Onconova Therapeutics, Inc. Reports Business Highlights and First Quarter 2019 Financial Results

May 14, 2019

NEWTOWN, Pa., May 14, 2019 (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 an initial focus on Myelodysplastic Syndromes (MDS), today provided a corporate update and reported financial results for the first quarter of 2019 ended March 31, 2019.

"As we progress through 2019, we are advancing the clinical development of our pipeline and executing on our collaboration strategy, as highlighted by the recently announced license agreement for investigational rigosertib in Greater China with HanX Biopharmaceuticals (HanX)," said Steven M. Fruchtman, M.D., President and Chief Executive Officer. "2019 is an important year for Onconova and we are working diligently to execute on our objectives. If successful, we believe rigosertib could be the first new treatment for higher-risk MDS (HR-MDS) in more than 15 years."

Dr. Fruchtman added, "Enrollment in our Phase 3 intravenous (IV) rigosertib trial in second-line HR-MDS patients is continuing and is our top priority. We exceeded 75 percent enrollment of the study during the first quarter and are focused on completing enrollment in the second half of 2019 and reporting top-line data following full enrollment and 288 death events. We believe the addition of sites in Brazil and China later this year could contribute significantly to achieving our goal of completing enrollment by year end. We also look forward in the future to initiating a Phase 3 trial with oral rigosertib in combination with azacitidine in first-line HR-MDS patients and to filing an IND in the U.S. for ON 123300, a first-in-class CDK4/6 + ARK5 inhibitor for the treatment of a variety of advanced tumors."

First Quarter 2019 and Recent Highlights

- Entered into rigosertib license agreement with HanX for the development and commercialization of rigosertib in Greater China, which, together with an equity investment at a premium, brings $4 million in cash to the Company. This new HanX license expands the existing collaboration between the two companies. Onconova and HanX previously signed a separate agreement in December 2017 for the pipeline compound ON 123300, Onconova's novel CDK 4/6 inhibitor, which is expected to enter a Phase 1 clinical trial in the U.S. during the second half of 2019.
- Achieved over 75 percent enrollment in the INSPIRE study during the first quarter, and remain focused on completing enrollment in the second half of 2019 and reporting top-line data following full enrollment and 288 death events.
- More than 140 trial sites in 23 countries across four continents are open, including 21 sites in Japan. Opened 19 new clinical trial sites in 8 already participating countries to support completion of enrollment of 360 patients in the Phase 3 INSPIRE study. Additional geographies are being opened during the coming months to add approximately 25 more sites.
- Attended MDS Symposium in Copenhagen May 8-11, 2019, for which five posters related to rigosertib were accepted for presentation.

Oral Rigosertib in Combination with Azacitidine for First-Line HR MDS Trial Progress and Near-Term Milestones

- In December 2018, Onconova submitted an application for a SPA to the FDA for a Phase 3 Trial of oral rigosertib in combination with azacitidine for treatment of first-line higher-risk MDS adult patients. The Company is currently in discussions with the FDA regarding the SPA. Upon agreement regarding the SPA, Onconova hopes to initiate the Phase 3 study with the support of a partnership.

Business Development Progress for Rigosertib and Pipeline Products

- Onconova entered into a new license agreement with HanX to develop and commercialize rigosertib in Greater China. HanX is a China-based pharmaceutical company focused on the development, registration, and commercialization of therapeutics for China. Under the terms of the agreement, Onconova has granted to HanX an exclusive license to develop and commercialize rigosertib in Greater China. In exchange for these rights, HanX will pay a $2 million up-front payment and make an additional $2 million equity investment in Onconova stock at a premium to market. In addition, HanX will initially dedicate $2 million in local currency to fund rigosertib development in China over the next two years and will be responsible for future development costs of the product in China pursuant to a joint development plan. HanX will make additional regulatory, developmental, and sales-based milestone payments to Onconova of up to $45.5 million and pay Onconova tiered royalties up to double digits on net sales in Greater China. If approval is received, Onconova will supply the finished product to HanX for development and commercialization. HanX also will support Onconova's other clinical trial initiatives in Greater China.
- ON 123300, an investigational first-in-class dual inhibitor of CDK4/6 + ARK5 with the potential to treat a variety of cancers, continues to make progress toward clinical development in the U.S. and China in partnership with HanX. HanX has

conducted toxicology studies to support an Onconova IND filing in the U.S., anticipated in the second quarter of 2019.

- Collaboration with the National Cancer Institute is ongoing for preclinical studies of rigosertib for treatment of pediatric cancer associated RASopathies.
- Scientific presentations related to rigosertib development and clinical trials were made at the American Association for Cancer Research Annual Meeting, the MDS Symposium in Copenhagen, and the Acute Leukemia Forum in Newport, CA; upcoming presentations will be made at the Acute Leukemia Forum in China, and the European Hematology Association Congress. Onconova will also attend the American Society of Clinical Oncology (ASCO) 2019 Annual Meeting in June.

First Quarter 2019 Financial Results

Cash and cash equivalents as of March 31, 2019, totaled $10.4 million compared to $17.0 million at December 31, 2018. The Company expects that cash and cash equivalents at March 31, 2019 will be sufficient to fund ongoing trials and operations into the fourth quarter of 2019. After receiving the upfront proceeds from the HanX rigosertib transaction, the Company expects that its cash and cash equivalents will be sufficient to fund ongoing trials and operations into the first quarter of 2020.

Net loss was $7.6 million for the quarter ended March 31, 2019, compared to $5.1 million for the first quarter ended March 31, 2018. Research and development expenses were $4.1 million for the quarter ended March 31, 2019, and $4.6 million for the comparable period in 2018. General and administrative expenses were $3.2 million for the quarter ended March 31, 2019, and $1.9 million for the comparable period in 2018.

Conference Call and Webcast Information

The Company will host a conference call today, May 14, at 9 a.m. Eastern Time, to provide a corporate update and discuss first quarter 2019 financial results. Interested parties may access the call by dialing toll-free (855) 428-5741 from the U.S., or internationally (210) 229-8823 and using conference ID: 4275108. The call will also be webcast live. Please click here to access the webcast. A replay will be available following the live webcast.

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 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). 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 Rigosertib

Rigosertib, Onconova's lead candidate, is a proprietary Phase 3 small molecule. A key publication presented 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 high-risk MDS patients (pivotal Phase 3 INSPIRE trial) and oral rigosertib plus azacitidine in first-line high-risk MDS patients (Phase 1/2). Patents covering oral and injectable rigosertib have been issued in the U.S. and are expected to provide coverage until at least 2037.

A**bout 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 high-risk MDS (HR-MDS), after failure of hypomethylating agent, or HMA, therapy.

About the INSPIRE Phase 3 Clinical Trial

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 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 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. The Company is in discussions with FDA regarding a Special Protocol Assessment for a pivotal Phase 3 study of oral rigosertib in combination with azacitidine.

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 our products, the INSPIRE Trial and other clinical trials, our other development plans and our collaboration with HanX. 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, our collaboration with HanX, and those discussed under the heading "Risk Factors" in Onconova's most recent Annual Report on Form 10-K and quarterly report 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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ONCONOVA THERAPEUTICS, INC.
Condensed Consolidated Balance Sheets
(in thousands)

	March 31, 2019	December 31, 2018
Assets	*(unaudited)*	
Current assets:		
Cash and cash equivalents	$ 10,396	$ 16,970
Receivables	35	35
Prepaid expenses and other current assets	759	760
Total current assets	11,190	17,765
Property and equipment, net	1	9
Other non-current assets	150	149
Total assets	$ 11,341	$ 17,923
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,334	$ 4,039
Accrued expenses and other current liabilities	3,849	4,173
Deferred revenue	226	226
Total current liabilities	8,409	8,438
Warrant liability	603	176
Deferred revenue, non-current	3,865	3,922
Total liabilities	12,877	12,536
Stockholders' (deficit) equity:		
Preferred stock	-	-
Common stock	59	57
Additional paid in capital	387,919	387,238
Accumulated other comprehensive income	(18)	(12)
Accumulated deficit	(389,496)	(381,896)
Total Onconova Therapeutics Inc., stockholders' deficit	(1,536)	5,387
Non-controlling interest	-	-
Total stockholders' (deficit) equity	(1,536)	5,387
Total liabilities and stockholders' (deficit) equity	$ 11,341	$ 17,923

ONCONOVA THERAPEUTICS, INC.

Condensed Consolidated Statements of Operations *(unaudited)*

(in thousands, except share and per share amounts)

	Three months ended March 31,	
	2019	**2018**
Revenue	$ 68	$ 564
Operating expenses:		
General and administrative	3,234	1,889
Research and development	4,075	4,577
Total operating expenses	7,309	6,466
Loss from operations	(7,241)	(5,902)
Change in fair value of warrant liability	(427)	812
Interest income	68	-
Net loss	(7,600)	(5,090)
Net loss attributable to non-controlling interest	-	-
Net loss attributable to Onconova Therapeutics, Inc	$ (7,600)	$ (5,090)
Net loss per share of common stock, basic and diluted	$ (1.29)	$ (5.04)
Basic and diluted weighted average shares outstanding	5,890,098	1,009,244