Onconova Therapeutics, Inc. Reports Business Highlights and Third Quarter 2017 Financial Results

November 9, 2017

NEWTOWN, Pa., Nov. 09, 2017 (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 provided a corporate update and reported financial results for the third quarter ended September 30, 2017.

“We are getting closer to two key milestones for the INSPIRE pivotal trial for IV rigosertib in patients with higher-risk MDS. A pre-planned interim analysis is anticipated in the coming months, and encouraging recent enrollment progress for this global trial suggests full enrollment may be achieved in the first half of 2018,” said Dr. Ramesh Kumar, President and Chief Executive Officer.

“We have also made good progress on our oral rigosertib-azacitidine combination program and plan to initiate a Special Protocol Assessment process with the Food and Drug Administration for a pivotal Phase 3 trial early next year. In addition, we are advancing our collaborative pre-clinical RASopathies drug program and look forward to two poster presentations at the 2017 American Society of Hematology conference.”

INSPIRE Trial of IV Rigosertib in 2nd Line Higher-risk (HR) MDS

Interim Analysis (IA)

- In the quarter, the Company received guidance from the Food and Drug Administration (FDA) and European Medicines Agency (EMA) concerning the statistical analysis plan (SAP) for both the interim and final (top-line) analysis in the INSPIRE trial. Based on guidance received, Onconova finalized the SAP in preparation for the IA.
- The IA will be triggered with the 88th death event in this trial of 225 patients. Based on internal modeling, this could occur in the fourth quarter of 2017 or early 2018. The date of the IA is tied to reaching a pre-identified number of death events. Accordingly, the precise time of completing the IA, which will take place approximately a couple of weeks after reaching the number of events, cannot be accurately forecast.
- This adaptive trial design permits several options after the IA, including a futility analysis, trial expansion using pre-planned statistical criteria, or choosing one of two endpoints (survival analysis of the Intent-to-Treat population or the pre-defined Very High Risk subpopulation).

Trial Progress

- As of October 31, 2017, the INSPIRE study is active at approximately 170 sites in 22 countries across four continents. A final five sites will be opened, which is expected to occur in November.
- The INSPIRE trial has stringent selection criteria so as to identify a more homogenous MDS patient population. Accordingly, extensive eligibility verification and trial site education are integral to the Company’s plan.
- Due to efforts undertaken to increase participation, including the addition and replacement of CROs and opening sites in additional countries, the enrollment rate for the trial increased recently. Consequently, Onconova expects full enrollment to be achieved in the first half of 2018, followed by top-line analysis after 176 death events in the second-half of 2018.

Oral Rigosertib in Combination with Azacitidine for 1st-line HR-MDS

Pivotal Phase 3 Trial Protocol

- Following input received from the FDA in an end-of-phase 2 meeting and from the EMA as part of the scientific advice process, Onconova has designed a Phase 3 protocol. The Company is awaiting the results of the ongoing Phase 1/2 Expansion Trial before engaging in further protocol development. Once the Expansion Trial is complete, which is expected to be in the fourth quarter of 2017, Onconova plans to submit the Pivotal Phase 3 protocol to the FDA in the first half of 2018 to initiate the SPA process with the FDA.

- Initiation of the Phase 3 trial, which is planned to be conducted globally, requires additional financing and/or business development transactions.

- This Expansion Trial is designed to enroll up to approximately 40 patients. More than half of the trial has been accrued in multiple sites in the USA. Based on this progress, the Company has decided to limit the trial to US sites.
- Onconova plans to present initial data from this study at a scientific conference in early 2018, highlighting the results of dose selection and optimization of the combination regimen.

Other Programs for Future Development or Partnership and Presentations

Rigosertib for Pediatric RASopathies

- On October 11, 2017, Onconova hosted a Key Opinion Leader meeting to discuss novel approaches to 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), who discussed new developments for pediatric patients with RASopathies, which are related genetic syndromes usually caused by mutations that alter the Ras subfamily and mitogen activated protein (MAP) kinases that control signal transduction. Onconova's Chief Medical Officer, Steven Fruchtman, M.D., provided an update on rigosertib, which is initially planned to be studied in pediatric patients with RASopathies complicated by the development of associated cancers.
- The Company has completed and expects to sign a Cooperative Research and Development Agreement with the US National Institutes of Health (NIH) to advance rigosertib in pediatric clinical trials at the National Cancer Institute. This trial is expected to start next year and will be funded by the NIH.

Onconova Enters into Strategic Collaboration with Cellectar Biosciences

- On September 21, 2017, the Company announced it had entered into a strategic collaboration with Cellectar Biosciences to develop new phospholipid drug conjugates combining select proprietary compounds or payloads from Onconova's early stage product pipeline with Cellectar' s patented phospholipid ether delivery platform.
- Under the terms of the collaboration, Onconova will provide Cellectar with several compounds, including some from the family of molecules that contains Briciclib, which is an EIF4E targeting small molecule with early Phase 1 data. Cellectar will link the molecules to its phospholipid ether to create new, more precisely targeted antitumor agents.

Upcoming Presentations

Two abstracts relating to the Company's lead product candidate, rigosertib, were accepted for poster presentation at the 59th American Society of Hematology (ASH) Annual Meeting in Atlanta, Georgia, which takes place December 9-12, 2017. Details of the presentations are listed below.

Long-term follow up of patients in a Phase 2 clinical trial of single agent oral rigosertib in lower-risk transfusion dependent MDS

Abstract Number: 1689

Title: Rigosertib Oral in Transfusion Dependent Lower Risk Myelodysplastic Syndromes (LR-MDS): Optimization of Dose and Rate of Transfusion Independence (TI) or Transfusion Reduction (TR) in a Single-Arm Phase 2 Study

Session Name: 637. Myelodysplastic Syndromes - Clinical Studies: Poster I

Date: Saturday, December 9, 2017; 5:30 - 7:30 PM EST

Studies on the mechanism of action of rigosertib azacitidine combination therapy for MDS

Abstract Number: 4235

Title: Effects of Rigosertib (RIGO) Alone or in Combination with Azacitidine or Vorinostat on Epigenetic Reprogramming of CD34+ Cells in the Myelodysplastic Syndrome

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

Date: Monday, December 11, 2017; 6:00 - 8:00 PM EST

Third-Quarter Financial Results:

- Cash, cash equivalents, and marketable securities as of September 30, 2017 totaled $7.6 million, compared to $21.4 million as of December 31, 2016. Based on our cash burn for the first three quarters of 2017 and our current projections, we expect that our cash and cash equivalents will be sufficient to fund our ongoing trials and operations through the end of 2017.
- Total net revenue was $0.1 million for the third quarter of 2017 and $0.6 million for the nine months ended September 30, 2017, compared to $1.7 million and $5.4 million, respectively, for the comparable periods in 2016.
- Research and development expenses were $5.1 million for the third quarter of 2017 and $14.6 million for the nine months ended September 30, 2017, compared to $4.0 million and $15.4 million, respectively, for the comparable periods in 2016.
- General and administrative expenses were $1.7 million for the third quarter of 2017 and $5.6 million for the nine months ended September 30, 2017, compared to $2.0 million and $7.2 million, respectively, for the comparable periods in 2016.

The Company will host a conference call on November 9th at 9:00 a.m. Eastern Time to provide a corporate update and discuss third quarter financial results. Interested parties may access the call by dialing toll-free (855) 428-5741 from the US, or (210) 229-8823 internationally and using conference ID: 3588306.

The call will also be webcast live. Please click here to access the webcast.

A replay will be available at this link until February 23, 2018.

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. Onconova has three product candidates in the clinical stage and several pre-clinical programs. The advanced clinical trial with the Company's lead compound, rigosertib, is 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 (HR) MDS, after failure of hypomethylating agent, or HMA, therapy.

About INSPIRE

The **IN**ternational **S**tudy of **P**hase III **I**V **R**igos**E**rtib, or INSPIRE, trial design was finalized following guidance received from the U.S. Food and Drug Administration and European Medicines Agency. 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 the National Comprehensive Cancer Network (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).

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 1/2 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.

ONCONOVA THERAPEUTICS, INC.

Condensed Consolidated Balance Sheets

(in thousands)

	September 30, 2017	December 31, 2016
Assets	**(unaudited)**	
Current assets:		
Cash and cash equivalents	$ 7,600	$ 21,400
Receivables	57	31
Prepaid expenses and other current assets	1,050	1,638
Total current assets	8,707	23,069
Property and equipment, net	83	152
Other non-current assets	12	12
Total assets	$ 8,802	$ 23,233
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 5,436	$ 5,323
Accrued expenses and other current liabilities	3,098	4,382
Deferred revenue	455	455
Total current liabilities	8,989	10,160
Warrant liability	1,686	3,401
Deferred revenue, non-current	4,205	4,545

Total liabilities	14,880	18,106
Stockholders' equity:		
Preferred stock	-	-
Common stock	99	68
Additional paid in capital	349,103	342,484
Accumulated other comprehensive income	(1)	(31)
Accumulated deficit	(356,109)	(338,224)
Total Onconova Therapeutics Inc. stockholders' (deficit) equity	(6,908)	4,297
Non-controlling interest	830	830
Total stockholders' (deficit) equity	(6,078)	5,127
Total liabilities and stockholders' (deficit) equity	$ 8,802	$ 23,233

ONCONOVA THERAPEUTICS, INC.

Condensed Consolidated Statements of Operations (unaudited)

(in thousands, except share and per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	**2016**	**2017**	**2016**
Revenue	$ 110	$ 1,651	$ 644	$ 5,373
Operating expenses:				
General and administrative	1,728	1,975	5,623	7,229
Research and development	5,141	3,991	14,641	15,377
Total operating expenses	6,869	5,966	20,264	22,606
Loss from operations	(6,759)	(4,315)	(19,620)	(17,233)
Change in fair value of warrant liability	(210)	2,706	1,716	2,985
Other income, net	8	10	19	28
Net loss	$ (6,961)	$ (1,599)	$ (17,885)	$ (14,220)
Net loss per share of common stock, basic and diluted	$ (0.71)	$ (0.29)	$ (2.09)	$ (3.90)
Basic and diluted weighted average shares outstanding	9,851,164	5,438,105	8,551,839	3,643,210

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Source: Onconova Therapeutics, Inc.