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Cascadian Therapeutics Announces FDA Orphan Drug Designation Granted to Tucatinib for the Treatment of HER2+ Metastatic Colorectal Cancer

SEATTLE, Sept. 27, 2017 (GLOBE NEWSWIRE) -- Cascadian Therapeutics, Inc. (NASDAQ:CASC), a clinical-stage biopharmaceutical company, today announced that the U.S. Food and Drug Administration ("FDA") has granted orphan drug designation to tucatinib for the treatment of HER2-positive (HER2+) metastatic colorectal cancer. Tucatinib is an investigational oral, small molecule kinase inhibitor that is highly selective for HER2 and is the Company's lead product in development.

The FDA's Orphan Drug Designation program provides orphan status to drugs defined as those intended for the safe and effective treatment, diagnosis or prevention of rare diseases that affect fewer than 200,000 people in the United States. Orphan designation qualifies the sponsor of the drug for certain development incentives, including tax credits for qualified clinical testing, prescription drug user fee exemption and 7-year marketing exclusivity upon FDA approval.

"We are pleased to receive FDA orphan drug designation for tucatinib in HER2+ metastatic colorectal cancer, our second orphan designation in addition to breast cancer with brain metastases," said Scott Myers, President and CEO of Cascadian Therapeutics. "We believe tucatinib has the potential to address unmet medical needs of patients in a broad range of HER2-positive cancers. We are currently supporting an investigator-initiated study evaluating tucatinib for the treatment of patients with HER2+ metastatic colorectal cancer."

"We are conducting a Phase 2 study called MOUNTAINEER evaluating tucatinib in combination with trastuzumab for patients with HER2 amplified metastatic colorectal cancer," said John Strickler, MD, Assistant Professor of Medicine, Division of Medical Oncology, Duke University Medical Center, and principal investigator of the MOUNTAINEER study. "There remains an unmet clinical need for new treatments for patients with HER2 amplified metastatic colorectal cancer. Tucatinib is a potent and selective inhibitor of HER2 *in vitro*, and is active in non-clinical HER2+ colorectal tumor models. Based on these data, we believe tucatinib has the potential to be a new therapeutic option for these patients."

The study, known as MOUNTAINEER, is currently recruiting participants. Additional details about MOUNTAINEER can be found at www.clinicaltrials.gov (NCT03043313).

The American Cancer Society estimates 135,430 new cases of colorectal cancer will be diagnosed in the U.S. in 2017, and expects colorectal cancer to cause over 50,000 deaths in 2017. Colorectal cancer is the third leading cause of cancer death in both men and women in the U.S. While the prevalence of HER2 amplified colorectal cancer varies depending on study methods and population, approximately 3-to-8 percent of these patients would be expected to potentially benefit from HER2-targeted therapy.[1]

About Tucatinib

Tucatinib is an investigational, orally bioavailable, potent tyrosine kinase inhibitor that is highly selective for HER2 without inhibition of EGFR. Inhibition of EGFR has been associated with clinical toxicities, including skin rash and diarrhea. Tucatinib has shown activity as a single agent and in combination with both chemotherapy and other HER2 directed agents such as trastuzumab.[2,3] Studies of tucatinib in these combinations have shown activity both systemically and in brain metastases. HER2 is a growth factor receptor that is overexpressed in multiple cancers, including breast, ovarian and gastric cancers. HER2 mediates cell growth, differentiation and survival. Tumors that overexpress HER2 (HER2+) are more aggressive and historically have been associated with poor overall survival, compared with HER2-negative cancers.

About Cascadian Therapeutics

Cascadian Therapeutics is a clinical-stage biopharmaceutical company dedicated to developing innovative product candidates for the treatment of cancer. Its lead product candidate, tucatinib, is an investigational oral, selective small molecule HER2 inhibitor. Cascadian Therapeutics is conducting a randomized, double-blind, controlled pivotal clinical trial called HER2CLIMB, which is comparing tucatinib vs. placebo, each in combination with capecitabine and trastuzumab, in patients with locally advanced or metastatic HER2+ breast cancer with and without brain metastases, who have previously

been treated with trastuzumab, pertuzumab and T-DM1. Additional details on HER2CLIMB can be found at www.HER2CLIMB.com or www.clinicaltrials.gov. For more information, please visit www.cascadianrx.com.

Forward-Looking Statements

In order to provide Cascadian Therapeutics' investors with an understanding of its current results and future prospects, this release contains statements that are forward-looking. Any statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "believes," "anticipates," "plans," "expects," "will," "intends," "potential," "possible" and similar expressions are intended to identify forward-looking statements, but the absence of these words does not necessarily mean that a statement is not forward-looking. Forward-looking statements include, without limitation, statements regarding clinical development activities, timing of additional data, the potential benefits of its product candidates, timing of regulatory filings, and potential regulatory approvals of its product candidates. Any statements that refer to expectations, projections or other characterizations of future events or circumstances are forward-looking statements. Forward-looking statements involve risks and uncertainties related to Cascadian Therapeutics' business and the general economic environment, many of which are beyond its control. These risks, uncertainties and other factors could cause Cascadian Therapeutics' actual results and timing of events to differ materially from those anticipated in forward-looking statements, including, without limitation, the risks associated with the costs and expenses of developing its product candidates, the adequacy of financing and cash, cash equivalents and investments, changes in general accounting policies, general economic factors, achievement of the results it anticipates from its preclinical development and clinical trials of its product candidates, the receipt of regulatory approvals, its ability to adequately obtain and protect its intellectual property rights, and other factors discussed under the caption "Risk Factors" in Cascadian Therapeutics' Quarterly Report on Form 10-Q for the quarterly period ended June 30, 2017 filed with the Securities and Exchange Commission. Although Cascadian Therapeutics believes that the forward-looking statements contained herein are reasonable as of the date hereof, it can give no assurance that its expectations are correct. All forward-looking statements are expressly qualified in their entirety by this cautionary statement. For a detailed description of Cascadian Therapeutics' risks and uncertainties, you should review the documents filed by Cascadian Therapeutics with the securities regulators in the United States on EDGAR and in Canada on SEDAR. Cascadian Therapeutics disclaims any obligation to publicly update or revise its forward-looking statements based on events or circumstances after the date hereof, except to the extent required by law.

1 Takegawa N and Yonesaka K. HER2 emerging oncotarget for colorectal cancer treatment after failure of anti-epidermal growth factor receptor therapy. Clin Colorectal Cancer 2017 Mar 9. Pii:S1533-0028(16)30240-7.
2 Moulder, S. et al., Phase 1 Study of ONT-380, a HER2 Inhibitor, in Patients with HER2+ Advanced Solid Tumors, with an Expansion Cohort in HER2+ Metastatic Breast Cancer. Clin Cancer Res. May 2017.
3 Hamilton, E. et al., Efficacy of a Phase 1b Study of Tucatinib (ONT-380), an Oral HER2-Specific Inhibitor, in Combination with Capecitabine and Trastuzumab in HER2+ Metastatic Breast Cancer, Including Patients with Brain Metastases. Presented at the San Antonio Breast Cancer Symposium (SABCS) Annual Meeting 2016, San Antonio, TX. December 9, 2016 (Poster P4-21-01).

Source: Cascadian Therapeutics, Inc.

Investor and Media Contact:
Monique Greer
Cascadian Therapeutics
206-801-2107
mgreer@cascadianrx.com