

CAMP4 Therapeutics: Sequencing Capital Across a Platform

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The case

The decision

On May 14, 2025, CAMP4 Therapeutics had three distinct demands on its capital: continue a clinical program for urea cycle disorders (UCDs), advance a newly nominated SYNGAP1 candidate toward toxicology and clinical testing, and sustain the discovery platform and partnership work that could generate future programs. The company had recently raised public capital, but reported less than \$50 million in cash and a substantial-doubt paragraph in its latest quarterly filing. It had also reported a company-claimed increase in SYNGAP1 protein in non-human primates (NHPs), while detailed supporting data were scheduled for a conference two days later.

For this classroom exercise, assume you are preparing a capital-sequencing recommendation for a **hypothetical CAMP4 board discussion**. Public sources do not disclose that the board posed this question or made a reallocation decision. Your task is to recommend what CAMP4 should protect, stage, partner, or defer using only what was publicly available at the cutoff.

Decision question: How should CAMP4 sequence scarce capital across CMP-CPS-001 for UCDs, CMP-SYNGAP-01 for SYNGAP1-related disorders, and the RAP discovery platform and partnership options?

Choose one approach, combine approaches, or present a coherent alternative. Identify the evidence and financial gates that would release the next commitment. Prepare a two-page recommendation memo and a five-minute oral defense. Do not invent asset-level costs, probability of success, patient outcomes, board intentions, or partnership terms. If you use numerical assumptions, label them as classroom assumptions and show the calculation.

Company and portfolio context

CAMP4 is a clinical-stage biotechnology company developing antisense oligonucleotides (ASOs) designed to increase expression of selected genes. Its proprietary RAP Platform identifies regulatory RNAs (regRNAs) associated with enhancers and promoters; CAMP4 says its therapeutic ASOs bind those regRNAs to alter transcription. This is distinct from ASOs designed to degrade messenger RNA or block translation. [P02, HTML lines 262-265, 401-411]

The October 2024 IPO prospectus described estimated net proceeds of approximately **\$26 million** for CMP-CPS-001, **\$18 million** for CMP-SYNGAP work, **\$10 million** for RAP and other

discovery programs, with the remainder for working capital and general corporate purposes. The prospectus estimated \$66.7 million of net proceeds before any underwriter option and \$77.1 million if fully exercised. These were management's intended uses, not restricted funds or actual program budgets. In Q1 2025 CAMP4 reported no material change in planned use. [P01, HTML lines 3651-3682; P03, lines 1073-1075]

At March 31, 2025, CAMP4 reported **\$49.3 million** in cash and cash equivalents and used **\$14.3 million** in operating cash during the quarter. Management estimated that cash would fund planned operating expenses and capital expenditures into Q2 2026. The same Form 10-Q said resources might not be sufficient to fund operations through at least the next twelve months and raised substantial doubt about the company's ability to continue as a going concern. These statements reflect different management assessments and assumptions; the record does not reduce the situation to either guaranteed runway or imminent insolvency. Q1 R&D expense was **\$10.1 million**, but CAMP4 reports one R&D operating segment and does not disclose program-by-program spending. [P03, Note 1, lines 411-422; MD&A lines 944-955; segment disclosure lines 750-766]

Partnership activity provides a potential source of support but not a replacement for a capital plan. BioMarin paid a **\$1 million** nonrefundable upfront payment and reimburses certain research activity under a collaboration; its future milestone and royalty payments are conditional, and BioMarin milestone consideration was fully constrained at quarter-end. CAMP4 reported **\$858,000** in Q1 research and collaboration revenue. Separately, an April 2025 event made CAMP4 eligible for a **\$600,000** Fulcrum milestone; eligibility is not the same as cash receipt. CAMP4 disclosed no new committed financing facility in its Q1 10-Q. [P03, Note 5, lines 632-646; P04, lines 23-24]

Three investment lanes

Lane	Position at cutoff	What is known and what remains open
CMP-CPS-001 / UCDs	Ongoing Phase 1 in healthy volunteers. Dosing completed in MAD cohort 3. Interim analysis of four SAD cohorts in 48 healthy volunteers reported no safety trends of concern.	Safety, pharmacokinetic and key pharmacodynamic biomarker data from the SAD/MAD study were expected in Q4 2025. The March 2025 10-K described an Australian Phase 1b expansion as planned for Q2 2025. Separately, the May 13 release reported that a European Phase 1b CTA for female OTC heterozygotes had been submitted; clearance was pending. Healthy-volunteer safety or biomarker results would not establish patient efficacy. [P02, lines 271, 610-611; P03, lines 813-824; P04, lines 4-7, 18-22]
CMP-SYNGAP-01 / SYNGAP1	Development candidate nominated; GLP toxicology was expected to begin in 2025, but had not started.	On May 13, CAMP4 reported a “meaningful increase” in SYNGAP1 protein after intrathecal dosing in NHPs. The release previewed further ASGCT information but gave no cohort size, methods or numerical result. Treat this as a company-reported preclinical signal. The May 16 presentations and details are outside the cutoff. [P03, lines 834-838; P04, lines 9, 14-20, 25]
RAP platform / discovery / collaboration	A GBA1 discovery program for Parkinson's disease and broader future discovery were disclosed. CAMP4 had a research collaboration with BioMarin for two genetic targets.	The public record does not report a full platform budget, target-level collaboration economics, or a program-level resource split. Future BioMarin milestones and royalties were contingent, not committed runway. [P02, lines 262-265, 273-274; P03, Note 5, lines 632-646]

The programs face different biological, delivery and development risks. CAMP4 describes CMP-CPS-001 as a subcutaneous, liver-directed ASO and its SYNGAP program as using intrathecal delivery. The ASO label alone does not make the programs comparable. [P02, lines 333, 401-411, 638-643]

Care and competition at the cutoff

For UCDs, CAMP4 described current care as nitrogen scavengers, supplemental citrulline, dietary protein restriction and, when needed, hemodialysis. It described liver transplant as the only curative treatment, generally limited to early-onset patients and available to fewer than 10%. Amgen's Ravicti (glycerol phenylbutyrate) was a marketed nitrogen-scavenger treatment. [P02, lines 458-464, 715-716]

Cutoff-eligible company sources described three OTC-focused investigational approaches in distinct populations and stages: Ultragenyx reported its Phase 3 DTX301 study had completed enrollment with 37 participants aged 12 and older; Arcturus reported an ongoing open-label Phase 2 study using five intravenous infusions over two months and a separate completed-dosing, placebo-controlled European study with eight participants; iECURE reported

preliminary findings from the first infant in an ongoing Phase 1/2 neonatal-onset OTC study. These are not like-for-like comparisons with CAMP4's planned Phase 1b expansion for female OTC heterozygotes, and the iECURE item is a company-reported single-participant update. [P07; P08, Exhibit 99.1, pp. 3-4; P09]

For SYNGAP1-related disorders, CAMP4 reported no FDA-approved disease-modifying therapy and described supportive therapies, antiseizure medicines and vagus nerve stimulation for some patients. The company identified Stoke Therapeutics and Praxis Precision Medicines as direct development competitors and Tevard, Regel and Quiver as discovery-stage programs. Cutoff-eligible filings describe Stoke's Acadia SYNGAP1 discovery/development collaboration and Praxis's PRAX-090 as an earlier-stage ASO with preclinical proof of mechanism and candidate nomination expected by year-end 2025. These do not establish an approved therapy or clinical efficacy; the Tevard, Regel and Quiver labels remain the dated March 2025 CAMP4 snapshot. [P02, lines 633-638, 715-716; P10, p. 22; P11, lines 622-629]

Constructed approaches

The following alternatives are constructed for teaching. They are not disclosed company proposals.

Approach	What you would do	Main trade-off	Possible decision gates
Protect the UCD clinical path	Continue CMP-CPS-001 toward the planned patient expansion, prepare for regulatory clearance and maintain the near-term clinical path. Stage discretionary SYNGAP and discovery spending.	Focuses resources on the most advanced program and a defined next patient-facing step; increases dependence on a single asset and may delay a high-need CNS program.	UCD MAD safety/PK/PD, European CTA clearance, an updated clinical budget and a financing plan that preserves adequate liquidity.
Maintain two lead programs with staged gates	Continue UCD work and fund SYNGAP through a bounded next proof point, such as GLP-tox preparation or initiation, while delaying later clinical commitments until data and cash visibility improve.	Preserves two scientific options and responds to the NHP signal; still places demands on a constrained balance sheet and requires transparent stop/go criteria.	NHP methods and reproducibility, toxicology readiness, the Q4 UCD readout, cash forecasts, and a specific trigger for further financing or partnering.
Preserve runway and externalize more development	Protect essential safety and maintenance work, slow selected internal commitments, seek partner support for eligible programs or platform work, and revisit internal spending after financing or evidence milestones.	Extends flexibility and may share development burden; partner terms and counterparties are unknown, and deferral could delay patient-relevant evidence or reduce strategic control.	Credible partner interest and terms, regulatory and scientific milestones, cash runway under downside scenarios, and explicit criteria for restarting internal investment.

Capital available for one lane is not automatically free cash: the company has shared operating expenses, obligations, research activity and clinical commitments. The public materials do not state which expenses are avoidable, how quickly they could be reduced, or what each next study would cost.

Exhibits

Exhibit 1. Capital and liquidity snapshot

Measure	Amount / statement	Basis
Estimated IPO net proceeds allocated across three stated uses	\$54 million (\$26m + \$18m + \$10m)	October 2024 prospectus; intended use estimates
Estimated IPO net proceeds	\$66.7m base; \$77.1m with full underwriter option	October 2024 prospectus estimates
Cash and cash equivalents	\$49.323m	March 31, 2025 balance sheet
Q1 operating cash used	\$14.286m	Three months ended March 31, 2025
Q1 R&D expense	\$10.146m	Consolidated R&D segment; not allocated by candidate
Management runway estimate	Into Q2 2026	Based on planned cash use; forward-looking
Going-concern disclosure	Substantial doubt; resources may not fund at least 12 months from filing issuance	Q1 2025 Form 10-Q

Sources: [P01, HTML lines 3651-3682]; [P03, lines 344-371, 411-422, 750-766, 944-983]. The \$54 million is the sum of three estimated use-of-proceeds amounts, not actual spending, a reserve, or a complete budget. Do not extrapolate an alternative runway from one quarter's operating cash flow.

Exhibit 2. Near-term evidence and funding gates

Lane	Evidence available May 14	Next evidence or dependency then expected	Limit of inference
CMP-CPS-001	Company-reported favorable interim SAD safety observations in 48 healthy volunteers; first three MAD cohorts dosed	Q4 2025 SAD/MAD safety, PK and key PD biomarkers; European CTA submitted with clearance pending; March 2025 10-K plan had called for an Australian Phase 1b expansion in Q2 2025	No patient efficacy result; no program cost or probability of success disclosed
CMP-SYNGAP-01	Nominated candidate; company-reported meaningful SYNGAP1 protein increase in NHPs, without detailed methods or magnitude in the May 13 release	GLP toxicology expected to begin in 2025; detailed ASGCT presentation scheduled May 16 but beyond the cutoff	Preclinical signal only; sample size and numerical effect unknown at cutoff
RAP / partners	GBA1 discovery program; BioMarin collaboration research revenue/reimbursement; contingent milestone economics	Future collaboration progress and potential new programs	No certain future milestone cash, program-level budget or partnership terms by target disclosed

Sources: [P02, lines 262-265, 271, 273-274, 610-611]; [P03, lines 813-846, 632-646]; [P04, lines 9-25].

Assignment

Prepare a two-page memo that:

1. Recommends how to sequence capital across UCD, SYNGAP1 and RAP/platform or partnership work.
2. Identifies what should be funded now, what should wait, and the evidence and financing conditions that would change the next decision.
3. Compares at least two constructed approaches and presents the strongest argument against your recommendation.
4. Separates reported facts, management expectations, your interpretation and any classroom assumptions.

Do not use the May 16 ASGCT presentations, later financing, later trial results or post-cutoff developments. All figures are in U.S. dollars unless noted.

Selected sources

- **P01 - CAMP4 Therapeutics, final IPO prospectus, Form 424B4, accepted October 11, 2024. [SEC filing](#)**
- **P02 - CAMP4 Therapeutics, 2024 Form 10-K, accepted March 27, 2025. [SEC filing](#)**
- **P03 - CAMP4 Therapeutics, Q1 2025 Form 10-Q, accepted May 13, 2025. [SEC filing](#)**
- **P04 - CAMP4 Therapeutics, Q1 2025 earnings release, Exhibit 99.1 to Form 8-K, released May 13, 2025. [SEC exhibit](#)**
- **P07 - Ultragenyx Pharmaceutical, Q4/FY2024 results and corporate update, February 13, 2025. [Company release](#)**
- **P08 - Arcturus Therapeutics, Q1 2025 update, May 12, 2025. [8-K Exhibit 99.1](#)**
- **P09 - iECURE, OTC-HOPE Phase 1/2 first-infant update, January 9, 2025. [Company release](#)**
- **P10 - Praxis Precision Medicines, Q1 2025 Form 10-Q, accepted May 2, 2025. [SEC filing](#) · [SEC filing detail](#) · [Company-hosted PDF](#)**
- **P11 - Stoke Therapeutics, Q1 2025 Form 10-Q, filed May 13, 2025. [SEC filing](#)**

The case freezes information at **May 14, 2025, 4:00 p.m. ET**. The company previewed May 16 materials in its release; the presentations and all details disclosed then are excluded.