



Deramiocele

**Parent
Project
Muscular
Dystrophy**

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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 discovery efforts and clinical trials; the pace of enrollment of clinical trials; plans regarding regulatory filings, future research and clinical trials; regulatory developments involving products, including the ability to obtain regulatory approvals or otherwise bring products to market; manufacturing capabilities; dates for regulatory meetings; statements about our financial outlook; 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; plans regarding current and future collaborative activities and the ownership of commercial rights; potential future agreements; scope, duration, validity and enforceability of intellectual property rights; future revenue streams and projections; expectations with respect to the expected use of proceeds from the recently completed offerings and the anticipated effects of the offerings; 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, 2024, as filed with the Securities and Exchange Commission on March 26, 2025, and in our Quarterly Report on Form 10-Q for the quarter ended March 31, 2025, as filed with the Securities and Exchange Commission on May 14, 2025. All forward-looking statements in this press release are based on information available to Capricor as of the date hereof, and Capricor assumes no obligation to update these forward-looking statements. *Deramioceol (CAP-1002) is an Investigational New Drug and is not yet approved for any indications. None of Capricor's exosome-based candidates have been approved for clinical investigation.*



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Capricor Therapeutics

- **Deramiocele** is a cellular therapy under review by the FDA for approval for the treatment of **cardiomyopathy** in patients with DMD
 - based on data from HOPE-2 and HOPE-2-OLE studies
 - **Deramiocele** is administered by IV infusion every 3 months
 - **Deramiocele** is a suspension of allogeneic Cardiosphere Derived Cells (CDCs) which are not stem cells and do not engraft
- **Deramiocele** mechanism of action is via exosome release which have anti-fibrotic, anti-inflammatory, immunomodulatory and pro-angiogenic activities
 - **Deramiocele** granted Orphan Drug, RMAT and Rare Pediatric Disease designations



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HOPE-2 and HOPE-2 OLE Study Overview

HOPE-2: Placebo controlled, randomized 12-month safety and efficacy study, final (n=20)

HOPE-2-OLE: Open label extension study with multiyear follow up (n=12)

Participants:

- Duchenne Muscular Dystrophy ≥ 10 years old
- Late Ambulatory & Non-ambulatory
- Left Ventricular Ejection Fraction $> 35\%$
- PUL 2.0 Entry Item score of 2 – 5

Key Endpoints:

- Cardiac Parameters by cMRI (Left Ventricular Ejection Fraction - **LVEF**)
- Performance of the Upper Limb (**PUL**)
- Safety

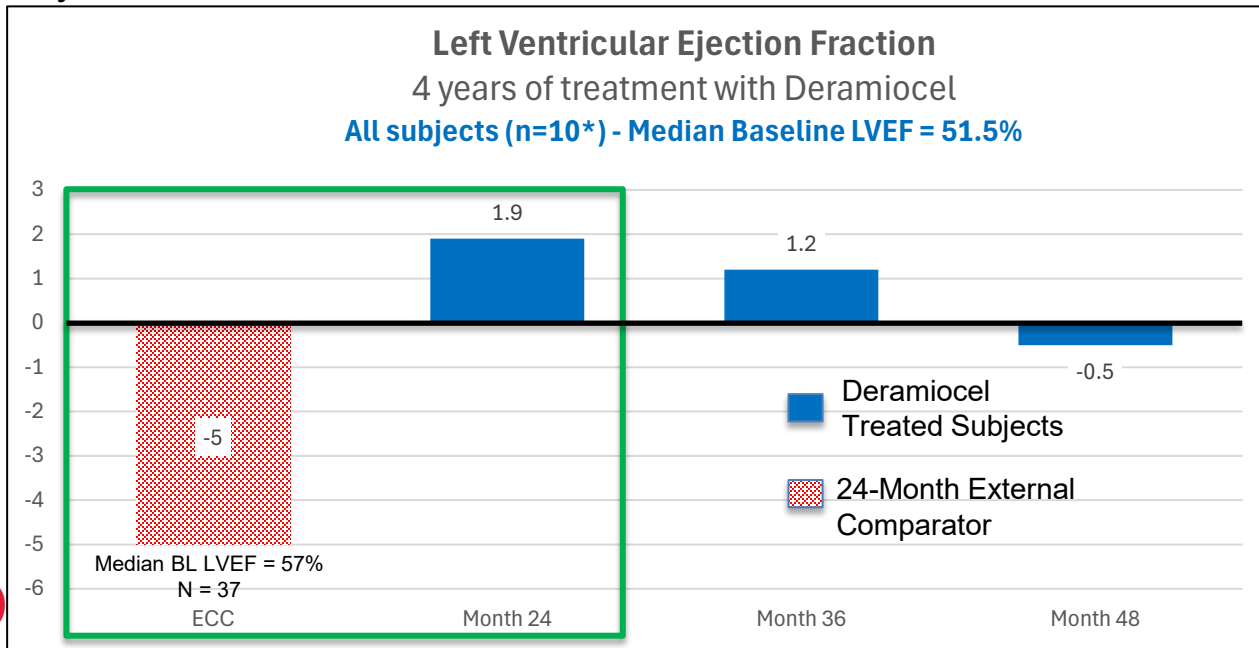


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HOPE-2-OLE

Long-Term stabilization of cardiac function as measured by LVEF using cMRI

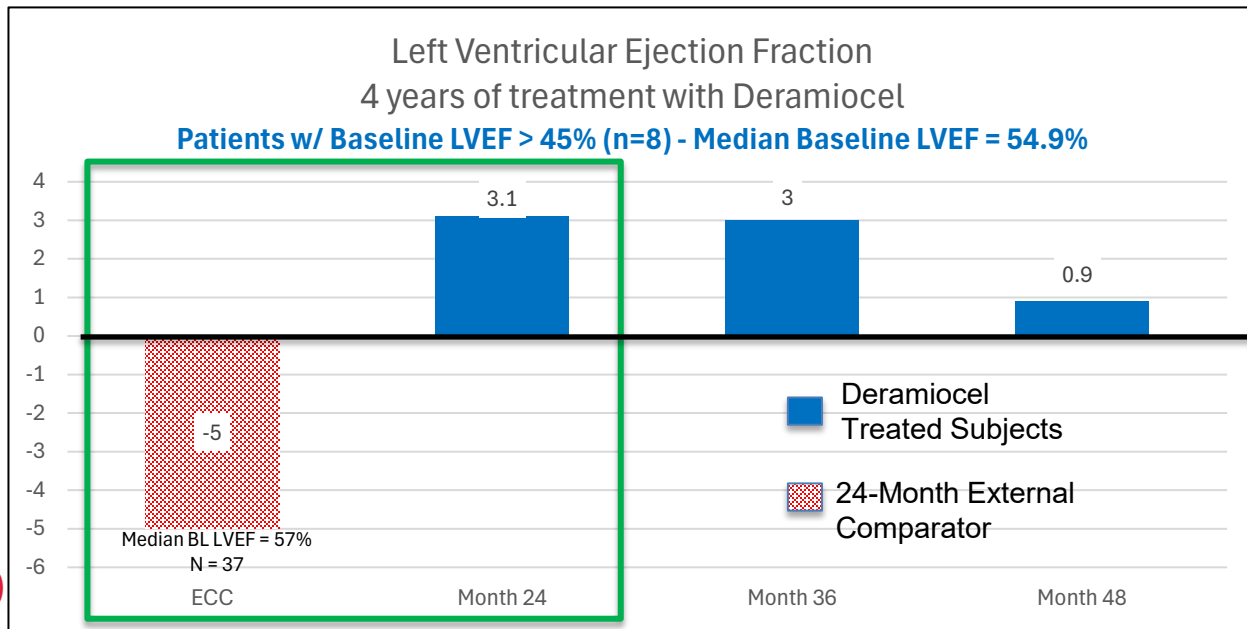
- External Comparator (ECC) from Vanderbilt University shows a median 2-year decline of 5% points in LVEF compared to a 2-year improvement of 1.9% points with deramiciol treated patents
- After 4 years of treatment with Deramiciol – **overall stabilization is observed**



HOPE-2-OLE

More marked differences in subgroup with >45% LVEF at baseline

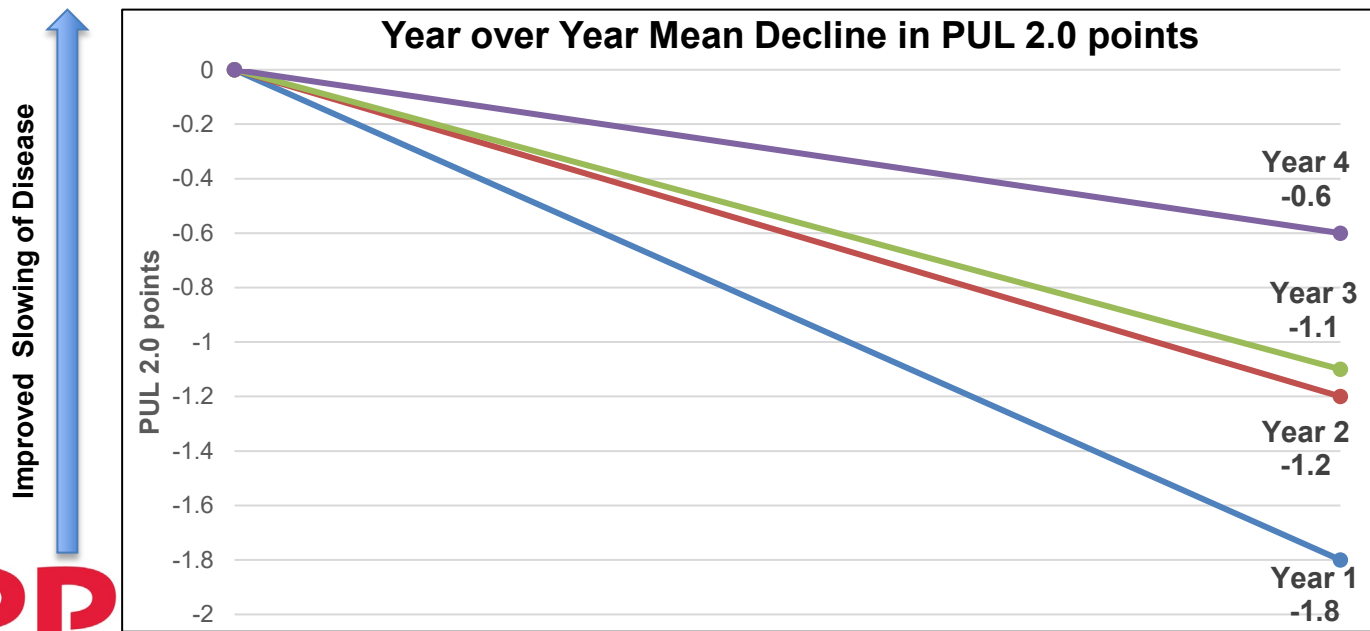
- Subgroup analysis of patients who initiated treatment with an LVEF >45% have greater stabilization in cardiac function over a 4-year treatment period
- **Suggests that early treatment with deramiocelel has a greater effect on cardiac function which is consistent with mechanism**



HOPE-2-OLE – Upper Limb Muscle Function

Potential Benefit / Data Highlights

- Long-Term benefit in slowing of decline of upper limb function (PUL 2.0)
- Average rate of decline of upper limb function decreases with longer treatment



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BLA currently under review by the FDA for:

***The treatment of cardiomyopathy
in patients with DMD***

PDUFA target date: **31 August 2025**



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