resTORbio Announces Positive End-of-Phase 2 Meeting with FDA and Planned Initiation of Global Phase 3 Program for RTB101

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Phase 3 program on track to start enrolling patients in the second quarter of 2019

Data from two Phase 2 clinical trials enrolling more than 900 patients support the design of the Phase 3 program

BOSTON, March 18, 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 plans to initiate its Phase 3 program of RTB101 10 mg once daily following its End-of-Phase 2 meeting with the U.S. Food and Drug Administration (FDA). The Phase 3 program will evaluate whether RTB101 decreases, as compared to placebo, the percentage of subjects with clinical symptoms consistent with a respiratory tract infection (RTI) with or without laboratory-confirmation of a pathogen.

"Our collaborative interactions with the FDA have us on track to start our Phase 3 program and advance RTB101 towards the potential submission of a New Drug Application (NDA). Initiating our planned Phase 3 program for RTB101 is a big step forward in the field of aging and immunosenescence, the decline in immune system function that occurs with age," said Chen Schor, Co-Founder, President and CEO of resTORbio. "In our Phase 2 clinical trials, RTB101 was observed to improve immune function and decrease the incidence of respiratory tract infections in elderly subjects. Given that RTIs are the fourth leading cause of hospitalizations in the elderly and significantly increase the risk of cardiovascular events, we believe RTB101, if successful in Phase 3 trials and approved, may offer a significant benefit to patients, payors and society."

Alignment was reached with the FDA on key elements of the Phase 3 program that will support the submission of an NDA for RTB101. The planned program includes two randomized, double-blinded, placebo-controlled Phase 3 clinical trials:

- Patient population: People 65 years of age and older, excluding current smokers and chronic obstructive pulmonary disease (COPD) patients
- Dose: Subjects will be randomized to RTB101 10 mg once daily or matching placebo (1:1 randomization)
- Duration of dosing: 16 weeks during winter cold and flu season
- Primary endpoint: Reduction in the percentage of subjects with clinical symptoms consistent with a RTI based on prespecified diagnostic criteria (defined as clinically symptomatic respiratory illness) with or without laboratory-confirmation of a pathogen
- Secondary endpoint: Reduction in the percentage of subjects with clinically symptomatic respiratory illness with laboratory-confirmation of a pathogen

Data from two Phase 2 clinical trials of RTB101 in more than 900 elderly subjects support the design of the Phase 3 program. RTB101 10 mg once daily improved immune function and reduced the incidence of RTIs in both healthy elderly and in elderly with comorbidities. Additionally, the Phase 2 clinical trials demonstrated a significant reduction in both self-reported and laboratory-confirmed RTIs. In an analysis of the Phase 2b trial results, a 46.6% reduction in the percentage of subjects with clinical symptoms consistent with an RTI was observed in subjects who did not smoke and did not have COPD, the proposed Phase 3 population, when treated with RTB101 10 mg once daily as compared to placebo (p=0.007). RTB101 10 mg once daily was observed to be well-tolerated.

"We are very excited to have reached alignment with the FDA on key elements of the Phase 3 clinical trials," said Dr. Joan Mannick, Co-Founder and Chief Medical Officer of resTORbio. "Most RTIs are caused by viruses that lack effective treatments. Since RTB101 was observed to upregulate antiviral innate immune pathways that target many different viruses, both our Phase 2b and Phase 3 primary endpoints include prespecified diagnostic criteria that encompass multiple different types of respiratory tract infections caused by multiple different types of viruses. We look forward to initiating our Phase 3 program and to developing RTB101 with the goal of improving the function of the aging immune system and thereby reducing the burden of respiratory illness in the elderly."

The first Phase 3 clinical trial is planned to begin in the southern hemisphere in the second quarter of 2019 and is expected to enroll approximately 1,000 subjects. The second Phase 3 clinical trial is planned to begin in the northern hemisphere in the fourth quarter of 2019 and is expected to enroll approximately 1,600 subjects. Each of the planned Phase 3 studies is expected to be powered at greater than or equal to 90% to demonstrate a 30% reduction in the percentage of subjects with clinically symptomatic respiratory illness between RTB101 and placebo using a two-sided test of 0.05 significance. The number of subjects who will have received RTB101 10 mg once daily in the Phase 2b clinical trial and planned Phase 3 program is expected to reach at least 1,500, which, based on communications with the FDA, is the size of the safety database that the Company believes will be sufficient to support an NDA filing, barring any safety signals observed in the Phase 3 trials. Depending on enrollment in the planned Phase 3 clinical trials, resTORbio expects top-line data in mid-2020.

About Respiratory Tract Infections

The reduced ability of the aging immune system to effectively detect and fight infections results in increased susceptibility of the elderly to RTIs. In the U.S., RTIs are the fourth leading cause of hospitalizations and seventh leading cause of death in people age 65 years and older. The majority of RTIs are caused by viruses, many of which have no currently approved therapies.

About RTB101

RTB101 is an investigational, oral, selective, and potent TORC1 inhibitor product candidate. RTB101 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 immune, cardiac and neurologic functions, 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 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. Learn more about resTORbio, Inc. at

https://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, express or implied statements or guidance regarding our proposed timing and trial design for our Phase 3 clinical trial of RTB101, including anticipated results of this clinical trial, 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 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 into Parkinson's disease and UTIs, the intended regulatory path for our product candidates and interactions with regulatory authorities, our ability to replicate results achieved in our clinical trials in any future trials, our cash position and our expectations regarding our uses of capital 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 planned Phase 3 clinical trials in RTIs and/or development of RTB101, either alone or in combination with a rapalog, such as everolimus or sirolimus; 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, including our planned Phase 3 clinical 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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