



Rapport Therapeutics Reports Fourth Quarter and Full Year 2024 Financial Results and Provides Business Update

March 11, 2025

- *Results of positron emission tomography (PET) trial and second multiple ascending dose (MAD-2) trial support RAP-219's transformative potential for epilepsy and other central nervous system (CNS) disorders*
- *Continued momentum in the Phase 2a trial of RAP-219 in patients with refractory focal epilepsy, with topline results expected in the third quarter of 2025*
- *Expect to initiate a Phase 2a trial in patients with bipolar mania in the third quarter of 2025, with topline results expected in the first half of 2027*
- *Appointed recognized translational and clinical drug development leader, Jeffrey Sevigny, M.D., as chief medical officer*
- *Ended the year with \$305.3 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, March 11, 2025 (GLOBE NEWSWIRE) -- Rapport Therapeutics, Inc. (Nasdaq: RAPP) ("Rapport" or the "Company"), 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 quarter and full year ended December 31, 2024, and provided a business update.

"With continued execution in the fourth quarter, Rapport is well positioned to deliver on multiple meaningful development milestones. The RAP-219 Phase 2a trial in patients with refractory focal epilepsy continues to advance with strong momentum; patient enrollment and dosing are progressing as planned, and the trial remains on track to deliver topline results in the third quarter of 2025. Based on data from 100 healthy subjects dosed, we continue to have confidence in RAP-219's potential as a transformational medicine. The recently released PET and MAD-2 trial results demonstrated neuroanatomical specificity and favorable tolerability, further reinforcing our belief in its differentiated precision profile," said Abraham N. Ceesay, CEO of Rapport. "We are committed to the pipeline-within-a-product strategy for RAP-219 and anticipate initiating a Phase 2a trial in bipolar mania in the third quarter of this year while continuing planning for our Phase 2a trial in diabetic peripheral neuropathic pain."

BUSINESS HIGHLIGHTS

RAP-219 Lead Program

- **Announced PET and MAD-2 Data, Further Supporting RAP-219's Transformative Potential for CNS Disorders:** In January, the Company announced results from its healthy volunteer PET and MAD-2 trials for RAP-219. Data demonstrated that neuroanatomical specificity was achieved through RAP-219's selective targeting of TARPy8. In Cohort 1 of the human PET trial, which used the dosing regimen utilized in the Company's ongoing Phase 2a trial in patients with refractory focal epilepsy, RAP-219 achieved target receptor occupancy associated with maximal seizure protection in preclinical models within five days and was generally well tolerated, which we believe further supports the use of such dosing regimen in the Phase 2a trial.
- **Favorable Tolerability Across Four Phase 1 Trials.** A total of four Phase 1 trials have been conducted to date -- 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 all of these trials, RAP-219 was generally well tolerated with no serious adverse events ("SAEs"). There were three treatment discontinuations (3%) that were attributed to treatment emergent adverse events ("TEAEs"), with no TEAEs greater than Grade 2. 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 exposed to 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

- **Presented Data at AES Demonstrating Consistent Association Between Decreases in RNS Measured Long Episodes and Meaningful Reductions in Clinical Seizures:** In December, Rapport presented novel findings on the association between the seizure biomarker used in the Company's proof-of-concept trial for RAP-219 and clinical seizures. Patients with refractory focal epilepsy who would meet the enrollment criteria for the ongoing RAP-219 Phase 2a trial were selected from [NeuroPace's](#) long-term study database for this post-hoc analysis. The analysis demonstrated a linear

relationship between changes in long episode (LE) frequency and clinical seizure frequency and identified the benchmark to predict clinically meaningful seizure reduction. Rapport presented the data at the American Epilepsy Society (AES) Annual Meeting in Los Angeles.

- **Phase 2a Trial Topline Results Expected in Third Quarter 2025.** The Company expects to announce topline results for its Phase 2a trial in patients with refractory focal epilepsy in the third quarter of 2025, narrowing the timeline from prior guidance.

Bipolar Disorder

- **Established Timeline for Initiation of Phase 2a Trial of RAP-219 in Bipolar Mania.** The Company expects to initiate a Phase 2a trial in patients with bipolar mania in the third quarter of 2025, with topline results expected in the first half of 2027.

Peripheral Neuropathic Pain

- **Finalizing Plans to Conduct Phase 2a Trial in Diabetic Peripheral Neuropathic Pain (DPNP).** In the fourth quarter of 2024, the Company was notified by the FDA that the IND submitted by the Company for the initiation of a Phase 2a proof-of-concept trial of RAP-219 for the treatment of DPNP was placed on clinical hold, and the FDA requested additional information and protocol design amendments. The Company is now finalizing its trial plans with a goal of advancing work in this area of high unmet need as quickly as possible.

Corporate Updates

- **Appointment of Dr. Jeffrey Sevigny as Chief Medical Officer.** Dr. Sevigny is a physician-scientist with more than 15 years of leadership in translational and clinical drug development, including as chief medical officer at Prevail Therapeutics, a wholly owned subsidiary of Eli Lilly, and senior vice president of neuroscience at Eli Lilly. Dr. Sevigny has spearheaded groundbreaking research across neuroscience and rare diseases. His experience spans the full spectrum of development, from discovery to late-stage clinical trials and regulatory approvals. With a strong record of portfolio development and building high-performing organizations, he brings deep strategic and operational experience to Rapport.

FOURTH QUARTER AND YEAR-END 2024 FINANCIAL RESULTS

- **Net Loss:** Net Loss for the fourth quarter of 2024 was \$20.0 million, as compared to \$13.5 million for the prior year period. For the full year 2024, net loss was \$78.3 million, which compared to a net loss of \$34.8 million for the full year 2023.
- **Research and Development (R&D) Expenses:** R&D expense was \$17.2 million for the fourth quarter of 2024, as compared to \$11.8 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. For the full year 2024, R&D expense was \$60.9 million, which compared to \$28.0 million for the full year 2023.
- **General and Administrative (G&A) Expenses:** G&A expense was \$6.3 million for the fourth quarter of 2024, as compared to \$3.0 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. For the full year 2024, G&A expense was \$22.1 million, which compared to \$8.2 million for the full year 2023.
- **Cash Position:** The Company ended the fourth quarter with \$305.3 million in cash, cash equivalents and short-term investments, compared to \$320.7 million as of September 30, 2024. The decrease was primarily due to cash outflows on operating activities in the fourth quarter of 2024.
- **Cash Runway:** The Company expects that cash, cash equivalents, and short-term investments as of December 31, 2024 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 TARPγ8, 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 Registration Statement on Form S-1, 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. 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.

Rapport Therapeutics, Inc.
Consolidated Balance Sheet Data
(In thousands)
(unaudited)

	December 31, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	\$ 56,805	\$ 70,169
Short-term investments	248,475	77,309
Restricted cash	105	85
Prepaid expenses and other current assets	4,417	3,309
Total current assets	<u>309,802</u>	<u>150,872</u>
Property and equipment, net	3,529	1,916
Operating lease right of use asset, net	1,442	2,084
Other assets	160	551
Total assets	<u>\$ 314,933</u>	<u>\$ 155,423</u>
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		

Current liabilities		
Accounts payable	\$ 1,954	\$ 2,502
Accrued expenses and other current liabilities	6,076	5,631
Operating lease liability	737	670
Total current liabilities	<u>8,767</u>	<u>8,803</u>
Series B preferred stock tranche right liability	—	4,200
Operating lease liability, net of current portion	739	1,476
Total liabilities	<u>9,506</u>	<u>14,479</u>
Commitments and contingencies		
Series A convertible preferred stock	—	89,487
Series B convertible preferred stock	—	77,091
Stockholders' equity (deficit)		
Undesignated preferred stock	—	—
Common Stock	37	4
Additional paid-in capital	429,657	19,796
Accumulated other comprehensive income	(522)	4
Accumulated deficit	<u>(123,745)</u>	<u>(45,438)</u>
Total stockholders' equity (deficit)	<u>305,427</u>	<u>(25,634)</u>
Total liabilities, convertible preferred stock, and stockholders' equity	<u>\$ 314,933</u>	<u>\$ 155,423</u>

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

	<u>For the three months ended</u>		<u>For the year ended</u>	
	<u>December 31,</u>		<u>December 31,</u>	
	<u>2024</u>	<u>2023</u>	<u>2024</u>	<u>2023</u>
Operating expenses				
Research and development	\$ 17,199	\$ 11,799	\$ 60,935	\$ 27,999
General and administrative	6,322	2,995	22,120	8,180
Total operating expenses	<u>23,521</u>	<u>14,794</u>	<u>83,055</u>	<u>36,179</u>
Loss from operations	(23,521)	(14,794)	(83,055)	(36,179)
Other income (expense):				
Interest income	3,541	1,375	12,138	2,527
Change in fair value of preferred stock tranche right liability	—	(94)	(7,390)	(1,124)
Total other income, net	<u>3,541</u>	<u>1,281</u>	<u>4,748</u>	<u>1,403</u>
Net loss before income taxes	(19,980)	(13,513)	(78,307)	(34,776)
Provision for income taxes	—	6	—	10
Net loss	<u>(19,980)</u>	<u>\$ (13,519)</u>	<u>\$ (78,307)</u>	<u>\$ (34,786)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (0.57)</u>	<u>\$ (8.01)</u>	<u>\$ (3.78)</u>	<u>\$ (23.10)</u>
Weighted-average common shares outstanding, basic and diluted	<u>35,069,296</u>	<u>1,687,087</u>	<u>20,738,338</u>	<u>1,505,774</u>

Rapport Therapeutics, Inc.
Condensed Consolidated Statements of Cash Flows
(In thousands)
(unaudited)

	<u>For the three months ended</u>		<u>For the year ended</u>	
	<u>December 31,</u>		<u>December 31,</u>	
	<u>2024</u>	<u>2023</u>	<u>2024</u>	<u>2023</u>

Net cash used in operating activities	\$ (14,446)	\$ (10,357)	\$ (64,828)	\$ (27,181)
Net cash used in investing activities	31,930	(78,661)	(170,141)	(78,860)
Net cash provided by financing activities	<u>7</u>	<u>(140)</u>	<u>221,625</u>	<u>145,136</u>
Net increase in cash, cash equivalents and restricted cash	<u>\$ 17,491</u>	<u>\$ (89,158)</u>	<u>\$ (13,344)</u>	<u>\$ 39,095</u>

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