Rapport Therapeutics Reports Third Quarter Financials and Provides Business Update

November 7, 2024

- *Biotech industry leaders added to the Board of Directors, bringing deep expertise in drug discovery, neuroscience clinical development, and operational leadership*
- *RAP-219 MAD-2 and PET trials ongoing; topline data expected in Q1 2025*
- *Actively recruiting patients for Phase 2a trial of RAP-219 in focal epilepsy; trial on track and topline data expected in mid-2025*
- *Investigational New Drug Application (IND) in diabetic peripheral neuropathic pain placed on clinical hold while the Company addresses U.S. Food and Drug Administration (FDA) Phase 2a trial protocol feedback*
- *Ended the quarter with $320.7 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, Nov. 07, 2024 (GLOBE NEWSWIRE) -- Rapport Therapeutics, Inc. (Nasdaq: RAPP), a clinical-stage biotechnology company focused on discovery and development of small molecule precision medicines for patients suffering from central nervous system (CNS) disorders, today announced financial results for the third quarter of 2024 and provided a business update.

"We are pleased with the progress we're making with RAP-219, particularly as we continue the execution of our Phase 2a proof-of-concept trial in focal epilepsy," said Abraham N. Ceesay, chief executive officer of Rapport. "The continued learnings from our ongoing clinical activities, including the MAD-2 and PET trials as well as our pharmaceutical development efforts, have only strengthened our confidence in the pipeline-in-a-product potential of RAP-219 as a potentially transformational treatment for focal epilepsy, peripheral neuropathic pain, and bipolar disorder."

BUSINESS UPDATES

Board of Directors

The Company announced the appointment of new members to its Board of Directors to help guide its next phase of growth and innovation, including Rob Perez, operating partner at General Atlantic and former chief executive officer of Cubist Pharmaceuticals; Raymond Sanchez, MD, former chief medical officer of Cerevel Therapeutics; Paul Silva, former chief accounting officer at Vertex Pharmaceuticals; and Wendy B. Young, PhD, former head of small molecule drug discovery at Genentech. With these appointments, Jeff Tong, PhD, partner at Third Rock Ventures, has resigned from the Board of Directors.

"On behalf of the entire Board of Directors, I extend our deep appreciation to Jeff for his incredible leadership in helping build Rapport from its inception," said Steve Paul MD, founder and chair of Rapport's Board of Directors. "We are pleased to welcome additional experienced biotech leaders to our board who will provide significant scientific, medical, and business expertise to help us realize the full potential of our science for patients and shareholders alike. With the extensive experience and accomplishments of Rob, Ray, Paul, and Wendy, together with our existing board members, we are well positioned for future success."

"I want to express my sincere gratitude to Jeff for his guidance and leadership in helping us launch and shape Rapport," said Ceesay. "It's a true honor to welcome such exceptional industry leaders to our Board of Directors. They bring a wealth of knowledge and experience in scaling biotech organizations, and their deep understanding of neuroscience drug development, operational excellence, and innovation will be instrumental in driving the Company's growth and long-term success. Their joining the Board of Directors is a testament to the promise of the science of receptor associated proteins and RAP-219."

Perez is currently an operating partner at General Atlantic. Previously, he was the chief executive officer of Cubist Pharmaceuticals where he led the launch of Cubicin® as well as multiple acquisitions and international expansion efforts leading up to its sale to Merck in 2015. Prior to that, Perez served as vice president of Biogen's US CNS business unit, where he was responsible for commercial leadership of an $800 million business. Perez is also the founder and chairman of Life Science Cares, and the co-founder of Biopharma Leaders of Color.

Sanchez is a psychiatrist with over 20 years of experience in academia, medicine, and the pharmaceutical industry, specializing in areas including CNS and analgesia. He served as the chief medical officer of Cerevel Therapeutics until its acquisition by AbbVie in 2024 and is now a senior advisor at Bain Capital Life Sciences among other roles. Sanchez has a proven track record in global asset development, leading to regulatory approvals for multiple compounds, including Abilify®. He has also played key roles in business development, capital formation, regulatory interactions, and strategic oversight of global medical portfolios across various therapeutic areas.

An experienced finance and operations executive, Silva played a key role in transforming Vertex Pharmaceuticals into a

high-growth company with over $6 billion in annual revenue. During his 15-year tenure, he led Vertex's accounting, tax, and treasury functions, supporting global growth and critical business development initiatives, including key international reimbursement agreements for its cystic fibrosis medicines. Silva also contributed to business development transactions that enabled Vertex to advance into genetic and cell therapies. Additionally, Silva was an inaugural Board Member of the Vertex Foundation and founding executive sponsor of Vertex “PRIDE.”

Young is a biotechnology and life science executive with over 30 years of experience in drug discovery and development. As former senior vice president of small molecule drug discovery at Genentech, she oversaw the advancement of over 25 clinical candidates in oncology, immunology, neurology, and anti-infectives. Notably, she led the BTK program and co-invented fenebrutinib. Young was recently inducted into the American Chemistry Society Hall of Fame, and she has also been named “One of the Top 20 Women in Biopharma” by *Endpoints News* and “Most Influential Women in SF Bay Area” by the *San Francisco Times*.

RAP-219 Lead Program

RAP-219 is designed to selectively target TARPγ8, a receptor-associated protein (RAP) which is associated with the neuronal AMPAR (neuronal α-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid receptor), a clinically validated target for epilepsy. The Company is also evaluating RAP-219 as a potential treatment for peripheral neuropathic pain and bipolar disorder.

- The Company is conducting a second multiple ascending dose trial (MAD-2) of RAP-219, expected to be completed in Q4 2024. The trial will assess additional dosing regimens specifically to inform dosing for its Phase 2a trial for the treatment of bipolar acute mania. The initial MAD trial demonstrated that RAP-219 was generally well tolerated at target therapeutic exposures, with no serious adverse events and no drug-related treatment-emergent adverse events (TEAEs) above Grade 1.
- A Phase 1 human positron emission tomography (PET) trial in healthy adult volunteers is currently underway, utilizing a companion PET radiotracer to confirm brain target receptor occupancy and brain region specificity across a range of RAP-219 dosing and exposure levels.
- Topline results from both the MAD-2 and PET trials will be released in Q1 2025.
- The Company continues to progress the development of a long-acting injectable formulation of RAP-219 as the first potential anti-seizure medication (ASM) in a depot formulation, offering greater ease-of-use and potentially improved patient adherence.

<u>Focal Epilepsy</u>

- Patient recruitment and screening is underway for the Company’s Phase 2a proof of concept trial in focal epilepsy. The trial is on track, and topline results are expected in mid-2025.
    - The Phase 2a trial is enrolling adult patients with drug-resistant focal epilepsy who have an implanted responsive neurostimulation (RNS) device and are demographically similar to those expected in future registrational trials.
    - The RNS device continually captures intracranial electroencephalography data and records the frequency of long episodes (LEs), which are often referred to as subclinical seizures and serve as a biomarker to clinical seizures. LEs function as a biomarker-based endpoint, supporting more efficient and objective efficacy results. After the initiation of new antiseizure medication, a 30% reduction of LEs has been shown to predict meaningful changes (≥ 50% reduction) in clinical seizure frequency.
- At the Annual Epilepsy Society meeting in December, the Company will have four poster presentations focused on RAP-219, highlighting preclinical data, SAD/MAD trial results, food effect data, and new analyses on the correlation between RNS-measured long episode and clinical seizure frequency in adult patients with refractory focal epilepsy.
- In September, the Company presented on the underlying mechanism of action for RAP-219 as well as the novel design of the Phase 2a proof-of-concept trial for the treatment of focal epilepsy at the International League Against Epilepsy 15th Annual European Epilepsy Congress and the 2024 Epilepsy Foundation Pipeline Conference.

<u>Peripheral Neuropathic Pain</u>

- The Company was recently 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 diabetic peripheral neuropathic pain (DPNP) was placed on clinical hold. The FDA requested additional information and amendments specific to the protocol design. The clinical hold is specific to the IND for DPNP and has not impacted the Company’s ongoing Phase 2a trial in focal epilepsy or planned proof-of-concept trial in bipolar disorder. The Company believes in its ability to resolve the clinical hold in DPNP and will provide an update on the anticipated timing of the Phase 2a trial initiation once available.

<u>Bipolar Disorder</u>

- The Company plans to initiate a Phase 2a trial in bipolar disorder patients with acute mania in 2025.

Preclinical and Discovery Programs

- With growing confidence in RAP-219 and a commitment to disciplined capital allocation, the Company is deferring further investment in RAP-199 and focusing resources on execution of its three RAP-219 proof-of-concept clinical trials.
- The Company continues to advance its RAP-enabled nicotinic acetylcholine receptor (nAChR) discovery-stage programs – modulators of a6 nAChR for the potential treatment of chronic pain, and modulators of a9a10 nAChR for the potential treatment of hearing loss.

THIRD QUARTER 2024 FINANCIAL RESULTS

- Net loss was $17.5 million for the third quarter of 2024, as compared to $8.7 million for the prior year period.
- Research and development expense was $15.5 million for the third quarter of 2024, as compared to $7.6 million for the prior year period. The increase in research and development 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 expense was $6.1 million for the third quarter of 2024, as compared to $2.0 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 becoming and operating as a public company.
- The Company ended the third quarter with $320.7 million in cash, cash equivalents and short-term investments, compared to $336.1 million as of June 30, 2024. The decrease was primarily due to cash outflows on operating activities in the third quarter of 2024.
- The Company expects that current cash, cash equivalents, and short-term investments as of September 30, 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 AMPAR (α-amino-3-hydroxy-5-methyl-4-isoxazolepropionic acid receptor) negative allosteric modulator (NAM) designed to achieve neuroanatomical specificity through its selective targeting of a RAP known as TARPγ8, which is associated with the neuronal AMPAR. Whereas AMPARs are distributed widely in the central nervous system (CNS), TARPγ8 is expressed only in discrete regions, including the hippocampus and cortex. Because of this restricted expression of TARPγ8 in forebrain regions, the Company believes RAP-219 has the potential to provide a differentiated clinical profile, including improved activity and tolerability along with a higher therapeutic index, potentially providing more patients with sustained therapeutic benefit without intolerable side effects, as compared to traditional neuroscience medications. Due to the role of AMPA biology in various neurological disorders and the precision approach of selective targeting of TARPγ8, the Company believes RAP-219 has significant pipeline-in-a-product potential and is currently evaluating the compound as a transformational treatment for patients with focal epilepsy, peripheral neuropathic pain, and bipolar disorder.

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 suffering from central nervous system (CNS) 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 clinical program, 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 advancing RAP-219 in clinical trials in focal epilepsy, peripheral neuropathic pain, and bipolar disorder. Additional preclinical and late-stage discovery stage programs are also underway, targeting CNS disorders including 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; the ongoing and planned development of RAP-199 and Rapport's discovery-stage programs; 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 (the SEC). 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.

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

	September 30, 2024	December 31, 2023
Assets		
Current assets		
Cash and cash equivalents	$ 39,314	$ 70,169
Short-term investments	281,347	77,309
Restricted cash	105	85
Prepaid expenses and other current assets	5,173	3,309
Total current assets	325,939	150,872
Property and equipment, net	3,409	1,916
Operating lease right of use asset, net	1,607	2,084
Other assets	189	551
Total assets	$ 331,144	$ 155,423
Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)		
Current liabilities		
Accounts payable	$ 1,323	$ 2,502
Accrued expenses and other current liabilities	5,056	5,631
Operating lease liability	720	670
Total current liabilities	7,099	8,803
Series B preferred stock tranche right liability	—	4,200
Operating lease liability, net of current portion	931	1,476
Total liabilities	8,030	14,479
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	36	4
Additional paid-in capital	426,443	19,796
Accumulated other comprehensive income	400	4
Accumulated deficit	(103,765)	(45,438)
Total stockholders' equity (deficit)	323,114	(25,634)
Total liabilities, convertible preferred stock, and stockholders' equity	$ 331,144	$ 155,423

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

	For the three months ended September 30,	
	2024	2023
Operating expenses		
Research and development	$ 15,543	$ 7,580
General and administrative	6,097	1,984
Total operating expenses	21,640	9,564
Loss from operations	(21,640)	(9,564)
Other income (expense):		
Interest income	4,103	856
Change in fair value of preferred stock tranche right liability	—	—
Total other income, net	4,103	856
Net loss before income taxes	(17,537)	(8,708)
Provision for income taxes	—	1
Net loss	$ (17,537)	$ (8,709)
Net loss per share attributable to common stockholders, basic and diluted	$ (0.50)	$ (5.70)
Weighted-average common shares outstanding, basic and diluted	34,855,907	1,529,216

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

	For the Three Months Ended September 30,	
	2024	2023
Net cash used in operating activities	$ (16,415)	$ (5,424)
Net cash used in investing activities	(53,041)	(52)
Net cash provided by (used in) financing activities	(1,394)	85,360
Net increase in cash, cash equivalents and restricted cash	$ (70,850)	$ 79,884

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