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Onconova Announces Successful End-of-Phase 2 Meeting with FDA for Oral Rigosertib and Azacitidine Combination

Pivotal Randomized Trial in 1st-line MDS Patients will Assess Overall Response Rate as Approval Endpoint

NEWTOWN, Pa., Sept. 26, 2016 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (NASDAQ:ONTX), a Phase 3 clinical-stage biopharmaceutical company focused on discovering and developing novel products to treat cancer, today announced receipt of the End-of-Phase 2 meeting minutes from the U.S. Food and Drug Administration (FDA) for the combination of oral rigosertib with azacitidine for the treatment of patients with higher-risk myelodysplastic syndromes (HR-MDS).

Based on these discussions, Onconova will design a randomized, controlled Phase 3 clinical trial comparing the combination of oral rigosertib plus azacitidine to azacitidine plus placebo in 1st-line HR-MDS patients. The primary endpoint of this pivotal trial will be overall response rate (ORR); ORR will be a composite of complete remission (CR) and partial remission (PR). Onconova could potentially pursue additional available paths from FDA for accelerated or enhanced review and further input on the development of a final trial protocol. Full details of the protocol will be available following completion of all regulatory discussions.

As part of the End-of-Phase 2 meeting with FDA, Onconova presented updated results from its Phase 2 trial of oral rigosertib and azacitidine. These results are expected to be presented by study investigators at a scientific conference later this year.

"We are encouraged that our interactions with FDA resulted in agreement on the patient population to be evaluated and the selection of overall response as the primary endpoint in this pivotal trial," stated Steven Fruchtman, M.D., Chief Medical Officer of Onconova. "We will provide additional details on the design of this trial and the full Phase 2 data set supporting our pivotal study plans at upcoming meetings and scientific conferences later in 2016."

Onconova is developing rigosertib for patients with MDS where there is a paucity of therapeutic options. Two hypomethylating agents (HMAs), azacitidine and decitabine, the mainstays for eligible 1st-line patients, were approved by the FDA more than a decade ago. In addition to the oral rigosertib and azacitidine combination program, Onconova is currently enrolling patients in the global, pivotal Phase 3 INSPIRE trial of IV rigosertib for 2nd-line MDS patients, who have failed HMA therapy. This trial of 225 patients is now enrolling in the U.S., Europe, Japan and Australia.

About Oral Rigosertib

The oral form of rigosertib provides a more convenient dosing for use where the duration of treatment may extend to multiple years. To date, more than 350 patients have been treated with the oral formulation of rigosertib, either as a single agent or in combination with other drugs. Phase 1 studies with 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.

About Trial 09-08 (NCT01926587): Combination therapy with oral rigosertib and azacitidine

The current standard of care for higher-risk MDS patients is one of two approved hypomethylating agents (Azacitidine and Decitabine, approved by the FDA in 2004 and 2006). Although these drugs are currently the mainstays in HR-MDS therapy, their overall response rate and duration of benefit is limited to a subset of eligible patients and all responding patients ultimately progress. Therefore, there is an urgent need for developing therapeutic options for newly diagnosed MDS patients. The 09-08 Phase 1/2 trial tested oral rigosertib in combination with injectable azacitidine in a dose ranging study (Phase 1), followed by an expansion cohort (Phase 2) to evaluate the efficacy and safety of the combination. Interim results from this trial were presented at the 2015 ASH conference indicating that the combination was generally well tolerated and that clinical responses were observed in 23 of 30 MDS patients evaluable for efficacy assessment.[1] Both 1st-line and 2nd-line patients were included in this study.

About Onconova Therapeutics, Inc.

Onconova Therapeutics is a Phase 3 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. The Company's most advanced product candidate, rigosertib, is a small molecule inhibitor of cellular signaling and acts as a RAS mimetic. These effects of rigosertib appear to be mediated by direct binding of the compound to the RAS-binding domain (RBD) found in many RAS effector proteins, including the Raf and PI3 kinases. Rigosertib is protected by issued patents (earliest expiry in 2026) and has been awarded Orphan Designation for MDS in the United States, Europe and Japan. In addition to rigosertib, two other candidates are in the clinical stage, and several candidates are in pre-clinical stages. For more information, please visit http://www.onconova.com.

References

[1]Navada S, et al. A phase 2 study of the combination of oral rigosertib and azacitidine in patients with myelodysplastic syndrome (MDS). ASH 2015; Abstract 910.

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," "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 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.

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