



ONCONOVA
THERAPEUTICS

Onconova Therapeutics Submits Application for Rigosertib to Participate in Federally Funded Human Studies in COVID-19 Disease

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Preclinical experiments with rigosertib in cellular models demonstrate marked inhibition of SARS-CoV-2 replication

NEWTOWN, Pa., July 27, 2020 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (NASDAQ: ONTX), a Phase 3 stage biopharmaceutical company focused on discovering and developing novel products to treat cancer, with an initial focus on myelodysplastic syndromes (MDS), today announced that it has submitted an application with the National Institute of Allergy and Infectious Disease (NIAID), with the goal of obtaining funding from the National Institutes of Health (NIH) to conduct human studies with rigosertib in COVID-19 disease patients.

"Recent preclinical studies conducted with rigosertib demonstrate impressive inhibition of SARS-CoV-2 replication in Vero Cells when compared to controls including agents currently being administered and studied in SARS-CoV-2 infection, and provide the Company with optimism that further research in humans infected with SARS-CoV-2 is warranted," said Steven M. Fruchtman, M.D., President and Chief Executive Officer. "While it is difficult to predict when the NIAID will consider the merits of rigosertib in this setting, a positive funding decision could allow Onconova to begin human studies in COVID-19 infected patients. As an oncology-focused company, Onconova does not currently intend to pursue clinical development in COVID-19 without additional sources of non-dilutive funding."

"By modulating the RAS/RAF/MEK/ERK pathway involved in proliferative signaling, rigosertib may play an important role in inhibiting COVID-19 replication in human cells, specifically lung tissue, a primary source of serious disease," said Richard C. Woodman, M.D., Chief Medical Officer of Onconova. "While our work in COVID-19 is in very early stages, Onconova believes it has an obligation to pursue these efforts given the impact of the pandemic on human health."

About Onconova Therapeutics, Inc.

Onconova Therapeutics, Inc. is a Phase 3-stage biopharmaceutical company focused on discovering and developing novel drugs to treat cancer, with an initial focus on myelodysplastic syndromes (MDS). Onconova has a pipeline of proprietary targeted agents designed to work against specific cellular pathways that are important in cancer cells. Advanced clinical trials with the Company's lead compound, rigosertib, are aimed at what the Company believes are unmet medical needs of patients with MDS. Onconova has conducted trials with two other research compounds and has a pre-clinical program with a CDK4/6 and ARK5 inhibitor, ON 123300.

For more information, please visit <https://www.onconova.com>.

About SARS-CoV-2 and COVID-19

Severe acute respiratory syndrome due to SARS-CoV-2 has impacted millions of people worldwide and has led to the death of hundreds of thousands of individuals. Collaborative efforts to test many experimental and health authority approved agents are ongoing worldwide to seek to address the global pandemic through the development of therapeutic antiviral drugs to treat COVID-19 infection and of vaccines to prevent infection with SARS-CoV-2.

About Myelodysplastic Syndromes

Myelodysplastic syndromes (MDS) are conditions that can occur when the blood-forming cells in the bone marrow become dysfunctional and thus produce an inadequate number of circulating blood cells. It is frequently associated with the presence of blasts or leukemic cells in the marrow. This leads to low numbers of one or more types of circulating blood cells, and to the need for blood transfusions. In MDS, some of the cells in the bone marrow are abnormal (dysplastic) and may have genetic abnormalities associated with them. Different cell types can be affected, although the most common finding in MDS is a shortage of red blood cells (anemia). Patients with higher-risk MDS may progress to the development of acute leukemia.

About Rigosertib

Rigosertib, Onconova's lead candidate, is a proprietary Phase 3 small molecule. A key publication in a preclinical model reported rigosertib's ability to block cellular signaling by targeting RAS effector pathways (Divakar, S.K., et al., 2016: "A Small Molecule RAS-Mimetic Disrupts RAS Association with Effector Proteins to Block Signaling." Cell 165, 643). Onconova is currently in the clinical development stage with oral and IV rigosertib, including clinical trials studying single agent IV rigosertib in second-line higher-risk MDS patients (pivotal Phase 3 INSPIRE trial) and oral rigosertib plus azacitidine in HMA naive and refractory higher-risk MDS patients (Phase 2). Patents covering oral and injectable rigosertib have been issued in the US and are expected to provide coverage until at least 2037.

About the INSPIRE Phase 3 Clinical Trial

The clinical trial **International Study of Phase 3 IV Rigosertib**, or INSPIRE, was finalized following guidance received from the U.S. Food and Drug Administration and European Medicines Agency. INSPIRE is a global, multi-center, randomized, controlled study to assess the efficacy and safety of IV rigosertib in higher-risk MDS (HR-MDS) patients who had progressed on, failed to respond to, or relapsed after previous treatment with a hypomethylating agent (HMA) within nine cycles over the course of one year after initiation of HMA treatment. This time frame optimizes the opportunity to respond to treatment with an HMA prior to declaring treatment failure, as per NCCN Guidelines. Patients are randomized at a 2:1 ratio

into two study arms: IV rigosertib plus Best Supportive Care versus Physician's Choice plus Best Supportive Care. The primary endpoint of INSPIRE is overall survival. The trial continued beyond the pre-specified interim analysis and is nearing its conclusion. Full details of the INSPIRE trial, such as inclusion and exclusion criteria, as well as secondary endpoints, can be found on clinicaltrials.gov (NCT02562443).

About IV Rigosertib

The intravenous form of rigosertib has been studied in Phase 1, 2, and 3 clinical trials involving more than 1000 patients, and is currently being evaluated in a randomized Phase 3 international INSPIRE trial for patients with HR-MDS after failure of HMA therapy.

About Oral Rigosertib

The oral form of rigosertib was developed to provide a potentially more convenient dosage form for use where the duration of treatment may extend to multiple years. This dosage form may also support combination therapy modalities. To date, over 400 patients have been dosed with the oral formulation of rigosertib in clinical trials. Combination therapy of oral rigosertib with azacitidine, the standard of care in HR-MDS, has also been studied. Currently, oral rigosertib is being developed as a combination therapy together with azacitidine for patients with higher-risk MDS who require HMA therapy. A Phase 1/2 trial of the combination therapy has been fully enrolled, and the updated efficacy and safety data was presented at the ASH 2019 Annual Meeting in December 2019.

Forward-Looking Statements

Some of the statements in this release are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, Section 21E of the Securities Exchange Act of 1934, as amended, and the Private Securities Litigation Reform Act of 1995, and involve risks and uncertainties. These statements relate to Onconova expectations regarding the INSPIRE Trial and Onconova's other development plans. Onconova has attempted to identify forward-looking statements by terminology including "believes," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," "approximately" or other words that convey uncertainty of future events or outcomes. Although Onconova believes that the expectations reflected in such forward-looking statements are reasonable as of the date made, expectations may prove to have been materially different from the results expressed or implied by such forward-looking statements. These statements are only predictions and involve known and unknown risks, uncertainties, and other factors, including Onconova's ability to continue as a going concern, maintain its Nasdaq listing, the need for additional financing, the success and timing of Onconova's clinical trials and regulatory approval of protocols, our collaborations including the effective termination of the HanX license and securities purchase agreements and plans for partnering certain territories, and those discussed under the heading "Risk Factors" in Onconova's most recent Annual Report on Form 10-K and quarterly reports on Form 10-Q. Any forward-looking statements contained in this release speak only as of its date. Onconova undertakes no obligation to update any forward-looking statements contained in this release to reflect events or circumstances occurring after its date or to reflect the occurrence of unanticipated events.

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