resTORbio Announces Interim Results for Phase 1b/2a Trial of RTB101 in Patients with Parkinson's Disease and Provides Corporate Update

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-- Interim data from three cohorts in the Phase 1b/2a study demonstrate that RTB101 is well tolerated, crosses the blood brain barrier, and reaches concentrations in cerebrospinal fluid observed in preclinical models to inhibit the activity of TORC1 and induce autophagy in neuronal cells --

-- TORC1 Inhibition in the brain with RTB101 may offer a new treatment paradigm for multiple neurodegenerative diseases associated with the accumulation of protein aggregates, such as Parkinson's, Huntington's and Alzheimer's disease --

BOSTON, Feb. 19, 2020 (GLOBE NEWSWIRE) -- resTORbio (Nasdaq: TORC) today announced interim results from the ongoing Phase 1b/2a trial of RTB101, an orally-administered small molecule potent target of rapamycin complex 1 (TORC1) inhibitor product candidate, alone or in combination with sirolimus, in Parkinson's disease (PD).

"We are pleased to have observed that RTB101 is well tolerated and crosses the blood brain barrier in Parkinson's disease patients at concentrations that have the potential to induce autophagy, the process by which cells break down toxic misfolded protein aggregates. Preclinical data suggest that induction of autophagy has the potential to slow the progression not only of Parkinson's disease but also of multiple other neurodegenerative diseases that are characterized by the accumulation of toxic protein aggregates in cells such as Huntington's and Alzheimer's disease," said Dr. Joan Mannick, Co-Founder and Chief Medical Officer of resTORbio.

Phase 1b/2a Trial of RTB101 alone and in combination with sirolimus in Parkinson's disease interim results
The multicenter, 2:1 randomized, double-blind, placebo-controlled Phase 1b/2a trial is evaluating the safety and tolerability of RTB101 alone or in combination with escalating doses of sirolimus (2 mg, 4 mg and 6 mg) once weekly for 4 weeks in patients with Parkinson's disease. To date, patients have been enrolled in three cohorts and dosed once weekly with 300 mg of RTB101 alone, 2 mg of sirolimus alone, or a combination of 300 mg RTB101 and 2 mg of sirolimus. Results of the interim study analysis indicated that all 3 dosing regimens were well tolerated and RTB101 300 mg once weekly was observed to cross the blood brain barrier. The concentrations of RTB101 in cerebrospinal fluid (CSF) in subjects dosed with RTB101 300 mg once weekly monotherapy were higher than expected and based on preclinical models, have the potential to induce autophagy in the brain. Sirolimus at the dose of 2 mg, alone or in combination with RTB101, was not detected in the CSF. Data from the first three cohorts in the study suggest that the concentrations of RTB101 observed in the CSF four hours after dosing were highest when RTB101 was given as a monotherapy. Enrollment of the RTB101 300 mg in combination with sirolimus 4 mg once weekly cohort is ongoing.

Corporate Update
The company has initiated a process to evaluate external opportunities, such as partnerships, acquisitions, mergers and other financial and strategic alternatives to maximize shareholder value. The company has engaged JMP Securities LLC to act as a strategic advisor for this process. There can be no assurance that this strategic review process will result in the company pursuing any transaction or that any transaction, if pursued, will be completed. The company has not set a timetable for completion of this strategic review process, and the company does not intend to comment further unless or until its Board of Directors has approved a definitive course of action, the review process is concluded, or it is determined other disclosure is appropriate.

About Parkinson's Disease
Parkinson's disease (PD) is a progressive neurodegenerative disease that affects approximately 7.5 million people worldwide. The incidence of PD increases rapidly in people 60 years of age and older, with a mean age at diagnosis of 70.5 years. Inhibition of TORC1 has been shown in multiple preclinical models to ameliorate neurodegenerative diseases including Parkinson's disease. TORC1 inhibition with RTB101 alone or in combination with sirolimus, may provide a therapeutic benefit to PD patients by ameliorating levodopa-induced dyskinesia and/or by inducing autophagy which leads to the breakdown of protein aggregates and improved neuronal survival.

About RTB101
RTB101 is an oral, selective, and potent TORC1 inhibitor product candidate that inhibits the phosphorylation of multiple targets downstream of TORC1. Inhibition of TORC1 has been observed to extend lifespan and healthspan in aging preclinical species and to improve the function of aging organ systems, including neurologic function, suggesting potential benefits in several aging-related diseases.

About resTORbio
resTORbio, Inc. is a clinical-stage biopharmaceutical company developing innovative medicines that target the biology of aging to treat aging-related diseases. resTORbio's lead program selectively inhibits TORC1, an evolutionarily conserved pathway that contributes to the decline in function of aging organ systems, including neurologic function. Learn more about resTORbio, Inc. at www.resTORbio.com.

Forward Looking Statements
This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended. Investors are cautioned that statements in this press release which are not strictly historical statements, including, without limitation, our proposed timing and anticipated results of our Phase 1b/2a clinical trial of RTB101 alone or in combination with sirolimus in patients with mild to severe Parkinson's disease, including the announcement of interim results; our future plans to develop RTB101 alone or in combination with rapalogs, such as everolimus or sirolimus, including the therapeutic potential and clinical benefits thereof; our expectations on the potential patient populations that may be addressed by our product candidates; our ability to replicate results achieved in our clinical trials in any future trials; and our engagement of JMP Securities LLC and our plans to explore and evaluate strategic alternatives and external opportunities, constitute forward-looking statements identified by words such as, but not limited to, "believe," "expect," "may," "will," "should," "seek," "anticipate," or "could" and similar words or expressions.

Any forward-looking statements in this statement are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially from those anticipated, including, without limitation, risks associated with: our ongoing Phase 1b/2a clinical trial of RTB101 alone or in combination with sirolimus in Parkinson's disease, including the announcement of interim results; the timing and anticipated results of our clinical trials; the risk that the results of our clinical trials will be predictive of future results in connection with future clinical trials; our ability to explore and evaluate strategic alternatives and external opportunities, the timing and outcome of our planned interactions with regulatory authorities; and obtaining, maintaining and protecting our intellectual property as well as those risks more fully discussed in the section entitled "Risk Factors" in the Annual Report on Form 10-K filed by resTORbio, Inc. with the Securities and Exchange Commission, as well as discussions of potential risks, uncertainties, and other important factors in our subsequent filings with the Securities and Exchange Commission. In addition, any forward-looking statements are neither historical facts nor assurances of future performance. Instead, they represent our beliefs, expectations, assumptions and views only as of today and should not be relied upon as representing our beliefs, expectations, assumptions and views as of any subsequent date. resTORbio explicitly disclaims any obligation to update any forward-looking statements. No representations or warranties (expressed or implied) are made about the accuracy of any such forward-looking statements.

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