resTORbio Announces Initiation of Phase 1b/2a Trial of RTB101 in Parkinson's Disease

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BOSTON, April 02, 2019 (GLOBE NEWSWIRE) -- resTORbio, Inc. (Nasdaq: TORC), a clinical-stage biopharmaceutical company developing innovative medicines that target the biology of aging to prevent or treat age-related diseases, today announced the initiation of a Phase 1b/2a trial of RTB101, its orally administered, small molecule, potent inhibitor of target of rapamycin complex 1 (TORC1) product candidate, alone or in combination with sirolimus, in Parkinson's disease (PD).

"We believe that TORC1 may be an important therapeutic target for several neurodegenerative diseases associated with aging, in which misfolded proteins aggregate and cause neuronal toxicity. As such, we are excited to initiate our first clinical trial in PD and expand our pipeline into neurodegenerative disease," said Chen Schor, Co-Founder, President and CEO of resTORbio. "Multiple preclinical models of PD have demonstrated the potential benefits of TORC1 inhibition, and intermittent TORC1 inhibition with a synergistic combination of RTB101 and sirolimus may serve as a promising approach for the treatment of PD. We look forward to reporting data from this trial in 2020."

Selective and broad inhibition of TORC1 has been shown to ameliorate neurodegenerative disease in several preclinical studies across multiple species, including models of PD. TORC1 inhibition with RTB101 in combination with sirolimus, a rapalog, may provide a therapeutic benefit to PD patients by potentially inducing autophagy to clear protein aggregates in neurons, increasing lysosomal biogenesis and decreasing glucosylceramide (GL1) synthesis.

Phase 1b/2a Trial Design
The four-week, 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 sirolimus in PD. The Company plans to enroll 45 patients with mild PD who are already on standard-of-care therapy, including those with and without glucocerebrosidase (GBA) mutations. Patients are expected to be enrolled into five cohorts and dosed once-weekly with RTB101 300mg alone or in combination with three dose levels of sirolimus (2 mg, 4 mg and 6 mg). The planned primary endpoint of the trial is safety and tolerability, and secondary endpoints will include exposure in blood, plasma and cerebrospinal fluid (CSF). The planned exploratory endpoints include biomarkers in plasma and CSF, and various clinical assessments. Data from this trial is expected in 2020.

About Parkinson's disease
Parkinson's disease, or 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. GBA gene mutations are the most common of the currently known PD genetic mutations and up to 10 percent of people with PD in the United States carry it.

About resTORbio
resTORbio, Inc. is a clinical stage biopharmaceutical company developing innovative medicines that target the biology of aging to prevent or treat age-related diseases. resTORbio's lead program selectively inhibits TORC1, an evolutionarily conserved pathway that contributes to the decline in function of multiple organ systems, including the immune, cardiovascular and central nervous systems.

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, express or implied statements or guidance regarding our proposed timing and trial design for our Phase 1b/2a clinical trial of RTB101, alone or in combination with sirolimus, in Parkinson's disease (PD), including anticipated results of this clinical trial, our expectations around the role and importance of TORC1 in neurodegenerative diseases, our estimates as to the number of people with PD worldwide and the United States and rates of diagnosis, our future plans to expand our pipeline and develop RTB101 alone or in combination with rapalogs, such as everolimus or sirolimus, including the therapeutic potential and clinical benefits thereof and the potential patient populations that may be addressed by our product candidates, our ongoing and future clinical trials for RTB101, including the timing of the initiation and anticipated results of these trials, the continued expansion of our pipeline, and our ability to replicate results achieved in our clinical trials in any future trials constitute forward-looking statements identified by words like "believe," "expect," "may," "will," "should," "seek," "anticipate," or "could" and similar expressions.

Such forward-looking statements 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 Phase 1b/2a clinical trial of RTB101, alone or in combination with sirolimus, in Parkinson's disease (PD); our ability to successfully demonstrate the efficacy and safety of our lead product candidate; the clinical results for our lead product candidate which may not support further development of additional indications; uncertainties related to the results of our clinical trials predictive of future results in connection with future trials; 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 represent our views only as of today and should not be relied upon as representing its views as of any subsequent date. resTORbio explicitly disclaims any obligation to update any forward-looking statements.

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