Clementia Announces Updated Phase 2 Part B Data on Palovarotene for FOP

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Updated 12-Week Flare-Up Data Presented at Mechanistic and Therapeutic Advances in Rare Skeletal Diseases and ASBMR 2018

Company Announces Additional Updated 12-Month WBCT Scan Data

Data Continue to Support Treatment Potential of Palovarotene for People with FOP

MONTREAL, Sept. 26, 2018 (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 updated data from the open label extension ("Part B") of its ongoing Phase 2 clinical trial of palovarotene in fibrodysplasia ossificans progressiva (FOP).

"These updated data continue to support the potential for palovarotene in FOP, an ultra-rare and devastating bone condition," said Clarissa Desjardins, Ph.D., founder and chief executive officer of Clementia. "Twelve-week flare-up outcomes for patients treated with palovarotene in our Phase 2 trial indicate a consistent treatment effect of approximately 73 percent reduction in mean new bone growth, or heterotopic ossification, and palovarotene was generally tolerated in both adult and pediatric patients. However, we observed that nearly half of the patients in Part B had flare-ups that went untreated mainly due to the criteria for flare-up treatment specified in the Part B protocol. The flare-up treatment criteria in the MOVE Trial, on the other hand, are expected to result in more frequent use of flare-up up dosing in response to flare-up symptoms. First and second interim analyses from the MOVE Trial are expected to occur in the second and third quarters of 2019, respectively, and are designed to assess new HO in treated patients as compared to external natural history study controls. We look forward to those data readouts next year and anticipate that they'll reflect the true potential of palovarotene in patients who urgently need a treatment option."

Updated 12-Week Flare-up Imaging Results

The final set of 12-week flare-up imaging data were presented today in a poster session at the Mechanistic and Therapeutic Advances in Rare Skeletal Diseases, a meeting organized by the Rare Bone Disease Alliance in association with American Society of Bone and Mineral Research (ASBMR), and will also be presented on Monday, Oct. 1, 2018 in a poster session at the ASBMR 2018 Annual Meeting (Presentation Number: MON-1066). The data demonstrate a consistent greater than 70 percent reduction in new heterotopic ossification (HO, or bone growth in abnormal places) across three different dosing regimens, as detailed below:

Treatment group	N=	Volume of new HO (mm^3) at 12 weeks	% reduction	p=value[1]
Placebo/untreated	46	11,014	-	-
Palovarotene 10/5mg	46	2,731	-75%	p=0.05
Palovarotene 20/10mg	15	3,045	-72%	p=0.02
Palovarotene chronic / flare-up	31	3,018	-73%	p=0.16

[1]ANOVA with BCa bootstrap and covariate adjustment

The poster also presents details on 12-week flare-up outcomes at the hips and shoulders, the two most frequent flare-up locations, and flare-ups at these locations result in substantially more bone than flare-ups at other locations. The treatment effect for flare-ups that had any new HO at these locations at 12-weeks varies between 60 and 97 percent reduction in new HO, depending on the treatment regimen.

As previously reported, palovarotene is generally tolerated in our ongoing Phase 2 study. There were dose-related increases in retinoid-associated adverse events (AEs) with most being mild or moderate in severity, and only one patient discontinued participation in the study because of an AE. There were no treatment-related effects on laboratory or ECG findings or on skeletal growth in pediatric patients.

Updated 12-month Whole-body CT Scans (WBCT)
In addition to the data presented in the poster, the company announced updated WBCT data that demonstrate new HO at 12 months in the palovarotene treated per protocol group of 23,335 mm^3, a reduction of 3,639 mm^3, or 13 percent (n=33, p=ns), versus the 26,974 mm^3 new HO volume observed in untreated NHS patients (n=58). Updated review of the data indicates that 47 percent of patients (n=15) had flare-up symptoms that were either untreated or under-treated. Due to the narrow definition of a flare-up in Part B, which required at least two flare-up symptoms and did not allow for flare-up dosing in connection with intercurrent flare-ups, these patients did not receive the 20/10mg regimen for their flare-up symptoms. These patients exhibited a mean new HO of 39,443mm^3. In those patients who did receive the 20mg/10mg regimen (n=9) for all reported flare-ups, the mean volume of new HO was 1,787mm^3, a 93 percent decrease versus untreated patients.

Clementia believes that the flare-up treatment definition in the ongoing MOVE Trial is expected to result in more frequent use of flare-up dosing in response to flare-up symptoms. Preliminary treatment rates (treated flare-ups per subject month exposure) from the MOVE Trial support this assessment, as the treatment rate in the MOVE Trial is much higher than that observed in our Part B study. As a result of this, the company does not believe that the Part B WBCT data are a reliable predictor of potential outcomes in the MOVE Trial.

About Palovarotene
Palovarotene is an RARγ agonist being developed as a 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 individuals, 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 orphan status for the treatment of FOP in the EU. In addition, palovarotene has been granted Fast Track and Breakthrough Therapy designations for FOP from the FDA.

About Clementia Pharmaceuticals Inc.
Clementia is a clinical-stage company innovating new treatments for people with ultra-rare bone disorders and other diseases with high medical need. The company's lead product candidate, palovarotene, a novel RARγ agonist, is currently being evaluated in the Phase 3 MOVE Trial to treat fibrodysplasia ossificans progressiva (FOP) and in the Phase 2 MO-Ped Trial to treat multiple osteochondromas (MO, also known as multiple hereditary exostoses/MHE). Clementia is also investigating palovarotene for the potential treatment of other conditions that may benefit from RARγ therapy. For more information, please visit www.clementiapharma.com and connect with us on Twitter @ClementiaPharma.

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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, 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.

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