Onconova Announces First Patient Dosed in Investigator-Initiated Phase 2 Study of Rigosertib in Recessive Dystrophic Epidermolysis Bullosa-Associated Squamous Cell Carcinoma

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NEWTOWN, Pa., April 22, 2021 (GLOBE NEWSWIRE) -- **Onconova Therapeutics, Inc. (NASDAQ: ONTX)**, a clinical-stage biopharmaceutical company focused on discovering and developing novel products for patients with cancer, announced today that the first patient has been dosed in an investigator-initiated Phase 2 study to assess the efficacy and safety of rigosertib in patients with recessive dystrophic epidermolysis bullosa (RDEB)-associated locally advanced/metastatic squamous cell carcinoma (SCC). The patient was dosed at the EB House Austria, a center of expertise for epidermolysis bullosa at the University Hospital Salzburg, Austria. Additional sites are anticipated to be opened in the UK and in the US to study this rare and genomically driven devastating disease.

In this open-label investigator-initiated study, 12 patients will receive either oral or intravenous rigosertib at the clinician's discretion given the various clinical manifestations of the disease, which may dictate the need for either oral or intravenous administration of rigosertib. These patients have skin desquamation making intravenous access difficult, or may form esophageal strictures, which make oral administration difficult. Patients will receive either oral rigosertib in four-week cycles (three weeks on, one week off) for up to 13 cycles, with 560 mg of oral rigosertib in the morning and again in the afternoon, for a total of 1,120 mg/day. Alternatively, patients will receive intravenous (IV) rigosertib as a 72-hour IV infusion on days 1, 2 and 3 of eight 2-week cycles, and on days 1, 2 and 3 of nine 4-week cycles thereafter, with each 24-hour infusion consisting of 1,800 mg of rigosertib.

The study has two primary endpoints. The first is to determine the anti-tumor activity of rigosertib in RDEB patients with advanced SCC who have failed prior standard of care through the overall response rate (ORR), defined as the proportion of patients who achieve either a complete response (CR) or a partial response (PR). The second primary endpoint is to evaluate the safety and tolerability of rigosertib in this population. Secondary study endpoints include quality of life and a biomarker analysis performed on archival tissue from all patients. Patients will be dosed for up to one year, with trial duration anticipated to be approximately two-and-a-half years.

"We are pleased with the advancement of our investigator-initiated programs with rigosertib, and to provide rigosertib in support of this important Phase 2 investigator-sponsored study," said Steven M. Fruchtman, M.D., President and Chief Executive Officer of Onconova Therapeutics. "Recessive dystrophic epidermolysis bullosa is a genetic skin blistering disease that often results in squamous cell carcinoma in severe subtypes. In this patient cohort, squamous cell carcinoma is the leading cause of death. We have previously identified polo-like kinase 1 as a therapeutic target in skin SCC, including RDEB SCC, so we are encouraged by the start of this trial. We hope rigosertib can prove beneficial to this rare patient population with a tremendous unmet medical need."

In addition to Onconova Therapeutics, the study is being supported by DEBRA International. "The aggressive course and poor prognosis of skin cancer in our patients emphasize the urgent need for potent therapies," stated Professor Johann W. Bauer, M.D., Principal investigator of the trial. "We hope that rigosertib as an innovative approach provides benefit to this devastating illness that currently lacks effective therapies."

"Basic research has provided understanding into the etiology of Recessive Dystrophic Epidermolysis Bullosa-associated cancer," stated Andrew South Ph.D., Associate Professor, Department of Dermatology & Cutaneous Biology, Thomas Jefferson University. "I would like to thank both the Debra Foundations for funding this work as well as Onconova for providing a research drug that may target the life-threatening cancers arising in these patients."

About Onconova Therapeutics

Onconova Therapeutics is a clinical-stage biopharmaceutical company focused on discovering and developing novel products for patients with cancer. The Company has proprietary targeted anti-cancer agents designed to disrupt specific cellular pathways that are important for cancer cell proliferation.

Onconova's novel, proprietary multi-kinase inhibitor ON 123300 is planned to begin a dose-escalation and expansion Phase 1 trial in the U.S. in 2Q21, and a dose-escalation and expansion Phase 1 trial is currently underway in China.

Onconova's product candidate oral rigosertib is being studied in an investigator-initiated study program, including in a dose-escalation and expansion Phase 1 investigator-initiated study targeting patients with KRAS+ lung adenocarcinoma in combination with nivolumab. In addition, Onconova continues to conduct preclinical work investigating rigosertib in COVID-19.

For more information, please visit www.onconova.com.

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's expectations regarding the registered direct offering, its patents and clinical development plans including patient enrollment timelines and indications for its product candidates. 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 the success and timing of Onconova's clinical trials and regulatory agency and institutional review board approvals of protocols, Onconova's ability to continue as a going concern, the need for additional financing, Onconova's collaborations, market conditions 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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