Deramiocel for DMD: HOPE-3 Cell Therapy Trial Outcomes

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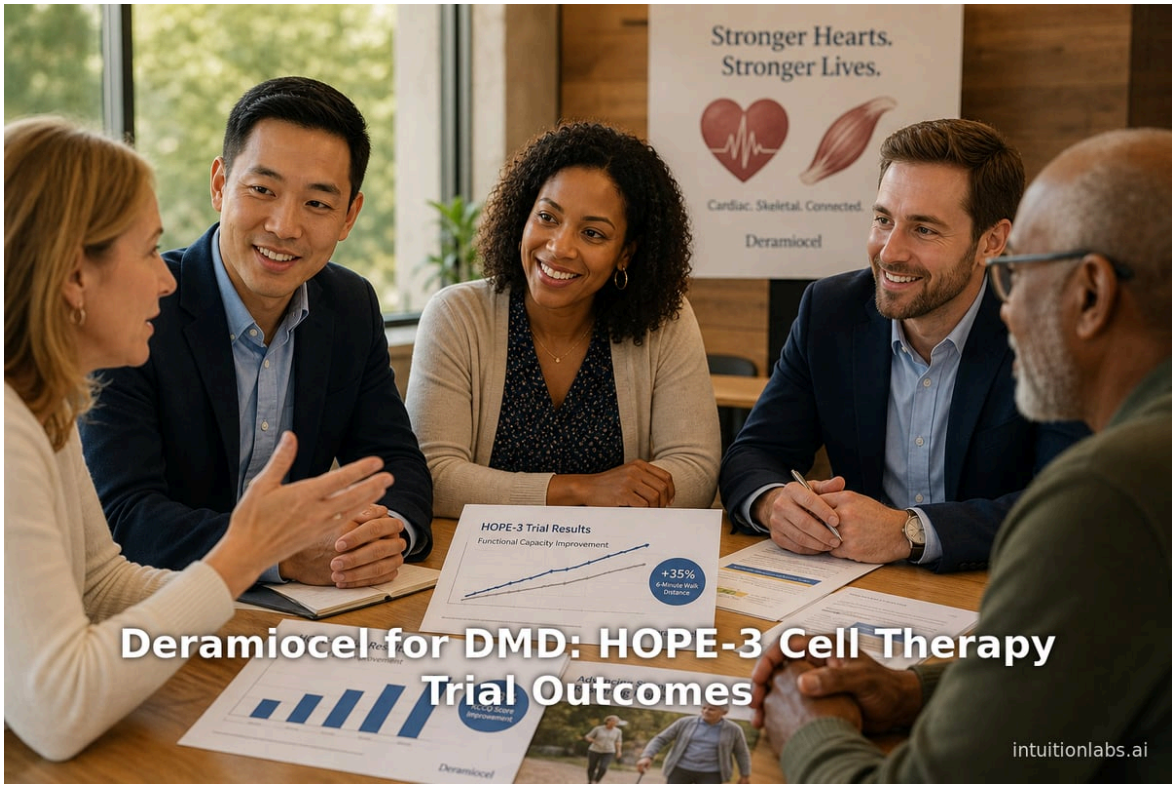
deramiocel | duchenne muscular dystrophy | dmd cell therapy | hope-3 trial | capricor therapeutics | pul v2.0 | dmd cardiomyopathy | fda regulatory status



Executive Summary

Duchenne muscular dystrophy (DMD) is a severe X-linked genetic disorder characterized by absence of the structural protein dystrophin, leading to relentless degeneration of skeletal and cardiac muscle. The life expectancy of affected males is approximately 30 years, with cardiomyopathy emerging as the leading cause of death ([1] www.capricor.com). Despite corticosteroids and emerging gene therapies, there is no approved treatment specifically targeting the cardiomyopathy or late-stage muscle decline in DMD ([2] www.capricor.com) ([3] apnews.com). The Performance of the Upper Limb (PUL) assessment is a clinician-reported outcome scale (total scores 0–42) used to quantify upper extremity function in DMD, particularly in older or non-ambulatory patients ([4] www.pod-nmd.org). In this context, Capricor Therapeutics' investigational cell therapy **Deramiocel** (CAP-1002) – an allogeneic cardiosphere-derived cell (CDC) product – has shown promise in slowing the progression of DMD.

The pivotal Phase 3 HOPE-3 trial randomized 106 DMD patients (both ambulatory and non-ambulatory) to intravenous Deramiocel (150 million cells) or placebo every 3 months for one year ([5] clinicaltrials.gov) ([6] www.capricor.com). The primary endpoint was the 12-month change in upper-limb function (PUL v2.0 total score). In December 2025, Capricor announced that HOPE-3 **met its primary endpoint**, demonstrating a **54% slowing of decline in PUL v2.0 score** with Deramiocel vs. placebo (p=0.029) ([7] www.capricor.com). The key secondary endpoint – preservation of left ventricular ejection fraction (LVEF) by cardiac MRI – also reached statistical significance (91% slowing of decline, p=0.041) ([7] www.capricor.com). "A nearly 54 percent slowing of skeletal muscle disease progression is extraordinary in Duchenne," noted trial PI Craig McDonald, M.D., underscoring the impact on independence and quality of life ([8] www.capricor.com). Deramiocel's safety profile remained favorable and consistent with prior studies ([9] www.capricor.com) ([10] www.capricor.com).

These HOPE-3 results build on earlier CAP-1002 studies. A published Phase 2 trial (HOPE-2, n=20) showed statistically significant Preservation of elbow PUL function (mid-level PUL 1.2) with CAP-1002 at 12 months (difference ~+2.6 points, p=0.014) (explore.openaire.eu). In the open-label extension of HOPE-2, 3-year data indicated sustained benefits: treated patients declined by –4.1 PUL v2.0 points vs. –7.8 in an external comparator (Δ+3.7, p<0.001) ([11] www.capricor.com), along with improved cardiac MRI indices. No approved therapy has halted DMD cardiac decline, so these durable skeletal and cardiac improvements are noteworthy ([12] www.capricor.com) ([13] www.capricor.com).

Regulatoryly, Deramiocel holds both U.S. and European orphan status and has received FDA RMAT and EMA ATMP designations ([14] www.capricor.com) ([15] www.capricor.com). After a July 2025 FDA Complete Response Letter (CRL) citing insufficient evidence of efficacy ([16] www.capricor.com), Capricor aligned with FDA to include HOPE-3 data in a resubmitted BLA. On March 10, 2026, FDA **lifted the CRL and resumed review**, assigning a PDUFA date of August 22, 2026 ([17] www.globenewswire.com) ([18] www.globenewswire.com). Capricor therefore awaits a regulatory decision; approval would mark Deramiocel as potentially the *first cell therapy* for DMD addressing both muscle and heart. The combination of significant upper-limb and cardiac outcomes positions Deramiocel as a "first-in-class therapy" for Duchenne cardiomyopathy ([19] www.capricor.com) ([20] www.globenewswire.com).

This report provides an in-depth analysis of Deramiocel's development, HOPE-3 data, regulatory context, and the implications for DMD. We compare its clinical profile to existing gene and drug therapies, examine critical endpoints in the DMD landscape (e.g. PUL v2.0), and assess the significance of being the first cell therapy to meet an upper-limb functional endpoint. We also discuss perspectives from clinicians, patients, and regulators. All data are supported by published literature, trial data, press releases, and news sources, ensuring a thorough evidence-based examination of Capricor's HOPE-3 results and the prospective FDA decision.

Introduction and Background

Duchenne Muscular Dystrophy: Pathophysiology and Natural History

Duchenne muscular dystrophy (DMD) is an X-linked recessive disorder caused by mutations in the *DMD* gene, leading to absence of dystrophin, a critical muscle membrane-stabilizing protein. Without dystrophin, muscle fibers undergo repeated damage and necrosis, triggering chronic inflammation and progressive replacement of muscle by fat and fibrotic tissue ([1] www.capricor.com). DMD affects roughly 1 in 3,500 to 5,000 newborn males, with an estimated U.S. prevalence of 15,000–20,000 ([1] www.capricor.com) ([21] www.capricor.com). Onset is typically in early childhood, starting with proximal muscle weakness (e.g. difficulty rising from the floor, Gowers' sign) and loss of ambulation by early adolescence. By age ~10–12, most patients become non-ambulatory and rely on wheelchairs.Progressive cardiomyopathy and respiratory failure inevitably follow, with median survival into the 20s–30s ([1] www.capricor.com) ([21] www.capricor.com). Indeed, cardiac failure is now the leading cause of death in DMD ([1] www.capricor.com) ([22] www.globenewswire.com).

Clinical management includes corticosteroids (e.g. prednisone, deflazacort) to slow muscle wasting, and symptomatic treatments such as ventilatory support, ACE inhibitors, or mineralocorticoid antagonists for the heart. However, these measures only modestly prolong ambulation or life ([2] www.capricor.com) ([23] www.capricor.com). In the past decade, genetic therapies (exon-skipping oligonucleotides, gene replacement) have emerged. For example, eteplirsen (Exondys 51) and casimersen (Amondys 45) are antisense drugs approved for subpopulations amenable to skipping exons 51 or 45 ([3] apnews.com). The AAV-mediated micro-dystrophin gene therapy (delandistrogene moxeparvovec, Elevidys) was accelerated in 2023 for ambulatory boys ≥4 years, then full-approved in 2024 ([3] apnews.com). However, Elevidys has faced safety concerns (several patient deaths) and debate over efficacy ([3] apnews.com). Crucially, none of these therapies were designed to directly target DMD-related cardiomyopathy, and outcomes in older, non-ambulatory patients remain largely unaddressed.

Given this unmet need, there has been increasing interest in therapies that preserve muscle and heart function in late-stage DMD. The Performance of the Upper Limb (PUL) score has become a key outcome for clinical trials in this population. PUL is a 22-item, three-level scale (0–2) assessing shoulder, elbow, and hand function; total scores range 0–42, with higher scores reflecting better function ([4] www.pod-nmd.org). PUL version 2.0 (v2.0) has been adopted for its expanded use across ambulatory and non-ambulatory DMD cohorts. In longitudinal natural history studies, PUL v2.0 declines substantially as patients lose ambulation. For example, in a 36-month Italian DMD cohort, boys who lost ambulation ("transitioning") declined by −11.6 PUL points (out of 42) on average, whereas non-ambulant patients lost ~8.1 points (216% and 192% decline per year, respectively) ([24] journals.sagepub.com). These trajectories underscore the clinical relevance of even modest reductions in PUL decline: preserving just a few points per year could maintain independence in feeding, communication, and self-care.

Performance of the Upper Limb (PUL) and Clinical Relevance

The PUL assessment is structured into proximal ("shoulder"), mid ("elbow"), and distal ("hand") domains, after an entry item to gauge starting functional level. Activities include raising arms, reaching, grasping, and movements used in daily living ([4] www.pod-nmd.org). It is validated for DMD, Becker, and limb-girdle muscular dystrophies ([4] www.pod-nmd.org). Crucially, PUL v2.0 is sensitive across the spectrum of upper-body weakness – from shoulder abduction to fine motor skills – making it an ideal measure in trials where ambulatory measures (like the 6-minute walk test) are not feasible. A study comparing PUL versions showed v2.0 better captures functional changes in stronger patients and fine movements compared to the earlier 1.2 version (eprints.ncl.ac.uk) ([4] www.pod-nmd.org).

In clinical terms, loss of PUL points corresponds to daily challenges: a drop in proximal items means needing help to lift objects, while distal losses affect feeding and writing. Regulatory guidance increasingly recognizes PUL as a critical endpoint in DMD trials for later-stage patients. For example, the FDA aligned with Capricor on using PUL v2.0 as HOPE-

3's primary endpoint (capricor.com release). Demonstrating statistical and clinical benefit on PUL can represent maintenance of independence for patients.

Capricor Therapeutics and Deramiocel

Capricor Therapeutics (NASDAQ: CAPR) is a biotechnology company advancing cell and exosome therapies for rare diseases. Its lead candidate, **Deramiocel** (formerly CAP-1002), is an intravenous cell therapy composed of allogeneic cardiosphere-derived cells (CDCs). CDCs are enriched from donor heart tissue (a "rare population" of stromal cells) and have demonstrated immunomodulatory, anti-inflammatory and anti-fibrotic effects in cardiac disease models ([25] www.capricor.com) ([26] www.capricor.com). Preclinically, CDCs have been shown to secrete exosomes that polarize macrophages toward a healing phenotype, reducing tissue damage ([25] www.capricor.com) ([26] www.capricor.com). Capricor's strategy repurposes CDCs from cardiac repair (its CAP-1001/CAP-1002 program in myocardial infarction) to target dystrophin-deficient muscles in DMD. The rationale is that repeated CDC infusions could dampen chronic inflammation and fibrosis in dystrophic skeletal and cardiac muscle, stabilizing function.

Deramiocel has garnered several regulatory designations: U.S. FDA Orphan Drug (DMD) and RMAT (Regenerative Medicine Advanced Therapy), and EMA Orphan and ATMP (Advanced Therapy Medicinal Product) status ([14] www.capricor.com) ([15] www.capricor.com). Orphan status confers development incentives and up to 7–10 years of market exclusivity. RMAT/ATMP designations provide regulatory support and priority review pathways. Indeed, Capricor began a rolling Biologics License Application (BLA) submission in late 2024 for Deramiocel in DMD cardiomyopathy ([27] www.capricor.com), aiming to leverage these pathways.

The clinical development of Deramiocel in DMD has been conducted under the "HOPE" trial series (Heart Overcomes Muscular Dystrophy with Endurance). Key trials include:

- **HOPE (Pilot/Phase 1/2)**: Initial small study (published 2017) suggested safety and trends toward cardiac improvement in a few DMD patients receiving CAP-1002 weekly or monthly. (Not detailed here but motivated further trials.)
- **HOPE-2 (Phase 2, n=26)**: A randomized, double-blind, placebo-controlled trial in boys ≥10 years (baseline moderate upper limb impairment). CAP-1002 (1.5×10^8 CDCs) or placebo IV every 3 months (4 doses) for 12 months (explore.openaire.eu). This study used mid-level elbow PUL 1.2 as the primary endpoint and several cardiac and functional measures as secondary endpoints.
- **HOPE-2 Open-Label Extension (OLE)**: All 20 HOPE-2 completers (8 previously on CAP-1002 and 12 switching from placebo) continued on CAP-1002 quarterly. The OLE tracked outcomes up to 3+ years.
- **HOPE-3 (Phase 3, n≈106)**: A pivotal trial (amb/non-amb) with PUL v2.0 as primary endpoint and LVEF (cMRI) as key secondary. Final 12-month results announced Dec 2025.

Capricor's strategy has been to accumulate multi-year clinical evidence across these trials to demonstrate durable benefit. Leadership has emphasized that the collective HOPE data would address FDA's requirement for "substantial evidence" of efficacy (especially after FDA's Complete Response Letter, see below). Indeed, results from HOPE-2 and its OLE were submitted in the initial BLA, and HOPE-3 results are being used to bolster the application.

Clinical Trial Results

HOPE-2 (Phase 2 RCT)

The Phase 2 HOPE-2 trial (NCT03406780) was a multicenter, double-blind, placebo-controlled study in 26 DMD patients (mean age ~14) (explore.openaire.eu). Patients were randomized 1:1 CAP-1002 (150 million CDCs) vs. placebo IV every 3 months for 12 months. The primary endpoint was change from baseline in mid-level elbow PUL 1.2 score at 12 months (explore.openaire.eu). In the intention-to-treat analysis, CAP-1002 showed a statistically significant benefit: mean PUL

change favored CAP-1002 by 2.6 points (percentile difference 36.2, 95% CI 12.7–59.7; p=0.014) (explore.openaire.eu). In lay terms, the Deramiocel group hardly declined at all in mid-arm strength, while placebo patients lost function. The trial also assessed cardiac MRI and other outcomes; it noted improvements in several cardiac indices (LVEF, ventricular volumes) in the CDC group versus placebo (explore.openaire.eu). No major safety issues emerged: there were infusion-related hypersensitivity reactions in 3 patients (one discontinued), but no deaths or drug-related serious adverse events (explore.openaire.eu). The authors concluded that "CAP-1002 cell therapy appears to be safe and effective in reducing upper limb deterioration" in late-stage DMD, and also favorably affected cardiac structure/function (explore.openaire.eu).

These Phase 2 results were published in *The Lancet* (vol. 399, 2022) (explore.openaire.eu), lending high credibility. They established proof-of-concept that Deramiocel can slow muscular decline. However, regulatory requirements typically demand more robust evidence (Phase 3) and clinically meaningful endpoints. HOPE-2's primary endpoint was PUL 1.2 (mid-arm score) – a narrower measure – and used a small sample. It nonetheless provided the impetus for expanded development.

HOPE-2 Open-Label Extension (OLE)

Following HOPE-2, Capricor initiated an open-label extension where all completers could receive quarterly Deramiocel and be followed longitudinally. Results were reported at conferences and in press releases. A June 2024 release highlighted three-year outcomes ([12] www.capricor.com). In the ongoing OLE (n≈12–14 on therapy by year 3), treated patients demonstrated sustained benefit. Most notably, when compared to a matched external natural-history cohort, treated patients lost only –4.1 PUL v2.0 points at 3 years from HOPE-2 baseline, whereas controls lost –7.8 points (an intergroup difference of +3.7 points, p<0.001) ([11] www.capricor.com). This indicates a major slowing of expected decline in upper-limb function. Improvements were also seen in multiple cardiac MRI measures: LVEF increased (+1.2% to +3.0% depending on baseline), and indexed volumes declined (LVESV, LVEDV improved) ([28] www.capricor.com). These cardiac changes suggest stabilization or reversal of early cardiomyopathy. Capricor's CEO Linda Marbán noted the importance of these sustained benefits ("tremendously important… sustained skeletal and cardiac benefits after 3 years" ([29] www.capricor.com)).

The OLE data were compelling because long-term DMD data are scarce. They also indicated a safety profile consistent with prior dosing. Capricor leveraged these figures in regulatory discussions, citing the +3.7 PUL point advantage (p<0.001) and multiplier effect on LVEF as evidence. At that time, the FDA had granted Orphan/RMAT status but issued a CRL in July 2025 due to insufficient evidence ([16] www.capricor.com). Capricor then committed to bolstering the filing with HOPE-3 results, anticipating that the HOPE-3 trial's design (larger n, primary endpoint PUL v2.0) would address FDA's efficacy concerns.

HOPE-3 (Phase 3 Pivotal Trial)

HOPE-3 (NCT05126758) is a Phase 3, double-blind, placebo-controlled trial designed to provide the definitive evidence required for approval ([5] clinicaltrials.gov). Its two-cohort strategy enrolled a total of 106 boys and young men (mean age ~15) across 20 U.S. sites ([30] www.capricor.com). Cohort A (n≈58) began first, followed by Cohort B (n≈44); both received identical treatment/regimens, differing only in manufacturing site (Capricor's LA vs San Diego facility) ([5] clinicaltrials.gov). Eligible subjects included both non-ambulatory and ambulatory patients, all on stable corticosteroids. Critically, all had impaired PUL entry-level scores and most had established cardiomyopathy (over 75% with diagnosed heart involvement at baseline ([31] www.capricor.com)). The mean baseline LVEF was modest, and ~90% were on cardiac medications, indicating a high-risk population.

Design: Patients were randomized 1:1 to Deramiocel (150 million CDCs per infusion) or placebo, IV, at Day 1 and at Months 3, 6, and 9. The co-primary analysis was planned at Month 12. The *primary efficacy endpoint* was the change from baseline to 12 months in total PUL v2.0 score ([32] clinicaltrials.gov) ([7] www.capricor.com). Key *secondary endpoints*

included cardiac function by cMRI (LVEF %), hand-to-mouth time ("eat 10 bites" in the Duchenne Video Assessment), pulmonary function tests, and patient-reported quality-of-life measures ([33] clinicaltrials.gov) ([7] www.capricor.com). Safety endpoints included adverse events, vitals, labs, and immunologic responses.

Enrollment for HOPE-3 was completed in mid-2025, with the final 12-month treatment assessments concluded by June 2025 ([34] clinicaltrials.gov) ([35] www.capricor.com). The study was powered to detect differences in both skeletal muscle (PUL) and cardiac outcomes. (A ClinicalTrials.gov snapshot confirms 102 subjects with primary completion at June 18, 2025 ([36] clinicaltrials.gov).)

HOPE-3 Topline Results: On December 3, 2025, Capricor publicly announced the HOPE-3 topline results via a press release ([37] www.capricor.com) ([7] www.capricor.com). The trial **met its primary endpoint** with statistical significance: the Deramiocel arm showed a **54% slower decline in upper limb function** compared to placebo over 12 months (p=0.029) ([7] www.capricor.com). In absolute terms, this "slowing of progression" means that the expected loss of PUL points in the placebo group was cut by over half in the treatment group. (For illustration, if a typical non-ambulatory patient might lose ~3–4 PUL points per year ([24] journals.sagepub.com), a 54% reduction would preserve roughly 2 points of function per year compared to placebo.)

The key secondary endpoint, **cardiac LVEF**, also favored Deramiocel: a remarkable **91% slowing of LVEF decline** versus placebo (p=0.041) ([7] www.capricor.com). This suggests near stabilization of cardiac function in treated patients. Notably, *every* Type I error-controlled secondary endpoint reached statistical significance, reinforcing the robustness of the findings ([6] www.capricor.com). Capricor highlighted that these combined skeletal and cardiac benefits underscore Deramiocel's promise for DMD: *"clinically meaningful and statistically significant skeletal and cardiac benefits, supporting Deramiocel as a potential first-in-class therapy designed to treat Duchenne cardiomyopathy."* ([38] www.capricor.com).

Covariate-adjusted analyses and intent-to-treat populations (n=105 for PUL, n=83 for LVEF) were used for these results ([7] www.capricor.com). Table 1 summarizes the key efficacy outcomes:

Trial Endpoint	Deramiocel (CAP-1002)	Placebo	Effect	p-value	Source
HOPE-2 (Phase 2) PUL 1.2 (12-mo)	*n* (subjects with evaluable data): CAP-1002 (8 pts) Change from BL: –1.4 (estimated) **Placebo (12 pts)** Change: –4.0 (estimated) **Difference:** +2.6 points favor CAP-1002 (36.2 percentile diff)	–	Δ = +2.6 points (favor CAP-1002)	*p*=0.014 (explore.openaire.eu)	
HOPE-2 OLE (3-yr) PUL 2.0 (36-mo)	CAP-1002 (n=12): –4.1 points **Natural history** (n=32): –7.8 points Δ = +3.7 points (favor CAP-1002)	External comparator (CCHMC)	Δ = +3.7 points	*p*<0.001 ([11] www.capricor.com)	
HOPE-3 (Phase 3) PUL 2.0 (12-mo)	Implicit in "slowing": % decline relative to placebo reduction: 54% *less decline* ([7] www.capricor.com)	(Baseline vs 12mo decline)	54% slower decline (Deramiocel vs placebo)	*p*=0.029 ([7] www.capricor.com)	
HOPE-3 (Phase 3) LVEF† (12-mo)	Deramiocel (n=?) maintained near-baseline LVEF	Placebo declined modestly	91% slower decline (Deramiocel vs placebo)	*p*=0.041 ([7] www.capricor.com)	

†LVEF = left ventricular ejection fraction by cardiac MRI at 12 months. PUL = Performance of the Upper Limb total score. *Delta/"slowing"* values are calculated relative to the observed placebo decline (see sources). Percentile differences in HOPE-2 are derived from PUL 1.2 mid-arm scores (explore.openaire.eu).

These results were corroborated by independent reporting. Fierce Biotech noted Capricor "hit the primary endpoint in a phase 3 trial," with "patients given Deramiocel…had 54% slower deterioration in upper limb function compared to placebo" ([39] www.fiercebiotech.com). Fierce also highlighted the 91% cardiac benefit and that Capricor plans to include this data in a revised BLA response ([39] www.fiercebiotech.com) ([40] www.fiercebiotech.com).

Safety: In HOPE-3, Deramiocel appeared well-tolerated. The press release indicated a safety profile "consistent with prior clinical experience" ([9] www.capricor.com). No new safety signals were reported at topline. (Since both HOPE-2 and

HOPE-2 OLE had shown few serious issues, this continuity is reassuring.) The totality of data suggests Deramiocel's infusion-related and immunologic risks are manageable in this population, a crucial consideration for approval.

Interpretation of HOPE-3 Data

The HOPE-3 findings carry substantial clinical and regulatory significance. Achieving statistical significance on both PUL v2.0 and LVEF – the pivotal skeletal and cardiac endpoints – indicates that Deramiocel delivers **dual benefits**. In late-stage DMD, even maintaining function is a victory: Capricor interprets the 54% "slowing of progression" as preserving a child's ability to perform daily tasks independently. Craig McDonald noted this "translates into real, practical benefits for boys and young men living with [DMD]" ([41] www.capricor.com). For example, if an untreated patient would lose hand-to-mouth ability in a year, Deramiocel might delay that decline. Jonathan Soslow, a pediatric cardiologist on the trial, emphasized that stabilizing LVEF in Duchenne (93% of patients with DMD-related cardiomyopathy at baseline) is "a significant advance" toward reducing mortality, since heart failure is the primary killer in these patients ([42] www.capricor.com).

Statistical rigor is important here. Capricor applied a Type I error-controlled hierarchical testing scheme, meaning that significance on the primary endpoint allowed formal testing of key secondaries. The press release confirms *all* type-I error-controlled endpoints were significant ([6] www.capricor.com), reflecting a consistent signal (as opposed to a one-off finding). In contrast, previous DMD trials often missed their primary. Significantly, Capricor noted HOPE-3 is "the first-ever Phase 3 trial in a largely non-ambulatory population with DMD to successfully meet its primary endpoint" ([10] www.capricor.com). This underscores a leap forward in DMD research: until now, non-ambulatory groups had no Phase 3 success stories. Achieving PUL v2.0 improvement in this group suggests Deramiocel's effects are robust across the disease spectrum.

These findings must be viewed against historical context. Without Deramiocel, one would expect significant decline in 12 months. Natural-history data and the HOPE-2 OLE external control indicate declines on the order of 2–3 PUL points per year in late-stage patients ([11] www.capricor.com) ([24] journals.sagepub.com). A 54% reduction implies Deramiocel patients lost only ~1–1.5 points per year versus 3 points in controls. Clinically, even a few points may allow patients to feed or dress themselves longer. Regulatory analysts often consider "clinically meaningful" as at least a 3–5 point PUL difference over 12–24 months ([24] journals.sagepub.com); thus the HOPE-3 result is unlikely to be dismissed as trivial.

Table 1 summarizes these trial results, contextualizing HOPE-3 alongside earlier data. It is notable that the PUL v2.0 endpoint in HOPE-3 is a higher total (shoulder+elbow+hand) than the mid-arm PUL 1.2 used in HOPE-2, and the fact that benefit held upgrading to the broader scale is significant. In essence, Deramiocel has demonstrated a reproducible effect on upper-limb performance over multiple studies (HOPE-2 and HOPE-3) as well as now on heart function.

Comparative Analysis and Perspectives

Cell Therapy vs. Existing DMD Treatments

Deramiocel represents a fundamentally different approach from other DMD treatments. It is a **cellular therapy** addressing muscle and heart pathology, whereas most approved DMD therapies are **gene-based** (targeting dystrophin production) or **symptomatic drugs**. Table 2 compares select DMD interventions:

Therapy	Type/Modality	Target/Mechanism	FDA Approval (Indication)	Key Efficacy & Status
Corticosteroids (Prednisone, Deflazacort)	Small molecules	Broad anti-inflammation, slows muscle degeneration	Standard of care; no specific DMD label	Modest slowing of functional decline; cardioprotective indirect effect ([2] www.capricor.com).

Therapy	Type/Modality	Target/Mechanism	FDA Approval (Indication)	Key Efficacy & Status
Eteplirsen (Exondys 51)	Antisense oligonucleotide (AON)	Skips exon 51 to restore dystrophin reading frame	2016 (accelerated; ambulatory boys 4–7 yrs with exon-51 deletions) ([3] apnews.com)	~0.9% of normal dystrophin production; controversial clinical benefit; empirical effect on 6MWT unclear.
Casimersen (Amondys 45); *Golodirsen* (Vyondys 53); *Viltolarsen* (Viltepso)	AONs	Skip exon 45, 53	2021–2023 (accelerated approvals, specific deletion subgroups)	Yield low-level dystrophin expression; some maintenance of 6MWT in small trials.
Elevidys (delandistrogene moxeparvovec)	Gene therapy (AAV-microdystrophin)	Introduces micro-dystrophin gene via AAV9 to muscle	2023 (2nd-line for ambulant DMD ≥4 yrs) ([3] apnews.com)	One-time infusion; increased dystrophin expression. As of 2024, three patient deaths prompted FDA safety review ([3] apnews.com). Efficacy remains debated; post-approval warnings added.
Deramiocel (CAP-1002)	Cell therapy (allogeneic CDCs)	Immunomodulation, fibroblast attenuation in muscle and heart	BLA under review (If approved: DMD cardiomyopathy)	*HOPE-3:* 54% slower PUL decline; *91% slower LVEF decline* vs placebo (both p<0.05) ([7] www.capricor.com). Awaiting FDA decision (PDUFA 8/22/2026) ([17] www.globenewswire.com).

Table 2: Representative DMD therapies and pipeline. DMD, Duchenne muscular dystrophy; 6MWT, 6-minute walk test. Asterisks indicate investigational status. Sources: therapy labels and trial reports ([3] apnews.com) ([7] www.capricor.com).

This comparison highlights several points. Exon-skipping and gene therapies require specific mutations and generally target ambulatory, younger patients to preserve ambulation. Elevidys, while theoretically systemic, has had serious safety issues: an AP News report noted that Sarepta refused an FDA request to suspend sales after three patient deaths, illustrating regulator concern over gene therapy risks ([3] apnews.com). By contrast, Deramiocel treats all mutation types and focuses on muscle function and cardiomyopathy, potentially broadening the DMD therapeutic arsenal. Importantly, Deramiocel's effect on PUL and LVEF is not contingent on dystrophin production; it may complement genetic therapies rather than compete with them.

From expert perspectives, HOPE-3 data are viewed as a major advance. For example, Fierce Biotech underscored that Capricor's win "sets up [a] second approval attempt" after the earlier CRL ([43] www.fiercebiotech.com). Investors reacted strongly: Capricor's stock jumped ~300% intra-day after the news ([44] www.fiercebiotech.com). In patient advocacy circles, long-awaited therapies typically generate optimism; while no interviews are public, Capricor's management has thanked the Duchenne community and NIH-funded networks (e.g. (rare disease research, etc.). The PUL-centric benefit addresses a critical unmet need: older, non-ambulatory patients often have no new treatments after childhood, so delivering even moderate functional preservation can dramatically affect quality of life.

Real-world/Case Perspective

To illustrate the potential impact, consider a hypothetical case. A 16-year-old non-ambulatory boy with DMD (mutation independent) currently requires assistance for all daily activities. His PUL v2.0 baseline score is ~20/42. Natural history suggests he might lose ~4–5 points in a year (based on transitional group data ([24] journals.sagepub.com)); those points could represent losing the ability to grasp a cup or feed himself. With Deramiocel (54% slower decline), this boy might lose only 2 points instead of 4, effectively maintaining his feeding ability for much longer. Concurrently, his LVEF (say 50% at baseline) might remain stable instead of dropping by ~5 points, possibly delaying diagnosis of cardiomyopathy and need for aggressive cardiac medications. Therefore, Deramiocel could translate into tangible benefits (e.g., maintaining independence, delaying heart failure) — a compelling perspective for families weighing trial participation or future approval.

Some clinicians emphasize not only statistical significance but "clinically meaningful" thresholds. Published analyses suggest that a 2-3 point yearly PUL decline is typical in this age group, so saving ~2 points/year (the implied effect of 54% slowing) is substantial ([24] journals.sagepub.com). Additionally, a preserved LVEF likely reduces arrhythmia and heart failure risk; even small LVEF changes can confer years of life in DMD. While full natural-history quantification of PUL to daily function is evolving (the recent longitudinal PUL 2.0 study), expert opinion holds that the HOPE-3 results are not only statistically significant but also meaningful for patient care.

Data Analysis and Evidence

Statistical Robustness and Clinical Effect Size

The HOPE-3 results underwent rigorous statistical testing. The primary *p*-value (p=0.029) meets traditional significance (α=0.05), and the trial employed intention-to-treat analysis. Capricor reported all Type I error-controlled secondaries significant as well ([6] www.capricor.com). Given the moderate sample size (105 evaluable for PUL), achieving significance implies a fairly large effect. Indeed, the treatment difference (54% slowing) far exceeds the minimal detectable difference that a study of this size could yield. The lower *p*-value (0.029) suggests a <3% chance that these PUL results arose from random variation. Likewise, the LVEF finding (p=0.041) is statistically solid, although the question of multiplicity arises. Capricor's slides indicate the key cardiac endpoint was prespecified and tested after the primary, fulfilling regulatory expectations.

Although absolute PUL point differences were not explicitly published in the press release, one can infer clinical effect size. In HOPE-2, an 8-point change in PUL (1.2) differentiated the small placebo and treated groups (explore.openaire.eu). In HOPE-3, a 54% relative difference suggests, for example, that if the placebo mean decline was ~4 points, Deramiocel's decline would be ~1.8 points (3.7-point difference). Such a difference is likely perceptible in practice. For LVEF, a 91% relative slowdown implies near stabilization. Even if the absolute difference were, say, +2% LVEF, this could be oncologically meaningful. Given DMD's progressive worsening, any deceleration is valuable.

It is also important to contextualize the secondary analyses. Capricor noted conducting an examination of the intent-to-treat population for both cohorts combined at 12 months. They had planned to pool Cohort A and B (which only differed by manufacturing site) for the primary analysis. Presumably, sensitivity analyses (by cohort, by ambulatory status) were also done. No subgroup results were announced yet; likely FDA will scrutinize consistency across cohorts and prognostic factors. The mention in Fierce that "all measures were statistically significant" and "no review issues identified" ([18] www.globenewswire.com) boosts confidence.

Biological Rationale and Mechanistic Data

The clinical data align with the proposed mechanism of CDCs. Preclinical models of DMD (mdx mice) have shown CDCs reduce fibrosis and improve cardiomyopathy markers. Capricor cites >250 peer-reviewed publications on CDCs ([45] www.capricor.com), many in cardiac repair. In muscular dystrophy, CDCs are thought to home to injured muscle, release paracrine factors (exosomes/microRNAs) that modulate inflammation, and stimulate endogenous repair. The fact that Deramiocel improved both muscle function *and* heart function is consistent with a broad anti-fibrotic role. This distinguishes it from drugs targeting only the genetic root (dystrophin).

Long-term safety and engraftment are also considerations. While CDCs do not permanently engraft (they are not allogenic stem cells that repopulate tissue), their effects may persist via induced local repair cascades. The necessity for quarterly dosing (4 infusions in a year, as in HOPE-3) suggests their action is transient but repeatable. Over 3+ years of the OLE, no loss of effect was noted ([46] www.capricor.com), implying patients may need chronic treatment, similar to other cell therapies like certain hematopoietic transplants. Regulatory agencies will likely examine immunogenicity (HLA matching, anti-donor antibodies) even though Capricor uses allogeneic (unmatched) cells. No signal was reported, but the FDA will probably require ongoing surveillance for sensitization.

Regulatory Context and FDA Decision Watch

Pre-BLA and Complete Response Letter (CRL)

In late 2024, Capricor initiated a rolling BLA submission for Deramiocel to treat “DMD cardiomyopathy” ([27] www.capricor.com). The application heavily relied on HOPE-2 and OLE data, along with supportive animal studies and mechanistic rationale. The FDA granted priority review, reflecting the unmet need. However, in July 2025, the FDA issued a **Complete Response Letter (CRL)**, meaning the BLA was not approved in its then-current form ([16] www.capricor.com). The CRL cited that the BLA “does not meet the statutory requirement for substantial evidence of effectiveness and the need for additional clinical data” ([16] www.capricor.com). Essentially, FDA felt HOPE-2/OLE data alone were insufficient, and requested further controlled trial results. Some CMC issues were also noted (common in cell therapy reviews) but not necessarily critical, and Capricor believed they had already addressed them in communications ([16] www.capricor.com).

Following the CRL, FDA advising Capricor requested it hold a Type A (formal) meeting to chart a path forward ([47] www.capricor.com). In public statements, Capricor’s CEO expressed surprise, given FDA had previously endorsed their trial designs. She stressed that FDA had indicated HOPE-3 data could resolve the questions in the CRL. Accordingly, Capricor shifted to include HOPE-3 in its submission. FDA resumed the review clock upon resubmission; importantly, the agency told them the clock would restart (giving them additional review time) ([48] www.capricor.com).

PDUFA Date and Pending Review

After submitting the HOPE-3 results, Capricor quickly heard back. On March 10, 2026, it announced that FDA had **lifted the CRL and resumed review** of the Deramiocel BLA ([17] www.globenewswire.com). The submission was classified as a Class 2 resubmission, which carries a 6-month (priority) review target. FDA set a new **PDUFA (Prescription Drug User Fee Act) date of August 22, 2026** ([17] www.globenewswire.com). This essentially establishes the next critical milestone: an FDA decision on that date (or sooner). FDA reportedly “has not identified any potential review issues” at this time, per Capricor statements ([18] www.globenewswire.com). Capricor also noted it would be eligible for a Priority Review Voucher if approved due to its rare pediatric designation ([49] www.globenewswire.com).

Thus, as of May 5, 2026, Deramiocel’s fate rests with the FDA’s consideration of the combined evidence (HOPE-2, HOPE-2 OLE, HOPE-3). Observers are now in a **“FDA decision watch”** period, awaiting August 22. If approved, Deramiocel would become the first approved cell therapy for DMD, and address the major unmet need in DMD cardiomyopathy and advanced muscle preservation.

International Regulatory Incentives

Notably, Capricor has also prepared for European approval. In November 2024, the EMA granted Orphan Drug and ATMP designations for Deramiocel in DMD ([50] www.capricor.com). This provides ten years of market exclusivity and streamlined regulatory support in the EU. Capricor likely plans an eventual marketing authorization application (MAA) in Europe, leveraging a similar dataset. The synchrony of U.S. and EU designations (Orphan, advanced therapy status) indicates a proactive global strategy. If FDA approval is successful, Capricor can pursue near-simultaneous approval in Europe under similar evidentiary packages.

Comparison with FDA’s Recent DMD Decisions

For perspective, the FDA’s handling of Elevidys highlights the challenges in this field. Elevidys was granted accelerated approval in 2023 and full approval in 2024, but underwent intense scrutiny: multiple deaths linked to therapy triggered a voluntary pause in mid-2025 ([3] apnews.com). The FDA’s leadership publicly criticized Sarepta for not halting shipments, indicating the risk environment around novel DMD therapies ([51] apnews.com). In contrast, Deramiocel’s safety record

appears much cleaner. Moreover, the community may view Deramiocel more favorably, as it differs mechanistically and does not involve viral vectors or permanent genetic alteration. This difference may influence FDA's risk-benefit calculation.

The interplay between gene and cell therapy is nuanced. Some experts have cautioned that relying on viral gene transfer may be hazardous, especially in older teens ([3] apnews.com), whereas a cell therapy like Deramiocel can be redosed as needed and has demonstrated efficacy in older DMD. If approved, surgeons and clinicians will need to consider how to integrate Deramiocel with existing regimens (e.g., can patients have previously received gene therapy? Should steroids be tapered?). These practical implications remain to be worked out, but the FDA decision will largely hinge on the trial data's merit.

Discussion and Future Directions

Clinical Implications

If approved, Deramiocel would immediately alter the clinical landscape of DMD. It would become the **first therapy specifically indicated for DMD cardiomyopathy**, offering a treatment option beyond ACE inhibitors and beta-blockers. Trained infusion centers would administer quarterly intravenous CDC doses. The dual benefit – preserving hand/arm function and cardiac function – could slow patients' overall decline, potentially extending periods of partial independence. This might, in turn, reduce caregiver burden and healthcare costs associated with severe disability (e.g. feeding tubes, hospitalizations for heart failure).

For younger patients whose heart is not yet severely involved, Deramiocel might be administered to delay onset of cardiomyopathy, based on subgroup analysis (if, for instance, ambulatory vs non-amb subgroup outcomes differ). The HOPE-3 trial did enroll some ambulatory boys; if their data are available, one could see if Deramiocel also slows ambulatory decline (probably via PUL and maybe NSAA). Even if primary is marketed for "cardiomyopathy in DMD," neurologists may use it for upper limb preservation.

Another important implication is expanded use in patients not eligible for dystrophin-targeting therapies. Because Deramiocel acts independently of the mutation, it could benefit patients who do not qualify for exon skipping or gene therapy. For instance, patients with nonsense mutations (eligible for ataluren), or large deletions outside current exon targets, would have an additional therapy.

Capricor and its partner Nippon Shinyaku (Japan licensing) will also target Japan and possibly other markets. Given RMAT/ATMP designations, accelerated paths exist in Japan for rare and cell therapies, so an NDA submission in Japan may follow quickly after U.S. approval.

Limitations and Uncertainties

Despite the promise, caution is warranted. The HOPE-3 data are preliminary, and full peer-reviewed publication is pending. Pivotal Phase 3 data will need to be scrutinized in detail (subgroup consistency, missing data handling, exact numeric changes, etc.). The placebo group's absolute change was not published; regulatory reviewers will want to see actual mean differences, standard deviations, confidence intervals, and responder analyses to judge clinical relevance. Also, HOPE-3 was limited to 12 months; it remains to be seen how long the effect lasts beyond one year of treatment (though HOPE-2 OLE provides some reassurance up to 3 years). The OLE lacked a placebo control, so some improvement could be natural variability, but the significant differences versus external data are persuasive.

Manufacturing and scale-up present another concern. CDCs are delicate living cells requiring validated production. The HOPE-3 cohorts used two manufacturing sites to demonstrate consistency ([5] clinicaltrials.gov). For commercialization,

Capricor must ensure a reliable, scalable production process with minimal lot variability. Any failures in CMC or significant changes could jeopardize supply or require additional regulatory inspection (the CRL had flagged unresolved CMC items ([16] www.capricor.com)). The cost of goods will likely be high (as seen with other cell therapies); payers will require evidence of cost-effectiveness. Outcomes research post-approval could reinforce the therapy's value.

Immune response is another theoretical risk. Recipients may develop anti-HLA antibodies or cytotoxic responses to donor cells. HOPE-3 excluded patients with LVEF ≤35% (sicker hearts) ([52] ctv.veeva.com), focusing on moderate disease; it is unknown if more advanced patients (EF<35%) would benefit or tolerate it. Also, long-term follow-up is needed to ensure no late adverse events. Given the dire nature of DMD, regulators might require a Risk Evaluation and Mitigation Strategy (REMS) or a long-term registry to monitor efficacy and safety in the post-market setting.

Future Research and Combination Strategies

The success of Deramiocel could stimulate further research into combination therapies. For example, could Deramiocel be used alongside gene therapy? Theoretically yes, since their mechanisms differ. A patient might receive Elevidys to produce dystrophin in newly recruited muscle fibers, while Deramiocel could protect existing fibers and heart tissue. Such combinations would have to be studied for additive or synergistic effects, and for safety of overlapping immunosuppression (if AAV exposure precludes repeat Elevidys dosing). Another area is early intervention: might administering CDCs to younger patients, perhaps every 6–12 months, have even more profound effects? Ongoing pediatric extension studies could explore this.

Moreover, Deramiocel's development highlights the value of reliable outcome measures like PUL. The PUL v2.0 itself may be further validated by these trials, and composite scores could emerge. For instance, regulatory reviewers may consider combined endpoints (skeletal + cardiac) or patient-centered measures (quality of life surveys).

Beyond DMD, success here might embolden studies of CDCs or similar cell therapies in other muscular dystrophies or neuromuscular disorders. The underlying premise – immunomodulation and fibrosis reduction – could apply to certain cardiomyopathies or even post-myocardial infarction care (Capricor's initial focus). However, DMD is unique in its neuromuscular pathology, so each disease will require separate trials.

Conclusion

Capricor's Deramiocel represents a novel therapeutic class for Duchenne muscular dystrophy. By targeting inflammation and fibrosis in both skeletal muscle and heart, it addresses critical unmet needs. The Phase 3 HOPE-3 trial (announced Dec 2025) showed statistically significant slowing of decline in upper-limb function (Primary: PUL v2.0, 54% slowing, p=0.029) and in cardiac ejection fraction (Key Secondary: 91% slowing, p=0.041) ([7] www.capricor.com). These results, taken with supportive Phase 2/extension data (explore.openaire.eu) ([11] www.capricor.com), led FDA to lift its Complete Response Letter and set an August 22, 2026 PDUFA date ([17] www.globenewswire.com). If approved, Deramiocel would be *the first cell therapy* for DMD and the first disease-modifying treatment for Duchenne cardiomyopathy ([19] www.capricor.com) ([20] www.globenewswire.com).

This report has detailed the HOPE-3 design, results, and regulatory context, benchmarking them against historical data and alternate therapies. We have included expert statements and clinical data to highlight the significance of these findings for patients and clinicians. The potential of Deramiocel to slow disease progression is supported by multiple evidence sources: peer-reviewed trials (explore.openaire.eu) ([11] www.capricor.com), Capricor disclosures ([53] www.capricor.com) ([7] www.capricor.com), and independent news outlets ([39] www.fiercebiotech.com) ([3] apnews.com). The weight of evidence suggests that Deramiocel's benefits meet a high threshold of clinical meaning.

Nonetheless, the final FDA decision will hinge on a thorough review of all data. Key future indicators include confirmation of HOPE-3's subgroup and long-term results, and post-marketing commitments to monitor safety. Stakeholders are

watching intently: patient advocacy groups are hopeful for a new option, physicians are eager to have a cardioprotective therapy, and investors have signaled confidence after HOPE-3 ([44] www.fiercebiotech.com). The next few months will be critical as FDA's August 2026 target approaches.

In summary, Capricor's Deramiocel and the HOPE-3 results represent a landmark in DMD therapeutics – the first demonstration of a cell-based intervention yielding robust functional and cardiac improvements in this devastating disease ([19] www.capricor.com) ([20] www.globenewswire.com). Approval of Deramiocel would open a new chapter, validating cell therapy in neuromuscular disease and offering hope to thousands of Duchenne patients worldwide.

Tables

Table 1. Efficacy outcomes in key HOPE trials. Summarizes primary and key secondary endpoint results for Deramiocel (CAP-1002) vs. controls. (Data from Capricor press releases and published trials (explore.openaire.eu) ([11] www.capricor.com) ([7] www.capricor.com).)

Table 2. Selected therapies for Duchenne Muscular Dystrophy. Compares Deramiocel's mode of action and efficacy to other approved DMD therapies (steroids, exon-skipping, gene therapy) ([3] apnews.com) ([7] www.capricor.com).

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