

Capricor Therapeutics - Readiness Before Resolution

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Decision scope: Sequencing incremental Deramiciol commercialization-readiness commitments

Case convention: The alternatives below are constructed for teaching. They are not disclosed board proposals or private deliberations. Figures and company statements are drawn from public sources available by the cutoff.

The decision

Capricor Therapeutics was preparing for a potential U.S. launch of Deramiciol, an investigational cell therapy for Duchenne muscular dystrophy (DMD), while the FDA continued reviewing its biologics license application (BLA). On August 24, 2026, the FDA extended the target action date from August 22 to November 22 after accepting a major amendment with 24-month HOPE-3 open-label-extension data and additional analyses. Capricor asked FDA to consider a refined proposed indication centered on upper-limb function, the trial's primary endpoint. The extension was a continuing review, not an approval. [P03]

The company had also expanded its San Diego manufacturing space in anticipation of potential demand. Yet approval remained uncertain and the exclusive U.S. distribution arrangement with Nippon Shinyaku was in dispute. The public record does not say how much incremental manufacturing or launch spending remained, what commitments were cancellable, or what a launch would cost. [P02, P09]

Your assignment: Recommend a rule for sequencing incremental commercialization-readiness spending while the FDA reviews the amendment. Choose among the constructed approaches below or propose a clear hybrid. Separate essential quality, regulatory and existing contractual work from new discretionary commitments. Explain how FDA feedback, inspection findings, distribution rights and liquidity should affect the sequence. Do not assume an approval, a distributor outcome or a disclosed program budget.

Constructed alternatives for discussion

1. **Milestone-gated tranches.** Release successive portions of any incremental readiness plan only as specified FDA, inspection and distribution milestones clear. Pause later tranches when a gate is unmet, while protecting critical-path quality and regulatory work.
2. **Accelerate readiness.** Commit earlier to incremental capacity and launch preparation to reduce time-to-market if approved. Define how leadership will manage affordability and the risk of delay or a restricted label.

3. **Liquidity-floor / maximum-burn policy.** Set a minimum liquidity reserve and maximum net new commitment level. Apply the rule irrespective of regulatory milestones, accepting slower readiness to preserve funds for obligations and other pipeline work. The threshold and forecast horizon are for students to propose; the company did not disclose them.

These alternatives do not imply that already-incurred construction or contractual obligations can be cancelled. They concern the governance of additional commitments whose amount and timing were not disclosed.

Evidence pulling in different directions

The pivotal HOPE-3 trial randomized 106 participants to Deramiciel or placebo. The peer-reviewed report found a 4.55 percentage-point between-group difference in 12-month PUL2.0 percentage change (95% CI 0.47-8.63; $p=.029$). Capricor's filing also described an updated left-ventricular ejection fraction analysis with $p=.09$; it said later outcomes in the testing hierarchy were not controlled for Type I error after a prespecified threshold was not met. [P02, P04]

At the July 29 advisory committee, members voted 3-9 against the question of whether the evidence established substantial effectiveness for **DMD cardiomyopathy**. The vote was nonbinding and was not a vote on the later upper-limb-focused proposed indication. In July, an FDA inspection generated one Form 483 observation about SOP/documentation, vendor oversight and audit-report timing. Capricor had submitted a response and was awaiting FDA feedback. [P02, P05]

The company reported that 82 of 106 HOPE-3 participants had reached 24 months by September 17: 40 originally randomized to Deramiciel and 42 to placebo. The WMS program listed a related poster for a September 30 session; the reviewed sources do not confirm that session occurred or when detailed results became public. Do not use unverified poster results. The planned oral presentation was October 3, after the case cutoff. [P07, P08]

Financing and operating context

At June 30, Capricor reported \$237.9 million in cash, cash equivalents and marketable securities, including \$217.0 million of marketable securities. Management said it believed those resources could fund operating capital requirements for at least twelve months from its August 14 filing date. That is a management estimate, not a guarantee through approval or launch. [P02]

During the first half of 2026, operating cash use was \$60.7 million. Investing cash use was \$203.4 million, largely reflecting purchases of marketable securities net of sales and maturities, plus \$16.6 million for property, equipment, leaseholds and construction in progress. The company reported no product revenue and a \$74.7 million net loss. These figures do not reveal future cash burn or remaining launch costs. [P02]

Capricor's H1 research and development table attributed \$26.1 million to Deramiciel and \$1.3 million to its exosome platform; the total also includes other personnel, facilities and R&D items. These are historical accounting categories, not future program budgets. The 2025 annual report described a 22,000-square-foot San Diego expansion intended for manufacturing scale-up, additional production suites, quality control and operations, subject to regulatory approval. It did not report the expansion's remaining cost to complete. [P02, P09]

Exhibit 1. Selected financial information

USD millions unless noted; reported periods end June 30.

Measure	H1 2026	H1 2025	Definition / note
Revenue	0.0	0.0	No product revenue reported
Operating expenses	79.7	52.7	GAAP consolidated measure
Net loss	74.7	50.3	GAAP consolidated measure; not the same as cash use
Net cash used in operating activities	60.7	26.6	Cash-flow measure
Net cash provided by (used in) investing activities	(203.4)	38.4	H1 2026 largely reflects net marketable-security purchases
Cash, equivalents and marketable securities	237.9 at June 30	318.1 at Dec. 31, 2025	Balance-sheet snapshot; includes marketable securities
H1 Deramioceol R&D	26.1	17.5	Company program attribution within R&D disclosure
H1 exosome-platform R&D	1.3	2.7	Company program attribution within R&D disclosure

Sources: [P02], consolidated statements, liquidity discussion and R&D table. Rounding may cause small differences. Investing cash use is not operating burn; GAAP loss is not cash burn.

Route to market and unresolved commitments

Under the U.S. agreement, Capricor is responsible for development and manufacturing; Nippon Shinyaku and its U.S. subsidiary hold exclusive U.S. distribution rights. Capricor had received \$30 million upfront and a \$10 million BLA-submission milestone. An additional \$80 million approval milestone and sales milestones/revenue share are contingent on future events and are not current liquidity. [P09]

Capricor disclosed a lawsuit seeking rescission or other relief concerning the U.S. arrangement. It withdrew an injunction motion without prejudice and said it chose arbitration after the FDA decision to develop a complete regulatory record; it expected arbitration to begin in the fall. The company's allegations are its position, not adjudicated findings. The route to market was therefore unresolved at cutoff. [P02]

The company's 2026 at-the-market (ATM) program had a \$125 million maximum after being reduced from \$150 million. It had sold \$77.5 million gross in 2025 and reported no sales from January 1 through the filing. Future issuance is not assured cash and could dilute shareholders. Capricor also warned that additional financing might require equity dilution, debt covenants or surrender of collaboration rights. [P02]

Questions for discussion

1. What spending is truly critical path before an FDA action, and which commitments can be sequenced?
2. Which alternative best balances speed, option value and financing flexibility? What is its strongest objection?
3. What evidence should trigger the next commitment: FDA feedback, inspection closure, distribution resolution, manufacturing validation, or some combination?
4. How should the recommendation change if the FDA approves a narrower label, requests further work, or issues another complete response letter?
5. Which figures can support a financial scenario, and what launch-cost, capacity, working-capital and demand inputs remain missing?

Sources

P02. Capricor, Q2 2026 Form 10-Q, quarter ended June 30, 2026; [SEC filing](#), accession 0001104659-26-097294. Relevant HTML locators in dossier source register.

P03. Capricor, Aug. 24, 2026 8-K and Exhibit 99.1, [SEC filing](#), accession 0001104659-26-100071.

P04. McDonald et al., “Deramiciel heart-derived cellular therapy in advanced Duchenne muscular dystrophy (HOPE-3),” *The Lancet* 408 (2026), 721-733, [publisher article](#).

P05. FDA, [Cellular, Tissue, and Gene Therapies Advisory Committee](#), July 29, 2026.

P07. Capricor, [Sept. 17, 2026 WMS announcement](#).

P08. WMS, [2026 full program, Sept. 20 PDF version](#).

P09. Capricor, 2025 Form 10-K, [SEC filing](#).

This case uses only information available by October 2, 2026, 4:00 p.m. ET. Source access, dates and detailed locators are documented in the research source register and evidence extraction.