



# ONCONOVA THERAPEUTICS

## Onconova Therapeutics Announces Presentations at the 15th International Symposium on Myelodysplastic Syndromes (MDS 2019) in Copenhagen, Denmark

May 6, 2019

NEWTOWN, Pa., May 06, 2019 (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 that five posters related to the Company's lead product candidate, rigosertib, were accepted for presentation at the 15th International Symposium on Myelodysplastic Syndromes taking place May 8-11, 2019 at the Tivoli Hotel & Congress Center in Copenhagen, Denmark.

Dr. Ric Woodman, CMO of Onconova, will be attending the Symposium and available to meet with interested parties.

### DETAILS OF THE POSTERS:

- 1) PHASE II STUDY OF ORAL RIGOSERTIB COMBINED WITH AZACITIDINE IN PATIENTS WITH HIGHER-RISK MYELODYSPLASTIC SYNDROMES (MDS)**  
Abstract: MDS19-0216  
May 8 – May 11, Room Vandsalen  
Presenter: Lewis R. Silverman, MD  
Tisch Cancer Institute, Division of Hematology/Oncology, Icahn School of Medicine at Mount Sinai, New York, NY
- 2) RIGOSERTIB /AZACITIDINE MODULATES INNATE IMMUNE SIGNALING AND HEMATOPOIETIC GENE PATHWAYS WHEN SEQUENCED IN VITRO, IN THE MYELODYSPLASTIC SYNDROME (MDS)**  
Abstract: MDS19-0201  
May 8 – May 11, Room Vandsalen  
Presenter: Lewis R. Silverman, MD  
Tisch Cancer Institute, Division of Hematology/Oncology, Icahn School of Medicine at Mount Sinai, New York, NY
- 3) EMERGENCE OF PTPN11 MUTATIONS IS ASSOCIATED WITH CLINICAL RESISTANCE TO RIGOSERTIB AND AZACITIDINE IN PATIENTS WITH MYELODYSPLASTIC SYNDROME (MDS)**  
Abstract: MDS19-0195  
May 8 – May 11, Room Vandsalen  
Presenter: Lewis R. Silverman, MD  
Tisch Cancer Institute, Division of Hematology/Oncology, Icahn School of Medicine at Mount Sinai, New York, NY
- 4) UTILITY OF ADAPTIVE TRIAL DESIGN IN HIGHER-RISK MYELODYSPLASTIC (HR-MDS) SYNDROMES IN A PHASE 3 TRIAL OF INTRAVENOUS RIGOSERTIB: INSPIRE TRIAL**  
Abstract: MDS19-0211  
May 8 – May 11, Room Vandsalen  
Presenter: Aref Al-Kali, MD  
Mayo Clinic, Hematology, Rochester, MN
- 5) EX VIVO RESPONSE PROFILING OF RIGOSERTIB IDENTIFIES DISTINCT CLASSES OF RESPONDERS IN ELODYSPLASTIC SYNDROME**  
Abstract: MDS19-0200  
Presenter: Marianne Santaguida, PhD, Director of Scientific Partnerships  
Notable Labs, Foster City, CA

### About Myelodysplastic Syndromes

Myelodysplastic syndromes (MDS) are conditions that can occur when the blood-forming cells in the bone marrow become dysfunctional and thus produce an inadequate number of circulating blood cells. It is frequently associated with the presence of blasts or leukemic cells in the marrow. This leads to low numbers of one or more types of circulating blood cells, and to the need for blood transfusions. In MDS, some of the cells in the bone marrow are abnormal (dysplastic) and may have genetic abnormalities associated with them. Different cell types can be affected, although the most common finding in MDS is a shortage of red blood cells (anemia). Patients with higher-risk MDS may progress to the development of acute leukemia.

## **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). 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. 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 Rigosertib**

Rigosertib, Onconova's lead candidate, is a proprietary Phase 3 small molecule. A key publication demonstrated rigosertib's ability to block cellular signaling by targeting RAS effector pathways (Divakar, S.K., et al., 2016: "A Small Molecule RAS-Mimetic Disrupts RAS Association with Effector Proteins to Block Signaling." Cell 165, 643). Onconova is currently in the clinic with oral and IV rigosertib, including single agent IV rigosertib in second-line high-risk MDS patients (pivotal Phase 3 INSPIRE trial) and oral rigosertib plus azacitidine in first-line high-risk MDS patients (Phase 2). Patents covering oral and injectable rigosertib have been issued in the US and are expected to provide coverage until at least 2037.

## **About IV Rigosertib**

The intravenous form of rigosertib has been studied in Phase 1, 2, and 3 clinical trials involving more than 1000 patients, and is currently being evaluated in a randomized Phase 3 international INSPIRE trial for patients with high-risk MDS (HR-MDS), after failure of hypomethylating agent, or HMA, therapy.

## **About the INSPIRE Phase 3 Clinical Trial**

The **IN**ternational **S**tudy of Phase 3 IV **R**igosertib, or **INSPIRE**, was finalized following guidance received from the U.S. Food and Drug Administration and European Medicines Agency. INSPIRE is a global 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 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. An interim analysis in early 2018 demonstrated a promising survival signal in the intent-to-treat population as reviewed by the Independent Data Monitoring Committee. The Committee recommended that the trial continue with an expansion in enrollment to 360 patients based on a pre-planned sample size re-estimation. Patients are randomized at a 2:1 ratio into two study arms: IV rigosertib plus Best Supportive Care versus Physician's Choice plus Best Supportive Care. The primary endpoint of INSPIRE is overall survival. Full details of the INSPIRE trial, such as inclusion and exclusion criteria, as well as secondary endpoints, can be found on [clinicaltrials.gov](http://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 may also support combination therapy modalities. To date, over 400 patients have been studied with the oral formulation of rigosertib. Combination therapy of oral rigosertib with azacitidine, the standard of care in HR-MDS, has also been studied. 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 1/2 trial of the combination therapy has been fully enrolled, and the preliminary efficacy and safety data was presented at The American Society of Hematology Annual Meeting in December 2018. A pivotal Phase 3 study design is under review by the FDA, and the Special Protocol Assessment is expected to conclude in the 1H of 2019.

## **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 expectations regarding the INSPIRE Trial and Onconova's other development plans. 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 Onconova's ability to continue as a going concern, the need for additional financing, 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**

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