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Dear FOP community,

I am very pleased to share with you the top-line results of our Phase 2 clinical trial. Overall, our study has exhibited positive trends in bone reduction and symptom reduction using palovarotene though these observations did not reach statistical significance. In the 40 subject placebo-controlled Phase 2 dose-ranging trial:

- No subject required a reduction in dose due to tolerability issues nor was discontinued from the trial.
- Those on placebo were at 2.6 times greater risk of forming heterotopic ossification (HO) than those on the palovarotene 10/5 mg treatment. In addition, those on either palovarotene regimen with new HO formed less HO than those on placebo.
- We observed palovarotene-related reductions in patient-reported pain associated with flare-ups and reductions in the time to resolution of flare-ups.

Together, we have achieved the first and likely hardest goal of our development program, which is to determine how we can measure palovarotene's effect and how we could potentially demonstrate efficacy of palovarotene in a Phase 3 clinical trial. We are extremely eager to move on to this next step in our clinical development program to hopefully achieve our goal of providing a therapy for those living with FOP.

The Phase 3 clinical trial is scheduled to begin in 2017 and will recruit patients from all over the world. We are currently planning and designing the features of this Phase 3 clinical trial as well as a surgical excision trial and will meet with regulatory authorities to ensure that the design of these trials meets with their expectations.

In most jurisdictions around the world, a sponsor, such as Clementia, is required to perform adequate and well-controlled studies that demonstrate a statistically significant impact of the drug on how a patient "feels, functions or survives." This is a high bar to achieve even when positive trends are detected such as in our initial Phase 2 clinical trial. However, we are determined to meet this challenge.

All of us here at Clementia continue to be inspired everyday by your stories, your collaboration, and your grit. We thank all of you who participated in this trial and our other clinical trials. You have provided answers and hope for the entire FOP community for many years to come. You are our heroes. We also want to thank all the dedicated clinical teams around the world who worked tirelessly to generate these results.

Let me leave you with these words from an unknown author, "Whatever you do, hold on to hope. The tiniest thread will twist into an unbreakable cord. Let hope anchor you in the possibility that this is not the end of your story, that change will bring you to peaceful shores."

Warm regards,

Clarissa

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Clementia Announces Top-line Results from Phase 2 Trial of Palovarotene for Treatment of Patients with Fibrodysplasia Ossificans Progressiva

MONTREAL, CANADA, October, 14, 2016 – Clementia Pharmaceuticals Inc. today announced top-line results from its Phase 2 clinical trial investigating palovarotene for the treatment of fibrodysplasia ossificans progressiva (FOP). FOP is an extremely rare, serious disease in which an accumulation of heterotopic ossification (HO, extraskeletal bone) in muscle and soft tissue progressively restricts movement by locking joints leading to loss of function, physical disability, and risk of early death. Several positive trends were detected in this 40-subject placebo-controlled trial, including palovarotene-related reductions in the proportion of subjects who developed new HO, reductions in volume of new HO, reductions in patient-reported pain associated with flare-ups, and reductions in the time to resolution of FOP-related flare-ups though none reached statistical significance. Palovarotene was well-tolerated, with all subjects completing the 12-week trial and enrolling into the open-label extension trial.

“The results of this landmark clinical trial are encouraging and closely mirror what was observed in previously reported animal studies with palovarotene,” said principal investigator, Frederick Kaplan, MD, the Isaac & Rose Nassau Professor of Orthopaedic Molecular Medicine and Chief of the Division of Molecular Orthopaedic Medicine in the Perelman School of Medicine at the University of Pennsylvania. “This study has considerably enhanced our knowledge of FOP and is a significant step forward for the entire FOP community.”

The 12-week Phase 2 trial randomized subjects to three dose groups: 10 mg palovarotene for 2 weeks followed by 5 mg for 4 weeks (10/5), 5 mg for 2 weeks followed by 2.5 mg for 4 weeks (5/2.5), or placebo. Treatment was initiated within 7 days of the onset of a flare-up with evaluations made at baseline, at the end of treatment (6 weeks), and after a 6-week observation period (12 weeks). Subjects on placebo were at 2.6 times greater risk of forming HO than those on palovarotene 10/5 mg treatment, while those on either palovarotene regimen with new HO formed less HO than those on placebo. Subjects on the 10/5 regimen reported a greater improvement in pain associated with flare-ups and a reduction in the duration of overall flare-up symptoms. Though a dose-related increase in the incidence of mucocutaneous adverse events was observed, no subject required a reduction in dose due to tolerability issues nor was discontinued from the trial.

Full results of the Phase 2 trial are expected to be published next year. Clementia continues to gather important additional data in the Phase 2 extension trial and in the ongoing observational Natural History Study. Data from these studies will inform the design of a Phase 3 registration trial, which is expected to start in 2017. “That patients were able to tolerate palovarotene with no discontinuations for safety reasons at a 10-mg dose encouraged us to implement a higher dose in the Phase 2 open-label

extension and to extend the number of days dosed," said Donna Grogan, MD, CMO of Clementia. The extension trial has also introduced a chronic daily dose based on new research conducted by scientists at Penn and The Children's Hospital of Philadelphia (CHOP) and findings from the completed Phase 2 trial.

Many years of laboratory research paved the way for this clinical trial. A leading investigator, Maurizio Pacifici, PhD, director of Orthopedic Research at CHOP, and his collaborators first showed that palovarotene produces powerful biological effects in transgenic mouse models of human FOP and inhibits HO markedly. "Those results have provided the basis and rationale for testing palovarotene to prevent HO in FOP patients in this trial," said Pacifici.

"We would like to thank the patients, their families, the investigators, and their research teams," said CEO of Clementia, Clarissa Desjardins. "Developing a potential treatment for FOP is our passion and our goal, and we will continue to press forward as rapidly and rigorously as possible to deliver a much needed potential therapy for all FOP patients."

Additional information about palovarotene and Clementia's clinical program can be found at clementiapharma.com.

Editor's Note: Kaplan declares no disclosures and is the Global Principal Investigator for Clementia's Phase 2 Study.

About Fibrodysplasia Ossificans Progressiva (FOP)

FOP is a rare, severely disabling congenital myopathy characterized by heterotopic ossification (HO) of muscle and soft tissues. Heterotopic ossification is bone that forms outside the normal skeleton and, in FOP, progressively restricts movement by locking joints leading to a cumulative loss of function, disability, and risk of early death. Virtually all newborns with FOP have a hallmark toe malformation in which both big toes are shortened and bent inwards. FOP is caused by a mutation in the ACVR1 gene resulting in increased activity of BMP Type I receptor or ALK2 receptor involved in the bone morphogenetic (BMP) pathway, a key pathway controlling bone growth and development. There are currently no approved treatments for FOP.

About Palovarotene

Palovarotene is a retinoic acid receptor gamma agonist (RARγ) being investigated as a treatment for FOP. Preclinical studies in mouse models of FOP demonstrated that palovarotene blocked both injury-induced and spontaneous heterotopic ossification, maintained mobility, and restored skeletal growth. Palovarotene received Fast Track designation from the U.S. Food and Drug Administration (FDA) and orphan designations for the treatment of FOP from both the FDA and the European Medicines Agency (EMA).

About Clementia Pharmaceuticals Inc.

Clementia is a clinical stage biopharmaceutical company committed to delivering treatments to people who have none. The company is developing its lead candidate palovarotene, a novel RARγ agonist, to treat fibrodysplasia ossificans progressiva (FOP) and other diseases. For more information, please visit www.clementiapharma.com.

http://clementiapharma.com/clinical-trials/.

Clementia reports the top-line results of its Phase 2 clinical trial for palovarotene in fibrodysplasia ossificans progressiva.

We would like to thank the FOP patient and medical communities without whom our Phase 2 program could not have succeeded. The investigators, their clinical research teams, and the subjects, families and caregivers provided leadership, support, commitment, and participation throughout the program. We enrolled 40 subjects in the Phase 2 trial and approximately 100 in the natural history study, and both trials have produced learnings that serve to advance the goals of the FOP community to understand the disease and to develop treatments.

1. What was the purpose of Clementia's Phase 2 trial?

   Answer. The purpose of the FOP clinical program is to develop evidence that supports marketing authorization (MA) for palovarotene as a treatment for FOP patients. The objective of our Phase 2 was to inform the design of a Phase 3 registrational trial by investigating whether palovarotene prevents heterotopic ossification (HO) following a flare-up, at what dose(s), and with what side effects.

2. What observations did you make from your Phase 2 trial?

   Answer. We observed several positive trends though none reached statistical significance in this study.

   - We observed that in the 40 subject placebo-controlled Phase 2 dose-ranging trial, no subject required a reduction in dose due to tolerability issues nor was discontinued from the trial.
   - We observed that those on placebo were at 2.6 times greater risk of forming HO than those on the palovarotene 10/5 mg treatment. In addition, those on either palovarotene regimen with new HO formed less HO than those on placebo.
   - We observed palovarotene-related reductions in patient-reported pain associated with flare-ups and reductions in the time to resolution of flare-ups.

3. What are the next steps for your FOP clinical program?

   Answer.

   - We are designing and plan to implement a Phase 3 registrational trial and a surgical excision trial as soon as possible in 2017.
   - We will continue to press forward with the collaboration of all stakeholders, including patients, physicians, and regulatory authorities, on our goal to deliver a much needed potential therapy for all FOP patients.