CervoMed Announces Orphan Drug Designation Granted to Neflamapimod by U.S. Food and Drug Administration for the Treatment of Frontotemporal Dementia

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Designation underscores significant unmet need in frontotemporal dementia and the potential role of neflamapimod in multiple neurologic disorders

On track to report topline data from the RewinD-LB Phase 2b clinical trial in early-stage dementia with Lewy bodies (DLB) in December 2024

BOSTON, Nov. 27, 2024 (GLOBE NEWSWIRE) -- CervoMed Inc. (NASDAQ: CRVO), a clinical-stage company focused on developing treatments for age-related neurologic disorders, today announced that its oral investigational drug neflamapimod has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) for the treatment of frontotemporal dementia (FTD).

"We are pleased to have received Orphan Drug Designation as it implicitly recognizes the scientific rationale and potential for neflamapimod to treat this debilitating condition. Patients diagnosed with frontotemporal dementia have no available treatment options, and this rare condition is extremely burdensome to patients and caregivers alike," said John Alam, MD, Chief Executive Officer of CervoMed. "Within this year, there have been multiple scientific presentations and publications that indicate neflamapimod targets specific pathogenic mechanisms associated with FTD. We are in active discussions with clinical thought leaders regarding the design of a proof-of-principle study in FTD, while continuing to prepare to advance neflamapimod into a Phase 3 trial in DLB in mid-2025."

FDA Orphan Drug Designation is granted to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the United States. Importantly, the FDA also requires sufficient preclinical and/or clinical data to establish a medically plausible basis for expecting the drug to be effective in the rare disease for which orphan drug status is granted. Orphan Drug status provides benefits to drug developers, including assistance in the drug development process, tax credits for clinical costs, exemptions from certain FDA fees and seven years of post-approval marketing exclusivity.

About Frontotemporal Dementia
FTD is a rare neurodegenerative disease, but it is one of the most common causes of early onset dementia. It affects an estimated 50,000 to 60,000 people in the United States and roughly 110,000 in the European Union, with potentially higher prevalence in Asia and Latin America. Pathologically FTD is characterized by significant neuronal loss (i.e. atrophy by MRI or at autopsy) in the frontal and temporal regions of the cortex. The pathogenic drivers underlying the neuronal loss are in most cases, in roughly equal proportions, intraneuronal inclusions in those two regions of the brain containing either the protein tau or TDP-43 (transactive response DNA binding protein); intranuclear inclusions of fused in sarcoma (FUS) are also seen, but in fewer than 10% of patients. Patients with FTD frequently develop symptoms such as behavioral changes, lapses in judgment, and diminished language skills when they are in their 40s and 50s with the disease running its course in 7-10 years. There are no FDA- or EMA-approved treatment options available for any form of FTD.

About Neflamapimod
Neflamapimod is an investigational, orally administered small molecule brain penetrant drug that inhibits alpha isoform of the p38MAP kinase. In preclinical studies, neflamapimod reversed synaptic dysfunction, including and particularly within the part of the brain most impacted in DLB – the basal forebrain cholinergic system. In Phase 1 and Phase 2 clinical studies involving more than 300 participants, neflamapimod has been shown to be generally well tolerated. Results from the AscenD-LB Phase 2a clinical study demonstrated that neflamapimod significantly improved dementia severity (assessed by Clinical Dementia Rating Sum-of-boxes, or CDR-SB) compared to placebo and significantly improved functional mobility (assessed by Timed Up and Go Test, or TUG test) compared to placebo. At the highest dose evaluated, neflamapimod also improved results on a cognitive test battery. The treatment response in AscenD-LB in patients with early-stage DLB (i.e., those without biomarker evidence of tau pathology in the brain) was substantial (effect size > 0.7) and greater than the overall patient population. Neflamapimod is currently being evaluated in the ongoing RewinD-LB Phase 2b study, a randomized, 16-week, double-blind, placebo-controlled clinical trial evaluating oral neflamapimod (40mg TID) in 159 patients with early-stage DLB. Topline results from the RewinD-LB study are expected in December 2024.

About CervoMed

CervoMed Inc. (the "Company") is a clinical-stage company focused on developing treatments for age-related neurologic disorders. The Company is currently developing neflamapimod, an investigational, orally administered small molecule brain penetrant that inhibits p38 mitogen-activated protein kinase alpha. Neflamapimod has the potential to treat synaptic dysfunction, the reversible aspect of the underlying neurodegenerative processes that causes disease in DLB and certain other major neurological disorders. Neflamapimod is currently being evaluated in a Phase 2b study in patients with early-stage DLB.

Forward-Looking Statements

This press release includes express and implied forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, regarding the intentions, plans, beliefs, expectations or forecasts for the future of the Company, including, but not limited to, the therapeutic potential of neflamapimod and the anticipated timing and achievement of clinical and development milestones, including neflamapimod's potential as a treatment for FTD, the completion and achievement of primary endpoints of the RewinD-LB Phase 2b clinical trial, the Company's announcement of topline or other data therefrom, and the Company's intent to initiate a Phase 3 trial in DLB in mid-2025 assuming positive topline results in the RewinD-LB trial. Terms such as "believes," "estimates," "anticipates," "expects," "plans," "aims," "seeks," "intends," "may," "might," "could," "might," "will," "should," "approximately," "potential," "target," "project," "contemplate," "predict," "forecast," "continue," or other words that convey uncertainty of future events or outcomes (including the negative of these terms) may identify these forward-looking statements. Although there is believed to be reasonable basis for each forward-looking statement contained herein, forward-looking statements by their nature involve risks and uncertainties, known and unknown, many of which are beyond the Company's control and, as a result, actual results could differ materially from those expressed or implied in any forward-looking statement. Particular risks and uncertainties include, among other things, those related to: the Company's available cash resources and the availability of additional funds on acceptable terms; the results of the Company's clinical trials, including RewinD-LB;

the likelihood and timing of any regulatory approval of neflamapimod or the nature of any feedback the Company may receive from the U.S. Food and Drug Administration; the ability to implement business plans, forecasts, and other expectations in the future; general economic, political, business, industry, and market conditions, inflationary pressures, and geopolitical conflicts; and the other factors discussed under the heading “Risk Factors” in the Company’s Annual Report on Form 10-K for the year ended December 31, 2023 filed with the U.S. Securities and Exchange Commission (SEC) on March 29, 2024, and other filings that the Company may file from time to time with the SEC. Any forward-looking statements in this press release speak only as of the date hereof (or such earlier date as may be identified). The Company does not undertake any obligation to update such forward-looking statements to reflect events or circumstances after the date of this press release, except to the extent required by law.

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