

March 27, 2017

Onconova Therapeutics, Inc. Reports Recent Business Highlights and Year-end 2016 Financial Results

NEWTOWN, Pa., March 27, 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, today provided a corporate update and reported financial results for the year ended December 31, 2016.

"We are pleased to report that our lead clinical candidate, rigosertib, is positioned for multiple key milestones, following the advancement of our late stage trials for patients with myelodysplastic syndromes (MDS). Enrollment for the targeted INSPIRE pivotal trial for patients with second-line higher-risk (HR) MDS is on track, with trial sites currently active across 17 countries on four continents. The trial eligibility criteria are highly selective and require extensive search to identify appropriate patients meeting the stringent entry criteria. We intend to maintain current momentum, with interim analysis expected in the second half of the year and full enrollment by the first quarter of 2018," said Dr. Ramesh Kumar, President and Chief Executive Officer.

"Development of oral rigosertib in combination with azacitidine for first line HR MDS patients is advancing as planned, with the protocol for a pivotal Phase 3 trial expected to be ready for submission to FDA for a Special Protocol Assessment during the second or third quarter of this year. MDS represents a growing and underserved market, with more than 10,000 patients diagnosed with HR-MDS annually in the U.S. No new therapy has been approved in over ten years and none has ever been approved for patients after the failure of hypomethylating agents."

"We will continue to focus on our lead programs and will look for partnering or other opportunities for our other pipeline and clinical programs. We will be presenting non-clinical data on pipeline compounds at the upcoming American Association for Cancer Research conference," added Dr. Kumar.

Recent Business Highlights:

Enrollment on Track for INSPIRE Pivotal Trial of IV Rigosertib in 2nd line HR-MDS

- 1 The global Phase 3 INSPIRE trial of IV rigosertib in patients who have failed to respond to or progressed following hypomethylating agent (HMA) therapy is active in sites across four continents in 16 countries, which is in line with the Company's plan. Our partner, Symbio Pharmaceuticals, has opened an additional 33 sites in Japan in collaboration on the INSPIRE protocol. The trial is expected to be active in all 19 countries in the second quarter of 2017 following the activation of sites in Switzerland and the Netherlands.
- 1 Pre-planned interim analysis for the global INSPIRE trial is currently on track and will be conducted after 88 death events have occurred.

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

- 1 Following a productive end of Phase 2 meeting with the Food and Drug Administration (FDA) in the third quarter of 2016, a protocol for a pivotal Phase 3 trial is being developed. The Company expects to submit this protocol for review by regulatory agencies in the US and Europe in the second or third quarter of 2017.
- 1 The pivotal trial will be designed as a 1:1 randomized placebo controlled trial of oral rigosertib plus azacitidine, compared to azacitidine plus placebo. The Company plans to use a full dose of azacitidine, as defined in the product insert. The patient population studied on this trial will be HMA naïve HR MDS patients. The primary endpoint for assessment of efficacy will be Response Rate of complete remission (CR) + partial remission (PR,) as per the International Working Group (IWG) 2006 Response criteria.
- 1 Formal FDA review will be sought via the Special Protocol Assessment (SPA) mechanism.
- 1 Further details, including sample size and other criteria will be available post regulatory review, anticipated in the second half of 2017. While the pivotal trial is being designed, we plan to expand the trial cohort with the view of further dose optimization for the planned pivotal Phase 3 Trial.

Upcoming Events and Conferences

- 1 Sachs Cancer Bio Partnering & Investor Forum, New York City: March 28, 2017

- | American Association for Cancer Research (AACR), Washington, D.C.: April 1-5, 2017

2016 Financial Results

- | Cash and cash equivalents as of December 31, 2016, totaled \$21.4 million, compared to \$19.8 million as of December 31, 2015. Onconova believes that its current cash and cash equivalents will be sufficient to fund its ongoing trials and operations into the fourth quarter of 2017.
- | Net loss was \$19.7 million for the year ended December 31, 2016, compared to \$24.0 million for the year ended December 31, 2015, primarily due to the change in fair value of warrant liability in the 2016 period related to warrants issued in the July 2016 rights offering.
- | Research and development expenses were \$20.1 million for the year ended December 31, 2016, compared to \$25.9 million for the year ended December 31, 2015.
- | General and administrative expenses were \$9.2 million for the year ended December 31, 2016, compared to \$9.5 million for the year ended December 31, 2015.

"During 2016, we sharpened our focus and were able to reduce costs while making progress on our lead programs. We are encouraged with the interest from potential partners and importantly, look forward to advancing our clinical programs," concluded Dr. Kumar.

Earnings Call

The Company will host a conference call on March 27th at 9:00 a.m. Eastern Time to provide a corporate update and discuss full year 2016 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: 79008554.

The call will also be webcast live at: <http://investor.onconova.com/events.cfm>

A replay will be available at that link until June 30, 2017.

[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 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 our lead compound, rigosertib, are aimed at what we believe 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 trial 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 and provides long duration of exposure and ensures dosing under a controlled setting.

[About INSPIRE](#)

The **I**nternational **S**tudy of Phase III **I**V **R**igos**E**rtib, 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 nine cycles of 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 a 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 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)

	December 31, 2016	December 31, 2015
	<i>(unaudited)</i>	
Assets		
Current assets:		
Cash and cash equivalents	\$ 21,400	\$ 19,799
Receivables	31	1,504
Prepaid expenses and other current assets	1,638	1,882
Total current assets	23,069	23,185
Property and equipment, net	152	248
Other non-current assets	12	12
Total assets	<u>\$ 23,233</u>	<u>\$ 23,445</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	\$ 5,323	\$ 3,421
Accrued expenses and other current liabilities	4,382	3,729
Deferred revenue	455	455
Total current liabilities	10,160	7,605
Warrant liability	3,401	-
Deferred revenue, non-current	4,545	5,000
Total liabilities	<u>18,106</u>	<u>12,605</u>
Stockholders' equity:		
Preferred stock	-	-
Common stock	68	25
Additional paid in capital	342,484	328,564

Accumulated other comprehensive income	(31)	(22)
Accumulated deficit	<u>(338,224)</u>	<u>(318,557)</u>
Total Onconova Therapeutics Inc. stockholders' equity	4,297	10,010
Non-controlling interest	<u>830</u>	<u>830</u>
Total stockholders' equity	<u>5,127</u>	<u>10,840</u>
Total liabilities and stockholders' equity	<u>\$ 23,233</u>	<u>\$ 23,445</u>

Year Ended December 31,

2016 2015

(unaudited)

Revenue	\$ 5,546	\$ 11,456
Operating expenses:		
General and administrative	9,178	9,533
Research and development	<u>20,071</u>	<u>25,895</u>
Total operating expenses	<u>29,249</u>	<u>35,428</u>
Loss from operations	(23,703)	(23,972)
Change in fair value of warrant liability	3,988	-
Other income, net	<u>62</u>	<u>(35)</u>
Net loss before income taxes	(19,653)	(24,007)
Income taxes	<u>14</u>	<u>16</u>
Net loss	(19,667)	(24,023)
Net loss attributable to non-controlling interest	-	44
Net loss attributable to Onconova Therapeutics, Inc	<u>\$ (19,667)</u>	<u>\$ (23,979)</u>
Net loss per share of common stock, basic and diluted	<u>\$ (4.44)</u>	<u>\$ (10.54)</u>
Basic and diluted weighted average shares outstanding	<u>4,426,639</u>	<u>2,273,976</u>

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INVESTOR RELATIONS CONTACT:

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