Onconova Therapeutics Announces the Presentation of Preliminary Clinical Data Providing Evidence of Rigosertib's Activity in RDEB-associated Squamous Cell Carcinoma

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Initial single patient data in this ultra-rare indication show that rigosertib monotherapy led to sustained complete response of all target lesions without signs of metastatic disease

NEWTOWN, Pa., Dec. 02, 2021 (GLOBE NEWSWIRE) -- Onconova Therapeutics, Inc. (NASDAQ: ONTX), a clinical-stage biopharmaceutical company focused on discovering and developing novel products for patients with cancer, today announced that early preliminary data from an investigator-initiated Phase 2 open label trial of rigosertib monotherapy in advanced squamous cell carcinoma complicating recessive dystrophic epidermolysis bullosa (RDEB-associated SCC) were presented at the Austrian Society of Dermatology and Venerology Annual Conference 2021, which took place from November 25 – 27, 2021.

RDEB is an ultra-rare condition caused by a lack of type VII collagen protein expression. Type VII collagen protein is responsible for anchoring the skin's inner layer to its outer layer, and its absence leads to extreme skin fragility and chronic wound formation in RDEB patients. Over time, many of these patients develop squamous cell carcinomas (SCCs) that typically arise in areas of chronic skin wounding and inflammation. Preclinical investigations demonstrated overexpression of polo like kinase 1 (PLK1) in RDEB-associated SCC tumor cells. These tumors show a highly aggressive, early metastasizing course, making them the primary cause of death for these patients, with a cumulative risk of death of 70% and 78.7% by age 45 and 55, respectively[1,2]. These neoplasms show limited response rates of mostly short duration to conventional chemo- and radiotherapy as well as targeted therapy with epidermal growth factor and tyrosine kinase inhibitors[1,3].

Data from the recent presentation are from a 24-year-old RDEB patient with a history of multiple, unresectable SCCs that were unresponsive to prior treatments including cemiplimab. Results showed that intravenously administered rigosertib had an acceptable safety profile and that the patient experienced sustained clinical and histological remission of all target lesions without signs of metastatic disease following 13 treatment cycles. The patient remains on study and the trial remains ongoing. The enrollment of additional patients is anticipated at sites in Salzburg, Austria; London, UK; and Philadelphia, Pennsylvania.

"Though the trial's currently available data are from only a single patient, they represent an exciting and powerful finding that warrants further study," said Andrew South Ph.D., Associate Professor, Department of Dermatology & Cutaneous Biology, Thomas Jefferson University. "RDEB-associated SCC is an indication with an extremely high unmet medical need, as it is invariably fatal with treatment options that have so far yielded disappointing results. The data generated in preclinical models suggesting rigosertib's robust activity against PLK1 have now been confirmed in the clinic and suggest that rigosertib may play a role in other more common cancers driven by PLK1."

The physicians caring for the patient, Dr. Bauer, Principal Investigator, and Dr. Laimer, Sponsor Medical Expert of the trial, added in a joint statement, "To see a complete response in a patient that has failed multiple prior therapies is highly encouraging and, together with preclinical data, suggests that rigosertib's ability to inhibit PLK1 may position it as a novel treatment option that can significantly improve upon the current standard-of-care. We look forward to the further evaluation of this hypothesis through the continued advancement of the trial."

A copy of the poster, titled "Rigosertib for locally advanced/metastatic EB-associated SCC," is available on the " Scientific Presentations" section of the Onconova website.

References

1. Mellerio et al. Br J Dermatol. 2016 Jan; 174(1):56-67. doi: 10.1111/bjd.14104.
2. Fine et al. J Am Acad Dermatol. 2009 Feb; 60(2):203-11. doi: 10.1016/j.jaad.2008.09.035.
3. Stratigos et al. Eur J Cancer. 2020 Mar;128:83-102. doi: 10.1016/j.ejca.2020.01.008.

About Onconova Therapeutics
Onconova Therapeutics is a clinical-stage biopharmaceutical company focused on discovering and developing novel products for patients with cancer. The Company has proprietary targeted anti-cancer agents designed to disrupt specific cellular pathways that are important for cancer cell proliferation.

Onconova's novel, proprietary multi-kinase inhibitor narazaciclib (formerly ON 123300) is being evaluated in two separate and complementary Phase 1 dose-escalation and expansion studies. These trials are currently underway in the United States and China.

Onconova's product candidate rigosertib is being studied in an investigator-initiated study program, including in a dose-escalation and expansion Phase 1/2a investigator-initiated study with oral rigosertib in combination with nivolumab for patients with KRAS+ non-small cell lung cancer.

For more information, please visit 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, and involve risks and

uncertainties. These statements relate to Onconova's expectations regarding the timing of Onconova's and investigator-initiated clinical development and data presentation plans, and the mechanisms and indications for Onconova's product candidates. Onconova has attempted to identify forward-looking statements by terminology including "believes," "estimates," "anticipates," "expects," "plans," "intends," "may," "could," "might," "will," "should," "preliminary," "encouraging," "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 the success and timing of Onconova's clinical trials, investigator-initiated trials and regulatory agency and institutional review board approvals of protocols, Onconova's collaborations, market conditions 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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