Onconova Therapeutics and Mission Bio Partner to Advance Precision Oncology Clinical Trials Employing Single-Cell Genomics

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The alliance will leverage Mission Bio's Tapestri Platform to investigate Onconova's novel cancer therapy rigosertib, through clinical trials

NEWTOWN, Pa. and SOUTH SAN FRANCISCO, Calif., Aug. 12, 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), and Mission Bio, the pioneer in targeted single-cell DNA analysis and precision genomics, today announced that they have formed a collaboration to utilize the Mission Bio Tapestri® Platform for targeted single-cell DNA analysis to study Onconova's novel cancer therapy, rigosertib, through clinical trials.

With the growing complexity of clinical trials, precision biomarkers are needed to reduce the time and costs associated with the drug development cycle. Broad-based sequencing technologies lack the sensitivity to identify the earlier initial single cell events that contain the driver mutations that initiate the oncologic disease. With the Mission Bio Tapestri Platform, researchers can detect rare cancer subclones and co-occurring cancer mutations at the single-cell level, offering a precise way to measure therapy response and disease progression. Supporting the pharma and biopharma industries through clinical trials and commercialization continues to be a focus for Mission Bio.

Ras proteins control cell proliferation, and mutation of this protein can lead to cancer in affected individuals. Ras is mutated in over 30 percent of patients with cancer, making it one of the most sought-after targets. Onconova is developing rigosertib, a first-in-class, small molecule Ras mimetic, to target this mutation. Rigosertib blocks the activation of Ras effector proteins, thus modulating the Ras pathway. Onconova's goal is to fully enroll INSPIRE, its phase 3 clinical trial studying rigosertib in higher-risk MDS patients who fail the current standard of care, by year-end.

"Through single-cell genomics, we can identify mutations with far better resolution than that of traditional sequencing methods. This allows a view into each patient's disease at a level never before achieved," explained Darrin Crisitello, CCO of Mission Bio. "The Tapestri Platform can identify subclones that help monitor a patient's response to research drugs in clinical trials, supporting the advancement of rigosertib to the clinic."

"Rigosertib has the potential to be the first new higher-risk MDS treatment in more than 15 years, for a condition affecting an estimated 59,000 patients with low and higher-risk MDS in the United States alone," said Dr. Steve Fruchtman, CEO of Onconova. "In adding the Tapestri Platform to our research and development program, we are including the opportunity to study single cell clones in MDS and determine the sequence of genetic events and the influence of rigosertib on these events along with clinical outcomes. These studies have the potential to make a meaningful difference in the lives of patients in need."

About Mission Bio

Mission Bio delivers targeted solutions for high impact applications with the Tapestri Platform. The Tapestri Platform is the industry's first single-cell DNA sequencing platform, enabling precise detection of heterogeneity in disease progression and treatment response. Application areas include blood cancers, solid tumors, and genome editing validation. The platform includes an instrument, consumables and software, which plug seamlessly into existing NGS workflows. LabCorp's Covance Drug Development business will be the first global contract research organization to offer services on the Mission Bio Tapestri Platform, supporting biopharmaceutical customers in their need for faster, more precise clinical trials.

The company's Tapestri Platform was also honored as a Top 10 Innovation of 2018 by The Scientist, as well as utilized by researchers at MD Anderson Cancer Center (MDACC) for the largest single-cell study completed to date. With Mission Bio, researchers have a highly sensitive, targeted, and customizable solution to move precision medicine forward.

To learn more about Mission Bio and how it's moving precision medicine forward, visit www.missionbio.com.

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 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 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 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 higher-risk MDS patients (pivotal Phase 3 INSPIRE trial) and oral rigosertib plus azacitidine in first-line 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 **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 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 (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 higher-risk MDS (HR-MDS), after failure of hypomethylating agent, or HMA, therapy.

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 Special Protocol Assessment for a pivotal Phase 3 study design is under review by the FDA.

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.

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