Clementia Pharmaceuticals Announces Open-Label Phase 2 Extension Study of Palovarotene in Patients with Fibrodysplasia Ossificans Progressiva

MONTREAL, CANADA, OCTOBER 27, 2014 – Clementia Pharmaceuticals, Inc. announced today the initiation of a Phase 2 extension study of palovarotene in patients with fibrodysplasia ossificans progressiva (FOP), 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 multi-center, open-label extension study is open only to patients who complete Clementia’s 12-week, randomized, double-blind, placebo-controlled, Phase 2 study of palovarotene, an investigational retinoic acid receptor gamma agonist, in FOP. The extension study is designed to evaluate the long-term safety and efficacy of palovarotene treatment in FOP patients. There is no placebo arm in the extension study, and all patients will be treated with palovarotene.

“Continuity of care is a high priority for patients with FOP because the disease is progressive and debilitating, with unpredictable flare-ups,” said Edward Hsiao, M.D., Ph.D., Assistant Professor at University of California San Francisco (UCSF) School of Medicine and one of the principal investigators in the extension study. “The extension trial provides access to palovarotene if patients experience flare-ups over the ensuing 12 months.”

“Providing continued access to palovarotene through this extension study is important for those patients who agreed to participate in the previous trial, which included the possibility of receiving either placebo or palovarotene treatment at the time of a flare-up,” commented Donna Grogan, MD, Chief Medical Officer of Clementia. “We are focused on understanding the role of palovarotene as a potential treatment for FOP, and on understanding the long-term effects of treatment. This study will advance our knowledge base in this area.”

For more information and answers to frequently asked questions, please visit www.clementiapharma.com. Additionally, details on the extension study will be available at www.clinicaltrials.gov (using the search terms, “palovarotene and extension study”) as soon as the extension study is listed.

About Fibrodysplasia Ossificans Progressiva (FOP)

FOP is a rare, severely disabling disease 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.

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