



# ONCONOVA THERAPEUTICS

## Onconova to Host Key Opinion Leader Meeting on Novel Approaches to the Diagnosis and Treatment of Pediatric RASopathies on Wednesday, October 11, in New York City

October 4, 2017

NEWTOWN, Pa., Oct. 04, 2017 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (NASDAQ:ONTX), a Phase 3-stage biopharmaceutical company focused on discovering and developing novel products to treat cancer, with a primary focus on myelodysplastic syndromes (MDS), today announced that it will host a Key Opinion Leader (KOL) breakfast meeting on the topic of Novel Approaches to the Diagnosis and Treatment of RASopathies. The event and live webcast will take place on Wednesday, October 11, from 8:00 AM-9:30 AM Eastern Time, in New York City.

The meeting will feature presentations by KOLs, Bruce D. Gelb, M.D. (Mount Sinai, New York), and Elliot Stieglitz, M.D. (University of California San Francisco), who will discuss new diagnostic and research developments for pediatric patients with RASopathies, which are related genetic syndromes usually caused by mutations that alter the Ras subfamily and mitogen activated protein kinases that control signal transduction.

Onconova's management team, including Steven Fruchtmann, M.D., the Chief Medical Officer, will also provide an update on rigosertib, a small molecule inhibitor of Ras signaling pathways (Saikumar et al, Cell, 2016) that is planned to be studied in pediatric patients with Rasopathies complicated by the development of Ras associated cancers. Currently, rigosertib, the Company's lead product candidate, is being evaluated in a global Phase 3 trial (INSPIRE) for higher risk-MDS patients after failure of therapy with hypomethylating agents.

A Q&A session with the featured experts and management will follow the presentations.

This event is intended for institutional investors, sell-side analysts, investment bankers, and business development professionals only. Please [RSVP](#) in advance if you plan to attend, as space is limited. For those who are unable to attend in person, a live webcast and replay will be accessible [here](#).

### KOL Biographical Information:

Bruce D. Gelb, M.D., is the Director and Gogel Family Professor of the Mindich Child Health and Development Institute at the Icahn School of Medicine at Mount Sinai. He is Professor of Pediatrics and of Genetics and Genomic Sciences. Dr. Gelb completed a pediatric residency and pediatric cardiology fellowship at Babies Hospital of Columbia-Presbyterian Medical Center and Texas Children's Hospital at the Baylor College of Medicine, respectively. He joined the faculty at Mount Sinai in 1991 following his fellowship and has remained there since. Dr. Gelb developed and now oversees an extensive program in genomics/gene discovery for congenital heart disease. He has received the E. Mead Johnson Award from the Society for Pediatric Research, and the Norman J. Siegel New Member Outstanding Science Award from the American Pediatric Society. Dr. Gelb was elected to the American Society of Clinical Investigation and the National Academy of Medicine (formerly, the Institute of Medicine). He is the President-Elect for the American Pediatric Society, President for the International Pediatric Research Foundation and a Board Member for the American Society of Human Genetics. In addition to his research, Dr. Gelb co-directs the Cardiovascular Genetics Program at Mount Sinai.

Elliot Stieglitz, M.D., is an assistant professor at the University of California San Francisco (UCSF) in the division of Pediatric Hematology & Oncology. His research focuses on improving outcomes for children diagnosed with myeloid malignancies, particularly those with juvenile myelomonocytic leukemia (JMML), an aggressive hematologic malignancy of childhood. As his primary clinical focus, Dr. Stieglitz is study chair for ADVL1512, a phase II clinical trial sponsored by the Children's Oncology Group (COG) that will evaluate the safety of trametinib in children with relapsed JMML. This trial has recently been approved by the COG and is expected to enroll patients by the end of 2017. His primary laboratory focus is elucidating the mechanism of mutant SETBP1-mediated leukemogenesis, which is associated with the most aggressive form of JMML and is the subject of a K08 award.

### **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 the Company believes 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 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. An expansion of this trial is currently enrolling patients at multiple sites in US, Europe and Australia. This novel combination therapy 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.

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