April 21, 2015

Onconova Therapeutics, Inc. Highlights Rigosertib and Early-stage Programs at the 2015 American Association of Cancer Research (AACR) Annual Meeting

NEWTOWN, Pa., April 21, 2015 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (Nasdaq:ONTX), a clinical-stage biopharmaceutical company focused on discovering and developing novel products to treat cancer, today announced the highlighting of data from its lead and other proprietary compounds at the 2015 AACR Annual Meeting being held April 18-22, 2015 at the Pennsylvania Convention Center in Philadelphia, PA.

Three abstracts related to Phase 3 stage compound, rigosertib, and Phase 1 stage compound, briciclib, and three abstracts related to two pre-clinical stage compounds, are currently available at http://www.aacr.org. The significance and brief summaries of each key abstract, and abstract titles and numbers, are listed below.

"These important pre-clinical data further advance our efforts to amass broad scientific evidence supporting the continued development of our clinical and pre-clinical pipeline," said Ramesh Kumar, Ph.D., President and CEO of Onconova. "We are excited by the potential each of the compounds highlighted at AACR holds as anti-cancer agents."

Bone marrow culture system for testing rigosertib activity in lower-risk myelodysplastic syndromes (LR-MDS) patients

Abstract number: 2425
Abstract title: An in vitro platform to dissect drug responsiveness in refractory anemia with ringed sideroblasts (RARS)
Significance: Rigosertib activity assessed in an in vitro bone marrow system; patient samples from an on-going Phase 2 clinical trial utilized to correlate in vitro activity with clinical results in LR-MDS patients.
Summary: Investigators from Columbia University Medical Center studied the underlying mechanisms for increased hemoglobin production, decreased transfusion requirements and re-sensitization to administered erythropoietin (EPO) noted in LR-MDS patients following rigosertib treatment in a Phase 2 trial (data presented at the 2013 ASH Annual Meeting). A co-culture of CD34+ stem cells and stromal cells isolated from the bone marrow of RARS LR-MDS patients was used to assess erythroid differentiation in the presence of EPO and rigosertib alone or in combination. RARS LR-MDS co-cultures showed no erythroid differentiation upon EPO stimulation, whereas co-cultures treated with rigosertib plus EPO showed increased erythroid differentiation, indicating the activity of rigosertib in this process. Further, co-cultures obtained from patients responsive to rigosertib in vivo showed increased differentiation in vitro following rigosertib/EPO stimulation. Investigators noted the potential use for this co-culture system to predict responsiveness to experimental drugs, such as rigosertib, in LR-MDS patients.

Development of orally bioavailable analog of briciclib

Abstract number: 1649
Abstract title: Potent anticancer activity of an orally bioavailable small molecule, ON 013100, and its water soluble derivative, briciclib, a clinical-stage eIF4E-targeted agent
Significance: Activity of briciclib IV, currently in a Phase 1 clinical trial in patients with advanced solid tumors refractory to current therapies, was comparable with an active analog suitable for oral delivery.
Summary: Following the recent development of an oral formulation for ON 013100, the parent compound of briciclib, researchers from Long Island University sought to compare the in vitro activities of ON 013100 and briciclib. Both compounds inhibited proliferation and induced killing of cancer cell lines with similar potency, and mechanistic studies demonstrated similar eIF4E targeting for the two compounds. Based on these experiments, investigators concluded that ON 013100 and briciclib share the same novel mechanism of action and noted the potential for development of an oral formulation of ON 013100 to complement the intravenous formulation of briciclib that is currently undergoing Phase 1 clinical testing.

Activity of pre-clinical CK2 inhibitor, ON 108600, against cancer stem cells and paclitaxel resistant triple-negative breast cancer (TNBC)

Abstract number: 4453
Abstract title: The dual CK2/TNIK inhibitor, ON 108600, targets cancer stem cells and induces apoptosis of paclitaxel resistant triple-negative breast cancer cells
Significance: Proof of concept for CK2 inhibition as a therapeutic target in paclitaxel resistant model breast cancer system.
Summary: Investigators from Mount Sinai School of Medicine tested ON 108600 against tumor-initiating stem cells and paclitaxel resistant TNBC cell lines in order to characterize its potential activity in TNBC. Notably, ON 108600 inhibited stem cell activity and self-renewal capacity of TNBC cell lines, while also targeting paclitaxel resistant cells through the inhibition of a novel target, Traf2 and Nck-interacting kinase (TNIK).

Development of nanosuspension formulation of pre-clinical PLK2 inhibitor, ON 1231320 (GBO-006), for testing in TNBC models

Abstract number: 1661
Abstract title: Development of lipid-based nanosuspension formulation of first-in-class PLK2 inhibitor GBO-006 to treat triple negative breast cancer
Significance: Formulation development of a novel PLK2 targeted compound.
Summary: Research conducted in collaboration with GVK Biosciences, India, highlighted the characterization of a nanosuspension preparation for ON 1231320 (GBO-006), undertaken in an effort to develop a formulation adequate for toxicology studies. Compared with previous formulations, the nanoformulation achieved similar efficacy in a xenograft model of TNBC. Ongoing studies are focused on further decreasing particle size.

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 chemical library and are designed to work against specific cellular pathways that are important in cancer cells, while causing minimal damage to normal cells. In addition to rigosertib, the Company's most advanced product candidate, two other candidates are clinical stage, and several candidates are in pre-clinical stages. For more information, please visit http://www.onconova.com.

About Rigosertib

Rigosertib is a small molecule that inhibits cellular signaling by acting as a Ras mimetic. This is believed to be mediated by direct binding of rigosertib to the Ras-binding domain (RBD) found in many Ras effector proteins, including the Raf kinases and PI3K. The initial therapeutic focus for rigosertib is myelodysplastic syndromes (MDS), a group of bone marrow disorders characterized by ineffective formation of blood cells that often converts into acute myeloid leukemia (AML). Clinical trials with intravenous (IV) and oral formulations of rigosertib are being conducted at leading institutions in the U.S. and Europe.

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, which 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 those discussed under the heading "Risk Factors" in our 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. We undertake 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.

CONTACT: Onconova Therapeutics

Benjamin Hoffman, 267-759-3036

bhoffman@onconova.us