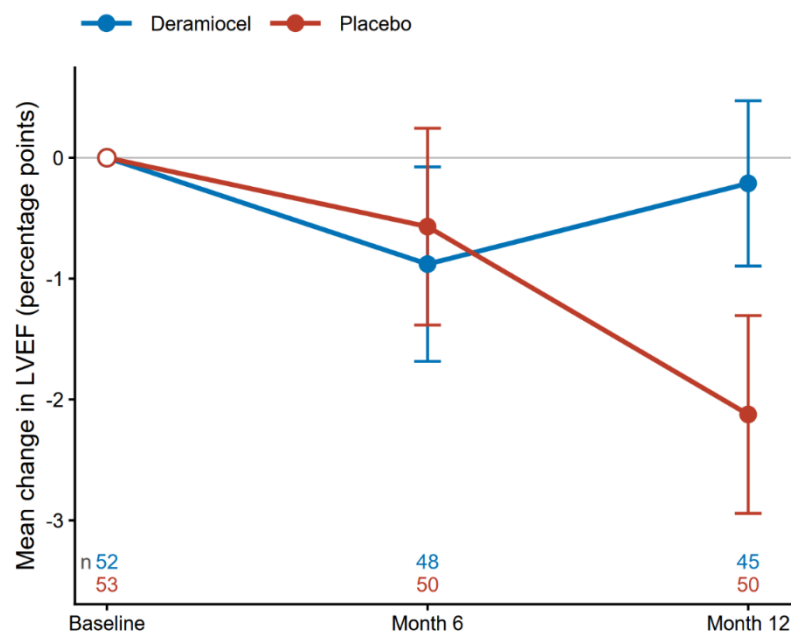
p < 0.02 for every analysis



ISE: LVEF at Month 12

ITT population

Mean change in LVEF (percentage points), $\pm$ SE



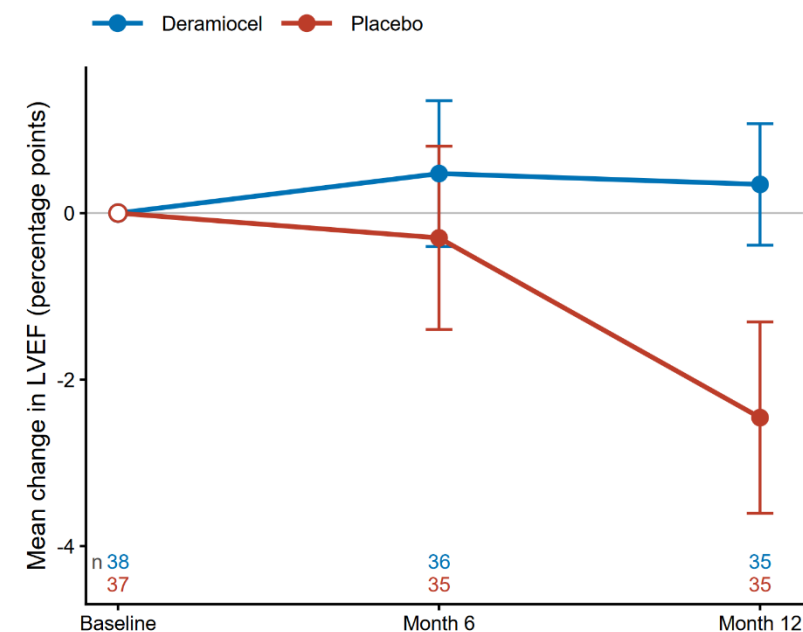
+2.21

percentage points

LS-mean difference at Month 12, Deramiciel vs placebo ($p = 0.05$)

Cardiomyopathy population

Mean change in LVEF (percentage points), $\pm$ SE



+3.13

percentage points

Observed difference at Month 12, Deramiciel vs placebo ($p = 0.04$)

The Deramiocele treatment effect is robust, not fragile

Robustness Report (HOPE-3)

- 600+ alternative analyses, same ~1-point PUL benefit
- Effect does not shift with analysis choices

Integrated Summary of Efficacy (ISE) (HOPE-2 & HOPE-3)

- More patients: 1.29-point PUL benefit, **p=0.0087**
- All 8 sensitivity analyses agree, every $p < 0.02$
- Heart function (LVEF) preserved vs placebo

We are grateful to the patients and families who took part in the HOPE studies

THEIR COMMITMENT MADE THIS RESEARCH POSSIBLE

Investigators and study teams

HOPE-3 investigators, coordinators and staff at 20 sites

Patient advocacy and scientific community

World Muscle Society (WMS)
Muscular Dystrophy Association (MDA)
Parent Project Muscular Dystrophy (PPMD)
CureDuchenne
Coalition Duchenne
Jett Foundation



HOPE-3 authors (The Lancet, 2026): Craig M McDonald, Chet Villa, Jonathan H Soslow, Kati Maharry, Nathaniel Hogan, Michael Binks, Kevin C Berth, Kristi A Elliott, Michael Taylor, Kan N Hor, James Signorovich, Erik K Henricson, Han C Phan, Susan Apkon, Partha S Ghosh, Cuixia Tian, Aravindhan Veerapandiyan, Leigh Ramos-Platt, Katheryn Gambetta, Saunder M Bernes, Arun S Varadhachary, Susan T Iannaccone, Chamindra G Lavery, Seth J Perlman, Nancy E Bass, Kathryn Mosher, Russell J Butterfield, Katherine D Mathews, Rebecca J Scharf, Edward C Smith, Jane Anne Emerson, Eugenio Mercuri, Mark S Awadalla, Linda Marbán*, Eduardo Marbán*, on behalf of the HOPE-3 Investigators



Q&A