



ONCONOVA THERAPEUTICS

Onconova Therapeutics Announces Cooperative Research and Development Agreement for Rigosertib with the National Cancer Institute

January 4, 2018

- Agreement related to study of rigosertib in pediatric RASopathies
- Clinical trial in pediatric patients to be conducted by the National Cancer Institute
- RASopathies are orphan diseases believed to be caused by molecular defects in the RAS pathway

NEWTOWN, Pa., Jan. 04, 2018 (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 it has entered into a Cooperative Research and Development Agreement (CRADA) with the National Cancer Institute (NCI), part of the National Institutes of Health (NIH). Under the terms of the CRADA, the NCI will conduct research, including preclinical laboratory studies and a clinical trial, on rigosertib in pediatric cancer associated RASopathies.

The RASopathies are a group of rare diseases which share a well-defined molecular basis in expression or defects involving Ras Effector Pathways. They are usually caused by germline mutations in genes that alter the RAS subfamily and mitogen-activated protein kinases that control signal transduction, and are among the most common genetic syndromes. Together, this group of diseases can impact more than 1 in 1000 individuals, according to [RASopathiesNet](#).

Dr. Steve Fruchtman, Chief Medical Officer of Onconova, noted: "We are excited about the potential of our collaboration with the Pediatric Oncology Branch at NCI's Center for Cancer Research in the study of rigosertib in children with both hematological and solid tumors that are driven by the Ras pathway. This collaboration could lead to important advances in the treatment of these refractory tumors. This novel approach directed at a specific mechanism driving the underlying neoplasm is the basis of personalized medicine for these indications".

As part of the CRADA, Onconova will provide rigosertib supplies and initial funding towards non-clinical studies. The NCI will fund the majority of the research, including the cost of the clinical trial, which is expected to start in 2018. A clinical trial protocol has been developed and will be reviewed by the Institutional Review Board.

Onconova is also collaborating with academic researchers and patient advocacy groups interested in developing novel therapeutics to address the needs of these patients. In October 2017, Onconova held a Key Opinion Leader Breakfast Symposium in New York City to bring attention to this unmet medical need and the potential for rigosertib in RASopathies. The meeting featured presentations by Bruce D. Gelb, M.D. (Mount Sinai, New York) and Elliot Stieglitz, M.D. (University of California, San Francisco), alongside Dr. Fruchtman. In July 2017, Onconova also presented a summary of its targeted approach at a symposium organized by RASopathiesNet.org.

While the NCI will conduct a trial for RASopathy related cancers in pediatric patients, Onconova will focus on Juvenile Myelomonocytic Leukemia (JMML), a well-described RASopathy affecting children which is incurable without an allogenic hematopoietic stem cell transplant.

Additional information highlighting Onconova's approach to studying rigosertib in RAS mediated diseases can be found in the presentation, "[Strategies to RASopathies and JMML](#)," located in the "[Scientific Presentations](#)" section of Onconova's website.

For Patients

Patients interested in enrolling please contact NCI's toll-free number 1-800-4-Cancer (1-800-422-6237) (TTY: 1-800-332-8615).

[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). Rigosertib, Onconova's lead candidate, is a proprietary Phase 3 small molecule agent, which we believe blocks cellular signaling by targeting RAS effector pathways. 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, while causing minimal damage to normal 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 IV Rigosertib](#)

The intravenous form of rigosertib has been employed in Phase 1, 2, and 3 clinical trials involving more than 800 patients, and is currently being evaluated in the randomized Phase 3 international INSPIRE trial for patients with higher-risk MDS, after failure of hypomethylating agent, or HMA, therapy. This formulation is intended for patients with advanced disease, provides long duration of exposure, and ensures dosing under a controlled setting.

[About INSPIRE](#)

The **IN**ternational **S**tudy of **P**hase III **IV** **R**igosertib, or INSPIRE, is based on guidance received from the U.S. Food and Drug Administration and European Medicines Agency and derives from the findings of the ONTIME Phase 3 trial. INSPIRE is a 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 the first 9 months or 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. The trial will enroll approximately 225 patients randomized at a 2:1 ratio into two treatment arms: IV rigosertib plus Best Supportive Care versus Physician's Choice plus Best Supportive Care. The primary endpoint of INSPIRE is overall survival and an interim analysis is anticipated. Full details of the INSPIRE trial, such as inclusion and exclusion criteria, as well as secondary endpoints, can be found on [clinicaltrials.gov \(NCT02562443\)](https://clinicaltrials.gov/ct2/show/study/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 also supports many combination therapy modalities. To date, 368 patients have been treated with the oral formulation of rigosertib. Initial studies with single-agent 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. 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 2 trial of the combination therapy has been fully enrolled and the preliminary results were presented in 2016. This novel combination is the subject of an issued US patent with earliest expiration in 2028.

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 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," "will," "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 ability to continue as a going concern, the 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.

General Contact

<http://www.onconova.com/contact/>

Investor Relations Contact

Katja Buhner, Affinity Growth Advisors on behalf of Onconova Therapeutics

Katja.Buhner@affinitygrowth.com / (212) 661-7004

 [Primary Logo](#)

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