



Rapport Therapeutics Reports First Quarter 2025 Financials and Provides Business Update

May 8, 2025

- *RAP-219 Phase 2a trial in patients with refractory focal epilepsy remains on track with topline results expected in the third quarter of 2025*
- *RAP-219 Phase 2a trial in patients with bipolar mania remains on track to commence in the third quarter of 2025, with topline results expected in the first half of 2027*
- *Company to host inaugural Investor and Analyst Day on June 2, 2025, featuring presentations from management team and key opinion leader [Dr. Jacqueline French](#)*
- *Ended the quarter with \$285.4 million in cash, cash equivalents, and short-term investments, excluding restricted cash, which is expected to fund operations through the end of 2026*

BOSTON and SAN DIEGO, May 08, 2025 (GLOBE NEWSWIRE) -- Rapport Therapeutics, Inc. (Nasdaq: RAPP), a clinical-stage biotechnology company dedicated to the discovery and development of small molecule precision medicines for patients with neurological or psychiatric disorders, today reported financial results for the first quarter ending March 31, 2025, and provided a business update.

"2025 represents a transformational year for Rapport as we prepare to deliver our initial proof-of-concept data for RAP-219 from the Phase 2a trial in patients with refractory focal epilepsy and progress our pipeline-in-a-product strategy with the initiation of our bipolar mania trial. Rapport's first quarter demonstrated our continued ability to execute our clinical development efforts, and we remain confident that we are on track to deliver on our development milestones. Enrollment in the Phase 2a trial in patients with refractory focal epilepsy is progressing as planned, and we are observing a growing level of excitement in the epilepsy community for the data, given the innovative trial design and its potential to evolve epilepsy drug development," said Abraham N. Ceasay, CEO of Rapport. "The recent PET and MAD-2 trial results further validate the neuroanatomical specificity of TARPγ8 and favorable tolerability profile of RAP-219. When combined with the strong preclinical anti-seizure effects seen, these findings reinforce our confidence in RAP-219's differentiated precision profile."

BUSINESS HIGHLIGHTS

RAP-219 Lead Program

- **PET Data Demonstrated Regionally Selective Expression of TARPγ8 in Epilepsy-Associated Brain Regions.** In January, the Company announced results from its healthy volunteer PET trial for RAP-219. The data showed that neuroanatomical specificity was achieved through RAP-219's selective targeting of TARPγ8, with robust expression in the areas of the brain associated with epilepsy and minimal expression in the hindbrain, where drug effects are often associated with intolerable adverse events.
- **RAP-219 Achieved Receptor Occupancy Associated with Maximal Seizure Protection Within Five Days of Dosing.** In Cohort 1 of the human PET trial—using the same dosing regimen as the ongoing Phase 2a trial in patients with refractory focal epilepsy—RAP-219 reached and exceeded receptor occupancy levels associated with maximal seizure protection in preclinical models and was generally well tolerated. In earlier preclinical studies, RAP-219 and other TARPγ8 AMPA receptor (AMPA) negative allosteric modulators (NAMs) demonstrated robust, dose-dependent antiseizure efficacy across a broad array of focal and generalized seizure models—including the corneal kindling model—with potent effects observed at 50–70% receptor occupancy, a range shown to be predictive of clinically meaningful outcomes. Data from both the PET and MAD-2 trials confirmed that target plasma concentrations and corresponding receptor occupancy was achieved within five days.
- **Favorable Tolerability Now Reported Across Four Phase 1 Trials.** A total of four Phase 1 trials have now been conducted—a single ascending dose trial, two multiple ascending dose trials, and a PET trial (final study report in progress)—with 100 healthy volunteers exposed to RAP-219. In these trials, RAP-219 was generally well tolerated with no serious adverse events ("SAEs") and no TEAEs greater than Grade 2. There were three treatment discontinuations (3%) that were attributed to treatment emergent adverse events. No clinically significant laboratory, electrocardiogram (ECG), or vital sign abnormalities were reported in the SAD or two MAD trials. While the final study report is in progress, PET trial TEAEs are generally consistent with other Phase 1 trials.
 - Among the 48 participants exposed to RAP-219 in the two MAD trials, the most common TEAEs were headache (n=5), sinus tachycardia (n=4), and brain fog, insomnia, bowel movement irregularity, dry mouth, and medical device site reaction (n=3 each). Among the 16 participants given placebo, the most common TEAEs were abdominal pain, brain fog, constipation, cough, decreased appetite, dizziness, medical device site reaction, and

second-degree atrioventricular block (n=1 each).

Focal Epilepsy

- **Phase 2a Topline Results Expected in Third Quarter 2025.** The trial remains on track and the Company expects to announce topline results for its Phase 2a trial in patients with refractory focal epilepsy in the third quarter of 2025.
- **Encore Presentations at the 2025 American Academy of Neurology (AAN) Annual Meeting Highlighted the Company's Ongoing Research and Development of RAP-219.**
 - [Optimal Cut Point for Reduction in Long Episode Frequency to Predict Meaningful Change in Clinical Seizure Frequency](#). A post hoc analysis was performed using data from patients with refractory focal epilepsy in Neuropace's long-term study database who met the enrollment criteria for the ongoing RAP-219 Phase 2a trial. Using a receiver operator characteristic (ROC) analysis, the analysis confirmed that a 30% reduction in LE frequency was the optimal cut point associated with a clinically meaningful ($\geq 50\%$) reduction in clinical seizures, regardless of the antiseizure medication initiated, revealing a linear relationship between long episode and clinical seizure frequencies.
 - [Antiseizure Effects with Selective TARP \$\gamma\$ 8 Negative Allosteric Modulators in Preclinical Seizure Models](#). RAP-219 provided potent, dose-dependent antiseizure effects in pentylenetetrazol (PTZ) and corneal kindling preclinical seizure models, with maximal protection observed with 70% receptor occupancy and RAP-219 mean plasma concentration of 7 ng/mL.

Bipolar Disorder

- **RAP-219 Advancing into the Clinic in Bipolar Mania.** The Phase 2a trial in patients with bipolar mania remains on track to initiate in the third quarter of 2025, with topline results expected in the first half of 2027.

Peripheral Neuropathic Pain

- **Plans for Phase 2a Trial in Diabetic Peripheral Neuropathic Pain (DPNP) Being Finalized.** An update on the timeline for initiation of the trial in DPNP is expected in 2025. In the fourth quarter of 2024, the IND submitted to the FDA for RAP-219 in DPNP was placed on clinical hold, and the FDA requested additional information and protocol design amendments.

CORPORATE UPDATES

- **Dr. Jeffrey Sevigny Appointed Chief Medical Officer.** In March, Dr. Sevigny joined Rapport as Chief Medical Officer (CMO). Most recently chief medical officer at Prevail Therapeutics, a wholly owned subsidiary of Eli Lilly, and senior vice president of Neuroscience at Eli Lilly, Dr. Sevigny is a physician-scientist with more than 15 years of leadership in translational and clinical drug development. His experience spans the full spectrum of development—from discovery to late-stage clinical trials and regulatory approvals—and with a strong record of portfolio development and building high-performing organizations, he brings deep strategic and operational experience to Rapport.
- **Investor and Analyst Day Scheduled in June.** Rapport plans to host its inaugural Investor and Analyst Day on Monday, June 2, 2025. Executive presentations will be accompanied by a fireside chat with leading epilepsy KOL, [Jacqueline A. French](#), M.D. Dr. French is professor of Neurology at NYU Langone's Comprehensive Epilepsy Center and is the founder and director of the [Epilepsy Study Consortium](#). She is the principal investigator for the RAP-219 Phase 2a trial.

FIRST QUARTER 2025 FINANCIAL RESULTS

- **Net Loss:** Net Loss for the first quarter of 2025 was \$24.1 million, as compared to \$22.7 million for the prior year period.
- **Research and Development (R&D) Expenses:** R&D expense was \$19.6 million for the first quarter of 2025, as compared to \$12.5 million for the prior year period. The increase in R&D expense was primarily driven by operational costs related to clinical development and costs to support the progression of the Company's overall pipeline.
- **General and Administrative (G&A) Expenses:** G&A expense was \$7.5 million for the first quarter of 2025, as compared to \$4.6 million for the prior year period. The increase in general and administrative expense was primarily driven by costs associated with the growth of the business, in addition to costs incurred to satisfy the requirements of operating as a public company.
- **Cash Position:** The Company ended the first quarter with \$285.4 million in cash, cash equivalents and short-term investments, compared to \$305.3 million as of December 31, 2024. The decrease was primarily due to cash outflows on operating activities in the first quarter of 2025.
- **Cash Runway:** The Company expects that cash, cash equivalents, and short-term investments as of March 31, 2025, will enable the Company to fund its operating expenses and capital expenditure requirements through the end of 2026.

About RAP-219

RAP-219 is a clinical-stage AMPA receptor (AMPA) negative allosteric modulator (NAM) designed to achieve neuroanatomical specificity through its selective targeting of a receptor associated protein (RAP) known as TARP γ 8, which is associated with

neuronal AMPARs. Whereas AMPARs are distributed widely in the central nervous system, TARPγ8 is expressed only in discrete regions, including the hippocampus and neocortex, where focal seizures often originate. By contrast, TARPγ8 has minimal expression in the hindbrain, where drug effects are often associated with intolerable adverse events. With this precision approach, the Company believes RAP-219 has the potential to provide a differentiated profile as compared to traditional neuroscience medications. Due to the role of AMPA biology in various neurological disorders and the selective targeting of TARPγ8, the Company believes RAP-219 has pipeline-in-a-product potential and is evaluating the compound as a transformational treatment for patients with focal epilepsy, bipolar disorder, and peripheral neuropathic pain.

Availability of Other Information About Rapport Therapeutics

Rapport Therapeutics uses and intends to continue to use its Investor Relations website and LinkedIn (Rapport Therapeutics) as a means of disclosing material nonpublic information and for complying with its disclosure obligations under Regulation FD. Accordingly, investors should monitor the Company's Investor Relations website and LinkedIn, in addition to following the Company's press releases, SEC filings, public conference calls, presentations, and webcasts. The contents of the Company's website or social media shall not be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended.

About Rapport Therapeutics

Rapport Therapeutics is a clinical-stage biotechnology company dedicated to discovering and developing small molecule precision medicines for patients with neurological or psychiatric disorders. The Company's founders have made pioneering discoveries related to the function of receptor associated proteins (RAPs) in the brain. Their findings form the basis of Rapport's RAP technology platform, which enables a differentiated approach to generate precision small molecule product candidates with the potential to overcome many limitations of conventional neurology drug discovery. Rapport's precision neuroscience pipeline includes the Company's lead investigational drug, RAP-219, designed to achieve neuroanatomical specificity through its selective targeting of a RAP expressed in only discrete regions of the brain. The Company is currently pursuing RAP-219 as a potential treatment for refractory focal epilepsy, bipolar mania and diabetic peripheral neuropathic pain. Additional preclinical and late-stage discovery stage programs are also underway, including targeting chronic pain and hearing disorders.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933 and Section 21E of the Securities Exchange Act of 1934, each as amended. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements include, but are not limited to, express or implied statements regarding: the clinical development of RAP-219 for the treatment of drug-resistant focal epilepsy, peripheral neuropathic pain and bipolar disorder, including the initiation, timing, progress and results of our ongoing and planned clinical trials; the Company's ability to resolve a clinical hold with the FDA; the potential activity and tolerability of RAP-219; the potential of Rapport's RAP technology platform; and expectations for Rapport's uses of capital, expenses and financial results, including its cash runway through the end of 2026.

Forward looking statements are based on management's current expectations and are subject to risks and uncertainties that could negatively affect Rapport's business, operating results, financial condition and stock value. Factors that could cause actual results to differ materially from those currently anticipated include: risks relating to the Company's research and development activities; Rapport's ability to execute on its strategy including obtaining the requisite regulatory approvals on the expected timeline, if at all; uncertainties relating to preclinical and clinical development activities; the Company's dependence on third parties to conduct clinical trials, manufacture its product candidates and develop and commercialize its product candidates, if approved; Rapport's ability to attract, integrate and retain key personnel; risks related to the Company's financial condition and need for substantial additional funds in order to complete development activities and commercialize a product candidate, if approved; risks related to regulatory developments and approval processes of the U.S. Food and Drug Administration and comparable foreign regulatory authorities; risks related to establishing and maintaining Rapport's intellectual property protections; and risks related to the competitive landscape for Rapport's product candidates; as well as other risks described in "Risk Factors," in the Company's Annual Report on Form 10-K and most recent Quarterly Report on Form 10-Q, as well as discussions of potential risks, uncertainties, and other important factors in Rapport's subsequent filings with the Securities and Exchange Commission. Any forward-looking statements represent Rapport's views only as of today and should not be relied upon as representing its views as of any subsequent date. Rapport expressly disclaims any obligation or undertaking to release publicly any updates or revisions to any forward-looking statements contained herein to reflect any change in its expectations or any changes in events, conditions or circumstances on which any such statement is based, except as required by law, and claims the protection of the safe harbor for forward-looking statements contained in the Private Securities Litigation Reform Act of 1995.

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Condensed Consolidated Balance Sheet Data (In thousands) (unaudited)

**March 31,
2025**

**December 31,
2024**

Assets

Current assets

Cash and cash equivalents	\$ 57,604	\$ 56,805
Short-term investments	227,778	248,475
Restricted cash	105	105
Prepaid expenses and other current assets	5,481	4,417
Total current assets	290,968.00	309,802
Property and equipment, net	3,404	3,529
Operating lease right of use asset, net	7,470	1,442
Other assets	211	160
Total assets	<u>\$ 302,053</u>	<u>\$ 314,933</u>

Liabilities, Convertible Preferred Stock and Stockholders' Equity

Current liabilities

Accounts payable	\$ 2,139	\$ 1,954
Accrued expenses and other current liabilities	6,236	6,076
Operating lease liability	1,008	737
Total current liabilities	9,383	8,767
Series B preferred stock tranche right liability	—	—
Operating lease liability, net of current portion	6,834	739
Total liabilities	16,217	9,506
Commitments and contingencies		
Common Stock	37	37
Additional paid-in capital	433,702	429,657
Accumulated other comprehensive income	(95)	(522)
Accumulated deficit	(147,808)	(123,745)
Total stockholders' equity	285,836	305,427
Total liabilities, convertible preferred stock, and stockholders' equity	<u>\$ 302,053</u>	<u>\$ 314,933</u>

Condensed Consolidated Statement of Operations
(In thousands, except share and per share data)
(unaudited)

	For the three months ended March 31,	
	2025	2024
Operating expenses		
Research and development	\$ 19,572	\$ 12,504
General and administrative	7,536	4,590
Total operating expenses	27,108	17,094
Loss from operations	(27,108)	(17,094)
Other income (expense):		
Interest income	3,045	1,815
Change in fair value of preferred stock tranche right liability	—	(7,390)
Total other income, net	3,045	(5,575)
Net loss	<u>\$ (24,063)</u>	<u>\$ (22,669)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.68)</u>	<u>\$ (11.07)</u>
Weighted-average common shares outstanding, basic and diluted	<u>35,266,577</u>	<u>2,046,889</u>

Condensed Consolidated Statements of Cash Flows
(In thousands)
(unaudited)

**For the three months ended
March 31,**

	2025	2024
Net cash used in operating activities	\$ (20,237)	\$ (17,615)
Net cash provided by (used in) investing activities	21,031	(41,926)
Net cash provided by financing activities	5	63,659
Net increase in cash, cash equivalents and restricted cash	<u>\$ 799</u>	<u>\$ 4,118</u>