Clementia Pharmaceuticals Opens Clinical Trial Site in Europe for Phase 2 Study of Palovarotene in Patients with Fibrodysplasia Ossificans Progressiva (FOP)

MONTREAL, CANADA, January 12, 2015 – Clementia Pharmaceuticals, Inc. today announced the opening of a new site in Paris, France for the Phase 2 clinical trial of palovarotene, the company’s lead product candidate for the treatment of fibrodysplasia ossificans progressiva (FOP). FOP is a rare, severely disabling genetic disease characterized by painful, recurrent episodes of soft tissue swelling (flare-ups) and new abnormal bone formation. This process, known as heterotopic ossification (HO), occurs in muscles, tendons and ligaments, causing significant morbidities and progressive disability.

The Hôpital Necker-Enfants Malades in Paris is the third clinical site opened for the Phase 2 trial, the other two sites being University of California San Francisco (UCSF) and the University of Pennsylvania in Philadelphia. Dr. Genevieve Baujat of Paris’ Centre of Reference for Skeletal Dysplasia, Imagine Institut, and Necker Hôpital is the principal investigator for the Paris-based site.

“We are pleased to conduct this important study evaluating palovarotene as a potential treatment for patients living with FOP,” said Dr. Baujat. “There are currently no approved treatments for those living with this severely disabling disease that often leads to fatal complications.”

In preclinical studies, palovarotene prevented heterotopic bone formation in mouse models of FOP. The ongoing Phase 2 clinical trial was designed to determine whether these effects can be replicated in patients with FOP.

“Six patients have been enrolled at the two U.S. sites,” said Jeff Packman, Chief Development Officer of Clementia. “The clinical trial site in Paris expands the reach of our Phase 2 clinical trial of palovarotene, making it easier for eligible European patients with this ultra-rare disease to participate and reducing the travel burden for patients.”

The double-blind, placebo-controlled Phase 2 clinical trial is evaluating the effect of different doses of palovarotene on new bone formation during and after a flare-up in 24 patients age 15 years or older. Treatment will be initiated within seven (7) days from flare-up onset and continue for six (6) weeks, with an additional six (6) weeks of follow-up. Efficacy and safety endpoints include imaging endpoints assessing for new bone formation, clinical measures of physical function and patient-reported outcomes. Patients who complete this study are eligible for an open label extension trial during which palovarotene would be administered for subsequent flare-ups. The company expects to complete the Phase 2 trial in 2015.

“The opening of the clinical trial site is welcome news for the European patient community who are eager to participate in research toward finding a potential treatment option for this
devastating disease,” said Antoine Lagoutte, IFOPA IPC member and officer of FOP France, the national patient organization for FOP.

For more information and answers to frequently asked questions, visit www.clementiapharma.com.

About Fibrodysplasia Ossificans Progressiva (FOP)
FOP is a rare, severely disabling congenital myopathy characterized by painful, recurrent episodes of soft tissue swelling (flare-ups) that result in new, abnormal bone formation in muscles, tendons, and ligaments. Flare-ups begin early in life and may occur spontaneously or after soft tissue trauma, vaccinations, or influenza infections. Recurrent flare-ups progressively restrict movement by locking joints, leading to cumulative loss of function and disability. FOP is caused by a point mutation in the ALK2/BMP type I receptor; the mutation results in over-activity of the receptor. Virtually all known patients have the same point mutation and have congenital malformations of the big toes at birth. FOP is thought to affect less than one individual for every million lives.

About Palovarotene
Palovarotene is a retinoic acid receptor gamma agonist in-licensed from Roche Pharmaceuticals, where it was previously evaluated in more than 800 individuals including healthy volunteers and patients with chronic obstructive pulmonary disease. Palovarotene has been shown to block bone formation in a variety of mouse models of FOP and is being investigated as a potential treatment for FOP.

About Clementia Pharmaceuticals Inc.
Clementia is a privately held, clinical-stage biopharmaceutical company focused on developing and commercializing innovative therapies for people living with rare diseases. The company is advancing a novel retinoic acid receptor gamma agonist to address diseases of heterotopic ossification, including fibrodysplasia ossificans progressiva. For more information, please visit www.clementiapharma.com.

Investor/Media Contact:
Smitha Dwarakanath
SmithSolve LLC
973-442-1555 ext. 122

Source: Clementia Pharmaceuticals, Inc.

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