



Clementia Granted Rare Pediatric Disease Designation by FDA for Palovarotene for Fibrodysplasia Ossificans Progressiva

February 11, 2019

MONTREAL, Feb. 11, 2019 (GLOBE NEWSWIRE) -- Clementia Pharmaceuticals Inc. (Nasdaq: CMTA), a clinical-stage biopharmaceutical company innovating treatments for people with ultra-rare bone disorders and other diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease designation to palovarotene for the treatment of fibrodysplasia ossificans progressiva (FOP). Palovarotene, an investigational therapy for FOP, has previously been granted Orphan Drug, Fast Track and Breakthrough designations by the FDA for FOP.

“FOP is a rare, devastating disease that begins to irreversibly impact individuals in childhood,” said Clarissa Desjardins, Ph.D., founder and chief executive officer of Clementia. “The receipt of this designation highlights the urgent need for a treatment for people with FOP. The entire Clementia team is working towards an NDA submission in the second half of 2019 in hopes of achieving our first regulatory approval, an important step toward achieving our mission of bringing innovative and effective treatments to individuals who currently have none.”

In October 2018, based on a meeting with the FDA, Clementia announced that the Agency was supportive of the company’s plan to submit a New Drug Application (NDA) for palovarotene for the prevention of heterotopic ossification (HO) associated with flare up symptoms in patients with FOP. The FDA based its assessment on the efficacy and safety data generated in the completed Phase 2 clinical program. The company’s NDA preparations are underway, with a submission targeted for the second half of 2019. If approved, Clementia expects a first commercial launch for palovarotene in 2020.

The Rare Pediatric Disease Designation qualifies investigational palovarotene for a Rare Pediatric Disease Priority Review Voucher (PRV), granted at the time of NDA approval. The PRV Program is intended to encourage development of therapies to prevent and treat rare pediatric diseases. This PRV can be redeemed to receive a priority review of a subsequent marketing application or sold to a third party.

About Palovarotene

Palovarotene is an RARy agonist being developed as a potential treatment for patients with ultra-rare and debilitating bone diseases, including fibrodysplasia ossificans progressiva (FOP) and multiple osteochondromas (MO), as well as other diseases. Palovarotene was in-licensed from Roche Pharmaceuticals, where it was previously evaluated in more than 800 subjects, including 450 patients treated for up to two years. Palovarotene has received Orphan Drug status for FOP and MO from the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA). In addition, palovarotene has been granted Fast Track, Breakthrough Therapy and Pediatric Rare Disease designations for FOP from the FDA.

About Fibrodysplasia Ossificans Progressiva (FOP)

FOP is a rare, severely disabling disorder characterized by heterotopic ossification (HO), or bone that forms outside the normal skeleton, in muscles, tendons or soft tissue. In FOP, HO progressively restricts

movement by locking joints, leading to a cumulative loss of function, progressive disability, and increased risk of early death. FOP is caused by a mutation in the ACVR1 gene, resulting in excess signaling in the bone morphogenetic pathway, a key pathway controlling bone growth and development, by way of both ligand-dependent and independent mechanisms. There are currently no approved treatments for FOP.

About Clementia Pharmaceuticals Inc.

Clementia is a clinical-stage company innovating treatments for people with ultra-rare bone disorders and other diseases with high medical need. The company is preparing to submit an NDA in the second half of 2019 to seek approval of its lead product candidate, palovarotene, a novel RAR γ agonist, for fibrodysplasia ossificans progressiva (FOP). The ongoing Phase 3 MOVE Trial is evaluating an additional dosing regimen of investigational palovarotene for FOP. Palovarotene is also in a Phase 2 trial, the MO-Ped Trial, for the potential treatment of multiple osteochondromas (MO, also known as multiple hereditary exostoses, or MHE). In addition, Clementia has commenced a Phase 1 trial for an eye drop formulation of palovarotene for the potential treatment of dry eye disease and is also investigating other conditions that may benefit from RAR γ therapy. For more information, please visit www.clementiapharma.com and connect with us on Twitter @ClementiaPharma.

Cautionary Note Regarding Forward-Looking Statements

This press release may include “forward-looking statements” within the meaning of the applicable securities laws, including with respect to the proposed timing of submission of the NDA for palovarotene. Each forward-looking statement contained in this press release is subject to known and unknown risks and uncertainties and other unknown factors that could cause actual results to differ materially from historical results and those expressed or implied by such statement. In addition to statements which explicitly describe such risks and uncertainties, readers are urged to consider statements labeled with the terms “believes,” “belief,” “expects,” “intends,” “anticipates,” “will,” or “plans” to be uncertain and forward-looking. Applicable risks and uncertainties include, among others, the Company’s ability to successfully complete in a timely manner the studies required to be completed in order to submit the NDA, the Company’s ability to generate revenue and become profitable; the risks related to its heavy reliance on palovarotene, its only current product candidate; the risks associated with the development of palovarotene and any future product candidate, including the demonstration of efficacy and safety; its dependence on licensed intellectual property, including the ability to source and maintain licenses from third-party owners; as well as the risks identified under the heading “Risk Factors” in the Company’s Annual Report on Form 20-F filed with the Securities and Exchange Commission (“SEC”), as well as the other information its file with the SEC or on SEDAR. Clementia cautions investors not to rely on the forward-looking statements contained in this press release when making an investment decision in its securities. Investors are encouraged to read the Company’s filings with the SEC or on SEDAR, available at www.sec.gov or www.sedar.com, for a discussion of these and other risks and uncertainties. The forward-looking statements in this press release speak only as of the date of this press release, and the Company undertakes no obligation to update or revise any of these statements, whether as a result of new information, future events or otherwise, except as required by law.

Investor/Media Contact:

Joseph Walewicz
Clementia Pharmaceuticals Inc.
+1-514-940-1080

Alicia Davis
THRUST Strategic Communications
+1-910-620-3302
alicia@thrustsc.com

 **Clementia**
Learning

Source: Clementia Pharmaceuticals Inc.