Recursion Announces Phase 2 Data of REC-994, a First-in-Disease Investigational Treatment for Symptomatic Cerebral Cavernous Malformation (CCM), has Met its Primary Endpoint of Safety and Tolerability

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REC-994 also demonstrates encouraging trends in objective MRI-based exploratory efficacy measures at the highest dose and the company plans to advance development of REC-994 for the potential treatment of symptomatic CCM in subsequent studies.

SALT LAKE CITY, Sept. 03, 2024 (GLOBE NEWSWIRE) -- Recursion (NASDAQ: RXRX), a leading clinical stage TechBio company decoding biology to radically improve lives, today announced top-line results of the SYCAMORE trial, a 12-month Phase 2 randomized double-blind, placebo-controlled, safety, tolerability and exploratory efficacy study for REC-994 in symptomatic CCM patients.

REC-994 met its primary endpoint of safety and tolerability, demonstrating a similar profile across placebo and both 200mg and 400mg dosage-arms with regard to the frequency and severity of adverse events after 12 months of treatment. Magnetic resonance imaging-based secondary efficacy endpoints showed a trend towards reduced lesion volume and hemosiderin ring size in patients at the highest dose (400mg) as compared to placebo. Time-dependent improvement in these trends at the 400mg dose was also observed in this signal-finding study. Improvements in either patient or physician-reported outcomes were not yet seen at the 12 month time point. A meeting with the FDA is anticipated as soon as practical to discuss plans for an additional clinical study. Recursion plans to present data from this trial at a forthcoming medical conference and intends to submit these data for publication in a peer reviewed scientific journal.

"These studies are making significant strides in the development of therapeutics for CCM. The data from this readout is an impressive start and will provide a valuable contribution to the existing CCM literature and strongly supports the need for a future study, with a longer duration and a larger patient cohort," said Dr. Jan-Karl Burkhardt, MD, Division Head, Cerebrovascular Surgery, University of Pennsylvania and Principal Investigator of the study. Connie Lee, Psy.D., founder and CEO of the Alliance to Cure Cavernous Malformation added: “I speak for the patients who have participated in the trial and those who have been cheering from the sidelines while waiting for news. This promising start is a critical step forward and will bring hope to thousands of families who currently have no options but brain or spinal cord surgery. The Alliance to Cure Cavernous Malformation looks forward to partnering with Recursion as they move to the next stage of the REC-994 program.”

“We are encouraged by the recent data from our signal-finding Phase 2 study in CCM, where the trial successfully met its primary safety endpoint and became the first investigational therapy to demonstrate safety alongside some promising trends in exploratory efficacy endpoints. These results provide critical insights that will inform our next study design, including exploring study duration, higher doses, and a larger cohort of patients," said Najat Khan, Ph.D., Chief R&D Officer and Chief Commercial Officer of Recursion. "This is the first of several key clinical readouts for the company and represents an early proof-of-platform milestone for our constantly evolving Recursion OS, as we build upon our success in drug discovery with expertise and execution in mid-phase development. We are deeply grateful to the patients and investigators, and we are committed to advancing potential transformational therapies for CCM and beyond.”

Background on Cerebral Cavernous Malformation (CCM)

CCM is a neurovascular condition that impacts approximately 360,000 symptomatic individuals in the US and EU5. The disease is often underdiagnosed and potentially affects over 1 million patients worldwide. CCM manifests as vascular malformations of the spinal cord and brain characterized by abnormally enlarged capillary cavities without intervening brain parenchyma. Patients with CCM lesions are at substantial risk for seizures, headaches, progressive neurological deficits, and potentially fatal hemorrhagic stroke. Currently, only non-pharmacologic treatments including microsurgical resection and stereotactic radiosurgery are available options for this high unmet need patient population. However, surgical resection or stereotactic radiosurgery is not always feasible because of location and may not be curative.

About REC-994

REC-994 is an orally bioavailable, superoxide scavenger small molecule under development for the treatment of symptomatic CCM. The potential of REC-994 in CCM was demonstrated using the earliest version of what would become the foundational technology underlying the Recursion OS. Subsequently, REC-994 demonstrated preclinical activity in models for CCM and tolerability and suitability for chronic dosing in Phase 1 single ascending dose escalation (SAD) and multiple ascending dose escalation (MAD) trials in healthy volunteers directed and executed by Recursion. Recursion has sought and received Orphan Drug Designation for REC-994 in symptomatic CCM in the US and Europe.

About the Trial

Our Phase 2 SYCAMORE clinical trial is a randomized, double-blind, placebo-controlled study of two doses of REC-994 in participants with CCM. The primary endpoint of the study is safety and tolerability. Secondary efficacy endpoints include MRI-based endpoints, clinician and patient reported outcomes, as well as selected biomarkers. This trial was fully enrolled in June 2023 with 62 participants, and 80% of participants who completed 12 months of treatment have entered the long-term extension study. This signal-finding study was not powered to demonstrate statistical significance.

About Recursion

Recursion (NASDAQ: RXRX) is a clinical stage TechBio company leading the space by decoding biology to radically improve lives. Enabling its mission is the Recursion OS, a platform built across diverse technologies that continuously generate one of the world’s largest proprietary biological and chemical datasets. Recursion leverages sophisticated machine-learning algorithms to distill from its dataset a collection of trillions of searchable

relationships across biology and chemistry unconstrained by human bias. By commanding massive experimental scale — up to millions of wet lab experiments weekly — and massive computational scale — owning and operating one of the most powerful supercomputers in the world, Recursion is uniting technology, biology and chemistry to advance the future of medicine.

Recursion is headquartered in Salt Lake City, where it is a founding member of BioHive, the Utah life sciences industry collective. Recursion also has offices in Toronto, Montréal, London, and the San Francisco Bay Area. Learn more at www.Recursion.com, or connect on X (formerly Twitter) and LinkedIn.

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Forward-Looking Statements

This document contains information that includes or is based upon "forward-looking statements" within the meaning of the Securities Litigation Reform Act of 1995, including, without limitation, those regarding Recursion's anticipated meeting with the FDA; Recursion's plans to present SYCAMORE trial data at a medical conference and submit the data for publication; the clinical relevance of the SYCAMORE trial data and obtaining additional confirmatory data; promising trends in REC-994 efficacy endpoints; advancing potential transformational therapies for CCM and beyond; subsequent REC-994 studies and their results and advancing Recursion's REC-994 program further; the size of the potential CCM patient population; Recursion OS and other technologies potential and advancement of the future of medicine; business and financial plans and performance; and all other statements that are not historical facts. Forward-looking statements may or may not include identifying words such as "plan," "will," "expect," "anticipate," "intend," "believe," "potential," "continue," and similar terms. These statements are subject to known or unknown risks and uncertainties that could cause actual results to differ materially from those expressed or implied in such statements, including but not limited to: challenges inherent in pharmaceutical research and development, including the timing and results of preclinical and clinical programs, where the risk of failure is high and failure can occur at any stage prior to or after regulatory approval due to lack of sufficient efficacy, safety considerations, or other factors; our ability to leverage and enhance our drug discovery platform; our ability to obtain financing for development activities and other corporate purposes; the success of our collaboration activities; our ability to obtain regulatory approval of, and ultimately commercialize, drug candidates; our ability to obtain, maintain, and enforce intellectual property protections; cyberattacks or other disruptions to our technology systems; our ability to attract, motivate, and retain key employees and manage our growth; inflation and other macroeconomic issues; and other risks and uncertainties such as those described under the heading "Risk Factors" in our filings with the U.S. Securities and Exchange Commission, including our most recent Annual Report on Form 10-K, our subsequent Quarterly Reports on Form 10-Q, and our Current Reports on Form 8-K. All forward-looking statements are based on management's current estimates, projections, and assumptions, and Recursion undertakes no obligation to correct or update any such statements, whether as a result of new information, future developments, or otherwise, except to the extent required by applicable law.

Source: Recursion Pharmaceuticals, Inc.