



# ONCONOVA THERAPEUTICS

## Onconova Announces Five Presentations on Rigosertib in Myelodysplastic Syndromes (MDS) at the ASH 2019 Annual Meeting & Exposition

November 7, 2019

- Oral presentation will feature efficacy and safety data from Expanded Phase 2 Trial of oral rigosertib in combination with azacitidine
- Four poster presentations to feature:
  - biomarker genomic studies from pivotal INSPIRE Trial
  - a novel adaptive trial design for a pivotal Phase 3 Study
  - trial design options for future combination study in first line higher risk MDS
  - Influence of rigosertib/azacitidine combination on key pathways involved in in-vitro hematopoiesis

NEWTOWN, Pa., Nov. 07, 2019 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (NASDAQ: ONTX), a Phase 3 stage biopharmaceutical company discovering and developing novel products to treat cancer, with a focus on Myelodysplastic Syndromes (MDS), today announced that five abstracts relating to the Company's lead product candidate, rigosertib, were accepted for presentation at the 61<sup>st</sup> American Society of Hematology (ASH) Annual Meeting & Exposition in Orlando, FL, which takes place December 7-10, 2019.

### Oral Presentation:

**1. Phase II Study of Oral Rigosertib Combined with Azacitidine (AZA) As First Line Therapy in Patients (Pts) with Higher-Risk Myelodysplastic Syndromes (HR-MDS)**

**Session Name:** 637. Myelodysplastic Syndromes – Clinical Studies: Combination Therapies

**Abstract:** 566

**Date:** Monday, December 9, 2019

**Presentation Time:** 7:00 AM - 8:30 AM

**Location:** W311 EFGH (Orange County Convention Center)

**Presenter:** Shyamala C. Navada, MD, Division of Hematology/Oncology, Icahn School of Medicine at Mount Sinai, New York, NY

### Poster Presentations:

**2. Genomic Profiling in Patients with Higher-Risk Myelodysplastic Syndrome (HR-MDS) Following HMA Failure: Baseline Results from the INSPIRE Study (04-30)**

**Session Name:** 637. Myelodysplastic Syndromes – Clinical Studies: Poster II

**Abstract:** 3015

**Date:** Sunday, December 8, 2019

**Session Time:** 6:00 PM – 8:00 PM

**Location:** Hall B (Orange County Convention Center)

**Presenter:** Guillermo Garcia-Manero, MD, Department of Leukemia, The University of Texas MD Anderson Cancer Center, Houston, TX

**3. The INSPIRE Study in Higher-Risk Myelodysplastic Syndrome (HR-MDS): A Novel Phase 3 Study Adaptive Design for Hematological Malignancies in Adults**

**Session Name:** 637. Myelodysplastic Syndromes – Clinical Studies: Poster III

**Abstract:** 4249

**Date:** Monday, December 9, 2019

**Presentation Time:** 6:00 PM - 8:00 PM

**Location:** Hall B (Orange County Convention Center)

**Presenter:** Anna Jonasova, MD, General University Hospital in Prague, 1st Internal Clinic - Clinic of Hematology, Prague, Czech Republic

**4. Phase 3, Multi-Center, International, Randomized, Double-Blind, Placebo Controlled Study of Oral Rigosertib + Injectable Azacitidine (AZA) Versus Injectable Azacitidine in Treatment-Naïve Patients with Higher-Risk Myelodysplastic Syndrome (HR-MDS)**

**Session Name:** 637. Myelodysplastic Syndromes – Clinical Studies: Poster III

**Abstract:** 4268

**Date:** Monday, December 9, 2019

**Session Time:** 6:00 PM – 8:00 PM

**Location:** Hall B (Orange County Convention Center)

**Presenter:** Guillermo Garcia-Manero, MD, Department of Leukemia, The University of Texas MD Anderson Cancer Center, Houston, TX

**5. The Sequenced Combination of Rigosertib and Azacitidine Has Modulatory Effects on CXCL8, RIG-I like Receptor (RLR) and Wnt/ $\beta$ -Catenin Signaling and Downstream Hematopoiesis Pathways in an *In Vitro* Model of the Myelodysplastic Syndrome**

**Session Name:** 636. Myelodysplastic Syndromes – Basic and Translational Studies: Poster III

**Abstract:** 4231

**Date:** Monday, December 9, 2019

**Session Time:** 6:00 PM – 8:00 PM

**Location:** Hall B (Orange County Convention Center)

**Presenter:** Richa Rai, Tisch Cancer Institute, Icahn School of Medicine at Mount Sinai, New York, NY

**About Onconova Therapeutics, Inc.**

Onconova Therapeutics, Inc. is a Phase 3-stage biopharmaceutical company discovering and developing novel small molecule drug candidates to treat cancer, with a 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. 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. Onconova has conducted trials with two other research compounds and has a pre-clinical program with a CDK4/6 and Ark5 inhibitor, ON 123300.

For more information, please visit <http://www.onconova.com>.

**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 Rigosertib**

Rigosertib, Onconova's lead candidate, is a proprietary Phase 3 small molecule. A key publication in a preclinical model 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 clinical development stage with oral and IV rigosertib, including clinical trials studying single agent IV rigosertib in second-line higher-risk MDS patients (pivotal Phase 3 INSPIRE trial) and oral rigosertib plus azacitidine in first-line and refractory higher-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 the INSPIRE Phase 3 Clinical Trial**

The clinical trial **International Study of Phase 3 IV Rigosertib, 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 higher-risk MDS (HR-MDS) patients who had progressed on, failed to respond to, or relapsed after previous treatment with a hypomethylating agent (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. 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. The trial continued beyond the pre-specified interim analysis and is nearing its conclusion. 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 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 HR-MDS after failure of HMA therapy.

**About Oral Rigosertib**

The oral form of rigosertib was developed to provide a potentially more convenient dosage form 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 dosed with the oral formulation of rigosertib in clinical trials. 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 (ASH) Annual Meeting in December 2018 and will be updated at the upcoming ASH Meeting in December 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, our collaborations, 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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