



Clementia Initiates Pivotal Phase 3 MOVE Trial for Palovarotene in Patients with Fibrodysplasia Ossificans Progressiva

December 12, 2017

Patient Dosing Has Commenced; Registration Study to Enroll 80 Patients Worldwide

Company to Host Conference Call at 8:30am ET Today

MONTREAL, Dec. 12, 2017 (GLOBE NEWSWIRE) -- [Clementia Pharmaceuticals Inc.](#) (NASDAQ:CMTA), a clinical-stage biopharmaceutical company innovating new treatments for people with ultra-rare bone disorders and other diseases, today announced the start of the MOVE Trial, its Phase 3 registration study evaluating the safety and efficacy of palovarotene for the treatment of patients with fibrodysplasia ossificans progressiva (FOP). Palovarotene, Clementia's lead product candidate, is a retinoic acid receptor gamma agonist (RAR γ) being investigated as a treatment for patients with ultra-rare and debilitating bone diseases, including FOP and multiple osteochondromas (MO), as well as other diseases.

"FOP is a rare, devastating, and debilitating disease for patients. It causes great hardship to families and caregivers. We are very excited to be part of this first-ever multicenter Phase 3 clinical trial for patients with FOP, for which there are no approved treatments," said Edward Hsiao, M.D., Ph.D., associate professor in endocrinology and the Institute for Human Genetics at the University of California San Francisco (UCSF) School of Medicine, and one of the principal investigators of the MOVE Trial. "Palovarotene has shown promise in its Phase 2 program. Our team looks forward to working with patients and our fellow investigators from around the world to complete this important study."

UCSF is the first site to enroll a patient in the MOVE Trial, which will be conducted at approximately 20 centers in 16 countries around the world (Argentina, Australia, Brazil, Canada, France, Germany, Italy, Japan, Netherlands, Russia, South Africa, South Korea, Spain, Sweden, United Kingdom and United States). Additional sites are expected to begin enrolling patients in the coming months, with completion of enrollment expected by the end of 2018.

"The initiation of this study is a landmark event for Clementia, fully transitioning us into a late-stage clinical organization," commented Donna Grogan, M.D., chief medical officer of Clementia. "To date we have treated over fifty FOP patients in our Phase 2 clinical program, and we are encouraged by palovarotene's safety profile and the efficacy data showing meaningful reductions in new heterotopic ossification (HO) volume following a flare-up." HO is a hallmark sign of FOP, in which bone grows where it should not, outside the skeleton and in abnormal places, such as muscles, tendons and soft tissue in patients with FOP.

"Palovarotene is rapidly advancing through clinical development and could be the first treatment to be approved for patients with FOP," said Clarissa Desjardins, Ph.D., chief executive officer of Clementia. "Our team is motivated by the medical need for patients with FOP and wishes to thank the patients, their families and caregivers, and the investigators around the world who have been working hand-in-hand with us over the past three years to advance palovarotene to this important milestone."

About the Phase 3 MOVE Trial

All patients in the MOVE Trial receive a single daily dose of palovarotene, with increased dosing at the time of a flare-up. Data from the FOP patients in Clementia's Natural History Study (NHS) will serve as the control. Patients

will be treated with palovarotene for 24 months, with three planned interim analyses. The first interim analysis will occur when the first 35 patients have completed their one-year CT scans, with the next two interim analyses coming 6 and 12 months afterwards. Clementia expects top-line results from the study in late 2020, with the first interim readout expected in early 2019.

The primary efficacy endpoint of the MOVE Trial is the annualized change in new HO volume as assessed by low-dose WBCT scan (excluding head) compared to untreated patients from the NHS. Secondary endpoints include the proportion of patients with any new HO, the change in the number of body regions with new HO, the proportion of patients reporting flare-ups and the rate of flare-up occurrence. Exploratory endpoints include functional assessments of joint function and changes in patient-reported physical function. Safety evaluations include adverse events, assessments of growth in children, clinical laboratory tests and vital signs. Full details of the study can be found at www.clinicaltrials.gov, NCT03312634.

About Palovarotene

Palovarotene is a retinoic acid receptor gamma agonist (RAR γ) being investigated as a treatment for patients with ultra-rare and debilitating bone diseases, including fibrodysplasia ossificans progressiva (FOP) and multiple osteochondromas (MO), and other diseases with high medical need. Preliminary Phase 2 data in patients with FOP mirror the decrease in heterotopic ossification (HO) volume observed in mouse models of FOP and support the initiation of the confirmatory Phase 3 MOVE Trial. Palovarotene also inhibits the formation of osteochondromas (OCs) in mouse models of multiple exostoses, supporting development in the MO indication. Palovarotene has received Orphan Drug designation for FOP and MO from the U.S. Food and Drug Administration (FDA). In addition, palovarotene has been granted Fast Track and Breakthrough Therapy designations for FOP from the FDA, and was granted orphan status for the treatment of FOP in the EU.

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.

Conference Call Information

To participate in the conference call, please dial (866) 916-2014 (domestic) or (636) 812-6655 (international) and refer to conference ID 5689444. The webcast can be accessed in the Investor Relations section of the company's website at www.clementiapharma.com. The replay of the webcast will be available in the investor section of the company's website at www.clementiapharma.com for 60 days following the call.

About Clementia Pharmaceuticals Inc.

Clementia is a clinical-stage biopharmaceutical company innovating new treatments for people with ultra-rare bone disorders and other diseases with high medical need. The company's lead candidate palovarotene, a novel RAR γ agonist, is currently being evaluated in the Phase 3 MOVE Trial to treat fibrodysplasia ossificans progressiva (FOP), with additional clinical studies planned in multiple osteochondromas (MO, also known as hereditary multiple exostoses) and other diseases. For more information, please visit www.clementiapharma.com.

UC Disclaimer

The information stated above was prepared by Clementia Pharmaceuticals Inc. to report on research conducted on its product and reflects solely the opinion of Clementia. Nothing in this statement shall be construed to imply any support or endorsement of Clementia, or any of its products, by The Regents of the University of California, its officers, agents and employees.

Cautionary Note Regarding Forward-Looking Statements

This press release may include “forward-looking statements” within the meaning of the applicable securities laws. 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, our ability to generate revenue and become profitable; the risks related to our heavy reliance on palovarotene, our only current product candidate; the risks associated with the development of palovarotene and any future product candidate, including the demonstration of efficacy and safety; our heavy dependence on licensed intellectual property, including our ability to source and maintain licenses from third-party owners; as well as the risks identified under the heading “Risk Factors” in our Prospectus on Form 424(b) filed with the Securities and Exchange Commission (“SEC”), as well as the other information we file with the SEC or on SEDAR. We caution investors not to rely on the forward-looking statements contained in this press release when making an investment decision in our securities. You are encouraged to read our 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 we undertake 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

Chelcie Lister
THRUST Investor Relations
+1-910-777-3049

[Primary Logo](#)

Source: Clementia Pharmaceuticals Inc.