

## Onconova Therapeutics, Inc. Reports Second Quarter 2014 Financial and Operational Results

NEWTOWN, Pa., Aug. 13, 2014 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (Nasdaq:ONTX), a clinical-stage biopharmaceutical company focused on discovering and developing novel products to treat cancer, today provided a corporate update and reported financial results for the second quarter ended June 30, 2014.

"Onconova made substantial progress during the second quarter toward our highest strategic priority, the efficient development of rigosertib for patients with myelodysplastic syndromes (MDS). Top-line results from the Phase 3 ONTIME trial of rigosertib suggest a treatment benefit for those patients who do not respond to initial treatment with the current standard of care, hypomethylating agents (HMAs); these patients are referred to as 'Primary HMA Failures.' We understand, based on our meeting with the FDA, that an indication could be sought specifically for the patients who had Primary HMA Failure," said Ramesh Kumar, Ph.D., President and CEO of Onconova. "Because the majority of MDS patients do not respond to initial treatment with HMAs, this patient population represents an unmet medical need as well as a significant market opportunity. Onconova expects to update its clinical development plan for rigosertib IV in patients with Primary HMA Failure in the fourth quarter of 2014. Consistent with our plan, we are also making adjustments to our work-force and expenditures."

### Recent Business Highlights:

#### Development of Rigosertib IV in Higher Risk MDS (HR-MDS)

- In the second quarter, Onconova met with the FDA to seek regulatory guidance for development of rigosertib IV for HR-MDS patients. Based on these discussions, we are designing an approval-track trial in the subset of patients with Primary HMA Failure.
- Together with our European partner, Baxter, Onconova met with several European national regulatory agencies and these discussions have also affirmed the unmet medical need in Primary HMA Failure patients.

#### Development of Oral Rigosertib in Lower Risk MDS (LR-MDS)

- Enrollment continues in a cohort of 20 LR-MDS patients aimed at expanding the data on the utility of a prognostic genomic methylation marker for identification of patients likely to respond to rigosertib.
- Recruitment is also continuing in a second Phase 2 trial (09-07) to explore dose and schedule optimization.
- Onconova is comparing continuous dosing with interrupted (two out of three weeks) dosing in a three-week treatment cycle in these ongoing trials.
- Based on the anticipated timing of genomic methylation signature and dosing optimization data, Onconova now believes that the earliest a pivotal study of oral rigosertib in LR-MDS patients could begin is in the first half of 2015.

#### Combination Therapy with Oral Rigosertib and Azacitidine (MDS & AML)

- All three Phase 1 cohorts combining the indicated dose of azacitidine (given during the second week of a four-week cycle) with escalating doses of rigosertib (given during the first three weeks of a four-week cycle) have been successfully completed. The combination therapy was well tolerated in the study population.
- The combination dosing schedule of oral rigosertib in the final cohort (560/280 mg BID) with the indicated dose of azacitidine has been selected for further exploration.
- The Phase 2 portion of this trial is now active at multiple sites (U.S. and Europe).

#### Development of Oral Rigosertib in Head and Neck (H&N) and Other Carcinomas

- A single-agent Phase 2 study in second and third line H&N and other refractory carcinoma patients indicated that oral rigosertib was well tolerated in these advanced cancer patients. Stable disease lasting up to nine months was the best response noted in H&N cancer. One stable disease each in lung and anal carcinomas were also noted. We have concluded, however, that there was not sufficient justification for further development of oral rigosertib as a single agent in these indications.
- A Phase 1 study of oral rigosertib in combination with chemoradiotherapy (platinum plus radiation) has been initiated in H&N and other carcinoma patients. We expect to have evaluable data from this study in 2015.

### Timeline of Planned Events

- Update on the development plan, following regulatory review, for rigosertib IV in HR-MDS: fourth quarter of 2014.
- Data from genomic signature and dose optimization studies in LR-MDS patients: fourth quarter of 2014.
- Presentation of results of Phase 1 portion of the rigosertib-azacitidine combination study: fourth quarter of 2014.
- Presentation of results of clinical trials (including ONTIME) and mechanism of action (biological plausibility) studies of rigosertib in MDS at scientific conferences: fourth quarter of 2014 and first quarter of 2015.

## Operational Updates

- The Company has implemented a reduction in headcount of approximately 15%.
- Consistent with our new development plan, the Company is also making appropriate adjustments to overall expenditures, primarily related to clinical programs beyond the focus area, CMC, non-clinical development and its earlier stage pipeline.

## Second Quarter and Six Months 2014 Financial Results

- Cash, cash equivalents, and marketable securities as of June 30, 2014 totaled \$70.5 million, compared to \$84.6 million as of March 31, 2014 and \$100.0 million as of December 31, 2013.
- Total net revenue was \$0.1 million for the second quarter of 2014 and \$0.6 million for the six months ended June 30, 2014, compared to \$0.6 million and \$1.7 million for the comparable periods in 2013.
- Research and development expenses were \$12.9 million for the second quarter of 2014 and \$27.2 million for the six months ended June 30, 2014, compared to \$10.0 million and \$22.8 million for the comparable periods in 2013.
- General and administrative expenses were \$4.0 million for the second quarter of 2014 and \$8.9 million for the six months ended June 30, 2014, compared to \$3.1 million and \$6.5 million for the comparable periods in 2013.

## Today's Conference Call at 4:30 PM ET

Onconova will host a conference call and audio webcast to discuss its second quarter 2014 financial results this afternoon at 4:30 PM ET. A live webcast will be available at [this link](#) or can be accessed by visiting "Events and Presentations" in the Investors and Media section of the Company's website at [www.onconova.com](http://www.onconova.com). The call can be accessed by dialing (877) 312-5881 (domestic) or (253) 237-1173 (international) five minutes prior to the start time and providing the Conference ID 84354714. A replay of the webcast will be available shortly after the conclusion of the call and will be archived on the Company's website for two weeks following the call.

Note: This will be our last quarterly earnings call for foreseeable future. We will communicate frequently with analysts and investors, but outside of the earnings season rush, and as and when significant disclosures and progress warrant.

## About Onconova Therapeutics, Inc.

Onconova Therapeutics is a clinical-stage biopharmaceutical company focused on discovering and developing novel products to treat cancer. Onconova's clinical and pre-clinical stage drug development candidates are derived from its proprietary chemical library and are designed to work against specific cellular pathways that are important in cancer cells, while causing minimal damage to normal cells. In addition to rigosertib, the Company's most advanced product candidate, two other candidates, briciclib and recilisib, are in clinical trials, and several candidates are in pre-clinical stages. For more information, please visit <http://www.onconova.com>.

## 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, which 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, expectations regarding the sufficiency of Onconova's cash balance 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 those discussed under the heading "Risk Factors" in our Annual Report on Form 10-K for the year ended December 31, 2013 filed by Onconova with the Securities and Exchange Commission on March 20, 2014.

Any forward-looking statements contained in this release speak only as of its date. We undertake 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)*

	<u>June 30,</u> <u>2014</u>	<u>December 31,</u> <u>2013</u>
<b>Assets</b>	<b>(unaudited)</b>	
Current assets:		
Cash and cash equivalents	\$ 55,499	\$ 60,009
Marketable securities	14,997	39,994
Prepaid expenses and other current assets	<u>3,331</u>	<u>4,387</u>
Total current assets	73,827	104,390
Property and equipment, net	609	626
Other non-current assets	<u>137</u>	<u>137</u>
Total assets	<u><u>\$ 74,573</u></u>	<u><u>\$ 105,153</u></u>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 5,027	\$ 3,710
Accrued expenses and other current liabilities	6,465	5,840
Deferred revenue	<u>455</u>	<u>788</u>
Total current liabilities	11,947	10,338
Deferred revenue, non-current	13,682	13,909
Other	<u>2</u>	<u>6</u>
Total liabilities	<u><u>25,631</u></u>	<u><u>24,253</u></u>
Stockholders' equity:		
Preferred stock	--	--
Common stock	217	215
Additional paid in capital	314,630	311,093
Accumulated other comprehensive income	--	1
Accumulated deficit	<u>(266,328)</u>	<u>(230,896)</u>
Total Onconova Therapeutics Inc. stockholders' equity	48,519	80,413
Non-controlling interest	<u>423</u>	<u>487</u>
Total stockholders' equity	<u><u>48,942</u></u>	<u><u>80,900</u></u>
Total liabilities and stockholders' equity	<u><u>\$ 74,573</u></u>	<u><u>\$ 105,153</u></u>

**Onconova Therapeutics, Inc.**  
**Condensed Consolidated Statements of Operations (unaudited)**  
*(in thousands, except share and per share amounts)*

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2014</u>	<u>2013</u>	<u>2014</u>	<u>2013</u>
Revenue	\$ 125	\$ 591	\$ 572	\$ 1,707
Operating expenses:				

General and administrative	3,985	3,117	8,917	6,463
Research and development	<u>12,904</u>	<u>10,047</u>	<u>27,152</u>	<u>22,803</u>
Total operating expenses	<u>16,889</u>	<u>13,164</u>	<u>36,069</u>	<u>29,266</u>
Income (loss) from operations	(16,764)	(12,573)	(35,497)	(27,559)
Change in fair value of warrant liability	3	(2)	19	12
Other income, net	<u>(19)</u>	<u>13</u>	<u>(18)</u>	<u>140</u>
Net loss	(16,780)	(12,562)	(35,496)	(27,407)
Net loss attributable to non-controlling interest	<u>27</u>	<u>--</u>	<u>64</u>	<u>--</u>
Net loss attributable to Onconova Therapeutics, Inc	(16,753)	(12,562)	(35,432)	(27,407)
Accretion of redeemable convertible preferred stock	<u>--</u>	<u>(1,032)</u>	<u>--</u>	<u>(2,051)</u>
Net loss applicable to common stockholders	<u>\$ (16,753)</u>	<u>\$ (13,594)</u>	<u>\$ (35,432)</u>	<u>\$ (29,458)</u>
Net loss per share of common stock, basic and diluted	<u>\$ (0.77)</u>	<u>\$ (5.21)</u>	<u>\$ (1.64)</u>	<u>\$ (11.29)</u>
Basic and diluted weighted average shares outstanding	<u>21,658,625</u>	<u>2,609,495</u>	<u>21,613,713</u>	<u>2,608,450</u>

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