June 5, 2017

Onconova Therapeutics Announces Positive Phase 2 Data in Rigosertib Treated Patients with Second-line Myelodysplastic Syndromes at the American Society of Clinical Oncology Annual Meeting

- ***Bone marrow response to rigosertib was evaluated as a surrogate for survival in this trial of 64 patients who had previously failed Hypomethylating Agent therapy***
- ***22% of patients achieved marrow complete response and 47% of patients in the study achieved disease stabilization.***
- ***In Landmark Analysis, Bone marrow response correlated with overall survival (mOS was 3.3 months for progressors, 6.3 months for stable disease and not reached for patients with marrow CR; Log rank P = 0.005)***

NEWTOWN, Pa., June 05, 2017 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (NASDAQ:ONTX), a Phase 3 stage biopharmaceutical company focused on discovering and developing novel small molecule drug candidates to treat cancer, with a primary focus on Myelodysplastic Syndromes (MDS), today announced the results of a Phase 2 study with rigosertib as a treatment for higher risk (HR-MDS) after failure of hypomethylating agents (HMAs). The study sought to evaluate bone marrow blast (BMBL) response to rigosertib as a surrogate for overall survival (OS) in this patient population. The results showed treatment with rigosertib resulted in a reduction in BMBL count, including complete bone marrow responses, confirming findings in earlier studies. Thus, BMBL response to rigosertib is a potential surrogate marker for improvement in overall survival in this patient population.

"In this new study for HR-MDS patients after failure of HMA therapy, we are excited to confirm a correlation between blast reduction and prolongation of survival in rigosertib treated patients. These results build upon our previous findings in the ONTIME trial showing improvement in overall survival in patients with the highest risk prognostic categories after failure of HMA treatment (ASH 2014 presentation),"said Ramesh Kumar, Ph.D., President and CEO of Onconova.

Rigosertib is currently being tested in a randomized, global, Phase 3 INSPIRE trial for this patient population.

Study Name: Relationship of Bone Marrow Blast response to Overall Survival in a Multicenter Study of Rigosertib in Patients with Myelodysplastic Syndromes with Excess Blasts Progressing on or After Treatment with a Hypomethylating Agent

Summary of Data from the 04-24 Trial

Patient Demographics:

- 64 patients treated, with a median age of 73, median prior HMA duration of 10.8 months.
- Eligible patients had 5%-30% BMBL confirmed within six weeks pre-study and disease. progression as per International Working Group (IWG) 2006 criteria, Cheson et al., *Blood* 2006) on or after HMAs within two years.

Safety/Tolerability:

- Intravenous rigosertib has been well tolerated to date.
- More than 1,100 patients have been treated with rigosertib.
- Adverse events in study 04-24 were similar to those observed in the preceding Phase 3 ONTIME Study

Objectives:

- **Primary Efficacy:** Evaluate the relationship between BMBL response and OS in MDS patients with excess blasts (5-30%) progressing on or after treatment with azacitidine or decitabine who are administered 72-hr continuous intravenous (IV) infusions of 1800 mg/24 hour rigosertib every 2 weeks for 8 cycles and every 4 weeks thereafter. BMBL response is defined according to the International Working Group (IWG) 2006 criteria; or stable BM response (no progression),
- **Secondary Efficacy:** Evaluate the following parameters: Overall response (CR, partial response/remission [PR], BMCR, and stable disease [SD]) according to 2006 IWG criteria; Population pharmacokinetics.
- **Safety:** safety and tolerability of rigosertib administered as 72-hour continuous IV (5%-30% BMBL) progressing on or after treatment with azacitidine or decitabine.

Trial Design:

- Phase 2 open-label, multi-center multi-national study (approximately 30-40 Centers in the US and Europe) of the efficacy and safety of Rigosertib administered as 72-hour continuous intravenous infusions in patients with myelodysplastic syndromes with excess blasts progressing on or after treatment with azacitidine or decitabine.
- Treatment will continue until IWG 2006 progression criteria are met (ie, 50% increase of BMBL or worsening of cytopenias) or until death from any cause, whichever comes first.

Following these results, all patients will be followed until death and/or progression, even if they have discontinued treatment for any reason. View the complete study poster HERE.

About Onconova Therapeutics, Inc.

Onconova Therapeutics, Inc. is a Phase 3-stage biopharmaceutical company focused on discovering and developing novel small molecule drug candidates to treat cancer, with a primary focus on Myelodysplastic Syndromes (MDS). Rigosertib, Onconova's lead candidate, is a proprietary Phase 3 small molecule agent, which we believe blocks cellular signaling by targeting RAS effector pathways. Using a proprietary chemistry platform, Onconova has created a pipeline of targeted agents designed to work against specific cellular pathways that are important in cancer cells, while causing minimal damage to normal cells. Onconova has three product candidates in the clinical stage and several pre-clinical programs. 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. For more information, please visit http://www.onconova.com.

About IV Rigosertib

The intravenous form of rigosertib has been employed in Phase 1, 2, and 3 clinical trials involving more than 800 patients, and is currently being evaluated in the randomized Phase 3 international INSPIRE trial for patients with higher-risk MDS, after failure of hypomethylating agent, or HMA, therapy. This formulation is intended for patients with advanced disease, provides long duration of exposure, and ensures dosing under a controlled setting.

About INSPIRE

The **IN**ternational **S**tudy of **P**hase III **I**V **R**igos**E**rtib, or INSPIRE, is based on guidance received from the U.S. Food and Drug Administration and European Medicines Agency and derives from the findings of the ONTIME Phase 3 trial. INSPIRE is a multi-center, randomized controlled study to assess the efficacy and safety of IV rigosertib in HR-MDS patients who had progressed on, failed to respond to, or relapsed after previous treatment with an HMA within the first 9 months or 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. The trial will enroll approximately 225 patients randomized at a 2:1 ratio into two treatment arms: IV rigosertib plus Best Supportive Care versus Physician's Choice plus Best Supportive Care. The primary endpoint of INSPIRE is overall survival and an interim analysis is anticipated. 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 Oral Rigosertib

The oral form of rigosertib was developed to provide more convenient dosing for use where the duration of treatment may extend to multiple years. This dosage form also supports many combination therapy modalities. To date, 368 patients have been treated with the oral formulation of rigosertib. Initial studies with single-agent oral rigosertib were conducted in hematological malignancies, lower-risk MDS, and solid tumors. Combination therapy of oral rigosertib with azacitidine and chemoradiotherapy has also been explored. 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 2 trial of the combination therapy has been fully enrolled and the preliminary results were presented in 2016. This novel combination is the subject of an issued US patent with earliest expiration in 2028.

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 future events or Onconova Therapeutics, Inc.'s future operations, clinical development of Onconova's product candidates and presentation of data with respect thereto, regulatory approvals, expectations regarding the sufficiency of Onconova's cash and other resources to fund operating expenses and capital expenditures, Onconova's anticipated milestones and future expectations and plans and prospects. 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. 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. 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, the need for additional financing and current plans and future needs to scale back operations if adequate financing is not obtained, the success and timing of Onconova's clinical trials and regulatory approval of protocols, 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.

GENERAL CONTACT:
http://www.onconova.com/contact/

INVESTOR RELATIONS CONTACT:
Lisa Sher, MBS Value Partners on behalf of Onconova Therapeutics
Lisa.Sher@mbsvalue.com / (212) 750-5800