June 1, 2013

Onconova Announces Presentation of Favorable Safety and Activity Data from Phase 2 Study of Oral Rigosertib in Lower Risk Myelodysplastic Syndromes

Interim Results Presented at 2013 ASCO Annual Meeting Indicate Strong Signal of Transfusion Independence

June 1, 2013 – NEWTOWN, PA: Onconova Therapeutics, Inc., a clinical-stage biopharmaceutical company, today announced the presentation of safety and clinical activity data at the American Society of Clinical Oncology Annual Meeting demonstrating an encouraging clinical profile for rigosertib in patients with myelodysplastic syndromes (MDS). The data reported today are interim data from the Phase 2 ONTARGET study which is designed to evaluate oral rigosertib as a single agent in transfusion-dependent lower risk MDS patients.

The results were discussed by Azra Raza, MD, Director, MDS Center, Columbia University Medical Center, New York, NY in a presentation entitled “Phase II study of orally administered rigosertib (ON 01910.Na) in transfusion-dependent lower risk myelodysplastic syndrome (MDS) patients.”

“These interim data from the ONTARGET study suggest that oral rigosertib may provide a new treatment option for patients with lower risk myelodysplastic syndrome who have become transfusion dependent,” commented Dr. Raza. “It is estimated that 77% of MDS patients are in the lower risk category, and currently, these patients lack effective therapeutic options beyond best supportive care and frequent transfusions. Our ongoing studies aim to correlate patient characteristics with transfusion independence, thus allowing for the selection of appropriate patients for future trials with rigosertib. We look forward to conducting additional clinical studies designed to optimize the dose and schedule for oral rigosertib in this patient population.”

ONTARGET is a randomized, two-arm Phase 2 study of oral rigosertib (560 mg bid) administered either intermittently (two out of three weeks) or continuously. Transfusion-dependent patients must have received at least four units of red blood cell (RBC) transfusions over eight weeks before randomization and can receive transfusions and erythrocyte stimulating agents (ESAs) while on study. Full results from the study are expected in the second half of 2013.

In the ONTARGET study, 13 of the 26 (50%) evaluable patients in the intermittent dosing arm and two of the eight (25%) evaluable patients in the continuous dosing arm achieved transfusion independence, defined as no RBC transfusions for at least eight consecutive weeks. Onset of transfusion independence ranged from 1-24 weeks following the initiation of rigosertib dosing, and the duration of transfusion independence ranged from eight to greater than 48 weeks, with two patients continuing to benefit from therapy more than nine months after starting rigosertib. None of the responders had a del5q karyotype. Eleven of the 13 transfusion-independent patients in the intermittent dosing arm received one or more injections of ESAs during the time of oral rigosertib administration, and the patterns of hemoglobin responses observed in a few patients suggest a possible synergy between oral rigosertib and ESAs.

Evaluation of the interim safety data indicated that oral rigosertib was generally well tolerated with the most frequently observed side effects being urologic in nature and believed to be related to dosing regimen. In the continuous dosing arm of the study, grade 2+ urinary side effects were observed in five of
the first nine patients. Accordingly, the study protocol was amended to allow all patients to be treated with intermittent dosing. The most frequent urinary adverse event in the intermittent dosing arm was grade 2+ urinary urgency/frequency (38% of patients), grade 2+ dysuria (15%), and hematuria (15%). Other grade 2+ adverse events included intermittent neutropenia (one grade 3 and one grade 4). Median onset of grade 2+ adverse events in the intermittent dosing arm was 28 weeks compared with 12 weeks in the continuous dosing arm. Median duration of treatment in the intermittent dosing arm has not yet been reached (greater than 48 weeks compared with 24 weeks in the continuous dosing arm). Renal function was unaffected and gastrointestinal adverse events and fatigue were infrequently observed.

“These new data presented at ASCO, combined with the demonstration of single agent activity of oral rigosertib in patients with solid tumors presented at the recent AACR 2013 Annual Meeting, provide the clinical basis for additional late-stage clinical trials for patients with MDS and solid tumors,” said Ramesh Kumar, PhD, President and Chief Executive Officer of Onconova. “We look forward to final data from the ONTARGET study and potential pivotal trials for oral rigosertib.”

ASCO 2013 rigosertib presentation information:
Saturday, June 1, 8:00 a.m. - 12:00 p.m. CT
Poster Title: Phase II study of orally administered rigosertib (ON 01910.Na) in transfusion-dependent lower risk myelodysplastic syndrome (MDS) patients (Abstract #7031)

Location: Room S405
Presented by: Dr. Azra Raza, Director, MDS Center, Columbia University Medical Center, New York, NY

About Rigosertib
Rigosertib is an inhibitor of two important cellular signaling pathways, phosphoinositide 3-kinase, or PI3K, and polo-like kinase, or PLK, both of which are frequently activated in cancer cells. Rigosertib is being developed in both oral and intravenous forms as a treatment for hematological diseases and solid tumors. Onconova recently announced reaching the enrollment goal in its randomized, controlled ONTIME Phase 3 Trial for intravenous rigosertib in adult patients with myelodysplastic syndromes whose disease has failed azacitidine or decitabine therapy. Rigosertib is also being evaluated in a Phase 3 trial for first-line treatment in combination with gemcitabine for patients with metastatic pancreatic cancer who had not previously received any chemotherapy. The oral form of rigosertib is currently being studied in Phase 2 trials in patients with transfusion-dependent lower risk myelodysplastic syndromes and in patients with head and neck cancer. Rigosertib has been granted orphan drug status for MDS in both the United States and Europe, as well as orphan drug status for pancreatic cancer in the United States. Rigosertib is being developed in partnership with Baxter International (commercialization rights in Europe) and SymBio Pharmaceuticals (Japan and Korea). Onconova has retained all other territories for commercialization.

About Onconova Therapeutics, Inc.
Onconova Therapeutics is a clinical-stage biopharmaceutical company focused on discovering and developing novel products to treat cancer. Onconova's clinical and pre-clinical stage drug development candidates are derived from its extensive proprietary chemical library and are designed to work against specific cellular pathways that promote cancer while causing minimal damage to normal cells. In addition to rigosertib, the Company's most advanced product candidate, two other candidates are in clinical trials, and several candidates are in pre-clinical stages. For more information, please visit http://www.onconova.com.

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