# Onconova Therapeutics and Pint Pharma Announce Brazilian Health Authority Approval for Initiating INSPIRE Trial with Intravenous Rigosertib in Higher-Risk Myelodysplastic Syndromes in Brazil

December 17, 2019

- The INSPIRE Trial enters the final stage of enrollment after surpassing 90% of the required number of randomized patients in November 2019
- Pint Pharma Facilitates Opening of Phase 3 INSPIRE Trial in Brazil
- Topline Data Expected in First Half 2020

NEWTOWN, Pa., Dec. 17, 2019 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (NASDAQ:ONTX), a Phase 3-stage biopharmaceutical company discovering and developing novel products to treat cancer, with an initial focus on myelodysplastic syndromes (MDS), and Pint Pharma, a European-based pharmaceutical company focused on the development, registration and commercialization of specialty-based treatments for the Latin American market, today announced the receipt of approval from the Brazilian Health authority to initiate the INSPIRE Trial in Brazil with intravenous rigosertib in Higher-Risk MDS (HR-MDS). In November 2019 enrollment in the INSPIRE Trial surpassed 90% percent of the required enrollment, and the Company continues to anticipate reporting topline data in the first half of 2020, following full enrollment and reaching the number of required survival events.

“As we enter the final stage of enrollment of the INSPIRE Trial, we thank our corporate partner Pint Pharma for their collaboration in opening the study in Brazil,” said Dr. Steven Fruchtman, President and CEO of Onconova Therapeutics, Inc. “The INSPIRE Trial surpassed 90% percent of the required enrollment in November 2019 and we expect the addition of up to 16 clinical trial sites in Brazil will provide further momentum to our anticipated reporting of topline data in the first half of 2020, following full enrollment and reaching the number of required survival events.”

“We are excited to collaborate with Onconova in opening new clinical sites and look forward to the recruitment of eligible patients for the INSPIRE Trial in Brazil,” said David Munoz, Chief Executive Officer of Pint Pharma. He added, “We are also pleased that local physicians will gain invaluable experience with rigosertib by their participation on the INSPIRE Trial.”

Dr. Ric Woodman, Chief Medical Officer of Onconova, is working closely with Dr. Valnei Canutti, Chief Scientific Officer of Pint. Dr. Woodman commented, “There is a great unmet medical need and interest to conduct studies in patients with HR-MDS in this geographical region. I look forward to a productive collaboration with Pint Pharma and working with Dr. Canutti, an expert in conducting trials in Brazil as well as an expert in MDS.”

Dr. Canutti added, “We anticipate meaningful contributions from Brazil to accrual to the INSPIRE Trial and are excited about receiving health authority approval to initiate INSPIRE in Brazil. There are significant numbers of patients with HR-MDS in Brazil with no approved approach following failure of the standard of care azacitidine. We at Pint, in collaboration with our partner Onconova, look forward to Brazil’s contributions to complete accrual to this important pivotal global trial.”

**About Pint Pharma**

Pint Pharma is devoted to the development, registration, and commercialization of specialty-based treatments. Pint Pharma benefits from leaders with extensive experience in the pharmaceutical sector and who are based strategically throughout Latin America and Europe. Pint Pharma has a long track record of developing strong relationships with global pharmaceutical and healthcare companies. Pint Pharma strives to be the first Pan-Latin American provider of innovative and high value-added treatments within Rare Diseases, Specialty Care, and Oncology.

**About Onconova Therapeutics, Inc.**

Onconova Therapeutics, Inc. is a Phase 3-stage biopharmaceutical company focused on discovering and developing novel products to treat cancer, with an initial 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 **IN**ternational **S**tudy of **P**hase 3 **I**V **R**igos**E**rtib, 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 (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 efficacy and safety data was presented at The American Society of Hematology (ASH) Annual 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, maintain its Nasdaq listing, 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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