Onconova Therapeutics, Inc. Reports Business Highlights and Full Year 2018 Financial Results

March 26, 2019

NEWTOWN, Pa., March 26, 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 a primary focus on Myelodysplastic Syndromes (MDS), today provided a corporate update and reported financial results for the fourth quarter and fiscal year ended December 31, 2018.

“This year was a period of important progress for Onconova, as we advanced our clinical pipeline, strengthened our balance sheet and senior leadership team, advanced our business development activities and expanded our intellectual property estate,” said Steven M. Fruchtman, M.D., President and Chief Executive Officer. “As we move through 2019, we anticipate continued progress across the Company, including recruiting for our Phase 3 IV rigosertib trial in second-line higher-risk MDS (HR-MDS) patients and advancing oral rigosertib in combination with azacitidine toward a pivotal phase 3 trial in first-line HR-MDS patients. In addition, we have surpassed the 75% accrual figure and anticipate completion of recruitment to the pivotal Phase 3 INSPIRE trial in the second half of 2019. We anticipate reporting top-line data following full enrollment and reaching 288 death events.”

2018 and Recent Highlights

- Executed succession plan by promoting Steven M. Fruchtman, M.D., who previously served as Chief Medical Officer, to President and Chief Executive Officer upon the transition of Ramesh Kumar, Ph.D., to an advisory role
- Strengthened the senior leadership team with the appointment of Richard Woodman, M.D., as Chief Medical Officer and Senior Vice President of Research and Development; and Avi Oler, J.D., M.B.A., as Vice President, Corporate Development and General Counsel
- Achieved greater than 75% enrollment in the INSPIRE study, and enrollment is expected to be completed in 2019
- Opening of new geographical areas to expedite completion of enrollment in the INSPIRE study
- A new patent was issued covering oral and IV formulations of rigosertib by the U.S. Patent and Trademark Office extending the Company’s patent for rigosertib to 2037
- Hosted a well-attended Key Opinion Leader Breakfast on February 7, 2019, focused on rigosertib development and earlier-stage programs

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

- Reported promising efficacy and acceptable safety profile from the Phase 2 study of a higher dose of oral rigosertib in combination with azacitidine (Vidaza®) in patients with HR-MDS at the 60th American Society of Hematology (ASH) Annual Meeting in December 2018
- Overall response rate of 90% reported in Onconova’s multi-institutional Phase 2 study in hypomethylating agent (HMA) naïve patients demonstrating a complete remission rate of 34%
- Submitted a Special Protocol Assessment (SPA) to the FDA for a Phase 3 Trial of oral rigosertib in combination with azacitidine (Vidaza®) for treatment of first-line HR-MDS, and are in discussions with the FDA to finalize the protocol

Business Development Progress for Rigosertib and Pipeline Products

- Entered into a license agreement with Pint Pharma to commercialize rigosertib in Latin America including an up to $2.5 million investment by Pint Pharma and up to $42.75 million in regulatory and sales milestones
- ON 123300, a first-in-class dual inhibitor of CDK4/6 + ARK5 with the potential to treat a variety of cancers, is advancing toward clinical development in partnership with HanX Biopharmaceuticals, Onconova’s greater China collaborator. HanX has begun manufacturing the compound and initiated toxicology studies to support an IND filing in the U.S., anticipated in the first half of 2019
- Collaboration ongoing with preclinical studies of rigosertib for pediatric cancer associated RASopathies
- Scheduled scientific presentations on rigosertib development and clinical trials at the American Association for Cancer Research (AACR) Annual Meeting, MDS Symposium in Copenhagen, European Hematology Association (EHA) Congress, and American Society of Clinical Oncology (ASCO) Annual Meeting
- Discussions with potential partners are ongoing

Year End 2018 Financial Results

Cash and cash equivalents as of December 31, 2018, totaled $17.0 million, compared to $4.0 million as of December 31, 2017. Based on current

projections, the Company expects that cash and cash equivalents will be sufficient to fund ongoing trials and operations into the fourth quarter of 2019.

Net loss was $20.4 million for the year ended December 31, 2018, compared to $24.1 million for the year ended December 31, 2017, primarily due to cost controls resulting in lower operating expenses and improved revenue in 2018 from collaboration agreements executed during the first half of 2018. Research and development expenses were $16.9 million for the year ended December 31, 2018, and $19.1 million for the comparable period in 2017. General and administrative expenses were $7.6 million for the year ended December 31, 2018, and $7.4 million for the comparable period in 2017.

Conference Call and Webcast Information

The Company will host a conference call today, March 26, at 9 a.m. Eastern Time, to provide a corporate update and discuss year-end 2018 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: 1878978. 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 agent. 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 high-risk MDS patients (pivotal Phase 3 INSPIRE trial) and oral rigosertib plus azacitidine in first-line high-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 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

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 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. 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 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 pivotal Phase 3 study design is under review by the FDA, and the Special Protocol Assessment is expected to conclude in the 1H of 2019.

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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ONCONOVA THERAPEUTICS, INC.

Condensed Consolidated Balance Sheets

(in thousands)

	December 31, 2018	December 31, 2017
Assets	*(unaudited)*	
Current assets:		
Cash and cash equivalents	$ 16,970	$ 4,024
Receivables	35	59
Prepaid expenses and other current assets	760	820
Total current assets	17,765	4,903
Property and equipment, net	9	64
Other non-current assets	149	12
Total assets	$ 17,923	$ 4,979
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,039	$ 6,186
Accrued expenses and other current liabilities	4,173	3,335
Deferred revenue	226	455
Total current liabilities	8,438	9,976
Warrant liability	176	1,773
Deferred revenue, non-current	3,922	4,091
Total liabilities	12,536	15,840
Stockholders' equity (deficit):		
Preferred stock	-	-
Common stock	57	8
Additional paid in capital	387,238	350,614
Accumulated other comprehensive income	(12)	3
Accumulated deficit	(381,896)	(362,316)
Total Onconova Therapeutics Inc. stockholders' equity (deficit)	5,387	(11,691)
Non-controlling interest	-	830
Total stockholders' equity (deficit)	5,387	(10,861)
Total liabilities and stockholders' equity	$ 17,923	$ 4,979

ONCONOVA THERAPEUTICS, INC.

Condensed Consolidated Statements of Operations

(in thousands, except share and per share amounts)

	Year Ended December 31,	
	2018	2017
	(unaudited)	
Revenue	$ 1,228	$ 787
Operating expenses:		
General and administrative	7,586	7,405
Research and development	16,924	19,119
Total operating expenses	24,510	26,524
Loss from operations	(23,282)	(25,737)
Change in fair value of warrant liability	1,597	1,628
Other income, net	1,151	30
Net loss before income taxes	(20,534)	(24,079)
Income tax (benefit) expense	(124)	13
Net loss	(20,410)	(24,092)
Net gain attributable to non-controlling interest	(163)	-
Net loss attributable to Onconova Therapeutics, Inc	$ (20,573)	$ (24,092)
Net loss per share of common stock, basic and diluted	$ (4.99)	$ (40.15)
Basic and diluted weighted average shares outstanding	4,124,073	600,022